Retrophin, Inc.
Form 10-K
March 28, 2014

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

FORM 10-K

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

For the Fiscal Year Ended December 31, 2013

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

Commission File Number: 000-53293

RETROPHIN, INC.
(Exact Name of Registrant as specified in its Charter)

Delaware	27-4842691
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
777 Third Avenue, 22nd Floor, New York, NY	10017
(Address of Principal Executive Offices)	(Zip code)

(646) 837-5863
(Registrant's telephone number, including area code)

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

Title of each class	Name of exchange on which registered
Common Stock, par value $0.0001 per share	The NASDAQ Global Market

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

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

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

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the

preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). þ Yes ¨ No

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

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

Large Accelerated Filer ¨	Accelerated Filer ¨
Non-Accelerated Filer ¨	Smaller Reporting Company þ

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

State the aggregate market value of the voting and non-voting common equity held by non-affiliates computed by reference to the price at which the common equity was last sold, or the average bid and asked price of such common equity, as of the last business day of the registrant's most recently completed second fiscal quarter. $41,994,436.

The number of shares of outstanding common stock, par value $0.0001 per share, of the Registrant as of March 19, 2014 was 24,262,716.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement, to be filed with the Securities and Exchange Commission within 120 days after the end of the registrant's fiscal year covered by this Annual Report on Form 10-K, with respect to the Annual Meeting of Stockholders to be held on May 9, 2014, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

Certain information contained in this Annual Report on Form 10-K of Retrophin, Inc., a Delaware corporation (the "Company") include forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The statements herein which are not historical reflect our current expectations and projections about the Company's future results, performance, liquidity, financial condition, prospects and opportunities and are based upon information currently available to the Company and 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 produce, market and generate sales of our products;
- our ability to develop, acquire and/or introduce new products;
- our projected future sales, profitability and other financial metrics;
- our future financing plans;
- our 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 the Company's operations. Actual events or results may differ materially from those discussed in forward-looking statements as a result of various factors, including, without limitation, the risks outlined under "Risk Factors" and matters described in this Annual Report generally. In light of these risks and uncertainties, there can be no assurance that the forward-looking statements contained in this Annual Report will in fact occur. Potential investors should not place undue reliance on any forward-looking statements. Except as expressly required by the federal securities laws, there is no undertaking to publicly update or revise any forward-looking statements, whether as a result of new information, future events, changed circumstances or any other reason.

Potential investors should not place undue reliance on any forward-looking statements. Except as expressly required by the federal securities laws, there is no undertaking to publicly update or revise any forward-looking statements,

whether as a result of new information, future events, changed circumstances or any other reason.

The specific discussions in this Annual Report about the Company include financial projections and future estimates and expectations about the Company's business. The projections, estimates and expectations are presented in this Annual Report only as a guide about future possibilities and do not represent actual amounts or assured events. All the projections and estimates are based exclusively on the Company management's own assessment of 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 the Company's projections, estimates or expectations.

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PART I

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

Item 1. Business

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

Overview

We are a fully integrated biopharmaceutical company focused on the development, acquisition and commercialization of therapies for the treatment of serious, catastrophic or rare diseases. We regularly evaluate and, where appropriate, act on opportunities to expand our pipeline through licenses and acquisitions of products in areas that will serve patients with serious, catastrophic or rare diseases and that we believe offer attractive growth characteristics.As a result of our acquisition of Manchester Pharmaceuticals, LLC, we sell Chenodal for the treatment of gallstones and Vecamyl for the treatment of hypertension. 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, or IS, and Nephrotic Syndrome, or NS. 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, or DMD.

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. We have filed an application for orphan drug designation from the FDA for RE-024 and sparsentan and plan to seek orphan drug designations from the FDA for RE-034 and Syntocinon. 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 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.

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 drugs approved by the United States Food and Drug Administration, or FDA, 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 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 plan to provide support to investigator studies of Syntocinon for the treatment of Autism Spectrum Disorders.

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- RE-034 (Tetracosactide Zinc).

  - RE-034 in Infantile Spasms ("IS"). 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 adrenocoricotropic hormone ("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 ("NS"). 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 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 pantothenate kinase-associated neurodegeneration ("PKAN"). 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. We intend to file a Investigational New Drug application, or IND, with the FDA for RE-024, so that we will be able to initiate a Company-sponsored Phase 1 clinical trial of RE-024. We expect that the first patients will be treated with RE-024 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 focal segmental glomerulosclerosis ("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.

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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.

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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 Pharmaceutical Corporation, or 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 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 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.

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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 plan to provide support to investigator studies of Syntocinon for the treatment of Autism Spectrum Disorders in.

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. We plan to submit an IND for RE-034 for the treatment of IS and NS to the FDA.

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.

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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. We intend to file an IND with the FDA for RE-024, so that we will be able to initiate a Company-sponsored Phase 1 clinical trial of RE-024. We expect that the first patients will be treated with RE-024 in early 2014 under an emergency IND.

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.

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.

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Chenodal™ (chenodiol tablets)

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

Chenodiol administration is known to reduce biliary cholesterol and the dissolution of radiolucent gallstones through suppression of hepatic synthesis of cholesterol, cholic acid and deoxycholic acid in the bile pool. Chenodiol was approved in 1983 for the management of gallstones and discontinued due to lack of commercial success. In 2009 Manchester Pharmaceuticals was granted approval of Chenodal for the treatment of gallstones and subsequently obtained Orphan Drug Designation for the treatment of cerebrotendinous xanthomatosis (CTX) in 2010.

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

Chenodal in Cerebrotendinous Xanthomatosis

We intend to obtain FDA approval of Chenodal for the treatment of cerebrotendinous xanthomatosis, a rare autosomal recessive lipid storage disease for which there are no FDA approved treatments. Pathogenesis of CTX involves deficiency of the enzyme 27-hydroxylase (CYP27A1), a rate-limiting enzyme in the synthesis of primary bile acids, including chenodeoxycholic acid, from cholesterol. The disruption of primary bile acid synthesis in CTX leads to toxic accumulation of cholesterol and cholestanol in most tissues. Most patients present with intractable diarrhea, premature cataracts, tendon xanthomas, atherosclerosis, and cardiovascular disease in childhood and adolescence. Neurological manifestations of the disease, including dementia and cognitive deficiencies, emerge during late adolescence and adulthood. Oral administration of chenodeoxycholic acid has been shown to normalize primary bile acid synthesis in patients with CTX. Chenodal has been used off-label as the mainstay of treatment for cerebrotendinous xanthomatosis since its clinical discovery in 1975. The epidemiology of CTX is not well understood; according to Orphanet, the prevalence of CTX is estimated to be 1-9/100,000.

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In 1984, a long term open-label study of chenodeoxycholic acid in patients with CTX showed correction of abnormal bile acid metabolism and resolution of symptoms. Patients received 750 mg of chenodeoxycholic acid daily for at least one year. This study showed that long term treatment with chenodeoxycholic acid improved neurologic function in some patients and reduced mean plasma cholestanol levels by a factor of three. This study suggests that treatment with chenodeoxycholic acid could correct the biochemical abnormalities and arrest and possibly reverse progression of cerebrotendinous xanthomatosis.

In 2001, The University Siena reported a follow-up of five adult CTX patients treated with chenodeoxycholic acid for eleven years. Four of five patients in this cohort presented neurological symptoms of the disease. Patients were maintained on 750 mg of chenodeoxycholic acid daily for at least eleven years. Chenodexoycholic acid therapy stabilized the clinical manifestations of CTX in these patients suggesting that patients would benefit from early diagnosis and treatment.

In 1998, The University Hospital Nijmegen reported the clinical effects of chenodeoxycholic acid treatment in children over the course of five years. All patients presented with chronic diarrhea, delay in motor function, and juvenile cataracts prior to treatment. Upon treatment with chenodeoxycholic acid, patients experienced immediate cessation of diarrhea and stabilization of cognitive function after one year of treatment. This study showed that early treatment of children with CTX is beneficial by slowing or halting the progression of the disease.

In 2013, the Chaim Sheba Medical Center reported the long-term neurological outcome conducted a cross sectional observational study of eighteen CTX patients. All patients treated with chenodeoxycholic acid experienced symptomatic improvement and neurological stabilization. It is suggested that early diagnosis and treatment initiation could help to halt disease progression.

Chenodal in Other Indications

We also plan to develop Chenodal for other indications, which may include the potential treatment of patients with primary biliary cirrhosis.

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Manufacturing

We intend to continue the relationship with Nexgen Pharma for the manufacture of Chenodal and Vecamyl and maintain distribution of the products through Dohmen Company.

Competition

Clinical studies of Cholic Acid as a potential treatment for inborn errors of bile acid synthesis, sponsored Asklepion Pharmaceuticals, have been reported. Intercept Pharmaceuticals is currently conducting clinical trials of its FXR agonist, obeticholic acid, in primary biliary cirrhosis, portal hypertension, NASH, and bile acid diarrhea. Additionally, we believe that Interecept Pharmaceuticals is working on a possible treatment of inborn errors of bile acid synthesis using FXR agonists.

Vecamyl (mecamylamine hydrochloride)

Vecamyl is an oral nicotinic parasympathetic ganglionic blocker indicated for the treatment of moderate to severe hypertension and uncomplicated cases of malignant hypertension. Mecamylamine was one of first orally available antihypertensive agents introduced in 1954 by Merck & Co., Inc. Oral mecamylamine is rapidly absorbed in the gastrointestinal tract and has a rapid onset and long duration of action. The antihypertensive effects of mecamylamine is a result of its blockade of impulse transmission at sympathetic ganglia due to competition of nicotinic acetylcholine receptors and stabilization of postsynaptic membranes against excitation by acetylcholine resulting in dilation of blood vessels resulting in reduced blood pressure.

Mecamylamine was removed from the market in 1996 for commercial reasons. In 2000, Manchester reintroduced 2.5-mg mecamylamine in the U.S. market for the treatment of hypertension. On March 26, 2014 we acquired the rights to sell the only approved form of mecamylamine in the U.S. We intend to maintain the supply of mecamylamine to the U.S. market.

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Licenses and Royalties

Novartis License

On December 12, 2013, we entered into an agreement with Novartis Pharma AG and Novartis AG pursuant to which Novartis and Novartis AG agreed to grant us an exclusive, perpetual, and royalty-bearing license for manufacture, development and commercialization of Syntocinon and related intranasal products in the United States. Under the license, Novartis Pharma AG 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 pay annual maintenance fees $3 million after each anniversary until there has been regulatory approval, up to $34 million in developmental milestones for the first indication and up to $32 million in developmental milestones for the second indication. Should we commercialize Syntocinon, we will be obligated to pay Novartis and Novartis AG a 10%-20% royalty of net sales of such products.

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 Pharmaceuticals Incorporated (“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. Through December 31, 2013, we made payments to Ligand of $2.5 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, 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 paid Dr. Weg an upfront fee, which amount included $1,000,000 in payments prior to and upon the execution of the Weg License Agreement. We are also obligated to pay Dr. Weg certain maintenance and sublicensing fees, as well as 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.

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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 had been paid as of December 31, 2013. 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 $1.54 million in fees to UCSD on a specified timeline, of which $0 has been paid as of December 31, 2013. 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.

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.

On March 18, 2014, US patent 8,673,883 was issued covering composition and use of RE-024 and its derivatives.

We have secured a license from Ligand and Bristol-Myers Squibb to a U.S. issued 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.

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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.

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.

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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.

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 we do 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.

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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 of patients 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.

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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.

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.

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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.

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.

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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 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.

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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 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.

Research and Development

Our expenditures for research and development activities were $7,084,009 and $662,502 during the years ended December 31, 2013 and 2012, respectively. These expenditures represent costs incurred to conduct research of our proprietary product candidates, including 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.

Employees

As of December 31, 2013, we employed 26 employees, each of whom was full-time, and we engaged five consultants who 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 35 additional full-time employees devoted to development activities and up to 15 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.

Organizational Background

We were incorporated as Desert Gateway, Inc., or Desert Gateway, an Oklahoma corporation, on February 8, 2008. Desert Gateway was originally a wholly owned subsidiary of American Merchant Data Services, Inc., or 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.

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On December 12, 2012, Desert Gateway completed a merger, in which former Retrophin, our predecessor, 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.

Item 1A. Risk Factors

Our business, as well as our common stock, are highly speculative in nature and involve a high degree of risk. Our securities should be purchased only by persons who can afford to lose their entire investment. 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(s), 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 December 31, 2013, we have incurred net losses of approximately $67.4 million and negative cash flows from operating activities of approximately $21.1 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 $33.8 million for the year ended December 31, 2013. As of December 31, 2013, we had an accumulated deficit of $67.4 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 and begin clinical trials 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;

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- 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.

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 of this filing, 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.

We face the problems, expenses, difficulties, complications and delays, many of which are beyond our control, associated with any business in its early stages and has no operating history on which an evaluation of our prospects can be made. Such prospects should be considered in light of the risks, expenses and difficulties frequently encountered in the establishment of a business in a new industry, characterized by a number of market entrants and intense competition, and in the shift from development to commercialization of new products based on innovative technologies. 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.

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

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

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

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

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

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

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

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

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

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Economic pressure on state budgets may result in states increasingly seeking to achieve budget savings through mechanisms that limit coverage or payment for our products. State Medicaid programs are increasingly requesting manufacturers to pay supplemental rebates and requiring prior authorization by the state program for use of any drug for which supplemental rebates are not being paid. Managed care organizations continue to seek price discounts and, in some cases, to impose restrictions on the coverage of particular drugs. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding constraint on prices and reimbursement for our products.

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

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

The illegal distribution and sale by third parties of counterfeit versions of our products or stolen products could have a negative impact on our reputation and business.

Third parties might illegally distribute and sell counterfeit versions of our products, which do not meet our rigorous manufacturing and testing standards. A patient who receives a counterfeit drug may be at risk for a number of dangerous health consequences. Our reputation and business could suffer harm as a result of counterfeit drugs sold under our brand name. In addition, thefts of inventory at warehouses, plants or while in transit, which are not properly stored and which are sold through unauthorized channels, could adversely impact patient safety, our reputation and our business.

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.

In connection with our acquisition of Manchester we granted the sellers a security interest in the equity and all of the assets of Manchester, including the rights to Chenodal and Vecamyl.

In connection with our acquisition of Manchester, we issued the sellers a $33 million senior secured note and granted the sellers a security interest in the equity and substantially all of the assets of Manchester, including the rights to Chenodal and Vecamyl, to secure our obligations under the secured note. The secured note is subject to acceleration and an increased interest rate upon the happening of customary events of default, including the failure to make timely payments of principal. The sellers would have the right to realize on the secured equity or assets to satisfy amounts owed under the note in an event of default. If the sellers were to realize on these secured assets, especially our rights to Chenodal and Vecamyl, it would have a material adverse effect on our business.

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, the year ended December 31, 2012 and the year ended December 31, 2013, we concluded that a material weakness existed in internal control over financial reporting and our disclosure controls. Specifically, our management concluded as of December 31, 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 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.

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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, the year ended December 31, 2012 and the year ended December 31, 2013 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.

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.

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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.

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.

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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 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.

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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.

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.

