As filed with the Securities and Exchange Commission on October 24, 2013

Registration No.

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

AMENDMENT NO. 2 TO FORM S-1 REGISTRATION STATEMENT UNDER THE SECURITIES ACT OF 1933

# **RETROPHIN, INC.**

(Exact name of registrant as specified in its charter)

Delaware (state or Other Jurisdiction of Incorporation or Organization) 2834 (Primary Standard Industrial Classification Code Number) 27-4842691 (I.R.S. Employer Identification Number)

777 Third Avenue, 22<sup>nd</sup> Floor, New York, NY 10017 (646) 837-5863

(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Martin Shkreli Chief Executive Officer 777 Third Avenue, 22<sup>nd</sup> Floor, New York, NY 10017 (646) 837-5863

(Name, address, including zip code, and telephone number including area code, of agent for service)

Copies of all communications, including communications sent to agent for service, should be sent to: Evan L. Greebel, Esq. Katten Muchin Rosenman LLP 575 Madison Avenue

New York, NY 10022 Tel.: (212) 940-6383

Approximate date of commencement of proposed sale to the public: From time to time after the effective date of this Registration Statement. If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933 check the following box. x

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

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 the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b2 of the Exchange Act.

Large accelerated filer  $\Box$ 

Accelerated filer  $\Box$ 

Non-accelerated filer

□ (Do not check if a smaller reporting company)

Smaller reporting company x

#### CALCULATION OF REGISTRATION FEE

Title of Securities to be Registered	Amount to be Registered (1)	Proposed Maximum Offering Price per Unit	Proposed Maximum Aggregate Offering Price (6)	Amount of Registration Fee (7)
Common Stock, par value \$0.0001 per share, offered by selling stockholders	9,147,014	\$4.83 (4)	\$44,180,077.62	\$6,026.16
Common Stock, par value \$0.0001 per share, underlying warrants held by selling stockholders	1,597,969 (2)	\$3.60 (5)	\$5,752,688.40	\$784.67
Common Stock, par value \$0.0001 per share, underlying warrants held by selling stockholders	2,867,235 (3)	\$6.00 (5)	\$17,136,738.00	\$2,337.45
Total	13,612,218		\$67,069,504.02	\$9,148.28

- (1) Pursuant to Rule 416 of the Securities Act of 1933, also registered hereby are such additional and indeterminable number of shares as may be issuable due to adjustments for changes resulting from stock dividends, stock splits and similar changes as well as anti-dilution provisions applicable to the warrants.
- (2) The 1,597,969 shares of common stock are being registered for resale by certain selling stockholders named in this registration statement, which shares are issuable by the registrant upon the exercise of the registrant's common stock purchase warrants issued on February 14, 2013.
- (3) The 2,867,235 shares of common stock are being registered for resale by certain selling stockholders named in this registration statement, which shares are issuable by the registrant upon the exercise of the registrant's common stock purchase warrants issued on August 15, 2013.
- (4) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(c) under the Securities Act of 1933, as amended. The calculation of the proposed maximum aggregate offering price of the common stock is based on the average of the bid and asked prices for the common stock as quoted on the OTC QB on September 11, 2013.
- (5) Estimated solely for the purpose of calculating the amount of the registration fee pursuant to Rule 457(g) of the Securities Act of 1933, as amended.
- (6) Estimated solely for the purpose of calculating the amount of the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.(7) Previously paid.
- The registrant hereby amends this registration statement on such date or dates as may be necessary to delay its effective date until the registrant shall file a further amendment which specifically states that this registration statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

**Preliminary Prospectus** 

Subject to Completion, Dated October 24, 2013

#### RETROPHIN, INC. 13,612,218 Shares of Common Stock

This prospectus relates to the sale of up to a total of 13,612,218 shares of common stock of Retrophin, Inc., a Delaware corporation, that may be sold from time to time by the selling stockholders named in this prospectus and their successors and assigns. The shares of common stock subject to this prospectus include: (i) 9,147,014 shares issued to certain selling stockholders, and (ii) 1,597,969 shares issuable upon the exercise by certain selling stockholders of our common stock purchase warrants issued on February 14, 2013 and 2,867,235 shares issuable upon the exercise by certain selling stockholders of our common stock purchase warrants issued on August 15, 2013, which we refer to herein as the "Warrants". The securities offered for resale hereby were issued, or are issuable, to the applicable selling stockholders in a private placement transaction completed prior to the filing of the registration statement of which this prospectus is a part.

The selling stockholders may sell all or a portion of their shares through public or private transactions at prevailing market prices or at privately negotiated prices. Information regarding the selling stockholders and the times and manner in which they may offer and sell the shares under this prospectus is provided under "Selling Stockholders" and "Plan of Distribution" in this prospectus. We have agreed to pay all the costs and expenses of this registration.

We will not receive any of the proceeds from the sale of shares by the selling stockholders. We may receive proceeds upon exercise of the Warrants, and any proceeds we receive will be used for general corporate purposes and for working capital.

Our common stock is listed for quotation on the OTC QB, under the symbol "RTRX". There is limited trading in our common stock. On October 23, 2013, the most recent day that our stock traded, the last reported price per share of our common stock was \$7.20. You are urged to obtain current market quotations of our common stock before purchasing any of the shares being offered for sale pursuant to this prospectus.

We are an "emerging growth company" under federal securities laws and are subject to reduced public company disclosure standards. See "Prospectus Summary — Implications of Being an Emerging Growth Company."

An investment in our securities is highly speculative, involves a high degree of risk and should be considered only by persons who can afford the loss of their entire investment. See "Risk Factors" beginning on page 4 of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The date of this prospectus is , 2013.

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

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

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#### PROSPECTUS SUMMARY

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

In this prospectus, unless the context requires otherwise, the terms "we", "our", "us", "Retrophin" and the "Company" refer to Retrophin, Inc., a Delaware corporation formerly known as Desert Gateway, Inc. ("Desert Gateway"), as well as our direct and indirect subsidiaries. References to "dollars" and "\$" are to United States dollars.

#### **Our Business**

#### Overview

We are a development stage company focused on developing pharmaceutical products primarily for the treatment of rare diseases. Our lead product in development, RE-021, a small molecule intended to treat focal segmental glomerulosclerosis (or "FSGS"), a disease that causes nephrotic syndrome and kidney failure, has completed Phase 2 clinical studies demonstrating safety and efficacy, and we expect to initiate a Phase 2 clinical study in 2013. We also have a number of programs in preclinical development. Our second most developed program (RE-024) for the treatment of pantothenate kinase-associated neurodegeneration ("PKAN"), a degenerative disease of the brain that primarily affects young children due to their inability to process phosphopantothenic acid, is in preclinical testing, and we will seek to initiate clinical trials of this product candidate as soon as is practical. We are also developing a treatment for Duchenne muscular dystrophy ("DMD"). Our focus is to seek treatment for serious, unmet, rare diseases. The majority of diseases on which we focus are considered "orphan" diseases because they affect fewer than 200,000 patients in the United States. However, such diseases have a profound impact on those that suffer from them and on their families. Currently, we believe that we are the only company that is focusing on developing treatments for these rare and ultra-rare diseases.

Our predecessor, former Retrophin, was formed as a Delaware limited liability company on March 11, 2011 and was converted to a Delaware corporation on September 20, 2012. Former Retrophin was a developmental stage biopharmaceutical company focused on the development and commercialization of therapies for catastrophic diseases.

#### **Organizational Background**

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

On December 12, 2012, Desert Gateway completed 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".

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, former Retrophin, with the Company continuing as the surviving corporation following the merger. On April 1, 2013, the Board of Directors of the Company determined to change the Company's fiscal year from a fiscal year ending in February of each year to a fiscal year ending on December 31 of each year.

Our principal offices are located at 777 Third Avenue, 22<sup>nd</sup> Floor, New York, NY 10017. Our telephone number is (646) 837-5863.



#### **Our Strategy**

Our goal is to become a leading biopharmaceutical company specializing in the development and commercialization of therapies for catastrophic diseases. Our commercialization strategy is to acquire pharmaceutical products for serious diseases and greatly increase patient and physician awareness to increase market penetration. Our development strategy is to focus on product opportunities which can take advantage of the shorter regulatory cycles that can be achieved with treatments for rare, life-threatening diseases. Beyond FSGS, PKAN and DMD, Retrophin has plans to discover and develop drug candidates for other orphan diseases, which may include cystic fibrosis and spinal muscular atrophy.

#### **Implications of Being an Emerging Growth Company**

The Company is an "emerging growth company" as defined in the Jumpstart Our Business Startups Act, or "JOBS Act." For as long as the Company is an emerging growth company, unlike other public companies, it will not be required to:

- provide an auditor's attestation report on management's assessment of the effectiveness of our system of internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002;
- comply with any new requirements adopted by the Public Company Accounting Oversight Board, or the PCAOB, requiring mandatory audit firm rotation or a supplement to the auditor's report in which the auditor would be required to provide additional information about the audit and the financial statements of the issuer;
- comply with any new audit rules adopted by the PCAOB after April 5, 2012, unless the SEC determines otherwise;
- · provide certain disclosure regarding executive compensation required of larger public companies; or
- obtain shareholder approval of any golden parachute payments not previously approved.

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

In addition, Section 107 of the JOBS Act also provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. In other words, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to take advantage of the benefits of this extended transition period.

	The Offering
Common stock offered by the selling stockholders:	Aggregate of 13,612,218 shares, consisting of 9,147,014 shares held by the selling stockholders, up to 1,597,969 shares underlying Warrants held by certain selling stockholders exercisable at \$3.60 per share (subject to adjustment) and expiring on the five year anniversary of the date of issuance, and up to 2,867,235 shares underlying Warrants held by certain selling stockholders exercisable at \$6.00 per share (subject to adjustment) and expiring on the five year anniversary of the five year anniversary of the date of issuance.
OTC QB Symbol:	RTRX. A limited market for the purchase and sale of our common stock currently exists.
Use of proceeds:	We will not receive any proceeds from the sale of the common stock offered hereby. However, we may receive up to a maximum of approximately \$22,956,098 of gross proceeds from the exercise of Warrants by selling stockholders, which proceeds we would expect to use for general working capital. No assurances can be given, however, that all or any portion of such warrants will ever be exercised.
Risk factors:	Investing in our securities involves a high degree of risk. As an investor you should be able to bear a complete loss of your investment. You should carefully consider the information set forth in the section of this prospectus entitled "Risk Factors."

#### **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 state and have not generated any revenues.

From inception through June 30, 2013, we have incurred net losses of approximately \$43.40 million and negative cash flows from operating activities of approximately \$10.46 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, it may never be profitable and, if it does become profitable, it may be unable to sustain profitability.

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

Since inception, we have incurred significant operating losses. Our net loss attributable to common stockholders was \$30.34 million for the year ended December 31, 2012. As of June 30, 2013 we had an accumulated deficit of \$43.40 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:

- · begin Phase 2 clinical development of RE-021 for the treatment of FSGS;
- continue our ongoing preclinical development of RE-024 for the treatment of PKAN, and potentially begin clinical trials of RE-024;
- 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 RE-021, RE-024, RE-001 and additional product candidates;
- establish a sales and marketing infrastructure to commercialize products for which we may obtain regulatory approval; and
- add operational, financial, and management information systems and personnel, including personnel to support of 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 large 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 would 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 RE-021, developing a viable manufacturing route for RE-001, planning pre-clinical studies and limited clinical studies of RE-001 and RE-024. 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. Consequently, any predictions you make about our future success or viability may not be as accurate as they would be if we had a longer operating history.

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

There can be no assurance that revenues from product sales will ever be achieved. 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 Federal Drug Administration (the "FDA"), selling and manufacturing these products, completing development of its 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.

# 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 business risk of exposure to significant product liability and other claims in the event that the use of our products cause, or is alleged to have caused, adverse effects. Furthermore, our products may cause, or may appear to have caused, adverse side effects (including death) or potentially dangerous drug interactions that we may not learn about or understand fully until the drug has been administered to patients for some time. The withdrawal of a product following complaints and/or incurring significant costs, including the requirement to pay substantial damages in personal injury cases or product liability cases, 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. We do not currently maintain product liability insurance, and will be required to put into place such insurance prior to commencing clinical trials or marketing and selling products. We are currently in the process of obtaining product liability insurance coverage of \$5,000,000 for our planned FSGS and PKAN clinical trials. Even once we have product liability insurance in place, such product liability insurance coverage may not be sufficient to cover our claims and we may not be able to obtain sufficient coverage at a reasonable cost in the future.

# 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.

#### 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 (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.

### 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 2 clinical study of RE-021, and as we continue toward Phase 1 clinical studies of RE-001 and RE-024, 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.

On September 18, 2013, we made a proposal to the board of directors of Transcept Pharmaceuticals, Inc. ("<u>Transcept</u>") to acquire all of the outstanding shares of Transcept's common stock for \$4.00 per share in cash. Although Transcept's board of directors rejected our proposal, we informed Transcept that we will be willing to continue to discuss the proposal. If Transcept accepts our proposal to acquire all of its outstanding shares of common stock, we will need to obtain additional equity or debt financing to consummate the acquisition and consolidation.

We believe that our existing cash and cash equivalents and marketable securities, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements until at least the first quarter of 2014. Additional funds may not be available to Retrophin when we need them on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.



Our future capital requirements will depend on many factors, including:

- the progress and results of our pre-clinical and clinical studies of RE-021, RE-001, RE-024, and other drug candidates;
- the costs, timing and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and
- · our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

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

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

# Our management has identified internal control deficiencies, which our management believes constitute material weaknesses. Any future material weaknesses or deficiencies in our internal control over financial reporting could harm stockholder and business confidence on our financial reporting, our ability to obtain financing and other aspects of our business.

In connection with the preparation of our audited financial statements for the period from March 11, 2011 (inception) through December 31, 2011 and the year ended December 31, 2012, our independent auditors advised management that a material weakness existed in internal control over financial reporting and our disclosure controls. Although we are committed to continuing to improve our internal control processes, and although we will continue to diligently and vigorously review our internal control over financial reporting, any control system, regardless of how well designed, operated and evaluated, can provide only reasonable, not absolute, assurance that its objectives will be met. Therefore, we cannot be certain that, in the future, additional material weaknesses or significant deficiencies will not exist or otherwise be discovered. If our efforts to address the weakness identified are not successful, or if other deficiencies occur, these weaknesses or deficiencies could result in misstatements of our results of operations, restatements of our financial statements, a decline in our stock price and investor confidence or other material effects on our business, reputation, results of operations, financial condition or liquidity.

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

The Independent Registered Public Accounting Firm's Report issued in connection with our audited financial statements for the period from March 11, 2011 (inception) through December 31, 2011 and the year ended December 31, 2012 stated that "the Company, as a development stage enterprise, is subject to risks and uncertainties as to whether it will be able to raise capital and commence its planned operations. These conditions raise substantial doubt about the Company's ability to continue as a going concern." Because we have been issued an opinion by our auditors that substantial doubt exists as to whether it can continue as a going concern, it may be more difficult to attract investors.

# If we are 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. Currently we have no patent protection on RE-001. We have composition of matter patents on RE-021 that were filed in July 1999, and we have filed a patent application on RE-024 in April 2012. We expect that in addition to the protection afforded by our patent filings that we will be able to extend our intellectual protection, by up to five years, via the provisions of the Hatch-Waxman Act.

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

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

We have negotiated a license agreement 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 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, and we cannot be certain 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 RE-021 we may not be able to stop competitors from marketing similar products.

We have filed a patent application in the United States on the composition of RE-024 as a treatment for pantothenate kinase associated neurodegeneration. We cannot be certain that this application will be granted, or that the claims we have made will be allowed by the patent office. Further, we have not filed for patent protection outside of the United States for RE-024. We cannot be certain that we will filed for patent protection outside the United States, or that even if we do any patents(s) will be granted.

We are in the process of licensing patents and patent applications on the core technology of RE-001 from both the University of Minnesota and the University of Wisconsin. We cannot be certain that we will be successful in securing these rights and we cannot be certain that we will be able to obtain these rights with beneficial terms. We also cannot be certain that a competing organization will obtain rights to these patents and applications. To the best of our knowledge, patent protection for the technology on which our lead compound, RE-001 is based have not been filed outside of the United States. We cannot be certain when or if we will file for patent protection outside of the United States, and we cannot be certain if we would be successful in obtaining these patents or if we will be able to enforce these patents. If patents are not issued in respect of our pending patent applications, we may not be able to stop competitors from marketing similar products in Europe and other countries in which we do not have issued patents.

We currently have no issued patents or pending applications covering methods of using or composition of RE-001 outside of the United States. We intend to seek orphan medicinal product designation and to rely on statutory data exclusivity provisions in jurisdictions outside the United States where such protections are available, including Europe. The patent rights that we are seeking to license relating to our product candidates are limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates.

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 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.

### 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 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 RE-021, 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 agreement with Ligand Pharmaceuticals or other third party licensors, and we cannot be certain that we will be able to maintain these rights with beneficial terms. Under our license agreement with Ligand Pharmaceuticals, composition of matter patents for the technology used in RE-021, are set to expire in 2019.

# 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.

# 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. If any third party patents were to be asserted against us we do not believe that our proposed products would be found to infringe any valid claim of these patents. If we were to challenge the validity of any issued United States patent in court, we would need to overcome a presumption of validity that attaches to every patent. This burden is high and would require us to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on infringement or validity.

In order to avoid or settle potential claims with respect to any of the patent rights described above or any other patent rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or our future collaborators were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. This could harm our business significantly.

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

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

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

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

### 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 FSGS and DMD indication 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 DMD, PKAN, and 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. 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.

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.

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. 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 its proposed products, new developments are expected to continue and there can be no assurance that discoveries by others will not render our potential products noncompetitive.

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

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

We do not own or operate manufacturing facilities for clinical or commercial production of our products. We have limited personnel with experience in drug manufacturing and we lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We outsource all manufacturing and packaging of our preclinical, clinical, and commercial products to third parties. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production and in maintaining required quality control. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate.

We do not currently have any agreements with third party manufacturers for the long-term commercial supply of any of our development stage product candidates. We may be unable to enter into agreements for commercial supply with third party manufacturers, or may be unable to do so on acceptable terms. Even if we enter into these agreements, the manufacturers of each product candidate will be single source suppliers to us for a significant period of time.

Reliance on third party manufacturers entails risks to which we may not be subject if we manufactured our product candidates or products ourselves, including:

- · reliance on the third party for regulatory compliance and quality assurance;
- · limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- · impact on our reputation in the marketplace if manufacturers of our products fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

Our contract manufacturers will be required to adhere to FDA regulations setting forth current good manufacturing processes (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 products candidates. In addition, if we are not able to obtain adequate supplies of our products 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.

### 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 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 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 busi

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

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

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

- we may not be able to control the amount and timing of resources that our distributors may devote to the commercialization of our product candidates;
- · our distributors may experience financial difficulties;
- business combinations or significant changes in a distributor's business strategy may also adversely affect a distributor's willingness or ability to complete its obligations under any arrangement; and
- these arrangements are often terminated or allowed to expire, which could interrupt the marketing and sales of a product and decrease our revenue.

### If material 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 products 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.

# 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 Medical Officer and Chief Financial Officer and are currently negotiating an employment agreement with our Chief Executive 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 15 full-time employees and five consultants that provide significant support and assistance to us as of the date of the Merger. Of these employees and consultants, eight work primarily in research and development and one provides 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. Assuming our plans and business conditions progress consistent with our current projections, we plan to grow to a total of 30 employees by the end of 2013. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

In the event that we attempt to acquire or develop our own in-house sales, marketing and distribution capabilities, factors that may inhibit our efforts to commercialize our products without strategic partners or licensees include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe our products;

the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;

- unforeseen costs associated with creating our own sales and marketing team or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

#### Risks Related to the Development and Commercialization of Our Product Candidates

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

We are engaged in the licensing and marketing of drugs for rare diseases. Specifically, we have licensed rights to 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, from Ligand Pharmaceuticals, and will be required to make future milestone and royalty payments to Ligand and Bristol Myers Squibb.

We are engaged in the development of new drugs, which is characterized by extensive research efforts and rapid technological progress. There can be no assurance that research and discoveries by others will not render our discovery programs noncompetitive or obsolete.

We will also depend on the success of our early product candidates RE-021, RE-024 and RE-001. RE-021 has not completed any clinical studies for the treatment of FSGS, and RE-001 and RE-024 are still in pre-clinical development. Clinical trials of our RE-021, RE-024 or RE-001 or subsequent product candidates may not be successful. If we are unable to commercialize RE-021, RE-024 or RE-001, or experience significant delays in doing so, our business may be materially harmed.

We have invested a significant portion of our efforts and financial resources in the development of our most advanced product candidates, RE-021, RE-024 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 RE-001, RE-021, RE-024, and subsequent product candidates for completion of our clinical trials on a timely basis;
- · successful completion of pre-clinical and clinical studies;
- obtaining marketing approvals from the FDA and similar regulatory authorities outside the United States;
- establishing commercial-scale manufacturing arrangements with third party manufacturers whose manufacturing facilities are operated in compliance with current good manufacturing practice, or 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, then our revenues may be adversely affected and our business may suffer.

A majority of the diseases that our current and future product candidates are being developed to address 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, and our assumptions on pricing are based on estimates.

Currently, most reported estimates of the prevalence of FSGS, PKAN 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 FSGS, PKAN or DMD, or in the study populations accurately reflect the prevalence of these diseases in the broader world population. If our estimates of the prevalence of FSGS, PKAN or DMD or of the number of patients who may benefit from treatment with RE-021, RE-024 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.

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 RE-021, RE-024 and RE-001---if they receive marketing approval ---may not gain market acceptance by physicians, patients, third party payors, and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;
- · relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- · publicity concerning our products or competing products and treatments; and
- · sufficient third party insurance coverage or reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate patients, the medical community, and third party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors.

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

We will only obtain regulatory approval to commercialize product candidates if we can demonstrate to the satisfaction of the FDA, or applicable non-United States regulatory authorities, in well-designed and conducted clinical trials, that our product candidates are safe and effective and otherwise meets 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 all 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 begun preclinical 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. Further, 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. We cannot assure you that any future clinical trials of RE-001, RE-021, or RE-024 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 FSGS, PKAN 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 nor commercialized this or any other product candidates. We are currently planning pre-clinical and eventual clinical studies for, RE-021, RE-024 and RE-001. We filed an IND for the FSGS indication on July 2, 2012 and have received FDA clearance to begin a clinical study of RE-021 in FSGS, but have not filed INDs for RE-024 or RE-001. We cannot be certain that we will ever file INDs for either 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.

Our lead development product candidates are intended to treat FSGS, PKAN and DMD, which are rare diseases. Given that our lead development candidates are in the early stages of required testing, we may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible patients willing and able to participate in the clinical trials required by the FDA or other non-United States regulatory agencies. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

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

Before obtaining regulatory approval for the sale of our product candidates, we must conduct, at our own expense, extensive preclinical and clinical tests to demonstrate the safety of our product candidates in animals 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 or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- conditions imposed on us by the FDA or any non-United States regulatory authority regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;

- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;
- our third party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- we might have to suspend or terminate one or more of our clinical trials if we, regulators or institutional review boards determine that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and
- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;
- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; and
- have the product removed from the market after obtaining marketing approval.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products or product candidates.

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

We face an inherent risk of product liability exposure related the testing of our product candidates in human clinical trials. We will face an even greater risk if we obtain new products for sales 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.

# 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 con

#### 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 the 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 be also be complex and time-consuming and, if such businesses, products and assets are not successfully integrated, we may not achieve the anticipated benefits, cost-savings or growth opportunities. Potential difficulties that may be encountered in the integration process include the following:

- integrating personnel, operations and systems, while maintaining focus on producing and delivering consistent, high quality products;
- · coordinating geographically dispersed organizations;
- · distracting employees from operations;
- · retaining existing customers and attracting new customers; and
- · managing inefficiencies associated with integrating the operations of the Company.

Furthermore, these acquisitions and other arrangements, even if successfully integrated, may fail to further our business strategy as anticipated, expose us to increased competition or challenges with respect to our products or geographic markets, and expose us to additional liabilities associated with an acquired business, product, technology or other asset or arrangement. Any one of these challenges or risks could impair our ability to realize any benefit from our acquisition or arrangement after we have expended resources on them.

#### **Risks Related to Regulatory Approval of Our Product Candidates**

### 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 commercial products 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 candidate and the activities associated with its 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 inspection of manufacturing facilities by, the FDA. Our future products may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

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

- our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable regulatory authorities for approval;
- our inability to demonstrate that a product candidate's benefits outweigh its risks;
- our inability to demonstrate that the product candidate presents an advantage over existing therapies;
- the FDA's or comparable regulatory authorities' disagreement with the manner in which we interpret the data from preclinical studies or clinical trials;
- the FDA's or comparable regulatory authorities' failure to approve the manufacturing processes, quality procedures or manufacturing facilities of third party manufacturers with which we contract for clinical or commercial supplies; and
- a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.

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

# Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.

Undesirable side effects caused by our product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product candidates and generating revenues from their sale.

In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may require the addition of restrictive labeling statements;
- · regulatory authorities may withdraw their approval of the product; and
- we may be required to change the way the product is administered or conduct additional clinical trials.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

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

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. We expect to seek orphan drug designations from the FDA for, RE-021, RE-024 and RE-001though there can be no assurance that the FDA will grant orphan status. We also expect to seek drug designation from the European Medicines Agency (the "EMEA"), for RE-021, RE-024 and RE-001, and there can be no assurance that we will be successful. 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-021, RE-024 and RE-001 may be important to the product candidate's success. Even if we obtain orphan drug exclusivity for, RE-021 for FSGS, RE-024 for PKAN and RE-001 for DMD we may not be able to maintain it. For example, if a competitive product that treats 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.

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

- · restrictions on such products, manufacturers or manufacturing processes;
- warning letters;
- withdrawal of the products from the market;
- · refusal to approve pending applications or supplements to approved applications that we submit;
- voluntary or mandatory recall;
- fines;

- suspension or withdrawal of regulatory approvals or refusal to approve pending applications or supplements to approved applications that we submit;
- · refusal to permit the import or export of our products;
- · product seizure or detentions;
- · injunctions or the imposition of civil or criminal penalties; and
- adverse publicity.

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

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

The business and financial condition of healthcare related businesses will continue to be affected by efforts of governments and third party payors to contain or reduce the cost of healthcare through various means. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval for RE-021, RE-024, RE-001 or any other product candidate that we develop, restrict or regulate post-approval activities and affect our ability to profitably sell RE-021, RE-024, RE-001 or any other product candidate for which it obtains 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.

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 payors 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.



#### **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 or our directors, is our largest stockholder. Together with other entities that he controls, Mr. Shkreli controls 3,213,299 shares of our common stock, or approximately 18% 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.

#### An active and visible trading market for our common stock may not develop.

We cannot predict whether an active market for shares of our common stock will develop in the future. If an active market for our common stock does not develop, it may be difficult for you to sell shares without depressing the market price for our common stock. In the absence of an active trading market:

- · investors may have difficulty buying and selling or obtaining market quotations;
- · market visibility for shares of our common stock may be limited; and
- a lack of visibility for shares of our common stock may have a depressive effect on the market price for shares of our common stock.

The OTC Market is an unorganized, inter-dealer, over-the-counter market that provides significantly less liquidity than, for example, NASDAQ or the NYSE AMEX. The trading price of our common stock is expected to be subject to significant fluctuations in response to variations in quarterly operating results, changes in analysts' earnings estimates, announcements of innovations by us or our competitors, general conditions in the industry in which we operate and other factors. These fluctuations, as well as general economic and market conditions, may have a material or adverse effect on the market price of shares of our common stock.

### Our common stock may be considered a "penny stock," and thereby be subject to additional sale and trading regulations that may make it more difficult to sell.

Our common stock may be deemed a "penny stock" (as that term is defined under Rule 3a51-1 of the Securities Exchange Act of 1934, as amended (the "Exchange Act")) in any market that may develop in the future. Generally, a "penny stock" is a common stock that is not listed on a securities exchange and trades for less than \$5.00 a share. Prices often are not available to buyers and sellers and the market may be very limited. Penny stocks in start-up companies are among the riskiest equity investments. Broker-dealers who sell penny stocks must provide purchasers of these stocks with a standardized risk-disclosure document prepared by the SEC. The document provides information about penny stocks and the nature and level of risks involved in investing in the penny stock market. A broker must also provide purchasers with bid and offer quotations and information regarding broker and salesperson compensation and make a written determination that the penny stock is a suitable investment for the purchaser and obtain the purchaser's written agreement to the purchase. Many brokers choose not to participate in penny stock transactions. Because of the penny stock rules, there may be less trading activity in penny stocks in any market that develops for our common stock in the future and stockholders are likely to have difficulty selling their shares.

