# Delivering a Promising Future




**RXi Pharmaceuticals**
Next Generation in RNAi®

2013 Annual Report

# RXi Pharmaceuticals
Next Generation in RNAi®

## CORPORATE OVERVIEW

RXi Pharmaceuticals Corporation is a biotechnology company focused on discovering, developing and commercializing innovative therapies based on its proprietary, next-generation RNAi platform. Therapeutics that use RNA interference, or "RNAi," have great promise because of their ability to "silence," or down-regulate, the expression of a specific gene that may be overexpressed in a disease condition. Building on the pioneering work of scientific founder and Nobel Laureate Dr. Craig Mello, RXi's first RNAi product candidate, RXI-109, entered into RXi's first human clinical trial in June 2012. RXI-109 targets connective tissue growth factor (CTGF), a key regulatory component of fibrosis and scar formation, and is initially being developed to reduce or inhibit scar formation in the skin following surgery.

RXi Pharmaceuticals believes it is well positioned to compete successfully in the RNAi-based therapeutics market with an experienced management team, an accomplished Scientific Advisory Board, and a strong, broad intellectual property position in RNAi chemistry and delivery.

## RNAI THERAPEUTICS

RNAi is a naturally occurring phenomenon by which short double-stranded RNAs interfere with the expression of targeted genes. The development of therapeutics based on RNAi technology takes advantage of this phenomenon and potentially allows us to reduce the expression of particular genes within living cells. The discovery of RNAi is regarded as a significant advancement in the scientific community, as evidenced by the selection of RNAi as the "Breakthrough of the Year" in 2002 by the journal *Science* and by the 2006 Nobel Prize in Medicine being awarded to the co-discoverers of RNAi, including Dr. Craig Mello. RNAi offers a novel approach to the drug development process because RNAi compounds can potentially be designed to target any one of the thousands of human genes, many of which are undruggable by other modalities.

## RXI NEXT-GENERATION THERAPEUTIC PLATFORM: SD-RXRNA®

A successful RNAi therapeutic platform includes stable, specific and potent RNAi compounds and the ability to deliver these compounds to the tissue(s) of choice. One conventional solution to the delivery problem involves encapsulation into a lipid-based particle, such as a liposome, to improve circulation time and cellular uptake. Scientists at RXi have used an alternative approach to delivery in which drug-like properties were built into the RNAi compound itself. These novel compounds are termed "self-delivering" RNAi compounds or sd-rxRNA. RXI-109, RXi's first clinical candidate, is based on the novel chemistry of the sd-rxRNA platform.

## RXI-109 FOR DERMAL SCARRING

RXI-109 is initially being developed to reduce fibrosis or scarring of the skin at a post-surgical wound site. Scarring represents a high unmet medical need as there are currently no FDA-approved therapies in the U.S. for the treatment and prevention of scars in the skin. If approved, RXI-109 would be a "first-in-class" RNAi treatment for the prevention or reduction of dermal scarring.

Together with leading scientists in the field of scarring, RXi Pharmaceuticals believes that reducing the level of CTGF early in the wound healing process will result in a reduced level of scarring. A therapeutic of this type could have great benefit for trauma and surgical patients (especially relating to raised or hypertrophic scarring), burn patients (including potential burn contractures), surgical revision of existing unsatisfactory scars, and in the treatment, removal and inhibition of keloids (scars which extend beyond the original skin injury).

## OPHTHALMOLOGY PRECLINICAL PROGRAMS

RXi is also exploring the use of sd-rxRNAs for treatment of ocular indications as the self-delivering technology lends itself well to indications that take advantage of local delivery. As a result, we have initially identified the following areas of focus for the development of targeted therapeutics for ocular indications: Retinal scarring related to conditions such as Proliferative Vitreoretinopathy (PVR) or Macular Degeneration, and Retinoblastoma.

The most advanced preclinical program is in the area of Retinal Scarring where we have shown that our dermal anti-scarring clinical candidate, RXI-109, can silence CTGF in the eye. The next steps in preclinical development for this program include evaluation of efficacy in an *in vivo* model of retinal scarring to quickly establish proof of concept.

## INTELLECTUAL PROPERTY

RXi's intellectual property estate includes patents and patent applications related to chemistries, sequences, configurations, compounds, delivery technologies, and therapeutic targets. This IP has been developed by RXi as well as in-licensed or acquired from third parties. We believe these patents and patent applications define broad coverage for the development and commercialization of advanced RNAi therapeutics. In particular, they relate to novel and proprietary structural and chemical modification patterns, used to introduce "drug-like" properties to rxRNA® compounds. Fundamental IP related to development of RXi's rxRNA compounds covers distinct (from conventional siRNAs) structures with duplex length shorter than 15 bases, or longer than 25 bases in the context of advanced and diverse chemical modification patterns. In 2013, RXi acquired the OPKO RNAi intellectual property estate. The OPKO RNAi estate includes 12 patent families and provides broad patent filings for siRNA compounds which target genes involved in angiogenesis, cancer, immune disorders and inflammatory diseases. Methods for siRNA delivery across the blood-brain and blood-retina barrier are also disclosed for therapeutic and diagnostic use.

Dear Shareholder,

On behalf of the Board of Directors and the employees of RXi Pharmaceuticals Corporation, we thank you for having been a shareholder for the past two years, or for having become a new shareholder in 2013. Indeed, the RXi shareholder base has become a lot broader and more diversified in the past year, and that evolution in the shareholder base is a reflection of the progress and achievements made by RXi Pharmaceuticals during that period.

Going forward, there are five pillars we expect to support the continued growth of our Company into the next several years:

1. Our unmatched intellectual property platform in the RNAi space;
2. Development of RXI-109, our lead compound currently in Phase 2 clinical trials, for dermal scarring in planned surgeries;
3. Formation of an Ophthalmology Franchise in addition to our existing dermatology focus;
4. Expanding our preclinical pipeline by moving discovery stage compounds based on RXi and OPKO targets into preclinical development; and
5. Potential acquisition of Phase 2 clinical assets in our area of clinical development focus.

These five pillars are supported by a nimble and dedicated professional group of employees, with strategic guidance provided by a seasoned and successful Board of Directors.

In the past 12 months, we have achieved progress with each of these pillars.

Our intellectual property platform has received significant attention in several conferences and publications. A key patent, for self-delivering RNAi compounds (sd-rxRNAs®) in the field of fibrosis, was granted by the U.S. Patent and Trademark Office.

Two Phase 1 studies were successfully completed with our lead compound, RXI-109, for dermal scarring. These studies have shown that RXI-109 is safe and well tolerated in healthy volunteers, and demonstrated a long-lasting reduction in Connective Tissue Growth Factor (CTGF), as well as statistically significant silencing of the mRNA for CTGF, which is consistent with the mechanism of action of RXI-109. The Company initiated its Phase 2 clinical program in late 2013, enrolling patients with abnormal scarring (hypertrophic scars).

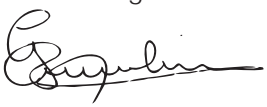
Our ophthalmology program has advanced to include a discovery-stage collaboration with the University of Southern California for the treatment of Retinoblastoma. Additionally, the Company has conducted dose finding toxicity studies with RXI-109 for intra-ocular injections in non-human primates.

The acquisition of substantially all of OPKO's RNAi-related assets, including an extensive intellectual property portfolio in the RNAi space, has provided RXi Pharmaceuticals instant access to well defined patented siRNA sequences that could potentially be adapted and optimized for delivery with our self-delivering platform, eliminating the need for special formulations to achieve clinical efficacy.

As a result of our corporate and clinical success thus far, RXi has been approached by third parties to evaluate additional Phase 2 clinical candidates for a possible acquisition or collaborative development effort associated with commercial rights. We will continue to evaluate these clinical candidates and others for the potential expansion of our clinical pipeline.

During the past two years, RXi Pharmaceuticals has maintained its focus on the key activities to achieve its goals. This has resulted in achieving our milestones on time and well within budget; rather unusual in the biotechnology space. We do not intend to lose that focus, and meeting our clinical milestones with RXI-109 in Phase 2 remains our highest priority. While doing that, we have started to prepare the next growth phase of our Company, which may include: an additional therapeutic area (ophthalmology), additional preclinical candidates (RXI IP or OPKO acquired IP), addition of Phase 2 clinical products (acquisition) and potential partnering activities (e.g. out-licensing) to discover and develop sd-rxRNA compounds for therapeutic areas outside of our core focus.

As we have done this past year, the Company will strive to continue to deliver against its milestones in a timely manner and within budget in 2014. We value the trust and support we receive from you, our shareholders, and hope to deliver a return on your investment by increasing the value of the Company as we evolve through our various stages of development.



Geert Cauwenbergh
*President & Chief Executive Officer*

Robert Bitterman
*Chairman of the Board of Directors*

# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

# FORM 10-K

**(Mark One)**

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

**For the fiscal year ended December 31, 2013**

**Or**

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

**For the transition period from to**

**Commission File Number 000-54910**

# RXi PHARMACEUTICALS CORPORATION

**(Exact name of registrant as specified in its charter)**

| **Delaware** | **45-3215903** |
|---|---|
| **(State or other jurisdiction of incorporation or organization)** | **(I.R.S. Employer Identification No.)** |

**1500 West Park Drive, Suite 210 Westborough, Massachusetts 01581**
**(Address of principal executive offices and Zip Code)**

**(508) 767-3861**
**(Registrant's telephone number, including area code)**

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

| **Title of each class** | **Name of exchange on which registered** |
|---|---|
| **Common stock, par value $0.0001 per share** | **The NASDAQ Capital Market** |

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

**Title of Each Class**

**None.**

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

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

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

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

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

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

Large accelerated filer ☐ Accelerated filer ☐

Non-accelerated filer ☐ (Do not check if a smaller reporting company) Smaller reporting company ☒

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

The aggregate market value of the voting common stock held by non-affiliates of the registrant, based on the closing sale price of the registrant's common stock as reported on OTCQX on June 30, 2013, was approximately $46,177,173. Shares of common stock held by each officer and director and by each person who is known to own 10% or more of the outstanding Common Stock have been excluded in that such persons may be deemed to be affiliates of the Company. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of March 21, 2014, RXi Pharmaceuticals Corporation had 13,373,239 shares of common stock, $0.0001 par value, outstanding.

**Documents incorporated by reference:**

Portions of the registrant's definitive proxy statement for its 2014 annual meeting of stockholders, to be filed with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2013, are incorporated by reference into Part III in this Form 10-K.

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**RXI PHARMACEUTICALS CORPORATION**
**ANNUAL REPORT ON FORM 10-K**
**For the fiscal year ended December 31, 2013**

## FORWARD-LOOKING STATEMENTS

This report contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as “intends,” “believes,” “anticipates,” “indicates,” “plans,” “intends,” “expects,” “suggests,” “may,” “should,” “potential,” “designed to,” “will” and similar references. Such statements include, but are not limited to, statements about: our ability to successfully develop RXI-109 and our other product candidates; the future success of our clinical trials with RXI-109; the timing for the commencement and completion of clinical trials; the future success of our strategic partnerships; and our ability to implement cost-saving measures. Forward-looking statements are neither historical facts nor assurances of future performance. These statements are based only on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, projections, anticipated events and trends, the economy and other future conditions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that are difficult to predict and many of which are outside of our control. Our actual results and financial condition may differ materially from those indicated in the forward-looking statements. Therefore, you should not rely on any of these forward-looking statements. Important factors that could cause our actual results and financial condition to differ materially from those indicated in the forward-looking statements include, among others: the risk that our clinical trials with RXI-109 may not be successful in evaluating the safety and tolerability of RXI-109 or providing evidence of increased surgical scar reduction compared to placebo; the successful and timely completion of clinical trials; uncertainties regarding the regulatory process; the availability of funds and resources to pursue our research and development projects, including our clinical trials with RXI-109; general economic conditions; and those identified in this Annual Report on Form 10-K under the heading “Risk Factors” and in other filings the Company periodically makes with the Securities and Exchange Commission. Forward-looking statements contained in this Annual Report on Form 10-K speak as of the date hereof and the Company does not undertake to update any of these forward-looking statements to reflect a change in its views or events or circumstances that occur after the date of this Annual Report on Form 10-K.

## PART I

### ITEM 1. *BUSINESS*

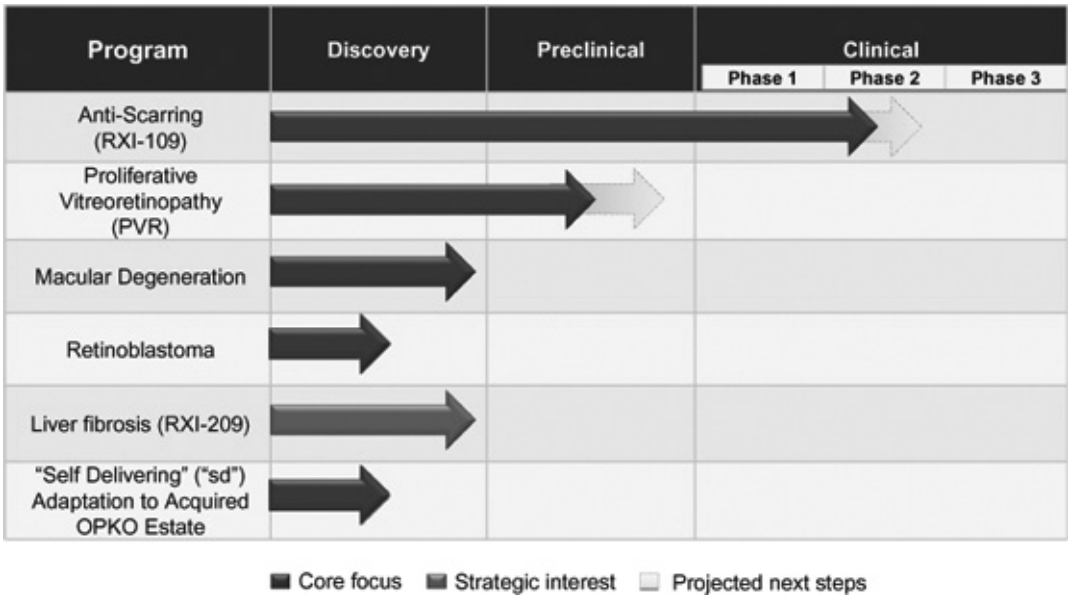
**Overview**

We are a biotechnology company focused on discovering, developing and commercializing innovative therapies based on our proprietary, next-generation RNA interference ("**RNAi**") platform. Therapeutics that use RNAi have great promise because of their ability to "silence," or down-regulate, the expression of a specific gene that may be over-expressed in a disease condition. Prior to September 2011, our business was operated as an unincorporated division within our former parent company. We were incorporated in Delaware as a wholly owned subsidiary of our former parent company on September 8, 2011 in preparation for our planned spin-off, which was completed on April 27, 2012. Since that date, we have operated as an independent, publicly traded company.

By utilizing the expertise in RNAi and the comprehensive RNAi platform that we have established, we believe that we have discovered and will be able to discover and develop lead compounds and progress them into and through clinical development for potential commercialization. Our proprietary therapeutic platform is comprised of novel RNAi compounds, referred to as rxRNA® compounds, that are distinct from, and we believe convey significant advantages over, conventional siRNA (conventionally-designed "small interfering RNA" compounds), and offer many of the properties that we believe are important to the clinical development of RNAi-based drugs. We have developed a number of unique forms of rxRNA compounds, all of which have been shown to be highly potent both *in vitro* and in preclinical *in vivo* models. These RNAi compounds include rxRNAori® and sd-rxRNA®, or "self-delivering" RNA. Based on our research, we believe that these different, novel siRNA configurations have various potential advantages for therapeutic use. These potential advantages include high potency, increased resistance to nucleases and modifications to eliminate off-target effects, and, in the case of the sd-rxRNA compounds, access to cells and tissues with no additional formulation required, and, hence, reduced cell toxicity, which is known to be an issue with unmodified siRNAs.

**Our Therapeutic Pipeline**

The following is a summary of our therapeutic development programs.



**RXI-109 Clinical Development Program**

Our lead clinical product candidate is RXI-109, a self-delivering RNAi compound (sd-rxRNA) being developed for the reduction of dermal scarring. RXI-109 is designed to reduce the expression of connective tissue growth factor ("**CTGF**"), a critical regulator of several biological pathways involved in scarring and fibrotic diseases. RXI-109 is being developed to prevent or reduce dermal scarring following surgery or trauma, as well as for the management of hypertrophic scars and keloids.

In June 2012, we initiated our first clinical trial of RXI-109, known as Study 1201. Study 1201 was designed to evaluate the safety and tolerability of several single-dose levels of RXI-109 in humans. Study 1201 enrolled fifteen subjects in a single-center, randomized, single-dose, double-blind, ascending dose, within-subject controlled study of RXI-109 for the treatment of incision scars, during which single, intradermal injections of escalating doses were administered. Subjects received an injection of RXI-109 in two separate areas on the abdomen and placebo injections in two other areas of the abdomen, followed by a small incision at each injection site. RXI-109 was well tolerated by intradermal injection. No serious local or systemic side effects were observed in the subjects at any of the doses administered, and maximum systemic exposure after intradermal administration was assessed at approximately 5% of the total dose administered. In this study, RXI-109 showed excellent safety and tolerability with ascending single doses and significantly reduced the expression of CTGF protein in the wounded area in a dose-dependent manner 84 days after a single dose, suggesting a potent and long lasting effect on this key biomarker for abnormal scarring.

In December 2012, we initiated a second Phase 1 clinical trial with RXI-109, known as Study 1202. Study 1202 was designed to evaluate the safety and tolerability of multi-dose administration of RXI-109 in healthy volunteers, including an evaluation of surrogate end points of clinical efficacy. In total, Study 1202 enrolled fifteen subjects (5 cohorts of 3 subjects each) in a single-center, randomized, multi-dose, double-blind, ascending dose, within-subject controlled study of RXI-109 for the treatment of incision scars. Eight small skin incisions were made in their abdomen and subjects received treatment with RXI-109 at the four incision sites on one side of the abdomen and placebo treatment at the four incision sites on the other side of the abdomen. Treatments were given by three intradermal injections over a 2-week period. Subjects were monitored for safety and local and systemic effects over a total study period of 84 days. Multiple dermal injections were well tolerated at all dose levels. Treatment with RXI-109 demonstrated a trend for dose-dependent silencing of CTGF mRNA in the treated areas, resulting in 43-50% reduction of CTGF mRNA levels compared to the placebo when measured three days after the last dose. In one of two highest-dose cohorts, the dosing period was delayed by two weeks after the incisions were made. No additional benefit was seen on mRNA reduction.

Based on the safety profile shown in our two Phase 1 clinical trials, we initiated a Phase 2 clinical trial for RXI-109 in November 2013 known as Study 1301. In this study, patients with a long hypertrophic scar in the lower abdominal area are eligible to receive scar revision surgery and subsequent treatment with RXI-109 in one of two treatment regimens. Patients will receive RXI-109 and placebo on a blinded basis at the distal ends of their revised scar, leaving a central untreated section of the scar. Each patient's revised scar area will provide the opportunity to compare the appearance of the revised areas after treatment with RXI-109 or placebo or when left untreated. This design allows for intra-subject comparison of the three revised scar segments, thereby increasing the power of the study.

In 2014, we expect to initiate two additional Phase 2 studies. The first additional Phase 2 study will evaluate the effect of RXI-109 on the recurrence of keloids after keloid revision surgery and the second additional Phase 2 study will evaluate the effect of RXI-109 on suppressing recurrence of hypertrophic scars after bilateral scar revision surgery in the breast area.

As there are currently no Food and Drug Administration ("**FDA**")-approved drugs to prevent scar formation, a therapeutic of this type could have great benefit for trauma and surgical patients, as a treatment during the surgical revision of existing unsatisfactory scars, and in the treatment, removal and inhibition of keloids (scars that extend beyond the original skin injury).

**Other Development Programs**

While focusing our efforts on our RXI-109 development program, we also intend to continue to advance additional development programs both on our own and through collaborations with academic and corporate third parties. On March 1, 2013, we entered into an asset purchase agreement with OPKO Health, Inc. ("**OPKO**") pursuant to which we acquired substantially all of OPKO's RNAi-related assets, including patents, licenses, clinical and preclinical data and other related assets (the "**OPKO Asset Purchase**").

Current programs in the discovery and preclinical stages include:

- a Small Business Innovation Research ("**SBIR**") grant to evaluate and develop sd-rxRNAs as potential therapeutics for the treatment of retinoblastoma;
- a collaboration evaluating the potential to use a CTGF-targeting sd-rxRNA as a therapeutic to reduce or inhibit retinal scarring, which often occurs as a consequence of some retinal diseases and following retinal detachment; and
- a discovery program to identify potential sd-rxRNA lead compounds and targets from the RNAi-related assets acquired from OPKO, which are at an early stage of development.

**Future Applications of RXI-109**

Overexpression of CTGF is implicated in dermal scarring and fibrotic disease, and because of this, we believe that RXI-109 or other CTGF-targeting RNAi compounds may be able to treat other fibrotic indications, including pulmonary fibrosis, liver fibrosis, acute spinal injury, ocular scarring, joint fibrosis and vascular restenosis. If the current clinical trials of RXI-109 produce successful results in dermal anti-scarring, we may explore opportunities in these additional indications, as well as other possible dermatology applications (e.g., cutaneous scleroderma).

**Market Opportunity**

There are currently no FDA-approved therapeutics in the United States for the treatment and prevention of scars in the skin. However, there are over 42 million procedures in the United States each year that could potentially benefit from a therapeutic treatment that could successfully reduce or prevent scarring; thus, the market potential is quite large. In addition to cosmetic and reconstructive surgeries, medical interventions which could incorporate an anti-scarring agent include scarring that results from trauma, surgery or burns (especially relating to raised or hypertrophic scarring or contracture scarring), surgical revision of existing unsatisfactory scars, and in the treatment, removal and inhibition of keloids (scars which extend beyond the original skin injury).

In November 2013, we signed a distribution agreement with Ethicor Ltd. ("**Ethicor**"), a UK-based unlicensed medicinal products ("**Specials**") pharmaceutical company. The agreement provides Ethicor with the distribution rights to RXI-109 in the European Union, with the possibility to negotiate in the future to extend such rights to other regions of the world, excluding the United States, Canada and Mexico. If approved for commercialization, Ethicor will pay us a double-digit percentage of any gross profits from its sales of RXI-109. Ethicor's distribution rights continue until the agreement is terminated; provided, however, that should we obtain marketing authorization for RXI-109 in any of the countries covered by the agreement, we have the option to terminate the agreement with respect to each such country in which marketing authorization has been obtained. Under the European medicines legislation (Directive 2001/83/EC, Article 5(1)), we expect that Ethicor will be able to supply, prior to regulatory approval, RXI-109 as a "Special" drug. A "Special" drug may be requested by an authorized health-care professional to meet the special needs of an individual patient under their direct responsibility. The collaboration is important for health-care professionals and patients who can get safe controlled early access to a development drug and is a significant milestone for the Company, not only in possible early revenue, but as increased exposure to RXI-109 may be key in accelerating the development of our drug.

**Reverse Stock Split**

On July 23, 2013, we effected a 1-for-30 reverse stock split of our outstanding common stock in connection with a listing of our common stock on the NASDAQ Capital Market. Stockholders who would otherwise have been entitled to fractional shares as a result of the reverse stock split received a cash payment in lieu of receiving fractional shares. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices, if applicable, were proportionately increased in accordance with the terms of the agreements governing such securities. Shares of common stock reserved for issuance upon the conversion of the Company's Series A convertible preferred stock ("**Series A Preferred Stock**") were proportionately reduced and the respective conversion prices were proportionately increased. All share and per share amounts in the financial statements have been retroactively adjusted for all periods presented to give effect to the reverse stock split, including reclassifying an amount equal to the reduction in par value to additional paid-in capital.

**Recent Business Developments**

During 2013 and in the first quarter of 2014, we announced several important developments that are outlined below.