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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 turing 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;
- 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.

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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;

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- 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.

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.

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Our investments may be risky or highly speculative, and we may not realize gains from our investments.

We may acquire equity securities for cash through our equity investment plan. Our goal is ultimately to dispose of such equity interests and realize gains upon our disposition of such interests. However, the equity interests we receive may not appreciate in value and, in fact, may decline in value. Accordingly, we may not be able to realize gains from our equity interests, and any gains that we do realize on the disposition of any equity interests may not be sufficient to offset any other losses we experience.

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.

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;

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- 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.

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.

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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.

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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 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 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 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.

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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 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.

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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;

- 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.

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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.

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;

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- 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 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.

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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;

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

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

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

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

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

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

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

Our 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.

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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 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;

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- 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 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.

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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 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.

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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 3,204,016 shares of our common stock, or approximately 13.06% 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.

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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.

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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Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

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 6,300 square feet of office space located in San Diego, CA.

Item 3. Legal Proceedings

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

Item 4. Mine Safety Disclosures

Not applicable.

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PART II

Item 5. Market For Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

We are a reporting company under the Exchange Act, and our public filings can be accessed at www.sec.gov. Our common stock is listed for quotation on the NASDAQ Global Market under the trading symbol "RTRX" ("DGTE" prior to December 17, 2012). Prior to January 10, 2014, our common stock was listed for quotation on the OTC QB market.

The following table sets forth the high and low bid prices for our common stock for each full quarterly period within the two most recent fiscal years 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 March 19, 2014)	$ 19.73	$ 7.89
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

As of March 19, 2014, we had approximately 262 holders of record of our common stock.

Dividends

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

Recent Sales of Unregistered Securities and Use of Proceeds

There were no sales of securities by the Company during the period covered by this Annual Report that have not previously been reported.

Purchases of Equity Securities by the Issuer

The following table summarizes the repurchases of our equity securities during the three month period ended December 31, 2013:

Period	Total number of shares purchased		Average price paid per share	Total number of shares purchased as part of publicly announced plans or programs	Approximate dollar value of shares that may yet be purchased under the share repurchase plan
October 1 to 31, 2013	-		-	-	-
November 1 to 30, 2013	2,340	*	$ 6.76	-	-
December 1 to 31, 2013	222,851	*	$ 7.38	-	-
Total	225,191	*		-	-

* Such shares were purchased on the open market pursuant to a stock repurchase plan approved by the Company's board of directors.

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Item 6. Selected Financial Data

Not applicable.

Item 7. 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.

Overview

We are a fully integrated 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.

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Plan of Operation

Our plan of operation for the years ending December 31, 2014 and 2015 is to continue implementing our business strategy, including the clinical development of our four drug candidates, focusing primarily on the development of Sparsentan for the treatment of FSGS, RE-024 for the treatment of PKAN, RE-034 for the treatment of Infantile Spasms and Nephrotic Syndrome, and Syntocinon for the treatment of Schizophrenia and possibly Autism. We also intend to expand our drug product portfolio by acquiring additional drugs for marketing or development. During the next 12 months, our principal expenditures may include the following:

- We expect to incur operating expenses, including expanded research and development and general and administrative expenses
- We expect to incur product development expenses, including the costs incurred with respect to applications to conduct clinical trials in the United States for our four products and the costs of ongoing and planned clinical trials. We expect to conduct multiple clinical trials for our assets, including a Phase 2 clinical trial for Sparsentan for the treatment of FSGS, a Phase 1 clinical trial for RE-024 for the treatment of PKAN, Phase 1 and 3 trials for RE-034 for the treatment of Infantile Spasms and Nephrotic Syndrome, and a Phase 2 trial for Syntocinon for the treatment of Schizophrenia. The expected costs associated with these trials amount to approximately $12.1 million through March 2015.
- We plan to re-introduce Syntocinon to the market for its original indication for aiding milk let-down. The re-launch is expected to cost approximately $3.2 million, which includes the contracting of a salesforce, market analysis, marketing and physician outreach and other related launch expenses.
- We plan to incur approximately $8.5 million in pre-clinical expenses in non-human studies to confirm safety and efficacy of our assets. Such amount includes sponsored research to which we have committed to.

As part of our planned expansion, we expect to aggressively increase our work staff by hiring up to 75 full-time employees by the end of 2014 for research and development activities and general and administrative activities. Total personnel costs through March 2015 are expected to be approximately $16.7 million, with $10.2 million in research and development and $6.5 million in general and administrative. We expect to incur approximately $2.2 million in expenses related to operating as a public entity. We will also continue to rely on outside counsel until we are ready to hire internal counsel. We also will incur $4 million in license maintenance fees due to Novartis ($3 million) for the Syntocinon license, and to Dr. Weg ($1 million) for the license of a product for the treatment of central nervous system disorders. In addition, we intend to use clinical research organizations and third parties to perform our clinical studies and manufacturing. At our current and desired pace of commercialization and clinical development of our drugs, through March 2015, we cannot assure you these amounts will be sufficient to fund our operations over the course of the next two years and we may need to expend significantly greater amounts to accomplish our goals.

Research and Development Projects

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.

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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 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 plan to provide support to investigator studies of Syntocinon for the treatment of Autism Spectrum Disorders.

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.

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 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.

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 o f 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.

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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. We intend to file an IND with the FDA for RE-024, so that we will be able to initiate a Company-sponsored Phase 1 clinical trial of RE-024. We expect that the first patients will be treated with RE-024 in early 2014 under an emergency IND.

RE-001

RE-001 is a recombinant, modified form of utrophin, a protein similar to the dystrophin protein that is missing in the muscles of DMD patients. RE-001 is a preclinical investigational program. Production scale-up the molecule is underway, and in vivo evaluation of clinical trial quality material may begin in 2014.

Financial Overview

The following discussion summarizes the key factors our management believes are necessary for an understanding of our financial statements as of December 31, 2013.

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 Costs

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, regulatory 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, 2013 and 2012 and for the period from March 11, 2011 (inception) through December 31, 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.

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	December 31, 2013	December 31, 2012	For the period from March 11, 2011 through December 31, 2013
External service provider costs:			
Sparsentan	$ 2,443,273	$ 297,833	$ 2,619,723
RE-024	1,548,957	124,635	1,673,592
Weg license in process R&D	1,000,000	-	1,000,000
Syntocinon	250,540	-	250,540
RE-034	230,279	-	230,279
General	159,080	240,034	493,569
Other product candidates	117,771	-	376,710
Total external service provider costs:	5,749,900	662,502	6,644,413
Internal personnel costs:	1,334,109	-	1,334,109
Total research and development	$ 7,084,009	$ 662,502	$ 7,978,522

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 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 compensation, professional fees, 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.

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As consideration for the license, we paid to Novartis and Novartis AG a $5 million upfront fee and are required to pay annual maintenance fees of $3 million after each anniversary until there has been regulatory approval, up to $34 million in developmental milestones for the first indication and up to $32 million in developmental milestones for the second indication. Should we commercialize Syntocinon, we will be obligated to pay Novartis and Novartis AG a 10%-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 December 31, 2013, we made payments to Ligand of $2.3 million under the license agreement.

Weg License Agreement

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 paid Dr. Weg an upfront fee, which amount included $1,000,000 in payments prior to and upon the execution of the Weg License Agreement. We are also obligated to pay Dr. Weg certain maintenance and sublicensing fees, as well as certain royalties on sales of FDA-approved products. Through December 31, 2013, we made payments to Dr. Weg of $1,000,000 under the Weg License Agreement.

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

We believe our ability to continue operations depends on our ability to raise capital. Our future depends on the costs, timing, and outcome of regulatory reviews of our product candidates and the costs of commercialization activities, including product marketing, sales and distribution. These conditions raise substantial doubt about our 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 we be unable to continue as a going concern.

Year ended December 31, 2013 Compared to the Year ended December 31, 2012

Revenue. We had no revenues for the year ended December 31, 2013 and 2012.

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Operating Expenses. Our operating expenses for the year ended December 31, 2013 were $24 million compared to $30.3 million for the year ended December 31, 2012 which represents a decrease of $6.3 million, or 21%. The expense decrease was principally attributable to a decrease in our compensation and related costs in the amount of $13.9 million, a decrease in our professional fees in the amount of $1.7 million, a decrease in our technology license fees of $1.5 million, offset by an increase in our research and development expenses in the amount of $6.4 million and an increase in other selling, general and administrative costs in the amount of $4.3 million. Our decrease in compensation and related costs of $13.9 million is a result of a decrease in stock based compensation of $14.8 million offset by an increase in cash compensation of $0.9 million. Our decrease in professional fees of $1.7 million is a result of a decrease in stock based compensation of $3.2 million offset by an increase in legal and related cash expenditures of $4.9 million. Our increase in research and development expenses of $6.4 million is a result of an increase in our internal personnel costs of $1.3 million and an increase in our external service provider costs of $5.1 million. Our increase in other selling, general, and administrative costs of $4.3 million is a result of an increase in settlement charges of $2.6 million and an increase in cash expenditures of $1.7. Our increase in cash expenditures of approximately $1.7 million is due to an increase in costs associated with business development expenses of $.9 million, an increase in travel and related expenses of $.25 million, an increase in recruitment fees of $.25 million, an increase in rent expense of $.3 million.

Other Expenses. Other expense for the year ended December 31, 2013 was $9.8 million compared to other expense of $.09 million for the year ended December 31, 2012, which represents an increase of $9.7 million. The increase was primarily attributable to the change in fair value of derivative financial instruments of $10.1 million and a decrease in interest expense of $.04 million, offset by a realized gain on the sale of marketable securities of $.37 million Included in other income is registration payment income of $0.36 million relating to a waiver we received for previous liquidated damages and expense of $0.36 million 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 year ended December 31, 2013 was $33.8 million compared to $30.3 million for the year ended December 31, 2012.

Impact of Inflation

The impact of inflation upon our revenue and income/(loss) from continuing operations during each of the past two fiscal years has not been material to our financial position or results of operations for those years because we have no products for sale and do not maintain any inventories whose costs are affected by inflation.

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 December 31, 2013, we had raised approximately $36.1 million through capital contributions and notes payable from Retrophin shareholders and related parties.

Since inception through December 31, 2013, we have incurred a net loss of approximately $67 million, including stock-based compensation charge of approximately $29 million for the period from March 11, 2011 (inception) to December 31, 2013. At December 31, 2013, we had a working capital deficit of approximately $28 million; however, the working capital deficit includes a derivative liability of approximately $25 million for warrants issued in financing transactions. Our accumulated deficit amounted to $67 million at December 31, 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 December 31, 2013 and December 31, 2012, our stockholders' deficit was $18 million and $3.4 million, respectively. Our net loss from operations for the years ended December 31, 2013 and 2012, and for the period March 11, 2011 (inception) through December 31, 2013 were approximately $33.8 million, $30.3 million and $67.4 million, respectively. Net cash used in operating activities were $17.6 million, $2.7 million and $21.1 million for the years ended December 31, 2013 and 2012, and for the period March 11, 2011 (inception) through December 31, 2013, respectively. Operations since inception have been funded entirely with the proceeds from equity and debt financings. As of December 31, 2013, we had cash of $6.0 million. 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.

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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 $2,284,511 of these settlements during the year ended December 31, 2013. 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 year ended December 31, 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.

On January 9, 2014, we completed a public offering of 4,705,882 shares of common stock at a price of $8.50 per share. We received net proceeds from the offering of $37,399,997, after deducting the underwriting discount and other estimated offering expenses.

On March 26, 2014, we acquired all of the assets and liabilities of Manchester Pharmaceuticals LLC, which we refer to as Manchester. In consideration for such acquisition, we agreed to pay to the sellers aggregate consideration of $62.5 million, plus an additional contingent payments based on net sales of the Chenodal and Vecamyl products.

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 11, 2013, we entered into an agreement with University of California, San Diego to provide an unrestricted charitable gift in support of translation psychiatric research at University of California, San Diego. The total charitable contribution in the amount of $530,000 will be paid in three contributions over a one-year period in 2014.

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 $1.54 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.

SickKids

On July 12, 2013, we agreed to sponsor a study with The Hospital for Sick Children ("SickKids"). We agreed to fund the study by providing CDN $750,000 over a three-year term.

University of Michigan

On October 2, 2013, we agreed to provide a grant to the University of Michigan in the amount of $2,000,000, payable in equal quarterly installments over a two-year period.

License Agreement Obligations

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 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. As consideration for the license, we are required to make substantial payments upon the achievement of certain milestones totaling up to $106.9 million, payable upon the achievement of certain milestones. Should we commercialize RE-021 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 sublicense any of these compounds to a third party, Retrophin shall pay to Ligand a percentage of the financial consideration in addition to the milestone and royalty payments required.

Novartis License Agreement

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. As consideration for the license, we paid to Novartis and Novartis AG a $5 million upfront fee and are required to pay annual maintenance fees of $3 million after each anniversary until there has been regulatory approval, up to $34 million in developmental milestones for the first indication and up to $32 million in developmental milestones for the second indication. Should the Company commercialize the Product, it will be obligated to pay Novartis and Novartis AG a 10%-20% royalty on net sales of such products.

Weg License Agreement

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.

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Funding Requirements

We believe that our available cash and short-term investments as of the date of this filing will not 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 entered into an agreement for the acquisition of Manchester LLC (“Manchester”), a privately held pharmaceutical company with two products on market generating approximately $5 million in revenue and positive cash flows. In accordance with the agreement, we paid $29.5 upon closing, of which $3.2 million was paid by Retrophin Therapeutics International LLC, a newly formed indirect wholly owned subsidiary, for rights of product sales outside of the United States. We will be required to pay an additional $33 million in three equal installments of $11 million within three, six, and nine months after the closing of the transaction.

In order to fund the Manchester acquisition and its planned development activities, we intend to raise additional capital through additional public offerings, private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, foundations and government agencies. Such financing may change our position on our ability to continue as a going concern.

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 our 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 we be unable to continue as a going concern.

Cash Flows

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

	For the year ended December 31,		For the period from March 11, 2011 (inception) through December 31, 2013
	2013	2012	
Net cash used in operating activities	$(17,589,168)	$(2,736,739)	$ (21,111,654)

Net cash used in investing activities	(5,406,425)	(1,699,593)	(7,118,890)
Net cash provided by financing activities	28,981,512	4,437,667	34,227,851
Net increase in cash	5,985,919	1,335	5,997,307
Cash, beginning of period	11,388	10,053	-
Cash, end of period	$5,997,307	$11,388	$ 5,997,307

Cash Flows from Operating Activities

Operating activities used approximately $17.6 million of cash during the year ended December 31, 2013 compared $2.7 million for the year ended December 31, 2012. The increase of $14.9 million was the result of an increase in net loss of $3.5 million, decrease in non-cash charges of $9.8 million, and a net change in operating assets and liabilities of $1.6 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.