#### The market price for shares of our common stock may be volatile and purchasers of our common stock could incur substantial losses.

The price of our stock is likely to be volatile. The stock market in general, and the market for biotechnology companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- · results of clinical trials of our product candidates or those of our competitors;
- our entry into or the loss of a significant collaboration;
- regulatory or legal developments in the United States and other countries, including changes in the health care payment systems;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;
- general economic, industry and market conditions;
- · results of clinical trials conducted by others on drugs that would compete with our product candidates;
- · developments or disputes concerning patents or other proprietary rights;
- · public concern over our product candidates or any products approved in the future;
- litigation;
- · future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this "Risk Factors" section.

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

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

### 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.

# A significant portion of our total outstanding shares of common stock is restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock.

Rule 144 under the Securities Act, which permits the resale, subject to various terms and conditions, of limited amounts of restricted securities after they have been held for six months will not immediately apply to our common stock because we were at one time designated as a "shell company" under SEC regulations. Pursuant to Rule 144(i), securities issued by a current or former shell company that otherwise meet the holding period and other requirements of Rule 144 nevertheless cannot be sold in reliance on Rule 144 until one year after the date on which the issuer filed current "Form 10 information" (as defined in Rule 144(i)) with the SEC reflecting that it ceased being a shell company, and provided that at the time of a proposed sale pursuant to Rule 144, the issuer has satisfied certain reporting requirements under the Exchange Act. We believe this requirement to file Form 10 information has been satisfied by the filing of this report on Form 8-K. Because, as a former shell company, the reporting requirements of Rule 144(i) will apply regardless of holding period, the restrictive legends on certificates for the shares of common stock issued in the Merger cannot be removed except in connection with an actual sale that is subject to an effective registration statement under, or an applicable exemption from the registration requirements of, the Securities Act.

Additional risks may exist as a result of our becoming a public reporting company through a "reverse merger." Certain SEC rules are more restrictive when applied to reverse merger companies, such as the ability of stockholders to re-sell their shares of common stock pursuant to Rule 144. In addition, securities analysts of major brokerage firms may not provide coverage of our capital stock or business. Because we became a public reporting operating company through a reverse merger, there is no incentive to brokerage firms to recommend the purchase of our common stock. We cannot assure you that brokerage firms will want to provide analyst coverage of our capital stock or business in the future.

The sale, or availability for sale, of our common stock in the public market may adversely affect the prevailing market price of our common stock and may impair our ability to raise additional capital by selling equity or equity-linked securities.

# 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.

#### We will incur increased costs as a result of being a public company.

As a public company, we will incur significant legal, accounting, reporting and other expenses that we did not incur as a private company, including costs related to compliance with the regulations of the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act"). We expect these rules and regulations to increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We also expect these new rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, we may experience more difficulty attracting and retaining qualified individuals to serve on our board of directors or as executive officers. We cannot predict or estimate the amount of additional costs we may incur as a result of these requirements or the timing of such costs.

# If we fail to maintain proper and effective internal controls, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, our ability to operate our business and investors' views of us.

We will be required to comply with Section 404 of 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, if our efforts to comply with new or changed laws, regulations, and standards differ from the activities intended by regulatory or governing bodies due to ambiguities related to practice, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

# We have considerable discretion in the use of proceeds from the selling stockholders' exercise of the Warrants, and we may use these proceeds in ways with which you may not agree.

Our Board of Directors and our management will have considerable discretion in the application of the net proceeds received by us from the selling stockholders' exercise of the Warrants. You will not have the opportunity, as part of your investment decision, to assess whether proceeds are being used appropriately. You must rely on the judgment of our Board of Directors and our management regarding the application of the net proceeds of this offering. The net proceeds may be used for corporate purposes that do not improve our efforts to maintain profitability or increase the price of our securities. The net proceeds from this offering may be placed in investments that do not produce income or that lose value. See "Use of Proceeds."

# 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.

#### CAUTIONARY NOTE ON FORWARD LOOKING STATEMENTS

Certain information contained in this prospectus include forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The statements herein which are not historical reflect our current expectations and projections about the 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 forwardlooking 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 "project" 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 prospectus generally. In light of these risks and uncertainties, there can be no assurance that the forward-looking statements contained in this prospectus will in fact occur. Potential investors should not place undue reliance on any forward-looking statements. Except as expressly required by the federal securities laws, there is no undertaking to publicly update or revise any forward-looking statements, whether as a result of new information, future events, changed circumstances or any other reason.

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

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

#### **USE OF PROCEEDS**

The selling security holders will receive all of the proceeds from the sale of shares of common stock pursuant to this prospectus. We will not receive any proceeds from the sale of the shares of common stock offered by the selling security holders to the public. However, we will receive proceeds from the exercise of the Warrants. If all of the Warrants issued on February 14, 2013 are exercised at the initial exercise price of \$3.60 per share (which exercise price is subject to adjustment under customary anti-dilution protections) and all of the Warrants issued on August 15, 2013 are exercised at the initial exercise price of \$6.00 per share (which exercise price is subject to adjustment under customary anti-dilution protections), then we will receive gross proceeds of approximately \$22,956,098. Any such proceeds will be used for working capital and general corporate purposes of the Company. No assurance can be given, however, that all or any portion of such Warrants will be exercised.

#### DETERMINATION OF OFFERING PRICE

The selling stockholders may sell these shares in the over-the-counter market or otherwise, at market prices prevailing at the time of sale, at prices related to the prevailing market price, or at negotiated prices. We will not receive any proceeds from the sale of shares by the selling stockholders but we may receive proceeds upon the conversion or exercise, if any, of the Notes and the Warrants.

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

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

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 (the "<u>Original Filing</u>"), 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 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.

#### Overview

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.

#### **Results of Operations**

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

### **Operating Expenses**

### For the period March 11, 2011 (inception) through December 31, 2011

Operating expenses were approximately \$3.27 million for the period from March 11, 2011 through December 31, 2011, which consisted of (i) compensation and related costs of approximately \$2.23 million which included approximately 431,000 shares of vested incentive shares granted to members and employees amounting to approximately \$1.72 million, (ii) professional fees of approximately \$0.91 million which included (a) approximately 60,000 shares of vested incentive shares granted to consultants amounting to approximately \$0.26 million for services rendered; (b) research and development fees of approximately \$0.35 million related to Retrophin's drug (RE-001) candidate for the treatment of Duchenne Muscular Dystrophy; (c) legal expense of approximately \$0.10 million related to formation of the company, employment and consulting agreements and general corporate work; and (d) consulting fees of approximately \$0.20 million related to outsourcing management roles, (iii) nine months rent expense of approximately \$0.06 million, and (iv) the remaining balance of \$0.07 million is related to travel and entertainment, depreciation, advertising and other operating expenses.

# For the year ended December 31, 2012

Operating expenses were approximately \$30.26 million for the year ended December 31, 2012, which consisted of (i) compensation and related costs of approximately \$18.13 million which included approximately 2,048,000 shares of vested incentive shares granted to members and employees amounting to approximately \$16.01 million, (ii) professional fees of approximately \$9.04 million which included (a) approximately 194,000 shares of vested incentive shares granted to consultants and direct transfers of shares to consultants by members amounting to approximately \$6.40 million for services rendered; (b) research and development fees of approximately \$0.52 million related to Retrophin's drug (RE-021 and RE-024) candidate for the treatment of FSGS and PKAN and evaluation of potential new technologies; (c) legal expense of approximately \$0.91 million related to licensing and production acquisition, employment and consulting agreements and general corporate work; (d) consulting fees of approximately \$0.83 million related to general accounting and audit work, (iii) twelve months rent expense of approximately \$0.1 million, (iv) license fee of approximately \$0.26 million, (v) depreciation and amortization expense of approximately \$0.12 million related to the Ligand licensing agreement, (vi) bad debt expense of \$0.56 million and (vii) the remaining balance of \$0.61 million is related to travel and entertainment, advertising and other operating expenses.

### For the period March 11, 2011 (inception) through December 31, 2012

Operating expenses were approximately \$33.52 million during the period from March 11, 2011 through December 31, 2012. The largest factors impacting our operating expenses during the period related compensation and related costs of approximately \$20.36 million and \$9.94 million in professional fees which included stock base compensation of approximately \$24.39 million, consisting of approximately 2,479,000 shares of vested incentive shares granted to members and employees amounting to approximately \$17.74 million and approximately 254,000 shares of vested incentive shares granted to consultants and direct transfers of shares to consultants by members amounting to approximately \$6.65 million for services rendered. Operating expenses also included rent expenses of approximately \$0.16 million, depreciation and amortization expenses of approximately \$0.13 million, license fee of approximately \$1.70 million, bad debt expense of \$0.56 million and travel and entertainment, other expenses and advertising fees of approximately \$0.67 million.

### For the three month period ended June 30, 2013 compared to the three month period ended June 30, 2012

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

Our operating expenses for the three month period ended June 30, 2013 were \$5,099,902 compared to \$4,739,692 for the three month period ended June 30, 2012 which represents an increase of \$360,210. The expense increase was attributable to a decrease in our compensation and related costs in the amount of \$1,040,812, a decrease in our professional fees in the amount of \$1,361,197, offset by an increase in our selling, general and administrative costs in the amount of \$2,762,219. Included in selling, general and administrative costs are settlement charges in the amount of \$2,284,511.

# For the six month period ended June 30, 2013 compared to the six month period ended June 30, 2012

We had no revenues during the six month period ended June 30, 2013 and 2012.

Our operating expenses for the six month period ended June 30, 2013 were \$7,350,386 compared to \$8,298,146 for the six month period ended June 30, 2012 which represents a decrease of \$947,760. The expense decrease was attributable to a decrease in our compensation and related costs in the amount of \$1,765,356, a decrease in our professional fees in the amount of \$2,377,049, offset by an increase in our selling, general and administrative costs in the amount of \$3,094,645 and an increase in our technology license contingent fee in the amount of \$100,000. Included in selling, general and administrative costs are settlement charges in the amount of \$2,284,511.

### Other Operating Expenses

Other operating expenses for the period March 11, 2011 (inception) through December 31, 2011, for the year ended December 31, 2012, and for the period March 11, 2011 (inception) through December 31, 2012 were as follows: (i) approximately \$0.005 million, \$0.003 and \$0.008 million, respectively which is related to a loss in foreign exchange in a vendor payment, (ii) approximately zero, \$0.022 million and \$0.022 million, respectively which related to \$.2 million note receivable with an interest rate of 12% per annum offset by approximately zero, \$0.106 million and \$0.106 million respectively of interest expense relate to a \$0.900 million and \$0.030 note payable with an interest rate of 12% and 15%, respectively, per annum.

Other income for the three month period ended June 30, 2013 was \$51,389 compared to other expense of \$20,487 for the three month period ended June 30, 2012 which represents an decrease of \$71,876. The decrease was primarily attributable to gain from the change in fair value of derivative financial instruments of \$56,041, a decrease in interest income of \$5,979, an increase in loss on transactions denominated in foreign currencies of \$4,657, offset by a decrease in interest expense of \$26,471.

Other expense for the six month period ended June 30, 2013 was \$2,441,049 compared to \$34,066 for the six month period ended June 30, 2012 which represents an increase of \$2,406,983. The expense increase was primarily attributable to the expense from the change in fair value of derivative financial instruments of \$2,395,618, a decrease in interest income of \$9,727, an increase in loss on transactions denominated in foreign currencies of \$3,873, offset by a decrease in interest expense of \$2,235.

#### Income Taxes

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

# 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.

# Net Loss

For the period March 11, 2011 (inception) through December 31, 2011, for the year ended December 31, 2012 and for the period March 11, 2011 (inception) through December 31, 2012, our net loss from operation were approximately \$3.27 million, \$30.26 million and \$33.52 million, respectively. Our net loss for the six month period ended June 30, 2013 was \$9,791,437 compared to \$8,332,212 for the six month period ended June 30, 2012.

### **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 by entering 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. Through June 30, 2013, we had raised approximately \$13.5 million through capital contributions and notes payable from Retrophin shareholders and related parties.

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.

On February 14, 2013, in connection with the closing of a private placement, we issued and sold an aggregate of 3,045,929 shares of common stock at \$3.00 per share, for an aggregate purchase price of \$9,137,787 in cash, and warrants to purchase up to an aggregate of 1,597,969 shares of common stock.

The Company concurrently entered into a registration payment arrangement requiring it to file a Form S-1 with the Securities and Exchange Commission within 30 days of the closing date of this transaction and cause it to be declared effective by no later than 90 days of the closing date of this transaction. The registration payment arrangement provided for by the Company to pay liquidated damages in the amount of 2% of the proceeds received in this transaction per month for each month that the Company is not in compliance with this requirement, not to exceed 10% in the aggregate. The Company determined that it was probable that it would not be in a position to comply with these requirements and therefore allocated approximately \$360,000 of the proceeds received in this transaction to a registration payment obligation.

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 June 30, 2013 and December 31, 2012, our stockholders' deficit was \$8,457,398 and \$3,407,815, respectively. Our net loss from operations for the period March 11, 2011 (inception) through December 31, 2011, for the year ended December 31, 2012 and for the period March 11, 2011 (inception) through December 31, 2012 were approximately \$3.27 million, \$30.26 million and \$33.52 million, respectively. Our net loss for the six month period ended June 30, 2013 was \$9,791,435compared to \$8,332,212 for the six month period ended June 30, 2012. Net cash used in operating activities were \$0.79, \$2.74 million and \$3.52 million for the period March 11, 2011 (inception) through December 31, 2011, for the year ended December 31, 2012 and for the period March 11, 2011 (inception) through December 31, 2012, respectively. Net cash used in operating activities was \$6,786,982 for the six month period ended June 30, 2013 compared to \$1,617,555 for the six month period ended June 30, 2012. Operations since inception have been funded entirely with the proceeds from equity and debt financings. As of June 30, 2013, we had cash equivalents of \$286,514. On August 15, 2013, we sold an aggregate of approximately 5,536,957 shares of common stock at \$4.50 per share in a private placement for an aggregate purchase price of approximately \$25,000,000 in cash, and warrants to purchase up to an aggregate of approximately 2,768,479 shares of common stock with an exercise price of \$6.00 per share underlying each warrant. 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.

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 (Note 13); 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.

On August 16, 2013, the Company announced that it had signed an agreement with a major pharmaceutical company for the exclusive right to negotiate a royalty-bearing U.S. license for a product to be developed for the treatment of Autism and Schizophrenia. Pursuant to the exclusivity agreement, the Company paid the major pharmaceutical company a non-refundable upfront fee and will have an exclusive period of 120 days to negotiate a license agreement (see Note 13).

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 \$2,286,511 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 June 30, 2013, which is entirely past due as of the date of this filing. The counter parties to these agreements reserve the right to demand payment at any time. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock to one of the counter parties as a separate component of one of these agreements. 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 9).

On August 29, 2013, the Company entered into an additional settlement agreement for \$300,000 due following execution of the agreement. As of the date of this filing, this settlement has been paid (see Note 13).

On September 18, 2013, we made a proposal to the board of directors of 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. We have invested approximately \$3 million and acquired approximately 4.96% of the outstanding common stock of Transcept as part of the proposal process.

Effective October 1, 2013, the Company signed a Sponsored Research Agreement ("SRA") with 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.

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

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

# **Cash Flows from Operating Activities**

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

Operating activities used approximately \$6,786,982 of cash during the six month period ended June 30, 2013 compared \$1,617,555 for the six month period ended June 30, 2012. The increase of \$5,169,427 was the result of a decrease in non-cash charges of \$3,843,070 and a net change in operating assets and liabilities of \$132,868, offset by an increase in net loss of \$1,459,225.

#### **Cash Flows from Investing Activities**

Cash used in investing activities for the year ended December 31, 2012 was approximately \$1.70 million, compared to approximately \$0.13 million from the period March 11, 2011 through December 31, 2011. The increase of approximately \$1.57 million was primarily the result of \$1.17 million to purchase intangible assets, primarily related to sublicense from Ligand of the technology used in RE-021.

Cash used in investing activities for the six month period ended June 30, 2013 was \$1,315,393, compared to \$1,279,961 for the six month period ended June 30, 2012. The increase of \$35,432 was primarily the result of repayment of a technology license liability of \$1,300,000 offset by a decrease in the purchase of intangible assets of \$1,077,271 and a decrease in a related party note receivable of \$193,500

### **Cash Flows from Financing Activities**

For the year ended December 31, 2012, financing activities provided approximately \$4.44 million, compared to proceeds of approximately \$0.8 million from the period March 11, 2011 through December 31, 2011. The increase of approximately \$3.6 million was primarily a result of an increase of approximately \$2.7 million of proceeds from the private sale of our equity securities and approximately \$0.9 million of proceeds from related parties' notes payable.

For six month period ended June 30, 2013, cash provided by financing activities was \$8,377,501, compared to \$2,918,879 during the six month period ended June 30, 2012. The increase of \$5,458,622 was primarily a result of an increase of \$7,230,350 in proceeds received from the private sale of our equity securities, offset by a decrease in activities associated with related party notes payable of \$1,736,728.

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

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

The Company concurrently entered into a registration rights agreement requiring it 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. The 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 the Company is not in compliance with the agreement, not exceeding 10% of gross proceeds in the aggregate. As described elsewhere herein, the Company is not in compliance with the registration payment arrangement and therefore recorded \$360,000 as registration payment obligation.

# **Plan of Operation**

Our plan of operation for the years ending December 31, 2013 and 2014 is to continue implementing our business strategy, including the clinical development of our three drug candidates, focusing primarily on the development of RE-021 for the treatment of FSGS. We also intend to expand our drug product portfolio by acquiring additional drugs for marketing or development. We expect our principal expenditures during the next 12 months to include:

- operating expenses, including expanded research and development and general and administrative expenses; and
- product development expenses, including the costs incurred with respect to applications to conduct clinical trials in the United States for our three products and the costs of ongoing and planned clinical trials.

As part of our planned expansion, we anticipate hiring additional full-time employees for research and development activities and for general and administrative activities. 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, for the next 12 months, we expect to spend approximately \$14 to \$16 million on clinical development and research and development activities, including approximately (i) \$5 million to \$6 million in license fees for the acquisition of technology related to our current and prospective products, (ii) \$3 million to \$4 million in expenses associated with our Phase 2 clinical trial for FSGS, (iii) \$2 million in expenses related to our Phase 1 clinical trial for PKAN, (iv) \$2 million in compensation expense for our research and development staff and (v) \$2 million in other research related expenses, including pre-clinical testing, formulation, toxicology, sponsored research and general research activities.

We also expect to spend approximately \$5 million to \$6 million on general and administrative expenses, including approximately (i) \$2 million to \$3 million in compensation expenses related to our employees and consultants, (ii) \$1.5 million in expenses associated with operating as a publicly traded company, such as legal fees, accounting fees, insurance premiums, investor and public relations fees and board of director fees and (iii) \$1.5 million in other general and administrative expenses including travel and entertainment, rent, patent prosecution expenses, taxes, recruiting, office related expenses and other general and administrative expenses. 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**

*RE-021*. We plan to initiate a Phase 2 clinical trial of RE-021 in patients with FSGS in 2013, with reduction in proteinuria as the primary endpoint. We expect it will take at least three years to complete development and obtain FDA approval of RE-021 for any indication, and we may never obtain such approval. Currently, we anticipate that we will need to expend approximately an additional \$5 to \$6 million in development costs over the next 12 months and at least an aggregate of approximately \$25 to \$35 million before we receive FDA approval for RE-021 for treatment of patients with FSGS.

*RE-024*. We intend to develop RE-024 as a potential treatment for pantothenate kinase-associated neurodegeneration (PKAN). RE-024 is a preclinical investigational program. In vivo animal testing of these molecules is ongoing. In August 2013, we announced that we received positive survival results from interim preclinical tests for the treatment of PKAN. RE-024 is a replacement therapy for phosphopantothenate, the substrate that is missing in patients with PKAN. Tests were conducted on mice that were administered a PANK inhibitor to induce a PKAN-like phenotype.

We expect to initiate a human study for RE-024 in the first quarter of 2014. We expect that it will take an additional five to seven years to complete development and obtain FDA approval of RE-024, if ever. Currently, we anticipate that we will need to expend approximately an additional \$3 to \$4 million in development costs over the next 12 months and at least an aggregate of approximately \$30 to \$50 million until we receive FDA approval for RE-024 should we choose to continue development.

*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 we expect that in vivo evaluation of clinical trial quality material may begin in 2013. Currently, we anticipate that we will need to expend approximately an additional \$3 million in development costs over the next 12 months.

# **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. Under the license agreement, Ligand is obligated to transfer to Retrophin certain information, records, regulatory filings, materials and inventory controlled by Ligand and relating to or useful for developing RE-021. We must use commercially reasonable efforts to develop and commercialize RE-021 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 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 based on 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. The license agreement contains other customary clauses and terms as are common in similar agreements in the industry.

# **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.

### **Share-Based Payments**

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

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

#### **Income Taxes**

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

The standard addresses the determination of whether tax benefits claimed or expected to be claimed on a tax return should be recorded in the financial statements. Under FASB ASC 740, we may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the tax authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position should be measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement. FASB ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. At the date of adoption, and as of June 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 six month period ended June 30, 2013 and the fiscal year end December 31, 2012, we had no accrued interest or penalties related to uncertain tax positions.

# **Registration Payment Arrangement**

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

### Net loss per share

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

# **Recently Issued Accounting Pronouncements**

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

Except as noted above, the Company has evaluated recent accounting pronouncements and their adoption has not had or is not expected to have a material impact on the Company's 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.

### Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

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

### Quantitative and Qualitative Disclosures about Market Risk

Our primary exposure to market risk is related to changes in interest rates. As of December 31, 2012, we had cash, cash equivalents and short- term investments of approximately \$11,000, consisting of money market funds, U.S. treasuries, certificates of deposit and cash equivalents. 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.

### Properties

Our principal executive offices are located at 777 Third Avenue, 22<sup>nd</sup> Floor, New York, NY10017.

On October 1, 2013 we entered into a building lease for approximately 4,232 square feet of office space located in Cambridge, MA.



### **DESCRIPTION OF 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 prospectus.

### Overview

We are a development stage company focused on developing pharmaceutical products primarily for the treatment of rare diseases. Our lead product in development, RE-021, a small molecule intended to treat FSGS, has completed Phase 2 clinical studies demonstrating safety and efficacy, and we expect to initiate a Phase 2 clinical study in 2013. We also have a number of programs in preclinical development. Our second most developed program (RE-024) for the treatment of PKAN is in preclinical testing, and we will seek to initiate clinical trials of this product candidate as soon as is practical. We are also developing a treatment for DMD. Our focus is to seek treatment for serious, unmet, rare diseases. The diseases on which we focus are considered "orphan" diseases because they affect fewer than 200,000 patients in the United States. However, such diseases have a profound impact on those that suffer from them and on their families. Currently, we believe that we are the only company that is focusing on developing treatments for these rare and ultra-rare diseases.

#### **Organizational Background**

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

On December 12, 2012, Desert Gateway completed the 2012 Merger, in which former Retrophin became a wholly-owned subsidiary and the principal operating subsidiary 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, former Retrophin, with the Company continuing as the surviving corporation following the merger. On April 1, 2013, the Board of Directors of the Company determined to change the Company's fiscal year from a fiscal year ending in February of each year to a fiscal year ending on December 31 of each year.

#### General

Our goal is to become a leading biopharmaceutical company specializing in the development and commercialization of therapies for catastrophic diseases. Our commercialization strategy is to acquire pharmaceutical products for serious diseases and greatly increase patient and physician awareness to increase market penetration. Our development strategy is to focus on product opportunities which can take advantage of the shorter regulatory cycles that can be achieved with treatments for rare, life-threatening diseases. Beyond FSGS, PKAN and DMD, Retrophin has plans to discover and develop drug candidates for other orphan diseases, which may include cystic fibrosis and spinal muscular atrophy.

We are a developmental stage biopharmaceutical company focused on the discovery, development and commercialization of novel molecules for the treatment of a range of human genetic disorders. Our lead product in development is RE-021, a small molecule intended to treat FSGS. We filed an IND for the FSGS indication on July 2, 2012 (IND Number 115,903) and expect that a Phase 2 clinical study of RE-021 to treat FSGS could begin in 2013. Our second development program is RE-024, a series of molecules designed to treat PKAN. Our preclinical development of RE-024 is being carried out in collaboration with St. Jude Children's Research Hospital. We expect to file an IND for a lead compound in the RE-024 program by 2014. Our third product in development is RE-001, a modified protein intended to treat DMD. We are planning to initiate first-in-human enabling studies of RE-001. Preclinical studies to date, in mice, have suggested that RE-001 improves muscles function and improves mortality. We expect to file for approval to begin human clinical trials of RE-001, to treat DMD, by the end of 2014. We created and developed both RE-024 and RE-001 and have filed a patent application in the United States for RE-024.

Our focus is to seek treatment for serious, unmet, rare diseases. FSGS, PKAN and others are orphan diseases affecting fewer than 200,000 patients in the United States and have profound impacts on sufferers.

We are initially focused on developing RE-021 for patients with FSGS. We have licensed the exclusive worldwide rights to 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, from Ligand Pharmaceuticals, Inc., which had previously been responsible for the development efforts.

During the next 12 to 18 months, we plan to:

- · initiate a placebo-controlled Phase 2 clinical trial in FSGS; and
- initiate an open-labeled Phase 2 clinical trial in other nephropathies.

# **Our Strategy**

In order to achieve our goals, we intend to:

- **Expand our product pipeline by pursuing additional acquisitions of niche orphan drugs.** 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 innovative orphan drugs. We focus on novel, life-saving orphan drug candidates in order to take advantage of our competitive strengths. We believe that drug development for orphan drug markets is particularly attractive because relatively small clinical trials can provide meaningful information regarding patient response and safety. Furthermore, the path to regulatory approval and commercial success for orphan drugs is less risky for an effective therapy, as compared to non-orphan drugs. Though we do not currently have any orphan drug designated product candidates, we expect to seek orphan drug designations from the FDA. However, there can be no assurance that the FDA will grant orphan status for our 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.
- **Build 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.

### **Industry Analysis**

The pharmaceutical industry in which we seek to compete is highly competitive, strictly regulated, and rapidly changing. In the U.S. and abroad, governments regulate how drugs are approved, manufactured, sold, and paid for. The cost to get a drug to market can be substantial, oftentimes approaching \$1 billion, and the pharmaceutical industry is characterized by long (often 7-10 years) time periods between the time an idea for a drug is conceived and the time that sale of said drug can legally begin. Despite the time required to discover and develop drugs, the pharmaceutical industry can afford substantial profit (global pharmaceutical sales are expected to reach \$1 trillion in the next few years) if drug development is carried out correctly. While the challenge of creating drugs can be daunting, the industry can afford advantages by giving pharmaceutical companies near monopolistic exclusivity. For example, we are seeking to develop drugs to treat orphan diseases which can afford freedom from competition (in the U.S. for 7 years) if the FDA grants "orphan drug status". Additionally, pharmaceuticals can enjoy strong freedom from competition based on the awarding of patents by the U.S. Patent and Trademark Office, which provides 20 years of intellectual protection.

In addition to government regulations, the pharmaceutical industry has elements of monopsony from managed care and government payers for drugs. Going forward, global efforts toward health care cost containment efforts are expected to continue to exert pressure on product pricing and market access. Further, the United States enacted major health care reform legislation in 2010, which began to be implemented in 2011. This new law is expected to expand access to health care to millions Americans by the end of the decade who did not previously have regular access to health care. The effect that this legislation will have on the pharmaceutical industry is uncertain.

Given the potential profits in the pharmaceutical industry, there is intense competition to succeed. Other large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations are similarly pursuing the development of novel drugs that target the same diseases that we are seeking to treat. Retrophin faces, and expects to continue to face, intense and increasing competition as new products enter the market and advanced technologies become available. Despite the challenges and uncertainties of the pharmaceutical industry, Retrophin believes that it is well-positioned to compete in this potentially lucrative field.

#### **Competitive strengths**

We seek to discover, develop and deliver to patients first-in-class or best-in-class medicines for the treatment of rare, life-threatening, diseases. A first-in-class drug refers to the first approved or marketed drug within a class of drug candidates that operate through a particular target or molecular mechanism in the body to affect a specific disease. A best-in-class drug refers to a drug, among all drugs within a class of drugs which operate through a particular target or molecular mechanism in the body to affect a particular disease that is superior to all other such drugs in the class by virtue of its superior efficacy, superior safety, ease of administration, or some combination of the foregoing. We believe that RE-021, a drug for the treatment of FSGS, has the potential to be a best-in-class drug due to its superior efficacy and ease of administration. We believe that RE-024, a drug for the treatment of PKAN, has the potential to be a first-in-class drug, because no drug currently uses the particular molecules of RE-024 in the treatment of PKAN.