- In March 2013, we entered into an asset purchase agreement with OPKO pursuant to which we have acquired substantially all of OPKO's RNAi-related assets, including patents, licenses, clinical and preclinical data and other assets.
- In March 2013, we raised approximately $16.4 million in a financing led by OPKO Health, Inc. and Frost Gamma Investments Trust, a trust controlled by Phillip Frost, M.D., as described more fully below.
- In April 2013, we appointed two independent directors to our Board of Directors, Mr. Paul Dorman and Dr. Curtis Lockshin.
- In June 2013, our common stock began trading on the OTCQX tier of the OTC Markets Group Inc.
- In June 2013, we announced that Study 1201 did not cause significant side effects or toxicities and showed that RXI-109 significantly reduced the expression of CTGF protein in the wound area in a dose-dependent manner 84 days after a single dose.
- In July 2013, we announced that multiple injections of RXI-109 were well tolerated with minimal and mild side effects in Study 1202. The treatment of RXI-109 in this study resulted in dose-dependent silencing of CTGF mRNA in the treated areas 3 days after the last dose.
- In July 2013, we completed a 1-for-30 reverse stock split of our outstanding common stock, which was effected on July 23, 2013.
- In July 2013, we applied to NASDAQ for approval to move the listing of our common stock from the OTCQX marketplace to The NASDAQ Capital Market.
- In August 2013, we entered into an exchange agreement with Tang Capital Partners, L.P. ("**TCP**") pursuant to which TCP exchanged a total of 2,000 shares of Series A Preferred Stock for a like number of shares of Series A-1 convertible preferred stock ("**Series A-1 Preferred Stock**") for the purpose of increasing stockholders' equity in support of the Company's efforts to list its common stock on the NASDAQ Capital Market.
- In November 2013, we signed a distribution agreement with Ethicor. The agreement provides Ethicor with the distribution rights to RXI-109 in the European Union, with the possibility to negotiate in the future to extend such rights to other regions of the world, excluding the United States, Canada and Mexico. Should we obtain marketing authorization for RXI-109 in any of the countries covered by the agreement, we have the option to terminate the agreement with respect to each such country in which marketing authorization has been obtained. The gross profits generated from any sales of RXI-109 by Ethicor will be shared between us and Ethicor.

- In November 2013, we initiated Study 1301, our first Phase 2 clinical trial for RXI-109. In this study, patients with long hypertrophic scars in the lower abdominal area will be eligible to receive scar revision surgery and subsequent treatment with RXI-109 in one of two treatment regimens to evaluate the effect of RXI-109 on the appearance of the revised areas when compared to placebo.
- In December 2013, we announced the results from additional cohorts in our second Phase 1 clinical trial, Study 1202. RXI-109 was dosed over the initial 2-week period following the incision which resulted in a 50% reduction of CTGF mRNA expression compared to the placebo when measured three days after the last dose. In the fifth cohort of the study, the dosing period was delayed by two weeks after the incisions were made. No additional benefit was seen on mRNA reduction. Multiple dermal injections were well tolerated at all doses, and treatment with RXI-109 resulted in dose-dependent silencing of CTGF mRNA in the treated areas.
- In January 2014, we entered into a second exchange agreement with TCP pursuant to which TCP exchanged a total of 3,000 shares of Series A Preferred Stock for a like number of shares of Series A-1 Preferred Stock for the purpose of increasing stockholders' equity in support of the Company's efforts to list its common stock on the NASDAQ Capital Market.
- In February 2014, we commenced trading our common stock on the NASDAQ Capital Market.
- In March 2014, we announced that we received the Issue of Notification from the United States Patent and Trademark Office on our unique self-delivering RNAi compounds (sd-rxRNA), for the treatment of fibrosis. The patent covers the use of sd-rxRNAs targeting CTGF for the treatment of fibrotic disorders. The patent (U.S. Patent Number 8,664,189) is scheduled to expire in 2029.

**Financial Condition**

We have generated significant losses to date, have not generated any product revenue to date and may not generate product revenue in the foreseeable future, if ever. We expect to incur significant operating losses as we advance our product candidates through the drug development and regulatory process. We will need to generate significant revenues to achieve profitability and might never do so. In the absence of product revenues, our potential sources of operational funding are expected to be the proceeds from the issuance of debt, sale of equity, funded research and development payments and payments received under partnership and collaborative agreements.

On March 6, 2013, we entered into a Securities Purchase Agreement (the "**SPA**") pursuant to which we agreed to issue 3,765,230 shares of common stock at a price of $4.35 per share (the "**Offering**"). The gross proceeds from the Offering, which closed on March 12, 2013, were approximately $16.4 million, and the net proceeds, after payment of commissions and other costs, were approximately $15.7 million. We have used the proceeds from the Offering for general corporate purposes, including the advancement of our RXI-109 program, research and development and general and administrative expenses.

We believe that our existing cash, cash equivalents and short-term investments should be sufficient to fund our operations, including the planned Phase 2 programs for RXI-109, into the second quarter of fiscal 2015. In the future, we will be dependent on obtaining funding from third parties, such as proceeds from the issuance of debt, sale of equity, funded research and development programs and payments under partnership and collaborative research and business development agreements, in order to maintain our operations and meet our obligations to licensors. There is no guarantee that debt, additional equity or other funding will be available to us on acceptable terms, or at all. If we fail to obtain additional funding when needed, we would be forced to scale back, or terminate, our operations or to seek to merge with or to be acquired by another company.

**Introduction to the Field of RNAi Therapeutics**

RNAi is a naturally occurring phenomenon where short, double-stranded RNA molecules interfere with the expression of targeted genes. RNAi technology takes advantage of this phenomenon and potentially allows us to effectively interfere with particular genes within living cells by designing RNA-derived molecules targeting those genes.

RNAi offers a novel approach to the drug development process because, as described below under "The RNAi Mechanism," RNAi compounds can potentially be designed to target any one of the thousands of human genes, many of which are "undruggable" by other modalities. The specificity of RNAi is achieved by an intrinsic, well-understood biological mechanism based on designing the sequence of an RNAi compound to match the sequence of the targeted gene. The sequence of the entire human genome is now known, and the mRNA coding sequence for many proteins is already available. Supported by numerous gene-silencing reports and our own research, we believe that this sequence information can be used to design RNAi compounds to interfere with the expression of almost any specific gene.

**The RNAi Mechanism**

The genome is made of double-stranded DNA (the double helix) with 20,000-25,000 protein coding genes that act as instruction manuals for the production of all human proteins. Proteins are important molecules that allow cells and organisms to live and function. With rare exceptions, each cell in the human body has the entire complement of genes. However, only a subset of these genes directs the production of proteins in any particular cell type. For example, a muscle cell produces muscle-specific protein, whereas a skin cell does not.

In order for a gene to guide the production of a protein, it must first be copied into a single-stranded chemical messenger (messenger RNA or mRNA), which is then translated into protein. RNAi is a naturally occurring process by which a particular messenger RNA can be destroyed before it is translated into protein. The process of RNAi can be artificially induced by introducing a small, double-stranded fragment of RNA corresponding to a particular messenger RNA into a cell. A protein complex within the cell called RISC (RNA-Induced Silencing Complex) recognizes this double-stranded RNA fragment and binds to it. RISC then splits the double strands apart, retaining one strand in the RISC complex. The RISC then helps this guide strand of RNA bind to and destroy its corresponding cellular messenger RNA target. Thus, RNAi provides a method to potentially block the creation of the proteins that cause disease.

Since gene expression controls most cellular processes, the ability to inhibit gene expression provides a potentially powerful tool to treat human diseases. Furthermore, since the human genome has already been decoded, and based on numerous gene-silencing reports, we believe that RNAi compounds can readily be designed to interfere with the expression of any specific gene. Based on our internal research and our review of certain scientific literature, we also believe that our RNAi platform may allow us to develop therapeutics with significant potential advantages over therapeutics developed using traditional methods, including:

- High specificity for targeted genes;
- High potency (low doses);
- Ability to interfere with the expression of potentially any gene;
- Accelerated generation of lead compounds; and
- Low toxicity due to a natural mechanism of action.

**RXi's RNAi Therapeutic Platform**

**RNAi Compound Design**

Synthetic RNAi compounds are made from a strand or strands of RNA that are manufactured by a nucleic acid synthesizer. The synthesizer is programmed to assemble a strand of RNA of a particular sequence using primarily four nucleotide units (Adenine ("**A**"), Uracil ("**U**"), Cytidine ("**C**") and Guanosine ("**G**")) that match a small segment of the targeted gene. The hallmark of an RNAi compound is that it has a double-stranded region. The compounds can be of various lengths of nucleotide units (nt) and can contain various modifications of the nucleotide units or linkages. The two strands can have overhangs or blunt ends. A single strand can form an RNAi compound by forming a structure referred to as a hairpin.

The length and shape of the compound can affect the activity and hence the potency of the RNAi in cells. The first design of RNAi compounds to be pursued for development as human therapeutics were short, double-stranded RNAs that included at least one overhanging single-stranded region and limited modifications, known as small-interfering RNA, or siRNA, which we also refer to as classic siRNA.

We believe that classic siRNAs have drawbacks that may limit the usefulness of those agents as human therapeutics, and that we may be able to utilize the technologies we have licensed and developed internally to optimize RNAi compounds for use as human therapeutic agents. It is the combination of the length, the nucleotide sequence and the configuration of chemical modifications that are important for effective RNAi therapeutics.

Our internal research leads us to believe that next generation rxRNA compounds offer significant advantages over classic siRNA used by other companies developing RNAi therapeutics, highlighted by the following characteristics:

- Potent RNAi activity in the absence of a delivery vehicle;
- More resistant to nuclease degradation;
- Readily manufactured;
- Potentially more specific for the target gene;
- More reliable at blocking immune side effects than classic siRNA; and
- In the case of sd-rxRNA®, the unique ability to be "self-delivering," without the need for any additional delivery vehicle.

Based on our own research, we have developed a variety of novel siRNA configurations with potential advantages for therapeutic use. The first of these has been termed rxRNAori. This configuration has some similarities to classic siRNA in that it is composed of two short RNA strands. We have found that by using a somewhat longer length (25-29 bp), removing the overhangs and using proprietary chemical modification patterns, we achieve a higher hit rate of very potent (picomolar potency) compounds in a given target sequence. These rxRNAori compounds are modified to increase resistance to nucleases and to prevent off-target effects including induction of an immune response. These novel RNAi compounds are distinct from the siRNA compounds used by many other companies developing RNAi therapeutics in that they are designed specifically for therapeutic use and offer many of the properties that we believe are important to the clinical development of RNAi-based drugs.

The second novel configuration has been called "sd-rxRNA" to indicate its novel "self-delivering" properties, which make additional delivery vehicles unnecessary for efficient cellular uptake and RISC-mediated silencing. A combination of at least three characteristics is required for activity: (1) specific, proprietary chemical modifications; (2) a precise number of chemical modifications; and (3) reduction in oligonucleotide content. Kinetic analyses of fluorescently-labeled compounds demonstrate that efficient cellular internalization is observed within minutes of exposure. These molecules are taken up efficiently and cause target gene silencing in diverse cell types (cell lines and primary cells). This novel class of RNAi compounds may afford a broad opportunity for therapeutic development.

We believe that both chemical modification and formulation of RNAi compounds may be utilized to develop RNA drugs suitable for therapeutic use. The route by which an RNAi therapeutic is brought into contact with the body depends on the intended organ or tissue to be treated. Delivery routes can be simplified into two major categories: (1) local (when a drug is delivered directly to the tissue of interest); and (2) systemic (when a drug accesses the tissue of interest through the circulatory system). Local delivery may avoid some hurdles associated with systemic approaches such as circulation clearance and tissue extravasation (crossing the endothelial barrier from the blood stream). However, the local delivery approach can only be applied to a limited number of organs or tissues (*e.g.*, skin, eye, lung and potentially the central nervous system).

The key to therapeutic success with RNAi lies in delivering intact RNAi compounds to the target tissue and the interior of the target cells. To accomplish this, we have developed a comprehensive platform that includes local and systemic delivery approaches. We work with chemically synthesized RNAi compounds that are optimized for stability and efficacy and combine delivery at the site of action and formulation with delivery agents to achieve optimal delivery to specific target tissues.

**Local Delivery**

sd-rxRNA molecules have unique properties that improve tissue and cell uptake. Delivery of sd-rxRNA by a local route of administration may avoid hurdles associated with systemic approaches such as rapid clearance from the bloodstream and inefficient extravasation (*e.g.*, crossing the endothelial barrier from the blood stream). We have studied sd-rxRNA molecules in a rat model of dermal delivery. Direct application of sd-rxRNA with no additional delivery vehicle to the skin (incision introduced) demonstrates that target gene silencing can be measured after local administration. The dose levels required for these direct-injection methods are small and suitable for clinical development, suggesting that local delivery indications will be very accessible with the sd-rxRNA technology platform. Target tissues that are potentially accessible for local delivery using sd-rxRNA compounds include the skin, the eye, the lung, the central nervous system, mucosal tissues, sites of inflammation and tumors (direct administration).

**Systemic Delivery**

Systemic delivery occurs when a drug accesses the targeted tissue through the circulatory system. In some cases, such as in targeting a treatment to the liver, the optimal route of delivery may be by a systemic route. We have developed a portfolio of systemic delivery solutions utilizing our RNAi therapeutic platforms. One novel approach involves the use of sd-rxRNA compounds. The self-delivering technology introduces properties required for *in vivo* efficacy such as cell and tissue penetration, reduced blood clearance and improved distribution properties. Systemic delivery of these sd-rxRNAs to mice has resulted in gene-specific inhibition in the liver with no additional delivery vehicle required, albeit at high concentrations. A proof-of-concept preclinical study using rxRNA® in conjunction with a standard lipid-based delivery vehicle has enabled us to demonstrate gene-specific inhibition in liver at much lower doses in a mouse model after intravenous, systemic delivery. While delivery of RNAi to the liver may be critical for the treatment of many diseases, additional target tissues that are potentially accessible using rxRNA® compounds by systemic delivery include kidney, fat, heart, lung, bone marrow, sites of inflammation, tumors and vascular endothelium.

**Intellectual Property**

We protect our proprietary information by means of United States and foreign patents, trademarks and copyrights. In addition, we rely upon trade secret protection and contractual arrangements to protect certain of our proprietary information and products. We have pending patent applications that relate to potential drug targets, compounds we are developing to modulate those targets (described throughout herein as rxRNA), methods of making or using those compounds and proprietary elements of our drug discovery platform.

Much of our technology and many of our processes depend upon the knowledge, experience and skills of key scientific and technical personnel. To protect our rights to our proprietary know-how and technology, we require all employees, as well as our consultants and advisors when feasible, to enter into confidentiality agreements that require disclosure and assignment to us of ideas, developments, discoveries and inventions made by these employees, consultants and advisors in the course of their service to us, and we vigorously defend that position with partners, as well as with employees who leave the Company.

We have also obtained rights to various patents and patent applications under licenses with third parties, which require us to pay royalties or milestone payments, or both. The degree of patent protection for biotechnology products and processes, including ours, remains uncertain, both in the United States and in other important markets, because the scope of protection depends on decisions of patent offices, courts and lawmakers

in these countries. There is no certainty that our existing patents or others, if obtained, will afford us substantial protection or commercial benefit. Similarly, there is no assurance that our pending patent applications or patent applications licensed from third parties will ultimately be granted as patents or that those patents that have been issued or are issued in the future will stand if they are challenged in court. We assess our license agreements on an ongoing basis, and may from time to time terminate licenses to technology that we do not intend to employ in our immunotherapy or RNAi technology platforms, or in our product discovery or development activities.

***Patents and Patent Applications***

We are actively prosecuting eleven patent families covering our rxRNA compounds and technologies, including RXI-109. Additionally, as part of our acquisition of the OPKO RNAi-related assets in March 2013, we acquired rights to a number of patents and patent applications. A summary of these patents and patent applications is set forth below in the following table.

| | RXi Platform | | OPKO Platform | |
|---|---|---|---|---|
| | Pending Applications | Issued Patents | Pending Applications | Issued Patents |
| United States | 13 | 1 | 6 | 17 |
| Canada | 5 | 0 | 3 | 1 |
| Europe | 5 | 0 | 5 | 21 |
| Japan | 4 | 0 | 4 | 1 |
| Other Markets | 8 | 0 | 2 | 1 |

***RXi RNAi Platform Patent and Patent Applications***

Our portfolio includes one issued patent, claiming CTGF-targeting sd-rxRNAs for the treatment of fibrotic disorders. This patent (U.S. Patent Number 8,664,189) is scheduled to expire in 2029. The patent applications encompass what we believe to be important new RNAi compounds and their use as therapeutics, chemical modifications of RNAi compounds that improve the compounds' suitability for therapeutic uses (including delivery) and compounds directed to specific targets (*i.e.*, that address specific disease states). Any patents that may issue from these pending patent applications will, if issued, be set to expire between 2028 and 2031, not including any patent term extensions that may be afforded under the Federal Food, Drug and Cosmetic Act (and the equivalent provisions in foreign jurisdictions) for any delays incurred during the regulatory approval process relating to human drug products (or processes for making or using human drug products).

***OPKO RNAi Platform Patents and Patent Applications***

The OPKO RNAi patents and patent applications encompass 12 patent families and includes 41 issued patents. The patents and patent applications cover RNAi compounds and their use as therapeutics and compounds directed to specific targets (*i.e*., that address specific disease states). The patents and any patents that may issue from the pending applications will be set to expire between 2022 and 2030, not including any patent term extensions that may be afforded under the Federal Food, Drug and Cosmetic Act (and the equivalent provisions in foreign jurisdictions) for any delays incurred during the regulatory approval process relating to human drug products (or processes for making or using human drug products).

***License Agreements***

We have secured exclusive and non-exclusive rights to develop RNAi therapeutics by licensing key RNAi technologies and patent rights from third parties. These rights relate to chemistry and configuration of RNAi compounds, delivery technologies of RNAi compounds to cells and therapeutic targets. As we continue to develop our own proprietary compounds, we continue to evaluate both our in-licensed portfolio as well as the field for new technologies that could be in-licensed to further enhance our intellectual property portfolio and unique position in the RNAi space.

*University of Massachusetts Medical School*. We hold a non-exclusive license from the University of Massachusetts Medical School ("**UMMS**"). This license grants to us rights under certain UMMS patent applications to make, use and sell products related to applications of RNAi technologies in particular fields, including HCMV and retinitis, amyotrophic lateral sclerosis, known as "ALS" or "Lou Gehrig's Disease," diabetes and obesity. Throughout the term of the license, we must pay UMMS an annual maintenance fee of $15,000. We also will be required to pay to UMMS customary royalties of up to 10% of: (i) any future net sales of licensed products; (ii) income received from any sublicensees under this license; and (iii) net sales of commercial clinical laboratory services, subject to a minimum royalty of $50,000 beginning in 2016. We also agreed to pay expenses incurred by UMMS in prosecuting and maintaining the licensed patents.

The UMMS license was effective on April 15, 2003, and will remain in effect until the expiration of all issued patents within the "patent rights" (as defined), unless earlier terminated in accordance with the provisions of the license. In the event that either party commits a material breach of its obligations under the UMMS license and fails to cure that breach within 60 days after receiving written notice thereof, the other party may terminate the UMMS license immediately upon written notice to the party in breach.

The UMMS license may be amended, supplemented, or otherwise modified only by signed written agreement of the parties.

*Advirna.* We have entered into agreements with Advirna, LLC pursuant to which Advirna assigned to us its existing patent and technology rights related to sd-rxRNA® technology in exchange for our agreement to pay Advirna an annual maintenance fee of $100,000 and a one-time milestone payment of $350,000 upon the issuance of the first patent with valid claims covering the assigned technology, which occurred on March 4, 2014. Additionally, we will be required to pay a 1% royalty to Advirna on any licensing revenue received by us with respect to future licensing of the assigned Advirna patent and technology rights. We also granted back to Advirna a license under the assigned patent and technology rights for fields of use outside human therapeutics and diagnostics, and issued to Advirna, upon the completion of the spin-off transaction in 2012, 1,394,997 shares of common stock.

Our rights under the Advirna agreement will expire upon the later of: (i) the expiration of the last-to-expire of the "patent rights" (as defined) included in the Advirna agreement; or (ii) the abandonment of the last-to-be abandoned of such patents, unless earlier terminated in accordance with the provisions of the agreement.

We may terminate the Advirna agreement at any time upon 90 days' written notice to Advirna, and Advirna may terminate the agreement upon 90 days' prior written notice in the event that we cease using commercially reasonable efforts to research, develop, license or otherwise commercialize the patent rights or "royalty-bearing products" (as defined), provided that we may refute such claim within such 90-day period by showing budgeted expenditures for the research, development, licensing or other commercialization consistent with other technologies of similar stage of development and commercial potential as the patent rights or royalty-bearing products. Further, either party at any time may provide to the other party written notice of a material breach of the agreement. If the other party fails to cure the identified breach within 90 days after the date of the notice, the aggrieved party may terminate the agreement by written notice to the party in breach.

The Advirna agreement may only be altered or supplemented by written mutual agreement by the parties.

*OPKO*. In March 2013, we acquired from OPKO substantially all of its RNAi-related assets, which included patents and patent applications, licenses, clinical and preclinical data and other related assets. In exchange for the assets that we purchased from OPKO, we issued to OPKO 1,666,666 shares of our common stock and agreed to pay, if applicable: (i) up to $50,000,000 in development and commercialization milestones for the successful development and commercialization of each "Qualified Drug" (as defined in the Asset Purchase Agreement with OPKO) (collectively, the "Milestone Payments"); and (ii) royalty payments equal to: (a) a mid-single-digit percentage of "Net Sales" (as defined in the Asset Purchase Agreement) with respect to each Qualified Drug sold for an ophthalmologic use during the applicable "Royalty Period" (as defined in the Asset Purchase Agreement);

and (b) a low-single-digit percentage of Net Sales with respect to each Qualified Drug sold for a non-ophthalmologic use during the applicable Royalty Period (collectively, the "Royalty Payments"). The assets purchased from OPKO are at an early stage of development, and we have established a discovery program to identify potential sd-rxRNA lead compounds and targets from these acquired RNAi-related assets.

**Research and Development**

To date, our research programs have focused on identifying product candidates and optimizing the delivery method and technology necessary to make RNAi compounds available by local or systemic administration, as appropriate, for diseases for which we intend to develop an RNAi therapeutic. Since we commenced operations, research and development has comprised a significant proportion of our total operating expenses and is expected to comprise the majority of our spending for the foreseeable future.

There are risks in any new field of drug discovery that preclude certainty regarding the successful development of a product. We cannot reasonably estimate or know the nature, timing and costs of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence from, any product candidate.

For more information on our research and development activity, see Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations—Research and Development" of this annual report on Form 10-K.

**Competition**

We believe numerous companies are investigating or plan to investigate a variety of proposed anti-scarring therapies in clinical trials. The companies include large and small pharmaceutical, chemical and biotechnology companies, as well as universities, government agencies and other private and public research organizations. Such companies include CoDa Therapeutics, Inc., Sirnaomics, Inc., FirstString Research, Inc., Merz Pharmaceuticals, LLC, Capstone Therapeutics, Halscion, Inc., Garnet Bio Therapeutics, Inc., AkPharma Inc., Promedior, Inc., Kissei Pharmaceutical Co., Ltd., Eyegene, Derma Sciences, Inc., Healthpoint Biotherapeutics, FibroGen, Inc. and Pharmaxon. In particular, Excaliard Pharmaceuticals, Inc., which has been acquired by Pfizer, Inc., has successfully advanced an anti-CTGF antisense oligonucleotide through several Phase 1 and Phase 2 trials and has demonstrated improved scar outcome over placebo.

We believe that other companies working in the RNAi area, generally, include Alnylam Pharmaceuticals, Inc., Marina Biotech, Inc., Tacere Therapeutics, Inc., Benitec Limited, Silence Therapeutics plc, Quark Pharmaceuticals, Inc., Rosetta Genomics Ltd., Lorus Therapeutics, Inc., Tekmira Pharmaceuticals Corporation, Arrowhead Research Corporation, Regulus Therapeutics Inc. and Santaris, as well as a number of large pharmaceutical companies. Many other companies are pursuing non-RNAi-based therapies for one or more fibrotic disease indications, including ocular scarring or other indications that we may seek to pursue. See Item 1A, "Risk Factors—Risks Relating to RXi's Business and Industry".

**Government Regulation**

The United States and other developed countries extensively regulate the preclinical and clinical testing, manufacturing, labeling, storage, record-keeping, advertising, promotion, export, marketing and distribution of drugs and biologic products. The FDA regulates pharmaceutical and biologic products under the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act and other federal statutes and regulations.

To obtain approval of our future product candidates from the FDA, we must, among other requirements, submit data supporting safety and efficacy for the intended indication as well as detailed information on the manufacture and composition of the product candidate. In most cases, this will require extensive laboratory tests

and preclinical and clinical trials. The collection of these data, as well as the preparation of applications for review by the FDA involve significant time and expense. The FDA also may require post-marketing testing to monitor the safety and efficacy of approved products or place conditions on any approvals that could restrict the therapeutic claims and commercial applications of these products. Regulatory authorities may withdraw product approvals if we fail to comply with regulatory standards or if we encounter problems at any time following initial marketing of our products.

The first stage of the FDA approval process for a new biologic or drug involves completion of preclinical studies and the submission of the results of these studies to the FDA. These data, together with proposed clinical protocols, manufacturing information, analytical data and other information submitted to the FDA, in an investigational new drug ("**IND**") application, must become effective before human clinical trials may commence. Preclinical studies generally involve FDA regulated laboratory evaluation of product characteristics and animal studies to assess the efficacy and safety of the product candidate.