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Cash Flows from Investing Activities

Cash used in investing activities for the year ended December 31, 2013 was $5.4 million compared to $1.7 million for the year ended December 31, 2012. The increase of $3.7 million was primarily the result of an increase in the purchase of intangible assets of $4.3 million, a purchase of marketable securities of $4.1 million, cover of securities sold, not yet purchased for $2.9 million, repayment of a technology license liability of $1.3 million, an increase in the purchase of fixed assets of $0.1 million, and an increase in our security deposit of $0.1 million, offset by the proceeds from the sale of marketable securities $4.4 million, proceeds from securities sold, not yet purchased for $4.2 million, a decrease in payment made on behalf of affiliate $0.1 million, and a decrease in repayments made on loans to stockholder of $0.4 million.

Cash Flows from Financing Activities

For the year ended December 31, 2013, cash provided by financing activities was $29 million compared to $4.4 million during the year ended December 31, 2012. The increase of $24.5 million was primarily a result of an increase of $27.5 million in proceeds received from issuance of common stock, offset by a purchase of treasury stock $1 million and a decrease in activities associated with related party notes payable of $2 million.

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 January 4, 2013, we entered into an agreement with Roth Capital Partners to act as our exclusive placement agent in connection with our February private placement. In connection with the agreement, we issued warrants to purchase up to an aggregate of 319,823 shares of our 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 we issue 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, we recorded a liability of $901,767 to derivative financial instruments in our 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 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 $2,816,313 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.

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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.

Other 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.

Effective May 20, 2013, we entered into an employment agreement with Marc L. Panoff (the “Panoff Employment Agreement”) pursuant to which Mr. Panoff was appointed as our Chief Financial Officer and Chief Accounting Officer. 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 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.

Subsequent Events

On February 28, 2014, we amended our lease agreement for our offices located in Carlsbad, CA. We increased our Carlsbad offices by approximately 3,800 square feet of office space for approximately $110,000 of additional annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes under a lease agreement expiring in June 2017.

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Public Offering

On January 9, 2014, we completed a public offering of 4,705,882 shares of common stock at a price of $8.50 per share. We received net proceeds from the offering of $37,399,997, after deducting the underwriting discount and other estimated offering expenses of $2,600,000. Our common stock is listed on the Nasdaq Global Market under the symbol “RTRX.”

Exclusivity Agreement

On March 26, 2014, we acquired 100% of the outstanding membership interests of Manchester Pharmaceuticals, LLC (“Manchester” or “acquiree”), a privately-held specialty pharmaceutical company that focuses on treatments for rare diseases. The acquisition of Manchester expands our ability to address the special needs of patients with ultra-rare diseases.

Under the terms of the agreement, we paid $29.5 million upon closing, of which $3.2 million was paid by Retrophin Therapeutics International LLC, a newly formed indirect wholly owned subsidiary, for rights of product sales outside of the United States. We entered into a promissory note with Manchester principals for $33 million to be paid in three equal installments of $11 million within three, six, and nine months after closing. Additional contingent payments will be made based on product sales. We expect to raise additional funds through a public equity offering, a private equity offering, and/or debt financing to satisfy its short term obligations.

The financial statements of the acquiree are not practicable to prepare at the time of filing due to the acquiree being privately held and not maintaining financial statements in accordance with U.S. GAAP. The initial accounting for the business combination is not yet complete and we are still performing procedures to determine the appropriate accounting. As such, we are unable to make the following disclosures, (i) pro forma data, (ii) purchase price allocation, (iii) expenses of the acquisition, and (iv) revenue and earnings of the acquiree since the acquisition date.

Exercise of Warrants

Subsequent to year end, an aggregate of 798,391 warrants were exercised for a total of $3,830,316 in cash received by the Company.

Covered Short Sales

Subsequent to December 31, 2013, we purchased $1,019,456 in marketable securities of thirteen publicly traded companies to “cover” securities sold, not yet purchased held as of December 31, 2013. As of the date of this filing, we have realized a gain on securities sold, not yet purchased in the amount of $45,885.

Stock Repurchases

Subsequent to December 31, 2013, we repurchased 248,801 shares of its common stock for an aggregate purchase price of $2,257,336. We currently recognize such repurchased common stock as treasury stock.

Consulting Agreements

On January 14, 2014, we entered into an agreement with a consultant to serve as an advisor to us. We granted 14,000 shares of common stock to such consultant, payable as follows (i) 3,500 shares of common stock to be issued on April 1, 2014, (ii) 3,500 shares of common stock to be issued on July 1, 2014, (iii) 3,500 shares of common stock to be

issued on October 1, 2014, (iv) 3,500 shares of common stock to be issued on January 1, 2015. In addition, such consultant shall receive $12,500 per month. The agreement expires on January 13, 2015.

On February 14, 2014, we entered into an agreement with a consultant to serve as an advisor to us. We granted 66,000 shares of common stock to such consultant, payable as follows (i) 16,500 shares of common stock to be issued on March 31, 2014, (ii) 16,500 shares of common stock to be issued on June 30, 2014, (iii) 16,500 shares of common stock to be issued on September 30, 2014, (iv) 16,500 shares of common stock to be issued on December 31, 2014. In addition, such consultant shall receive $200,000 upon the execution of the agreement. The agreement expires on December 31, 2014.

On March 6, 2014, we entered into an agreement with a consultant to serve as an advisor to us. We granted 200,000 shares of common stock to such consultant. The agreement expires on December 31, 2014.

Bonus

On February 24, 2014, our Board of Directors approved an aggregate cash bonus pool of $1,100,000 to officers and employees of record as of December 31, 2013.

Employee Equity Issuance

Subsequent to year end, we issued 400,000 shares of restricted common stock to three officers and 1,210,000 options to purchase shares of our common stock to four officers and other employees.

Securities Sold, Not Yet Purchased

As of the date of this filing, the Company has $1,218,800 and $498,146 of securities sold, not yet purchased and unrealized loss related to securities sold, not yet purchased, respectively.

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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.

Restatement of Prior Quarters

In the fourth quarter of 2013, we discovered that certain warrants issued to a placement agent in connection with our February Private Placement was not recorded and certain expenses were overstated in our condensed consolidated financial statements for the first, second, and third quarters of 2013. The overstated expenses include costs related to the February 14, 2013 Private Placement Offering and costs associated with our consultants. The adjustments necessary to reflect such issuance were recorded in the fourth quarter of 2013.

The following table (in thousands) sets forth the effects of the restatement on affected items within our previously reported Consolidated Balance Sheets for the periods ended March 31, 2013, June 30, 2013 and September 30, 2013 had the adjustments been made in the corresponding quarters.

	March 31, 2013		June 30, 2013		September 30, 2013	
	As Reported	As Restated	As Reported	As Restated	As Reported	As Restated
Non-derivative liabilities	$1,575	$1,460	$4,229	$4,114	$4,935	$4,935
Derivative financial instruments, warrants	7,000	8,392	6,901	8,283	22,234	23,853
Total Liabilities	8,575	9,852	11,130	12,397	27,169	28,788
Additional paid in capital	34,773	33,621	34,946	33,794	48,652	47,500
Deficit accumulated during the development stage	(38,355)	(38,480)	(43,404)	(43,519)	(54,301)	(54,768)
Stockholder’s deficit	$(3,582)	$(4,859)	$(8,458)	$(9,725)	$(5,804)	$(7,423)

The following table (in thousands) sets forth the effects of the restatement on affected items within our previously reported Consolidated Statement of Operations for the three months ended March 31, 2013, June 30, 2013 and September 30, 2013 had the adjustments been made in the corresponding quarters.

	March 31, 2013		June 30, 2013		September 30, 2013	
	As Reported	As Restated	As Reported	As Restated	As Reported	As Restated
Operating loss	$(2,251)	$(1,886)	$(5,100)	$(4,985)	$(5,155)	$(5,155)
Non-operating loss	(2,492)	(2,982)	51	61	(5,743)	(5,980)
Net loss	(4,743)	(4,868)	(5,049)	(4,924)	(10,898)	(11,135)
Net loss per common share, basic and diluted	$(0.44)	$(0.46)	$(0.41)	$(0.40)	$(0.71)	$(0.72)

The following table (in thousands) sets forth the effects of the restatement on affected items within our previously reported Consolidated Statement of Operations for the six and nine months ended June 30, 2013 and September 30,

2013, respectively had the adjustments been made in the corresponding quarters.

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	June 30, 2013		September 30, 2013	
	As Reported	As Restated	As Reported	As Restated
Operating loss	$(7,351)	$(6,986)	$(7,351)	$(6,986)
Non-operating loss	(2,441)	(2,921)	(2,441)	(2,921)
Net Loss	$(9,792)	$(9,907)	$(9,792)	$(9,907)
Net loss per common share, basic and diluted	$(0.85)	$(0.86)	$(1.62)	$(1.65)

Restatement of Previously Issued Financial Statements for Additional Disclosures

We, while undergoing a review of our condensed consolidated financial statements for the three and six months periods ended June 30, 2013, commenced an evaluation of our accounting for a series of settlement agreements that we, along with certain related parties, entered into between April 2013 and June 2013. On September 13, 2013, we, under the authority of the board of directors, determined that these agreements should have been disclosed in the footnotes to our consolidated financial statements for the year ended December 31, 2012. Accordingly, we have restated the consolidated financial statements to include these disclosures.

We also determined that it’s our obligation to pay liquidated damages under a registration payment that we entered into in connection with a financing transaction completed on February 14, 2013, which required us 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, we restated the condensed consolidated financial statements for the quarter ended March 31, 2013 to disclose that we 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.

Marketable Securities

We account for marketable securities held as “available-for-sale” pursuant to ASC 320 Investments — Debt and Equity Securities (“ASC 320”). We classified 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 our investment policy as approved by our Board of Directors. Fair values of marketable securities are based on quoted market prices.

Based on our liquidity position, our CEO and CFO are authorized to make various investment transaction decisions for prudent investment of our excess funds. The ability to conduct investments is limited to our CEO and CFO. The current policy limits marketable securities investments with a maturity, credit quality, and concentration that is authorized only by our CEO and CFO.

Securities Sold, Not Yet Purchased

Securities sold, not yet purchased consist of marketable securities that we have sold short. In order to facilitate a short sale, we borrow the securities from another party and deliver the securities to the buyer. We will be required to "cover" its short sale in the future through the purchase of the security in the market at the prevailing market price and deliver it to the counterparty from which it borrowed. We are exposed to a loss to the extent that the security price increases during the time from when we borrowed the security to when we purchase it in the market to cover the short sale. Securities sold, not yet purchased are presented on the consolidated balance sheets with gains and losses reported in realized and unrealized gains on marketable securities on the consolidated statement of operations and comprehensive loss.

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.

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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 fiscal years ended December 31, 2013 and 2012, we had no accrued interest or penalties related to uncertain tax positions.

Long-Lived Assets

Long-lived assets, other than goodwill, are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of the assets might not be recoverable. Conditions that would necessitate an impairment assessment include a significant decline in the observable market value of an asset, a significant change in the extent or manner in which an asset is used, or any other significant adverse change that would indicate that the carrying amount of an asset or group of assets may not be recoverable. Application of alternative assumptions, such as changes in estimate of future cash flows, could produce significantly different results. Because of the significance of the judgments and estimation processes, it is likely that materially different amounts could be recorded if we used different assumptions or if the underlying circumstances were to change.

For long-lived assets used in operations, impairment losses are only recorded if the asset’s carrying amount is not recoverable through its undiscounted, probability-weighted future cash flows. We measure the impairment loss based on the difference between the carrying amount and estimated fair value.

Derivative Instruments

We do not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. We evaluate all of our 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, we calculate the fair value of the financial instruments using 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.

Registration Payment Arrangement

We 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”.

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Patents

We capitalize external cost, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. We expense cost associated with maintaining and defending patents subsequent to their issuance in the period incurred. We amortize patent cost once issued on a straight-line basis over the estimate useful lives of the patents. We 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. We account 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 our marketable securities available-for-sale and securities sold, not yet purchased, we use quoted prices in active markets.

In estimating the fair value of our derivative liabilities, we use the Binomial Lattice 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 Update ("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. We early adopted this guidance in the second quarter of 2013.

The FASB has issued ASU No. 2013-11, Income Taxes (Topic 740): Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists (a consensus of the FASB Emerging Issues Task Force). The amendments in this ASU state that an unrecognized tax benefit, or a

portion of an unrecognized tax benefit, should be presented in the financial statements as a reduction to a deferred tax asset for a net operating loss carryforward, a similar tax loss, or a tax credit carryforward, except as follows. To the extent a net operating loss carryforward, a similar tax loss, or a tax credit carryforward is not available at the reporting date under the tax law of the applicable jurisdiction to settle any additional income taxes that would result from the disallowance of a tax position or the tax law of the applicable jurisdiction does not require the entity to use, and the entity does not intend to use, the deferred tax asset for such purpose, the unrecognized tax benefit should be presented in the financial statements as a liability and should not be combined with deferred tax assets. The amendments in this ASU are effective for fiscal years, and interim periods within those years, beginning after December 15, 2013. Early adoption is permitted. The amendments should be applied prospectively to all unrecognized tax benefits that exist at the effective date. Retrospective application is permitted. We have not yet determined the effect of the adoption of this standard and it is not expected to have a material impact on our consolidated financial position and results of operations.

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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.

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.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

Our primary exposure to market risk is related to changes in interest rates. As of December 31, 2013, we had cash and short-term investments of approximately $6 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.

Item 8. Financial Statements and Supplementary Data

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

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Item 9. 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 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.

Item 9A. Controls and Procedures

(a) Evaluation of Disclosure Controls and Procedures

Management, with the participation of our Principal Executive Officer and Principal Financial Officer, carried out an evaluation of the effectiveness of our "disclosure controls and procedures" (as defined in the Securities Exchange Act of 1934, as amended (the "Exchange Act")) Rules 13a-15(e) and 15d-15(e)) as of the end of the period covered by this Annual Report on Form 10-K (the "Evaluation Date"). Based upon that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that as of the Evaluation Date, our disclosure controls and are 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, including our Principal Executive Officer and Principal Financial Officer, as appropriate to allow timely decisions regarding required disclosure.

(b) Management's Report on Internal Control Over Financial Reporting

Our management is also responsible for establishing and maintaining adequate internal controls over financial reporting (as such term is defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act). Our internal controls over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles.

As of December 31, 2013, we carried out an assessment of the effectiveness of our internal control over financial reporting based on the framework in "Internal Control-Integrated Framework" issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation, our management concluded that our internal control over financial reporting was not effective as of December 31, 2013.

As of December 31, 2013, we had identified certain matters that constituted 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 hiring of a Chief Financial Officer who has significant experience with publicly held companies. Although this is an important step towards improving the application of complex accounting principles, the preparation of financial reports and the segregation of duties, additional time is still required to fully implement additional internal controls procedures and test their operating effectiveness before we can definitively conclude that we have remediated our

deficiencies. Because these remediation steps have not yet been completed, we have performed additional analyses and other procedures to ensure that our consolidated financial statements contained in this Annual Report were prepared in accordance with GAAP and applicable SEC regulations.

We believe that our weaknesses in internal control over financial reporting and our disclosure controls relate in part to the fact that prior to the 2012 Merger with Desert Gateway, Retrophin was a small, privately-held company and was not subject to public company disclosure requirements, including the requirement to report on internal control over financial reporting in compliance with Section 404 of the Sarbanes-Oxley Act of 2002 and Item 308 of Regulation S-K. Our internal controls are still in a state of transition as we work diligently to integrate and assimilate all of our operations and work to remedy the significant deficiencies that together constitute a material weakness in our internal control over financial reporting.