We have developed or acquired innovative product candidates for multiple rare disease indications, all of which represent proprietary applications of the expertise of our management and scientists, 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. Our product candidates result from a mixture of discoveries by in-house scientists and through judicious in-licensing of assets from other organizations, including other biotech and pharmaceutical companies, universities, or research institutes. We believe that our small molecule technologies, including the technologies that we are developing to treat FSGS, PKAN and DMD, and our team of experienced management and scientists allow us to seek to deliver first-in-class and best-in-class treatments.

Our lead product candidate (RE-021) has completed Phase 2 clinical studies demonstrating safety and efficacy, and we expect to initiate a Phase 2 clinical trial in 2013. Additionally, our second most developed program (RE-024) is in preclinical testing, and we will seek to initiate clinical trials of this product candidate as soon as is practical.

# **Research and Product Development Pipeline**

# RE-021

RE-021 is our lead development stage compound. RE-021 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. We have secured a license to 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, from Ligand and Bristol-Myers Squibb. We are developing RE-021 as a treatment for focal segmental glomerulosclerosis (FSGS) and other nephropathies. We also intend to develop RE-021 for resistant hypertension and in other therapeutic areas.

ARBs and ERAs have a rich history of clinical development. ARBs have a relatively narrow mechanistic purview: they are known to be antihypertensive agents with positive downstream effects on proteinuria , an excess of serum proteins in the urine, and end-organ (kidney and heart) prognosis. ERAs represent a less well-understood clinical mechanism. Over a dozen ERAs have been trialed clinically, for a diverse array of diseases including the successfully approved Tracleer (bosentan) and Letairis (ambrisentan) for pulmonary arterial hypertension (PAH), the unsuccessful darusentan for resistant hypertension and heart failure, the withdrawn-from-market Thelin (sitaxsentan) for PAH, the failed avosentan for diabetic nephropathy, the failed zibotentan in prostate cancer, the failed clazosentan in subarachnoid hemorrhage, the failed tezosentan in heart failure, the failed atrasentan in prostate cancer, the failed enrasentan in heart failure and the continuing trials of macitentan. We believe the ERA selectivity of RE-021 may provide benefits in terms of safety (i.e. less edema) over non-selective ERAs.

#### Ligand Phase 2 Clinical Trials

Ligand Pharmaceuticals has completed two controlled Phase 2 clinical trials for the treatment of Stage 1 and Stage 2 hypertension with the technology we license for RE-021. A total of 374 subjects were enrolled in these two trials, 95 of whom received placebo, 77 of whom received one of three escalating doses of RE-021 for up to four weeks, 144 of whom received one of three escalating doses of RE-021 for up to 12 weeks and 58 of whom received up to 12 weeks of irbesartan 300mg as an active control. In summary, once daily treatment with all doses of RE-021 was well tolerated in such subjects.

Overall, Treatment Emergent Adverse Events ("TEAE"s) were reported by 21% to 23% patients receiving RE-021 for four weeks, 34% to 61% of patients receiving RE-021 for 12 weeks, 45.3% of patients receiving placebo and 48.3% receiving Irbesartan 300 mg for 12 weeks. The most frequent system organ TEAEs with 5% or higher incidences were nervous system disorders, infections and infestations, general disorders and administration site conditions, gastrointestinal disorders, respiratory, thoracic and mediastinal disorders, investigations, musculoskeletal and connective tissue disorders, vascular disorders were observed in the placebo group, potentially due to uncontrolled high blood pressures. A higher rate of gastrointestinal disorders, vascular disorder, infections and infestations were observed in the highest dose of 12 week RE-021; and higher rate of general disorders and administration site conditions observed in the two highest doses of 12-Week RE-021.

The incidence rates of individual Adverse Events ("AE"s) were <14%. The most frequent AEs with 5% or higher rate were headache, edema peripheral, dizziness, fatigue, nausea, diarrhea, and abdominal pain. Headaches were reported more in placebo than other groups. Edema, dizziness, nausea, abdominal pain and diarrhea were reported more often in the highest doses of 12-week RE-021 than other groups.

In addition to the safety data obtained, it was observed that proteinuria was markedly reduced in subjects receiving RE-021 and we believe such results warrant further development of the product for FSGS and other nephropathies associated with elevated proteinuria.

# RE-021 in FSGS

We intend to develop RE-021 as a treatment for FSGS. FSGS is a leading cause of end stage renal disease (ESRD) and nephrotic syndrome. There are no FDA-approved treatments for FSGS and the off-label armamentarium is limited to ARBs, steroids, and immunosuppressant agents which are only effective for some patients. We estimate that there are at least 40,000 FSGS patients in the United States, which we believe could result in potential annual revenue of greater than \$1 billion/year for RE-021.

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 RE-021 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-021 in other indications

In addition to developing RE-021 as a potential treatment for FSGS, Retrophin intends to seek to begin clinical development of RE-021 in, IgA nephropathy, diabetic nephropathy, resistant hypertension, and other rare nephropathies as soon as possible.

# IgA Nephropathy

IgA nephropathy is a form of glomerulonephritis, a renal disease typically characterized by inflammation of the glomeruli or small blood vessels in the kidneys, with high proteinuria as its key symptom. There is no FDA approved therapy for IgA nephropathy. The prognosis of this disease is directly related to proteinuria level, with roughly one-third to one-fifth of patients losing their kidney within 10 years, with risk continuing linearly as age progresses. Most patients are diagnosed young, so dialysis, transplant and death are inevitable in these patients. There is a range of estimated patients from 40,000 to 150,000 in the United States. Assuming 35% of these patients have very severe proteinuria, and a \$25,000 per-patient per-year price, peak global sales of RE-021 in IgA nephropathy can exceed over \$1 billion.

There has never been a large clinical trial in IgA nephropathy. We believe that it is widely accepted and evidence-based that proteinuria is an appropriate endpoint for measuring the progress of this disease. Following completion of a small, open-label study, we would seek to begin a pivotal trial evaluating RE-021 in IgA nephropathy patients having proteinuria >1g/day. We believe that an acceptable primary endpoint for such a trial would be change in proteinuria at three months. Based on other IgA nephropathy studies, we believe that approximately 150 patients could be enrolled in about one year. Retrophin could be in a position to start a pivotal clinical study in IgA nephropathy in 2014.

#### Resistant Hypertension

Retrophin intends to mirror a previous darusentan Phase 2 trial seeking to treat resistant hypertension with RE-021. We believe that the potential potency of RE-021 and an increased sample size compared to a previous study could allow for improvement in the expected primary endpoint of systolic blood pressure change at 10 weeks. In this population, trial design is a key concern. Because resistant hypertension is a complex clinical "situation," it requires exponentially more clinical trial programming and design. Twenty-four hour ambulatory blood pressure automated monitoring is a more accurate assay for blood pressure than sitting blood pressure. We estimate that this study could begin enrolling in 2014. If results of this study are positive, Retrophin would target a partnership with a major pharmaceutical company to continue development.

# 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 per million people. The devastating effects of PKAN—most sufferers end up wheelchair bound, as well as suffering from dementia and other psychiatric problems, and typically don't live past age 20—are clear. There are currently no viable treatment options for patients with PKAN: the opportunity with RE-024 is to transform treatment of PKAN with a potentially life changing and life-extending impact on 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 idiopathology 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.

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

#### Pantothenic acid pro-phosphates, a potential solution

PKAN is caused by a misregulation in a single protein responsible for neurological function, namely, pantothenate kinase-2 (PANK2). PANK is the first enzyme responsible for the synthesis of Coenzyme A (CoA), and specifically phosphorylates pantothenic acid (vitamin B5).

# Retrophin's Approach to Treating PKAN: RE-024

PKAN is caused by dysregulation of the pantothenate kinase (PANK) enzyme, which converts pantothenic acid to phosphopantothenic acid. The reaction catalyzed by PANK is depicted in Figure 1.

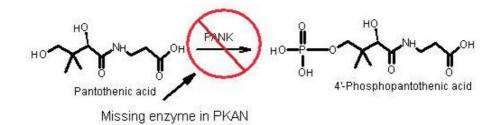


Figure 1: Reaction catalyzed by PANK.

RE-024 is a small molecule "prophosphate" designed to circumvent the need for PANK, the dysfunctional enzyme responsible for PKAN, that is, to directly supply cells with the product of the reaction, namely phosphopantothenic acid. A simple approach to this could be to use the product of the enzymatic reaction, namely, 4"-phosphopantothenic acid. This approach has been mentioned in the literature, but it has been recognized that the highly charged molecule would not be able to permeate the lipohilic cell membrane. The approach taken with RE-024 is to follow the lead of nucleotide chemistry, and to generate prodrugs of phosphates ("pro-phosphates"). At physiological pH, phosphates have a negative charge, which makes it very difficult to diffuse through any cellular membranes. By converting the phosphate to a phosphoramidate pro-phosphate, such negative charge is masked. The masking groups will be cleaved once in the cell, revealing the phosphate at its desired site of action. The approach described has been successfully used in improving the bioavailability of nucleotides.

Retrophin is in the process of synthesizing a library of derivatives of RE-024, via a CRO. The library is designed to define the optimal characteristics of molecule, specifically, with a view to striking a balance between extra and intracellular stability and lipophilicity. A similar idea, in the nucleoside case, has been described for potential HCV treatments, for example, GS-7977.

#### 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 Duchenne muscular dystrophy (DMD) patients. In RE-001, micro-utrophin is 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.

### Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a severe recessive X-linked form of muscular dystrophy characterized by rapid progression of muscle degeneration, eventually leading to loss of ambulation and death. This affliction affects one in 3,500 males, making it the most prevalent of muscular dystrophies. In general, only males are affected, though females can be carriers. Females may be afflicted if the father is afflicted and the mother is also a carrier/affected. The disorder is caused by a mutation in the dystrophin gene, located in humans on the X chromosome.

Symptoms of DMD usually appear in male children before age five and may be visible in early infancy. Progressive proximal muscle weakness of the legs and pelvis associated with a loss of muscle mass is observed first. Eventually this weakness spreads to the arms, neck, and other areas. As the condition progresses, muscle tissue experiences wasting and is eventually replaced by fat and fibrotic tissue. By age 10, braces may be required to aid in walking but most patients are wheelchair dependent by age 12. Later symptoms may include abnormal bone development that lead to skeletal deformities, including curvature of the spine. Due to progressive deterioration of muscle, loss of movement occurs, eventually leading to paralysis. The average life expectancy for patients afflicted with DMD varies from late teens to early to mid-twenties. There have been reports of a few DMD patients surviving to the age of 40, but this is extremely rare.

There is no known cure for DMD. Treatment is generally aimed at controlling the onset of symptoms to maximize quality of life. Corticosteroids such as prednisolene and deflazacort are commonly used for DMD to increase energy and strength and defer severity of some symptoms. However, the benefits are temporary, modest and are accompanied by detrimental side effects including muscle wasting, fat deposition and bone loss. Physical therapy is also used to help maintain muscle strength, flexibility and function. Orthopedic appliances such as braces and wheelchairs help to provide structural support and improve mobility, and respirators and ventilators assist with managing breathing. There are new treatments in development to potentially restore the functionality of a gene containing a mutation resulting in DMD by a process called "exon skipping." The goal of exon-skipping is to realign the translation of genetic information in the dystrophin gene and promote synthesis of a shortened, but functional, version of the protein. Exon-skipping drugs are still in development stage, and if successful it is expected that they could slow the course of DMD and reduce the severity of the muscle disease. It is also possible that these exon-skipping therapies, if successful, may be appropriate only for those patients with very specific mutations in the dystrophin gene.

# RE-001

RE-001 is a novel compound that is being developed to replace dystrophin, the missing protein that has been identified as causing DMD. Protein replacement therapy is a well-known tool for many diseases such as insulin for diabetes, erythropoietin (EPO) for anemia resulting from chronic kidney disease and myelodysplasia, and human growth hormone (HGH) for short stature, chronic renal failure, and Prader-Willi syndrome, among other conditions.

Figure 2 demonstrates the role of dystrophin in cell stability, that is, to bind the muscle cell membrane to the actin filaments required for the mechanical function of muscle cells.

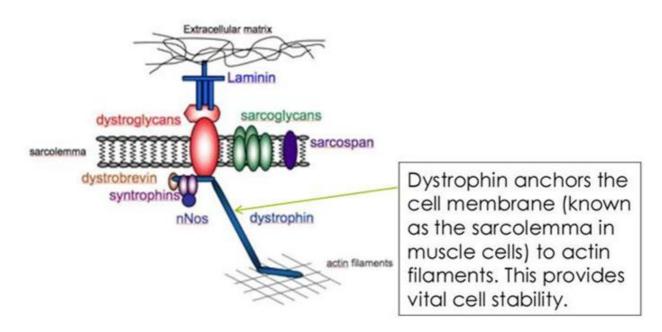


Figure 2: Role of dystrophin in muscle cell stabilization. RE-001 is designed to replace dystrophin in DMD boys.

RE-001 is designed to replace dystrophin by providing a recombinant supply of a modified form of a very similar protein, utrophin, fused to a cellpenetrating peptide (TAT) which allows for delivery of the utrophin protein into the cell where it is needed for structural support and integrity. In pre-clinical studies, treatment with RE-001 in "mdx" mice (a strain of mice that lack the muscle protein dystrophin), an animal model for DMD, resulted in reduced creatine kinase excretion, a marker of muscle damage. Retrophin will seek to replicate this result in humans, with creatine kinase as a possible primary endpoint or co-primary endpoint for a Phase 2 trial.

# **RE-001** Development Activities

Two papers on use of TAT-m-UTR have been published. In the first study (Ervasti *et al.*, *PLoS*, **2009**), the treated mice in the above study showed markedly less muscle degradation, as measured by muscle fiber diameter than those treated with placebo. Additionally, TAT-m-UTR treated mice exhibited better physical muscle strength, as measured by muscle force assays. In a second study with a more severe muscle impairment (Ervasti *et al.*, *J. Appl. Physiol.*, **2011**), mice with DMD treated with TAT-m-UTR had a median overall survival of 43.5 days  $\pm$  2.0 days, compared to 30 days  $\pm$  1.8 days for PBS treated mice.

Direct protein replacement as a potential therapy for Duchenne muscular dystrophy has, to the best of our knowledge, not been attempted to date.

## Planned Phase 1 Clinical Trial

We expect to initiate a Phase 1 clinical study of RE-001 in DMD patients by the end of 2014. We can provide no assurances that we can successfully start this study. The Phase 1 clinical study will initially explore the tolerability and pharmacokinetic behavior of RE-001. Dose amount and frequency will be informed by our initial animal studies.

# Licenses and Royalties

### 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. 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 RE-021. 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 RE-021. We must use commercially reasonable efforts to develop and commercialize RE-021 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 upon the achievement of certain milestones totaling up to \$106.7 million, if all such milestones are achieved. 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 based on 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.

The license agreement will continue until neither party has any further obligations to make payments under the agreement. 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 RE-021 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.

### **Research Agreement**

# St. Jude Sponsored Research Agreement

On September 1, 2013, we entered into an agreement (the "Sponsored Research Agreement") with St. Jude Children's Research Hospital ("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 fees to St. Jude on a specified timeline. The Sponsored Research 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.

# **Intellectual Property**

We hold a worldwide exclusive license under our license agreement with Ligand for 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, to three granted U.S. patents as well as foreign counterparts thereof and other patent applications and patents claiming priority therefrom.

We have licenses to three issued U.S. patents, which will currently expire in 2020-2023 before any patent term extension, and three pending U.S. patent applications covering RE-021, our lead compound, and there are issued patents or pending applications covering RE-021 in the following foreign jurisdictions: Australia, Austria, Belgium, Bulgaria, Canada, China, Cyprus, the Czech Republic, Denmark, Finland, France, Germany, Great Britain, Greece, Hungary, Ireland, Italy, Japan, Latvia, Liechtenstein, Luxembourg, Monaco, Netherlands, Poland, Portugal, Romania, Slovakia, South Africa, Spain, Sweden, Switzerland and Turkey. We have filed one application covering RE-024 in the United States, and a PCT counterpart filing has been made. The RE-021 patents and patent applications relate to method of use, and the RE-024 application is for composition of matter. We do not own or license any issued or pending applications for patents covering 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 RE-021 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 RE-021.

If we obtain marketing approval for RE-021 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, 8 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 Regulations" 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 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

A portion of our future end-user demand for our drugs is for patients covered under Medicaid, Medicare and other government-related programs such as TRICARE and the Veterans Administration (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 sales with respect to Medicaid sales, but we do generate net sales with respect to Medicare sales, TRICARE sales and sales made to the VA.

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

#### Competition

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

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

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

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.

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

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

# **Clinical Testing of Our Products in Development**

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

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

# **Government Regulations**

# **United States – Marketed Products**

In the United States, the FDA regulations govern the research, development, testing, manufacture, quality control, labeling, storage, record-keeping, approval, sale, distribution, advertising and promotion of our products.

The FDA may withdraw product approval for non-compliance with regulatory requirements or if safety or efficacy problems occur after the product reaches the market. The FDA also has the power to require changes in labeling or to prevent further marketing of a product based on the results of post-marketing programs.

The facilities, procedures, and operations of our contract manufacturers must be determined to be adequate by the FDA before a new drug application (an "NDA") or supplemental new drug application ("sNDA") is approved. Additionally, manufacturing facilities are subject to inspections by the FDA for compliance with current good manufacturing practices, licensing specifications, and other FDA regulations on an on-going basis. Vendors that supply our finished products or components used to manufacture, package and label products are subject to similar regulations and periodic inspections.

Following such inspections, the FDA may issue notices on Form 483 and issue Warning Letters that could cause us to modify certain activities identified during the inspection. The FDA generally issues a Form 483 notice at the conclusion of an FDA inspection and lists conditions the FDA investigators believe may violate cGMP or other FDA regulations. FDA guidelines specify that a Warning Letter be issued only for violations of "regulatory significance" for which the failure to adequately and promptly achieve correction may be expected to result in an enforcement action.

In addition, the FDA imposes a number of complex regulatory requirements on entities that advertise and promote pharmaceuticals, including but not limited to, standards and regulations for direct-to-consumer advertising, payments to physicians, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet.

Failure to comply with FDA and governmental regulations can result in fines, unanticipated compliance expenditures, recall or seizure of products, total or partial suspension of production and/or distribution, suspension of the FDA's review of NDAs or sNDAs, injunctions, disqualification from participation in government reimbursement programs and criminal prosecution. Any of these actions or events could have a material adverse effect on us both financially and reputationally.

### United States – Drug Candidates

### FDA Process

The research, development, testing, manufacture, labeling, promotion, advertising, distribution and marketing, among other things, of drug products are extensively regulated by governmental authorities in the United States and other countries. In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act (the "FDCA"), and its implementing regulations. Failure to comply with the applicable U.S. requirements may subject us to administrative or judicial sanctions, such as FDA refusal to approve pending NDAs, warning letters, fines, civil penalties, product recalls, product seizures, total or partial suspension of production or distribution, injunctions and/or criminal prosecution.

### Drug Approval Process.

None of our drug product candidates may be marketed in the United States until the drug has received FDA approval. The steps required before a drug may be marketed in the United States generally include the following:

- Completion of extensive pre-clinical laboratory tests, animal studies, and formulation studies in accordance with the FDA's GLP regulations;
- · Completion of extensive pre-clinical laboratory tests, animal studies, and formulation studies in accordance with the FDA's GLP regulations;



- Submission to the FDA of an IND for human clinical testing, which must become effective before human clinical trials may begin;
- Performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the drug for each proposed indication;
- Submission to the FDA of an NDA after completion of all pivotal clinical trials;
- Satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the active pharmaceutical ingredient, or API, and finished drug product are produced and tested to assess compliance with cGMPs; and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

The development and approval process requires substantial time, effort and financial resources, and we cannot be certain that any approvals for our product candidates will be granted on a timely basis, if at all.

# Expedited Review and Approval.

The FDA has various programs, including Fast Track, priority review and accelerated approval, which are intended to expedite or simplify the process for reviewing drugs, and/or to provide for approval on the basis of surrogate endpoints. Even if a drug qualifies for one or more of these programs, the FDA may later decide that the drug no longer meets the conditions for qualification or that the time period for FDA review or approval will be shortened. Generally, drugs that may be eligible for these programs are those for serious or life- threatening conditions, those with the potential to address unmet medical needs, and those that offer meaningful benefits over existing treatments. For example, Fast Track is a process designed to facilitate the development, and expedite the review of drugs to treat serious diseases and fill an unmet medical need. Priority review is designed to give drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists an initial review within 6 months as compared to a standard review time of 12 months. Although Fast Track designated drug and expedite review of the application for a drug designated for priority review. Accelerated approval provides an earlier approval of drugs to treat serious diseases, and that fill an unmet medical need based on a surrogate endpoint, which is a laboratory measurement or physical sign used as an indirect or substitute measurement representing a clinically meaningful outcome. As a condition of approval, the FDA may require that a sponsor of a drug receiving accelerated approval perform post-marketing clinical trials.

# Orphan Drug Designation

The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 patients in the United States. Orphan drug designation must be requested before submitting an NDA. After the FDA grants Orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Other than guidance concerning the nonclinical laboratory studies and clinical investigations necessary for approval of an NDA, orphan drug status does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The FDA may grant orphan drug status to multiple competing product candidates targeting the same indications. However, an orphan drug that subsequently receives the first FDA approval is entitled to a seven year period of market exclusivity during which the FDA may not approve any other applications to market the same drug for the same indication, except in very limited circumstances.

# Patent Term Restoration and Marketing Exclusivity.

Depending upon the timing, duration and specifics of FDA approval of the use of our drugs, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND, and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the extension must be applied for prior to expiration of the patent. The U.S. Patent and Trademark Office, or the USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we intend to apply for restorations of patent term for some of our currently owned or licensed patents to add patent life beyond their current expiration date, depending on the expected length of clinical trials and other factors involved in the submission of the relevant NDA.

Data and market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the pre-clinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

### Foreign Regulation

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

#### **Other Laws and Regulatory Processes**

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

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

#### Employees

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

# Organization and Consolidated Subsidiaries.

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

# **DESCRIPTION OF PROPERTY**

Our principal executive offices are located at 777 Third Avenue, 22nd Floor, New York, NY 10017.

# LEGAL PROCEEDINGS

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

# MANAGEMENT

The following table sets forth the name, age, and position of our directors and officers as of the date of this prospectus. Executive officers are elected annually by our board of directors. Each executive officer holds his office until he resigns, is removed by the board, or his successor is elected and qualified. Directors are elected annually by our stockholders at the annual meeting. Each director holds his office until his successor is elected and qualified or his earlier resignation or removal. Each person listed below was appointed to his respective office and/or director position as of December 17, 2012.

Name	Age	Position
Martin Shkreli	30	Chief Executive Officer and Director
Marc Panoff	43	Chief Financial Officer
Horacio Plotkin, M.D.	48	Chief Medical Officer
Stephen Aselage	62	Director
Steve Richardson	59	Director
Cornelius E. Golding	65	Director

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

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

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

STEPHEN ASELAGE has served as a director of the Company since December 17, 2012. Previously, Mr. Aselage was a director of our predecessor, Retrophin, Inc., since October 2012. Prior to joining Retrophin, Mr. Aselage served as the Executive Vice President and Chief Business Officer at BioMarin, a biotechnology company, from December 2009 through September 2012. And from June 2005 to December 2009, Mr. Aselage served as BioMarin's Senior Vice President of Global Commercial Development. From February 2004 to June 2005, Mr. Aselage served as Executive Vice President of Global Commercial Operations at Cell Therapeutics, a biotechnology company focused on cancer therapeutics. From September 2003 to January 2004, Mr. Aselage served as Senior Vice President of North American Sales and Marketing for Genzyme Corporation, a biotechnology company, following Genzyme's acquisition of Sangstat Medical Corporation where he had worked since February 1999. While at Sangstat, Mr. Aselage restructured the company's sales, marketing and medical affairs groups. From 1996 through 1999, Mr. Aselage served as Director of Sales and Marketing at Advanced Tissue Sciences, a biotechnology company. Earlier in his career, Mr. Aselage held a variety of sales and sales management positions at biotechnology and pharmaceutical companies including Rhône-Poulenc Rorer Pharmaceuticals (now Sanofi-Aventis), Genentech, Inc., and Bristol Laboratories, a biopharmaceutical company. Mr. Aselage holds a B.S. in biology from the University of Notre Dame. Mr. Aselage was selected as a director because of his business and professional experience, including but not limited to his leadership of BioMarin in drug commercialization, private and public financings and a successful turnaround of multiple businesses.

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

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

### **Director Independence**

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

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

# **Compensation of Directors**

We have not established a policy to provide compensation to our directors for their services in such capacity. Our board will consider developing such a policy in the future.

# EXECUTIVE COMPENSATION

The following table sets forth all cash compensation paid by the Company for the fiscal years 2011 and 2012. The table below sets forth the positions and compensation for each officer and director of the Company.

SUMMARY COMPENSATION TABLE										
Name and Principal Position	Fiscal Year	Salary (\$)	Bonus (\$)	Stock Awards (\$) (1)	Option Awards (\$) (1)	Non-Equity Incentive Plan Compensation (\$)	Change in Pension Value and Nonqualified Deferred Compensation Earnings (\$)	All Other Compensation (\$)	Total (\$)	
Martin Shkreli, Chief Executive Officer and Director (2)	2012	250,000	565,231	14,444,100					15,259,331	
	2011	187,500	34,900	1,608,300					1,830,700	
Stephen Aselage, Director (2)	2012	83,333		2,000,000					2,083,333	
	2011									
Steven Richardson, Director (2)	2012									
	2011									
Robert Wilson, former Chief Executive Officer, President and Director (3)	2012									
	2011									
Gary Lyons, former Director (3)	2012									
	2011									

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

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

(3) Prior to December 12, 2012, Robert Wilson served as the principal executive officer of Desert Gateway and, prior to December 17, 2012, Robert Wilson and Gary Lyons served as directors of Desert Gateway. Desert Gateway did not paid its officers and directors any salary or consulting fees in fiscal years 2011 and 2012.

# Compensation Arrangements

Mr. Shkreli received an annual base salary of \$250,000 pursuant to an employment agreement with our predecessor, former Retrophin. Mr. Shkreli received a bonus of \$34,900 for fiscal 2011 and a bonus of \$565,231 for fiscal 2012. Mr. Shkreli's bonus for 2012 included a payment of \$250,000 in connection with his performance. In addition to the \$250,000 of base salary and \$250,000 of performance bonus in 2012, the Company reclassified \$407,900 in stockholders' equity as of December 31, 2012, of which approximately \$93,000 related to expenses that should have been classified as operating expense. Of the remaining \$315,231, we determined that our write-off in 2012 of a \$210,000 note due from a related party that Mr. Shkreli managed prior to our merger with Desert Gateway should be deemed to be a bonus to him. Also, we determined that payments that we made prior to our merger with Desert Gateway in the amount of \$105,231 were for operating expenses of the aforementioned related party and should be deemed to be a bonus to Mr. Shkreli did not receive direct payments in connection with the \$315,000 in charges described in the prior two sentences, and elected for the Company to treat these payments as compensation.

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

# Grants of Stock Awards

On March 31, 2011, the Company granted 1,608,300 incentive shares to Mr. Shkreli, which vested on the final day of each calendar quarter over three years, commencing on June 30, 2011. On September 11, 2012, the Company accelerated the vesting of 938,175 of the shares issued to Mr. Shkreli.

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

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

### **Employment Agreements**

# Plotkin Employment Agreement

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

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

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

In the event Dr. Plotkin's employment is terminated (i) by the Company without cause (as such term is defined in the Plotkin Employment Agreement) or (ii) by Dr. Plotkin's resignation following a material breach of a material term of the Plotkin Employment Agreement by the Company which has not been cured within 10 days following notice thereof, then Dr. Plotkin will be entitled to a severance payment in an amount equal to \$60,000 plus reimbursement of certain expenses. If Dr. Plotkin chooses to resign for reasons other than a material breach of the Plotkin Employment Agreement by the Company then Dr. Plotkin will forfeit any unvested stock grants or stock options that he received and will not be entitled to severance or any additional payments.

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

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

# Panoff Employment Agreement

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

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

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

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

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

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

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

### **Compensation Committee Interlocks and Insider Participation**

We do not have a compensation committee or a committee performing similar functions. All compensation matters are determined by our board of directors. We plan to have a compensation committee when we elect additional independent persons to our board of directors.

# CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

### 2012 Merger

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

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

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

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

## **Private Placements**

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

On August 15, 2013, the Company completed a private placement transaction (the "August 2013 Private Placement"), pursuant to a Securities Purchase Agreement, dated as of August 14, 2013, by and among the Company and the selling stockholders, including Mr. Shkreli, Mr. Panoff, Mr. Plotkin, Mr. Aselage and Mr. Richardson. In such private placement transaction, (i) Mr. Shkreli purchased 11,112 shares of our common stock and Warrants to purchase up to 5,556 shares of our common stock, for an aggregate purchase price of \$50,000, (ii) Mr. Panoff purchased 1,100 shares of our common stock and Warrants to purchase up to 550 shares of our common stock, for an aggregate purchase price of \$4,950, (iii) Mr. Plotkin purchased 2,200 shares of our common stock and Warrants to purchase up to 1,100 shares of our common stock, for an aggregate purchase price of \$9,900, (iv) Mr. Aselage purchased 556 shares of our common stock and Warrants to purchase up to 278 shares of our common stock, for an aggregate purchase of our common stock, for an aggregate purchase up to 555 shares of our common stock and Warrants to purchase up to 278 shares of our common stock, for an aggregate purchase price of \$4,950. In connection with the closing of the August 2013 Private Placement, the Company also entered into a Registration Rights Agreement with the selling stockholders, including Mr. Shkreli, Mr. Panoff, Mr. Plotkin, Mr. Aselage and Mr. Richardson, pursuant to which the Company agreed to register all of the shares of common stock, and shares of common stock issuable upon the exercise of the Warrants, sold in the August 2013 Private Placement.

# PRINCIPAL AND SELLING STOCKHOLDERS

The following table sets forth certain information regarding the beneficial ownership of our common stock as of the date hereof, by (i) each person known by us to be the beneficial owner of 5% or more of the outstanding common stock, (ii) each executive officer and director of the Company, (iii) all of our executive officers and directors as a group, and (iv) the selling stockholders.

In January 2013, we sold an aggregate of 272,221 shares of common stock in certain private placement transactions. On February 14, 2013, we closed the February 2013 Private Placement, in which we sold 3,045,929 shares of common stock and Warrants to purchase up to 1,597,969 shares of common stock. The Warrants sold in the February 2013 Private Placement are immediately exercisable, expire on the fifth anniversary of their issuance and entitle their holders to purchase the number of shares of our common stock specified therein at \$3.60 per share (subject to customary full-ratchet anti-dilution protections). On August 15, 2013, we closed the August 2013 Private Placement, in which we sold 5,536,957 shares of common stock and Warrants to purchase up to 2,768,479 shares of common stock. The Warrants sold in the August 2013 Private Placement are immediately exercisable, expire on the fifth anniversary of their issuance and entitle their holders to purchase the number of shares sold in the August 2013 Private Placement are immediately exercisable, expire on the fifth anniversary of their issuance and entitle their holders to purchase the number of shares of our common stock specified therein at \$6.00 per share (subject to customary full-ratchet anti-dilution protections).

The amounts and percentages of common stock beneficially owned are reported on the basis of regulations of the SEC governing the determination of beneficial ownership of securities. Under the rules of the SEC, a person is deemed to be a "beneficial owner" of a security if that person has or shares "voting power," which includes the power to vote or to direct the voting of such security, or "investment power," which includes the power to dispose of or direct the disposition of such security. Certain shares may be deemed to be beneficially owned by more than one person (if, for example, persons share the power to vote or the power to dispose of the shares). In addition, shares are deemed to be beneficially owned by a person if the person has the right to acquire the shares (for example, upon exercise of an option) within 60 days of the date as of which the information is provided. In computing the percentage ownership of any person, the amount of shares outstanding is deemed to include the amount of shares beneficially owned by such person (and only such person) by reason of these acquisition rights.

The below table assumes that all of the currently outstanding Warrants will be exercised in their entirety for common stock and all of the securities will be sold in this offering. However, any or all of the securities listed below may be retained by any of the selling stockholders, and therefore, no accurate forecast can be made as to the number of securities that will be held by the selling stockholders upon termination of this offering. The selling stockholders are not making any representation that any shares covered by this prospectus will be offered for sale. Subject to applicable community property laws, we believe that all persons listed have sole voting and investment power with respect to their shares unless otherwise indicated.

	Shares Beneficially Owned Prior to Offering (1)		Shares Being Registered		Beneficial Ownership After Offering (1)(2)	
Name of Beneficial Owner	Shares	8	Percent	for Sale	Shares	Percent
Executive Officers and Directors (3)						
Martin Shkreli, Chief Executive Officer and						
Director (4)	3,213,299		17.64%	199,572	3,013,727	16.60%
Marc Panoff, Chief Financial Officer	121,650		*	1,650	120,000	*
Horacio Plotkin, M.D., Chief Medical Officer	23,300	(5)	*	3,300	20,000	*
Stephen Aselage, Director	262,034		1.44% *	834	261,200	1.44%
Steven Richardson, Director All directors and executive officers as a group	99,720 3,720,003		20.39%	1,665 207,021	98,055 3,512,982	19.33%
Other Non-Selling 5% Beneficial Owner						
Ligand Pharmaceuticals Incorporated (6)	620,000		3.42%	0	620,000	3.42%
Other Selling Stockholders						
Other Selling Stockholders						
Sabby Healthcare Volatility Master Fund, Ltd.	1,517,344	(7)	8.13%	1,517,344		*
Sabby Volatility Warrant Master Fund, Ltd.	312,366		1.71%	312,366		*
Auriga Global Investors SV, SA (9)	885,422		4.80%	885,422	0	*
RRC Bio Fund, LP (10)	1,725,006		9.21%	1,725,006	0	*
Perceptive Life Sciences Master Fund Ltd (11) Prosight Fund, LP (12)	1,575,005 487,503		8.43% 2.66%	1,575,005 487,503	0	*
Sphera Global Healthcare Master Fund	254,034		1.39%	254,034	0	*
Hartz Capital Investments, LLC (13)	410,518		2.24%	410,518	0	*
Empery Asset Master, LTD (14)	101,808		*	101,808	0	*
Ryan M. Lane	25,405		*	25,405	0	*
Susan B. Lane Revocable Trust of 2010 (15)	25,405		*	25,405	0	*
Medical Strategy GmbH for PHARMA/wHealth Management Company S.A on behalf of Pharma/wHealth Oppenheim Asset Management Services S. à r.l.	35,000		*	35,000	0	*
on behalf of FCP OP Medical BioHealth- Trends	170,000		*	170,000	0	*
IPConcept Luxembourg S.A. (IPConcept Fund	17 0,000			1,0,000	Ŭ	
Management S.A. is the former name and						
changed to IPConcept Luxembourg S.A. (16)	33,000		*	33,000	0	*
Michael Chill	8,414		*	8,414	0	*
John Chambers	8,414		*	8,414	0	*
Michael Margolis Thomas E. Fernandez	8,415 370,770		2.04%	8,415 70,770	0 300,000	1.65%
Marek Biestek	484,118		2.66%	146,618	337,500	1.86%
Antoine Verglas	88,888		*	88,888	0	*
Harley Lippman	33,333		*	33,333	0	*
9W Investment Fund I LP (17)	733,332		3.99%	733,332	0	*
AmTrust International Insurance, Ltd. (18)	833,333		4.52%	833,333	0	*
Andrew Left	33,333		*	33,333	0	*
Andrew Vaino	332,170		1.83%	3,000	329,170	1.81%
APO Medical Opportunities Medical Strategy (19)	71,617		*	71,617	0	*
Broadfin Healthcare Master Fund, Ltd. (20)	666,668		3.63%	666,668	0	*
David Moradi	150,000		*	150,000	0	*
Dos Mineiros Capital Fund (21) IPConcept (Luxembourg) S.A., acting in its own name but on behalf of APO Medical	329,017		1.80%	329,017	0	
Opportunities – Medical Strategy Hauck & Aufhäuser Banquiers Luxembourg S.A.	215,502		1.18%	215,502	0	*
on behalf of RIM Global Bioscience	20,000		*	20,000	0	*
Michael Smith	7,167	(	*	3,000	4,167	*
PHARMA/wHealth Management Company S.A	55,250	(22)	*	55,250	0	*
Fourth Avenue Capital Partners LP	216,669	(23)	1.19% *	216,669	0	*
Quintessence Fund L.P. QVT Fund IV LP	176,073 204,738	(23) (23)	1.12%	176,073 204,738	0	*
QVT Fund V LP	1,285,857	(23)	6.92%	204,738	0	*
Roman Roik	1,205,057	(23)	*	1,205,057	0	*
Ronald Gottesman	16,667		*	16,667	0	*
venBio Select Fund, LLC	200,000		1.10%	200,000	0	*
Zachary Prensky	181,000		*	174,000	7,000	*
Loon Capital Management (24)	33,333		*	33,333	0	*

\* Less than 1%, unless otherwise specified.

- (1) Percentages are based on 18,386,919 shares of common stock issued and outstanding as of October 24, 2013.
- (2) Assumes that all securities registered will be sold.
- (3) As reported on the beneficial owner's Schedule 13G, filed with the SEC on January 11, 2013. The address of each officer and director is c/o Retrophin, Inc., 777 Third Avenue, 22<sup>nd</sup> Floor, New York, NY 10017.
- (4) Includes an aggregate of 473,687 shares of common stock held by MSMB Healthcare LP and MSMB Healthcare Investors LLC. Mr. Shkreli is the managing member of MSMB Healthcare Investors LLC, which is the general partner of MSMB Healthcare LP. Mr. Shkreli disclaims beneficial ownership of the shares held by MSMB Healthcare LP and MSMB Healthcare Investors LLC.
- (5) Includes 20,000 shares of common stock issuable upon exercise of employee stock options which have vested or will vest within 60 days of the date hereof.
- (6) The address of the beneficial owner is 11119 North Torrey Pines Road, Suite 200, La Jolla, California, 92037.
- (7) Represents the stockholder's beneficial ownership of 9.99% of the Company's issued and outstanding shares of common stock, as the Warrants held by such investor are subject to a 9.99% beneficial ownership limitation. Notwithstanding any restrictions on such beneficial ownership limitations, the number of shares offered includes (i) 833,334 shares of common stock and (ii) 416,667 shares of common stock issuable upon exercise of the Warrants. Sabby Management, LLC shares voting and investment power with respect to these shares on behalf of this stockholder. As manager of Sabby Management, LLC, Hal Mintz also shares voting and investment power on behalf of this stockholder. Each of Sabby Management, LLC and Hal Mintz disclaim beneficial ownership over the securities covered by this prospectus except to the extent of their pecuniary interest therein. Except as described above, none of the selling stockholders has had, within the past three years, any position, office or other material relationship with the Company or any of our predecessors or affiliates.
- (8) Includes (i) 166,666 shares of common stock and (ii) 83,333 shares of common stock issuable upon exercise of the Warrants. Sabby Management, LLC shares voting and investment power with respect to these shares on behalf of this stockholder. As manager of Sabby Management, LLC, Hal Mintz also shares voting and investment power on behalf of this stockholder. Each of Sabby Management, LLC and Hal Mintz disclaim beneficial ownership over the securities covered by this prospectus except to the extent of their pecuniary interest therein.
- (9) Inigo Resusta shares voting and investment power with respect to the shares held by this stockholder.
- (10) James Silverman shares voting and investment power with respect to the shares held by this stockholder.
- (11) Joseph Edelman shares voting and investment power with respect to the shares held by this stockholder.
- (12) William Lawrence Hawkins shares voting and investment power with respect to the shares held by this stockholder.
- (13) Empery Asset Management LP, the authorized agent of Hartz Capital Investments, LLC ("HCI"), has discretionary authority to vote and dispose of the shares held by HCI and may be deemed to be the beneficial owner of these shares. Martin Hoe and Ryan Lane, in their capacity as investment managers of Empery Asset Management LP, may also be deemed to have investment discretion and voting power over the shares held by HCI. Mr. Hoe and Mr. Lane disclaim any beneficial ownership of these shares.
- (14) Empery Asset Management LP, the authorized agent of Empery Asset Master Ltd ("EAM"), has discretionary authority to vote and dispose of the shares held by EAM and may be deemed to be the beneficial owner of these shares. Martin Hoe and Ryan Lane, in their capacity as investment managers of Empery Asset Management LP, may also be deemed to have investment discretion and voting power over the shares held by EAM. EAM, Mr. Hoe and Mr. Lane each disclaim any beneficial ownership of these shares.
- (15) Susan Lane shares voting and investment power with respect to the shares held by this stockholder.
- (16) Michael Borelbach shares voting and investment power with respect to the shares held by this stockholder.
- (17) Brandon Lee Jones shares voting and investment power with respect to the shares held by this stockholder.
- (18) Adam Karkowsky shares voting and investment power with respect to the shares held by this stockholder.
- (19) Michael Borelbach shares voting and investment power with respect to the shares held by this stockholder.
- (20) Kevin Kotter shares voting and investment power with respect to the shares held by this stockholder.
- (21) Pedro Mendes Machado shares voting and investment power with respect to the shares held by this stockholder.
- (22) Includes 4,167 shares of common stock issuable upon exercise of employee stock options which have vested or will vest within 60 days of the date hereof.
- (23) Quintessence Fund L.P., QVT Fund IV LP, QVT Fund V LP and Fourth Avenue Capital Partners LP, together with their affiliates (and any other persons or entities whose beneficial ownership of common stock may be aggregated with them for purposes of Section 13(d) of the Exchange Act) (collectively, the "QVT Affiliates"), are collectively subject to a 9.99% beneficial ownership limitation with respect to the Warrants held by the QVT Affiliates. This beneficial ownership limitation prohibits the QVT Affiliates from acquiring shares of common stock upon conversion of the Warrants held by the QVT Affiliates to the extent that, upon such exercise, the number of shares of common stock then beneficially owned by the

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

(24) Patrick Wilmerding shares voting and investment power with respect to the shares held by this stockholder.

Except as described above, none of the selling stockholders has had, within the past three years, any position, office or other material relationship with the Company or any of our predecessors or affiliates.

# PLAN OF DISTRIBUTION

Each selling stockholder of the securities and any of their pledgees, assignees and successors-in-interest may, from time to time, sell any or all of their securities covered hereby on the principal OTC Market or any other stock exchange, market or trading facility on which the securities are traded or in private transactions. These sales may be at fixed or negotiated prices. A selling stockholder may use any one or more of the following methods when selling securities:

- · ordinary brokerage transactions and transactions in which the broker-dealer solicits purchasers;
- block trades in which the broker-dealer will attempt to sell the securities as agent but may position and resell a portion of the block as principal to facilitate the transaction;

- purchases by a broker-dealer as principal and resale by the broker-dealer for its account;
- · an exchange distribution in accordance with the rules of the applicable exchange;
- privately negotiated transactions;
- settlement of short sales;
- in transactions through broker-dealers that agree with the Selling Stockholders to sell a specified number of such securities at a stipulated price per security;
- through the writing or settlement of options or other hedging transactions, whether through an options exchange or otherwise;
- a combination of any such methods of sale; or
- any other method permitted pursuant to applicable law.

The selling stockholders may also sell securities under Rule 144 under the Securities Act, if available, rather than under this prospectus.

Broker-dealers engaged by the selling stockholders may arrange for other brokers-dealers to participate in sales. Broker-dealers may receive commissions or discounts from the selling stockholders (or, if any broker-dealer acts as agent for the purchaser of securities, from the purchaser) in amounts to be negotiated, but, except as set forth in a supplement to this prospectus, in the case of an agency transaction not in excess of a customary brokerage commission in compliance with FINRA Rule 2440; and in the case of a principal transaction a markup or markdown in compliance with FINRA IM-2440.

In connection with the sale of the securities or interests therein, the selling stockholders may enter into hedging transactions with broker-dealers or other financial institutions, which may in turn engage in short sales of the securities in the course of hedging the positions they assume. The selling stockholders may also sell securities short and deliver these securities to close out their short positions, or loan or pledge the securities to broker-dealers that in turn may sell these securities. The selling stockholders may also enter into option or other transactions with broker-dealers or other financial institutions or create one or more derivative securities which require the delivery to such broker-dealer or other financial institution of securities offered by this prospectus, which securities such broker-dealer or other financial institution may resell pursuant to this prospectus (as supplemented or amended to reflect such transaction).

The selling stockholders and any broker-dealers or agents that are involved in selling the securities may be deemed to be "underwriters" within the meaning of the Securities Act in connection with such sales. In such event, any commissions received by such broker-dealers or agents and any profit on the resale of the securities purchased by them may be deemed to be underwriting commissions or discounts under the Securities Act. Each selling stockholder has informed the Company that it does not have any written or oral agreement or understanding, directly or indirectly, with any person to distribute the securities. In no event shall any broker-dealer receive fees, commissions and markups which, in the aggregate, would exceed eight percent (8%).

The Company is required to pay certain fees and expenses incurred by the Company incident to the registration of the securities. The Company has agreed to indemnify the selling stockholders against certain losses, claims, damages and liabilities, including liabilities under the Securities Act.

Because selling stockholders may be deemed to be "underwriters" within the meaning of the Securities Act, they will be subject to the prospectus delivery requirements of the Securities Act including Rule 172 thereunder. In addition, any securities covered by this prospectus which qualify for sale pursuant to Rule 144 under the Securities Act may be sold under Rule 144 rather than under this prospectus. The selling stockholders have advised us that there is no underwriter or coordinating broker acting in connection with the proposed sale of the resale securities by the selling stockholders.

We agreed to keep this prospectus effective until the earlier of (i) the date on which the securities may be resold by the selling stockholders without registration and without regard to any volume or manner-of-sale limitations by reason of Rule 144, without the requirement for the Company to be in compliance with the current public information under Rule 144 under the Securities Act or any other rule of similar effect or (ii) all of the securities have been sold pursuant to this prospectus or Rule 144 under the Securities Act or any other rule of similar effect. The resale securities will be sold only through registered or licensed brokers or dealers if required under applicable state securities laws. In addition, in certain states, the resale securities covered hereby may not be sold unless they have been registered or qualified for sale in the applicable state or an exemption from the registration or qualification requirement is available and is complied with.

Under applicable rules and regulations under the Exchange Act, any person engaged in the distribution of the resale securities may not simultaneously engage in market making activities with respect to the common stock for the applicable restricted period, as defined in Regulation M, prior to the commencement of the distribution. In addition, the selling stockholders will be subject to applicable provisions of the Exchange Act and the rules and regulations thereunder, including Regulation M, which may limit the timing of purchases and sales of securities of the common stock by the selling stockholders or any other person. We will make copies of this prospectus available to the selling stockholders and have informed them of the need to deliver a copy of this prospectus to each purchaser at or prior to the time of the sale (including by compliance with Rule 172 under the Securities Act).

#### DESCRIPTION OF OUR COMMON STOCK

#### **Capital Structure**

We currently have authorized capital stock of 120,000,000 shares, of which 100,000,000 shares are designated as common stock, par value \$0.0001 per share, and 20,000,000 shares are designated as preferred stock, par value \$0.0001 per share, of which 1,000 shares are designated as Class A Preferred, par value \$0.001 per share. As of October 24, 2013, 18,386,919 shares of our common stock and 0 shares of our preferred stock were issued and outstanding. As of October 23, 2013, there were 291 holders of record of our common stock.

The holders of our common stock are entitled to one vote per share on matters on which our stockholders vote. There are no cumulative voting rights. Subject to any preferential dividend rights of any outstanding shares of preferred stock, holders of our common stock are entitled to receive dividends, if declared by our board of directors, out of funds that we may legally use to pay dividends. Generally, all matters to be voted on by stockholders must be approved by a majority (or, in the case of election of directors, by a plurality) of the votes entitled to be cast by all shares of our Common Stock that are present in person or represented by proxy. Holders representing 50 percent (50%) of our Common Stock issued, outstanding and entitled to vote, represented in person or by proxy, are necessary to constitute a quorum at any meeting of our stockholders. A vote by the holders of a majority of our outstanding shares is required to effectuate certain fundamental corporate changes such as liquidation, merger or an amendment to our Articles of Incorporation. If we liquidate or dissolve, holders of our common stock are entitled to share ratably in our assets once our debts and any liquidation preference owed to any thenoutstanding preferred stockholders are paid. Our certificate of incorporation does not provide our common stock with any redemption, conversion or preemptive rights.

The Board of Directors of the Company has the authority to designate one or more series of preferred stock with such voting powers, if any, and with such rights, preferences and privileges as the Board of Directors shall determine.

# MARKET PRICE OF AND DIVIDENDS ON THE COMPANY'S COMMON EQUITY AND RELATED STOCKHOLDER MATTERS

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 OTC QB under the trading symbol "RTRX" ("DGTE" prior to December 17, 2012). There has been limited trading in our shares since they became eligible for trading on the OTC QB during the third quarter of 2008.

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

Quarter Ending	H	igh	Low		
Fiscal Year 2013					
Fourth Quarter (through October 23, 2013)	\$	7.25	\$	6.45	
Third Quarter	\$	7.25	\$	4.50	
Second Quarter	\$	9.99	э \$	4.75	
First Quarter	\$	5.78	\$	3.00	
First Quarter	ψ	5.70	φ	5.00	
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	
T' 1 X 2014					
Fiscal Year 2011	¢	0.00	¢	0.00	
First Quarter	\$	0.90	\$	0.90	
Second Quarter	\$	0.90	\$	0.90	
Third Quarter	\$	1.05	\$	0.90	
Fourth Quarter	\$	1.05	\$	0.90	
Fiscal Year 2010					
First Quarter		N/A		N/A	
Second Quarter	\$	0.90	\$	0.90	
-	\$	0.90	э \$	0.90	
Third Quarter	,		•		
Fourth Quarter	\$	0.90	\$	0.90	

As of October 23, 2013, we had approximately 291 holders of record of our common stock.

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.

# DISCLOSURE OF COMMISSION POSITION ON INDEMNIFICATION FOR SECURITIES ACT LIABILITIES

Our company is incorporated under the laws of the State of Delaware. Section 145 of the Delaware General Corporation Law provides that a corporation may indemnify directors and officers as well as other employees and individuals against expenses including attorneys' fees, judgments, fines and amounts paid in settlement in connection with various actions, suits or proceedings, whether civil, criminal, administrative or investigative other than an action by or in the right of the corporation, a derivative action, if they acted in good faith and in a manner they reasonably believed to be in or not opposed to the best interests of the corporation, and, with respect to any criminal action or proceeding, if they had no reasonable cause to believe their conduct was unlawful. A similar standard is applicable in the case of derivative actions, except that indemnification only extends to expenses including attorneys' fees incurred in connection with the defense or settlement of such actions, and the statute requires court approval before there can be any indemnification that may be granted by a corporation's certificate of incorporation, bylaws, agreement, a vote of stockholders or disinterested directors or otherwise.

The Company's bylaws provide that the Company will indemnify and hold harmless, to the fullest extent permitted by Section 145 of the Delaware General Corporation Law, as amended from time to time, each person that such section grants us the power to indemnify.

The Delaware General Corporation Law permits a corporation to provide in its certificate of incorporation that a director of the corporation shall not be personally liable to the corporation or its stockholders for monetary damages for breach of fiduciary duty as a director, except for liability for:

- · any breach of the director's duty of loyalty to the corporation or its stockholders;
- · acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;
- · payments of unlawful dividends or unlawful stock repurchases or redemptions; or
- · any transaction from which the director derived an improper personal benefit.

The Company's certificate of incorporation provides that, to the extent permitted by applicable law, none of our directors will be personally liable to the Company or its stockholders for monetary damages for breach of fiduciary duty as a director. Any repeal or modification of this provision will be prospective only and will not adversely affect any limitation, right or protection of a director of our company existing at the time of such repeal or modification.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers or persons controlling the Company pursuant to the foregoing provisions, the Company has been informed that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event a claim for indemnification against such liabilities (other than payment by us for expenses incurred or paid by a director, officer or controlling person of our company in successful defense of any action, suit, or proceeding) is asserted by a director, officer or controlling person in connection with the securities being registered, we will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction, the question of whether such indemnification by it is against public policy in the Securities Act and will be governed by the final adjudication of such issue.

## LEGAL MATTERS

The validity of our common stock offered hereby has been passed upon by Katten Muchin Rosenman LLP, New York, New York.

# EXPERTS

The audited consolidated financial statements of Retrophin, Inc., our predecessor, and its subsidiaries as of December 31, 2012 and 2011 and the related statements of operations, changes in stockholder's deficiency and cash flows for the year ended December 31, 2012 and the periods from March 11, 2011 (inception) through December 31, 2012 and March 11, 2011 (inception) through December 31, 2012 and March 11, 2011 (inception) through December 31, 2011, and the unaudited financial statements for the period from March 11, 2011 (inception) through December 31, 2011, for the six months ended June 30, 2013 and for the period from March 11, 2011 (inception) through June 30, 2013, and related footnotes appearing in this registration statement, have been so included in reliance on the Report of Independent Registered Public Accounting Firm of Marcum LLP, an independent registered public accounting firm, appearing elsewhere in this prospectus, given on the authority of such firm as experts in accounting and auditing.