After the IND becomes effective, a company may commence human clinical trials. These are typically conducted in three sequential phases, but the phases may overlap. Phase 1 trials consist of testing the product candidate in a small number of patients or healthy volunteers, primarily for safety at one or more doses. Phase 2 trials, in addition to safety, evaluate the efficacy of the product candidate in a patient population somewhat larger than Phase 1 trials. Phase 3 trials typically involve additional testing for safety and clinical efficacy in an expanded population at multiple test sites. A company must submit to the FDA a clinical protocol, accompanied by the approval of the Institutional Review Boards ("**IRB**") at the institutions participating in the trials, prior to commencement of each clinical trial.

To obtain FDA marketing authorization, a company must submit to the FDA the results of the preclinical and clinical testing, together with, among other things, detailed information on the manufacture and composition of the product candidate, in the form of a new drug application (an "**NDA**"), or, in the case of a biologic, a biologics license application (a "**BLA**").

The amount of time taken by the FDA for approval of an NDA or BLA will depend upon a number of factors, including whether the product candidate has received priority review, the quality of the submission and studies presented, the potential contribution that the compound will make in improving the treatment of the disease in question and the workload at the FDA.

The FDA may, in some cases, confer upon an investigational product the status of a fast track product. A fast track product is defined as a new drug or biologic intended for the treatment of a serious or life threatening condition that demonstrates the potential to address unmet medical needs for this condition. The FDA can base approval of an NDA or BLA for a fast track product on an effect on a surrogate endpoint, or on another endpoint that is reasonably likely to predict clinical benefit. If a preliminary review of clinical data suggests that a fast track product may be effective, the FDA may initiate review of entire sections of a marketing application for a fast track product before the sponsor completes the application.

We anticipate that our products will be manufactured by our strategic partners, licensees or other third parties. Before approving an NDA or BLA, the FDA will inspect the facilities at which the product is manufactured and will not approve the product unless the manufacturing facilities are in compliance with the FDA's current good manufacturing practices ("**cGMP**"), which are regulations that govern the manufacture, holding and distribution of a product. Manufacturers of biologics also must comply with the FDA's general biological product standards. Our manufacturers also will be subject to regulation under the Occupational Safety and Health Act, the Nuclear Energy and Radiation Control Act, the Toxic Substance Control Act and the Resource Conservation and Recovery Act and other applicable environmental statutes. Following approval, the FDA periodically inspects drug and biologic manufacturing facilities to ensure continued compliance with the good manufacturing practices regulations. Our manufacturers will have to continue to comply with those requirements. Failure to comply with these requirements subjects the manufacturer to possible legal or regulatory

action, such as suspension of manufacturing or recall or seizure of product. Adverse patient experiences with the product must be reported to the FDA and could result in the imposition of marketing restrictions through labeling changes or market removal. Product approvals may be withdrawn if compliance with regulatory requirements is not maintained or if problems concerning safety or efficacy of the product occur following approval.

The labeling, advertising, promotion, marketing and distribution of a drug or biologic product also must be in compliance with FDA and Federal Trade Commission requirements which include, among others, standards and regulations for off-label promotion, industry sponsored scientific and educational activities, promotional activities involving the internet, and direct-to-consumer advertising. We also will be subject to a variety of federal, state and local regulations relating to the use, handling, storage and disposal of hazardous materials, including chemicals and radioactive and biological materials. In addition, we will be subject to various laws and regulations governing laboratory practices and the experimental use of animals. In each of these areas, as above, the FDA has broad regulatory and enforcement powers, including the ability to levy fines and civil penalties, suspend or delay issuance of product approvals, seize or recall products and deny or withdraw approvals.

We will also be subject to a variety of regulations governing clinical trials and sales of our products outside the United States. Whether or not FDA approval has been obtained, approval of a product candidate by the comparable regulatory authorities of foreign countries and regions must be obtained prior to the commencement of marketing the product in those countries. The approval process varies from one regulatory authority to another and the time may be longer or shorter than that required for FDA approval. In the European Union, Canada and Australia, regulatory requirements and approval processes are similar, in principle, to those in the United States.

**Environmental Compliance**

Our research and development activities involve the controlled use of potentially harmful biological materials as well as hazardous materials, chemicals and various radioactive compounds. We are subject to federal, state and local laws and regulations governing the use, storage, handling and disposal of these materials and specific waste products. We are also subject to numerous environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. The cost of compliance with these laws and regulations could be significant and may adversely affect capital expenditures to the extent we are required to procure expensive capital equipment to meet regulatory requirements.

**Human Resources**

As of March 15, 2014, we had twelve full-time employees, eight of whom were engaged in research and development, and four of whom were engaged in management, administration and finance. None of our employees is represented by a labor union or covered by a collective bargaining agreement, nor have we experienced any work stoppages.

## ITEM 1A. *RISK FACTORS*

**Risks Relating to Our Business and Industry**

***We are dependent on the success of our lead drug candidate, which may not receive regulatory approval or be successfully commercialized.***

RXI-109, our first RNAi-based product candidate, targets CTGF and may have a variety of medical applications. We began Phase 1 clinical trials for RXI-109 in June 2012 and began Phase 2 clinical trials for RXI-109 in November 2013. The FDA did not require additional information prior to the commencement of our ongoing Phase 2 clinical trial, but may require additional information from the Company regarding our current or planned at any time, and such information may be costly to provide or cause potentially significant delays in development. There is no assurance that we will be able to successfully develop RXI-109 or any other product candidate.

We have no commercial products and currently generate no revenue from commercial sales or collaborations and may never be able to develop marketable products. The FDA or similar foreign governmental agencies must approve our products in development before they can be marketed. The process for obtaining FDA approval is both time-consuming and costly, with no certainty of a successful outcome. Before obtaining regulatory approval for the sale of any drug candidate, we must conduct extensive preclinical tests and successful clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Although the results of our Phase 1 clinical trials of RXI-109 are promising, additional clinical trials will be required to establish the safety and efficacy of RXI-109. We have not yet shown safety or efficacy in humans for any of our other RNAi-based product candidates. A failure of any preclinical study or clinical trial can occur at any stage of testing. The results of preclinical and initial clinical testing of these products may not necessarily indicate the results that will be obtained from later or more extensive testing. Additionally, any observations made with respect to blinded clinical data are inherently uncertain as we cannot know which set of data come from patients treated with an active drug versus the placebo vehicle. Investors are cautioned not to rely on observations coming from blinded data and not to rely on initial clinical trial results as necessarily indicative of results that will be obtained in subsequent clinical trials.

***A number of different factors could prevent us from obtaining regulatory approval or commercializing our product candidates on a timely basis, or at all.***

We, the FDA or other applicable regulatory authorities or an IRB may suspend clinical trials of a drug candidate at any time for various reasons, including if we or they believe the subjects or patients participating in such trials are being exposed to unacceptable health risks. Among other reasons, adverse side effects of a drug candidate on subjects or patients in a clinical trial could result in the FDA or other regulatory authorities suspending or terminating the trial and refusing to approve a particular drug candidate for any or all indications of use.

Clinical trials of a new drug candidate require the enrollment of a sufficient number of patients, including patients who are suffering from the disease or condition the drug candidate is intended to treat and who meet other eligibility criteria. Rates of patient enrollment are affected by many factors, and delays in patient enrollment can result in increased costs and longer development times.

Clinical trials also require the review and oversight of IRBs, which approve and continually review clinical investigations and protect the rights and welfare of human subjects. An inability or delay in obtaining IRB approval could prevent or delay the initiation and completion of clinical trials, and the FDA may decide not to consider any data or information derived from a clinical investigation not subject to initial and continuing IRB review and approval.

Numerous factors could affect the timing, cost or outcome of our drug development efforts, including the following:

- Delays in filing or acceptance of initial drug applications for our product candidates;
- Difficulty in securing centers to conduct clinical trials;
- Conditions imposed on us by the FDA or comparable foreign authorities regarding the scope or design of our clinical trials;
- Problems in engaging IRBs to oversee trials or problems in obtaining or maintaining IRB approval of studies;
- Difficulty in enrolling patients in conformity with required protocols or projected timelines;
- Third-party contractors failing to comply with regulatory requirements or to meet their contractual obligations to us in a timely manner;
- Our drug candidates having unexpected and different chemical and pharmacological properties in humans than in laboratory testing and interacting with human biological systems in unforeseen, ineffective or harmful ways;

- The need to suspend or terminate clinical trials if the participants are being exposed to unacceptable health risks;
- Insufficient or inadequate supply or quality of our drug candidates or other necessary materials necessary to conduct our clinical trials;
- Effects of our drug candidates not being the desired effects or including undesirable side effects or the drug candidates having other unexpected characteristics;
- The cost of our clinical trials being greater than we anticipate;
- Negative or inconclusive results from our clinical trials or the clinical trials of others for similar drug candidates or inability to generate statistically significant data confirming the efficacy of the product being tested;
- Changes in the FDA's requirements for testing during the course of that testing;
- Reallocation of our limited financial and other resources to other clinical programs; and
- Adverse results obtained by other companies developing similar drugs.

It is possible that none of the product candidates that we may attempt to develop will obtain the appropriate regulatory approvals necessary to begin selling them or that any regulatory approval to market a product may be subject to limitations on the indicated uses for which we may market the product. The time required to obtain FDA and other approvals is unpredictable, but often can take years following the commencement of clinical trials, depending upon the complexity of the drug candidate. Any analysis we perform of data from clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit or prevent regulatory approval. Any delay or failure in obtaining required approvals could have a material adverse effect on our ability to generate revenue from the particular drug candidate.

We also are subject to numerous foreign regulatory requirements governing the conduct of clinical trials, manufacturing and marketing authorization, pricing and third-party reimbursement. The foreign regulatory approval process includes all of the risks associated with the FDA approval described above as well as risks attributable to the satisfaction of local regulations in foreign jurisdictions. Approval by the FDA does not assure approval by regulatory authorities outside of the United States.

***The approach we are taking to discover and develop novel therapeutics using RNAi is unproven and may never lead to marketable products.***

RNA interference is a relatively new scientific discovery. Our RNAi technologies have been subject to only limited clinical testing. To date, no company has received regulatory approval to market therapeutics utilizing RNAi, and a number of clinical trials of RNAi technologies by other companies have been unsuccessful. The scientific evidence to support the feasibility of developing drugs based on these discoveries is both preliminary and limited. To successfully develop RNAi-based products, we must resolve a number of issues, including stabilizing the RNAi material and delivering it into target cells in the human body. We may spend large amounts of money trying to resolve these issues and may never succeed in doing so. In addition, any compounds that we develop may not demonstrate in patients the chemical and pharmacological properties ascribed to them in laboratory studies, and they may interact with human biological systems in unforeseen, ineffective or even harmful ways.

***The FDA could impose a unique regulatory regime for RNAi therapeutics.***

The substances we intend to develop may represent a new class of drug, and the FDA has not yet established any definitive policies, practices or guidelines in relation to these drugs. While we expect any product candidates that we develop will be regulated as a new drug under the Federal Food, Drug, and Cosmetic Act, the FDA could decide to regulate them or other products we may develop as biologics under the Public Health Service Act. The

lack of policies, practices or guidelines may hinder or slow review by the FDA of any regulatory filings that we may submit. Moreover, the FDA may respond to these submissions by defining requirements that we may not have anticipated.

***Even if we receive regulatory approval to market our product candidates, our product candidates may not be accepted commercially, which may prevent us from becoming profitable.***

The RNAi product candidates that we are developing are based on new technologies and therapeutic approaches. RNAi products may be more expensive to manufacture than traditional small molecule drugs, which may make them more costly than competing small molecule drugs. Additionally, RNAi products do not readily cross the so-called blood brain barrier and, for various applications, are likely to require injection or implantation, which will make them less convenient to administer than drugs administered orally. Key participants in the pharmaceutical marketplace, such as physicians, medical professionals working in large reference laboratories, public health laboratories and hospitals, third-party payors and consumers may not accept products intended to improve therapeutic results based on RNAi technology. As a result, it may be more difficult for us to convince the medical community and third-party payors to accept and use our products or to provide favorable reimbursement. If medical professionals working with large reference laboratories, public health laboratories and hospitals choose not to adopt and use our RNAi technology, our products may not achieve broader market acceptance.

Additionally, although we expect that we will have intellectual property protection for our technology, certain governments may elect to deny patent protection for drugs targeting diseases with high unmet medical need (e.g., as in the case of HIV) and allow in their country internationally unauthorized generic competition. If this were to happen, our commercial prospects for developing any such drugs would be substantially diminished in these countries.

***We are subject to significant competition and may not be able to compete successfully.***

We believe numerous companies are investigating or plan to investigate a variety of proposed anti-scarring therapies in clinical trials. The companies include large and small pharmaceutical, chemical and biotechnology companies, as well as universities, government agencies and other private and public research organizations. Such companies include CoDa Therapeutics, Inc., Sirnaomics, Inc., FirstString Research, Inc., Merz Pharmaceuticals, LLC, Capstone Therapeutics, Halscion, Inc., Garnet Bio Therapeutics, Inc., AkPharma Inc., Promedior, Inc., Kissei Pharmaceutical Co., Ltd., Eyegene, Derma Sciences, Inc., Healthpoint Biotherapeutics, FibroGen, Inc. and Pharmaxon. In particular, Excaliard Pharmaceuticals, Inc., which has been acquired by Pfizer, Inc., has successfully advanced an anti-CTGF antisense oligonucleotide through several Phase 1 and Phase 2 trials and has demonstrated improved scar outcome over placebo.

We believe other companies working in the RNAi area, generally, include Alnylam Pharmaceuticals, Inc., Marina Biotech, Inc., Tacere Therapeutics, Inc., Benitec Limited, Silence Therapeutics plc, Quark Pharmaceuticals, Inc., Rosetta Genomics Ltd., Lorus Therapeutics, Inc., Tekmira Pharmaceuticals Corporation, Arrowhead Research Corporation, Regulus Therapeutics Inc. and Santaris, as well as a number of large pharmaceutical companies. Many other companies are pursuing non-RNAi-based therapies for one or more fibrotic disease indications, including ocular scarring or other indications that we may seek to pursue.

Most of these competitors have substantially greater research and development capabilities and financial, scientific, technical, manufacturing, marketing, distribution and other resources than we have, and we may not be able to successfully compete with them. In addition, even if we are successful in developing our product candidates, in order to compete successfully we may need to be first to market or to demonstrate that our RNAi based products are superior to therapies based on different technologies. A number of our competitors have already commenced clinical testing of RNAi product candidates and may be more advanced than we are in the process of developing products. If we are not first to market or are unable to demonstrate superiority, any products for which we are able to obtain approval may not be successful.

***We are dependent on technologies we license, and if we lose the right to license such technologies or fail to license new technologies in the future, our ability to develop new products would be harmed.***

Many patents in the RNAi field have already been exclusively licensed to third parties, including our competitors. If any of our existing licenses are terminated, the development of the products contemplated by the licenses could be delayed or terminated and we may not be able to negotiate additional licenses on acceptable terms, if at all, which would have a material adverse effect on our business.

***We may be unable to protect our intellectual property rights licensed from other parties; our intellectual property rights may be inadequate to prevent third parties from using our technologies or developing competing products; and we may need to license additional intellectual property from others.***

Therapeutic applications of gene silencing technologies, delivery methods and other technologies that we license from third parties are claimed in a number of pending patent applications, but there is no assurance that these applications will result in any issued patents or that those patents would withstand possible legal challenges or protect our technologies from competition. The United States Patent and Trademark Office and patent granting authorities in other countries have upheld stringent standards for the RNAi patents that have been prosecuted so far. Consequently, pending patents that we have licensed and those that we own may continue to experience long and difficult prosecution challenges and may ultimately issue with much narrower claims than those in the pending applications. Third parties may hold or seek to obtain additional patents that could make it more difficult or impossible for us to develop products based on RNAi technology without obtaining a license to such patents, which licenses may not be available on attractive terms, or at all.

In addition, others may challenge the patents or patent applications that we currently license or may license in the future or that we own and, as a result, these patents could be narrowed, invalidated or rendered unenforceable, which would negatively affect our ability to exclude others from using RNAi technologies described in these patents. There is no assurance that these patent or other pending applications or issued patents we license or that we own will withstand possible legal challenges. Moreover, the laws of some foreign countries may not protect our proprietary rights to the same extent as do the laws of the United States. Any patents issued to us or our licensors may not provide us with any competitive advantages, and there is no assurance that the patents of others will not have an adverse effect on our ability to do business or to continue to use our technologies freely. Our efforts to enforce and maintain our intellectual property rights may not be successful and may result in substantial costs and diversion of management time. Even if our rights are valid, enforceable and broad in scope, competitors may develop products based on technology that is not covered by our licenses or patents or patent application that we own.

There is no guarantee that future licenses will be available from third parties for our product candidates on timely or satisfactory terms, or at all. To the extent that we are required and are able to obtain multiple licenses from third parties to develop or commercialize a product candidate, the aggregate licensing fees and milestones and royalty payments made to these parties may materially reduce our economic returns or even cause us to abandon development or commercialization of a product candidate.

Our success depends upon our ability to obtain and maintain intellectual property protection for our products and technologies.

The applications based on RNAi technologies claim many different methods, compositions and processes relating to the discovery, development, delivery and commercialization of RNAi therapeutics. Because this field is so new, very few of these patent applications have been fully processed by government patent offices around the world, and there is a great deal of uncertainty about which patents will issue, when, to whom and with what claims. Although we are not aware of any blocking patents or other proprietary rights, it is likely that there will be significant litigation and other proceedings, such as interference and opposition proceedings in various patent offices, relating to patent rights in the RNAi field. It is possible that we may become a party to such proceedings.

***We may not be able to obtain sufficient financing and may not be able to develop our product candidates.***

In March 2013, we raised $16.4 million in gross proceeds from an offering of our common stock (the "**Offering**"). With the proceeds from the Offering, we believe that we have sufficient working capital to fund our currently planned operations, including the planned Phase 2 program for RXI-109, into the second quarter of fiscal 2015. However, in the future, we may need to incur debt or issue equity in order to fund our planned expenditures as well as to make acquisitions and other investments. We cannot assure you that debt or equity financing will be available to us on acceptable terms or at all. If we cannot, or are limited in the ability to, incur debt, issue equity or enter into strategic collaborations, we may be unable to fund the discovery and development of our product candidates, address gaps in our product offerings or improve our technology.

We anticipate that we will need to raise substantial amounts of money to fund a variety of future activities integral to the development of our business, which may include but are not limited to the following:

- To conduct research and development to successfully develop our RNAi technologies;
- To obtain regulatory approval for our products;
- To file and prosecute patent applications and to defend and assess patents to protect our technologies;
- To retain qualified employees, particularly in light of intense competition for qualified scientists;
- To manufacture products ourselves or through third parties;
- To market our products, either through building our own sales and distribution capabilities or relying on third parties; and
- To acquire new technologies, licenses or products.

We cannot assure you that any needed financing will be available to us on acceptable terms or at all. If we cannot obtain additional financing in the future, our operations may be restricted and we may ultimately be unable to continue to develop and potentially commercialize our product candidates.

***Future financing may be obtained through, and future development efforts may be paid for by, the issuance of debt or equity, which may have an adverse effect on our stockholders or may otherwise adversely affect our business.***

If we raise funds through the issuance of debt or equity, any debt securities or preferred stock issued will have rights, preferences and privileges senior to those of holders of our common stock in the event of a liquidation. In such event, there is a possibility that once all senior claims are settled, there may be no assets remaining to pay out to the holders of common stock. In addition, if we raise funds through the issuance of additional equity, whether through private placements or public offerings, such an issuance would dilute your ownership in us.

The terms of debt securities may also impose restrictions on our operations, which may include limiting our ability to incur additional indebtedness, to pay dividends on or repurchase our capital stock, or to make certain acquisitions or investments. In addition, we may be subject to covenants requiring us to satisfy certain financial tests and ratios, and our ability to satisfy such covenants may be affected by events outside of our control.

***We expect to continue to incur significant research and development expenses, which may make it difficult for us to attain profitability, and may lead to uncertainty as to our ability to continue as a going concern.***

We expend substantial funds to develop our RNAi technologies, and additional substantial funds will be required for further research and development, including preclinical testing and clinical trials of any product candidates, and to manufacture and market any products that are approved for commercial sale. Because the

successful development of our products is uncertain, we are unable to precisely estimate the actual funds we will require to develop and potentially commercialize them. In addition, we may not be able to generate enough revenue, even if we are able to commercialize any of our product candidates, to become profitable.

If we are unable to achieve or sustain profitability or to secure additional financing, we may not be able to meet our obligations as they come due, raising substantial doubts as to our ability to continue as a going concern. Any such inability to continue as a going concern may result in our common stock holders losing their entire investment. There is no guarantee that we will become profitable or secure additional financing. Our financial statements contemplate that we will continue as a going concern and do not contain any adjustments that might result if we were unable to continue as a going concern. Changes in our operating plans, our existing and anticipated working capital needs, the acceleration or modification of our expansion plans, increased expenses, potential acquisitions or other events will all affect our ability to continue as a going concern.

***We will rely upon third parties for the manufacture of our clinical product candidates.***

We do not have the facilities or expertise to manufacture supplies of any of our potential product candidates for clinical trials. Accordingly, we will be dependent upon contract manufacturers for these supplies. We currently obtain supplies for RXI-109 from a single supplier, Agilent Technologies, Nucleic Acid Solutions Division. If for any reason we are unable to obtain RXI-109 from this supplier, we would have to seek to obtain it from another major oligonucleotide manufacturer. There is no assurance that we will be able to timely secure needed supply arrangements on satisfactory terms, or at all. Our failure to secure these arrangements as needed could have a material adverse effect on our ability to complete the development of our product candidates or, if we obtain regulatory approval for our product candidates, to commercialize them.

***We may not be able to establish or maintain the third-party relationships that are necessary to develop or potentially commercialize some or all of our product candidates.***

We expect to depend on collaborators, partners, licensees, clinical research organizations and other third parties to support our discovery efforts, to formulate product candidates, to manufacture our product candidates and to conduct clinical trials for some or all of our product candidates. We cannot guarantee that we will be able to successfully negotiate agreements for or maintain relationships with collaborators, partners, licensees, clinical investigators, vendors and other third parties on favorable terms, if at all. Our ability to successfully negotiate such agreements will depend on, among other things, potential partners' evaluation of the superiority of our technology over competing technologies, the quality of the preclinical and clinical data that we have generated and the perceived risks specific to developing our product candidates. If we are unable to obtain or maintain these agreements, we may not be able to clinically develop, formulate, manufacture, obtain regulatory approvals for or commercialize our product candidates. We cannot necessarily control the amount or timing of resources that our contract partners will devote to our research and development programs, product candidates or potential product candidates, and we cannot guarantee that these parties will fulfill their obligations to us under these arrangements in a timely fashion. For example, Ethicor may never achieve significant profits, if any, from its distribution of RXI-109 pursuant to our distribution agreement, and we may receive limited or no revenue under the profit-sharing provisions of our agreement. We may not be able to readily terminate any such agreements with contract partners even if such contract partners do not fulfill their obligations to us.

***We are subject to potential liabilities from clinical testing and future product liability claims.***

If any of our future products are alleged to be defective, they may expose us to claims for personal injury by patients in clinical trials of our products or as a result of our distribution agreement with Ethicor. If our products are approved by the FDA, users may claim that such products caused unintended adverse effects. We will seek to obtain clinical trial insurance for clinical trials that we conduct, as well as liability insurance for any products that we market. There is no assurance that we will be able to obtain insurance in the amounts we seek, or at all. We anticipate that licensees who develop our products will carry liability insurance covering the clinical testing and

marketing of those products. There is no assurance, however, that any insurance maintained by us or our licensees will prove adequate in the event of a claim against us. Even if claims asserted against us are unsuccessful, they may divert management's attention from our operations and we may have to incur substantial costs to defend such claims.

***Any drugs we develop may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which could have a material adverse effect on our business.***

If approved, we intend to sell our products to physicians, plastic surgeons and dermatologists, as well as hospitals, oncologists and clinics that receive reimbursement for the healthcare services they provide to their patients from third-party payors, such as Medicare, Medicaid and other domestic and international government programs, private insurance plans and managed care programs. Most third-party payors may deny reimbursement if they determine that a medical product was not used in accordance with cost-effective treatment methods, as determined by the third-party payor, was used for an unapproved indication or if they believe the cost of the product outweighs its benefits. Third-party payors also may refuse to reimburse for experimental procedures and devices. Furthermore, because our programs are still in development, we are unable at this time to determine their cost-effectiveness and the level or method of reimbursement for them. Increasingly, the third-party payors who reimburse patients are requiring that drug companies provide them with predetermined discounts from list prices, and are challenging the prices charged for medical products. If the price we are able to charge for any products we develop is inadequate in light of our development and other costs, our profitability could be adversely affected.