This annual report does not include an attestation report of our registered public accounting firm regarding internal control over financial reporting. Management’s report was not subject to attestation by our registered public accounting firm pursuant to rules of the SEC that permit us to provide only management’s report herein.

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Change In Internal Control Over Financial Reporting

Except for the changes described below, there were no changes in internal control over financial reporting that occurred during the year ended December 31, 2013, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

On May 20, 2013, Marc Panoff became our Chief Financial Officer (CFO). Mr. Panoff has extensive experience in accounting and finance, and provides additional depth in each area.

On July 1, 2013, we hired a controller to further segregate duties within the Company.

Effective October 1, 2013, we appointed Cornelius Golding as an independent member of the Board of Directors who will also serve as Chairman of the Audit Committee. Mr. Golding has more than 44 years of experience in finance and accounting.

We are designing processes and internal controls to address changes in internal controls over financial reporting.

Item 9B. Other Information

None.

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PART III

Item 10. Directors, Executive Officers, and Corporate Governance of the Registrant

Information with respect to this item is incorporated by reference from our definitive Proxy Statement to be filed with the SEC not later than 120 days after the end of our fiscal year.

Item 11. Executive Compensation

Information with respect to this item is incorporated by reference from our definitive Proxy Statement to be filed with the SEC not later than 120 days after the end of our fiscal year.

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

Information with respect to this item is incorporated by reference from our definitive Proxy Statement to be filed with the SEC not later than 120 days after the end of our fiscal year.

Item 13. Certain Relationships and Related Transactions

Information with respect to this item is incorporated by reference from our definitive Proxy Statement to be filed with the SEC not later than 120 days after the end of our fiscal year.

Item 14. Principal Accountant Fees and Services

Information with respect to this item is incorporated by reference from our definitive Proxy Statement to be filed with the SEC not later than 120 days after the end of our fiscal year.

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PART IV

Item 15. Exhibits and Financial Statement Schedules

(a) (1) The financial statements at page F-1 are filed as a part of this Annual Report on Form 10-K.

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

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

(b) Description of Exhibits

Exhibit No.	Description
1.1	Form of Underwriting Agreement(1)
2.1	Agreement and Plan of Merger, dated December 12, 2012, by and among Desert Gateway, Inc. (now known as Retrophin, Inc.) (the “Company”), Desert Gateway Acquisition Corp., and Retrophin Inc. (2)
3.1	Certificate of Incorporation of the Company (3)
3.2	Amended and Restated Bylaws of the Company (4)
4.1	Form of Warrant issued to the purchasers (the “February 2013 Purchasers”) in the private placement of 3,045,929 shares of common stock, dated February 14, 2013 (5)
4.2	Form of Common Stock Purchase Warrant, dated August 15, 2013, issued to the purchasers (the “August 2013 Purchasers”) of securities in the private placement of the Company closed on August 15, 2013 (6)
10.1	Securities Purchase Agreement, dated February 12, 2013, by and among the Company and the February 2013 Purchasers (7)
10.2	Registration Rights Agreement, dated February 12, 2013, by and among the Company and the February 2013 Purchasers (8)
10.3	Sublicense Agreement, dated February 16, 2012, by and among Ligand Pharmaceuticals Incorporated, a Delaware corporation, Pharmacopeia, Inc., a Delaware limited liability company, and Retrophin, LLC, a Delaware limited liability company (9)
10.4	Employment Agreement, dated April 24, 2013, by and between Retrophin, Inc. and Horacio Plotkin, M.D. (10)
10.5	Employment Agreement, dated May 7, 2013, by and between Retrophin, Inc. and Marc Panoff (11)
10.6	Amendment to Employment Agreement, dated as of June 30, 2013 (12)
10.7	Securities Purchase Agreement, dated August 14, 2013, by and among the Company and the August 2013 Purchasers (13)
10.8	Registration Rights Agreement, dated August 15, 2013, by and among the Company and the August 2013 Purchasers (14)
10.9	First Amendment to Securities Purchase Agreement, dated August 14, 2013, by and among the Company and the purchasers signatory thereto (15)
10.10	First Amendment to Registration Rights Agreement, dated August 14, 2013, by and among the Company and the purchasers signatory thereto (16)
10.11	Sponsored Research Agreement between St. Jude Children's Research Hospital and the Company, dated October 1, 2013. (17) (Portions of Sections 1, 4, 6, Appendix A and

	Appendix B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)
10.12	Form of Settlement and Release Agreement between the Company, MSMB Capital Management, LP, MSMB Capital Management LLC, MSMB Healthcare LP, MSMB Healthcare Investors LLC, MSMB Healthcare Management LLC and the other parties thereto (18)
10.13	Form of Indemnification Agreement between the Company, MSMB Capital Management, LP, MSMB Capital Management LLC, MSMB Healthcare LP, MSMB Healthcare Investors LLC and MSMB Healthcare Management LLC (19)
10.14	Form of Promissory Note made by MSMB Capital Management, LP, MSMB Capital Management LLC, MSMB Healthcare LP, MSMB Healthcare Investors LLC and MSMB Healthcare Management LLC in favor of the Company (20)
10.15	License Agreement, dated December 12, 2013, by and among Retrophin, Inc., Novartis Pharma AG and Novartis AG. (21) (Portions of Sections 1, 4, 10, 11, 16, Schedule A and Schedule B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)

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10.16	Exclusive License Agreement, dated December 12, 2013, by and between Retrophin, Inc. and Stuart Weg, MD. (22) (Portions of Sections 2, 3, 4, 6, 7, Appendix A and Appendix B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)
10.17	Sponsored Research Agreement, dated December 12, 2013, by and between Retrophin, Inc. and The Regents of the University of California, on behalf of its San Diego Campus. (23) (Portions of the Recital, Sections 1, 2, 3, 4, 5, 6, 8, 9, 14, Exhibit A and Exhibit B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)
10.18	Employment Agreement, dated December 16, 2013, by and between Retrophin, Inc. and Martin Shkreli. (24)
10.19	Stock Purchase Agreement, dated December 23, 2013, by and among Retrophin, Inc., Kyalin Biosciences, Inc. and the Sellers party thereto. (25)
16.1	Letter from Michael F. Cronin, CPA, to the Securities and Exchange Commission (the "SEC") (26)
21.1	List of the Company's Subsidiaries *
31.1	Certifications pursuant to Section 302 of Sarbanes Oxley Act of 2002 *
32.1	Certifications pursuant to Section 906 of Sarbanes Oxley Act of 2002 *
101.INS	XBRL Instance Document *
101.SCH	XBRL Taxonomy Extension Schema Document *
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document *
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document *
101.LAB	XBRL Taxonomy Extension Label Linkbase Document *
101.PRE	Taxonomy Extension Presentation Linkbase Document *
*	Filed herewith.
(1)	Incorporated by reference to the Company's Amendment No. 1 to Registration Statement on Form S-1 filed with the SEC on January 1, 2014.
(2)	Incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K filed with the Securities and Exchange Commission (the "SEC") on December 19, 2012.
(3)	Incorporated by reference to Exhibit 3.1 to Amendment No. 2 to the Company's General Form for Registration of Securities on Form 10-12G filed with the SEC on October 28, 2010.
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(12)	Incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed with the SEC on July 30, 2013.

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(18) Incorporated by reference to Exhibit 10.12 to the Company's Registration Statement on Form S-1 filed with the SEC on November 15, 2013.
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(26) Incorporated by reference to Exhibit 16.1 to the Company's Current Report on Form 8-K filed with the SEC on October 29, 2012.

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SIGNATURES

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

Date: March 28, 2014

RETROPHIN, INC.

By: /s/ Martin Shkreli
Name: Martin Shkreli
Title: Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Martin Shkreli Martin Shkreli	Chief Executive Officer and Director (Principal Executive Officer)	March 28, 2014
/s/ Marc Panoff Marc Panoff	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 28, 2014
/s/ Stephen Aselage Stephen Aselage	Director	March 28, 2014
/s/ Steven Richardson Steven Richardson	Director	March 28, 2014
/s/ Cornelius Golding Cornelius Golding	Director	March 28, 2014
/s/ Jeffrey Paley Jeffrey Paley	Director	March 28, 2014

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EXHIBIT INDEX

Exhibit No.	Description
1.1	Form of Underwriting Agreement(1)
2.1	Agreement and Plan of Merger, dated December 12, 2012, by and among Desert Gateway, Inc. (now known as Retrophin, Inc.) (the "Company"), Desert Gateway Acquisition Corp., and Retrophin Inc. (2)
3.1	Certificate of Incorporation of the Company (3)
3.2	Amended and Restated Bylaws of the Company (4)
4.1	Form of Warrant issued to the purchasers (the "February 2013 Purchasers") in the private placement of 3,045,929 shares of common stock, dated February 14, 2013 (5)
4.2	Form of Common Stock Purchase Warrant, dated August 15, 2013, issued to the purchasers (the "August 2013 Purchasers") of securities in the private placement of the Company closed on August 15, 2013 (6)
10.1	Securities Purchase Agreement, dated February 12, 2013, by and among the Company and the February 2013 Purchasers (7)
10.2	Registration Rights Agreement, dated February 12, 2013, by and among the Company and the February 2013 Purchasers (8)
10.3	Sublicense Agreement, dated February 16, 2012, by and among Ligand Pharmaceuticals Incorporated, a Delaware corporation, Pharmacopeia, Inc., a Delaware limited liability company, and Retrophin, LLC, a Delaware limited liability company (9)
10.4	Employment Agreement, dated April 24, 2013, by and between Retrophin, Inc. and Horacio Plotkin, M.D. (10)
10.5	Employment Agreement, dated May 7, 2013, by and between Retrophin, Inc. and Marc Panoff (11)
10.6	Amendment to Employment Agreement, dated as of June 30, 2013 (12)
10.7	Securities Purchase Agreement, dated August 14, 2013, by and among the Company and the August 2013 Purchasers (13)
10.8	Registration Rights Agreement, dated August 15, 2013, by and among the Company and the August 2013 Purchasers (14)
10.9	First Amendment to Securities Purchase Agreement, dated August 14, 2013, by and among the Company and the purchasers signatory thereto (15)
10.10	First Amendment to Registration Rights Agreement, dated August 14, 2013, by and among the Company and the purchasers signatory thereto (16)
10.11	Sponsored Research Agreement between St. Jude Children's Research Hospital and the Company, dated October 1, 2013. (17) (Portions of Sections 1, 4, 6, Appendix A and Appendix B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)
10.12	Form of Settlement and Release Agreement between the Company, MSMB Capital Management, LP, MSMB Capital Management LLC, MSMB Healthcare LP, MSMB Healthcare Investors LLC, MSMB Healthcare Management LLC and the other parties thereto (18)
10.13	Form of Indemnification Agreement between the Company, MSMB Capital Management, LP, MSMB Capital Management LLC, MSMB Healthcare LP, MSMB Healthcare Investors LLC and MSMB Healthcare Management LLC (19)
10.14	Form of Promissory Note made by MSMB Capital Management, LP, MSMB Capital Management LLC, MSMB Healthcare LP, MSMB Healthcare Investors LLC and MSMB Healthcare Management LLC in favor of the Company (20)

10.15	License Agreement, dated December 12, 2013, by and among Retrophin, Inc., Novartis Pharma AG and Novartis AG. (21) (Portions of Sections 1, 4, 10, 11, 16, Schedule A and Schedule B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)
10.16	Exclusive License Agreement, dated December 12, 2013, by and between Retrophin, Inc. and Stuart Weg, MD. (22) (Portions of Sections 2, 3, 4, 6, 7, Appendix A and Appendix B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)
10.17	Sponsored Research Agreement, dated December 12, 2013, by and between Retrophin, Inc. and The Regents of the University of California, on behalf of its San Diego Campus. (23) (Portions of the Recital, Sections 1, 2, 3, 4, 5, 6, 8, 9, 14, Exhibit A and Exhibit B of the Exhibit have been omitted pursuant to a request for confidential treatment and filed separately with the Commission.)
10.18	Employment Agreement, dated December 16, 2013, by and between Retrophin, Inc. and Martin Shkreli. (24)
10.19	Stock Purchase Agreement, dated December 23, 2013, by and among Retrophin, Inc., Kyalin Biosciences, Inc. and the Sellers party thereto. (25)

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16.1	Letter from Michael F. Cronin, CPA, to the Securities and Exchange Commission (the "SEC") (26)
21.1	List of the Company's Subsidiaries *
31.1	Certifications pursuant to Section 302 of Sarbanes Oxley Act of 2002 *
32.1	Certifications pursuant to Section 906 of Sarbanes Oxley Act of 2002 *
101.INS	XBRL Instance Document *
101.SCH	XBRL Taxonomy Extension Schema Document *
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document *
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document *
101.LAB	XBRL Taxonomy Extension Label Linkbase Document *
101.PRE	Taxonomy Extension Presentation Linkbase Document *

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)

INDEX TO FINANCIAL STATEMENTS

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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, 2013 and 2012 and the related consolidated statements of operations and comprehensive loss, changes in shareholders' deficit and cash flows for the years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our 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 as of December 31, 2013 and 2012, and the consolidated results of its operations and its cash flows for the year ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, in conformity with accounting principles generally accepted in the United States of America.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 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, that might be necessary should the Company be unable to continue as a going concern.