# WHERE YOU CAN FIND ADDITIONAL INFORMATION

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

# **RETROPHIN, INC.** (A DEVELOPMENT STAGE COMPANY)

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# Item 1. Financial Statements

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

	ne 30, 2013 .inaudited)	Dece	ember 31, 2012
Assets			
Current assets:			
Cash	\$ 286,514	\$	11,388
Prepaid expenses and other current assets	 117,895		21,830
Total current assets	404,409		33,218
Property and equipment, net	28,642		23,790
Patents pending	18,093		18,093
Due from affiliate	-		137,547
Security deposit	137,547		-
Technology license, net	2,083,851		2,178,617
Total assets	\$ 2,672,542	\$	2,391,265
Liabilities and Stockholders' Deficit			
Current liabilities:			
Technology license liability	\$ -	\$	1,300,000
Accounts payable	1,425,707		1,023,320
Accrued expenses	741,610		2,467,796
Registration payment obligation	360,000		-
Settlement payable	1,691,400		-
Note payable - related party	-		884,764
Investors' deposits	-		100,000
Due to related parties	10,000		23,200
Derivative financial instruments, at estimated fair value - warrants	6,901,223		-
Total current liabilities	 11,129,940		5,799,080
Stockholders' Deficit:			
Preferred stock Series A \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding	-		-
Common stock \$0.0001 par value; 100,000,000 shares authorized; 12,321,973 and 8,952,905			
issued and outstanding, respectively	1,232		895
Additional paid-in capital	34,944,917		30,203,402
Deficit accumulated during the development stage	(43,403,547)		(33,612,112)
Total stockholders' deficit	 (8,457,398)		(3,407,815)
Total liabilities and stockholders' deficit	\$ 2,672,542	\$	2,391,265

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

	For the three months ended June30,For the six months ended June 30,2013201220132012								fro	or the period om March 11, 2011 (inception) nrough June 30, 2013
Operating expenses:		2010		=01=		2010				50, 2015
Compensation and related costs - inclusive of share base compensation \$57,516, \$1,391,863, \$78,349, \$2,943,767 and \$17,816,166	\$	492,131	\$	1,532,943	\$	1,537,418	\$	3,302,774	\$	21,898,171
Professional fees - inclusive of share based compensation		,								
\$70,872, \$2,647,001, \$209,243, \$3,650,125 and \$6,860,948		1,701,264		3,062,461		2,393,841		4,770,890		12,497,693
Selling, general and administrative		2,906,507		144,288		3,319,127		224,482		4,675,235
Technology license contingent fee		-		-		100,000		-		1,800,000
Total operating expenses		5,099,902		4,739,692		7,350,386		8,298,146		40,871,099
							-			
Operating loss		(5,099,902)		(4,739,692)	\$	(7,350,386)		(8,298,146)		(40,871,099)
Other income (expense):										
Interest income		5		5,984		5		9,732		21,910
Interest expense		-		(26,471)		(41,563)		(43,798)		(147,480)
Change in fair value of derivative financial instruments -										
warrants		56,041		-		(2,395,618)		-		(2,395,618)
Loss on transactions denominated in foreign currencies		(4,657)		-		(3,873)		-		(11,260)
Total other income (expense), net		51,389		(20,487)		(2,441,049)		(34,066)		(2,532,448)
							_			
Net loss	\$	(5,048,513)	\$	(4,760,179)	\$	(9,791,435)	\$	(8,332,212)	\$	(43,403,547)
					_		_		_	
Net loss per common share, basic and diluted	\$	(0.41)	\$	(1.55)	\$	(0.85)	\$	(3.00)		
Weighted average common shares outstanding, basic and diluted	_	12,253,599	_	3,064,764		11,492,475	_	2,781,992		

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

	Common	Common stock Additional Receivables due		Rocoivables due	Accumulated	Total Stockholders'
	Shares	Amount	paid in capital	from stockholder	deficit	deficit
					•	*
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		-	100
Incentive shares granted- employees	1,758,300	176	(176)	-	-	-
Incentive shares granted- non employees	381,000	38	(38)	-	-	-
Incentive shares forfeited - employees	(45,835)	(5)	5	-	-	-
Share based compensation - employees	-	-	1,724,967	-	-	1,724,967
Share based compensation - non employees	-	-	254,332	-	-	254,332
Issuance of shares in connection with March 2011 private placement, net of fess of \$66,061	253,750	25	658,914	-	-	658,939
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	-	-	103,633
Loan made to stockholder	-	-	-	(10,000)	-	(10,000)
Net loss	-	-	-	-	(3,268,256)	(3,268,256)
Balance - December 31, 2011	4.042.265	404	2,766,567	(35,000)	(3,268,256)	(536,285)
	,- ,		,,	(	(-,,,	()
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	-	-	1,806,677
Prior Issuance of Series A preferred in connection with May 2012 private	520,505	00	1,000,011			1,000,077
placement, net of feees of \$12,275, exchanged to common stock	470,764	47	1.668.979	-	-	1,669,026
Shares transferred to consultants by founder for services	470,704		4,400,000	_	_	4,400,000
Shares transferred to employees by founder for services		-	1,375,000			1,375,000
Shares issued in accordance with license agreement	620,000	62	1,549,938			1,550,000
Shares outstanding at time of reverse merger date December 12, 2012	2,585,583	259	1,142	-	-	1,401
Incentive shares granted- employees	2,505,505	259	(86)	-	-	1,401
Incentive shares granted - non employees	87,503	9	(80)	-	-	-
Incentive shares forfeited - employees			(9)	-	-	-
	(46,353)	(5)		-	-	14 027 050
Share based compensation - employees	-	-	14,637,850	-	-	14,637,850
Share based compensation - non employees	-	-	1,997,372	-	-	1,997,372
Receivable due from stockholder charged to compensation	-	-	-	407,900	-	407,900
Loan made to stockholder	-	-	-	(372,900)	-	(372,900)
Net loss	-		-		(30,343,856)	(30,343,856)
Balance - December 31, 2012	8,952,905	895	30,203,402	-	(33,612,112)	(3,407,815)
Incentive shares granted - non employees (unaudited)	12,500	1	(1)	-	-	-
Incentive shares granted - employees (unaudited)	120,000	12	(12)	-	-	-
Share based compensation - employees (unaudited)	-	-	78,349	-	-	78,349
Share based compensation - non employees (unaudited)	-	-	209,243	-	-	209,243
Incentive shares forfeited - employees (unaudited)	(20,833)	(2)	2	-	-	-
Incentive shares forfeited - non employees (unaudited)	(72,082)	(7)	7	-	-	-
Issuance of common stock in connection with January 2013 private placement						
at \$3.00 per share, net of fees of \$0 (unaudited)	272,221	27	816,637	-	-	816,664
Issuance of common stock in connection with February 2013 private placement						
at \$3.00 per share, net of fees of \$678,986						
and registration payment obligation of \$360,000 (unaudited)	3,045,929	305	3,592,891	-	-	3,593,196
Shares issued on behalf of related party	6,000	1	44,399	-	-	44,400
Adjustment to existing shareholder (unaudited)	5,333	-	-	_	-	-
Net loss (unaudited)		-	-	-	(9,791,435)	(9,791,435)
Balance - June 30, 2013 (unaudited)	12,321,973	\$ 1.232	\$ 34,944,917	\$ -	\$ (43,403,547)	\$ (8,457,398)
	12,021,070	φ <u>1,202</u>	\$ 57,577,517	÷	¢ (+0,+00,0+7)	¢ (0,407,000)

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

		For the six month	ıs ende	d June 30,	Μ	the period from arch 11, 2011 eption) through
		2013		2012		une 30, 2013
Cash Flows From Operating Activities:						
Net loss	\$	(9,791,435)	\$	(8,332,212)	\$	(43,403,547)
Adjustments to reconcile net loss to net cash used in operating activities:						
Depreciation and amortization		105,307		37,695		230,547
Compensation in lieu of receivable		-		-		407,900
Share based compensation - employees		78,349		2,943,767		17,816,166
Share based compensation - non-employees		209,243		3,650,125		6,860,947
Share based payment - Technology license contingent fee		-		-		1,550,000
Change in estimated fair value of derivative financial instruments - warrants		2,395,618		-		2,395,618
Changes in operating assets and liabilities:				(6.022)		(115,005)
Prepaid expenses		(96,065)		(6,032)		(117,895)
Accounts payable and accrued expenses		312,001		89,102		3,800,796
Net cash used in operating activities		(6,786,982)		(1,617,555)		(10,459,468)
Cash Flows From Investing Activities:						
Purchase of fixed assets		(9,693)		(3,490)		(37,339)
Purchase of intangible assets		(5,700)		(1,082,971)		(1,173,793)
Repayment of technology license liability		(1,300,000)		-		(1,150,000)
Cash received in merger transaction		(1,000,000)		-		3,721
Payments made on behalf of affiliate		-		-		(137,547)
Loans made to stockholder		-				(382,900)
Increase in note receivable - related party		-		(193,500)		-
Net cash used in investing activities		(1,315,393)		(1,279,961)		(2,877,858)
- · · · · · · · · · · · · · · · · · · ·		(_,,,)		(_, ; ; ; ; ; ; ; ; )		(_,,)
Cash Flows From Financing Activities:						
Proceeds from related parties		-		-		56,500
Repayment of net amounts due to related parties		(13,200)		-		(46,500)
Proceeds from note payable - related party		-		838,764		930,000
Repayment of note payable - related party		(884,764)		-		(930,000)
Investors' deposits		-		-		100,000
Proceeds received from issuance of common stock, net of fees of \$678,986,						
\$73,952, and \$820,367, respectively		9,275,465		2,045,115		13,513,840
Repayment of stock subscription receivable		-		35,000		-
Net cash provided by financing activities		8,377,501		2,918,879		13,623,840
Net increase in cash		275,126		21,363		286,514
Cash, beginning of period		11,388		10,053		-
Cash, end of period	\$	286,514	\$	31,416	\$	286,514
Supplemental Disclosure of Cash Flore Information						
Supplemental Disclosure of Cash Flow Information: Cash paid for interest	\$	28,263	\$	-	\$	43,027
•	Ψ	20,205	Ψ		Ψ	45,027
Non-cash investing and financing activities:	¢		¢		¢	25.000
Forfeiture of subscription receivable	\$	-	<u>э</u>	-	\$	25,000
Shares issued on behalf of related party	\$	44,400	\$		\$	44,400
Allocation of proceeds from issuance of common stock to registration payment arrangment	\$	360,000	\$	-	\$	360,000
						· · · ·

#### RETROPHIN, INC. AND SUBSIDIARY (A DEVELOPMENT STAGE COMPANY) NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

# NOTE 1. DESCRIPTION OF BUSINESS

## Organization and Description of Business

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

## NOTE 2. BASIS OF PRESENTATION

The accompanying unaudited condensed consolidated financial statements of the Company should be read in conjunction with the audited consolidated financial statements and notes thereto included in the Company's Annual Report on Form 10-K /A for the year ended December 31, 2012 (the "2012 10-K/A") filed with the Securities and Exchange Commission (the "SEC") on September 13, 2013. The accompanying condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP") for interim financial information, the instructions to Form 10-Q and the rules and regulations of the SEC. Accordingly, since they are interim statements, the accompanying condensed consolidated financial statements do not include all of the information and notes required by GAAP for annual financial statements, but reflect all adjustments consisting of normal, recurring adjustments, that are necessary for a fair presentation of the financial position, results of operations and cash flows for the interim periods presented. Interim results are not necessarily indicative of results for a full year. The December 31, 2012 balance sheet information was derived from the audited financial statements as of that date.

## NOTE 3. LIQUIDITY, FINANCIAL CONDITION AND MANAGEMENT PLANS

The Company incurred a net loss of approximately \$43 million, including stock-based compensation charge of approximately \$25 million for the period from March 11, 2011 (inception) to June 30, 2013. At June 30, 2013, the Company had a cash balance of approximately \$286,500 and a working capital deficit of approximately \$10.7 million. The Company's accumulated deficit amounted to \$43,403,547 at June 30, 2013.

The Company has principally financed its operations from inception using proceeds from sales of its equity securities in a series of private placement transactions (see Note 10 and Note 12). The Company to date has no revenues, significantly limited capital resources and is subject to all of the risks and uncertainties that are typical of a development stage enterprise. Significant uncertainties include, among others, whether it will be able to raise the capital it needs to finance the start of its planned operations and whether such operations, if launched, will enable the Company to become a profitable enterprise.

On 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 (Note 13); 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.

The Company entered into an additional settlement agreement on August 29, 2013 (Note 13).

On August 16, 2013, the Company announced that it had signed an agreement with a major pharmaceutical company for the exclusive right to negotiate a royalty-bearing U.S. license for a product to be developed for the treatment of Autism and Schizophrenia. Pursuant to the exclusivity agreement, the Company paid the major pharmaceutical company a non-refundable upfront fee and will have an exclusive period of 120 days to negotiate a license agreement (see Note 13).

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 \$2,286,511 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 June 30, 2013, which is entirely past due as of the date of this filing. The counter parties to these agreements reserve the right to demand payment at any time. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the second quarter ended June 30, 2013. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary (see Note 9).

On August 29, 2013, the Company entered into an additional settlement agreement for \$300,000 due following execution of the agreement. As of the date of this filing, this settlement has been paid (see Note 13).

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

Management believes the Company's ability to continue its operations depends on its ability to raise capital. In February 2012, the Company entered into a license agreement providing it with the use of certain technology. The Company is currently developing pre-clinical and clinical studies of drug candidates. The license agreement (described in Note 7) also enables the Company to sell the licensed technology as a research product or sublicense the technology to other third parties as alternative sources of revenue to its own product development efforts. The Company's future depends on the costs, timing, and outcome of regulatory reviews of its product candidates and the costs of commercialization activities, including product marketing, sales and distribution. During the first quarter of 2013, the Company raised an aggregate of approximately \$9.95 million in certain private placement transactions. During the third quarter of 2013, the Company raised an additional \$25 million in aggregate proceeds in connection with a private placement transaction. The Company expects to continue to finance its cash needs through additional private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, 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 4. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

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

#### Principles of Consolidation

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

#### Cash and Cash Equivalents

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

#### Property and Equipment

Property and equipment are stated at cost. Depreciation is provided for using the straight-line method over the estimated useful lives of the assets. At June 30, 2013 and 2012, property and equipment consisted of computers with an estimated useful life of three years, leasehold improvements with an estimated life of four years and furniture with an estimated life of seven years.

#### Employee Stock-Based Compensation

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

#### Non-Employee Stock-Based Compensation

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

#### Use of Estimates

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

#### Research and Development Costs

Research and development costs are charged to operations as incurred and consist primarily of consulting costs, contract research and development costs, and compensation costs. For the three and six months ended June 30, 2013 and 2012, and for the period from March 11, 2011 (inception) through June 30, 2013, the Company recognized \$605,203, \$141,962, \$713,937, \$176,233, and \$1,608,451, respectively, of research and development costs.

#### Patents

The Company capitalized external cost, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company expense cost associated with maintaining and defending patents subsequent to their issuance in the period incurred. The Company amortizes patent cost once issued on a straight-line basis over the estimate useful lives of the patents. The Company assess the potential impairment to all capitalized patent cost when events or changes in circumstances indicate that the carrying amount of our patent may not be recoverable.

#### 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 1,597,969 and 0 warrants were excluded from the computation of diluted net loss per common share for the three and six months ended June 30, 2013 and 2012 because to do so would have an anti-dilutive effect for the periods presented.

An aggregate of 120,000 and 0 stock options were excluded from the computation of diluted net loss per common share for the three and six months ended June 30, 2013 and 2012 because they were contingent options subject to recall and to do so would have an anti-dilutive effect for the periods presented.

An aggregate of 231,493 and 1,973,142 common stock equivalents (incentive shares) were excluded from the computation of diluted net loss per common share for the three and six months ended June 30, 2013 and 2012 because they were contingent shares subject to recall.

#### Derivative Instruments

The Company does not use derivative instruments to hedge exposures to cash flow, market or foreign currency risks. The Company evaluates all of its financial instruments to determine if such instruments are derivatives or contain features that qualify as embedded derivatives. For derivative financial instruments that are accounted for as liabilities, the derivative instrument is initially recorded at its fair value and is then revalued at each reporting date, with changes in the fair value reported in the statements of operations. For stock-based derivative financial instruments, the Company calculates the fair value of the financial instruments using a probability-weighted Black-Scholes option pricing model, which is comparable to the Binomial Lattice options pricing model at inception and on each subsequent valuation date. The classification of derivative instruments, including whether such instruments should be recorded as liabilities or as equity, is evaluated at the end of each reporting period. (See Note 5 and Note 6).

#### Joint and Several Liability Assessment

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

## Financial Instruments and Fair Value

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

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

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

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

In estimating the fair value of the Company's derivative liabilities, the Company used a probability-weighted Black-Scholes option pricing model. (See Note 5 and Note 6).

Financial assets with carrying values approximating fair value include cash and cash equivalents as well as prepaid expenses and other current assets. Financial liabilities with carrying values approximating fair value include accounts payable and other accrued liabilities.

## Registration Payment Arrangement

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

#### Subsequent Events

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

## Recently Issued Accounting Pronouncements

In February 2013, the FASB issued Accounting Standards Updated ("ASU") 2013-04 "Obligations Resulting from Joint and Several Liability Arrangements for Which the Amount at the Reporting Date is Fixed") ("ASU 2013-04"). The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount the entity has (a) contractually agreed to pay, and (b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company early adopted this guidance in the second quarter of 2013 (Note 9 and Note 13).

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

## NOTE 5. DERIVATIVE FINANCIAL INSTRUMENTS

In accordance with ASC Topic 815-40, "Derivative and Hedging – Contracts in Entity's Own Equity" ("ASC Topic 815-40"), instruments which do not have fixed settlement provisions are deemed to be derivative instruments. Based upon the Company's analysis of the criteria contained in ASC Topic 815-40, the warrants issued in connection with the sale of the common stock during the period ended June 30, 2013 that do not have fixed settlement provisions, are not indexed to Company's own stock. The fair value of the warrants are classified as derivative liabilities due to a ratchet provision that allows for a favorable adjustment to the exercise price if the Company issues additional equity instruments in the future at an effective price per share less than the exercise price then in effect.

The warrants are re-measured at each balance sheet date based on estimated fair value. Changes in estimated fair value are recorded as non-cash valuation adjustments within other income (expense) in the Company's results of operations. The Company recorded a gain and loss on a change in the estimated fair value of warrants of \$56,041 and \$2,395,618 for the three and six months ended June 30, 2013, respectively.

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

	As	of
	Date of issuance	June 30, 2013
	February 14, 2013	
Fair market price of common stock	\$3.75	\$5.50
Expected option term	5 years	4.63 years
Risk-free interest rate	0.86%	1.41%
Expected volatility	101%	98.59%

Expected volatility is based on historical stock volatilities of several comparable publicly-traded companies over a period equal to the expected terms of the warrants, as the Company does not have a long trading history to estimate the volatility of its own common stock. The warrants have a transferability provision. Based on guidance provided in SEC Staff Accounting Bulletin No. 107 ("SAB 107") for options issued with such a provision, the Company used the full contractual term as the initial expected term of the warrants. The risk free interest rate is based on the U.S. Treasury security rates for the remaining term of the warrants at the measurement date.

## NOTE 6. FAIR VALUE MEASUREMENTS

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

		Fair Value Measurements at June 30, 2013							
	al carrying e at June 30, 2013	acti	ted prices in ve markets Level 1)	obser	ficant other vable inputs Level 2)	un	ignificant observable uts (Level 3)		
Derivative liabilities related to warrants	\$ 6,901,223	\$	-	\$	-	\$	6,901,223		

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

	Common Stoc Significant Un	leasurements of k Warrants Using observable Inputs evel 3)
Balance at December 31, 2012	\$	-
Issuance of common stock warrants		4,505,605
Change in fair value of common stock warrant liability		2,395,618
Balance at June 30, 2013	\$	6,901,223

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. LICENSE AGREEMENT

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

In accordance with the agreement as amended most recently as of January 7, 2013, the Company made two non-refundable payments totaling \$2,550,000, the first payment of \$1,150,000 made upon execution and the second payment of \$1,400,000 made in February 2013, which includes a \$250,000 fee payable to the sublicensee in exchange for extended due date of this payment from October 1, 2012 to February 2013. As of June 30, 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 of extension fees were expensed to operations in February 2013. In addition, the Company issued 620,000 common shares to Ligand valued at \$1,550,000 as a result of the merger transaction, the amount of which was expensed to operations in December 2012. For the three and six months ended June 30, 2013 and 2012, and for the period from March 11, 2011 (inception) through June 30, 2013, the Company recognized amortization expense of the license related to this agreement of \$50,511, \$36,581, \$100,466, \$36,581, and \$221,849 respectively.

## NOTE 8. NOTES PAYABLE

#### Note Payable - related party

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

Total interest expense recognized for the three and six months ended June 30, 2013 and 2012, and for the period from March 11, 2011 (inception) through June 30, 2013 amounted to \$0, \$26,471, \$19,733, \$43,798 and \$147,480, respectively.

## NOTE 9. RELATED PARTY TRANSACTIONS

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

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

In the second quarter of 2013, the Company, its Chief Executive Officer and a related party , which is a former investor in the Company that was previously managed by the Company's Chief Executive Officer, became party to a series of agreements to settle up to \$2,286,511 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 \$2,286,511 charge to operations during the quarter ended June 30, 2013 and a corresponding liability of \$1,691,400 for the difference between (a) the aggregate amount of all such settlements, and (b) \$593,111 of cash and non-cash consideration that the Company paid to immediately settle a portion of the agreement on behalf of the related party. The \$1,691,400 is past due as of the August 9, 2013. The counter parties to these agreements reserve the right to demand payment at any time. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the second quarter ended June 30, 2013. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary. As of the date of this filing, the Company owes \$1,655,000 in cash and 5,000 shares of common stock valued at \$36,400, due immediately.

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

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

On August 15, 2013, the Company closed a private placement and sold 5,536,957 shares of the Company's common stock, at a purchase price of \$4.50 per share, or approximately \$25,000,000 in the aggregate, and warrants to purchase up to an aggregate of 2,768,479 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 16,078 shares of common stock and warrants to purchase up to an aggregate of 8,039 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 10. STOCKHOLDERS' DEFICIT

#### Issuances

Common Stock

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

On February 14, 2013, the Company closed a private placement (the "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.

On February 14, 2013, in connection with the closing of the Private Placement, the Company entered into a Registration Rights Agreement (the "Registration Rights Agreement") with the purchasers in the 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 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 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 Notes 2 and 13, 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.

Subsequent to June 30, 2013, in connection with a private placement transaction which closed in August 2013, the Company amended the February 2013 Registration Rights Agreement (see Note 13).

On May 13, 2013, the Company issued options (the "Options") to purchase 120,000 shares of common stock in connection with an employment agreement with Horacio Plotkin, M.D. (the "Plotkin Employment Agreement") pursuant to which Dr. Plotkin was appointed as Chief Medical Officer of the Company. The options vest quarterly on a pro rata portion during the 3 years following the effective date of July 1, 2013. The Company valued these Options using the Black-Scholes options pricing model and the following assumption terms: risk-free interest rate of .83% (based on the US Treasury note yield), expected term (in years) of 5.81 (based on guidance provided in SAB 107 that allows the Company to use the simplified method for "plain vanilla" options for this calculation), expected volatility of 98.56% (based on historical stock volatilities of several comparable publicly-traded companies over a period equal to the expected term of the options, as the Company does not have a long trading history to estimate the volatility of its own common stock), and an exercise price equal to the fair value of the stock on the date of issuance of \$8.70 per share. For the three and six months ended June 30, 2013 the Company recognized \$36,683 as compensation expense related to the Options. At June 30, 2013, the unrecognized compensation expense, remaining amortization period, intrinsic value and remaining contract life of the Options are \$768,049, 2.75 years, \$0 and 9.87 years, respectively.

## NOTE 11. INCENTIVE SHARES

At June 30, 2013, the Company did not have any active share-based compensation plans available for grants to employees, non-employee directors and consultants. Since its inception, the Company has granted incentive shares.

For the three and six months ended June 30, 2013 and 2012, and for the period from March 11, 2011 (inception) through June 30, 2013, the Company recognized \$91,705, \$4,038,864, \$250,909, \$6,593,892 and \$24,640,431 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 June 30, 2013, there was approximately \$1,114,984, of unrecognized compensation cost related to incentive shares issued. This amount is expected to be recognized over a weighted average of 2.17 years.

	Employee - number of shares	Non Employee - number of shares	Total number of shares	0	ted Average ir Value
Unvested December 31, 2011	1,281,225	321,165	1,602,390	\$	4.00
Granted	866,180	87,503	953,683		12.89
Vested	(2,048,280)	(193,672)	(2,241,952)		7.34
Forfeited	(46,353)	-	(46,353))		9.06
Unvested December 31, 2012	52,772	214,996	267,768		5.79
Granted	120,000	12,500	132,500		6.17
Vested	(11,112)	(64,748)	(75,860)		7.04
Forfeited	(20,833)	(72,082)	(92,915)		4.00
Unvested June 30, 2013	140,827	90,666	231,493	\$	4.82

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

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

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

## NOTE 12. COMMITMENTS AND CONTINGENCIES

The Company assumed a building lease from an affiliate in April 2013 and is responsible for rent of approximately \$275,000 annually through August 2016 (see Note 9).



The Company is obligated to pay \$1,835,000 and 73,710 shares of Common Stock in liquidated damages in accordance with the February 14, 2013 Registration Rights Agreement (see Note 10).

The Company is required to make an upfront fee payment in connection with an exclusivity agreement entered into with a major pharmaceutical company in August 2013 (see Note 13).

# NOTE 13. SUBSEQUENT EVENTS

#### First Amendment to the February Registration Rights Agreement

As described in Notes 3 and 10, 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 from its February 14, 2013 private placement to be declared effective within certain contractually defined time-frames; (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 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 will record 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 to be taken as a charge to operations and the remaining a

#### Private Placement Transaction

On August 15, 2013, the Company closed a private placement and sold 5,536,957 shares of the Company's common stock, at a purchase price of \$4.50 per share, or approximately \$25,000,000 in the aggregate, and warrants to purchase up to an aggregate of 2,768,479 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 16,078 shares of common stock and warrants to purchase up to an aggregate of 8,039 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.

#### Exclusivity Agreement

On August 16, 2013, the Company announced that it had signed an agreement with a major pharmaceutical company for the exclusive right to negotiate a royalty-bearing U.S. license for a product to be developed for the treatment of Autism and Schizophrenia. Pursuant to the exclusivity agreement, the Company paid the major pharmaceutical company a non-refundable upfront fee and will have an exclusive period of 120 days to negotiate a license agreement. Upon execution of a license agreement, the Company would receive the exclusive right to the intellectual property to develop, manufacture and sell the product in the United States and would pay an additional fee to the major pharmaceutical company.

#### Additional Settlement Agreement

On August 29, 2013, the Company entered into an additional settlement agreement for \$300,000 due following execution of the agreement. The Company charged this amount to selling, general and administrative expense in its statement of operations. As of the date of this filing, this settlement has been paid.

#### Additional Events Subsequent to September 13, 2013 (Unaudited)

#### Acquisition Proposal

On September 18, 2013, the Company made a proposal to the board of directors of Transcept Pharmaceuticals, Inc. ("<u>Transcept</u>") to acquire all of the outstanding shares of Transcept's common stock for \$4.00 per share in cash. The proposal has been rejected by Transcept's board of directors. The Company has invested approximately \$3 million and acquired approximately 4.96% of the outstanding common stock of Transcept as part of the proposal process.

#### Leases

On October 1, 2013 the Company entered into a building lease for approximately 4,232 square feet of office space located in Cambridge, MA. The Company is responsible for approximately \$216,000 annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes, under a lease agreement expiring September 2016.

On October 8, 2013, the Company amended its lease agreement for its principal office located in New York City. The Company expanded its principal office is responsible for an additional rent of approximately \$225,000 annual base rent plus rent escalations, common area maintenance, insurance, and real estate taxes, under a lease agreement expiring September 2016.

#### Research Agreement

Effective October 1, 2013, the Company signed a Sponsored Research Agreement ("SRA") with 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.

#### Settlement Payments

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

In October 2013, 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.

## REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

We have audited the accompanying consolidated balance sheets of Retrophin, Inc. and Subsidiary (a development stage company) (the "Company") as of December 31, 2012 and 2011 and the related consolidated statements of operations, changes in stockholder's deficit and cash flows for the year ended December 31, 2012, for the periods from March 11, 2011 (inception) through December 31, 2011 and March 11, 2011 (inception) through December 31, 2012. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

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

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the consolidated financial position of Retrophin, Inc. and Subsidiary (a development stage company) as of December 31, 2012 and 2011, and the consolidated results of its operations and its cash flows for the year ended December 31, 2012, for the periods from March 11, 2011 (inception) through December 31, 2011 and from March 11, 2011 (inception) through December 31, 2012, in conformity with accounting principles generally accepted in the United States of America.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 2 to the consolidated financial statements, the Company is a development stage enterprise with no revenues, historical losses and limited capital resources. The Company, as a development stage enterprise, is subject to risks and uncertainties as to whether it will be able to raise capital and commence its planned operations. These conditions raise substantial doubt about the Company's ability to continue as a going concern. Management's plans regarding these matters also are described in Note 2. The consolidated financial statements do not include any adjustments relating to the recovery of assets or classification of liabilities might be necessary should the Company be unable to continue as a going concern.

/s/ Marcum LLP

New York, NY June 13, 2013, except paragraph numbers 3, 4, 5 and 6 of Note 2 and paragraph numbers 3, 8, 9 and 10 of Note 12, as to which the date is September 13, 2013.