We currently expect that any drugs we develop may need to be administered under the supervision of a physician. Under currently applicable law, drugs that are not usually self-administered may be eligible for coverage by the Medicare program if:

- They are "incidental" to a physician's services;
- They are "reasonable and necessary" for the diagnosis or treatment of the illness or injury for which they are administered according to accepted standard of medical practice;
- They are not excluded as immunizations; and
- They have been approved by the FDA.

Insurers may refuse to provide insurance coverage for newly approved drugs, or insurance coverage may be delayed or be more limited than the purpose for which the drugs are approved by the FDA. Moreover, eligibility for insurance coverage does not imply that any drug will be reimbursed in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim payments for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement may be based on payments for other services and may reflect budgetary constraints or imperfections in Medicare data. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates. Our inability to promptly obtain coverage and profitable reimbursement rates from both government-funded and private payors for new drugs that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to develop products and our overall financial condition.

Additionally, third-party payors are increasingly attempting to contain healthcare costs by limiting both coverage and the level of reimbursement for medical products and services. Levels of reimbursement may decrease in the future, and future legislation, regulation or reimbursement policies of third-party payors may adversely affect the demand for and price levels of our products. If our customers are not reimbursed for our products, they may reduce or discontinue purchases of our products, which could have a material adverse effect on our business, financial condition and results of operations.

Comprehensive healthcare reform legislation, which became law in 2010, could adversely affect our business and financial condition. Among other provisions, the legislation provides that a "biosimilar" product may be approved by the FDA on the basis of analytical tests and certain clinical studies demonstrating that such product is highly similar to an existing, approved product and that switching between an existing product and the biosimilar product will not result in diminished safety or efficacy. This abbreviated regulatory approval process may result in increased competition if we are able to bring a product to market. The legislation also includes more stringent compliance programs for companies in various sectors of the life sciences industry with which we may need to comply and enhanced penalties for non-compliance with the new healthcare regulations. Complying with new regulations may divert management resources, and inadvertent failure to comply with new regulations may result in penalties being imposed on us.

Some states and localities have established drug importation programs for their citizens, and federal drug import legislation has been introduced in Congress. The Medicare Prescription Drug Plan legislation, which became law in 2003, required the Secretary of Health and Human Services to promulgate regulations for drug reimportation from Canada into the United States under some circumstances, including when the drugs are sold at a lower price than in the United States. The Secretary, however, retained the discretion not to implement a drug reimportation plan if he finds that the benefits do not outweigh the costs, and has so far declined to approve a reimportation plan. Proponents of drug reimportation may attempt to pass legislation that would directly allow reimportation under certain circumstances. Legislation or regulations allowing the reimportation of drugs, if enacted, could decrease the price we receive for any products that we may develop and adversely affect our future revenues and prospects for profitability.

***Even if we obtain regulatory approvals, our marketed drugs will be subject to ongoing regulatory review. If we fail to comply with continuing U.S. and foreign regulations, we could lose our approvals to market drugs and our business would be materially and adversely affected.***

Following regulatory approval of any drugs we may develop, we will remain subject to continuing regulatory review, including the review of adverse drug experiences and clinical results that are reported after our drug products are made available to patients. This would include results from any post-marketing tests or vigilance required as a condition of approval. The manufacturer and manufacturing facilities we use to make any of our drug products will also be subject to periodic review and inspection by the FDA. The discovery of any new or previously unknown problems with the product, manufacturer or facility may result in restrictions on the drug or manufacturer or facility, including withdrawal of the drug from the market. We would continue to be subject to the FDA requirements governing the labeling, packaging, storage, advertising, promotion, recordkeeping and submission of safety and other post-market information for all of our product candidates, even those that the FDA had approved. If we fail to comply with applicable continuing regulatory requirements, we may be subject to fines, suspension or withdrawal of regulatory approval, product recalls and seizures, operating restrictions and other adverse consequences.

***If we fail to attract, hire and retain qualified personnel, we may not be able to design, develop, market or sell our products or successfully manage our business.***

Our business prospects are dependent on our management team and all of our employees. The loss of any of our key employees, including Drs. Cauwenbergh and Pavco, who serve as our Chief Executive Officer and our Chief Development Officer, respectively, or our inability to identify, attract, retain and integrate additional qualified key personnel, could make it difficult for us to manage our business successfully and achieve our business objectives.

Competition for skilled research, product development, regulatory and technical personnel is intense, and we may not be able to recruit and retain the personnel we need. The loss of the services of any key research, product development, regulatory and technical personnel, or our inability to hire new personnel with the requisite skills, could restrict our ability to develop our product candidates.

**Risks Relating to Our Common Stock**

***The price of our common stock has been and may continue to be volatile and our recent relisting on the Nasdaq Capital Market may further increase volatility.***

The stock markets, in general, and the markets for drug delivery and pharmaceutical company stocks, in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. These broad market fluctuations may adversely affect the trading price of our common stock. In addition, the limited trading volume of our stock may contribute to its volatility.

Further, in February 2014 our common stock commenced trading on the Nasdaq Capital Market. Our stock price may be subject to additional volatility as a result of this listing on the Nasdaq Capital Market.

In the past, following periods of volatility in the market price of a particular company's securities, litigation has often been brought against that company. If litigation of this type is brought against us, it could be extremely expensive and divert management's attention and our company's resources.

***We have issued preferred stock in the past and possibly may issue more preferred stock in the future, and the terms of the preferred stock may reduce the value of our common stock.***

We are authorized to issue up to 10,000,000 shares of preferred stock in one or more series. There were 4,754 and 4,570 shares of our Series A Preferred Stock and Series A-1 Preferred Stock issued and outstanding at March 21, 2014, respectively. Our board of directors may determine the terms of future preferred stock offerings without further action by our stockholders. The issuance of our preferred stock could affect your rights or reduce the value of our outstanding common stock. In particular, rights granted to holders of preferred stock may include voting rights, preferences as to dividends and liquidation, conversion and redemption rights and restrictions on our ability to merge with or sell our assets to a third party. Additionally, the sale of a significant number of shares of common stock received upon conversion of our Series A or Series A-1 Preferred Stock could cause the market price of our common stock to decline.

***We may acquire other businesses or form joint ventures that may be unsuccessful and could dilute your ownership interest in our company.***

As part of our business strategy, we may pursue future acquisitions of other complementary businesses and technology licensing arrangements. We also may pursue strategic alliances. We have no experience with respect to acquiring other companies and limited experience with respect to the formation of collaborations, strategic alliances and joint ventures. We may not be able to integrate such acquisitions successfully into our existing business, and we could assume unknown or contingent liabilities. We also could experience adverse effects on our reported results of operations from acquisition related charges, amortization of acquired technology and other intangibles and impairment charges relating to write-offs of goodwill and other intangible assets from time to time following the acquisition. Integration of an acquired company requires management resources that otherwise would be available for ongoing development of our existing business. We may not realize the anticipated benefits of any acquisition, technology license or strategic alliance. For example, pursuant to the asset purchase agreement we entered into with OPKO, we acquired substantially all of OPKO's RNAi-related assets, including patents, licenses, clinical and preclinical data and other assets. These assets are at an early stage of development and will require a significant investment of time and capital if we are to be successful in developing them. There is no assurance that we will be successful in developing the assets that we acquired in the OPKO Asset Purchase, and a failure to successfully develop these assets could diminish our prospects. Further, if we fail to use commercially reasonable efforts to develop the OPKO assets for at least one clinical indication, OPKO would have the right, after a 180-day cure period, to reacquire the assets from us without any consideration.

To finance future acquisitions, we may choose to issue shares of our common stock or preferred stock as consideration, which would dilute your ownership interest in us. Alternatively, it may be necessary for us to raise additional funds through public or private financings. Additional funds may not be available on terms that are

favorable to us and, in the case of equity financings, may result in dilution to our stockholders. Any future acquisitions by us also could result in large and immediate write-offs, the incurrence of contingent liabilities or amortization of expenses related to acquired intangible assets, any of which could harm our operating results.

***The holders of our Series A and Series A-1 Preferred Stock may be able to delay or prevent a change in corporate control that would be beneficial to our stockholders.***

The holders of our Series A and Series A-1 Preferred Stock have the right to convert at any time their shares of our Series A Preferred Stock and Series A-1 Preferred Stock into shares of our common stock, except to the extent that the holder would own more than 9.999% of our common stock outstanding immediately after giving effect to the conversion. Although our Series A and Series A-1 Preferred Stock generally are non-voting stock, the holders of our Series A and Series A-1 Preferred Stock will be entitled to vote on an as-converted basis together with our common stock with respect to any transaction that would constitute a deemed liquidation event under our charter, including any proposed merger or sale of Company. Although the Series A and Series A-1 Preferred Stock holders have no rights to influence our day-to-day operations or even vote on the election of directors, by virtue of their voting rights in the context of a deemed liquidation event, the holders of our Series A and Series A-1 Preferred Stock will be able to significantly influence the outcome of the vote on any such extraordinary transaction that is required to be submitted to a vote of our stockholders. This right may adversely affect the market price of our common stock by:

- Delaying, deferring or preventing a change in control of our company;
- Impeding a merger, consolidation, takeover or other business combination involving our company; or
- Discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of our company in a "hostile" transaction.

***We do not anticipate paying cash dividends in the foreseeable future.***

Our business requires significant funding. We currently plan to invest all available funds and future earnings in the development and growth of our business and do not anticipate paying any cash dividends on our common stock in the foreseeable future. As a result, capital appreciation, if any, of our common stock will be your sole source of potential gain for the foreseeable future.

***Provisions of our certificate of incorporation and bylaws and Delaware law might discourage, delay or prevent a change of control of our company or changes in our management and, as a result, depress the trading price of our common stock.***

Our certificate of incorporation and bylaws contain provisions that could discourage, delay or prevent a change of control of our company or changes in our management that the stockholders of our company may deem advantageous. These provisions:

- Authorize the issuance of "blank check" preferred stock that our board could issue to increase the number of outstanding shares and to discourage a takeover attempt;
- Prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- Provide that the board of directors is expressly authorized to adopt, alter or repeal our bylaws; and
- Establish advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings.

Although we believe these provisions collectively provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board, they would apply even if the offer may be considered

beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management team by making it more difficult for stockholders to replace members of our board, which is responsible for appointing the members of our management.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

## ITEM 1B. *UNRESOLVED STAFF COMMENTS*

None.

## ITEM 2. *PROPERTIES*

On June 27, 2012, we entered into a lease (the "**Office Lease**") with Westborough Associates Building Five Limited Partnership to lease office space in the building known as Building Five in Westborough Office Park, located at 1500 West Park Drive, Westborough, Massachusetts, 01581, covering approximately 2,150 square feet. The premises will be used for office space, replacing our previous office space in Worcester, Massachusetts. The term of the lease commenced upon signing and will continue through April 30, 2014. The base rent for the premises is $37,625 per annum, payable monthly. As provided in the Office Lease, we may renew the Office Lease for one two-year term by giving six months prior written notice to the landlord. The rental rate for this renewal period shall be 95% of the then fair market rental rate.

On June 28, 2012, we entered into a sublease (the "**Laboratory Sublease**") with Massachusetts Biomedical Initiatives, Inc., a Massachusetts not-for-profit organization, to lease certain laboratory and office space at Gateway Park, 60 Prescott Street, Worcester, Massachusetts, 01605, covering approximately 526 square feet. The premises will be used for laboratory space and supporting functions, replacing our existing laboratory space in Worcester, Massachusetts. The term of the lease commenced on October 1, 2012 and will continue for a one-year period. The base rent for the premises is $25,200 per annum, payable monthly. As provided in the Laboratory Sublease, we may extend the Laboratory Sublease on a month-to-month basis and have the ability to terminate the Laboratory Sublease upon delivery of a sixty-day notice. The base rent during each year of any option term shall increase by 3.5% over the base rent for the prior year.

On December 17, 2013, we entered into a lease (the "**Lease**") with 257 Simarano Drive, LLC, Brighton Properties, LLC, Robert Stubblebine 1, LLC and Robert Stubblebine 2, LLC to lease office and laboratory space in the building known as the "Main Building" located at 257 Simarano Drive, Marlborough, Massachusetts, covering approximately 7,581 square feet. The premises will be used by the Company for office and laboratory space. The term of the Lease will commence on April 1, 2014 and continue for five years, expiring on March 31, 2019. The base rent for the premises during the first year of the Lease is $107,709.50 per annum, payable monthly. Each year thereafter, the base rent shall increase by approximately 3% over the base rent from the prior year.

We believe that our facilities are suitable for our current and future needs.

## ITEM 3. *LEGAL PROCEEDINGS*

Although we are not currently involved in any legal proceedings, from time to time, we may become a party to various legal actions and complaints arising in the ordinary course of business.

## ITEM 4. *MINE SAFETY DISCLOSURES*

Not applicable.

## PART II.

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

**Market Information**

Our common stock is listed on the NASDAQ Capital Market under the symbol "RXII". Prior to May 10, 2012, there was no established public trading market for our common stock. On July 23, 2013, we effected a 1-for-30 reverse stock split. The share prices in the table below are shown on a post-split basis. The following table shows the high and low per-share sale prices of our common stock for the periods indicated:

| | High | Low |
|---|---|---|
| **2012** | | |
| Second Quarter (from May 10, 2012) | $ 7.05 | $0.90 |
| Third Quarter | 7.65 | 2.85 |
| Fourth Quarter | 3.59 | 1.50 |
| **2013** | | |
| First Quarter | $10.74 | $2.10 |
| Second Quarter | 8.67 | 5.10 |
| Third Quarter | 6.23 | 3.23 |
| Fourth Quarter | 3.60 | 2.55 |

On March 21, 2014, the last reported sale price per share of our Common Stock on the NASDAQ Capital Market was $5.32.

**Holders**

As of March 21, 2014, there were approximately 172 holders of record of our common stock. Because many of our shares are held by brokers and other institutions on behalf of stockholders, we are unable to estimate the total number of individual stockholders represented by these holders of record.

**Dividends**

We have never paid any cash dividends and do not anticipate paying any cash dividends on our common stock in the foreseeable future. We expect to retain future earnings, if any, for use in our development activities and the operation of our business. The payment of any future dividends will be subject to the discretion of our board of directors and will depend, among other things, upon our results of operations, financial condition, cash requirements, prospects and other factors that our board of directors may deem relevant.

**Securities Authorized for Issuance Under Equity Compensation Plans**

We intend to file with the SEC a definitive Proxy Statement, which we refer to herein as the Proxy Statement, not later than 120 days after the close of the fiscal year ended December 31, 2013. The information required by this item relating to our equity compensation plans is incorporated herein by reference to the information contained under the section captioned "Equity Compensation Plan Information" of the Proxy Statement.

**Unregistered Sales of Securities**

No sales or issues of unregistered securities occurred that have not previously been disclosed in a Quarterly Report on Form 10-Q or in a Current Report on Form 8-K.

**Repurchases of Equity Securities**

We did not repurchase any shares of our common stock during fiscal 2013 or fiscal 2012.

## ITEM 6. *SELECTED FINANCIAL DATA*

As a smaller reporting company, we are not required to provide this information.

## ITEM 7. *MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS*

*The following discussion of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and the notes to those consolidated financial statements included in Item 8 of this Annual Report on Form 10-K. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth under "Risk Factors" and elsewhere in this Annual Report on Form 10-K, our actual results may differ materially from those anticipated in these forward-looking statements. Please refer to the discussion under the heading "Forward-Looking Statements" above.*

### Overview

We are a biotechnology company focused on discovering, developing and commercializing innovative therapies based on our proprietary, next-generation RNAi platform. Therapeutics that use RNAi have great promise because of their ability to "silence," or down-regulate, the expression of a specific gene that may be over-expressed in a disease condition. Prior to September 2011, our business was operated as an unincorporated division within our former parent company. We were incorporated in Delaware as a wholly owned subsidiary of our former parent company on September 8, 2011 in preparation for our planned spinoff, which was completed on April 27, 2012. Since that date, we have operated as an independent, publicly traded company.

By utilizing the expertise in RNAi and the comprehensive RNAi platform that we have established, we believe that we have discovered and will be able to discover and develop lead compounds and progress them into and through clinical development for potential commercialization. Our proprietary therapeutic platform is comprised novel RNAi compounds, referred to as rxRNA compounds, that are distinct from, and we believe convey significant advantages over, conventional siRNA (conventionally-designed "small interfering RNA" compounds), and offer many of the properties that we believe are important to the clinical development of RNAi-based drugs. We have developed a number of unique forms of rxRNA compounds, all of which have been shown to be highly potent both *in vitro* and in preclinical *in vivo* models. These RNAi compounds include rxRNAori and sd-rxRNA, or "self-delivering" RNA. Based on our research, we believe that these different, novel siRNA configurations have various potential advantages for therapeutic use. These potential advantages include high potency, increased resistance to nucleases and modifications to off-target effects, and, in the case of the sd-rxRNA compounds, access to cells and tissues with no additional formulation required, and, hence, reduced cell toxicity, which is known to be an issue with unmodified siRNAs.

Our lead clinical product candidate is RXI-109, a self-delivering RNAi compound (sd-rxRNA) being developed for the reduction of dermal scarring. RXI-109 is designed to reduce the expression of CTGF, a critical regulator of several biological pathways involved in scarring and fibrotic diseases. RXI-109 is being developed to prevent or reduce dermal scarring following surgery or trauma, as well as for the management of hypertrophic scars and keloids.

In June 2012, we initiated our first clinical trial of RXI-109, known as Study 1201. Study 1201 was designed to evaluate the safety and tolerability of several single-dose levels of RXI-109 in humans. Study 1201 enrolled fifteen subjects in a single-center, randomized, single-dose, double-blind, ascending dose, within-subject controlled study of RXI-109 for the treatment of incision scars, during which single, intradermal injections of escalating doses were administered. Subjects received an injection of RXI-109 in two separate areas on the abdomen and placebo injections in two other areas of the abdomen, followed by a small incision at each injection site. RXI-109 was well tolerated by intradermal injection. No serious local or systemic side effects were observed in the subjects at any of the doses administered, and maximum systemic exposure after intradermal

administration was assessed at approximately 5% of the total dose administered. In this study, RXI-109 showed excellent safety and tolerability with ascending single doses and significantly reduced the expression of CTGF protein in the wounded area in a dose-dependent manner 84 days after a single dose, suggesting a potent and long lasting effect on this key biomarker for abnormal scarring.

In December 2012, we initiated a second Phase 1 clinical trial with RXI-109, known as Study 1202. Study 1202 was designed to evaluate the safety and tolerability of multi-dose administration of RXI-109 in healthy volunteers, including an evaluation of surrogate end points of clinical efficacy. In total, Study 1202 enrolled fifteen subjects (5 cohorts of 3 subjects each) in a single-center, randomized, multi-dose, double-blind, ascending dose, within-subject controlled study of RXI-109 for the treatment of incision scars. Eight small skin incisions were made in their abdomen and subjects received treatment with RXI-109 at the four incision sites on one side of the abdomen and placebo treatment at the four incision sites on the other side of the abdomen. Treatments were given by three intradermal injections over a 2-week period. Subjects were monitored for safety and local and systemic effects over a total study period of 84 days. Multiple dermal injections were well tolerated all at dose levels. Treatment with RXI-109 demonstrated a trend for dose-dependent silencing of CTGF mRNA in the treated areas, resulting in 43-50% reduction of CTGF mRNA levels compared to the placebo when measured three days after the last dose. In one of two highest-dose cohorts, the dosing period was delayed by two weeks after the incisions were made. No additional benefit was seen on mRNA reduction.

Based on the safety profile shown in our two Phase 1 clinical trials, we initiated a Phase 2 clinical trial for RXI-109 in November 2013 known as Study 1301. In this study, patients with a long hypertrophic scar in the lower abdominal area are eligible to receive scar revision surgery and subsequent treatment with RXI-109 in one of two treatment regimens. Patients will receive RXI-109 or placebo on a blinded basis at the distal ends of their revised scar, leaving a central untreated section of the scar. Each patient's revised scar area will provide the opportunity to compare the appearance of the revised areas after treatment with RXI-109 or placebo when left untreated. This design allows for intra-subject comparison of the three revised scar segments, thereby increasing the power of the study.

In 2014, we expect to initiate two additional Phase 2 studies. The first additional Phase 2 study will evaluate the effect of RXI-109 on the recurrence of keloids after keloid revision surgery and the second additional Phase 2 study will evaluate the effect of RXI-109 on suppressing recurrence of hypertrophic scars after bilateral scar revision surgery in the breast area.

Overexpression of CTGF is implicated in dermal scarring and fibrotic disease, and because of this, we believe that RXI-109 or other CTGF-targeting RNAi compounds may be able to treat additional fibrotic indications, including pulmonary fibrosis, liver fibrosis, acute spinal injury, ocular scarring, joint fibrosis and vascular restenosis. If the current clinical trials of RXI-109 produce successful results in dermal anti-scarring, we may explore opportunities in these indications, as well as other possible dermatology applications (*e.g.*, cutaneous scleroderma).

While focusing our efforts on our RXI-109 development program, we also intend to continue to advance additional development programs both on our own and through collaborations with academic and corporate third parties. Current programs in the discovery and preclinical stages include an SBIR grant to evaluate and develop sd-rxRNAs as potential therapeutics for the treatment of retinoblastoma and a collaboration evaluating the potential to use a CTGF-targeting sd-rxRNA as a therapeutic to reduce or inhibit retinal scarring, which often occurs as a consequence of some retinal diseases and following retinal detachment.

On March 1, 2013, we entered into an asset purchase agreement with OPKO pursuant to which we have acquired substantially all of OPKO's RNAi-related assets, including patents, licenses, clinical and preclinical data and other assets. The assets purchased from OPKO are at an early stage of development, and we have established a discovery program to identify potential sd-rxRNA lead compounds and targets from these acquired RNAi-related assets.

In November 2013, we signed a distribution agreement with Ethicor Ltd. ("**Ethicor**"), a UK-based unlicensed medicinal products ("**Specials**") pharmaceutical company. The agreement provides Ethicor with the distribution rights to RXI-109 in the European Union, with the possibility to negotiate in the future to extend such rights to other regions of the world, excluding the United States, Canada and Mexico. If approved, Ethicor will pay us a double-digit percentage of any gross profits from its sales of RXI-109 by Ethicor. Ethicor's distribution rights continue until the agreement is terminated; provided, however, that should we obtain marketing authorization for RXI-109 in any of the countries covered by the agreement, we have the option to terminate the agreement with respect to each such country in which marketing authorization has been obtained. Under the European medicines legislation (Directive 2001/83/EC, Article 5(1)), we expect that Ethicor will be able to supply, prior to regulatory approval, RXI-109 as a "Special" drug. A "Special" drug may be requested by an authorized health-care professional to meet the special needs of an individual patient under their direct responsibility. The collaboration is important for health-care professionals and patients who can get safe controlled early access to a development drug and is a significant milestone for the Company, not only in possible early revenue, but as increased exposure to RXI-109 may be key in accelerating the development of our drug.

**Reverse Stock Split**

On July 23, 2013, we effected a 1-for-30 reverse stock split of our outstanding common stock in connection with a listing of our common stock on the NASDAQ Capital Market. Stockholders who would otherwise have been entitled to fractional shares as a result of the reverse stock split received a cash payment in lieu of receiving fractional shares. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices, if applicable, were proportionately increased in accordance with the terms of the agreements governing such securities. Shares of common stock reserved for issuance upon the conversion of the Company's Series A Preferred Stock were proportionately reduced and the respective conversion prices were proportionately increased. All share and per share amounts in the financial statements have been retroactively adjusted for all periods presented to give effect to the reverse stock split, including reclassifying an amount equal to the reduction in par value to additional paid-in capital.

**Research and Development**

To date, our research programs have focused on identifying product candidates and optimizing the delivery method and technology necessary to make RNAi compounds available by local or systemic administration, as appropriate, for diseases for which we intend to develop an RNAi therapeutic. Since we commenced operations, research and development has comprised a significant proportion of our total operating expenses and is expected to comprise the majority of our spending for the foreseeable future.

There are risks in any new field of drug discovery that preclude certainty regarding the successful development of a product. We cannot reasonably estimate or know the nature, timing and costs of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence from, any product candidate. Our inability to make these estimates results from the uncertainty of numerous factors, including but not limited to:

- Our ability to advance product candidates into preclinical research and clinical trials;
- The scope and rate of progress of our preclinical program and other research and development activities;
- The scope, rate of progress and cost of any clinical trials we commence;
- The cost of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights;
- Clinical trial results;
- The terms and timing of any collaborative, licensing and other arrangements that we may establish;

- The cost and timing of regulatory approvals;
- The cost of establishing clinical and commercial supplies of our product candidates and any products that we may develop;
- The cost and timing of establishing sales, marketing and distribution capabilities;
- The effect of competing technological and market developments; and
- The effect of government regulation and insurance industry efforts to control healthcare costs through reimbursement policy and other cost management strategies.

Failure to complete any stage of the development of our product candidates in a timely manner could have a material adverse effect on our operations, financial position and liquidity.