/s/ Marcum LLP

New York, NY
March 28, 2014

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

	December 31, 2013	December 31, 2012
Assets		
Current assets:		
Cash	$ 5,997,307	$ 11,388
Marketable securities	132,994	-
Prepaid expenses and other current assets	1,370,943	21,830
Total current assets	7,501,244	33,218
Property and equipment, net	127,427	23,790
Due from affiliate	-	137,547
Security deposits	244,058	-
Restricted cash	40,000	-
Indefinite lived intangible assets	10,560,355	-
Other Amortizable intangible assets, net	2,025,795	2,196,710
Total assets	$ 20,498,879	$ 2,391,265
Liabilities and Stockholders' Deficit		
Current liabilities:		
Technology license liability	$ -	$ 1,300,000
Deferred technology purchase liability, current portion	1,634,630	-
Accounts payable	3,553,567	1,023,320
Accrued expenses	2,779,695	2,467,796
Offering expense liability	746,739	
Securities sold, not yet purchased	1,457,901	-
Note payable - related party	-	884,764
Investors' deposits	-	100,000
Due to related parties	-	23,200
Derivative financial instruments, warrants	25,037,346	-
Total current liabilities	35,209,878	5,799,080
Deferred technology purchase liability	1,000,000	-
Deferred income tax liability, net	2,600,899	-
Total liabilities	38,810,777	5,799,080
Commitments and contingencies		
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; 18,546,363 and 8,952,905 issued and 18,415,573 and 8,952,905 outstanding, respectively	1,855	895
Additional paid-in capital	50,189,127	30,203,402
Treasury stock, at cost, 130,790	(957,272)	-
Deficit accumulated during the development stage	(67,435,621)	(33,612,112)
Accumulated other comprehensive loss	(109,987)	-
Total stockholders' deficit	(18,311,898)	(3,407,815)
Total liabilities and stockholders' deficit	$ 20,498,879	$ 2,391,265

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

	For the year ended December 31,		For the period from March 11, 2011 (inception) through December 31,
	2013	2012	2013
Operating expenses:			
Selling, general and administrative - inclusive of share base compensation $4,384,220, $22,410,222, and $28,773,741	$16,888,064	$29,594,515	$ 49,514,264
Research and development - inclusive of share based compensation $259,076, $0, and $259,076	7,084,009	662,502	7,978,522
Total operating expenses	23,972,073	30,257,017	57,492,786
Operating loss	(23,972,073)	(30,257,017)	(57,492,786)
Other income (expenses):			
Interest expense, net	(46,344)	(84,087)	(130,356)
Registration payment obligation income	360,000	-	360,000
Registration payment obligation expense	(360,000)	-	(360,000)
Realized gain on sale of marketable securities, net	374,482	-	374,482
Change in fair value of derivative financial instruments - warrants	(10,099,926)	-	(10,099,926)
Loss on transactions denominated in foreign currencies	(3,873)	(2,752)	(11,260)
Total other expense, net	(9,775,661)	(86,839)	(9,867,060)
Loss before provision for income taxes	(33,747,734)	(30,343,856)	(67,359,846)
Provision for income taxes	(75,775)	-	(75,775)
Net loss	$(33,823,509)	$(30,343,856)	$ (67,435,621)
Net loss per common share, basic and diluted	$(2.38)	$(8.29)	
Weighted average common shares outstanding, basic and diluted	14,205,264	3,662,114	
Comprehensive Loss:			
Net loss	$(33,823,509)	$(30,343,856)	$ (67,435,621)
Unrealized loss on marketable securities	(109,987)	-	(109,987)
Comprehensive Loss	$(33,933,496)	$(30,343,856)	$ (67,545,608)

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)
CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' DEFICIT
FOR THE PERIOD FROM MARCH 11, 2011 (INCEPTION) THROUGH DECEMBER 31, 2013

	Common stock		Common stock in treasury				
	Shares	Amount	Shares	Amount	Additional paid in capital	Receivables due from stockholder	Accumulated other comprehensiv loss
Balance - March 11, 2011 (inception)	-	$-	-	-	$-	$-	$-
Issuance of common shares	1,608,300	161	-	-	24,839	(25,000)	-
Issuance of common shares to founders inconnection with the initial capital contribution	50,000	5	-	-	95	-	-
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	-	-
Share based compensation - non employees	-	-	-	-	254,332	-	-
Issuance of shares in connection with March 2011 private placement, net of fess of $66,061	253,750	25	-	-	658,914	-	-
Issuance of Series A preferred in connection with March 2011 private placement, net of feees of $1,367, recapitalization to common stock	36,750	4	-	-	103,629	-	-
Loan made to stockholder	-	-	-	-	-	(10,000)	-
Net loss	-	-	-	-	-	-	-
Balance - December 31, 2011	4,042,265	404	-	-	2,766,567	(35,000)	-
Prior Issuance of Series A preferred in connection with January 2012 private placement, net of feees of $61,677, exchanged to common stock	326,963	33	-	-	1,806,644	-	-
Prior Issuance of Series A preferred in connection with May 2012 private placement, net of feees of $12,275, exchanged to common stock	470,764	47	-	-	1,668,979	-	-
Shares transferred to consultants by founder for services	-	-	-	-	4,400,000	-	-
Shares transferred to employees by founders for services	-	-	-	-	1,375,000		-
	620,000	62	-	-	1,549,938	-	-

Shares issued in accordance with license agreement							
Shares outstanding at time of reverse merger date December 12, 2012	2,585,583	259	-	-	1,142	-	-
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	-	-
Share based compensation - non employees	-	-	-	-	1,997,372	-	-
Loan made to stockholder	-	-	-	-	-	(372,900)	-
Receivable due from stockholder charged to compensation	-	-	-	-	-	407,900	-
Net loss	-	-	-	-	-	-	-
Balance - December 31, 2012	8,952,905	895	-	-	30,203,402	-	-
Incentive shares granted - employees	135,000	14	-	-	(14)	-	-
Share based compensation - employees	-	-	-	-	1,424,528	-	-
Share based compensation - non employees	359,000	36	-	-	3,218,732	-	-
Incentive shares forfeited - employees	(20,833)	(2)	-	-	2	-	-
Incentive shares forfeited - non employees	(37,500)	(4)	-	-	4	-	-
Issuance of common stock in connection with January 2013 private placement at $3.00 per share, net of fees of $0	272,221	27	-	-	816,637	-	-
Issuance of common stock in connection with February 2013 private placement at $3.00 per share, net of fees of $928,986 and registration payment obligation of $360,000	3,045,929	305	-	-	2,441,124	-	-
Issuance of common stock in connection with August 2013 private placement at $4.50 per share, net of fees of $2,780,563 and payment made to February investors for inducement to participate in August financing of $2,238,681	5,531,401	553	-	-	10,670,020	-	-
Issuance of common stock in connection with payment made to February investors for inducement to participate in August financing, 271,222 shares at $4.50 per share and 20,685 shares at $5.00 per share	291,907	29	-	-	1,323,894	-	-
Treasury stock	-	-	(130,790)	(957,272)	-	-	-
Shares issued on behalf of related party	11,000	1	-	-	80,799	-	-

Adjustment to existing shareholders	5,333	1	-	-	9,999	-	-
Unrealized loss on marketable securities	-	-	-	-	-	-	(109,987)
Net loss	-	-	-	-	-	-	-
Balance - December 31, 2013	18,546,363	$1,855	(130,790)	$(957,272)	$50,189,127	$-	$(109,987)

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

	For the year ended December 31,		For the period from March 11, 2011 (inception) through December 31,
	2013	2012	2013
Cash Flows From Operating Activities:			
Net loss	$ (33,823,509)	$ (30,343,856)	$ (67,435,621)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	215,993	124,885	341,233
Realized gain on marketable securities	(374,482)	-	(374,482)
Compensation in lieu of stockholder receivable	-	407,900	407,900
Provision for income taxes	75,775	-	75,775
Share based compensation - employees	1,424,528	16,012,850	19,162,345
Share based compensation - non-employees	3,218,768	6,397,372	9,870,472
Shares issued on behalf of related party	80,800	-	80,800
Registration payment obligation expense	360,000	-	360,000
Reversal of registration payment obligation liability	(360,000)	-	(360,000)
Share based payment - Technology license contingent fee	-	1,550,000	1,550,000
Change in estimated fair value of liability classified warrants	10,099,926	-	10,099,926
Changes in operating assets and liabilities:			-
Prepaid expenses and other current assets	(1,349,113)	(14,830)	(1,370,943)
Technology license fees	-	150,000	150,000
Accounts payable and accrued expenses	2,842,146	2,978,940	6,330,941
Net cash used in operating activities	(17,589,168)	(2,736,739)	(21,111,654)
Cash Flows From Investing Activities:			
Purchase of fixed assets	(117,033)	(24,774)	(144,679)
Purchase of indefinite lived intangible assets	(5,400,601)	-	(5,400,601)
Purchase of amortizable intangible asset	(31,682)	(1,168,093)	(1,199,775)
Security deposits	(106,511)	-	(106,511)
Repayment of technology license liability	(1,300,000)	-	(1,300,000)
Proceeds from the sale of marketable securities	4,385,425	-	4,385,425
Purchase of marketable securities	(4,124,482)	-	(4,124,482)
Proceeds from securities sold, not yet purchased	4,193,719		4,193,719
Cover securities sold, not yet purchased	(2,865,260)		(2,865,260)
Increase in restricted cash	(40,000)		(40,000)
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)	(382,900)
Net cash used in investing activities	(5,406,425)	(1,699,593)	(7,118,890)

Cash Flows From Financing Activities:			
Proceeds from related parties	-	10,500	56,500
Repayment of net amounts due to related parties	(13,200)	(33,300)	(46,500)
Proceeds from note payable - related party	-	930,000	930,000
Repayment of note payable - related party	(884,764)	(45,236)	(930,000)
Investors' deposits	(100,000)	100,000	-
Proceeds received from issuance of common stock, net	30,936,748	3,475,703	35,175,123
Purchase of treasury stock, at cost	(957,272)	-	(957,272)
Net cash provided by financing activities	28,981,512	4,437,667	34,227,851
Net increase in cash	5,985,919	1,335	5,997,307
Cash, beginning of year	11,388	10,053	-
Cash, end of year	$ 5,997,307	$ 11,388	$ 5,997,307
Supplemental Disclosure of Cash Flow Information:			
Cash paid for interest	$ 28,263	$ 14,764	$ 43,027
Non-cash investing and financing activities:			
Unrealized gain on marketable securities	$ 3,292	$ -	$ 3,292
Unrealized loss on securities sold, not yet purchased	$ (113,279)	$ -	$ (113,279)
Forfeiture of subscription receivable	$ -	$ -	$ 25,000
Reclassification of due from related parties	$ -	$ 500	$ -
Technology license liability	$ -	$ 1,300,000	$ -
Adjustment to existing shareholders	$ 10,000	$ -	$ 10,000
Purchase of Kyalin in exchange for future consideration	$ 2,634,630	$ -	$ 2,634,630
Affiliate receivable applied to security deposit	$ 137,547	$ -	$ 137,547
Share based payment made to February investors for inducement to participate in August financing	$ 1,323,923	$ -	$ 1,323,923
Offering expense liability	$ 746,739	$ -	$ 746,739
Increase in basis of indefinite lived intangible assets acquired from Kyalin due to accrual of deferred tax liability	$ 2,525,124	$ -	$ 2,525,124

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RETROPHIN, INC. AND SUBSIDIARY
(A DEVELOPMENT STAGE COMPANY)

NOTES TO AUDITED CONSOLIDATED FINANCIAL STATEMENTS

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 was 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 14) 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 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 was 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 December 31 in order to conform 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. The Company is considered to be a development stage company and, as such, the Company’s financial statements are prepared in accordance with Accounting Standards Codification (“ASC”) 915, “Development Stage Entities” (“ASC 915”). The Company is subject to all of the risks and uncertainties associated with development stage companies.

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NOTE 2. LIQUIDITY AND FINANCIAL CONDITION AND MANAGEMENT'S PLANS

The Company incurred a net loss of approximately $67.4 million, including stock-based compensation of $29.0 million for the period from March 11, 2011 (inception) to December 31, 2013. At December 31, 2013, the Company had a cash balance of approximately $6.0 million and a working capital deficiency of approximately $27.7 million; however, the working capital deficit includes a derivative liability of approximately $25.04 million for warrants issued in financing transactions. The Company's accumulated deficit amounted to approximately $67.4 million at December 31, 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 14). On January 9, 2014, the Company raised gross proceeds of approximately $40 million.

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 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 the 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 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 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 12).

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

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 disease and other infectious diseases (see Note 13).

On December 12, 2013, the Company entered into an agreement with Novartis Pharma AG 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.

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As consideration for the license, the Company paid to Novartis and Novartis AG a $5 million upfront fee and is required to pay annual maintenance fees of $3 million after each anniversary until there has been regulatory approval, up to $34 million in developmental milestones for the first indication and up to $32 million in developmental milestones for the second indication. Should the Company commercialize the Product, it will be obligated to pay Novartis and Novartis AG a 10%-20% royalty on net sales of such products (see Note 7).

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 paid Dr. Weg $1,000,000, as well as certain maintenance and sublicensing fees. The Company is also obligated to pay Dr. Weg certain royalties on sales of Food and Drug Administration (the "FDA") approved products.

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 $1.54 million in fees to UCSD on a specified timeline, of which $0 has been paid as of the date hereof. As of December 31, 2013, the Company has accrued $40,082 in relation to the agreement. The Company is obligated to pay $192,500 per quarter through 2015. 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 within 60 days.

On December 16, 2013 (the "Effective Date"), 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. The Company realized a gain of $235,839 on the sale of Transcept shares for the year ended December 31, 2013.

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 ("CEO").

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.

On December 23, 2013, the Company entered into a stock purchase agreement (the "Stock Purchase Agreement") with Kyalin Biosciences, Inc., a Delaware corporation ("Kyalin"), pursuant to which the Company acquired all of the issued and outstanding shares of capital stock (the "Shares") from the Kyalin Sellers ("Sellers"). 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 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 (see Note 8.)

On March 26, 2014, the Company acquired 100% of the outstanding membership interests of Manchester Pharmaceuticals, LLC ("Manchester" or "acquiree"), a privately-held specialty pharmaceutical company that focuses on treatments for rare diseases. The acquisition of Manchester expands the Company's ability to address the special needs of patients with ultra-rare diseases.

Under the terms of the agreement, the Company paid $29.5 million upon closing, of which $3.2 million was paid by Retrophin Therapeutics International LLC, a newly formed indirect wholly owned subsidiary, for rights of product sales outside of the United States. The Company entered into a promissory note with Manchester principals for $33 million to be paid in three equal installments of $11 million within three, six, and nine months after closing. Additional contingent payments will be made based on product sales. The Company expects to raise additional funds through a public equity offering, a private equity offering, and/or debt financing to satisfy its short term obligations.

The financial statements of the acquiree are not practicable to prepare at the time of filing due to the acquiree being privately held and not maintaining financial statements in accordance with U.S. GAAP. The initial accounting for the business combination is not yet complete and the Company is still performing procedures to determine the appropriate accounting. As such, the Company is unable to make the following disclosures, (i) pro forma data, (ii) purchase price allocation, (iii) expenses of the acquisition, and (iv) revenue and earnings of the acquiree since the acquisition date.

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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. 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.

During the year ended December 31, 2013, the Company raised approximately $30.9 million in certain private placement transactions and raised an additional $40 million in gross proceeds through a public offering in January 2014. The Company expects to continue to finance its cash needs through additional public offerings, 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 United States of America generally accepted accounting principles ("U.S. GAAP"). All intercompany accounts and transactions have been eliminated in consolidation.

Restatement of Prior Quarters

In the fourth quarter of 2013, the Company discovered that certain warrants issued to a placement agent in connection with the Company's February Private Placement were not recorded (see Note 14) and certain expenses were overstated in the Company's condensed consolidated financial statements for the first, second, and third quarters of 2013. The overstated expenses include costs related to the February 14, 2013 Private Placement Offering (see Note 14) and costs associated with the Company's consultants. The adjustments necessary to reflect such issuance were recorded in the fourth quarter of 2013.

The following table (in thousands) sets forth the effects of the restatement on affected items within the Company's previously reported Consolidated Balance Sheets for the periods ended March 31, 2013, June 30, 2013 and September 30, 2013 had the adjustments been made in the corresponding quarters.