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

	Dece	mber 31, 2012	December 31, 2011		
Assets					
Current assets					
Cash	\$	11,388	\$	10,053	
Other current assets		21,830		7,000	
Total current assets		33,218		17,053	
Property and equipment, net		23,790		2,517	
Patents pending		18,093		-	
Due from affiliate		137,547			
Technology license, net		2,178,617		-	
Total assets	\$	2,391,265	\$	19,570	
Liabilities and Stockholders' Deficit					
Liabilities					
Current liabilities					
Technology license liability	\$	1,300,000	\$	-	
Accounts payable		1,023,320		340,134	
Accrued expenses		2,467,796		169,721	
Note payable – related party		884,764		-	
Investors deposit		100,000		-	
Due to related parties		23,200		46,000	
Total liabilities		5,799,080		555,855	
Stockholders' Deficit					
Preferred stock Series A \$0.001 par value; 20,000,000 authorized; 0 and 0 issued and outstanding, respectively		_		-	
Common stock \$0.0001 par value; 100,000,000 authorized; 8,952,905 and 4,042,265 issued and					
outstanding, respectively		895		404	
Additional paid-in capital		30,203,402		2,766,567	
Subscription receivable - Stockholder		-		(35,000)	
Deficit accumulated during the development stage		(33,612,112)		(3,268,256)	
Total stockholders' deficit		(3,407,815)		(536,285)	
Total liabilities and stockholders' deficit	\$	2,391,265	\$	19,570	

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

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

	For the year ended December 31, 2012		Ma (ince	For the period from March 11, 2011 (inception) through December 31, 2011		the period from farch 11, 2011 ception) through cember 31, 2012
Operating expenses:						
Compensation and related costs - inclusive of share based compensation						
\$16,012,850, \$1,724,967 and \$17,737,817	\$	18,133,550	\$	2,227,203	\$	20,360,753
Professional fees - inclusive of share based compensation \$6,397,372, \$254,332,						
and \$6,651,704		9,035,702		909,681		9,945,383
Selling, general and administrative		1,292,296		63,812		1,356,108
Technology license contingent fees		1,700,000		-		1,700,000
Rent		95,469		63,000		158,469
Total operating expenses		30,257,017		3,263,696		33,520,713
Other income (expense):						
Interest income		21,830		75		21,905
Interest expense		(105,917)		-		(105,917)
Loss on transactions denominated in foreign currencies		(2,752)		(4,635)		(7,387)
Total other expense		(86,839)		(4,560)		(91,399)
•	_				-	
Net loss	\$	(30,343,856)	\$	(3,268,256)	\$	(33,612,112)
Net loss per common share - basic and diluted	\$	(8.29)	\$	(1.59)		
Weighted average number of common shares outstanding during the period - basic and diluted		3,662,114		2,053,402		

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

# RETROPHIN, INC. AND SUBSIDIARY (A DEVELOPMENT STAGE COMPANY) CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' DEFICIT

			Receivable due Additional paid from		Accumulated	Total Stockholders'	
	Shares	Amount	in capital	stockholder	deficit	deficit	
Balance - March 11, 2011 (inception)	- 3	\$-	\$-	\$-	\$-	\$-	
Issuance of common shares	1,608,300	161	24,839	(25,000)	-	-	
Issuance of common shares to founders in							
connection with the initial capital							
contribution	50,000	5	95	-	-	100	
Incentive shares granted – employees	1,758,300	176	(176)	-	-	-	
Incentive shares granted– non employees	381,000	38	(38)	-	-	-	
Incentive shares forfeited – employees	(45,835)	(5)	5	-	-	-	
Share based compensation - employees	-	-	1,724,967	-	-	1,724,967	
Share based compensation - non employees	-	-	254,332	-	-	254,332	
Issuance of shares in connection with							
March 2011 private placement, net of fees							
of \$66,061	253,750	25	658,914	-	-	658,939	
Issuance of Series A preferred in connection							
with March 2011 private placement, net							
of fees of \$1,367, recapitalization to							
common stock	36,750	4	103,629	-	-	103,633	
Loan made to stockholder	-	-	-	(10,000)	-	(10,000)	
Net loss	-	-	-	-	(3,268,256)	(3,268,256)	
Balance - December 31, 2011	4,042,265	404	2,766,567	(35,000)	(3,268,256)	(536,285)	
Issuance of Series A preferred in connection	1,012,200	101	2,700,007	(55,000)	(0,200,200)	(000,200)	
with January 2012 private placement, net of fees of \$61,677 recapitalized into							
common stock	326,963	33	1,806,644	-	-	1,806,677	
Issuance of Series A preferred in connection							
with May 2012 private placement, net of fees of \$12,275, recapitalized into							
common stock	470,764	47	1,668,979	-	-	1,669,026	
Shares transferred to consultants by founder	., .,,	.,	1,000,070			1,000,010	
for services rendered to the Company	-	-	4,400,000	-	-	4,400,000	
Shares transferred to employees by			.,,			.,,	
founders for services rendered to the							
Company	-	-	1,375,000	-	-	1,375,000	
Shares issued in accordance with			_,			_,,	
technology license agreement	620,000	62	1,549,938	-	-	1,550,000	
Shares outstanding at time of reverse	,		,,			,	
merger completed on December 12, 2012	2,585,583	259	1,142	-	-	1,401	
Incentive shares granted – employees	866,180	86	(86)	-	-	-	
Incentive shares granted– non employees	87,503	9	(9)	-	-	-	
Incentive shares forfeited – employees	(46,353)	(5)	5	-	-	-	
Share based compensation - employees	-	-	14,637,850	-	-	14,637,850	
Share based compensation - non employees	-	-	1,997,372	-	-	1,997,372	
Receivable due from stockholder charged to			,			,	
compensation	-	_	-	407,900	_	407,900	
Loan made to stockholder	-	-	-	(372,900)		(372,900)	
Net loss	-	-	-	-	(30,343,856)	(30,343,856)	
Balance - December 31, 2012	8,952,905	\$ 895	\$ 30,203,402	\$-	\$ (33,612,112)	\$ (3,407,815)	
Summer December 51, 2012	0,002,000	÷ 335	\$ 50,205,702	÷	¢ (00,012,112)	¢ (0,107,010)	

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

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

Cook Elevis Even Operating Activities		For the year ended December 31, 2012		For the period from March 11, 2011 (inception) through December 31, 2011		For the period from March 11, 2011 (inception) through December 31, 2012	
Cash Flows From Operating Activities: Net loss	\$	(30,343,856)	\$	(3,268,256)	\$	(33,612,112)	
Adjustments to reconcile net loss to net cash used in operating activities:	Э	(30,343,050)	Э	(3,200,230)	Ф	(55,012,112)	
Depreciation and amortization		124,885		355		125,240	
Compensation in lieu of stockholder receivable		407,900		222		407,900	
Share based compensation - employees		16,012,850		- 1,724,967		17,737,817	
Share based compensation - employees		6,397,372					
Share based payment - Technology license contingent fee				254,332		6,651,704	
		1,550,000		-		1,550,000	
Changes in operating assets and liabilities:		(14,020)		(7,000)		(21,020)	
Other assets		(14,830)		(7,000)		(21,830)	
Technology license fee		150,000		-		150,000	
Accounts payable		680,865		340,134		1,020,999	
Accrued expenses		2,298,075		169,721		2,467,796	
Net cash (used) in operating activities		(2,736,739)		(785,747)		(3,522,486)	
Cash Flows From Investing Activities:							
Purchase of fixed assets		(24,774)		(2,872)		(27,646)	
Purchase of intangible assets		(1,168,093)		-		(1,168,093)	
Cash received in merger transaction		3,721		-		3,721	
Payments made on behalf of affiliate		(137,547)		-		(137,547)	
Loans made to stockholder		(372,900)		(10,000)		(382,900)	
Net cash (used) in investing activities		(1,699,593)		(12,872)		(1,712,465)	
Cash Flows From Financing Activities:							
Proceeds from advances from related parties		10,500		46,000		56,500	
Repayment of advances from related parties		(33,300)		40,000		(33,300)	
Proceeds from note payable - related party		930,000		-		930,000	
Repayment of note payable - related party		(45,236)		-		(45,236)	
Investor deposit		(45,256)		-		100,000	
Proceeds received from issuance of common stock , net of cost of \$73,952,		100,000		-		100,000	
\$67,428 and \$141,380, respectively		3,475,703		762,672		4,238,375	
Net cash provided in financing activities		4,437,667	_	808,672	_	5,246,339	
	_	-,,-,-,007		000,072		3,240,333	
Net decrease in cash		1,335		10,053		11,388	
Cash, beginning of period		10,053		-		-	
Cash, end of period	\$	11,388	\$	10,053	\$	11,388	
Supplemental Disclosure of Cash Flow Information:							
Cash paid for interest	\$	14,764	\$	-	\$	14,764	
Non-cash investing and financing activities:	¥	1,701				11,704	
Issuance of common stock for subscription receivable	\$	-	\$	25,000	\$	25,000	
issuance of common stock for subscription receivable	Ψ		Ψ	20,000	\$	23,000	

The accompanying notes are an integral part of these 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 is not considered a predecessor company of the Desert Gateway for accounting or legal purposes. Following the 2008 reorganization, Desert Gateway re-domiciled to Delaware. Since inception and until Desert Gateway's merger with Retrophin, Inc., a private company ("Former Retrophin") in December 2012 (as described below), Desert Gateway had no existing operations, and its sole purpose was to locate and consummate a merger or acquisition with a private entity.

Former Retrophin, Inc. was originally organized as a Delaware limited liability company, named Retrophin, LLC, on March 11, 2011 ("Inception"). On September 20, 2012, Retrophin filed a Certificate of Conversion to change its legal form of organization from a limited liability company to a corporation in the State of Delaware. This conversion (as more fully described in Note 8) into a corporation, which preceded the Merger on December 12, 2012, resulted in no change of ownership and was therefore considered a recapitalization of the LLC's equity.

On September 13, 2012, Former Retrophin formed a new entity, Retrophin Pharmaceutical, Inc., a Delaware corporation and a wholly-owned subsidiary of Retrophin, Inc.

On December 12, 2012, Desert Gateway completed the transactions contemplated under the Agreement and Plan of Merger, dated as of December 12, 2012 (the "Merger Agreement"), by and among Desert Gateway, Desert Gateway Acquisition Corp., a Delaware corporation and wholly-owned subsidiary of Desert Gateway, and Former Retrophin, our predecessor, in which Former Retrophin became a wholly-owned subsidiary and the principal operating subsidiary of the Company. The transactions contemplated by the Merger Agreement are collectively referred to herein as the "2012 Merger". The Merger became effective on December 12, 2012, upon the filing of a certificate of merger with the Secretary of State of the State of Delaware. Accordingly, the Merger resulted in a change in control of Desert Gateway. Desert Gateway's net assets amounted to \$1,401 at the time of the merger, including \$3,721 of cash and \$2,320 of trade liabilities. The merger is being accounted for as a reverse merger and recapitalization of Former Retrophin into Desert Gateway, whereby Desert Gateway is the legal acquirer and Former Retrophin is the legal acquiree and the accounting acquirer in this transaction.

Upon the consummation of the Merger all of the issued and outstanding Class A Preferred shares of Former Retrophin were exchanged into the Company's common shares at the rate of 1 to 7 (each Class A Preferred stockholder received 7 shares of the Company's common stock) and all of the issued and outstanding share of common stock of Former Retrophin were exchanged for shares of the Company's common stock on exchange ratio of 1 to 5 (each Common stockholder of Former Retrophin received 5 shares of the Company's common stock).

The consolidated financial statements give retroactive effect to these changes as if the merger occurred at the inception of the Company.

On February 14, 2013, the Company changed its name to "Retrophin, Inc." through a short-form merger pursuant to Section 253 of the Delaware General Corporation Law, with its then wholly owned subsidiary, and our predecessor, Retrophin, with the Company continuing as the surviving corporation following the merger.

On April 1, 2013, the Company changed its fiscal year end from the last day of February to a fiscal year end of December 31 in order to confirm its reporting cycle to that of Former Retrophin.

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



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

The Company incurred a net loss of approximately \$33.6 million, including stock-based compensation charge of \$24,389,521 for the period from March 11, 2011 (inception) to December 31, 2012. At December 31, 2012, the Company had a cash balance of approximately \$11,000 and a working capital deficiency of approximately \$5,766,000. The Company's accumulated deficit amounted to approximately \$33,600,000 at December 31, 2012.

The Company has principally financed its operations from inception using proceeds from sales of its equity securities in a series of private placement transactions (see Note 7). The Company to date has no revenues, significantly limited capital resources and is subject to all of the risks and uncertainties that are typical of a development stage enterprise. Significant uncertainties include, among others, whether it will be able to raise the capital it needs to finance the start of its planned operations and whether such operations, if launched, will enable the Company to become a profitable enterprise.

On February 14, 2013, in connection with the closing of a private placement, we issued and sold an aggregate of 3,045,929 shares of common stock, for an aggregate purchase price of \$9,137,787 in cash, and warrants to purchase up to an aggregate of 1,522,969 shares of common stock. The Company concurrently entered into a registration payment arrangement requiring it to file a Form S-1 with the Securities and Exchange Commission within 30 days of the closing date of this transaction and cause it to be declared effective by no later than 90 days of the closing date of this transaction. The registration payment arrangement provided for the Company to pay liquidated damages in the amount of 2% of the proceeds received in this transaction per month for each month that the Company is not in compliance with this requirement, not to exceed 10% in the aggregate. The Company determined that it was probable that it would not be in a position to comply with these requirements and therefore allocated approximately \$360,000 of the proceeds received in this transaction.

In the second quarter of 2013, the Company, its Chief Executive Officer and a related party became party to a series of agreements to settle up to \$2,286,511 of liabilities, which Company management believes are the primary obligation of the related party. The Company and the related party have entered into indemnification agreements whereby the related party has agreed to defend and hold the Company harmless against all such obligations and amounts, whether paid or unpaid, arising from these agreements. The Company paid \$593,111 of the total settlement liabilities in the second quarter of 2013 and had \$1,691,400 in outstanding settlement liabilities, \$300,000 of which is past due, \$713,900 of which was due in July 2013, and \$677,500 of which was due in August 2013. The counter parties to these agreements reserve the right to demand payment at any time. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock in the Company to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the second quarter ended June 30, 2013.

There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

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

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

Management believes the Company's ability to continue its operations depends on its ability to raise capital. The Company entered into a licensing agreement providing it with the use of certain technology. The Company is currently developing pre-clinical and clinical studies of drug candidates. The licensing agreement described in Note 4 also enables the Company to sell the licensed technology as a research product or sublicense the technology to other third parties as alternative sources of revenue to its own product development efforts. The Company's future depends on the costs, timing, and outcome of regulatory reviews of its product candidates and the costs of commercialization activities, including product marketing, sales and distribution. During the first quarter of 2013, the Company has raised approximately \$9.95 million in certain private placement transactions. The Company expects to continue to finance its cash needs through additional private equity offerings and debt financings, corporate collaboration and licensing arrangements and grants from patient advocacy groups, foundations and government agencies. Although management believes that the Company has access to capital resources, there are no commitments for financing in place at this time, nor can management provide any assurance that such financing will be available on commercially acceptable terms, if at all.

## NOTE 3. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

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

# Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiary in conformity with U.S. GAAP. All intercompany accounts and transactions have been eliminated in consolidation.

## Restatement of Previously Issued Financial Statements for Additional Disclosures

The Company, while undergoing a review of its condensed consolidated financial statements for the three and six months periods ended June 30, 2013, commenced an evaluation of its accounting for a series of settlement agreements that the Company, along with certain of its related parties, entered into between April 2013 and June 2013. These agreements, which Company management originally deemed to be the primary obligation of a related party, are more fully described in Notes 2 and 12. On September 13, 2013, Company management, under the authority of the board of directors, determined that these agreements should have been disclosed in the footnotes to its consolidated financial statements for the year ended December 31, 2012. Accordingly, the Company has restated the consolidated financial statements to include these disclosures.

The Company also determined that its obligation to pay liquidated damages under a registration payment that it entered into in connection with a financing transaction completed on February 14, 2013, which required the Company to cause a registration statement to be declared effective by the Securities and Exchange Commission by May 15, 2013, should have also been disclosed. Accordingly, the Company is restating these consolidated financial statements to disclose that it allocated approximately \$360,000 of the proceeds received in this financing transaction to a registration payment obligation that was deemed probable at the date that the financing transaction was completed.

## Cash and Cash Equivalents

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

## Property and Equipment

Property and equipment are stated at cost. Depreciation is provided for using the straight-line method over the estimated useful lives of the assets. At December 31, 2012 and 2011, property and equipment consisted of computers with an estimated useful life of three years and leasehold improvements with an estimated life of four years.

## Employee Stock-Based Compensation

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

# Non-Employee Stock-Based Compensation

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

#### Income Taxes

The Company accounts for income taxes under ASC 740 Income Taxes ("ASC 740"). ASC 740 requires the recognition of deferred tax assets and liabilities for both the expected impact of differences between the financial statements and tax basis of assets and liabilities and for the expected future tax benefit to be derived from tax loss and tax credit carry forwards. ASC 740 additionally requires a valuation allowance to be established when it is more likely than not that all or a portion of deferred tax assets will not be realized.

ASC 740 also clarifies the accounting for uncertainty in income taxes recognized in an enterprise's financial statements and prescribes a recognition threshold and measurement process for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. For those benefits to be recognized, a tax position must be more-likely-than-not to be sustained upon examination by taxing authorities. ASC 740 also provides guidance on derecognition, classification, interest and penalties, accounting in interim periods, disclosure and transition. Based on the Company's evaluation, it has been concluded that there are no significant uncertain tax positions requiring recognition in the Company's unaudited financial statements. Since the Company was incorporated on March 11, 2011, all of its years of operations will be subject to examination. The Company believes that its income tax positions and deductions would be sustained on audit and does not anticipate any adjustments that would result in a material changes to its consolidated financial position.

The Company's policy for recording interest and penalties associated with audits is to record such expense as a component of income tax expense. There were no amounts accrued for penalties or interest as of or during the period from March 11, 2011 (inception) through December 31, 2012. Management is currently unaware of any issues under review that could result in significant payments, accruals or material deviations from its position.

Prior to conversion into a corporation on September 20, 2012, as a limited liability company, the Company was treated as a partnership for Federal and state income tax purposes. Accordingly, no provision has been made for Federal and state income taxes in the accompanying financial statements for any periods preceding September 20, 2012, since all items of income or loss are required to be reported on the income tax returns of the members, who are responsible for any taxes thereon. Profits and losses are allocated based upon capital in accordance with the permissible methods under Internal Revenue Code Section 706. Further, the Company incurred losses since inception through September 20, 2012, that would have resulted in the recognition of deferred tax assets that would have been fully reserved had the Company been subject to income taxes.

The Company is subject to the New York City Unincorporated Business Tax through September 19, 2012. Subsequent to Company's conversion to a corporation from a limited liability company on September 20, 2012, the Company will report and pay taxes based on its income or loss.

## Use of Estimates

In preparing financial statements in conformity with accounting principles generally accepted in the United States of America, management is required to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting period. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions. These estimates and assumptions include valuing equity securities in share-based payments, estimating the useful lives of depreciable and amortizable assets and estimating the fair value of long-lived assets to assets whether impairment charges may apply.



## Foreign Currency Translation and Remeasurement

Under ASC 830 Foreign Currency Matters, functional currency assets and liabilities are translated into the reporting currency, US Dollars, using period end rates of exchange and the related translation adjustments are recorded as a separate component of accumulated other comprehensive income. Functional statements of operations amounts expressed in functional currencies are translated using average exchange rates for the respective periods. Remeasurement adjustments and gains or losses resulting from foreign currency transactions are recorded as foreign exchange gains or losses in the consolidated statements of operations.

## Research and Development Costs:

Research and development costs are charged to operations as incurred and consist primarily of consulting services. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011 and for the period from March 11, 2011 (inception) through December 31, 2012, the Company incurred approximately \$524,000, \$353,000, and \$877,000, respectively, relating to research and development costs that are included in professional fees in the accompanying consolidated statements of operations.

#### Patents

The Company capitalized external cost, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company expense cost associated with maintaining and defending patents subsequent to their issuance in the period incurred. The Company amortizes patent cost once issued on a straight-line basis over the estimate useful lives of the patents. The Company assess the potential impairment to all capitalized patent cost when events or changes in circumstances indicate that the carrying amount of our patent may not be recoverable. For the years ended December 31, 2012 and 2011 patents costs \$18,093 and \$0, respectively, are included in the accompanying consolidated balance sheets.

## Basic and diluted Net Loss Per Share

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

An aggregate of 267,768 and 1,602,390 common stock equivalents (incentive shares) were excluded from the computation of diluted net loss per common share for the year ended December 31, 2012 and for the period from March 11, 2011 (inception) through December 31, 2011, because they were contingent shares subject to recall.

## Recently Issued Accounting Pronouncements

In February 2013, the FASB issued Accounting Standards Updated ("ASU") 2013-04 "Obligations Resulting from Joint and Several Liability Arrangements for Which the Amount at the Reporting Date is Fixed") ("ASU 2013-04"). The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount the entity has (a) contractually agreed to pay, and (b) any additional amounts that the entity expects to pay on behalf of its co-obligors. The Company early adopted this guidance in the second quarter of 2013 (Note 12).

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

## NOTE 4. ACCRUED EXPENSES

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

	De	December 31, 2012		March 11, 2011 through December 31, 2011	
Compensation related costs	\$	1,022,716	\$	-	
Consulting fees		679,800		169,721	
Legal fees		563,380		-	
Finders' fee liability		100,000		-	
Interest		90,650		-	
Other		11,250		-	
	\$	2,467,796	\$	169,721	

# NOTE 5. LICENSE AGREEMENT

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

In accordance with the agreement as amended most recently as of January 7, 2013, the Company is obligated to make two non-refundable payments totaling \$2,450,000, the first payment of \$1,150,000 due upon execution and the second payment of \$1,300,000 due January 31, 2013, which includes a \$150,000 fee payable to the sublicensee in exchange for extending due date of this payment from October 1, 2012 to January 31, 2013. If the Company makes the second payment after January 31, 2013 but before February 28, 2013 the payment due is \$1,400,000, if before March 31, 2013 the payment due is \$1,450,000. As of December 31, 2012, the Company has recognized \$2,300,000 for the cost of the License Agreement which is presented in the accompanying consolidated balance sheet as an intangible asset that is being amortized on a straight-line basis over the term of the License Agreement which expires on September 30, 2023. As of December 31, 2012, the Company made one payment of \$1,150,000. The Company has recorded a \$1,300,000 liability in the accompanying consolidated balance sheet at December 31, 2012 for the remaining payment of \$1,150,000 plus \$150,000 of extension fees. In addition, as more fully described below, the Company issued 620,000 common shares to Ligand valued at \$1,550,000 as a result of the merger transaction. For the year ended December 31, 2012, the Company recognized amortization expense of the license related to this agreement totaling \$121,383.

In addition, the Company is obligated to make series of milestone payments upon the achievement of each development milestone events set forth in the sublicense agreement which could amount to an aggregate of up to \$106.9 million. Milestone payments as they become due will be recognized as license expense, pro-rata over the period through September 2023.

Per the sublicense agreement, starting from the first commercial sale of any licensed product (as defined in the agreement), the Company is obligated to pay the Sublicensor royalty payments equal to 15% of annual worldwide net sales of licensed product up to \$300,000. For worldwide net sales of licensed product exceeding \$300,000, a royalty percentage of 17% is applied. Royalties are payable on a quarterly basis, and are payable on a product-by-product and country-by country basis on the net sales of licensed products. Royalties terms will be in effect until the later of (i) ten years after the first commercial sale of any licensed product in such country or (ii) the expiration of any patent rights licensed under the license agreement (iii) the expiration of all periods of market exclusivity. Currently, the last to expire issued patent covered by such arrangement expires in September 2023; however, the Company expects such date may be extended by patent-term extensions. The sublicense agreement contains other customary clauses and terms as are common in similar agreements in the industry.

In the event the Company's Exit Transaction defined in the agreement as (i) sale of all or substantially all of the Company's assets or business or (ii) a merger, reorganization or consolidation involving the Company in which the stockholders or members of the Company immediately prior to such transaction cease to own collectively a majority of the voting equity securities or membership interests of a successor entity or (iii) a registered public offering of Company's common stock under the Securities Act of 1933 or (iv) a reverse merger of Company into an existing public company), the Company is obligated to pay the Sublicensor \$1,500,000 no later than fifteen business days prior to the closing of the Exit Transaction. The Company has an option to issue capital stock in lieu of a cash payment to the Sublicensor. Should the Company choose to issue capital stocks, the number of shares of capital stock issue shall be equal to \$1,500,000 divided by the per share price of the capital stock to be agreed upon between the Company and the Sublicensor on the date such election is made.

## NOTE 6. NOTES PAYABLE

#### *Note Payable - related party*

On February 1, 2012, the Company entered into a secured promissory note with a related party in the amount of \$900,000, with an interest rate of 12% per annum, compounded monthly. The note plus accrued unpaid interest shall become due i) on or prior to December 31, 2012 or ii) upon consummation of a Sale of the Company to acquire (a) a majority of the outstanding equity securities, or (b) all or substantially all of the Company's assets on a consolidated basis.

In addition, the Company has the right to repay a portion of the outstanding obligation without penalty or premium. The repayment amount shall be applied in the following order: (i) any expenses to be reimbursed to the related party, (ii) all unpaid interest through the date of repayment and (iii) against the principal amount. On March 5, 2012, an aggregate payment of \$25,000 was made by the Company, of which \$9,764 was applied to accrued interest and the remaining balance of \$15,236 was applied to the principal balance. The remaining principal balance of this note amounts to \$884,764 as of December 31, 2012. The remaining principal balance of the note was repaid subsequent to year end.

On December 28, 2012, the secured promissory note was extended to June 30, 2013.

#### Note Payable - employee

On September 30 2012, the Company received an advance of \$30,000 from a related party in the form of a promissory note, with an interest rate of 15% per annum, compounded monthly. The note expired on the earlier of i) December 31, 2012 or ii) upon a significant change in the Company's ownership (as defined in the promissory note). On December 3, 2012, the Company repaid \$30,000 plus any unpaid interest.

The accrued interest payable related to the two notes payable at December 31, 2012 and 2011 was aggregated to \$90,650 and \$0, respectively.

Total interest expense recognized for the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011 and for the period from March 11, 2011 (inception) through December 31, 2012 were aggregated to \$105,917, \$0 and \$105,917, respectively.

## NOTE 7. RELATED PARTY TRANSACTIONS

In October and November 2011, the Company was advanced \$7,500, from a company related through common ownership. The advance is due on demand.

In November 2011, the Company was advanced \$30,000 from a company related through common ownership. The advances were repaid in February 2012.

On December 8, 2011, the Company received advances of funds aggregating \$8,500 from entities related through common ownership. The advances are due on demand. Balance remaining at December 31, 2012 was \$5,700.

In August 2012, the Company paid a security deposit on behalf of an affiliate of \$137,547 in connection with a building lease entered into by such affiliate. The Company assumed the lease from its affiliate in April 2013, whereby the security deposit was assigned to the Company.

During the year 2012, the Company paid an aggregate amount of \$563,380 in legal fees on behalf of the same affiliate. The affiliate is currently in the process of dissolving and the Company does not expect to collect the amount outstanding. As a result, the Company has written-off \$563,380 to bad debt expense in 2012. Such charge is included in selling general and administrative expense in the statement of operations. Subsequent to December 31, 2012, the Company became a party to certain settlement agreements, which management believes are the primary obligation of this affiliate (Note 12).

## NOTE 8. STOCKHOLDERS' DEFICIT

#### Post Merger Capitalization with Desert Gateway

## Common Stock

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

## Preferred Stock

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

#### Issuances

# Common Stock

On March 30, 2011, the Company issued to its founder 1,608,300 shares of Common Stock for a \$25,000 capital contribution.

On March 31, 2011, the Company issued to a member 50,000 shares of Common Stock for a \$100 capital contribution.

#### Private Placement Offering - March 2011

On March 31, 2011, the Company offered for sale, pursuant to a Private Placement Memorandum ("PPM"), up to 500,000 of the Company's Common Stock at \$4 per share, for an aggregate offering price of \$2,000,000. The Common Stock was entitled to one (1) vote per each unit outstanding. The termination date of this offer was originally May 3, 2011. On June 15, 2011, the Company extended the termination date of the PPM to August 31, 2011.

In April, May and June 2011, the Company sold shares of Common Stock in a private placement for \$4 per share, yielding aggregate proceeds of \$725,000. In addition, the Company incurred aggregate fees of \$66,061 in connection with the private placement. These common shares were subsequently exchanged for Series A Preferred shares (subsequently recapitalized into 253,750 shares of common stock).

#### Incentive Stock Awards

Since Inception, the Company entered into various incentive unit agreements for issuances of Incentive Common Shares with certain individuals for future services (see note 9).

#### Preferred Stock

On June 30, 2011, the Company amended its PPM to sell a new series of units of membership interest known as the "Series A Preferred Stock," instead of common stock. The Series A Preferred Shares have a liquidation priority over the Common Shares with a preference equal to two (2) times the amount originally invested in such shares (including any prior cash distributions of any operating profits) before any amounts are paid with respect to any Common Stock. In conjunction with the amended PPM, the Company amended the subscription agreements of the prior Common Stockholders and changed the Stock ownership to the newly issued Series A Preferred Stocks.

In July, October and December 2011, the Company sold shares of Series A Preferred Stock (subsequently recapitalized into 36,750 shares of common stock) related to the amended private placement for approximately \$2.86 per share, yielding aggregate proceeds of \$105,000 of which 10,500 shares sold and \$30,000 proceeds were from a related party through common ownership. In addition, the Company incurred aggregate fees of \$1,367 in connection with the private placement.

On January 25, 2012, the Company, in connection with a January 2012 private placement offered for sale up to 875,000 shares of the Company's Series A Preferred Shares at approximately \$5.71 per share with similar terms and conditions as the amended PPM.

From January 1, 2012 through May 14, 2012, the Company sold shares of Series A Preferred Stock (subsequently recapitalized into 326,963 shares of common stock) related to the January 2012 private placement at approximately \$5.71 per Share, yielding aggregate proceeds of \$1,868,354 of which 128,163 shares sold and \$732,353 proceeds were from a related party through common ownership. In addition, the Company incurred aggregate fees of \$61,677 in connection with the private placement.

On May 18, 2012, the Company, in connection with the May 2012 private placement, offered for sale up to 875,000 shares of the Company's Series A Preferred Stock at approximately \$11.43 per share with similar terms and conditions as the amended PPM.



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 Chairman of the Board of Directors. The shares in both of these transactions, which have an aggregate fair value of \$4,400,000, are fully vested and non-forfeitable.

In November 2012 and December 2012, the Company's founding shareholder personally transferred 275,000 shares of his common stock to several employees. The shares, which had an aggregate fair value of \$1,375,000, are fully vested and non-forfeitable.