**License Agreements**

We have entered into licensing relationships with academic institutions, research foundations and commercial entities, and may seek to enter into additional licenses with pharmaceutical and biotechnology companies. We also may enter into strategic alliances to expand our intellectual property portfolio and to potentially accelerate our development programs by gaining access to technology and funding, including equity sales, license fees and other revenues. For each product that we develop that is covered by the patents licensed to us, including our material licenses discussed elsewhere in this Annual Report on Form 10-K, we are obligated to make additional payments upon the attainment of certain specified product development milestones.

See "Business — Intellectual Property" and note 13 to our consolidated financial statements for information on our material license agreements.

**Critical Accounting Policies and Estimates**

The discussion and analysis of our financial condition and results of operations is based upon our financial statements, which have been prepared in accordance with United States generally accepted accounting principles ("**GAAP**"). The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses, and related disclosure of contingent assets and liabilities. On an on-going basis, we evaluate our estimates, including those related to the impairment of long-lived assets, certain accrued expenses and stock-based compensation. We base our estimates on historical experience and various other assumptions that are believed to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions and could have a material impact on our reported results. While our significant accounting policies are more fully described in the Notes to our financial statements included elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies to be the most critical in understanding the judgments and estimates we use in preparing our financial statements,

***Research and Development Expenses***

Research and development costs are charged to expense as incurred and relate to salaries, employee benefits, facility-related expenses, supplies, stock-based compensation related to employees and non-employees involved in the Company's research and development, external services, other operating costs and overhead related to our research and development departments, costs to acquire technology licenses and expenses associated with preclinical activities and our clinical trials. Payments made by the Company in advance for research and development services not yet provided and/or for materials not yet received are recorded as prepaid expenses. Accrued liabilities are recorded related to those expenses for which vendors have not yet billed us with respect to services provided and/or materials that we have received.

Preclinical clinical trial expenses relate to third-party services, patient-related fees at the sites where our clinical trials are being conducted, laboratory costs, analysis costs, toxicology studies and investigator fees. Costs associated with these expenses are generally payable on the passage of time or when certain milestones are achieved. Expense is recorded during the period incurred or in the period in which a milestone is achieved. In order to ensure that we have adequately provided for preclinical and clinical expenses during the proper period, we maintain an accrual to cover these expenses. These accruals are assessed on a quarterly basis and are based on such assumptions as expected total cost, the number of patients and clinical trial sites and length of the study. Actual results may differ from these estimates and could have a material impact on our reported results. Our historical accrual estimates have not been materially different from our actual costs.

***Stock-based Compensation***

We follow the provisions of Financial Accounting Standards Board ("**FASB**") Accounting Standards Codification ("**ASC**") Topic 718, "*Compensation – Stock Compensation*" ("**ASC 718**"), which requires the measurement and recognition of compensation expense for all stock based payment awards made to employees, non-employee directors, and consultants, including employee stock options. Stock compensation expense based on the grant date fair value estimated in accordance with the provisions of ASC 718 is recognized as an expense over the requisite service period. Determining the amount of stock-based compensation to be recorded requires us to develop highly subjective estimates to be used in calculating the grant-date fair value of stock options. We use the Black-Scholes option pricing model to value our option grants and determine the related compensation expense. The use of the model requires us to make estimates of the following assumptions:

*Expected volatility* — Due to our limited trading history, we are responsible for estimating volatility and currently use the expected volatilities of similar entities. We have considered a number of factors in making our determination as to entities that are considered similar, such as the industry, stage of development, size of the company, and financial leverage.

*Expected term* — We use the simplified method to estimate the expected term assumption. The simplified method is based on the vesting period and the contractual term for each grant, or for each vesting-tranche for awards with graded vesting.

*Risk-free interest rate* — The yield on zero-coupon U.S. Treasury securities for a period that is commensurate with the expected term assumption is used as the risk-free interest rate.

*Dividend Yield* — We utilize a dividend yield of zero based on the fact that we have never paid cash dividends and currently have no intention to pay cash dividends.

The amount of stock-based compensation expense recognized during a period is based on the value of the portion of the awards that are ultimately expected to vest. ASC 718 requires forfeitures to be estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates. In developing a forfeiture rate estimate, the Company considered forfeiture rates used by similar entities as well as its historical experience and actual forfeitures for the year. We have estimated an annualized forfeiture rate of 5.0% for options granted to our employees and no forfeiture rate for the directors as of December 31, 2013. The Company will continue to evaluate its forfeiture rate as compared to the actual number of forfeitures in future periods to determine if adjustments to compensation expense may be required.

Stock based compensation expense prior to the completion of the spinoff was allocated to the carved out financial statements based on an estimate of time spent by Galena employees, board members, scientific advisory board members, and outside consultants on RXi related matters. Galena options held by current RXi employees were cancelled at the date of the completion of the spinoff except for options to purchase an aggregate of 477,191 shares of Galena common stock. The Company will continue to recognize stock compensation expense on the non-cancelled options as they vest. Under the terms of the option awards, these options will continue to vest as long as the individuals are employed by RXi.

For stock options granted as consideration for services rendered by non-employees, the Company recognizes compensation expense in accordance with the requirements of FASB ASC Topic 505-50, "*Equity Based Payments to Non-Employees"*. Non-employee option grants that do not vest immediately upon grant are recorded as an expense over the requisite service period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, will be re-measured using the fair value of the Company's common stock and the non-cash compensation recognized during the period will be adjusted accordingly. Since the fair market value of options granted to non-employees is subject to change in the future, the amount of the future compensation expense will include fair value re-measurements until the stock options are fully vested.

**Results of Operations**

The following data summarizes the results of our operations for the periods indicated, in thousands:

| | For the Years Ended December 31, | |
|---|---|---|
| | **2013** | **2012** |
| Revenue | $ 399 | $ 97 |
| Research and development expenses | (17,651) | (10,451) |
| General and administrative expenses | (3,697) | (2,621) |
| Operating loss | (20,949) | (12,975) |
| Net loss | (20,925) | (12,880) |
| Net loss applicable to common stockholders | $(29,535) | $(25,695) |

**Comparison of the Years Ended December 31, 2013 and 2012**

***Revenue***

We generate revenue through government grants. The following table summarizes our total revenues from government grants, for the periods indicated, in thousands:

| | For the Years Ended December 31, | |
|---|---|---|
| | **2013** | **2012** |
| Grant revenues | $399 | $97 |
| **Total revenues** | $399 | $97 |

Total revenues were approximately $399,000 for the year ended December 31, 2013, compared with $97,000 for the year ended December 31, 2012. The increase of $302,000 was due to the recognition of work completed on the Company's government grants during the year. Work increased significantly on the grants during the year ended December 31, 2013 as compared with the same period in the prior year as two of the Company's three grants had project end dates in 2013.

We also had $118,000 of deferred revenue at December 31, 2013, which consists of receipt of grant awards from the government, but which we have not yet recognized, pursuant to our revenue recognition policies, as the work has not been completed.

*Operating Expenses*

The following table summarizes our total operating expenses, for the periods indicated, in thousands:

| | For the Years Ended December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Research and development expenses | $17,651 | $10,451 |
| General and administrative expenses | 3,697 | 2,621 |
| **Total operating expenses** | $21,348 | $13,072 |

**Research and Development Expenses**

Research and development expenses consist of compensation-related costs for our employees dedicated to research and development activities, fees related to our Scientific Advisory Board ("**SAB**") members, expenses related to our ongoing research and development efforts primarily related to our clinical trials, drug manufacturing, outside contract services, licensing and patent fees and laboratory supplies and services for our research programs. We expect research and development expenses to increase as we expand our discovery, development and clinical activities.

Total research and development expense was approximately $17,651,000 for the year ended December 31, 2013, compared with $10,451,000 for the year ended December 31, 2012. The increase of $7,200,000, or 69%, was primarily due an increase of $6,077,000 in expense related to the fair value of common stock issued in exchange for patent and technology rights, $743,000 in research and development expenses largely due to costs related to the manufacture of RXI-109 for use in the Company's on-going clinical trials and an increase of $451,000 in employee stock-based compensation expense offset by a decrease of $71,000 in non-employee stock-based compensation related to the change in the fair value of stock options.

**General and Administrative Expenses**

General and administrative expenses consist primarily of compensation-related costs for our employees dedicated to general and administrative activities, legal fees, audit and tax fees, consultants, professional services and general corporate expenses.

General and administrative expense was approximately $3,697,000 for the year ended December 31, 2013, compared with $2,621,000 for the year ended December 31, 2012. The increase of $1,076,000, or 41%, was primarily due to an increase of $631,000 in employee stock-based compensation and $458,000 in general and administrative expense due to an increase in headcount, employee compensation and benefits, an increase in board fees due to the addition of two new members in 2013, annual Delaware franchise tax and professional services such as outside contract services and consultants, offset by a decrease of $13,000 related to the fair value of common stock warrants issued in exchange for services.

*Interest Income (Expense)*

The key objectives of our investment policy are to preserve principal and ensure sufficient liquidity, so our invested cash may not earn as high of a level of income as longer-term or higher risk securities, which generally have less liquidity and more volatility.

Interest income was approximately $24,000 for the year ended December 31, 2013, compared with interest expense of $30,000 for the year ended December 31, 2012. The increase of $54,000 was primarily due to interest earned on the Company's cash equivalents and short-term investments in 2013, compared with interest expense accrued in 2012 related to the bridge notes funded by the Series A Preferred Stock holders in 2012. The bridge notes were converted into shares of Series A Preferred Stock at the completion of the spinout from our former parent company in the second quarter of 2012.

***Series A and Series A-1 Preferred Stock Accretion and Dividends***

The following table summarizes our Series A and Series A-1 Preferred Stock transactions for the periods indicated, in thousands:

| | For the Years Ended December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Accretion of Series A Preferred Stock | $ — | $ 9,500 |
| Series A and Series A-1 Preferred Stock dividends | 8,610 | 3,315 |
| Accretion of Series A and Series A-1 Preferred Stock and dividends | $8,610 | $12,815 |

Accretion of the Series A and Series A-1 Preferred Stock and dividends was approximately $8,610,000 for the year ended December 31, 2013, compared with $12,815,000 for the year ended December 31, 2012. The decrease of $4,205,000, or 33%, is primarily due to the one-time charge of $9,500,000 related to the beneficial conversion feature of the Series A Preferred Stock in 2012 offset by an increase of $5,295,000 in Series A and Series A-1 Preferred Stock dividends in 2013 due to changes in the Company's closing common stock price on the dividend payment dates and the number of preferred shares earning dividends each year, as well as a full year of dividends issued as compared with 2012.

The rights and preferences of the Series A and Series A-1 Preferred Stock and the calculation of the dividend payable, are described further in Note 8 to the notes of the financial statements.

**Liquidity and Capital Resources**

We had cash, cash equivalents and short-term investments of approximately $14.4 million as of December 31, 2013, compared with approximately $5.1 million as of December 31, 2012.

On March 6, 2013, we entered into the SPA pursuant to which we agreed to issue 3,765,230 shares of common stock at a price of $4.35 per share. The gross proceeds from the Offering, which closed on March 12, 2013, were approximately $16.4 million, and the net proceeds, after payment of commissions and other costs, were approximately $15.7 million.

We believe that our existing cash, cash equivalents and short-term investments should be sufficient to fund the Company's operations, including the planned Phase 2 programs for RXI-109, into the second quarter of fiscal 2015.

We expect to incur significant operating losses as we advance our product candidates through the drug development and regulatory process. We have generated significant losses to date, have not generated any product revenue to date and may not generate product revenue in the foreseeable future, if ever. In the future, we will be dependent on obtaining funding from third parties, such as proceeds from the issuance of debt, sale of equity, funded research and development programs and payments under partnership and collaborative agreements, in order to maintain our operations and meet our obligations to licensors. There is no guarantee that debt, additional equity or other funding will be available to the Company on acceptable terms, or at all. If we fail to obtain additional funding when needed, we may be forced to scale back or terminate operations or to seek to merge with or to be acquired by another company.

***Net Cash Flow from Operating Activities***

Net cash used in operating activities was approximately $6,311,000 for the year ended December 31, 2013, compared with $5,062,000 for the year ended December 31, 2012. The increase of approximately $1,249,000 related primarily to the net loss of $20,925,000 for the year ended December 31, 2013 as compared to $12,880,000 for the same period in 2012, as described above, adjusted for non-cash items to arrive at the net cash

used in operating activities. The non-cash items adjusted for the year ended December 31, 2013 were approximately $14,328,000, compared with $7,293,000 for the year ended December 31, 2012. The increase of $7,035,000 in non-cash items in 2013 as compared to the same period in the prior year is primarily related to the increase of $6,077,000 in non-cash expense related to the fair value of common stock issued in exchange for patent and technology rights.

***Net Cash Flow from Investing Activities***

Net cash used in investing activities was $3,093,000 for the year ended December 31, 2013, compared with net cash provided by investing activities of $16,000 for the year ended December 31, 2012. Net cash used in investing activities during the year ended December 31, 2013 primarily included $9,000,000 used to purchase short-term investments, $78,000 used to purchase capital equipment, and $18,000 used to for the lease deposit of the Company's new facilities in 2014 partially offset by the maturity of short-term investments of $6,000,000. Net cash provided by investing activities during the year ended December 31, 2012 primarily included $33,000 of proceeds from the sale of capital equipment partially offset by $15,000 used to purchase equipment.

***Net Cash Flow from Financing Activities***

Net cash provided by financing activities was $15,667,000 for the year ended December 31, 2013, compared with $9,670,000 for the year ended December 31, 2012. Net cash provided by financing activities during 2013 was primarily attributable to the net proceeds received of $15,651,000 from the issuance of the Company's common stock to certain investors completed during March 2013. Net cash provided by financing activities during 2012 was primarily attributable to proceeds of $8,500,000 from the issuance of Series A Preferred Stock and proceeds of $500,000 from the issuance of convertible notes payable.

**Recently Issued Accounting Standards**

In February 2013, the FASB issued amendments to the accounting guidance for presentation of comprehensive income to improve the reporting of reclassifications out of accumulated other comprehensive income. The amendments do not change the current requirements for reporting net income or other comprehensive income, but do require an entity to provide information about the amounts reclassified out of accumulated other comprehensive income by component. In addition, an entity is required to present, either on the face of the statement where the net income is presented or in the notes, significant amounts reclassified out of accumulated other comprehensive income by the respective line items of net income but only if the amount reclassified is required under GAAP to be reclassified to net income in its entirety in the same reporting period. For other amounts that are not required under GAAP to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures required under GAAP that provide additional detail about these amounts. This new standard is required to be applied retrospectively and is effective for fiscal years and interim periods within those years beginning after December 15, 2012. The adoption of this standard did not impact the Company's financial statements as the Company's comprehensive loss is equal to its net loss for all periods presented.

**Off-Balance Sheet Arrangements**

In connection with certain license agreements, we are required to indemnify the licensor for certain damages arising in connection with the intellectual property rights licensed under the agreement. In addition, we are a party to a number of agreements entered into in the ordinary course of business that contain typical provisions that obligate us to indemnify the other parties to such agreements upon the occurrence of certain events. These indemnification obligations are considered off-balance sheet arrangements in accordance with ASC Topic 460 ("**ASC 460**"), "*Guarantor's Accounting and Disclosure Requirements for Guarantees, Including Indirect Guarantees of Indebtedness of Others*." To date, we have not encountered material costs as a result of such obligations and have not accrued any liabilities related to such obligations in our financial statements. See Note 13 of the notes to our financial statements for further discussion of these indemnification agreements.

## ITEM 7A. *QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK*

As a smaller reporting company, we are not required to provide this information.

**ITEM 8.** ***FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA***

**REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

Board of Directors and Stockholders
RXi Pharmaceuticals Corporation
Westborough, Massachusetts

We have audited the accompanying balance sheets of RXi Pharmaceuticals Corporation (the "Company"), a development stage company, as of December 31, 2013 and 2012, and the related statements of operations and cash flows for the years then ended and for the period from January 1, 2003 (date of inception) through December 31, 2013, and convertible preferred stock and stockholders' equity (deficit) for the period from September 24, 2011 to December 31, 2013, divisional equity for the period from April 3, 2006 to September 23, 2011 and the parent company's net deficit for the period from January 1, 2003 (date of inception) to December 31, 2006. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. Our audits 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 presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of the Company at December 31, 2013 and 2012 and the results of its operations and its cash flows for the years then ended and for the period from inception (January 1, 2003) through December 31, 2013, in conformity with accounting principles generally accepted in the United States of America.

/s/ BDO USA, LLP

Boston, Massachusetts
March 28, 2014

**RXi PHARMACEUTICALS CORPORATION**
(A Development Stage Company)
**BALANCE SHEETS**
**(Amounts in thousands, except share data)**

| | December 31, 2013 | December 31, 2012 |
|---|---|---|
| **ASSETS** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 11,390 | $ 5,127 |
| Restricted cash | 50 | 53 |
| Short-term investments | 3,000 | — |
| Prepaid expenses and other current assets | 303 | 212 |
| Total current assets | 14,743 | 5,392 |
| Equipment and furnishings, net of accumulated depreciation of $632 and $585, in 2013 and 2012, respectively | 177 | 198 |
| Other assets | 18 | 2 |
| Total assets | $ 14,938 | $ 5,592 |
| **LIABILITIES, CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)** | | |
| Current liabilities: | | |
| Accounts payable | $ 163 | $ 416 |
| Accrued expenses and other current liabilities | 1,795 | 767 |
| Deferred revenue | 118 | 491 |
| Current maturities of capital lease obligations | — | 5 |
| Total current liabilities | 2,076 | 1,679 |
| Deferred revenue, net of current portion | — | 27 |
| Total liabilities | 2,076 | 1,706 |
| Commitments and contingencies (Note 7) | | |
| Series A convertible preferred stock, $0.0001 par value, 15,000 shares authorized; 7,920 and 9,726 shares issued and outstanding at December 31, 2013 and 2012, respectively (at liquidation value) | 7,920 | 9,726 |
| Stockholders' equity (deficit): | | |
| Preferred stock, $0.0001 par value; 10,000,000 authorized (Note 8) | | |
| Series A-1 convertible preferred stock, $0.0001 par value, 5,000 shares authorized; 2,054 shares issued and outstanding at December 31, 2013 (at liquidation value) | 2,054 | — |
| Common stock, $0.0001 par value, 1,500,000,000 shares authorized; 11,788,045 and 5,289,007 shares issued and outstanding at December 31, 2013 and 2012, respectively | 1 | — |
| Additional paid-in capital | 40,969 | 11,317 |
| Deficit accumulated during the developmental stage | (38,082) | (17,157) |
| Total stockholders' equity (deficit) | 4,942 | (5,840) |
| Total liabilities, convertible preferred stock and stockholders' equity (deficit) | $ 14,938 | $ 5,592 |

See accompanying notes to financial statements.

**RXi PHARMACEUTICALS CORPORATION AND PREDECESSOR (RNAi)**
(A Development Stage Company)
**STATEMENTS OF OPERATIONS**
**(Amounts in thousands, except share and per share data)**

| | Years Ended December 31, | | Predecessor (RNAi) and RXi |
|---|---|---|---|
| | 2013 | 2012 | Period from January 1, 2003 (Date of Inception) to December 31, 2013 |
| Revenues: | | | |
| Grant revenues | $ 399 | $ 97 | $ 496 |
| Total revenues | 399 | 97 | 496 |
| Operating Expenses: | | | |
| Research and development expenses (1) | 17,651 | 10,451 | 73,833 |
| General and administrative expenses (1) | 3,697 | 2,621 | 43,534 |
| Total operating expenses | 21,348 | 13,072 | 117,367 |
| Operating loss | (20,949) | (12,975) | (116,871) |
| Interest income (expense), net | 24 | (30) | 622 |
| Other income, net | — | 125 | 6,441 |
| Loss before provision for income taxes | (20,925) | (12,880) | (109,808) |
| Provision for income taxes | — | — | — |
| Net loss | (20,925) | (12,880) | (109,808) |
| Accretion of Series A and Series A-1 convertible preferred stock and dividends | (8,610) | (12,815) | (21,425) |
| Net loss applicable to common stockholders | $ (29,535) | $ (25,695) | $(131,233) |
| Net loss per common share applicable to common stockholders (Note 2): | | | |
| Basic and diluted | $ (2.88) | $ (5.62) | |
| Weighted average common shares: | | | |
| Basic and diluted | 10,263,954 | 4,573,787 | |

(1) Non-cash stock-based compensation expenses included in operating expenses are as follows:

| | | |
|---|---|---|
| Research and development | 912 | 532 |
| General and administrative | 1,067 | 436 |

See accompanying notes to financial statements.

**RXi PHARMACEUTICALS CORPORATION AND PREDECESSOR (RNAi)**
**(A Development Stage Company)**
**STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STATEMENTS OF STOCKHOLDERS' EQUITY FOR THE PERIOD FROM SEPTEMBER 24, 2011 TO DECEMBER 31, 2013, DIVISIONAL EQUITY FOR THE PERIOD FROM APRIL 3, 2006 TO SEPTEMBER 23, 2011 AND PARENT COMPANY'S NET DEFICIT FOR THE PERIOD FROM JANUARY 1, 2003 (DATE OF INCEPTION) TO DECEMBER 31, 2006**
**(Amounts in thousands, except share data)**

| | RXi | | | | | | | | Predecessor (RNAi) | Predecessor (CytRx) | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | Series A Convertible Preferred Stock | | Series A-1 Convertible Preferred Stock | | Common Stock | | Additional Paid-in Capital | Deficit Accumulated Since Incorporation | Divisional Equity | Parent Company's Net Deficit | |
| | Shares Issued | Amount | Shares Issued | Amount | Shares Issued | Amount | | | | | Total |
| Inception, January 1, 2003 | | | | | | | | | $ — | $ — | $ — |
| Net loss | | | | | | | | | — | (89) | (89) |
| Balance at December 31, 2003 | | | | | | | | | — | (89) | (89) |
| Net loss | | | | | | | | | — | (3,272) | (3,272) |
| Net transactions with Parent Company | | | | | | | | | — | 2,393 | 2,393 |
| Balance at December 31, 2004 | | | | | | | | | — | (968) | (968) |
| Net loss | | | | | | | | | — | (2,209) | (2,209) |
| Net transactions with Parent Company | | | | | | | | | — | 2,727 | 2,727 |
| Balance at December 31, 2005 | | | | | | | | | — | (450) | (450) |
| Net loss | | | | | | | | | — | (2,405) | (2,405) |
| Net transactions with Parent Company | | | | | | | | | — | 2,587 | 2,587 |
| Balance at December 31, 2006 | | | | | | | | | $ — | $ (268) | $ (268) |
| Balance at April 3, 2006 | | | | | | | | | $ — | $ — | $ — |
| Cash contributions from Parent Company | | | | | | | | | 2 | — | 2 |
| Balance at December 31, 2006 | | | | | | | | | 2 | — | 2 |
| Non-cash equity adjustments from Parent Company | | | | | | | | | 4,318 | — | 4,318 |
| Cash contributions from Parent Company | | | | | | | | | 15,679 | — | 15,679 |
| Stock-based compensation expense | | | | | | | | | 1,814 | — | 1,814 |
| Net loss | | | | | | | | | (10,990) | — | (10,990) |
| Balance at December 31, 2007 | | | | | | | | | 10,823 | — | 10,823 |
| Non-cash equity adjustments from Parent Company | | | | | | | | | 750 | — | 750 |
| Cash contributions from Parent Company | | | | | | | | | 7,944 | — | 7,944 |
| Stock based compensation expense | | | | | | | | | 3,824 | — | 3,824 |
| Net loss | | | | | | | | | (14,373) | — | (14,373) |
| Balance at December 31, 2008 | | | | | | | | | 8,968 | — | 8,968 |
| Non-cash equity adjustments from Parent Company, net | | | | | | | | | (1,756) | — | (1,756) |
| Cash contributions from Parent Company | | | | | | | | | 7,714 | — | 7,714 |
| Stock based compensation expense | | | | | | | | | 4,202 | — | 4,202 |
| Net loss | | | | | | | | | (18,387) | — | (18,387) |
| Balance at December 31, 2009 | | | | | | | | | 741 | — | 741 |
| Non-cash equity adjustments from Parent Company, net | | | | | | | | | (2,326) | — | (2,326) |
| Cash contributions from Parent Company, net | | | | | | | | | 11,640 | — | 11,640 |
| Stock-based compensation expense | | | | | | | | | 4,368 | — | 4,368 |
| Net loss | | | | | | | | | (11,993) | — | (11,993) |
| Balance at December 31, 2010 | | | | | | | | | 2,430 | — | 2,430 |
| Non-cash equity adjustments from Parent Company, net | | | | | | | | | (8,083) | — | (8,083) |
| Cash contributions to Parent Company, net | | | | | | | | | 369 | — | 369 |
| Stock-based compensation expense | | | | | | | | | 1,987 | — | 1,987 |
| Reclassification of derivative liability upon elimination of obligation | | | | | | | | | 9,249 | — | 9,249 |
| Net loss—Predecessor (RNAi) | | | | | | | | | (7,682) | — | (7,682) |
| Recapitalization of divisional deficit | — | $ — | — | $ — | 3,347,996 | $ — | $ 10 | $ (1,740) | 1,730 | — | — |
| Stock-based compensation | — | — | — | — | — | — | 122 | — | — | — | 122 |
| Cash contribution from Parent Company | — | — | — | — | — | — | 1,500 | — | — | — | 1,500 |
| Expenses paid by Parent Company for RXi | — | — | — | — | — | — | 2,058 | — | — | — | 2,058 |
| Net loss | — | — | — | — | — | — | — | (2,537) | — | — | (2,537) |