	March 31, 2013		June 30, 2013		September 30, 2013	
	As Reported	As Restated	As Reported	As Restated	As Reported	As Restated
Non-derivative liabilities	$1,575	$1,460	$4,229	$4,114	$4,935	$4,935
Derivative financial instruments, warrants	7,000	8,392	6,901	8,283	22,234	23,853
Total Liabilities	8,575	9,852	11,130	12,397	27,169	28,788
Additional paid in capital	34,773	33,621	34,946	33,794	48,652	47,500
Deficit accumulated during the development stage	(38,355)	(38,480)	(43,404)	(43,519)	(54,301)	(54,768)

Stockholder's deficit	$(3,582)	$(4,859)	$(8,458)	$(9,725)	$(5,804)	$(7,423)

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The following table (in thousands) sets forth the effects of the restatement on affected items within the Company's previously reported Consolidated Statement of Operations for the three months ended March 31, 2013, June 30, 2013 and September 30, 2013 had the adjustments been made in the corresponding quarters.

	March 31, 2013		June 30, 2013		September 30, 2013	
	As Reported	As Restated	As Reported	As Restated	As Reported	As Restated
Operating loss	$(2,251)	$(1,886)	$(5,100)	$(4,985)	$(5,155)	$(5,155)
Non-operating loss	(2,492)	(2,982)	51	61	(5,743)	(5,980)
Net loss	(4,743)	(4,868)	(5,049)	(4,924)	(10,898)	(11,135)
Net loss per common share, basic and diluted	$(0.44)	$(0.46)	$(0.41)	$(0.40)	$(0.71)	$(0.72)

The following table (in thousands) sets forth the effects of the restatement on affected items within the Company's previously reported Consolidated Statement of Operations for the six and nine months ended June 30, 2013 and September 30, 2013, respectively had the adjustments been made in the corresponding quarters.

	June 30, 2013		September 30, 2013	
	As Reported	As Restated	As Reported	As Restated
Operating loss	$(7,351)	$(6,986)	$(12,505)	$(12,255)
Non-operating loss	(2,441)	(2,921)	(8,184)	(8,901)
Net Loss	$(9,792)	$(9,907)	$(20,689)	$(21,156)
Net loss per common share, basic and diluted	$(0.85)	$(0.86)	$(1.62)	$(1.65)

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 restated the condensed consolidated financial statements for the quarter ended March 31, 2013 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

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. The Company minimizes its credit risk associated with cash by periodically evaluating the credit quality of its primary

financial institution. The balance at times may exceed federally insured limits.

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Marketable Securities

The Company accounts for marketable securities held as “available-for-sale” in accordance with ASC 320, “Investments Debt and Equity Securities” (“ASC 320”). The Company classifies these investments as current assets and 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 4.

Securities Sold, Not Yet Purchased

Securities sold, not yet purchased consist of marketable securities that the Company has sold short. In order to facilitate a short sale, the Company borrows the securities from another party and delivers the securities to the buyer. The Company will be required to "cover" its short sale in the future through the purchase of the security in the market at the prevailing market price and deliver it to the counterparty from which it borrowed. The Company is exposed to a loss to the extent that the security price increases during the time from when the Company borrowed the security to when the Company purchases it in the market to cover the short sale.

The Company accounts for securities sold, not yet purchased in accordance with ASC 815 – Derivative and Hedging (“ASC 815”), which states such transactions have two of the three characteristics of a derivative instrument and do not generally involve derivative instruments. Securities sold, not yet purchased are presented on the consolidated balance sheets with gains and losses reported in realized and unrealized gains on marketable securities on the consolidated statement of operations and comprehensive loss.

Restricted Cash

The Company holds restricted cash as a rent guarantee on an operating lease through September 2016.

Employee Stock-Based Compensation

The Company accounts for stock-based compensation in accordance with ASC 718, “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 ASC 505, “Share 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 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.

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Research and Development Costs

The Company develops new products through research and development and the licensing and acquisition of third-party businesses and technologies. Research and development costs are charged to operations as incurred and consist primarily of internal and external expenses related to conducting clinical trials, pre-clinical studies, regulatory activities, consulting, contract research and development, and compensation. For the years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized $7,084,009, $662,502 $7,978,522, respectively, of research and development costs.

Income Taxes

The Company accounts for income taxes in accordance with 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 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, 2013. 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 704. 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 the Company’s conversion to a corporation from a limited liability company on September 20, 2012, the Company reports and pays taxes based on its income or loss.

Foreign Currency Translation and Remeasurement

The Company accounts for foreign currency translation and remeasurement in accordance with ASC 830, “Foreign Currency Matters” (“ASC 830”). Under ASC 830, 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.

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 assesses the carrying amounts of its patents for potential impairment when events or changes in circumstances indicate that the carrying amounts of our patents may not be recoverable. As of December 31, 2013 and 2012, patents costs of $49,775 and $18,093, respectively, are included in the accompanying consolidated balance sheets.

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Long-Lived Assets

The Company accounts for long-lived assets in accordance with ASC 360, “Long-lived assets, other than goodwill, are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of the assets might not be recoverable. Conditions that would necessitate an impairment assessment include a significant decline in the observable market value of an asset, a significant change in the extent or manner in which an asset is used, or any other significant adverse change that would indicate that the carrying amount of an asset or group of assets may not be recoverable. Application of alternative assumptions, such as changes in estimate of future cash flows, could produce significantly different results. Because of the significance of the judgments and estimation processes, it is likely that materially different amounts could be recorded if we used different assumptions or if the underlying circumstances were to change.

For long-lived assets used in operations, impairment losses are only recorded if the asset’s carrying amount is not recoverable through its undiscounted, probability-weighted future cash flows. The Company measures the impairment loss based on the difference between the carrying amount and estimated fair value.

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 years ended December 31, 2013 and 2012, respectively, because their inclusion would have an anti-dilutive effect for the periods presented.

An aggregate of 172,667 and 0 stock options were excluded from the computation of diluted net loss per common share for the years ended December 31, 2013 and 2012, respectively, because they would have an anti-dilutive effect for the periods presented.

An aggregate of 168,643 and 267,768 incentive shares were excluded from the computation of diluted net loss per common share for the years ended December 31, 2013 and 2012, respectively, because they were contingent shares subject to recall.

On January 9, 2014, the Company completed a public offering of 4.7 million shares of common stock at a price of $8.50 per share (see Note 17).

Subsequent to year end, an aggregate of 798,391 warrants were exercised for a total of $3,830,316 in cash received by the Company (see Note 17).

Derivative Instruments

The Company does not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. The Company evaluates all of its financial instruments to determine if such instruments are derivatives or contain features that qualify as embedded derivatives. For derivative financial instruments that are accounted for as liabilities, the derivative instrument is initially recorded at its fair value and is then revalued at each reporting date, with changes in the fair value reported in the statements of operations. For stock-based derivative financial instruments, the Company calculates the fair value of the financial instruments using the 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 5 and Note 6).

Joint and Several Liability Arrangements

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

The Company accounts for financial instruments in accordance with ASC Topic 820, “Fair Value Measurements and Disclosures” (“ASC Topic 820”). 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 and securities sold, not yet purchased, the Company used quoted prices in active markets (see Note 4 and Note 6).

In estimating the fair value of the Company's derivative liabilities, the Company used the Binomial Lattice pricing model (see Note 5 and Note 6).

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.

Treasury Stock

Treasury stock is recorded at cost. Issuance of treasury stock is accounted for on a first-in, first-out basis. Differences between the cost of treasury stock and the re-issuance proceeds are charged to additional paid-in capital.

Registration Payment Arrangements

The Company accounts for registration payment arrangements in accordance with ASC 825-20, "Registration Payment Arrangements" ("ASC 825-20") 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" ("ASC 450").

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. The Company reclassified research and development expenses and selling, general, and administrative expenses.

Subsequent Events

The Company follows the provisions of ASC Topic 855-10, "Subsequent Events" ("ASC 855-10") 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 Update ("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 2 and Note 12).

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The FASB has issued ASU No. 2013-11, Income Taxes (Topic 740): Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists (a consensus of the FASB Emerging Issues Task Force). The amendments in this ASU state that an unrecognized tax benefit, or a portion of an unrecognized tax benefit, should be presented in the financial statements as a reduction to a deferred tax asset for a net operating loss carryforward, a similar tax loss, or a tax credit carryforward, except as follows. To the extent a net operating loss carryforward, a similar tax loss, or a tax credit carryforward is not available at the reporting date under the tax law of the applicable jurisdiction to settle any additional income taxes that would result from the disallowance of a tax position or the tax law of the applicable jurisdiction does not require the entity to use, and the entity does not intend to use, the deferred tax asset for such purpose, the unrecognized tax benefit should be presented in the financial statements as a liability and should not be combined with deferred tax assets. The amendments in this ASU are effective for fiscal years, and interim periods within those years, beginning after December 15, 2013. Early adoption is permitted. The amendments should be applied prospectively to all unrecognized tax benefits that exist at the effective date. Retrospective application is permitted. The Company has not yet determined the effect of the adoption of this standard and it is not expected to have a material impact on the Company's consolidated financial position and results of operations.

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. MARKETABLE SECURITIES AND SECURITIES SOLD, NOT YET PURCHASED

The Company measures marketable securities and securities sold, not yet purchased 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 and securities sold, not yet purchased at December 31, 2013 consisted of the following:

	Cost	Unrealized Gains	Unrealized Losses	Estimated Fair Value
Marketable securities available-for-sale:	$ 129,702	$ 3,292	$ -	$ 132,994
Securities sold, not yet purchased	$ 1,344,622	$ 13,256	$ 126,535	$ 1,457,901

For the year ended December 31, 2012, marketable securities and securities sold, not yet purchased were $0.

NOTE 5. DERIVATIVE FINANCIAL INSTRUMENTS

The Company accounts for 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 year ended December 31, 2013 that do not have fixed settlement provisions, are not indexed to the Company's own stock. The fair value of the warrants are classified as liability instruments due to a non-standard anti-dilution provision that provides for a reduction to the exercise price of the warrants if the Company issues additional equity or equity linked instruments in the future at an effective price per share less than the exercise price then in effect.

The warrants are re-measured at each balance sheet date based on estimated fair value. Changes in estimated fair value are recorded as non-cash valuation adjustments within other income (expense) in the Company's accompanying consolidated statements of operations. The Company recorded a loss on a change in the estimated fair value of warrants of $10,099,926 during the year ended December 31, 2013

The Company calculated the fair value of the warrants using the Binomial Lattice pricing model. The assumptions used at the date of issuance and at December 31, 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	December 31, 2013
Fair market price of common stock	$3.75	$4.50	$4.50	$7.00
Contractual term	5 years	5 years	5 years	4.12 – 4.62 years
Risk-free interest rate	0.86%	1.48%	1.48%	1.39%
Expected volatility	101%	106%	106%	93%-97%

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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.

The following tables illustrates the Company's derivative warrant issuances and balances outstanding as of, and during the years ended December 31, 2013 and 2012:

	Warrants	Weighted Average Exercise Price	Fair Value
Outstanding at December 31, 2011	-	$ -	$ -
Issued	-	-	-
Canceled	-	-	-
Exercised	-	-	-
Outstanding at December 31, 2012	-	$ -	$ -
Issued	4,782,249	5.04	3.13
Canceled	-	-	-
Exercised	-	-	-
Outstanding at December 31, 2013	4,782,249	$ 5.04	$ 5.23

The following information applies to derivative warrants outstanding at December 31, 2013:

Exercise Price	Number of Warrants	Weighted Average Remaining Contractual Life (years)	Number Exercisable
$ 3.60	1,917,792	4.12	1,917,792
$ 6.00	2,864,457	4.62	2,864,457

The total intrinsic value of derivative warrants outstanding and exercisable as of December 31, 2013 is $9,384,950. The Company's closing stock price was $7 on December 31, 2013.

NOTE 6. FAIR VALUE MEASUREMENTS

The following table presents the Company's asset and liabilities that are measured and recognized at fair value on a recurring basis classified under the appropriate level of the fair value hierarchy as of December 31, 2013:

	As of December 31, 2013	Fair Value Hierarchy at December 31, 2013		
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)

Asset:					
Marketable securities, available-for-sale	$	132,994	$132,994	$-	$-
Liabilities:					
Derivative liability related to warrants	$	25,037,346	$-	$-	$25,037,346
Securities sold, not yet purchased	$	1,457,901	$1,457,901	$-	$-

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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 year ended December 31, 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	5,407,372
August 14, 2013	328,561
August 15, 2013	9,201,487
Total value upon issuance	14,937,420
Change in fair value of common stock warrant liability	10,099,926
Balance at December 31, 2013	$ 25,037,346

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 7. INTANGIBLE ASSETS

Indefinite lived intangible assets

The Company purchased, and currently owns (i) an exclusive, perpetual, and royalty-bearing license exclusive for the manufacture, development and commercialization of a product in the United States (ii) technology related to an intranasal formulation compound. The intellectual property underlying the license and formula is held in perpetuity. These intangible assets are measured initially at cost not subject to amortization and are tested for impairment annually or in interim reporting periods if events or changes in circumstances indicate that the carrying amounts of these intangible asset might not be recoverable. FASB ASC 740-10-55 ("ASC 740") addresses the accounting treatment when an asset is acquired outside of a business combination, and the tax basis of that asset differs from the amount paid. As a result of this guidance, the Company has stepped-up the basis of its intangible assets and has recorded a deferred tax liability in the same amount, to account for the basis difference resulting from the Kyalin acquisition.

Indefinite lived intangible assets as of December 31, 2013 consist of the following:

Syntocinon License	$5,000,000
Carbetocin Assets	5,560,355
Total	$10,560,355

2013 Activity

Syntocinon License Agreement

On December 12, 2013 (the “Effective Date”), the Company entered into an agreement with Novartis Pharma AG 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 (the “Product”) 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 trials and other development of Syntocinon at its expense, and must use commercially reasonably efforts to develop Syntocinon in the United States.

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As consideration for the license, the Company paid to Novartis Pharma AG and Novartis AG a $5 million upfront fee and is required to make substantial payments at certain dates and upon the achievement of certain milestones as follows: (i) annual payments of $3,000,000 after each anniversary of the Effective Date until there has been Regulatory Approval of the Product for use in an autism or schizophrenia indication from the U.S. Food and Drug Administration, (ii) a milestone payment of $2,000,000 after the first patient is enrolled in a clinical trial for any indication not set forth in the New Drug Application ("NDA") filed by Novartis on January 19, 1960 for the following indications: initial milk let-down, milk retention, incipient mastitis, impaired milk let-down, (the "Initial NDAregistration"), (iii) a milestone payment of $2,000,000 after the first patient is enrolled in a registration study in the first indication for the Product for any indication not set forth in the Initial NDA, (iv) a milestone payment of $2,000,000 after the first patient is enrolled in a registration study in the second indication for the Product for any indication not set forth in the Initial NDA, (v) a milestone payment of $5,000,000 after the receipt of positive results from a registration study in the first indication for the Product for any indication not set forth in the Initial NDA (vi) a milestone payment of $5,000,000 after receipt of positive results from a registration study in the second indication for the Product for any indication not set forth in the Initial NDA, (vii) a milestone payment of $10,000,000 after filing an NDA for the first indication for any indication not set for the in the Initial NDA, (viii) a milestone payment of $10,000,000 after filing an NDA for the second indication for any indication not set for the in the Initial NDA, (ix) a milestone payment of $15,000,000 after Regulatory Approval of the Product in the first indication from FDA for any indication not set forth in the Initial NDA, (x) a milestone payment of $15,000,000 after Regulatory Approval of the Product in the second indication from FDA for any indication not set forth in the Initial NDA. Should the Company commercialize the Product, it will be obligated to pay Novartis and Novartis AG a 10%-20% royalty on net sales of such products.