#### Receivables from Shareholders

In November of 2011, the Company advanced \$10,000 to a related party, with an interest rate of 0.001% and a five year term. The advance is classified as a note receivable from related party on the balance sheet at December 31, 2011 and is due on November 3, 2016, the note is classified as a reduction of stockholders' equity in the accompanying consolidated balance sheet.

On February 3, 2012, the Company entered into a note receivable with a related party in the amount of \$200,000. The note receivable is unsecured, bearing an interest rate of 12% per annum and due to mature on February 3, 2013. The note is classified as a reduction of stockholders' equity in the accompanying consolidated balance sheet.

Advances to shareholders consist of payments made by the Company for entities commonly controlled by the shareholders for operating expenses aggregating \$172,900.

## NOTE 9. INCENTIVE SHARES

On March 31, 2011, the Company granted 1,849,300 incentive shares to several executive and non-executive employees, and certain consultants, with an aggregate fair value of \$7,397,200 or \$4 per share. The incentive shares vested on the final day of each calendar quarter over three years, commencing on June 30, 2011. On September 11, 2012, the Company accelerated the vesting of 938,175 shares issued to its founder and Chief Executive Officer, which resulted in a charge of \$3,216,600 included in compensation and related costs in the accompanying statement of operations.

In August and November 2011, the Company granted an aggregate of 290,000 incentive shares to two consultants, with an aggregate fair value of \$1,160,000 or \$4 per share, for consulting services. The incentive shares vested on the final day of each calendar quarter over three years, commencing on June 30, 2011 and December 31, 2011.

In January 2012, the Company granted 826,600 incentive shares to the Chief Executive Officer, an employee and a consultant, with an aggregate fair value of \$9,919,200 or \$12 per share. The incentive shares vested on the final day of each calendar quarter over three years, commencing on March 31, 2012. On September 11, 2012, the Company immediately vested the Chief Executive Officer's unvested incentive shares totaling 28,185 for continuing services. On December 11, 2012, the Chief Executive Officer's remaining unvested incentive shares totaling 573,015 were vested immediately due to the merger, which resulted in an aggregate charge of \$7,214,400 included in compensation and related costs in the accompanying statement of operations.

On March 7, 2012, the Company granted 83,333 incentive shares to a third party consultant, with an aggregate fair value of \$2,000,000 or approximately \$24 per share, for consulting services. The incentive shares vested (i) 50% immediately and (ii) on the final day of each calendar quarter over two years, commencing on March 31, 2012.

On July 7, 2012, the Company granted 43,750 incentive shares to an employee, with an aggregate fair value of \$375,000 or approximately \$8.6 per share. The incentive shares vested on the final day of each calendar quarter over three years, commencing on September 30, 2012.

For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and for the period from March 11, 2011 (inception) through December 31, 2012, the Company recognized \$22,410,222, \$1,979,299, and \$24,389,521 as compensation expense related to incentive shares granted in the consolidated statements of operations, respectively. Share compensation for non-employee awards subject to vesting is being accrued at current fair value as of December 31, 2012, there was approximately \$844,973 of unrecognized compensation cost related to incentive shares issued. This amount is expected to be recognized over a weighted average of 1.69 years.

	Employee - number of shares	Non Employee - number of shares	Total number of shares	Weighted verage Fair Value
Unvested March 11, 2011 ("inception")			-	\$ -
Granted	1,758,300	381,000	2,139,300	4.00
Vested	(431,240)	(59,835)	(491,075)	4.00
Forfeited	(45,835)	-	(45,835)	-
Unvested December 31, 2011	1,281,225	321,165	1,602,390	\$ 4.00
Granted	866,180	87,503	953,683	12.89
Vested	(2,048,280)	(193,672)	(2,241,952)	7.34
Forfeited	(46,353)	-	(46,353)	-
Unvested December 31, 2012	52,772	214,996	267,768	\$ 3.20

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

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

## NOTE 10. COMMITMENTS AND CONTINGENCIES

## Sublease

During March 2011, the Company began subleasing offices on a month -to-month basis for \$7,000 per month. On June 31, 2011, the Company entered into a sublease agreement with a company affiliated by common ownership, where the Company will pay \$7,000 a month or 75% of the space used, pro-rated, according to the aggregate cost of the shared offices with the affiliated entities of the related party leasing company, whichever is greater. According to the agreement, the Company is responsible for incidental costs and for rent or lease of office furniture and equipment. The sublease is on a six month rolling basis and termination of the agreement can be made by a mutual agreement of both parties or by the related party leasing company. The month-to-month lease was terminated in September 2012.

In October 2012, the Company entered into a sublease with a company ("Sublessor") affiliated by common ownership that expires on November 29, 2016. The sublease agreement requires the Company to pay 50% of the rent and related escalations and for the Company to pay for 50% of the utilities incurred by the Sublessor.

Rent expense for the year ended December 31, 2012, for the periods from March 11, 2011 (inception) through December 31, 2011 and from March 11, 2011 (inception) through December 31, 2012 were \$95,469, \$63,000 and \$158,469, respectively, which is recorded as rent expense in the consolidated statement of operations.

As of December 31, 2012 minimum future rental commitments under non-cancelable operating leases follow:

Year Ending December 31,	
2013	\$ 138,200
2014	140,161
2015	140,161
2016	 128,481
Total	\$ 547,003

On April 11, 2013, the lease was assigned to the Company by the Sublessor inclusive of the security deposit held.

## **Consulting Agreements**

On August 15, 2011, the Company entered into an agreement with a consultant to serve as a senior advisor of strategy.

The agreement's initial term is for one year and automatically renews on an annual basis. Pursuant to this agreement the compensation to the consultant is comprised of (a) a fee of \$37,500 per calendar quarter, payable commencing September 30, 2011, (b) 25,000 shares of the Company Common Stock with an estimated fair value of \$100,000, which vest over twelve (12) quarters so long as the agreement remains in effect, and (c) 25,000 additional common stock, (i) upon the Company's completion of its initial financing at a pre-financing value of \$20 million, and (ii) which vest in accordance with certain schedules of milestones as described in the consulting agreement. At December 31, 2012, the financing and milestones have not yet occurred or been achieved. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and December 31, 2012, the Company recognized professional fees related to this agreement in the amounts of \$150,000, \$75,000, and \$225,000, respectively, of which amounts comprised of fee payable of \$155,000 at December 31, 2012 and 2011, respectively.

On November 1, 2011, the Company granted to the consultant an additional 120,000 shares of common stock with an estimate fair value of \$480,000, which vest in over twelve (12) calendar quarters commencing December 31, 2011. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and for the period from March 11, 2011 (inception) through December 31, 2012, the Company recognized professional fees related to this share based compensation of \$210,000, \$40,000, and \$250,000.

On August 25, 2011, the Company entered into an agreement with a consultant to serve as chief scientific officer of the Company.

The agreement's initial term is for one year and automatically renews on an annual basis. Pursuant to this agreement the compensation to the consultant is comprised of (a) a fee of \$50,000 per calendar quarter, (b) 75,000 incentive shares with an estimated fair value of \$300,000, which vest over twelve (12) quarters so long as the agreement remains in effect, and (c) receive 70,000 additional incentive shares, (i) upon the Company's completion of its initial financing at a prefinancing value of \$20 million, and (ii) which vest in accordance with certain schedules of milestones as described in the consulting agreement. At December 31, 2012, the financing and milestones have not yet occurred or been achieved. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2011, and for the period from March 11, 2011 (inception) through December 31, 2012, the Company recognized professional expense related to this agreement in amounts of \$200,000, \$100,000, and 300,000, respectively, of which amounts comprise of fee payable of \$200,000 and \$100,000 at December 31, 2012 and December 31, 2011, respectively.

On November 1, 2011, the Company granted to the consultant an additional 70,000 incentive shares with an estimated fair value of \$280,000, which vest in over twelve (12) calendar quarters commencing December 31, 2011. For the year ended December 31, 2012, for the period from March 11, 2011 (inception) through December 31, 2012, the Company recognized professional expense related to this share based compensation of \$122,500, \$23,333, and \$145,833.

## Sponsored Research Agreement

On July 1, 2012, the Company entered into a Sponsored Research Agreement with an organization that expires on July 1, 2013, unless extended by written agreement between the parties. The Company has agreed to pay a sponsor fee of \$203,169 to the organization to perform the research program stated in the Sponsored Research Agreement. The sponsor fee payments are as follows: \$101,855 within 30 days of the execution of the agreement and the remaining \$101,314 will be due on January 1, 2013. As of December 31, 2012, the Company included the first payment of \$101,855 in accounts payable and accrued expenses, as no payment have been made by the Company.

Sponsor fee totaling \$203,169 will be recognized as professional expense, pro-rata over the one year term of the Sponsored Research Agreement. Total professional expense recorded related to the Sponsored Research Agreement totaled \$101,855 for the year ended December 31, 2012.

#### Employment agreement

Effective March 1, 2011, the Company entered into a three-year employment agreement with Martin Shkreli, who serves as the Company's Chief Executive Officer. The Agreement provides for (a) a base salary of \$250,000 per year, (b) annual cash bonus award at the discretion of the Board equal to one month salary, (c) three weeks' vacation paid per calendar year, (d) accelerated vesting of options in the event of (i) a merger or consolidation, (ii) a sale of all or substantially all of the assets or (iii) any other change in control of the Company, and (e) all group insurance plans and other benefit plans and programs made available to the Company's management employees.

## NOTE 11. INCOME TAXES

From the Company's inception in March 11, 2011 to September 20, 2012, the Company was not subject to federal and state income taxes since it was operating as a Limited Liability Company (LLC). On September 20, 2012, the company converted from an LLC to a C corporation and, as a result, became subject to corporate federal and state income taxes. This conversion is considered a recapitalization of the equity structure of the company and was treated as a nontaxable transaction. As a result of the conversion to a taxable entity, the Company recorded a deferred tax liability on the balance sheet and in income tax expense as of the date of the change in tax status in the amount of \$1,079,000 related to the technology license. The company files its taxes on a cash basis method.

For the period ended December 31, 2012, the Company incurred net operating losses and, accordingly, no provision for income taxes has been recorded. In addition, no benefit for income taxes has been recorded due to the uncertainty of the realization of any tax assets including NOL carryovers. At December 31, 2012, the Company had approximately \$5.9 million dollars of federal and state and local net operating losses. The net operating loss carryforwards, if not utilized, will begin to expire in 2032 for federal purposes.

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

	 2012	
Current		
Federal	\$ -	
State	-	
	-	
Deferred		
Federal	(1,173)	
State	 (733)	
	(1,906)	
Total	\$ (1,906)	
Change in valuation allowance	1,906	
Income tax (benefit)	-	
Total	\$ -	

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

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

	2012
Statutory rate - federal	(35.00%)
State taxes, net of federal benefit	(1.81%)
Partnership Losses preceding the conversion to a C Corp	19.39%
Stock Based Compensation related to profits interest	9.52%
Meals & Entertainment	0.01%
Deferred tax adjustment upon conversion to taxable status	1.61%
Change in valuation allowance	6.28%
Income tax provision (benefit)	0.00%

The tax effects of "temporary differences" give rise to deferred tax assets and liabilities as of December 31, 2012. (in thousands):

	 2012	Asset	Liability
Net operating loss and capital loss carry forward	\$ 2,748	\$ 2,748	
Technology license	\$ (466)		(466)
Organizational costs	\$ 9		9
Accrual to Cash	\$ (385)		(385)
Valuation allowance	\$ (1,906)		
Total	\$ -	\$ 2,748	\$ (842)

# NOTE 12. SUBSEQUENT EVENTS

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

On February 14, 2013, Desert Gateway Inc. closed a private placement of 3,045,929 shares of Desert Gateway Inc. common stock, at a purchase price of \$3.00 per share, or \$9,137,787 in the aggregate, and Warrants to purchase up to an aggregate of 1,522,969 shares of common stock with an exercise price of \$3.60 per such share underlying any Warrant. The issuance of the shares of common stock in such private placement was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

The Company concurrently entered into a registration payment arrangement requiring it to file a Form S-1 with the Securities and Exchange Commission within 30 days of the closing date of this transaction and cause it to be declared effective by no later than 90 days of the closing date of this transaction. The registration payment arrangement provided for the Company to pay liquidated damages in the amount of 2% of the proceeds received in this transaction per month for each month that the Company is not in compliance with this requirement, not to exceed 10% in the aggregate. The Company determined that it was probable that it would not be in a position to comply with these requirements and therefore allocated approximately \$360,000 of the proceeds received in this transaction to a registration payment obligation.

Effective May 13, 2013, the Company entered into an employment agreement with Horacio Plotkin, M.D. (the "Plotkin Employment Agreement") pursuant to which Dr. Plotkin was appointed as Chief Medical Officer of the Company.

In accordance with the terms of the Plotkin Employment Agreement, Dr. Plotkin's initial base salary is \$350,000 and he is eligible to receive a discretionary annual bonus of up to 50% of his then applicable base salary. Additionally, Dr. Plotkin received \$20,000 in connection with signing the Plotkin Employment Agreement. Dr. Plotkin will also be awarded options to purchase 120,000 shares of restricted common stock of the Company at an exercise price of \$8.70 per share, a pro rata portion of which will vest quarterly during the 3 years following the effective date.

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

In accordance with the terms of the Panoff Employment Agreement, Mr. Panoff's initial base salary is \$230,000 and he is eligible to receive a discretionary annual bonus of up to 50% of his then applicable base salary. Mr. Panoff will also be granted 120,000 units of restricted common stock of the Company, a pro rata portion of which will vest quarterly during the 3 years following the effective date.

In the second quarter of 2013, the Company, its Chief Executive Officer and a related party became party to a series of agreements to settle up to \$2,286,511 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 \$2,286,511 charge to operations during the quarter ended June 30, 2013 that was offset by a corresponding liability of \$1,691,400 for the difference between (a) the aggregate amount of all such settlements, and (b) \$593,111 of cash and non-cash consideration that the Company paid to immediately settle a portion of the agreement on behalf of the related party. Of the total outstanding \$1,691,400 settlement liability, \$300,000 is past due, \$713,900 was due in July 2013, and \$677,500 was due in August 2013. The counter parties to these agreements reserve the right to demand payment at any time. The Chief Executive Officer also agreed to deliver or cause to be delivered 47,128 shares of common stock in the Company to one of the counter parties as a separate component of one of these agreements. Accordingly, the Company does not believe it is required to record a liability for the shared-based component of this specific agreement during the second quarter ended June 30, 2013. There is uncertainty as to whether the related party will have sufficient liquidity to repay the Company or fund the indemnification agreements should it become necessary.

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

The Company applied the accounting guidance provided in ASU 2013-04. The guidance in this update is effective for fiscal years beginning after December 15, 2013 with early adoption permitted. The guidance in this update requires companies to measure obligations resulting from joint and several liability arrangements as the sum of the amount that the entity 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.

In accordance with ASC 855-10, Company management reviewed all material events through the date of this report and there are no material subsequent events to report, other than those listed in the Note.

You should rely only on the information contained in this document. We have not authorized anyone to provide you with information that is different. This document may only be used where it is legal to sell these securities. The information in this document may only be accurate on the date of this document.

Additional risks and uncertainties not presently known or that are currently deemed immaterial may also impair our business operations. The risks and uncertainties described in this document and other risks and uncertainties which we may face in the future will have a greater impact on those who purchase our common stock. These purchasers will purchase our common stock at the market price or at a privately negotiated price and will run the risk of losing their entire investment.

# **RETROPHIN, INC.**

# 13,612,218 Shares Common Stock

# PROSPECTUS

\_\_\_\_\_, 2013

## PART II

# INFORMATION NOT REQUIRED IN PROSPECTUS

#### Item 13. Other Expenses of Issuance and Distribution

The following table sets forth the costs and expenses payable by the Company in connection with the offer and sale of the securities being registered. All amounts are estimates except the SEC registration fee.

Nature of Expense	
SEC registration fee	\$ 9,148.28
Legal fees and expenses	\$ 50,000.00
Accounting fees and expenses	\$ 50,000.00
Total	\$ 109,148.28

## Item 14. Indemnification of Directors and Officers

Our company is incorporated under the laws of the State of Delaware. Section 145 of the Delaware General Corporation Law provides that a corporation may indemnify directors and officers as well as other employees and individuals against expenses including attorneys' fees, judgments, fines and amounts paid in settlement in connection with various actions, suits or proceedings, whether civil, criminal, administrative or investigative other than an action by or in the right of the corporation, a derivative action, if they acted in good faith and in a manner they reasonably believed to be in or not opposed to the best interests of the corporation, and, with respect to any criminal action or proceeding, if they had no reasonable cause to believe their conduct was unlawful. A similar standard is applicable in the case of derivative actions, except that indemnification only extends to expenses including attorneys' fees incurred in connection with the defense or settlement of such actions, and the statute requires court approval before there can be any indemnification where the person seeking indemnification has been found liable to the corporation. The statute provides that it is not exclusive of other indemnification that may be granted by a corporation's certificate of incorporation, bylaws, agreement, a vote of stockholders or disinterested directors or otherwise.

The Company's bylaws provide that the Company will indemnify and hold harmless, to the fullest extent permitted by Section 145 of the Delaware General Corporation Law, as amended from time to time, each person that such section grants us the power to indemnify.

The Delaware General Corporation Law permits a corporation to provide in its certificate of incorporation that a director of the corporation shall not be personally liable to the corporation or its stockholders for monetary damages for breach of fiduciary duty as a director, except for liability for:

- any breach of the director's duty of loyalty to the corporation or its stockholders;
- · acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;
- · payments of unlawful dividends or unlawful stock repurchases or redemptions; or
- any transaction from which the director derived an improper personal benefit.

The Company's certificate of incorporation provides that, to the extent permitted by applicable law, none of our directors will be personally liable to the Company or its stockholders for monetary damages for breach of fiduciary duty as a director. Any repeal or modification of this provision will be prospective only and will not adversely affect any limitation, right or protection of a director of our company existing at the time of such repeal or modification.

#### Item 15. Recent Sales of Unregistered Securities

The following summarizes all sales of unregistered securities by the Company and former Retrophin, our predecessor, within the past three years:

#### Sales by Retrophin, our predecessor

On March 2011, former Retrophin, in connection with its original formation as a limited liability company, issued an aggregate of 1,608,300 common stock to Martin Shkreli, our Chief Executive Officer, for aggregate consideration of \$25,000.

On March 31, 2011, former Retrophin offered for sale, pursuant to a Private Placement Memorandum ("PPM"), up to 500,000 of Retrophin'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 PPM was restated to extend the termination date to August 31, 2011.

In April, May and June 2011, former Retrophin sold 181,250 shares of common stock in a private placement for \$4 per share, yielding aggregate proceeds of \$725,000. In addition, former Retrophin incurred aggregate fees of \$66,061 in connection with the private placement. These common shares were subsequently exchanged for Series A Preferred.

In July, October and December 2011, former Retrophin sold 36,750 shares of Series A Preferred Stock related to the amended private placement for approximately \$2.86 per share, yielding aggregate proceeds of \$105,000 of which 10,500 shares sold and \$30,000 proceeds were from a related party through common ownership. In addition, former Retrophin incurred aggregate fees of \$1,367 in connection with the private placement.

On January 25, 2012, former Retrophin, in connection with a January 2012 private placement, offered for sale up to 875,000 shares of former Retrophin'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, former Retrophin sold 326,963 shares of Series A Preferred Stock related to the January 2012 private placement at approximately \$5.71 per share, yielding aggregate proceeds of \$1,868,353 of which 128,163 shares sold and \$732,353 proceeds were from a related party through common ownership. In addition, former Retrophin incurred aggregate fees of \$61,677 in connection with the private placement.

From May 31, 2012 through September 25, 2012, former Retrophin sold 271,824 shares of its Series A Preferred Stock in a private placement at \$5 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.

On March 31, 2011, former Retrophin 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, former Retrophin accelerated the vesting of 93,175 shares issued to Mr. Shkreli, its founder and Chief Executive Officer, which resulted in a charge of \$3,216,600 included in compensation and related costs in the accompanying unaudited statement of operations.

In August and November 2011, former Retrophin 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, former Retrophin granted 826,600 incentive shares to Mr. Shkreli, its 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, former Retrophin immediately vested Mr. Shkreli's unvested incentive shares totaling 28,185 for continuing services.



On March 7, 2012, former Retrophin granted 83,335 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, former Retrophin granted 43,750 incentive shares to an employee, with an aggregate fair value of 375,000 or approximately \$8.60 per share. The incentive shares vested on the final day of each calendar quarter over three years, commencing on September 31, 2012.

The sales of the securities identified above were made pursuant to privately negotiated transactions that did not involve a public offering of securities and, accordingly, we believe that these transactions were exempt from the registration requirements of the Securities Act pursuant to Section 4(2) thereof and the rules promulgated thereunder. Each of the above-referenced investors in former Retrophin's stock represented to Retrophin in connection with their investment that they were "accredited investors" (as defined by Rule 501 under the Securities Act) and were acquiring the shares for investment and not distribution, that they could bear the risks of the investment and could hold the securities for an indefinite period of time. The investors received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration or an available exemption from such registration. All of the foregoing securities are deemed restricted securities for purposes of the Securities Act.

#### 2012 Merger

On December 12, 2012, we closed the 2012 Merger, pursuant to which Retrophin became a wholly-owned subsidiary of the Company. At the effective time of the 2012 Merger, all of the issued and outstanding shares of common stock, par value \$0.001 per share, and Series A Preferred Stock, par value \$0.001 per share, of Retrophin, our predecessor, that was outstanding immediately prior to such effective time were cancelled. In consideration, the Company issued to the former holders of capital stock of Retrophin, our predecessor, an aggregate of 5,434,120 shares of the Company's common stock. Such securities were not registered under the Securities Act. These securities qualified for exemption under Section 4(2) of the Securities Act because the issuance of securities by us did not involve a public offering.

#### January 2013 Private Placement

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

#### February 2013 Private Placement

On February 14, 2013, the Company closed a private placement of 3,045,929 shares of the Company's 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. 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.

#### August 2013 Private Placement

On August 15, 2013, the Company closed a private placement of 5,536,957 shares of the Company's common stock, at a purchase price of \$4.50 per share, or approximately \$25,000,000 in the aggregate, and Warrants to purchase up to an aggregate of 2,768,479 shares of common stock with an exercise price of \$6.00 per such share underlying any Warrant. The issuance of the shares of common stock in such private placement was not registered under the Securities Act as such issuance was exempt from registration under Section 4(2) of the Securities Act and Regulation D promulgated thereunder.

### Warrants

On February 14, 2013, in connection with the closing of the private placement transaction described above, the Company issued and sold Warrants to purchase up to an aggregate of 1,597,969 shares of common stock. Each Warrant entitles the purchaser (or subsequent holder) thereof to purchase up to 50% of the number of shares of common stock purchased by such purchaser in such private placement. The Warrants are exercisable in whole or in part, at an initial exercise price per share of \$3.60, which is subject to customary full-ratchet anti-dilution protections. The Warrants may be exercised at any time upon the election of the holder, beginning on the date of issuance and ending of the fifth anniversary of the date of issuance. The issuance of the Warrants 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, in connection with the closing of the private placement transaction described above, the Company issued and sold Warrants to purchase up to an aggregate of 2,768,479 shares of common stock. Each Warrant entitles the purchaser (or subsequent holder) thereof to purchase up to 50% of the number of shares of common stock purchased by such purchaser in such private placement. The Warrants are exercisable in whole or in part, at an initial exercise price per share of \$6.00, which is subject to customary full-ratchet anti-dilution protections. The Warrants may be exercised at any time upon the election of the holder, beginning on the date of issuance and ending of the fifth anniversary of the date of issuance. The issuance of the Warrants 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.

## Item 16. Exhibits and Financial Statement Schedules

Exhibit No.	Description
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. (1)
3.1	Certificate of Incorporation of the Company (2)
3.2	Bylaws of the Company (3)
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 (4)
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 (5)
5.1	Opinion of Katten Muchin Rosenman LLP *
10.1	Securities Purchase Agreement, dated February 12, 2013, by and among the Company and the February 2013 Purchasers (6)
10.2	Registration Rights Agreement, dated February 12, 2013, by and among the Company and the February 2013 Purchasers (7)
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 (8)
10.4	Employment Agreement, dated April 24, 2013, by and between Retrophin, Inc. and Horacio Plotkin, M.D. (9)
10.5	Employment Agreement, dated May 7, 2013, by and between Retrophin, Inc. and Marc Panoff (10)
10.6	Amendment to Employment Agreement, dated as of June 30, 2013 (11)
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10.9	First Amendment to Securities Purchase Agreement, dated August 14, 2013, by and among the Company and the purchasers signatory thereto (14)



10.10 First Amendment to Registration Rights Agreement, dated August 14, 2013, by and among the Company and the purchasers signatory thereto (15)

10.11 Sponsored Research Agreement between St. Jude Children's Research Hospital and the Company, dated September 1, 2013.\* (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 on the date of filing of this Registration Statement on Form S-1.)

- 21.1 List of the Company's Subsidiaries \*
- 23.1 Consent of Katten Muchin Rosenman LLP (included in Exhibit 5.1)\*
- 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.
  - **\*\*** To be filed by amendment.
- (1) 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.
- (2) Incorporated by reference to Exhibit 3.1 to Amendment No. 2 to the Company's General Form for Registration of Securities on Form 10-12G filed with the SEC on October 28, 2010.
- (3) Incorporated by reference to Exhibit 3.2 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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- (15) Incorporated by reference to Exhibit 10.4 to the Company's Current Report on Form 8-K filed with the SEC on August 20, 2013.

## Item 17. Undertakings

- (a) The undersigned registrant hereby undertakes:
- (1) To file, during any period in which offers or sales are being made, a post-effective amendment to this registration statement:
  - (i) To include any propectus required by section 10(a)(3) of the Securities Act of 1933;

(ii) To reflect in the prospectus any facts or events arising after the effective date of the registration statement (or the most recent posteffective amendment thereof) which, individually or in the aggregate, represent a fundamental change in the information set forth in the registration statement. Notwithstanding the foregoing, any increase or decrease in volume of securities offered (if the total dollar value of securities offered would not exceed that which was registered) and any deviation from the low or high end of the estimated maximum offering range may be reflected in the form of prospectus filed with the SEC pursuant to Rule 424(b) if, in the aggregate, the changes in volume and price represent no more than 20 percent change in the maximum aggregate offering price set forth in the "Calculation of Registration Fee" table in the effective registration statement;

(iii) To include any material information with respect to the plan of distribution not previously disclosed in the registration statement or any material change to such information in the registration statement.

(2) That, for the purpose of determining any liability under the Securities Act of 1933, each such post-effective amendment shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

(3) To remove from registration by means of a post-effective amendment any of the securities being registered which remain unsold at the termination of the offering.

(4) That, for the purpose of determining liability under the Securities Act of 1933 to any purchaser, if the registrant is subject to Rule 430C, each prospectus filed pursuant to Rule 424(b) as part of a registration statement relating to an offering, other than registration statements relying on Rule 430B or other than prospectuses filed in reliance on Rule 430A, shall be deemed to be part of and included in the registration statement as of the date it is first used after effectiveness. Provided, however, that no statement made in a registration statement or prospectus that is part of the registration statement or made in a document incorporated or deemed incorporated by reference into the registration statement or prospectus that is part of the registration statement will, as to a purchaser with a time of contract of sale prior to such first use, supersede or modify any statement that was made in the registration statement or prospectus that was part of the registration statement or made in any such document immediately prior to such date of first use.

(b) The undersigned registrant hereby undertakes that:

(1) For the purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

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# SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of New York, State of New York, on October 24, 2013.

# **RETROPHIN, INC.**

By: /s/ Martin Skhreli

Name:	Martin Shkreli
Title:	Chief Executive Officer

## POWER OF ATTORNEY

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities and on the date indicated.