| | RXi | | | | | | | | Predecessor (RNAi) | Predecessor (CytRx) | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | Series A Convertible Preferred Stock | | Series A-1 Convertible Preferred Stock | | Common Stock | | Additional Paid-in Capital | Deficit Accumulated Since Incorporation | Divisional Equity | Parent Company's Net Deficit | |
| | Shares Issued | Amount | Shares Issued | Amount | Shares Issued | Amount | | | | | Total |
| Balance at December 31, 2011 | — | — | — | — | 3,347,996 | — | 3,690 | (4,277) | — | — | (587) |
| Issuance of Series A convertible preferred stock | 9,500 | 9,500 | — | — | — | — | — | — | — | — | — |
| Beneficial conversion feature related to Series A convertible preferred stock | — | (9,500) | — | — | — | — | 9,500 | — | — | — | 9,500 |
| Accretion of beneficial conversion feature related to Series A convertible preferred stock | — | 9,500 | — | — | — | — | (9,500) | — | — | — | (9,500) |
| Issuance of common stock in exchange for patent and technology rights | — | — | — | — | 1,394,997 | — | 6,173 | — | — | — | 6,173 |
| Stock-based compensation expense | — | — | — | — | — | — | 968 | — | — | — | 968 |
| Issuance of common stock warrants in exchange for services | — | — | — | — | — | — | 13 | — | — | — | 13 |
| Expenses paid by Parent Company for RXi | — | — | — | — | — | — | 699 | — | — | — | 699 |
| Conversion of Series A convertible preferred stock to common stock | (224) | (224) | — | — | 546,014 | — | 224 | — | — | — | 224 |
| Fair value of Series A convertible preferred stock dividends | — | — | — | — | — | — | (3,315) | — | — | — | (3,315) |
| Dividends paid on Series A convertible preferred stock | 450 | 450 | — | — | — | — | 2,865 | — | — | — | 2,865 |
| Net loss | — | — | — | — | — | — | — | (12,880) | — | — | (12,880) |
| Balance at December 31, 2012 | 9,726 | 9,726 | — | — | 5,289,007 | — | 11,317 | (17,157) | — | — | (5,840) |
| Issuance of common stock, net of offering costs of $727 | — | — | — | — | 3,765,230 | 1 | 15,650 | — | — | — | 15,651 |
| Issuance of common stock in exchange for patent and technology rights | — | — | — | — | 1,666,666 | — | 12,250 | — | — | — | 12,250 |
| Stock-based compensation expense | — | — | — | — | — | — | 1,979 | — | — | — | 1,979 |
| Cash paid in lieu of fractional shares for 1:30 reverse stock split | — | — | — | — | (2,807) | — | (12) | — | — | — | (12) |
| Common stock issued upon exercise of stock options | — | — | — | — | 2,000 | — | 5 | — | — | — | 5 |
| Issuance of common stock under employee stock purchase plan | — | — | — | — | 11,265 | — | 28 | — | — | — | 28 |
| Exchange of Series A convertible preferred stock into Series A-1 convertible preferred stock | (2,000) | (2,000) | 2,000 | 2,000 | — | — | — | — | — | — | 2,000 |
| Conversions of Series A and Series A-1 convertible preferred stock into common stock | (434) | (434) | — | — | 1,056,684 | — | 434 | — | — | — | 434 |
| Fair value of Series A and Series A-1 convertible preferred stock dividends | — | — | — | — | — | — | (8,610) | — | — | — | (8,610) |
| Dividends issued on Series A and Series A-1 convertible preferred stock | 628 | 628 | 54 | 54 | — | — | 7,928 | — | — | — | 7,982 |
| Net loss | — | — | — | — | — | — | — | (20,925) | — | — | (20,925) |
| Balance at December 31, 2013 | 7,920 | $ 7,920 | 2,054 | $2,054 | 11,788,045 | $ 1 | $40,969 | $(38,082) | $— | $— | $ 4,942 |

See accompanying notes to financial statements.

**RXi PHARMACEUTICALS CORPORATION AND PREDECESSOR (RNAi)**
(A Development Stage Company)
**STATEMENTS OF CASH FLOWS**
**(Amounts in thousands)**

| | Predecessor (RNAi) and RXi | | |
|---|---|---|---|
| | Years Ended December 31, | | Period from January 1, 2003 (Date of Inception) through December 31, |
| | 2013 | 2012 | 2013 |
| Cash flows from operating activities: | | | |
| Net loss | $(20,925) | $(12,880) | $(109,808) |
| Adjustment to reconcile net loss to net cash used in operating activities: | | | |
| Depreciation and amortization expense | 99 | 147 | 910 |
| (Gain) Loss on disposal of equipment | — | (8) | 44 |
| Non-cash rent expense | — | — | 29 |
| Accretion and receipt of bond discount | — | — | 35 |
| Non-cash stock-based compensation expense | 1,979 | 968 | 20,913 |
| Fair value of common stock warrants issued in exchange for services | — | 13 | 13 |
| Fair value of common stock issued in exchange for patent and technology rights | 12,250 | 6,173 | 18,423 |
| Loss on exchange of Parent Company derivatives | — | — | 900 |
| Fair value of Parent Company's shares mandatorily redeemable for cash upon exercise of warrants | — | — | (785) |
| Fair value of Parent Company common stock and common stock warrants issued in exchange for services | — | — | 2,689 |
| Change in fair value of derivatives of Parent Company issued in connection with various equity financings | — | — | (5,604) |
| Fair value of Parent Company's common stock issued in exchange for licensing rights | — | — | 3,954 |
| Changes in operating assets and liabilities: | | | |
| Prepaid expenses and other assets | (89) | (26) | (285) |
| Accounts payable | (253) | 29 | 163 |
| Due to former Parent Company | — | 597 | 390 |
| Accrued expenses and other current liabilities | 1,028 | 223 | 2,431 |
| Deferred revenue | (400) | (298) | 118 |
| **Net cash used in operating activities** | (6,311) | (5,062) | (65,470) |
| Cash flows from investing activities: | | | |
| Change in restricted cash | 3 | — | (50) |
| Purchase of short-term investments | (9,000) | — | (46,532) |
| Maturities of short-term investments | 6,000 | — | 43,497 |
| Cash paid for purchase of equipment and furnishings | (78) | (15) | (838) |
| Proceeds from disposal of equipment and furnishings | — | 33 | 32 |
| Cash paid for lease deposit | (18) | (2) | (65) |
| **Net cash provided by (used) in investing activities** | (3,093) | 16 | (3,956) |
| Cash flows from financing activities: | | | |
| Cash contributions from Parent Company, net | — | 699 | 55,923 |
| Proceeds from the issuance of Series A convertible preferred stock | — | 8,500 | 8,500 |
| Proceeds from issuance of convertible notes payable | — | 500 | 1,000 |
| Net proceeds from the issuance of common stock | 15,651 | — | 15,651 |
| Proceeds from exercise of stock options | 5 | — | 5 |
| Proceeds from issuance of common stock in connection with employee stock purchase plan | 28 | — | 28 |
| Cash paid in lieu of fractional shares for 1:30 reverse stock split | (12) | — | (12) |
| Repayments of capital lease obligations | (5) | (29) | (279) |
| **Net cash provided by financing activities** | 15,667 | 9,670 | 80,816 |
| Net increase in cash and cash equivalents | 6,263 | 4,624 | 11,390 |
| Cash and cash equivalents, beginning of period | 5,127 | 503 | — |
| Cash and cash equivalents, end of period | $ 11,390 | $ 5,127 | $ 11,390 |

| | Years Ended December 31, | | Predecessor (RNAi) and RXi (Registrant) Period from January 1, 2003 (Date of Inception) through December 31, |
|---|---|---|---|
| | 2013 | 2012 | 2013 |
| **Supplemental disclosure of cash flow information:** | | | |
| Cash received during the period for interest | $ 24 | $ — | $ 748 |
| Cash paid during the period for interest | $ — | $ 30 | $ 38 |
| **Supplemental disclosure of non-cash investing and financing activities:** | | | |
| Settlement of corporate formation expenses in exchange for Parent Company common stock | $ — | $ — | $ 978 |
| Fair value of derivatives issued in connection with Parent Company common stock | $ — | $ — | $14,051 |
| Fair value of Parent Company shares mandatorily redeemable for cash upon exercise of warrants | $ — | $ — | $ 785 |
| Allocation of management expenses | $ — | $ — | $ 551 |
| Equipment and furnishings exchanged for Parent Company common stock | $ — | $ — | $ 48 |
| Equipment and furnishings acquired through capital lease | $ — | $ — | $ 277 |
| Non-cash lease deposit | $ — | $ — | $ 50 |
| Value of Parent Company restricted stock units and common stock issued in lieu of bonuses included in accrued expenses | $ — | $ — | $ 427 |
| Value of Parent Company restricted stock units issued in lieu of cash bonuses | $ — | $ — | $ 207 |
| Fair value of Parent Company stock options modified | $ — | $ — | $ 960 |
| Reclassification of derivative liability upon elimination of obligation | $ — | $ — | $ 9,249 |
| Fair value of Series A convertible preferred stock beneficial conversion feature | $ — | $9,500 | $ 9,500 |
| Accretion of Series A convertible preferred stock | $ — | $9,500 | $ 9,500 |
| Conversion of notes payable into Series A convertible preferred stock | $ — | $1,000 | $ 1,000 |
| Fair value of Series A and Series A-1 convertible preferred stock dividends | $8,610 | $3,315 | $11,925 |
| Conversion of Series A and Series A-1 convertible preferred stock into common stock | $ 434 | $ 224 | $ 658 |
| Exchange of Series A convertible preferred stock into Series A-1 convertible preferred stock | $2,000 | $ — | $ 2,000 |

See accompanying notes to financial statements.

**RXi PHARMACEUTICALS CORPORATION AND PREDECESSOR (RNAi)**
**(A Development Stage Company)**

**NOTES TO FINANCIAL STATEMENTS**

## 1. Nature of Business

Prior to April 13, 2011, Galena Biopharma, Inc. ("**Galena**" or the "**Parent Company**") (formerly known as RXi Pharmaceuticals Corporation) was engaged primarily in conducting discovery research and preclinical development activities based on RNAi, and Galena's financial statements for periods prior to April 13, 2011 reflected solely the assets, liabilities and results of operations attributable to its RNAi-based assets, liabilities and results of operations. On April 13, 2011, our former Parent Company broadened its strategic direction by adding the development and commercialization of cancer therapies that utilize peptide-based immunotherapy products, including a main product candidate, NeuVax, for the treatment of various cancers. On September 24, 2011, Galena contributed to RXi Pharmaceuticals Corporation ("**RXi**" or the "**Company**"), a newly formed subsidiary, substantially all of its RNAi-related technologies and assets. The newly formed RXi was incorporated on September 8, 2011 in preparation for the planned spinoff, which was completed on April 27, 2012. RXi was not engaged in any activities other than its initial incorporation from September 8, 2011 to September 23, 2011.

To date, the Company's principal activities, including that of its predecessor, have consisted of conducting discovery research and preclinical development activities utilizing its RNAi therapeutic platform, initiating clinical development for its first lead therapeutic candidate, acquiring RNAi technologies and patent rights through exclusive, co-exclusive and non-exclusive licenses, recruiting an RNAi-focused management and scientific/clinical advisory team, capital raising activities and conducting business development activities aimed at establishing research and development partnerships with pharmaceutical and larger biotechnology companies.

On March 6, 2013, the Company entered into a Securities Purchase Agreement (the "**SPA**") pursuant to which the Company agreed to issue 3,765,230 shares of common stock at a price of $4.35 per share (the "**Offering**"). The gross proceeds from the Offering, which closed on March 12, 2013, were approximately $16.4 million, and the net proceeds, after payment of commissions and other costs, were approximately $15.7 million.

On July 23, 2013, the Company effected a 1-for-30 reverse stock split of our outstanding common stock in connection with a listing of our common stock on the NASDAQ Capital Market. Stockholders who would have otherwise been entitled to fractional shares as a result of the reverse stock split received a cash payment in lieu of receiving fractional shares. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices, if applicable, were proportionately increased in accordance with the terms of the agreements governing such securities. Shares of common stock reserved for issuance upon the conversion of the Company's Series A convertible preferred stock ("**Series A Preferred Stock**") were proportionately reduced and the respective conversion prices were proportionately increased. All share and per share amounts in the financial statements have been retroactively adjusted for all periods presented to give effect to the reverse stock split, including reclassifying an amount equal to the reduction in par value to additional paid-in capital.

The Company expects to incur significant operating losses as it advances its product candidates through the drug development and regulatory process. The Company has generated significant losses to date, has not generated any product revenue to date and may not generate product revenue in the foreseeable future, if ever. In the future, RXi will be dependent on obtaining funding from third parties, such as proceeds from the issuance of debt, sale of equity, funded research and development programs and payments under partnership and collaborative agreements, in order to maintain RXi's operations and meet RXi's obligations to licensors. There is no guarantee that debt, additional equity or other funding will be available to the Company on acceptable terms, or at all. If the Company fails to obtain additional funding when needed, RXi would be forced to scale back, or terminate the Company's operations or to seek to merge with or to be acquired by another company. The

Company believes that its existing cash, cash equivalents and short-term investments will be sufficient to fund the Company's operations, including the planned Phase 2 programs for RXI-109, into the second quarter of fiscal 2015.

**2. Summary of Significant Accounting Policies**

*Basis of Presentation* — Historical financial information from the period January 1, 2003 through September 23, 2011 included in the Statements of Operations, Convertible Preferred Stock and Stockholders' Equity, Divisional Equity, and Parent Company's Net Deficit and Cash Flows for the cumulative period from inception (January 1, 2003) through December 31, 2013, has been "carved out" of the financial statements of our former Parent Company for such periods. Such financial information is limited to RNAi-related activities, assets and liabilities only, and excludes activities, assets and liabilities that are attributable to our former Parent Company's cancer therapy activities. RXi was formed on September 8, 2011 and was not engaged in any activities other than its initial incorporation from September 8, 2011 to September 23, 2011.

*Uses of estimates in preparation of financial statements* — The preparation of financial statements in accordance with accounting principles generally accepted in the United States of America requires management 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 and the reported amounts of revenues and expenses during the reporting period. Actual results could differ materially from those estimates.

*Cash and Cash Equivalents* — The Company considers all highly liquid debt instruments with an original maturity of three months or less to be cash equivalents. Cash equivalents consist primarily of amounts invested in money market accounts and certificates of deposits.

*Restricted Cash* — Restricted cash consists of certificates of deposit on hand with the Company's financial institutions as collateral for its corporate credit cards.

*Short-term Investments* — The Company's short-term investments consist of certificates of deposit with original maturities ranging from 6 months to 1 year.

*Concentrations of Credit Risk* — Financial instruments that potentially subject the Company to concentrations of credit risk consist principally of cash, cash equivalents and short-term investments. The Company maintains cash balances in several accounts with one bank, which at times are in excess of federally insured limits. The Company has established guidelines related to credit ratings and maturities intended to safeguard principal balances and maintain liquidity. The Company's investments are maintained in accordance with the Company's investment policy, which defines allowable investments, specifies credit quality standards and limits the exposure of any single issuer.

*Fair Value of Financial Instruments* — The carrying amounts reported in the balance sheet for cash equivalents, restricted cash, short-term investments, accounts payable and capital leases approximate their fair values due to their short-term nature or market rates of interest.

*Equipment and Furnishings* — Equipment and furnishings are stated at cost and depreciated using the straight-line method based on the estimated useful lives (generally three to five years for equipment and furniture) of the related assets.

Depreciation and amortization expense for the years ended December 31, 2013 and 2012 was approximately $99,000 and $147,000, respectively.

*Impairment of Long-Lived Assets* — The Company reviews long-lived assets, including finite-lived intangible assets, for impairment on an annual basis, as of December 31, or on an interim basis if an event occurs that might reduce the fair value of such assets below their carrying values. An impairment loss would be recognized based on the difference between the carrying value of the asset and its estimated fair value, which

would be determined based on either discounted future cash flows or other appropriate fair value methods. The Company believes no impairment existed as of December 31, 2013 and 2012.

*Stock-based Compensation* — The Company follows the provisions of the Financial Accounting Standards Board ("**FASB**") Accounting Standards Codification ("**ASC**") Topic 718, "*Compensation – Stock Compensation*" ("**ASC 718**") which requires the measurement and recognition of compensation expense for all stock-based payment awards made to employees, officers and non-employee directors, including stock options. Stock compensation expense based on the grant date fair value estimated in accordance with the provisions of ASC 718 is recognized as an expense over the requisite service period.

For stock options granted as consideration for services rendered by non-employees, the Company recognizes compensation expense in accordance with the requirements of FASB ASC Topic 505-50, "*Equity Based Payments to Non-Employees*" ("**ASC 505-50**"). Non-employee option grants that do not vest immediately upon grant are recorded as an expense over the requisite service period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, will be re-measured using the fair value of the Company's common stock and the non-cash compensation recognized during the period will be adjusted accordingly. Since the fair market value of options granted to non-employees is subject to change in the future, the amount of the future compensation expense will include fair value re-measurements until the stock options are fully vested.

*Revenue Recognition* — Principal sources of revenue consist of government research grants. Revenue from government grants is recognized over the respective contract periods as the services are performed, provided there is persuasive evidence of an arrangement, the fee is fixed or determinable and collection of the related receivable is reasonably assured, and no contingencies remain outstanding. Monies received prior to the recognition of revenue are recorded as deferred revenue.

*Research and Development Expenses* — Research and development costs are charged to expense as incurred and relate to salaries, employee benefits, facility-related expenses, supplies, stock-based compensation related to employees and non-employees involved in the Company's research and development, external services, other operating costs and overhead related to our research and development departments, costs to acquire technology licenses and expenses associated with preclinical activities and our clinical trials. Payments made by the Company in advance for research and development services not yet provided and/or for materials not yet received are recorded as prepaid expenses. Accrued liabilities are recorded related to those expenses for which vendors have not yet billed us with respect to services provided and/or materials that we have received.

Preclinical and clinical trial expenses relate to third-party services, patient-related fees at the sites where our clinical trials are being conducted, laboratory costs, analysis costs, toxicology studies and investigator fees. Costs associated with these expenses are generally payable on the passage of time or when certain milestones are achieved. Expense is recorded during the period incurred or in the period in which a milestone is achieved. In order to ensure that we have adequately provided for preclinical and clinical expenses during the proper period, we maintain an accrual to cover these expenses. These accruals are assessed on a quarterly basis and are based on such assumptions as expected total cost, the number of patients and clinical trial sites and length of the study. Actual results may differ from these estimates and could have a material impact on our reported results. Our historical accrual estimates have not been materially different from our actual costs.

*Patents and Patent Application Costs* — Although the Company believes that its patents and underlying technology have continuing value, the amount of future benefits to be derived from the patents is uncertain. Patent costs are, therefore, expensed as incurred.

*Income Taxes* — The Company recognizes assets or liabilities for the deferred tax consequences of temporary differences between the tax basis of assets or liabilities and their reported amounts in the financial statements in accordance with FASB ASC 740, *"Accounting for Income Taxes"* ("**ASC 740**"). These temporary differences will result in taxable or deductible amounts in future years when the reported amounts of the assets or

liabilities are recovered or settled. ASC 740 requires that a valuation allowance be established when management determines that it is more likely than not that all or a portion of a deferred asset will not be realized. The Company evaluates the realizability of its net deferred income tax assets and valuation allowances as necessary, at least on an annual basis. During this evaluation, the Company reviews its forecasts of income in conjunction with other positive and negative evidence surrounding the realizability of its deferred income tax assets to determine if a valuation allowance is required. Adjustments to the valuation allowance will increase or decrease the Company's income tax provision or benefit. The recognition and measurement of benefits related to the Company's tax positions requires significant judgment, as uncertainties often exist with respect to new laws, new interpretations of existing laws, and rulings by taxing authorities. Differences between actual results and the Company's assumptions or changes in the Company's assumptions in future periods are recorded in the period they become known.

*Comprehensive Loss* — The Company's comprehensive loss is equal to its net loss for all periods presented.

*Parent Company's Net Deficit* — The Parent Company's Net Deficit of the Predecessor consists of CytRx Corporation's ("**CytRx**") initial investment in Galena and subsequent changes in Galena's net investment resulting from Galena being an integrated part of CytRx. All disbursements for the Predecessor were made by CytRx.

*Non-cash equity adjustments from Parent Company* — Non-cash equity adjustments from Parent Company consist of credits for issuance of common stock for operational purposes, common stock and warrants issued to individuals engaged in RNAi activities, net of charges for the fair value of our former Parent Company's warrants that were allocated to the RNAi business and accounted for as a cost of equity at the time of issuance.

*Net loss per share* — The Company accounts for and discloses net loss per common share in accordance with FASB ASC Topic 260, "*Earnings per Share."* Basic and diluted net loss per common share is computed by dividing net loss attributable to common stockholders by the weighted average number of common shares outstanding. When the effects are not anti-dilutive, diluted earnings per share is computed by dividing the Company's net earnings by weighted average number of common shares outstanding and the impact of all dilutive common shares. There were no potential dilutive common shares for all periods presented.

The following table sets forth the potential common shares excluded from the calculation of net loss per common share because their inclusion would be anti-dilutive:

| | December 31, | |
|---|---|---|
| | **2013** | **2012** |
| Options to purchase common stock | 2,556,269 | 2,128,266 |
| Common stock underlying Series A and Series A-1 convertible preferred stock | 24,313,108 | 23,707,454 |
| Warrants to purchase common stock | 4,615 | 4,615 |
| Total | 26,873,992 | 25,840,335 |

**3. Recent Accounting Pronouncements**

In February 2013, the FASB issued amendments to the accounting guidance for presentation of comprehensive income to improve the reporting of reclassifications out of accumulated other comprehensive income. The amendments do not change the current requirements for reporting net income or other comprehensive income, but do require an entity to provide information about the amounts reclassified out of accumulated other comprehensive income by component. In addition, an entity is required to present, either on the face of the statement where the net income is presented or in the notes, significant amounts reclassified out of accumulated other comprehensive income by the respective line items of net income but only if the amount reclassified is required under GAAP to be reclassified to net income in its entirety in the same reporting period.

For other amounts that are not required under GAAP to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures required under GAAP that provide additional detail about these amounts. This new standard is required to be applied retrospectively and is effective for fiscal years and interim periods within those years beginning after December 15, 2012. The adoption of this standard did not impact the Company's financial statements as the Company's comprehensive loss is equal to its net loss for all periods presented.

**4. Fair Value Measurements**

The Company follows the provisions of FASB ASC Topic 820, "*Fair Value Measurements and Disclosures*."

The Company's financial assets and liabilities are re-measured and reported at fair value at each reporting period, and are re-measured and reported at fair value at least annually using a fair value hierarchy that is broken down into three levels. Level inputs are as defined as follows:

Level 1 — quoted prices in active markets for identical assets or liabilities.

Level 2 — other significant observable inputs for the assets or liabilities through corroboration with market data at the measurement date.

Level 3 — significant unobservable inputs that reflect management's best estimate of what market participants would use to price the assets or liabilities at the measurement date.

The Company categorized its restricted cash, cash equivalents and short-term investments as Level 2 hierarchy. The assets classified as Level 2 have initially been valued at transaction price and subsequently valued, at the end of each reporting period, using other market observable data. Observable market data points include quoted prices, interest rates, reportable trades and other industry and economic events. Financial assets measured at fair value on a recurring basis are summarized as follows, in thousands:

| Description | December 31, 2013 | Quoted Prices in Active Markets (Level 1) | Significant Other Observable Inputs (Level 2) | Unobservable Inputs (Level 3) |
|---|---|---|---|---|
| Assets: | | | | |
| Cash equivalents | $ 9,500 | $— | $ 9,500 | $— |
| Restricted cash | 50 | — | 50 | — |
| Short-term investments | 3,000 | — | 3,000 | — |
| Total assets measured and recorded at fair value | $12,550 | $— | $12,550 | $— |

| Description | December 31, 2012 | Quoted Prices in Active Markets (Level 1) | Significant Other Observable Inputs (Level 2) | Unobservable Inputs (Level 3) |
|---|---|---|---|---|
| Assets: | | | | |
| Restricted cash | $ 53 | $— | $ 53 | $— |
| Total assets measured and recorded at fair value | $ 53 | $— | $ 53 | $— |

**5. Capital Lease Obligations**

The Company acquires equipment under capital leases, which is included in equipment and furnishings in the balance sheet. The cost and accumulated amortization of capitalized leased equipment was approximately $26,000 and $26,000 at December 31, 2013, respectively, and $26,000 and $17,000 at December 31, 2012, respectively. Amortization expense for capitalized leased equipment was approximately $9,000 and $19,000 for

the years ended December 31, 2013 and 2012, respectively. During the years ended December 31, 2013 and 2012, the interest expense on these capital leases was negligible. At December 31, 2013, the Company had no outstanding capital leases.