The Company capitalized the $5,000,000 upfront fee as an indefinite lived intangible asset. The Company will test for impairment when events and circumstances indicate that the assets might be impaired. As of December 31, 2013, the Company has accrued the annual maintenance fee in the amount of $150,000.

Kyalin - Carbetocin Technology Purchase

On December 23, 2013 (the "Closing Date"), the Company entered into a Stock Purchase Agreement with Kyalin to acquire substantially all of Kyalin's assets which include patents, patent applications, contracts and data related to the intranasal formulation of the compound Carbetocin (collectively, the "Carbetocin Assets") Carbetocin, similar to Oxytocin, has potential utility for the treatment of milk let-down in post pregnant women, inducing contractions during labor, postpartum hemorrhage, as well as for autism and schizophrenia.

The Company determined to treat the Stock Purchase Agreement as an asset acquisition in accordance with ASC 805, "Business Combinations" ("ASC 805"). In accordance with ASC 805, the Company is required to determine whether the transaction meets the definition of a business combination, which requires that the assets acquired and liabilities assumed constitute a business. The form in which the transaction takes place typically will not affect the determination of whether the acquisition is a business. A business is identified by evaluating whether there is sufficient continuity of operations so that disclosure of prior financial information is material to an understanding of future operations. Kyalin has had significantly limited activity since its formation on October 4, 2011.

The Company agreed to pay to the Sellers $3,000,000 of fixed minimum payments consisting of: (i) $500,000 of cash at closing, (ii) $500,000 of cash on the first anniversary of the Closing Date, (iii) $1,000,000 of the Company's common stock, par value $0.0001 per share common stock on the first anniversary of the Closing Date, (iv) $1,000,000 of the Company's common stock, par value $0.0001 per share common stock on the second anniversary of the Closing Date, and up to $2,000,000 of additional consideration thereafter, subject to the attainment of following milestones, (v) $800,000 of the Company's common stock, par value $0.0001 per share common stock after completion of the first Phase II study of a compound that meets its primary endpoint and would enable the Company

to subsequently move the respective compound into a Phase III Study, (vi) $800,000 of the Company's common stock, par value $0.0001 per share common stock after completion of the first Phase III Study of a compound that meets its primary endpoint such that the safety and efficacy data from such Phase III Study may be included in an NDA for a drug with the FDA, (vii) $400,000 of the Company's common stock, par value $0.0001 per share common stock after receipt of FDA approval of an NDA for a drug. On the closing date of the acquisition, the Company paid $365,370 and recorded a liability of $2,634,630. As of December 31, 2013, the Company has recorded a liability of $634,630 for the cash and $2,000,000 for value of shares payable at certain future dates in accordance with the stock purchase agreement. Share payments shall be issued based on the greater of (a) the average price per share quoted 30 trading days immediately preceding the date such shares first become issuable or (b) $7.0938 per share. The Company has recorded a deferred purchase asset liability of $2,634,630.

In accordance with ASC 740, the Company has stepped-up the basis of its intangible assets in the amount of $2,525,124 and has recorded a deferred tax liability in the same amount, to account for the basis difference resulting from the Kyalin acquisition (see Note 16).

Amortizable intangible assets

2012 Activity

Ligand License Agreement

On February 16, 2012 the Company entered into an agreement pursuant to which a biotechnology company ('the Sublicensor') with license rights to certain drug technologies agreed to grant the Company a worldwide sublicense for the development, manufacture and commercialization of Sparsentan (DARA). The license 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 Sparsentan. The Company must use commercially reasonable efforts to develop and commercialize Sparsentan 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.

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In accordance with the agreement as amended most recently as of January 7, 2013, the Company was obligated to make two non-refundable payments totaling $2,550,000, the first payment of $1,150,000 was made upon execution and the second payment of $1,400,000 was made in February 2013, and includes a $250,000 fee payable to the sublicensee in exchange for extending the due date of this payment from October 1, 2012 to February 2013. As of December 31, 2013, 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. The $250,000 extension fee was expensed in February 2013. In addition, the Company issued 620,000 common shares to Ligand valued at $1,550,000 following the completion of the Merger transaction as required under the terms of the agreement. The fair value of these shares was expensed to operations in December 2012. Amortization expense on intangibles was $202,597, $121,383, and $323,890 for the year ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, respectively, and was recorded in the statement of operations as Research and Development expense.

In addition, the Company is obligated to make series of milestone payments upon the achievement of each development milestone event set forth in the sublicense agreement which could amount to an aggregate of up to $106.9 million. 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.

Amortizable intangible assets as of December 31, 2013 and 2012 consist of the following:

	December 31, 2013		
	Gross Carrying Amount	Accumulated Amortization	Net Book Value
Ligand License	2,300,000	(323,980)	1,976,020
Patent Costs*	49,775	-	49,775
Total	$ 2,349,775	$ (323,980)	$ 2,025,795

	December 31, 2012		
	Gross Carrying Amount	Accumulated Amortization	Net Book Value
Ligand License	$ 2,300,000	$ (121,383)	$ 2,178,617
Patent Costs*	18,093	-	18,093
Total	$ 2,318,093	$ (121,383)	$ 2,196,710

*Patent costs will be amortized when a patent is procured and a life is assigned to the asset
As of December 31, 2013, amortization expense for the next five years is expected to be as follows:

2014	$202,597

2015	202,597
2016	202,597
2017	202,597
2018	202,597
thereafter	1,012,810
Total	$2,025,795

As of December 31, 2013 the remaining weighed average period of amortization is 9.54 years.

NOTE 8. ACCRUED EXPENSES

Accrued expenses consist of the following at December 31, 2013 and 2012:

	December 31, 2013	December 31, 2012
Compensation related costs	1,144,983	1,022,716
Research and development	1,035,875	679,800
Business development	300,000	-
License fee	150,000	-
Accounting and legal fees	75,000	563,380
Finder's fee	-	100,000
Other	73,837	11,250
Interest	-	90,650
	$ 2,779,695	$ 2,467,796

NOTE 9. SELLING, GENERAL, AND ADMINISTRATIVE

Selling, general, and administrative expenses consist of the following at December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013:

	December 31, 2013	December 31, 2012	For the period from March 11, 2011 (inception) through December 31, 2013
Professional fees (inclusive of share base compensation $3,218,768, $6,397,372, and $9,870,472)	$ 7,333,830	$ 9,035,702	$ 16,982,798
Other	5,029,491	725,263	5,852,152
Compensation and related costs (inclusive of share base compensation $1,165,452, $16,012,850, and $18,903,269)	4,274,743	18,133,550	24,729,314
Technology license contingent fees	250,000	1,700,000	1,950,000
Total selling, general, and administrative expenses	$ 16,888,064	$ 29,594,515	$ 49,514,264

NOTE 10. RESEARCH AND DEVELOPMENT

Research and development expenses consist of the following at December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013:

	December 31, 2013	December 31, 2012	For the period from March 11, 2011 through December 31, 2013
External service provider costs:			
Sparsentan	$ 2,443,273	$ 297,833	$ 2,619,723
RE-024	1,548,957	124,635	1,673,592
Weg license in process R&D (Note 13)	1,000,000	-	1,000,000
Syntocinon	250,540	-	250,540
RE-034	230,279	-	230,279
General	159,080	240,034	493,569
Other product candidates	117,771	-	376,710
Total external service provider costs:	5,749,900	662,502	6,644,413
Internal personnel costs (inclusive of share base compensation $259,076, $0, and $259,076):	1,334,109	-	1,334,109
Total research and development	$ 7,084,009	$ 662,502	$ 7,978,522

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NOTE 11. 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 was originally 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. 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, was repaid during the quarter ended March 31, 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. On December 3, 2012, the Company repaid $30,000 plus any unpaid interest.

Total interest expense recognized for the years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013 were aggregated to $41,563, $105,917, and $147,480, respectively.

NOTE 12. RELATED PARTY TRANSACTIONS

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.

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 for the year ended December 31, 2013 for the (a) $2,203,711 of cash consideration, and (b) 11,000 shares of common stock valued at $80,800 of non-cash consideration. The $2,284,511 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. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

Concurrent with the execution of such settlement agreements, the Company and the related party entered into promissory notes whereby the related party agreed to pay the Company the principal amount of $2,284,511 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 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.

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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 13. COMMITMENTS AND CONTINGENCIES

Leases and Sublease

In October 2012, the Company entered into a sublease with a company ("Sublessor") affiliated by common ownership for 4,216 square foot of office space and its principal offices. The sublease agreement required 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. The Company assumed the building lease from such 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 12).

On October 1, 2013, the Company entered into building lease for approximately 4,232 square foot of office space located in Cambridge, Massachusetts under which we are responsible for rent of approximately $216,000 annually plus rent escalations, common area maintenance, insurance, and real estate taxes through September 2016.

On October 8, 2013, the Company entered into an amended lease agreement for an approximately 4,000 square foot of 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.

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.

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, and (b) 25,000 shares of the Company common stock with an estimated fair value of $100,000, which vests over twelve (12) quarters so long as the agreement remains in effect. For the years ended December 31, 2013 and 2012, for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized professional fees related to this agreement in the amounts of approximately $153,000, $150,000, and $378,500, respectively, of which amounts comprised of fee payable of $0, $155,000 and $0 at December 31, 2013 and 2012 and for the period from March 11 ,2011 (inception) through December 31, 2013, respectively.

On November 1, 2011, the Company granted to the same above 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 years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized professional fees related to this share based

compensation of $195,000, $210,000, and $445,000, respectively.

On October 26, 2013 and December 1, 2013, the Company amended the above consulting agreements to issue the consultant 200,000 additional shares of the Company common stock to the consultant that payable as follows: (i) 100,000 shares on December 31, 2013, (ii) 50,000 shares on March 30, 2014, (iii) 50,000 shares on June 30, 2014. In addition, the consultant amended the fee and shall receive $26,666 per month. The agreement expires on October 25, 2013, and shall automatically extend for one year unless notice of non-extension is given. For the year ended December 31, 2013 and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized professional expense related to this agreement in the amount of approximately $780,000.

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On August 25, 2011 and November 1, 2011, the Company entered into two agreements with a consultant to serve as chief scientific officer of the Company. The agreements' initial terms were for one year and automatically renewed on an annual basis. Pursuant to the agreements the compensation to the consultant was comprised of (a) a fee of $50,000 per calendar quarter, and (b) 145,000 incentive shares with an estimated fair value of $580,000, which vested over twelve (12) quarters so long as the agreements remained in effect. For the years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized professional expense related to these agreements in amounts of $225,000, $200,000, and 525,000, respectively. These agreements terminated on December 31, 2012. The Company recorded professional expense for the year ended December 31, 2013 for 34,575 vested shares in 2013 that were issued upon execution of the agreements.

On February 15, 2013, the Company entered into an agreement with a consultant to provide certain advisory services. The Company granted 12,500 shares of common stock with an estimated value of $52,500. For the years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized professional expense related to these agreements in amounts of $52,500, $0, and $52,500, respectively

On September 20, 2013, the Company entered into an agreement with a consultant to serve as an advisor to the Company. The Company granted 331,500 shares of common stock issued and payable as follows (i) 131,500 shares of common stock issued upon execution of the agreement, (ii) 50,000 shares of common stock issued on September 30, 2013, (iii) 50,000 shares of common stock issued on December 31, 2013, (iv) 50,000 shares of common stock issued on March 31, 2014, and (v) 50,000 shares issued on June 30, 2014. The agreement expires on June 30, 2014. For the years ended December 31 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized professional expense related to this agreement in the amount of $1,628,375, $0, and $1,628,375.

On November 8, 2013, the Company entered into an agreement with a consultant to serve as an advisor to the Company. The Company shall pay the consultant $15,000 per quarter and expires in six months from the date entered into.

On December 31, 2013, the Company entered into an agreement with a consultant to serve as an advisor to the Company. The Company granted 15,000 shares of common stock issued and paid upon the date of execution. During the term of the agreement, the Company shall pay the consultant $50,000 per month. The agreement expires on April 30, 2014. For the years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized professional expense related to these agreements in amounts of $105,000, $0, and $105,000, respectively.

Sponsored Research Agreements

St. Jude

On July 1, 2012, the Company entered into a Sponsored Research Agreement with St. Jude Children's Research Hospital ("St. Jude") that expired on July 1, 2013. The Company paid sponsor fees totaling $203,169 to the organization to perform the research program stated in the Sponsored Research Agreement.

Sponsor fees totaling $203,169 was 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 $101,314, $101,855, $203,169 for the years ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, respectively.

Effective October 1, 2013, the Company signed a new Sponsored Research Agreement 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. On October 22, 2013, the Company paid $195,169 in accordance with this agreement. For the year ended December 31, 2013, the Company recognized professional expense related to this agreement in the amount of $97,584.

SickKids

On July 12, 2013, the Company agreed to sponsor a study with The Hospital for Sick Children (“SickKids”). The Company agreed to fund the study by providing a CAD $750,000 (USD $721,470) amount matching Brain Canada’s contribution over a three-year term. The Company paid CAD $250,000 (USD $234,490) on December 3, 2013. The Company is obligated to pay a total of CAD $500,000 (USD $467,425) in CAD $250,000 (USD $233,713) yearly installments through 2015. For the year ended December 31, 2013, the Company recognized professional expense related to this agreement in the amount of $113,327.

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University of Michigan

On October 2, 2013, the Company agreed to provide a charitable contribution to the University of Michigan up to $2,000,000 to be funded in equal quarterly installments over a two-year period commending in October, 2013. On October 2, 2013, the Company paid and expensed $250,000 to the University of Michigan as part of this agreement. The Company reserves the right to cancel this arrangement with no further funding obligation.

UCSD

On December 11, 2013, the Company entered into an agreement with UC San Diego to provide an unrestricted charitable gift in support of translation psychiatric research at UC San Diego. The total charitable contribution in the amount of $530,000 will be paid in three contribution over a one year period in beginning on January 31, 2014. As of December 31, 2013, the Company has accrued and expensed $530,000 as part of this 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 over a period of two years. As consideration for the research program, the Company is obligated to pay an aggregate of approximately $1.54 million in fees to UCSD on a specified timeline, of which $0 has been paid as of the date hereof. As of December 31, 2013, the Company has expense and accrued $40,082 in relation to the agreement. The Company is obligation to pay $192,500 per quarter through 2015. 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 within 60 days.

License Agreements

Weg License Agreement

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 paid Dr. Weg $1,000,000 upon execution of the agreement. The Company is also obligated to pay Dr. Weg certain royalties on sales of FDA-approved products.

Prior to receipt from the FDA of approval for a product for depression indications, the Company agrees to pay Weg an annual license maintenance fee of $1 million. Upon the receipt of FDA approval for any product for depression indication, no further annual maintenance fees are due. The Company agrees to pay a royalty for FDA approved products at a rate of 3% to 8%.