Signature	Title	Date
/s/ Martin Shkreli Martin Shkreli	Chief Executive Officer and Director (Principal Executive Officer)	October 24, 2013
* Marc Panoff	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	October 24, 2013
* Stephen Aselage	Director	October 24, 2013
* Steven Richardson	Director	October 24, 2013
Cornelius E. Golding	Director	October 24, 2013
*By:/s/ Martin Shkreli Name: Martin Shkreli <b>Title: Attorney-in-fact</b>		

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575 Madison Avenue New York, NY 10022-2585 212.940.8800 tel 212.940.8776 fax www.kattenlaw.com

### FORM OF OPINION

October \_\_, 2013

Retrophin, Inc. 777 Third Avenue, 22nd Floor New York, New York 10017

Re: <u>Retrophin, Inc. Registration Statement on Form S-1</u>

Ladies and Gentlemen:

We have acted as counsel to Retrophin, Inc., a Delaware corporation (the "Company"), in connection with the preparation and filing of a Registration Statement on Form S-1 (the "Registration Statement") with the Securities and Exchange Commission (the "SEC") under the Securities Act of 1933, as amended (the "Securities Act"), relating to the offer and sale by the selling stockholders identified in the Registration Statement of up to 13,612,218 shares (the "Securities") of common stock, par value \$0.0001 per share, of the Company (the "Common Stock"). The Securities consist of (i) 9,147,014 shares issued to certain selling stockholders (the "Shares") and (ii) 1,597,969 shares issuable upon the exercise by certain selling stockholders of the Company's common stock purchase warrants issued on February 14, 2013 and 2,867,235 shares issuable upon the exercise by certain selling stockholders of the Company's common stock purchase warrants issued on August 15, 2013 (together, the "Warrants"). The Shares and the Warrants were issued to the selling stockholders pursuant to (i) that certain Securities Purchase Agreement (the "February 2013 Purchase Agreement"), dated as of February 12, 2013, between the Company and the purchasers party thereto, (ii) that certain Securities Purchase Agreement (the "August 2013 Purchase Agreement"), dated as of August 14, 2013, to the Registration Rights Agreement (the Gourdance with the requirements of Item 601(b)(5) of Regulation S-K under the Securities Act.

AUSTIN CENTURY CITY CHARLOTTE CHICAGO HOUSTON IRVING LOS ANGELES NEW YORK ORANGE COUNTY SAN FRANCISCO BAY AREA SHANGHAI WASHINGTON, DC LONDON: KATTEN MUCHIN ROSENMAN UK LLP A limited liability partnership including professional corporations

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In connection with this opinion, we have relied as to matters of fact, without investigation, upon certificates of public officials and others and upon affidavits, certificates and written statements of directors, officers and employees of, and the accountants for, the Company. We have also examined originals or copies, certified or otherwise identified to our satisfaction, of such instruments, documents and records as we have deemed relevant and necessary to examine for the purpose of this opinion, including (a) the Registration Statement, (b) the prospectus constituting a part of the Registration Statement, (c) the February 2013 Purchase Agreement, (d) the August 2013 Purchase Agreement, (e) the Amended Registration Rights Agreement, (f) the Warrants, (g) a specimen certificate representing the Common Stock, (h) the Certificate of Incorporation of the Company, as currently in effect, (i) the Bylaws of the Company, as currently in effect, and (j) minutes and corporate records of proceedings of the Board of Directors of the Company relating to the February 2013 Purchase Agreement, the August 2013 Purchase Agreement, the Amended Registration Rights Agreement, the authorization and issuance of the Shares and the Warrants pursuant thereto, and related matters.

In connection with this opinion, we have assumed the legal capacity of all natural persons, the accuracy and completeness of all documents and records that we have reviewed, the genuineness of all signatures, the due authority of the parties signing such documents, the authenticity of the documents submitted to us as originals and the conformity to authentic original documents of all documents submitted to us as certified, conformed or reproduced copies. In making our examination of documents executed or to be executed by parties other than the Company, we have assumed that such parties had the power, corporate or other, to enter into and perform all obligations thereunder and have also assumed the due authorization by all requisite action, corporate or other, and execution and delivery by such parties of such documents and the validity and binding effect thereof.

Based upon and subject to the foregoing, it is our opinion that the Securities have been duly authorized and validly issued and are fully paid and nonassessable.

Our opinion expressed above is limited to the General Corporation Law of the State of Delaware and we do not express any opinion concerning any other laws. This opinion is given as of the date hereof and we assume no obligation to advise you of changes that may thereafter be brought to our attention.

We hereby consent to the reference to our firm under the heading "Legal Matters" in the prospectus forming a part of the Registration Statement and to the filing of this opinion with the SEC as an exhibit to the Registration Statement. In giving this consent, we do not thereby admit that we are included in the category of persons whose consent is required under Section 7 of the Securities Act or the related rules and regulations thereunder.

Very truly yours,

KATTEN MUCHIN ROSENMAN LLP

### SPONSORED RESEARCH AGREEMENT

This Sponsored Research Agreement ("Agreement"), effective as of this 1<sup>st</sup> day of October 2013 ("Effective Date"), is entered into by and between St. Jude Children's Research Hospital, 262 Danny Thomas Place, Memphis, Tennessee 38105 ("St. Jude") and Retrophin, Inc., a Delaware Corporation located at 777 Third Avenue, 22<sup>nd</sup> Floor, New York, New York 10017 ("Sponsor" and collectively with St. Jude, the "Parties"),

## 1. <u>Background</u>

1.1 St. Jude is the owner, by assignment from ######## ("Principal Investigator"), of her entire right, title, and interest in any and all inventions, discoveries and materials which are created or developed by her as a result of research performed at St. Jude.

1.2 Principal Investigator has expertise in the study of PKAN disease and other infectious diseases and desires to conduct a Research Program, as hereinafter described, at St. Jude.

1.3 Sponsor desires to provide support for such Research Program in return for certain rights granted herein, and St. Jude is willing to grant such rights under the terms and conditions set forth herein.

St. Jude and Sponsor do hereby agree as follows:

### 2. <u>Definitions</u>

2.1 "Research Program" shall include, and be limited to, the research program to be conducted by the Principal Investigator as described in **Appendix A**; which shall be updated as appropriate, and as mutually agreed by the parties.

2.2 "IPR" shall mean St. Jude's intellectual property rights in and to any U. S. and foreign patent applications, and patents which issue therefrom, which claim any St. Jude Invention, Joint Invention or other invention conceived or first reduced to practice in the performance of the Research Program during the term of this Agreement, as well as St. Jude rights in and to any unpatentable invention, discovery, process, method or St. Jude Materials conceived or first reduced to practice in the performance of the Research Program during the term of this Agreement.

2.3 "St. Jude Materials" shall mean biological materials, whether patentable or unpatentable, which are created or discovered as a result of the Research Program during the term of this Agreement.

2.4 "Option" shall mean the exclusive right to acquire an exclusive, worldwide, royalty-bearing license, with the right to sublicense, for any and all IPR, upon commercially reasonable terms. Such option shall be exclusive and St. Jude shall not discuss licensing the IPR with any third party during the Option Period or Negotiation Period (as defined below).

\* ######## = Material omitted pursuant to a request for Confidential Treatment and submitted separately to the Commission on the date of submission of this Registration Statement on Form S-1.

2.5 "License Agreement" means the exclusive license agreement to be negotiated between Sponsor and St. Jude upon exercise of the Option granted herein.

2.6 "Patent Costs" shall mean any and all expenses incurred by St. Jude in the filing, prosecution and maintenance of patent applications or patents claiming any IPR for which Sponsor has elected to exercise its Option.

2.7 "Compound" shall mean any molecules, products, compounds, cells, tissues or products created by Sponsor or for which sponsor files a patent application, that Sponsor provides to St. Jude or Principal Investigator in connection with the Research Program

3. <u>Performance of the Research Program</u>. St. Jude shall undertake the Research Program, which shall be conducted under the direct supervision of Principal Investigator. The Research Program may be modified upon mutual written agreement of the parties. Performance of the Research Program is estimated to take approximately two (2) years.

########

5. <u>Intellectual Property</u>.

5.1 The Parties hereto acknowledge and agree that the existing inventions, technologies and other intellectual property of Sponsor and St. Jude are their separate property, respectively, and are not affected by this Agreement and neither party shall have any claims to or rights in such existing inventions, technologies and intellectual property of the other party, except as otherwise set forth in this Agreement and other written agreements between the Parties, if any.

5.2 <u>Inventions</u>. Any new invention, suggestion, idea, development, creation or discovery of any type directly or indirectly resulting from the work of the Research Program during the term of this Agreement, or disclosed in writing by St. Jude or the Principal Investigator as a Research Project invention-in-progress and completed after the term of this Agreement, shall be promptly disclosed in writing to Sponsor and identified as an Invention under this Agreement.

5.1.1 Any IPR resulting from the Research Program for which St. Jude employees are the sole inventors under U.S. patent law ("St. Jude Invention") shall be the exclusive property of St. Jude.

\* ######### = Material omitted pursuant to a request for Confidential Treatment and submitted separately to the Commission on the date of submission of this Registration Statement on Form S-1.

5.1.2 Any IPR resulting from the Research Program for which St. Jude employees and Sponsor employees are joint inventors ("Joint Invention") shall be jointly owned by St. Jude and Sponsor.

5.1.3 Any IPR resulting from the Research Program for which Sponsor employees are the sole inventors under U.S. patent law (the "Sponsor Invention") shall be the exclusive property of the Sponsor.

Inventorship (as opposed to ownership) of any Invention arising out of the Research Program shall be determined in accordance with United States' patent laws, or, in the event that an Invention is not patentable, by mutual agreement of the Parties based upon their relative contributions to the Research Program.

Notwithstanding any other provision of this Agreement, in no event shall St. Jude have any right or interest in or to any Sponsor Invention. Sponsor shall grant St. Jude and Principal Investigator a royalty-free research license during the term of this Agreement to any Inventions (including Rights of Sponsor embodied therein) solely for academic research purposes; provided that, St. Jude shall not license or sublicense to any third party nor commercially exploit such Sponsor Invention in any manner.

During the Term of this Agreement and subject to the terms set forth herein, Sponsor shall grant to St. Jude a non-exclusive, royalty-free research license to the Compounds solely for academic research purposes; provided that, St. Jude shall not license or sublicense to any third party nor commercially exploit such Compounds in any manner. If the Principal Investigator wishes to publish or otherwise disclose the results of any test or analysis of the Compound, the Principal Investigator and St. Jude shall comply with the provisions of Article 10 of this Agreement

5.2 Patents. St. Jude and Sponsor shall consult with each other and consider the reasonable comments of the non-filing party, on the filing of all U.S. and foreign patent applications which claim St. Jude Inventions or Joint Inventions. St. Jude shall file, prosecute and maintain, at its own expense and discretion, all U.S. and foreign patent applications and patents issuing thereon claiming any St. Jude Invention. Sponsor shall file (on behalf of St. Jude and Sponsor), prosecute and maintain, at its own expense, all U.S. and foreign patent applications claiming any Joint Invention. Sponsor shall consult with and keep St. Jude informed of such efforts, and it shall provide St. Jude with copies of all relevant documentation with respect thereto. Sponsor shall give St. Jude reasonable time to review and comment upon any draft application, official paper from any patent office requiring a response, and any proposed response thereto. Should Sponsor elect not to file, continue prosecution of, or maintain a patent application or patent claiming a Joint Invention, it will promptly notify St. Jude and St. Jude may file, prosecute or maintain such patent application or patent at its own expense and shall have sole rights thereafter to the subject Joint invention, notwithstanding the provisions of Paragraph 7.2.

6. <u>Option</u>.

- 6.1 In consideration for Sponsor's funding of the ########\*.
- 6.2 #########\*.
- 6.3 ########\*.
- 7. <u>Retained Rights</u>.

7.1 St. Jude shall retain the right to use any IPR exclusively licensed to Sponsor for internal research and educational purposes only and shall not be permitted to sublicense such right to any party without the prior written consent of Sponsor. St. Jude shall retain the right to transfer any such St. Jude Materials and related information or technology developed or created by St. Jude employees during the course of the Research Program to other not-for-profit institutions, solely for internal non-commercial research use and educational purposes; provided, that such other not-for-profit institutions are not permitted or granted the right to sublicense or transfer such material without the prior written consent of Sponsor. St. Jude agrees that it will not transfer such St. Jude Materials and related information or technology to any commercial entity without the prior written approval of Sponsor.

7.2 For any IPR covered under this agreement which is made in part using funds or resources obtained from the U.S. Government, the U.S. shall retain certain rights as set forth in the Bayh-Dole Act, 35 U.S.C. §§200 et seq.

7.3 Sponsor shall retain the right to use any IPR developed within the Research Program for internal research purposes.

# 8. <u>Confidentiality and Use of Names</u>.

8.1 "Confidential Information" shall mean any nonpublic information disclosed by one party to the other for the purpose of supporting performance under this Agreement. In the absence of express permission to the contrary, a party in receipt of Confidential information from the other party shall not transfer such information to any third party, will use such information for internal research purposes only and shall take, and cause its employees and agents, including Principal Investigator, to take all reasonable measures including but not limited to: (i) limit access to and use of Confidential Information to the Principal Investigator and other Institution employees for whom such access and use is required for performance of the Research Program; (ii) use Confidential Information only for the purposes approved by Sponsor in writing; (iii) prevent the access, transfer or disclosure of Confidential Information to any other person or entity without Sponsor's prior written approval; (iv) upon request by Sponsor, return to Sponsor all Confidential Information owned or provided by Sponsor, except that St. Jude may retain one copy of the Confidential Information solely to monitor its continuing obligations hereunder; and (v) immediately inform Sponsor of any unauthorized disclosure or misappropriation of Confidential Information and assist Sponsor in taking all reasonable and lawful actions necessary to prevent or abate such unauthorized disclosure or misappropriation.

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8.2 Nothing contained herein shall in any way restrict or impair the right of Sponsor or St. Jude to use, disclose, or otherwise deal with any information or data which:

(a) at the time of disclosure to a receiving party is generally available to the public or thereafter becomes generally available to the public by publication or otherwise through no act of the receiving party; or

- (b) the receiving party can show by written record was in its possession prior to the time of disclosure to it hereunder; or
- (c) is independently made available to the receiving party as a matter of right by a third party; or
- (d) is subject to disclosure by requirements of law.

8.3 Neither of the parties shall use the name of the other party or the name of a current or former staff member, employee, student or affiliated physician or faculty in any public disclosure, publicity or advertising involving the fact of this Agreement or its subject matter without the prior written approval of the other party, unless required to do so by law.

8.4 This Article 8 shall remain in effect for five (5) years following disclosure of any Confidential Information. St. Jude may, but only to the extent such disclosures are required by law, disclose Confidential Information in required reports to Regulatory Authorities (e.g. the FDA, Department of Health, Institutional Review Boards, Ethics and Biosafety Committees) or other Governmental agencies; provided, however, that St. Jude shall first inform Sponsor and seek to limit the scope of disclosure of Confidential Information and obtain confidential treatment by such agencies of the Confidential Information required to be disclosed. St. Jude acknowledges and agrees that all Confidential Information is of important commercial and competitive value to the Sponsor, and may be maintained by the Sponsor as confidential trade secrets.

9. <u>Reports</u>. St. Jude agrees that the Principal Investigator will furnish Sponsor with a written report summarizing all of the results of the Research Program within sixty (60) days of the one year anniversary and expiration of this Agreement. Sponsor shall have the unrestricted right to use all of the information included in any such report for internal research purposes only.

10. <u>Publications</u>. Sponsor recognizes that Principal Investigator may wish to publish or otherwise publicly disclose the results of the Research Program. St. Jude agrees to submit all such intended publications or presentations to Sponsor at least thirty (30) days prior to any submission or other public disclosure and to delete any Confidential Information belonging to Sponsor. If Sponsor determines that the publication or intended presentation contains patentable subject matter related to a St. Jude Invention or to a Joint Invention, St. Jude agrees to postpone publication or presentation for an additional sixty (60) days to permit the filing of a patent application or the deletion of such Confidential information. The Publication shall be reasonably modified or corrected to prevent disclosure of Sponsor's Confidential Information. Each Publication shall appropriately acknowledge Sponsor's contribution to the Research Project if so requested by Sponsor or required for such Publication.

11. <u>Term and Termination</u>

11.1 <u>Term</u>. Unless otherwise terminated by operation of law or by acts of the parties in accordance with the terms of this Agreement, this Agreement shall be in full force and effect for a period of two (2) years from the Effective Date. The term may be extended by written agreement between the parties.

# 11.2 <u>Termination</u>.

a) by St. Jude. If Sponsor fails to meet any of its material obligations under this Agreement and fails to remedy these failures within sixty (60) days after receipt of written notice thereof, St. Jude may terminate this Agreement upon written notice thereof, which shall terminate any further Option granted to Sponsor under this Agreement. Any termination of this Agreement shall not affect the rights or obligations granted to Sponsor under any Option previously exercised, or License Agreement executed, prior to the termination of this Agreement. If this Agreement is terminated by St. Jude for any reason other than Sponsor's failure to meet its obligations hereunder, St. Jude shall repay to Sponsor the uncommitted balance, if any, of the Research Program funding which was paid by Sponsor to St. Jude pursuant to Paragraph 4.

b) by Sponsor. In the event Principal Investigator is unable to continue supervision of the Research Program for a period in excess of sixty (60) days, St. Jude shall so notify Sponsor and may nominate a replacement. If St. Jude does not nominate a replacement or if that replacement is unsatisfactory to Sponsor, Sponsor may terminate this Agreement upon thirty (30) days written notice to St. Jude. In the event St. Jude fails to meet its obligations under this Agreement and shall fail to remedy these failures within sixty (60) days after receipt of written notification thereof, Sponsor shall have the option of terminating this Agreement upon written notice thereof, and such right to terminate shall be Sponsor's sole remedy at law or in equity. If this Agreement is terminated by Sponsor due to St. Jude's failure to meet its obligations hereunder, St. Jude shall repay to Sponsor the uncommitted balance, if any, of the Research Program funding which was paid by Sponsor to St. Jude pursuant to Paragraph 4. Any other termination of this Agreement by Sponsor shall not relieve Sponsor of its obligation to pay any monies due or owing to St. Jude at the time of such termination and shall not impair any accrued right of St. Jude. This Agreement may be terminated by either party at any time upon thirty (30) days advance written notice to the other party.

c) <u>Either Party</u>. If this Agreement is terminated by either party, (i) such termination shall be without prejudice to the rights and remedies of either party with respect to any breach of this Agreement by the other party, where such a breach occurred prior to the termination of this Agreement; (ii) the provisions of clauses 5-10, together with those other provisions of this Agreement which are incidental to, and required in order to give effect to those clauses, shall remain in full force and effect; and (iii) Sponsor shall be relieved of all outstanding payment obligations under Section 6 of this Agreement.

11.3 Survival. Sections 5.2, 6, 7, 8, 9, 10, 11.2, 12, 13 and 15 shall survive any termination of this Agreement.

12. <u>Indemnification</u>.

12.1 Sponsor agrees to indemnify, defend and hold harmless St. Jude and the American Lebanese Syrian Associated Charities (ALSAC) and their respective corporate affiliates and Boards of Governors, officers, staff, representatives and agents against all damages, expenses (including without limitation reasonable legal expenses), claims, demands, suits or other actions such as product liability claims (collectively, the "Claims"), arising in connection with this Agreement or the Research Program; <u>provided</u>, that such Claims are not the result of the negligence, misconduct or bad faith of St. Jude, the Principal Investigator or its or their employees, consultants, agents or representatives. St. Jude shall promptly notify Sponsor of any such claim and shall cooperate with Sponsor and its insurance carrier in the defense of the claim. Sponsor agrees to submit any proposed settlement to St. Jude in advance of its approval. Upon the first commercial sale of any product developed under the IPR herein, Sponsor agrees to procure, retroactive to the sale date, and maintain in an amount standard to the industry and the type of research being conducted comprehensive general liability insurance against any claims or expenses for which it is obligated to indemnify as provided above, or maintain a comparable program of self-insurance and upon the request of St. Jude, Sponsor shall provide St. Jude with a certificate of insurance or letter of self-insurance, evidencing the same.

12.2 St. Jude agrees to indemnify, defend and hold harmless the Sponsor and its affiliates, officers, employees, directors, representatives, managers, members, consultants, advisors and agents against any and all Claims that result from the negligence, misconduct or bad faith of St. Jude, the Principal Investigator or its or their affiliates, employees, consultants, agents or representatives. Sponsor shall promptly notify St. Jude of any such claim and shall cooperate with St. Jude and its insurance carrier in the defense of the claim. St. Jude agrees to submit any proposed settlement to Sponsor in advance of its approval.

## 13. <u>Warranties</u>.

13.1 EXCEPT AS PROVIDED BELOW, ST. JUDE MAKES NO WARRANTIES, EXPRESS OR IMPLIED AS TO ANY MATTER WHATSOEVER, INCLUDING WITHOUT LIMITATION THE CONDITION, ORIGINALITY, OR ACCURACY OF THE RESEARCH PROGRAM OR ANY INVENTION(S) OR PRODUCT(S), WHETHER TANGIBLE OR INTANGIBLE CONCEIVED, DISCOVERED, OR DEVELOPED UNDER THIS AGREEMENT; OR THE OWNERSHIP, MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OF THE RESEARCH PROGRAM OR ANY SUCH INVENTION OR PRODUCT. ST. JUDE SHALL NOT BE LIABLE FOR ANY DIRECT, CONSEQUENTIAL, OR OTHER DAMAGES SUFFERED BY SPONSOR, ANY SUB-LICENSEE, OR ANY OTHERS RESULTING FROM THE USE OF RESULTS OF THE RESEARCH PROGRAM OR ANY INVENTION OR PRODUCT RESULTING THEREFROM.

13.2 Nothing in this Agreement shall be construed as:

(a) a warranty or representation by St. Jude as to the validity or scope of any IPR; or

(b) a warranty or representation that anything made, used, sold or otherwise disposed of under any license of IPR from St. Jude is or will be free from infringement of patents of third parties; or

(c) conferring by implication or otherwise any license or rights under any patents of St. Jude other than IPR as defined herein, regardless of whether such patents are dominant or subordinate to IPR; or

(d) an obligation to furnish any know-how not provided under IPR.

13.3 St. Jude represents and warrants that it has the authority and legal right: (i) to enter into this Agreement and that it has taken all necessary actions in connection with the execution of this Agreement, (ii) to perform its obligations hereunder, and (iii) to grant the rights to Sponsor as herein granted. St. Jude also represents and warrants that all necessary consents, approvals and authorizations of all governmental authorities and other persons required to be obtained in connection with the execution, delivery and performance of this Agreement have been and shall be obtained. The execution and delivery of this Agreement and the performance of St. Jude's obligations hereunder do not conflict with or violate any requirement of applicable laws or regulations and do not and shall not conflict with, violate or reach or constitute a default or require any consent under any contractual obligation of St. Jude.

14. <u>Notices</u>. Notices hereunder shall be deemed sufficient if given by registered mail, postage prepaid, and addressed to the party to receive such notice at the address given herein, or such other address as may hereinafter be designated by notice in writing, or telecopied with a return telecopy confirmation.

To St. Jude: J. Scott Elmer Office of Technology Licensing – MS742 St. Jude Children's Research Hospital 262 Danny Thomas Place Memphis, TN 38105

To Sponsor: Retrophin, Inc. 777 Third Avenue 22<sup>nd</sup> Floor New York, NY 10017

> With a copy to (which shall not constitute notice): Katten Muchin Rosenman LLP 575 Madison Avenue New York, NY 10022 Attention: Evan L. Greebel, Esq.

15. <u>Title to Equipment</u>. St. Jude shall retain any equipment purchased and/or fabricated by it with funds provided by Sponsor under this Agreement.

16. <u>Waiver</u>. None of the terms of this Agreement may be waived or modified except by an express agreement in writing signed by both parties. The failure or delay of either party in enforcing any of its rights under this Agreement shall not be deemed a continuing waiver or a modification by such party of such right.

17. <u>Severability</u>. If one or more provisions of this Agreement shall be found to be illegal or invalid, it shall not affect the legality or validity of any of the remaining provisions hereof and such provision shall be interpreted in accordance with applicable law.

18. <u>Independent Contractors</u>. The relationship created by this Agreement shall be that of independent contractors with neither party having the authority to bind or act as agent for the other party or its employees for any purpose.

19. <u>Amendment</u>. The parties may, from time to time during the term of this Agreement, modify any of the provisions of this Agreement only by an instrument duly executed by both parties.

20. <u>Assignment</u>. This Agreement shall not be assignable by either party without the prior written consent of the other party, except by operation of law or to affiliates, or successors in interest to the business to which this Agreement pertains. Any and all assignments not made in accordance with this section shall be void.

21. <u>Expenses</u>. Except as otherwise expressly set forth herein, all expenses incurred by the parties in connection with this Agreement shall be borne wholly by the party incurring such expenses.

## 22. <u>Remedies</u>.

22.1 Given the nature of the obligations set forth in Sections 5 and 8 of this Agreement, St. Jude acknowledges and agrees that Sponsor may be irreparably damaged by St. Jude's alleged breach or violation of such sections. Without prejudice to the rights and remedies otherwise available to the Sponsor, it shall be entitled, without the requirement of a posting of a bond or other security to seek equitable relief, including an injunction or specific performance, in the event of any breach of the provisions of Sections 5 or 8 of this Agreement.

22.2 This Agreement shall be construed and enforced under the laws of the State of New York without regard to conflicts or choice of law provisions. The parties hereby agree that any dispute, controversy or claim arising under, out of or relating to this Agreement and any amendments, shall be referred to and finally determined by arbitration in accordance with the Rules of Arbitration of the International Chamber of Commerce's International Court or Arbitration (ICC). The arbitration shall be conducted in English, the Arbitrator shall; be admitted to the Bar of the State of New York, the Arbitrator shall be experienced in the matters set forth in this Agreement and the arbitration shall occur at the ICC offices in New York. Each Party shall bear its own attorneys fees and expenses and each Party to the arbitration will pay an equal share of the expenses and fees of the arbitrator, together with other expenses of the arbitration incurred or approved by the arbitrator. The Parties agree to abide by all decisions and awards rendered in such proceedings. Such decisions and awards rendered by the arbitrator shall be final and conclusive. All such controversies, claims or dispute shall be settled in this manner in lieu of any action at law or equity; provided, however, that nothing in this subsection shall be construed as precluding the bringing an action for injunctive relief as provided in Section 22.1. Any court of competent jurisdiction may enter judgment upon the award. IF FOR ANY REASON THIS ARBITRATION CLAUSE BECOMES NOT APPLICABLE OR IF THE PARTIES ARE SEEKING INJUNCTIVE OR EQUITABLE RELIEF AS PROVIDED ABOVE, THEN EACH PARTY, (i) TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, HEREBY IRREVOCABLY WAIVES ALL RIGHT TO TRIAL BY JURY AS TO ANY ISSUE RELATING HERETO IN ANY ACTION, PROCEEDING, OR COUNTERCLAIM ARISING OUT OF OR RELATING TO THIS AGREEMENT OR ANY OTHER MATTER INVOLVING THE PARTIES HERETO.

23. <u>Entire Agreement</u>. This Agreement constitutes the entire understanding of both parties with respect to the subject matter herein contained and supersedes any previous agreements on this subject matter executed by these parties.

24. <u>U.S. Securities Laws</u>. St. Jude\_is aware, and will advise its employees, affiliates, independent contractors, agents and consultants who are provided with information about Sponsor in connection with this Agreement, of the restrictions imposed by the United States securities law on the purchase and sale of securities by any person who has received material, non-public information from the issuer of such securities and on the communication of such information to any other person when it is reasonably foreseeable that such other person is likely to purchase or sell such securities in reliance upon such information.

25. <u>Warranty Of Non-Exclusion</u>. Sponsor represents and warrants that neither it nor any of its employees are or have been excluded, terminated, suspended, or debarred from a federal or state health care program or from participation in any federal or state procurement or non-procurement programs. Sponsor further represents that no final adverse action by the federal or a state government is threatened or pending, or has occurred, against Sponsor, its affiliates, or to its knowledge, against any employee, or any agent employed to provide goods or services pursuant to this transaction. Sponsor further represents and warrants that it neither it nor any employee has been convicted of a criminal offense as set forth in 42 U.S.C. § 1320a-7, including but not limited to an offense related to the provision of healthcare items or services. Sponsor will immediately notify St. Jude of any change in the status of the representations and warranty set forth in this section. Any breach of this section shall give St. Jude the right to terminate this transaction immediately for cause.

IN WITNESS WHEREOF, the Parties hereto have caused this Agreement to be executed by their respective duly authorized officers or representatives on the respective dates indicated below.

Retrophin, Inc.	St. Jude Children's Research Hospital
By:	By: J. Scott Elmer Director, Technology Licensing
Printed Name	
Title:	Date:
Date:	Agreed to by Dr. Suzanne Jackowski
	Date:
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\* ######## = Material omitted pursuant to a request for Confidential Treatment and submitted separately to the Commission on the date of submission of this Registration Statement on Form S-1.

\* ######## = Material omitted pursuant to a request for Confidential Treatment and submitted separately to the Commission on the date of submission of this Registration Statement on Form S-1.

Retrophin, Inc.

List of Subsidiaries

Subsidiary

Jurisdiction of Incorporation

1. Retrophin Pharmaceutical, Inc.

Delaware