**6. Accrued Expenses and Other Current Liabilities**

Accrued expenses and other current liabilities consist of the following, in thousands:

| | December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Employee compensation and benefits | $ 627 | $403 |
| Clinical development expenses | 665 | 153 |
| Professional fees | 190 | 107 |
| Research and development costs | 130 | 104 |
| Other | 183 | — |
| Total accrued expenses and other current liabilities | $1,795 | $767 |

**7. Commitments and Contingencies**

*License Commitments*

The Company acquires assets under development and enters into research and development arrangements with third parties that often require milestone and royalty payments based on the progress of the asset through development stages. Milestone payments may be required, for example, upon approval of the product for marketing by a regulatory agency. In certain agreements, the Company is required to make royalty payments based upon a percentage of the sales of the products licensed pursuant to such agreements. Because of the contingent nature of these payments, they are not included in the table of contractual obligations shown below (see also Note 13).

These arrangements may be material individually, and in the unlikely event that milestones for multiple products covered by these arrangements were reached in the same period, the aggregate charge to expense could be material to the results of operations. In addition, these arrangements often give the Company the discretion to unilaterally terminate development of the product, which would allow the Company to avoid making the contingent payments; however, the Company is unlikely to cease development if the compound successfully achieves clinical testing objectives.

License agreements generally relate to the Company's obligations associated with RNAi. The Company continually assesses the progress of its licensed technology and the progress of its research and development efforts as it relates to its licensed technology and may terminate with notice to the licensor at any time. In the event these licenses are terminated, no amounts will be due.

The Company's contractual license obligations that will require future cash payments as of December 31, 2013 are as follows, in thousands:

| Year Ending December 31, | |
|---|---|
| 2014 | $ 503 |
| 2015 | 153 |
| 2016 | 153 |
| 2017 | 153 |
| 2018 | 150 |
| 2019 and thereafter | 575 |
| Total | $1,687 |

*Operating Leases*

The Company leases certain office and laboratory space under various operating leases.

Total rent expense under the Company's operating leases was $62,900 and $138,000 for the years ended December 31, 2013 and 2012, respectively.

At December 31, 2013, the Company's future minimum payments required under operating leases are as follows, in thousands:

| **Year Ending December 31,** | |
|---|---|
| 2014 | $110 |
| 2015 | 118 |
| 2016 | 119 |
| 2017 | 117 |
| 2018 | 120 |
| 2019 and thereafter | 30 |
| Total | $614 |

The Company applies the disclosure provisions of FASB ASC Topic 460, "*Guarantor's Accounting and Disclosure Requirements for Guarantees, Including Indirect Guarantees of Indebtedness of Others*" ("**ASC 460**"), to its agreements that contain guarantee or indemnification clauses. The Company provides: (i) indemnifications of varying scope and size to certain investors and other parties for certain losses suffered or incurred by the indemnified party in connection with various types of third-party claims; and (ii) indemnifications of varying scope and size to officers and directors against third party claims arising from the services they provide to us. These indemnifications give rise only to the disclosure provisions of ASC 460. To date, the Company has not incurred costs as a result of these obligations and does not expect to incur material costs in the future. Accordingly, the Company has not accrued any liabilities in its financial statements related to these indemnifications.

## 8. Preferred Stock

The Company has authorized up to 10,000,000 shares of preferred stock, $0.0001 par value per share, for issuance. The Company's Board of Directors is authorized under the Company's Amended and Restated Articles of Incorporation, to designate the authorized preferred stock into one or more series and to fix and determine such rights, preferences, privileges and restrictions of any series of preferred stock, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, as shall be determined by the Company's Board of Directors upon its issuance.

***Series A Preferred Stock***

At December 31, 2013, 15,000 shares of Series A Preferred Stock, $0.0001 par value per share, were authorized for issuance.

*Issuance*

On April 27, 2012, upon the completion of the planned spinoff, the Company issued 9,500 shares of Series A Preferred Stock to Tang Capital Partners, L.P. ("**TCP**") and RTW Investments, LLC. Upon the issuance of the Series A Preferred Stock, the Series A Preferred Stock was assessed under FASB ASC Topic 480, "*Distinguishing Liabilities from Equity*" ("**ASC 480**") and it was determined that it was not within the scope of ASC 480. Therefore, the Series A Preferred Stock was not considered a liability under ASC 480.

The Series A Preferred Stock was then assessed under FASB ASC Topic 815, "*Derivatives and Hedging*" ("**ASC 815**"). The Series A Preferred Stock is convertible into common stock at the holders' option, subject to the terms of the Certificate of Designations. This embedded feature meets the definition of a derivative. The Company believes that the Series A Preferred Stock is an equity host for the purposes of assessing the embedded conversion option for potential bifurcation. The Company concluded that the conversion option feature is clearly and closely related to the preferred stock host. As such, the conversion feature did not require bifurcation under ASC 815.

The Company has recorded the Series A Preferred Stock in temporary equity as the Company may not be able to control the actions necessary to issue the maximum number of common shares needed to provide for a conversion in full of the then outstanding Series A Preferred Stock, at which time a holder of the Series A Preferred Stock may elect to redeem their preferred shares outstanding in the amount equal to the face value per share, plus unpaid accrued dividends.

*Dividends*

Holders of Series A Preferred Stock are entitled to receive cumulative mandatory dividends at the rate per share of seven percent (7%) of the face amount ($1,000 per share) per annum, payable quarterly on each March 31, June 30, September 30 and December 31. Dividends shall be payable in additional shares of Series A Preferred Stock valued for this purpose at the face amount. In the event there are not sufficient authorized Series A Preferred Shares available to pay such a dividend, the dividend shall instead accrete to and increase the value of the outstanding Series A Preferred Stock. The fair value of the Series A Preferred Stock dividend, which is included in the Company's net loss applicable to common shareholders, is calculated by multiplying the number of common shares that a preferred holder would receive upon conversion by the closing price of the Company's common stock on the dividend payment date.

The Company recorded Series A Preferred Stock dividends of $8,198,000 and $3,315,000 during the years ended December 31, 2013 and 2012, respectively.

*Liquidation Preference*

The "Liquidation Preference" with respect to a share of Series A Preferred Stock represents an amount equal to the face amount of the shares plus all accrued and unpaid dividends on the Series A Preferred Stock (as adjusted for any stock dividends, combinations, splits, recapitalizations and the like with respect to such shares). In the event of a liquidation, dissolution, or winding up, whether voluntary or involuntary, no distribution shall be made to the holders of any shares of capital stock of the Corporation (other than Senior Securities pursuant to the rights, preferences and privileges thereof) unless prior thereto the holders of shares of Series A Preferred Stock have received the Liquidation Preference with respect to each share then outstanding.

*Conversion*

Each holder of shares of Series A Preferred Stock may, at any time and from time to time, convert each of its shares into a number of fully paid and non-assessable shares of common stock at the defined conversion rate. Initially, each share of Series A Preferred Stock is convertible into 2,437.57 shares of common stock. In no event shall any holder of shares of Series A Preferred Stock have the right to convert shares of Series A Preferred Stock into shares of common stock to the extent that, after giving effect to such conversion, the holder, together with any of its affiliates, would beneficially own more than 9.999% of the then-issued and outstanding shares of common stock.

*Voting*

The holders of Series A Preferred Stock do not have any right to elect directors and have only limited voting rights, which consist primarily of the right to vote under certain protective provisions set forth in the Certificate

of Designations, regarding: (i) any proposed amendment to the Series A Preferred Stock or its right and preferences; and (ii) any proposed "Deemed Liquidation Event" as defined in the Certificate of Designations.

***Series A-1 Preferred Stock***

At December 31, 2013, 5,000 shares of Series A-1 convertible preferred stock ("**Series A-1 Preferred Stock**"), $0.0001 par value per share, were authorized for issuance.

*Exchange Transaction*

On August 13, 2013, we entered into an exchange agreement (the "**Exchange Agreement**") with TCP pursuant to which TCP agreed to exchange a total of 2,000 shares of Series A Preferred Stock for a like number of shares of Series A-1 Preferred Stock. The terms of the Series A-1 Preferred Stock are identical in all respects to the Series A Preferred Stock, other than the elimination of cash penalties that would potentially be due and payable upon the failure of the Company to have enough shares of common stock available to permit the conversion of Series A-1 Preferred Stock into common stock. The exchange transaction was recognized as a decrease of $2,000,000 in Series A Preferred Stock and a corresponding increase of $2,000,000 in Series A-1 Preferred Stock, which represents the face value of the shares exchanged.

Upon the issuance of the Series A-1 Preferred Stock, the Series A-1 Preferred Stock was assessed under ASC 480 and it was determined that it was not within the scope of ASC 480. Therefore, the Series A-1 Preferred Stock was not considered a liability under ASC 480.

The Series A-1 Preferred Stock was then assessed under ASC 815. The Series A-1 Preferred Stock is convertible into common stock at the holders' option, subject to the terms of the Certificate of Designations. This embedded feature meets the definition of a derivative. The Company believes that the Series A-1 Preferred Stock is an equity host for the purposes of assessing the embedded conversion option for potential bifurcation. The Company concluded that the conversion option feature is clearly and closely related to the preferred stock host. As such, the conversion feature did not require bifurcation under ASC 815.

The Company has recorded the Series A-1 Preferred Stock in permanent equity as the Company is not required to effect a net cash settlement in the instance that the Company does not have enough shares of common stock available to permit the conversion of Series A-1 Preferred Stock into common stock.

*Dividends*

Holders of Series A-1 Preferred Stock are entitled to receive cumulative mandatory dividends at the rate per share of seven percent (7%) of the face amount ($1,000 per share) per annum, payable quarterly on each March 31, June 30, September 30 and December 31. Dividends shall be payable in additional shares of Series A-1 Preferred Stock valued for this purpose at the face amount. In the event there are not sufficient authorized Series A-1 Preferred Shares available to pay such a dividend, the dividend shall instead accrete to and increase the value of the outstanding Series A-1 Preferred Stock. The fair value of the Series A-1 Preferred Stock dividend, which is included in the Company's net loss applicable to common shareholders, is calculated by multiplying the number of common shares that a preferred holder would receive upon conversion by the closing price of the Company's common stock on the dividend payment date.

The Company recorded Series A-1 Preferred Stock dividends of $412,000 during the year ended December 31, 2013 and no Series A-1 Preferred Stock dividends were recorded during the year ended December 31, 2012.

*Liquidation Preference*

The "Liquidation Preference" with respect to a share of Series A-1 Preferred Stock represents an amount equal to the face amount of the shares plus all accrued and unpaid dividends on the Series A-1 Preferred Stock

(as adjusted for any stock dividends, combinations, splits, recapitalizations and the like with respect to such shares). In the event of a liquidation, dissolution, or winding up, whether voluntary or involuntary, no distribution shall be made to the holders of any shares of capital stock of the Corporation (other than Senior Securities pursuant to the rights, preferences and privileges thereof) unless prior thereto the holders of shares of Series A-1 Preferred Stock have received the Liquidation Preference with respect to each share then outstanding. The liquidation preference of the Series A Preferred Stock is *pari passu* with the liquidation preference of the Series A-1 Preferred Stock.

*Conversion*

Each holder of shares of Series A-1 Preferred Stock may, at any time and from time to time, convert each of its shares into a number of fully paid and non-assessable shares of common stock at the defined conversion rate. Initially, each share of Series A-1 Preferred Stock is convertible into 2,437.57 shares of common stock. In no event shall any holder of shares of Series A-1 Preferred Stock have the right to convert shares of Series A-1 Preferred Stock into shares of common stock to the extent that such issuance or sale or right to effect such conversion would result in the holder or any of its affiliates together beneficially owning more than 9.999% of the then issued and outstanding shares of common stock.

If, at any time, the number of outstanding shares of common stock is increased by a stock split, stock dividend, combination, reclassification or other similar event (in each case, whether by merger or otherwise), then the conversion price shall be proportionately reduced. If the number of outstanding shares of common stock is decreased by a reverse stock split, combination or reclassification of shares, or other similar event (in each case, whether by merger or otherwise), then the conversion price shall be proportionately increased. Holders of Series A-1 Preferred Stock are also entitled to adjustments to the conversion price and other rights in the event of a merger, change of control and other defined events.

*Voting*

The holders of Series A-1 Preferred Stock do not have any right to elect directors and have only limited voting rights, which consist primarily of the right to vote under certain protective provisions set forth in the Certificate of Designations, regarding: (i) any proposed amendment to the Series A-1 Preferred Stock or its right and preferences; and (ii) any proposed "Deemed Liquidation Event" as defined in the Certificate of Designations.

**9. Common Stock**

*Common Stock Issuances*

On March 1, 2013, the Company entered into an asset purchase agreement with OPKO Health, Inc. ("**OPKO**") pursuant to which the Company acquired substantially all of OPKO's RNAi-related assets, including patents, licenses, clinical and preclinical data and other related assets (the "**OPKO Asset Purchase**"). Upon the close of the asset purchase agreement with OPKO on March 12, 2013, the Company issued to OPKO 1,666,666 shares of common stock. Under the asset purchase agreement, the Company will make, if applicable, up to $50 million per product in development and commercialization milestones for the successful development and commercialization of products utilizing the acquired OPKO intellectual property. In addition, if applicable, upon commercialization of these products the Company will make royalty payments to OPKO.

The Company assessed the OPKO Asset Purchase under FASB ASC Topic 805, *"Business Combinations"* ("**ASC 805**"), and it was determined that the transaction be accounted for as a purchase of assets, as the acquired assets did not constitute a business under the guidance of ASC 805. The assets purchased from OPKO are at an early stage of development, and, as such, determining the future economic benefit of the OPKO RNAi assets at the date of acquisition is highly uncertain. The fair value of the assets was determined using the quoted market price of the Company's common stock, on the date of the transfer of the assets, of March 12, 2013. Accordingly, the fair value of the OPKO Asset Purchase of $12,250,000 was expensed as in-process research and development during the year ended December 31, 2013.

On March 6, 2013, the Company entered into a SPA, pursuant to which the Company agreed to issue a total of 3,765,230 shares of common stock at a price of $4.35 per share. The gross proceeds from the Offering, which closed on March 12, 2013, were approximately $16.4 million, and the net proceeds, after payment of commissions and other costs, were approximately $15.7 million.

**10. Stock-Based Compensation**

*Stock Plans*

On January 23, 2012, the Company's board of directors and sole stockholder adopted the RXi Pharmaceuticals Corporation 2012 Long-Term Incentive Plan (the "**2012 Incentive Plan**"). Under the 2012 Incentive Plan, the Company may grant incentive stock options, nonqualified stock options, cash awards, stock appreciation rights, restricted and unrestricted stock and stock unit awards and other stock-based awards. The Company's board of directors currently acts as the administrator of the Company's 2012 Incentive Plan. The administrator has the power to select participants from among the key employees, directors and consultants of and advisors to the Company, establish the terms, conditions and vesting schedule, if applicable, of each award and to accelerate vesting or exercisability of any award.

As of December 31, 2013, an aggregate of 5,000,000 shares of common stock were reserved for issuance under the Company's 2012 Incentive Plan, including 2,556,269 shares subject to outstanding common stock options granted under the 2012 Incentive Plan and 2,443,731 shares available for future grants. Each option shall expire within ten years of issuance. Stock options granted by the Company to employees generally vest as to 12.5% of the shares on the six-month and first anniversary of the grant date and 25% of the shares at the end of each successive three-year period until fully vested.

*Stock-Based Compensation*

The Company follows the provisions of ASC 718, which requires the measurement and recognition of compensation expense for all stock-based payment awards made to employees and non-employee directors including employee stock options. Stock compensation expense based on the grant date fair value estimated in accordance with the provisions of ASC 718 is recognized as an expense over the requisite service period.

For stock options granted as consideration for services rendered by non-employees, the Company recognizes compensation expense in accordance with the requirements of ASC 505-50. Non-employee option grants that do not vest immediately upon grant are recorded as an expense over the requisite service period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, will be re-measured using the fair value of the Company's common stock and the non-cash compensation recognized during the period will be adjusted accordingly. Since the fair market value of options granted to non-employees is subject to change in the future, the amount of the future compensation expense will include fair value re-measurements until the stock options are fully vested.

The Company is currently using the Black-Scholes option-pricing model to determine the fair value of all its option grants. For option grants for the year ended December 31, 2013 and 2012, the following assumptions were used:

| | Year Ended December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Weighted average risk-free interest rate | 1.26% | 0.94% |
| Weighted average expected volatility | 76.36% | 88.70% |
| Weighted average expected option term (years) | 5.93 | 6.05 |
| Weighted average expected dividend yield | 0.00% | 0.00% |

The weighted-average fair value of options granted during the years ended December 31, 2013 and 2012 was $4.05 and $2.10 per share, respectively.

The risk-free interest rate used for each grant was based upon the yield on zero-coupon U.S. Treasury securities with a term similar to the expected life of the related option. The Company's expected stock price volatility assumption is based upon the volatility of a composition of comparable companies. The expected life assumption for employee grants were based upon the simplified method provided for under ASC 718-10 and the expected life assumptions for non-employees were based upon the contractual term of the option. The dividend yield assumption of zero is based upon the fact that the Company has never paid cash dividends and presently has no intention of paying cash dividends. The Company has estimated an annualized forfeiture rate of 5.0% for options granted to its employees and 0% forfeiture rate for the directors. The Company will record additional expense if the actual forfeitures are lower than estimated and will record a recovery of prior expense if the actual forfeitures are higher than estimated.

The following table summarizes the activity of Company's stock option plan for the period January 1, 2012 to December 31, 2013:

| | Stock Options | Weighted Average Exercise Price |
|---|---|---|
| Balance at January 1, 2012 | — | $ — |
| Granted | 2,128,266 | 3.00 |
| Exercised | — | — |
| Cancelled | — | — |
| Balance at December 31, 2012 | 2,128,266 | $3.00 |
| Granted | 430,003 | 6.16 |
| Exercised | (2,000) | 2.55 |
| Cancelled | — | — |
| Outstanding, December 31, 2013 | 2,556,269 | $3.47 |
| Exercisable, December 31, 2013 | 1,023,597 | $3.26 |

Stock-based compensation expense for the years ended December 31, 2013 and 2012 was approximately $1,979,000 and $968,000, respectively. Of this, the Company recognized approximately $43,000 and $114,000 of expense related to non-employee stock options for the same period. There is no income tax benefit as the Company is currently operating at a loss and an actual income tax benefit may not be realized.

The weighted-average remaining contractual life of options outstanding and exercisable at December 31, 2013 was 8.59 years and 8.48 years, respectively.

The aggregate intrinsic value of outstanding options as of December 31, 2013 was $555,000, of which $234,000 related to exercisable stock options. The intrinsic value of stock options exercised was $2,000 for the year ended December 31, 2013. No options were exercised during the year ended December 31, 2012.

As of December 31, 2013, the compensation expense for all unvested stock options in the amount of approximately $3,627,000 will be recognized in our results of operations over a weighted average period of 2.48 years.

*Employee Stock Purchase Plan*

On June 7, 2013, the Compensation Committee approved an employee stock purchase plan ("**ESPP**"), subject to the approval of the Company's stockholders within twelve months of the date the ESPP was adopted. The ESPP allows employees to contribute a percentage of their cash earnings, subject to certain maximum

amounts, to be used to purchase shares of the Company's common stock on each of two semi-annual purchase dates. The purchase price is equal to 90% of the market value per share on either (a) the date of grant of a purchase right under the ESPP or (b) the date on which such purchase right is deemed exercised, whichever is lower. The maximum number of shares available for issuance pursuant to the ESPP is equal to the lesser of: (a) 50,000 shares, increased on each anniversary of the adoption of the ESPP by one percent (1%) of the total shares of stock then outstanding, and (b) 113,333 shares.

The fair value was estimated using the Black-Scholes option-pricing model.

The following assumptions were used to value the shares under the ESPP for the period ended December 31, 2013:

| | |
|---|---|
| Weighted average risk-free interest rate | 0.09% |
| Weighted average expected volatility | 88.68% |
| Weighted average expected lives (years) | 0.50 |
| Weighted average expected dividend yield | 0.00% |

The risk-free interest rate used was based upon the prevailing short-term interest rates. The Company's expected volatility is based upon the volatility of a composition of comparable companies for the expected term. The expected life assumption was based upon the purchase period and the dividend yield assumption of zero is based upon the fact that the Company has never paid cash dividends and presently has no intention of paying cash dividends.

The weighted average fair value of stock purchase rights granted as part of the ESPP was $2.04 for the year ended December 31, 2013.

The Company recorded $10,200 of stock-based compensation expense for the year ended December 31, 2013 related to the ESPP. The Company issued 11,265 shares during the year ended December 31, 2013 and at December 31, 2013, 38,735 shares were available for issuance under the ESPP.

*Predecessor Stock-Based Compensation Expense*

Stock-based compensation expense prior to the completion of the spinoff on April 27, 2012 was allocated to the Company's carved-out financial statements based on an estimate of time spent by Galena employees, board members, scientific advisory board members and outside consultants on RXi related matters. Galena options held by current RXi employees were cancelled at the date of the completion of the spin-off except for options to purchase an aggregate of 477,191 shares of Galena common stock. The Company will continue to recognize stock compensation expense on the non-cancelled options as they vest. Under the terms of the option awards, these options will continue to vest as long as the individuals are employed by RXi. As of December 31, 2013, 468,941 options remain outstanding with a range of exercise prices from $0.65 to $7.50.

Of the total stock-based compensation expense recorded by the Company, approximately $13,100 and $283,000 related to options issued by our former Parent Company for the years ended December 31, 2013 and 2012, respectively.

## 11. Development Stage Supplemental Equity Disclosure

Summarized below are the Company's equity (common stock and common stock options) transactions since the Company's inception through December 31, 2013.

| Type of Security | Date of Issuance | Shares of Common Stock | Dollar Amount of Consideration ($) | Price per Share or Exercise Price per Share ($) | Counter Party to Transaction | Nature of Non-Cash Consideration | Basis of Assigning Cost |
|---|---|---|---|---|---|---|---|
| Common Stock | September 8, 2011 | 100 | — | 0.01 | Galena | NA | Cash |
| Common Stock | April 27, 2012 | 3,347,996 | NA | 0.27 | Galena | NA | Independent Third Party Valuation |
| Common Stock | April 27, 2012 | 1,394,997 | NA | 4.43 | Advirna | Intellectual Properties | Market Value |
| Common Stock | Various - 2012 | 546,014 | NA | 0.41 | Conversion of Series A Preferred Stock | NA | Cost Basis |
| Common Stock | March 12, 2013 | 3,765,230 | 15,651 | 4.35 | PIPE | NA | Net Cash |
| Common Stock | March 12, 2013 | 1,666,666 | NA | 4.35 | Opko | Intellectual Properties | Market Value |
| Common Stock | Various - 2013 | 1,056,684 | NA | 0.41 | Conversion of Series A Preferred Stock | NA | Cost Basis |
| Common Stock | September 11, 2013 | 2,000 | 5 | 2.55 | Exercise of Stock Options | NA | Cash |
| Common Stock | December 31, 2013 | 11,265 | 28 | 2.61 | ESPP | NA | Cash |

## 12. Income Taxes

The components of federal and state income tax expense are as follows (in thousands):

| | Year Ended December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Current | | |
| Federal | $ — | $ — |
| State | — | — |
| Total current | — | — |
| Deferred | | |
| Federal | (9,123) | (1,903) |
| State | (2,002) | (484) |
| Total deferred | (11,125) | (2,387) |
| Valuation allowance | 11,125 | 2,387 |
| Total income tax expense | $ — | $ — |

The components of net deferred tax assets are as follows (in thousands):

| | As of December 31, | |
|---|---|---|
| | 2013 | 2012 |
| Net operating loss carryforwards | $ 6,590 | $ 3,054 |
| Tax credit carryforwards | 146 | 3 |
| Stock based compensation | 861 | 282 |
| Licensing deduction deferral | 6,864 | 57 |
| Other timing differences | 165 | 105 |
| Gross deferred tax assets | 14,626 | 3,501 |
| Valuation allowance | (14,626) | (3,501) |
| Net deferred tax asset | $ — | $ — |

The Company's deferred tax assets at December 31, 2013 and 2012 consisted primarily of its net operating loss carryforwards, tax credit carryforwards, Section 197 intangible assets capitalized for federal income tax purposes and certain accruals that for tax purposes are not deductible until future payment is made.