The following one-time milestones are due in accordance with the Weg agreement:

- $1 million after a patent covering products under the agreement is granted by the U.S. Patent and Trademark Office;
- $2.5 million for the first NDA acceptance for depression indication;
- $2.5 million after the first FDA approval for products for depression indication;
- $2.5 million for the first NDA acceptance of product for an indication not included in the Company's previously accepted NDA;
- $2.5 million for the first FDA approval for products not included in the Company's previous FDA approval.

Other License Agreements

During the years ended December 31, 2013 and 2013, the Company entered into license agreements with Novartis and Ligand. The Company is required to pay annual maintenance fees and milestone payments in accordance with these agreements (see Note 2 and 7.)

Kyalin Technology Acquisition

On December 23, 2013, the Company entered into a Stock Purchase Agreement with Kyalin to acquire substantially all of Kyalin's assets. The Company is required to pay annual maintenance fees and milestone payments in accordance with the agreement (see Note 2 and 7.)

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Shkreli Employment agreement

On December 16, 2013, the Company entered into a new 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. Shkreli 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.

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.

Withdrawal of Transcept Proposal

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 invested approximately $3 million and

acquired approximately 4.96% of the outstanding common stock of Transcept as part of the proposal process.

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. The Company realized a gain of $235,839 on the sale of Transcept shares for the year ended December 31, 2013.

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Contract Commitments

The following table summarizes our principal contractual commitments, excluding open orders that support normal operations, as of December 31, 2013:

Year Ending December 31,	Research and Development and other Charitable Donations	Consultants	Operating Leases
2014	$4,560,092	$831,660	$777,538
2015	2,950,294	-	811,149
2016	-	-	724,292
2017	-	-	12,850
2018	-	-	-
2019 and thereafter	-	-	-
Total	$7,510,386	$831,660	$2,325,829

NOTE 14. 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.

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).

Private Placement Offerings - 2013

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 January 4, 2013, the Company entered into an agreement with Roth Capital Partners to act as its exclusive placement agent in connection with the February Private Placement. In connection with the agreement, the Company paid cash fees in the amount of $624,033 and issued warrants to purchase up to an aggregate of 319,823 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 $901,767 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 (see Note 2).

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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 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 2, 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.

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First Amendment to the February Registration Rights Agreement

As described in Note 2, 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 the 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 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. The Registration Statement was declared effective by the SEC on December 6, 2013.

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 15).

Preferred Stock

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.

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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.

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 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 member 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 and were recorded as a charge to operations in the accompanying statement of operations.

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 and were recorded as a charge to operations in the accompanying statement of operations.

Receivables from Shareholders

On February 3, 2012, the Company entered into a note receivable with a related party in the amount of $200,000. The note receivable was unsecured, bearing an interest rate of 12% per annum and due to mature on February 3, 2013. The note was originally classified as a reduction of stockholders' equity in the accompanying consolidated balance sheet and later treated as compensation in the amount of $372,900 for the year ended December 31, 2012

Stock Options

On May 13, 2013, the Company issued options (the "Options") to purchase 120,000 shares of common stock with an estimated fair value of $804,732 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 with the following assumptions: risk-free interest rate of .83% (based on the U.S. 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 year ended December 31, 2013, the Company recognized $170,500 as compensation expense related to the Options.

On August 13, 2013, the Company issued options to purchase 50,000 shares of common stock with an estimated fair value of $193,765 to an employee. These options vest quarterly in pro rata portions during the 3 years following the effective date of October 1, 2013. The Company valued these options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest rate of 1.49% (based on the U.S. 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 $5.00 per share. For the year ended December 31, 2013, the Company recognized $24,774 as compensation expense related to the Options.

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On September 9, 2013, the Company issued options to purchase 90,000 shares of common stock with an estimated fair value of $448,354 to two employees. The options vest quarterly in pro rata portions during the 3 years following the effective date of October 1, 2013. The Company valued these options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest rate of 1.71% (based on the U.S. 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 year ended December 31, 2013, the Company recognized $46,268 as compensation expense related to the Options.
On December 6, 2013 and December 9, 2013, the Company issued options to purchase 330,000 shares of common stock with an estimated fair value of $2,236,848 to six employees. The options vest quarterly in pro rata portions during the 3 years following the effective date of January 1, 2014. The Company valued these options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest rate of 1.51% (based on the U.S. 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 101.66% (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 and $8.10, respectively, per share. For the year ended December 31, 2013, the Company recognized $50,019 as compensation expense related to the Options.

On December 6, 2013, the Company issued options to purchase 51,000 shares of common stock with an estimated fair value of $350,085 to four Board of Directors. The options vest immediately. The Company valued these options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest rate of 1.51% (based on the U.S. 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 101.66% (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 year ended December 31, 2013, the Company recognized $350,085 as compensation expense related to the Options.

On December 16, 2013, the Company issued options (the "Options") to purchase 1,080,000 shares of common stock with an estimated fair value of $6,350,400 in connection with an employment agreement with Martin Shkreli (the "Shkreli Employment Agreement"). The options vest quarterly in pro rata portions during the 3 years following the effective date of December 31, 2013. The Company valued these Options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest rate of 1.55% (based on the U.S. 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 101.66% (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 $7.45 per share. For the year ended December 31, 2013, the Company recognized $529,200 as compensation expense related to the Options.

The following tables illustrates the Company's stock option issuances and balances outstanding as of, and during the years ended December 31, 2013 and 2012, respectively:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding at January 1, 2013	-	-	-	-
Granted	1,721,000	$ 7.66	-	$ -
Canceled	-	-	-	
Exercised	-	-	-	
Outstanding at December 31, 2013	1,721,000	$ 7.66	$ 9.89	$ 172,000
Exercisable at December 31, 2013	172,667	$ 7.85	$ 9.86	$ 14,333

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The weighted average grant date fair value of option granted during the year ended December 31, 2013 is $6.03 The aggregate intrinsic value of stock options outstanding and exercisable was calculated based on a closing stock price of $7 on December 31, 2013. Unrecognized compensation cost associated with unvested options amounts to $9, 213,338, as of December 31, 2013, which will be expensed over a weighted average remaining vesting period of 2.7 years.

Share based compensation

For the year ended December 31, 2013, the Company issued 359,000 shares to consultants. The weighted average fair value of the shares issued is $6.92. The Compensation expense for these shares was determined based on the closing market price of the Company's stock at the date of grant. Compensation expense for the year ended December 31, 2013 amounted to $2,485,875.

Stock Repurchases

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

NOTE 15. 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.

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. In December 2012, the employee was terminated and the shares were accordingly forfeited.

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 was granted 120,000 shares with an estimated fair value of $768,000 on May 20, 2013 of restricted common stock of the Company, a pro rata portion of which 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 with an estimated fair value of $75,000. The stock will vest quarterly in pro rata portions beginning September 30, 2013 during the 3 years following the grant date.

For the year ended December 31, 2013 and 2012, and for the period from March 11, 2011 (inception) through December 31, 2013, the Company recognized $986,575 ($253,682 to employees and $732,893 to non-employees), $22,410,222, and $25,295,054 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, 2013, there was approximately $1,105,967 of unrecognized compensation cost related to incentive shares issued. This amount is expected to be recognized over a weighted average of 2.19 years. The Company issued these shares with an initial vesting period of 2 to 3 years.

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	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
Granted	135,000	-	135,000	6.24
Vested	(36,724)	(139,069)	(175,793)	5.44
Forfeited	(20,833)	(37,500)	(58,333)	4.00
Unvested December 31, 2013	130,215	38,427	168,642	$6.44

From inception through September 2012, 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 16. INCOME TAXES

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

	2013	2012
Current		
Federal	$-	$-
State	-	-
	-	-
Deferred		
Federal	(6,293)	(1,173)
State	(3,435)	(733)
	(9,728)	(1,906)
Total	(9,728)	(1,906)
Change in valuation allowance	9,804	1,906
Income tax (benefit)	76	-
Total	$-	$-

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A reconciliation of the statutory federal income tax expense (benefit) to the effective tax rate is as follows:

	2013		2012	
Statutory rate - federal	-35.00	%	-35.00	%
State taxes, net of federal benefit	-6.70	%	-1.81	%
Change in FV of derivative liability (warrants)	10.46	%	0.00	%
Stock Based Compensation related to profits interest	2.30	%	9.52	%
Other	0.17	%	1.62	%
Partnership losses preceding conversion	0.00	%	19.39	%
Change in valuation allowance	29.00	%	6.28	%
Income tax provision (benefit)	0.23	%	0.00	%

The tax effects of "temporary differences" giving rise to deferred tax assets and liabilities as of December 31, 2013 and 2012 are as follows (in thousands):

	2013	2012
Net operating loss and capital loss carryforward	$11,498	$2,748
Intangible assets	(2,999)	(466)
Other	610	(376)
Valuation allowance	(11,710)	(1,906)
Total Deferred tax liability	$(2,601)	$-

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.

For the periods ended December 31, 2012 and 2013, the Company incurred net operating losses and, accordingly, no federal current provision for income taxes has been recorded. As a result of indefinite-lived intangibles that are not subject to amortization for tax purposes, the Company recorded a deferred provision for the period ended December 31, 2013. 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, 2013, the Company had approximately $25.3million of federal, state and local net operating losses. The Company's utilization of the net operating loss carryforwards may be subject to annual limitations due to the ownership change limitations provided by Internal Revenue Code (IRC) Section 382 and similar state provisions. Pursuant to IRC Section 382, the annual use of the Company’s net operating loss credit carryforwards may be limited in the event a cumulative change in ownership of more than 50% occurs within a three-year period. The annual limitations may result in the expiration of net operating losses and credits prior to utilization. The annual limitation is determined based upon the fair market value of the Company as of the date of such ownership change. Based on the value of the Company at all relevant dates, the computed annual limitation that would result from an ownership change of the Company is not expected to prevent us from utilizing our net operating losses prior to their expiration if we can generate sufficient taxable income to do so in the future. The net operating loss carry forwards, if not utilized, will begin to expire in 2032 for federal purposes. The Company recorded a deferred income tax provision of approximately $76,000 million in the year ended December 31, 2013 related to the different book and tax treatment for other intangible assets. For tax purposes, intangible assets are subject to different amortization allowances than for book purposes. Further, pursuant to the guidance in FASB ASC 740-10-55, the

Company has stepped-up the basis of its intangible assets by $2,525,124 and has recorded a deferred tax liability in the same amount, to account for the book/tax basis difference resulting from the Kyalin acquisition.

The Company files income tax returns in the U.S. federal jurisdiction and various state and local jurisdictions. The Company's income tax returns are open to examination by federal, state and foreign tax authorities, generally for the years ended December 31, 2011 and later. The Company has no amount recorded for any unrecognized tax benefits as of December 31, 2013 and 2012, nor did the Company record any amount for the implementation of ASC 740. The Company's policy is to record estimated interest and penalty related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision. During the years ended 2013 and 2012, the Company did not recognize any interest or penalties in its statements of operations and there are no accruals for interest or penalties at December 31, 2013 or 2012.

NOTE 17. SUBSEQUENT EVENTS

In accordance with ASC 855-10, Company management reviewed all material events through the date of this report.

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On February 28, 2014, the Company amended its lease agreement for its offices located in Carlsbad, CA. The Company increased its Carlsbad offices by approximately 3,800 square feet of office space for approximately $110,000 of additional annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes under a lease agreement expiring in June 2017.

Public Offering

On January 9, 2014, the Company completed a public offering of 4,705,882 shares of common stock at a price of $8.50 per share. The Company received net proceeds from the offering of $37,399,997, after deducting the underwriting discount and other estimated offering expenses.

Acquisition of Manchester Pharmaceuticals, LLC

On March 26, 2014, the Company acquired 100% of the outstanding membership interests of Manchester Pharmaceuticals, LLC (“Manchester” or “acquiree”), a privately-held specialty pharmaceutical company that focuses on treatments for rare diseases. The acquisition of Manchester expands the Company’s ability to address the special needs of patients with ultra-rare diseases.

Under the terms of the agreement, the Company paid $29.5 million upon closing, of which $3.2 million was paid by Retrophin Therapeutics International LLC, a newly formed indirect wholly owned subsidiary, for rights of product sales outside of the United States. The Company entered into a promissory note with Manchester principals for $33 million to be paid in three equal installments of $11 million within three, six, and nine months after closing. Additional contingent payments will be made based on product sales. The Company expects to raise additional funds through a public equity offering, a private equity offering, and/or debt financing to satisfy its short term obligations.

The financial statements of the acquiree are not practicable to prepare at the time of filing due to the acquiree being privately held and not maintaining financial statements in accordance with U.S. GAAP. The initial accounting for the business combination is not yet complete and the Company is still performing procedures to determine the appropriate accounting. As such, the Company is unable to make the following disclosures, (i) pro forma data, (ii) purchase price allocation, (iii) expenses of the acquisition, and (iv) revenue and earnings of the acquiree since the acquisition date.

Exercise of Warrants

Subsequent to year end, an aggregate of 798,391 warrants were exercised for a total of $3,830,316 in cash received by the Company.

Covered Short Sales

Subsequent to December 31, 2013, the Company purchased $1,019,456 in marketable securities of thirteen publicly traded companies to “cover” securities sold, not yet purchased held as of December 31, 2013. As of the date of this filing, the Company has realized a gain on securities sold, not yet purchased in the amount of $45,885.

Stock Repurchases

Subsequent to December 31, 2013, the Company repurchased 248,801 shares of its common stock for an aggregate purchase price of $2,257,336. The Company currently recognizes such repurchased common stock as treasury stock.

Consulting Agreements

On January 14, 2014, the Company entered into an agreement with a consultant to serve as an advisor to the Company. The Company granted 14,000 shares of common stock payable as follows (i) 3,500 shares of common stock issued on April 1, 2014, (ii) 3,500 shares of common stock issued on July 1, 2014, (iii) 3,500 shares of common stock issued on October 1, 2014, (iv) 3,500 shares of common stock issued on January 1, 2015. In addition, the Consultant shall receive $12,500 per month. The agreement expires on January 13, 2015.

On February 14, 2014, the Company entered into an agreement with a consultant to serve as an advisor to the Company. The Company granted 66,000 shares of common stock payable as follows (i) 16,500 shares of common stock issued on March 31, 2014, (ii) 16,500 shares of common stock issued on June 30, 2014, (iii) 16,500 shares of common stock issued on September 30, 2014, (iv) 16,500 shares of common stock issued on December 31, 2014. In addition, the Consultant shall receive $200,000 upon the execution of the agreement. The agreement expires on December 31, 2014.

In March 2014, the Company entered into an agreement with a consultant to serve as an advisor to the Company. In exchange for the services, the Company issued 200,000 shares of common stock.

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Bonus

On February 24, 2014, the Company' Board of Directors approved an aggregate cash bonus pool of $1,100,000 to officers and employees of record as of December 31, 2013.

Employee Equity Issuance

Subsequent to year end, the Company issued 400,000 shares of restricted common stock to three officers and 1,210,000 options to purchase shares of the Company's common stock to four officers and other employees of the Company.

Securities sold, not yet purchased

As of the date of this filing, the Company has $1,218,800 and $498,146 of securities sold not yet purchased and unrealized loss related to securities sold, not yet purchased, respectively.