The Company has incurred net operating losses since inception. At December 31, 2013, the Company had federal and state net operating loss carryforwards of approximately $30.3 million, which are available to reduce future taxable income expiring in 2033. Based on an assessment of all available evidence including, but not limited to the Company's limited operating history in its core business and lack of profitability, uncertainties of the commercial viability of its technology, the impact of government regulation and healthcare reform initiatives, and other risks normally associated with biotechnology companies, the Company has concluded that it is more likely than not that these net operating loss carryforwards and credits will not be realized and, as a result, a full deferred income tax valuation allowance has been recorded against these assets.

Under the provisions of the Internal Revenue Code, certain substantial changes in the Company's ownership may result in a limitation on the amount of net operating loss carryforwards and research and development credit carryforwards which could be utilized annually to offset future taxable income and taxes payable.

The Company files income tax returns in the U.S. and Massachusetts. The Company is subject to tax examinations for the 2013 tax year. The Company has not recorded any uncertain tax positions as of December 31, 2013 or 2012. The Company does not believe there will be any material changes in its unrecognized tax positions over the next 12 months. RXi has not incurred any interest or penalties. In the event that the Company is assessed interest or penalties at some point in the future, they will be classified in the financial statements as general and administrative expenses.

**13. License Agreements**

As part of its business, the Company enters into licensing agreements with third parties that often require milestone and royalty payments based on the progress of the asset through development stages. Milestone payments may be required, for example, upon approval of the product for marketing by a regulatory agency. In certain agreements, the Company is required to make royalty payments based upon a percentage of the sales of the products licensed pursuant to such agreements.

The expenditures required under these arrangements may be material individually in the event that the Company develops product candidates covered by the intellectual property licensed under any such arrangement, and in the unlikely event that milestones for multiple products covered by these arrangements were reached in the same period, the aggregate charge to expense could be material to the results of operations. In addition, these arrangements often give the Company discretion to unilaterally terminate development of the product, which would allow the Company to avoid making the contingent payments; however, the Company is unlikely to cease development if the compound successfully achieves clinical testing objectives.

*University of Massachusetts Medical School*. We hold a non-exclusive license from the University of Massachusetts Medical School ("**UMMS**"). This license grants to us rights under certain UMMS patent applications to make, use and sell products related to applications of RNAi technologies in particular fields, including HCMV and retinitis, amyotrophic lateral sclerosis, known as "ALS" or "Lou Gehrig's Disease," diabetes and obesity. Throughout the term of the license, we must pay UMMS an annual maintenance fee of $15,000. We also will be required to pay to UMMS customary royalties of up to 10% of: (i) any future net sales of licensed products; (ii) income received from any sublicensees under this license; and (iii) net sales of commercial clinical laboratory services, subject to a minimum royalty of $50,000 beginning in 2016. We also agreed to pay expenses incurred by UMMS in prosecuting and maintaining the licensed patents.

The UMMS license was effective on April 15, 2003, and will remain in effect until the expiration of all issued patents within the "patent rights" (as defined), unless earlier terminated in accordance with the provisions of the license. In the event that either party commits a material breach of its obligations under the UMMS license and fails to cure that breach within 60 days after receiving written notice thereof, the other party may terminate the UMMS license immediately upon written notice to the party in breach.

*Advirna.* We have entered into agreements with Advirna, or their surviving entity, pursuant to which Advirna assigned to us its existing patent and technology rights related to sd-rxRNA technology in exchange for our agreement to pay Advirna an annual maintenance fee of $100,000 and a one-time milestone payment of $350,000 upon the issuance of the first patent with valid claims covering the assigned technology. Additionally, we will be required to pay a 1% royalty to Advirna on any licensing revenue received by us with respect to future licensing of the assigned Advirna patent and technology rights. We also granted back to Advirna a license under the assigned patent and technology rights for fields of use outside human therapeutics and diagnostics and issued to Advirna, upon the completion of the spin-off transaction, 1,394,997 shares of common stock. The Company recorded research and development expense of $6,173,000 during the year ended December 31, 2012 to recognize the fair value of the common shares issued in exchange for the sd-rxRNA® patent and technology rights assigned to RXi by Advirna.

Our rights under the Advirna agreement will expire upon the later of: (i) the expiration of the last-to-expire of the "patent rights" (as defined) included in the Advirna agreement; or (ii) the abandonment of the last-to-be abandoned of such patents, unless earlier terminated in accordance with the provisions of the agreement.

We may terminate the Advirna agreement at any time upon 90 days' written notice to Advirna, and Advirna may terminate the agreement upon 90 days' prior written notice in the event that we cease using commercially reasonable efforts to research, develop, license or otherwise commercialize the patent rights or "royalty-bearing products" (as defined), provided that we may refute such claim within such 90-day period by showing budgeted expenditures for the research, development, licensing or other commercialization consistent with other technologies of similar stage of development and commercial potential as the patent rights or royalty-bearing products. Further, either party at any time may provide to the other party written notice of a material breach of the agreement. If the other party fails to cure the identified breach within 90 days after the date of the notice, the aggrieved party may terminate the agreement by written notice to the party in breach.

**14. Related Party Transactions**

On September 24, 2011, the Company entered into an agreement with Advirna, which was co-founded by the Company's former Senior Vice President and Chief Scientific Officer, pursuant to which Advirna assigned to RXi its existing patent and technology rights related to *sd*-rxRNA® technology in exchange for the Company's agreement to pay Advirna an annual maintenance fee, other consideration upon the achievement of certain milestones and issued to Advirna 1,394,997 shares of common stock at the date of the completion of the spinoff. We also granted back to Advirna a license under the assigned patent and technology rights for fields of use outside human therapeutics and diagnostics (see also Note 13).

**15. Subsequent Events**

On January 24, 2014, the Company entered into an exchange agreement with TCP pursuant to which TCP exchanged a total of 3,000 shares of Series A Preferred Stock for a like number of shares of Series A-1 Preferred Stock. The terms of the Series A-1 Preferred Stock are identical in all respects to the Series A Preferred Stock, other than the elimination of cash penalties that would potentially be due and payable upon the failure of the Company to have enough shares of Common Stock available to permit the conversion of Series A Preferred Stock into Common Stock. As a result of the elimination of this penalty, the face value of the Series A-1 Preferred Stock will be reclassified on our balance sheet from mezzanine to stockholders' equity, which reclassification will be reflected in the quarter ending March 31, 2014 and will result in the addition of $3 million to stockholders' equity.

Also on January 24, 2014, the Company filed a Certificate of Increase with the Secretary of State of the State of Delaware, increasing the total authorized shares of Series A-1 Preferred Stock from 5,000 shares to 10,000 shares.

## ITEM 9. *CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURES*

None.

## ITEM 9A. *CONTROLS AND PROCEDURES*

### Evaluation of Disclosure Controls and Procedures

Based on an evaluation as of the end of the period covered by this report, Dr. Geert Cauwenbergh, our Chief Executive Officer and acting Chief Financial Officer, has concluded that our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) were effective as of the end of the period covered by this report to ensure that information that we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in SEC rules and forms.

Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their objectives, and Dr. Cauwenbergh has concluded that these controls and procedures are effective at the "reasonable assurance" level. We believe that a control system, no matter how well designed and operated, cannot provide absolute assurance that the objectives of the control system are met, and no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within a company have been detected.

### Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles.

There are inherent limitations in the effectiveness of any system of internal control, including the possibility of human error and the circumvention or overriding of controls. Accordingly, even effective internal controls can provide only reasonable assurances with respect to financial statement preparation. Further, because of changes in conditions, the effectiveness of internal control may vary over time.

Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2013. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations ("**COSO**") of the Treadway Commission in *Internal Control — Integrated Framework* . Based on our assessment using the COSO criteria, the Company's Chief Executive Officer and Chief Financial Officer concluded that, as of December 31, 2013, our internal control over financial reporting is effective.

This Annual Report on Form 10-K provides only management's report. As a smaller reporting company, we are not required to provide an attestation report by our independent registered public accounting firm regarding internal control over financial reporting.

### Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the quarter ended December 31, 2013 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

**ITEM 9B.** ***OTHER INFORMATION***

In November 2013, the Company signed a distribution agreement with Ethicor Ltd. ("**Ethicor**"), a UK-based unlicensed medicinal products ("**Specials**") pharmaceutical company. The agreement provides Ethicor with the distribution rights to RXI-109 in the European Union, with the possibility to negotiate in the future to extend such rights to other regions of the world, excluding the United States, Canada and Mexico. Ethicor must pay us a double-digit percentage of any gross profits from its sales of RXI-109. Ethicor's distribution rights continue until the agreement is terminated; provided, however, that should we obtain marketing authorization for RXI-109 in any of the countries covered by the agreement, we have the option to terminate the agreement with respect to each such country in which marketing authorization has been obtained. Under the European medicines legislation (Directive 2001/83/EC, Article 5(1)), we expect that Ethicor will be able to supply, prior to regulatory approval, RXI-109 as a "Special" drug. A "Special" drug may be requested by an authorized health-care professional to meet the special needs of an individual patient under their direct responsibility. The collaboration is important for health-care professionals and patients who can get safe controlled early access to a development drug and is a significant milestone for the Company, not only in possible early revenue, but as increased exposure to RXI-109 may be key in accelerating the development of our drug.

## PART III

**ITEM 10.** ***DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE***

We will file a definitive proxy statement for our 2014 annual meeting of stockholders (the "**Proxy Statement**") not later than 120 days after the fiscal year end of December 31, 2013. The information required by this item is incorporated herein by reference to the information contained in the Proxy Statement.

**ITEM 11.** ***EXECUTIVE COMPENSATION***

The information required by this item is incorporated herein by reference to the information contained in the Proxy Statement.

**ITEM 12.** ***SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS***

The information required by this item is incorporated herein by reference to the information contained in the Proxy Statement.

**ITEM 13.** ***CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE***

The information required by this item is incorporated herein by reference to the information contained in the Proxy Statement.

**ITEM 14.** ***PRINCIPAL ACCOUNTANT FEES AND SERVICES***

The information required by this item is incorporated herein by reference to the information contained in the Proxy Statement.

## PART IV

### ITEM 15. *EXHIBITS AND FINANCIAL STATEMENT SCHEDULES*

**Financial Statements**

Our financial statements are set forth in Item 8 to this Annual Report on Form 10-K.

**Financial Statement Schedules**

Certain schedules are omitted because they are not applicable, or are not required by smaller reporting companies.

**Exhibits**

The exhibits listed on the Exhibit Index beginning on page II-4, which is incorporated herein by reference, are filed or furnished as part of this report or are incorporated into this report by reference.

## SIGNATURES

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

RXi PHARMACEUTICALS CORPORATION

By: / s / GEERT CAUWENBERGH

**Geert Cauwenbergh, Dr. Med. Sc.**
**President, Chief Executive Officer**
**and Chief Financial Officer**

Date: March 28, 2014

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

| Signatures | Title | Date |
|---|---|---|
| / s / GEERT CAUWENBERGH<br>Geert Cauwenbergh, Dr. Med. Sc. | President, Chief Executive Officer,<br>Chief Financial Officer and Director<br>(Principal Executive Officer and Principal Financial Officer) | March 28, 2014 |
| / s / CAITLIN KONTULIS<br>Caitlin Kontulis | Controller and Secretary<br>(Principal Accounting Officer) | March 28, 2014 |
| / s / KEITH L. BROWNLIE<br>Keith L. Brownlie | Director | March 28, 2014 |
| / s / ROBERT J. BITTERMAN<br>Robert J. Bitterman | Director | March 28, 2014 |
| / s / H. PAUL DORMAN<br>H. Paul Dorman | Director | March 28, 2014 |
| / s / CURTIS A. LOCKSHIN<br>Curtis A. Lockshin | Director | March 28, 2014 |

**Exhibits**

## EXHIBIT INDEX

| Exhibit Number | Description | Incorporated by Reference Herein | |
|---|---|---|---|
| | | Form | Date |
| 2.1 | Contribution Agreement, dated as of September 24, 2011, between RXi Pharmaceuticals Corporation (formerly RNCS, Inc.) and Galena Biopharma, Inc. (formerly RXi Pharmaceuticals Corporation). | Amendment No. 1 to the Registration Statement on Form S-1 (File No. 333-177498) | December 8, 2011 |
| 2.2 | Securities Purchase Agreement, dated as of September 24, 2011, among RXi Pharmaceuticals Corporation (formerly RNCS, Inc.), Galena Biopharma, Inc. (formerly RXi Pharmaceuticals Corporation), Tang Capital Partners, LP and RTW Investments, LLC. | Amendment No. 1 to the Registration Statement on Form S-1 (File No. 333-177498) | December 8, 2011 |
| 2.3 | Asset Purchase Agreement, dated March 1, 2013, between RXi Pharmaceuticals Corporation and OPKO Health, Inc. + | Quarterly Report on Form 10-Q (File No. 000-54910) | March 15, 2013 |
| 3.1 | Amended and Restated Certificate of Incorporation of RXi Pharmaceuticals Corporation. | Amendment No. 4 to the Registration Statement on Form S-1 (File No. 333-177498) | February 7, 2012 |
| 3.2 | Certificate of Amendment to the Amended and Restated Certificate of Incorporation of RXi Pharmaceuticals Corporation. | Current Report on Form 8-K (File No. 000-54910) | July 22, 2013 |
| 3.3 | Certificate of Designations, Preferences and Rights of Series A Convertible Preferred Stock of RXi Pharmaceuticals Corporation. | Amendment No. 4 to Registration Statement Form S-1 (File No. 333-177498) | February 7, 2012 |
| 3.4 | Certificate of Designations, Preferences and Rights of Series A-1 Convertible Preferred Stock of RXi Pharmaceuticals Corporation. | Quarterly Report on Form 10-Q (File No. 000-54910) | August 14, 2013 |
| 3.5 | Certificate of Increase, filed with the Secretary of State of the State of Delaware on January 24, 2014. | Current Report of Form 8-K (File No. 000-54910) | January 24, 2014 |
| 3.6 | Amended and Restated Bylaws of RXi Pharmaceuticals Corporation. | Quarterly Report on Form 10-Q (File No. 333-177498) | May 14, 2012 |
| 4.1 | Secured Convertible Promissory Note, dated September 24, 2011 of RXi Pharmaceuticals Corporation (formerly RNCS, Inc.), issued to Tang Capital Partners, LP. | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |
| 4.2 | Secured Convertible Promissory Note, dated September 24, 2011 of RXi Pharmaceuticals Corporation (formerly RNCS, Inc.), issued to RTW Investments, LLC. | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |

| Exhibit Number | Description | Incorporated by Reference Herein | |
|---|---|---|---|
| | | Form | Date |
| 10.1 | Employment Agreement, dated September 24, 2011, between RXi Pharmaceuticals Corporation (formerly, RNCS, Inc.) and Pamela Pavco, Ph.D.* | Current Report on Form 8-K of Galena Biopharma, Inc. (File No. 001-33958) | September 26, 2011 |
| 10.2 | License Agreement between RXi Pharmaceuticals Corporation and Dharmacon, Inc. (now part of Thermo Fisher Scientific Inc.), dated October 29, 2007.(+) | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |
| 10.3 | Non-Exclusive License Agreement, between CytRx Corporation and the University of Massachusetts Medical School, related to UMMS disclosure number 01-36, dated April 15, 2003, as amended February 1, 2004.(+) | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |
| 10.4 | Patent and Technology Assignment Agreement between RXi Pharmaceuticals Corporation (formerly RNCS, Inc.) and Advirna, LLC, effective as of September 24, 2011. | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |
| 10.5 | Lease between RXi Pharmaceuticals Corporation and Newgate Properties, LLC for One Gateway Place, Worcester, Massachusetts 01605, dated September 25, 2007. | Amendment No. 1 to the Registration Statement on Form S-1 of Galena Biopharma, Inc. (File No. 333-147009) | November 19, 2007 |
| 10.6 | Amendment to Lease between RXi Pharmaceuticals Corporation and Newgate Properties, LLC for One Gateway Place, Worcester, Massachusetts 01605, dated January 23, 2009. | Current Report on Form 8-K of Galena Biopharma, Inc. (File No. 001-33958) | January 29, 2009 |
| 10.7 | Amendment to Lease between RXi Pharmaceuticals Corporation and Newgate Properties, LLC for One Gateway Place, Worcester, Massachusetts 01605, dated March 5, 2009. | Quarterly Report on Form 10-Q of Galena Biopharma, Inc. (File No. 001-33958) | May 15, 2009 |
| 10.8 | Amendment to Lease between RXi Pharmaceuticals Corporation and Newgate Properties, LLC for One Gateway Place, Worcester, Massachusetts 01605, dated August 28, 2008. | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |
| 10.9 | Amendment to Lease between RXi Pharmaceuticals Corporation and Newgate Properties, LLC for One Gateway Place, Worcester, Massachusetts 01605, dated November 4, 2008. | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |
| 10.10 | Amendment to Lease between RXi Pharmaceuticals Corporation and Newgate Properties, LLC for One Gateway Place, Worcester, Massachusetts 01605, dated June 9, 2011. | Registration Statement on Form S-1 (File No. 333-177498) | October 25, 2011 |
| 10.11 | RXi Pharmaceuticals Corporation 2012 Long Term Incentive Plan.* | Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177498) | January 23, 2012 |
| 10.12 | Form of Incentive Stock Option Award.* | Registration Statement on Form S-1 (File No. 333-191236 | September 18, 2013 |
| 10.13 | Form of Non-qualified Stock Option Award.* | Registration Statement on Form S-1 (File No. 333-191236 | September 18, 2013 |

| Exhibit Number | Description | Incorporated by Reference Herein | |
|---|---|---|---|
| | | Form | Date |
| 10.14 | Form of Restricted Stock Unit Award.* | Registration Statement on Form S-1 (File No. 333-191236 | September 18, 2013 |
| 10.15 | Amendment to RXi Pharmaceuticals Corporation Long-Term Incentive Plan.* | Registration Statement on Form S-1 (File No. 333-191236 | September 18, 2013 |
| 10.16 | RXi Pharmaceuticals Corporation Employee Stock Purchase Plan.* | Registration Statement on Form S-1 (File No. 333-191236 | September 18, 2013 |
| 10.17 | Form of Indemnification Agreement.* | Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177498) | January 23, 2012 |
| 10.18 | Employment Agreement, dated April 27, 2012, between RXi Pharmaceuticals Corporation and Geert Cauwenbergh, Dr. Med. Sc.* | Current Report on Form 8-K (File No. 333-177498) | May 3, 2012 |
| 10.19 | Securities Purchase Agreement, dated as of March 6, 2013, among RXi Pharmaceuticals Corporation and the purchasers named therein. | Current Report on Form 8-K (File No. 000-54910) | March 7, 2013 |
| 10.20 | Lease Agreement dated December 17, 2013 between RXi Pharmaceuticals Corporation and 257 Simarano Drive, LLC, Brighton Properties, LLC, Robert Stubblebine 1, LLC and Robert Stubblebine 2, LLC | Current Report on Form 8-K (File No. 000-54910) | December 20, 2013 |
| 10.21 | Manufacturing and Distribution Agreement, dated November 14, 2013 between RXi Pharmaceuticals Corporation and Ethicor Pharmaceuticals Ltd.**** + | | |
| 23.1 | Consent of BDO USA, LLP, an Independent Registered Public Accounting Firm.**** | | |
| 31.1 | Sarbanes-Oxley Act Section 302 Certification of Chief Executive Officer and Chief Financial Officer.**** | | |
| 32.1 | Sarbanes-Oxley Act Section 906 Certification of Chief Executive Officer and Chief Financial Officer.**** | | |
| 101 | The following financial information from the Annual Report on Form 10-K of RXi Pharmaceuticals Corporation for the year ended December 31, 2013, formatted in XBRL (eXtensible Business Reporting Language): (1) Balance Sheets as of December 31, 2013 and December 31, 2012; (2) Statements of Operations for the years ended December 31, 2013 and 2012 and for the period from January 1, 2003 (inception) to December 31, 2013; (3) Statements of Convertible Preferred Stock and Statements of Stockholders' Equity for the period from September 24, 2011 to December 31, 2013, Divisional Equity for the period from April 3, 2006 to September 23, 2011 and Parent Company's Net Deficit for the period from January 1, 2003 (date of inception) to December 31, 2006; (4) Statements of Cash Flows for the years ended December 31, 2013 and 2012 and for the cumulative period from January 1, 2003 (inception) to December 31, 2013; and (4) Notes to Financial Statements. | | |

* Indicates a management contract or compensatory plan or arrangement.

**** Filed herewith.

\+ Confidential treatment has been requested or granted for certain portions which have been blanked out in the copy of the exhibit filed with the Securities and Exchange Commission. The omitted information has been filed separately with the Securities and Exchange Commission.

## MANAGEMENT TEAM

**Geert Cauwenbergh, Dr. Med. Sc.**
*President and Chief Executive Officer*

Vice President, Research & Development,
Johnson & Johnson's Skin Research Center

Founder, Chairman & CEO of Barrier Therapeutics, Inc.
(acquired by Stiefel Laboratories, Inc.)

Chairman & CEO of Rhei Pharmaceuticals HK Ltd.

**Pamela Pavco, Ph.D.**
*Chief Development Officer*

VP of Pharmaceutical Development of Galena Biopharma, Inc.

Senior Director, R&D Project Management,
Sirna Therapeutics, Inc., a subsidiary of Merck & Co., Inc.

Responsible for Sirna-027, 1st chemically modified siRNA to enter clinical trials

**Lyn Libertine, M.D.**
*Vice President Medical Affairs & Safety Assessment*

Director Pharmacology of Galena Biopharma, Inc.

Scientist, Critical Therapeutics, DMPK and clinical development for pulmonary & cardiovascular programs

Contributed to development of Zyflo CR®, the only FDA-approved leukotriene synthesis inhibitor for asthma

**Karen Bulock, Ph.D.**
*Vice President Research*

Associate Director Discovery of Galena Biopharma, Inc.

Project Lead for program leading to discovery of RXI-109

Group Leader, Discovery/HTS of Cytrx Corp.

## SCIENTIFIC ADVISORY BOARD

**Craig Mello, Ph.D.**
*Chairman, Scientific Advisory Board*

Co-recipient of the 2006 Nobel Prize in Medicine for RNAi, co-discovered RNAi and co-invented RNAi therapeutics

Blais University Chair in Molecular Medicine at the University of Massachusetts Medical School, a Howard Hughes Investigator and a member of the National Academy of Sciences

**Leroy Young, M.D.**
*Scientific Advisory Board*

Director of the BodyAesthetic Research Center in St. Louis, Missouri

Immediate past President of the Aesthetic Surgery Education and Research Foundation (ASERF)

**Jeannette Graf, M.D.**
*Scientific Advisory Board*

Assistant Clinical Professor of Dermatology at the Mount Sinai School of Medicine

Independent consultant and advisory board member for a number of cosmetic and pharmaceutical companies, including Neutrogena, Johnson & Johnson, RoC, Allergan, Aveeno, Merz/Bioform and Medicis

## BOARD OF DIRECTORS

**Robert Bitterman**
*Chairman of the Board and Chairman of the Compensation Committee*

President & CEO of Cutanea Life Sciences, Inc., a wholly owned subsidiary of Maruho Company, LTD., a specialty pharma development company focused on diseased and aging skin

More than 18 years of executive leadership experience in the pharmaceutical and biologic life science industry

**Keith Brownlie**
*Chairman of the Audit Committee*

Distinguished career with Ernst & Young that spanned 36 years

Held the position of Metro New York Area Life Sciences Industry Leader at Ernst & Young

**H. Paul Dorman**
*Chairman of the Nominating Committee*

Chairman and CEO of DFB Pharmaceuticals, a holding company that has successfully invested and operated multiple pharmaceutical businesses

More than 30 years of executive experience in the pharmaceutical industry

**Curtis Lockshin, Ph.D.**
*Chairman of the Governance Committee*

CEO of Guardum Pharmaceuticals, a wholly owned U.S. subsidiary of OJSC Pharmsynthez

Vice President, Corporate R&D Initiatives, OPKO Health, Inc. with operational responsibilities inside several of OPKO's R&D units

**Geert Cauwenbergh, Dr. Med. Sc.**
*President and Chief Executive Officer*

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**Annual Meeting**
Monday, June 2, 2014
11:00 am
NASDAQ OMX
One Liberty Plaza
165 Broadway
New York, NY 10006

**Transfer Agent**
Computershare Trust Company, N.A.
250 Royall Street
Canton, MA 02021

**Auditor**
BDO USA, LLP
Boston, MA

**Legal Counsel**
Ropes & Gray
San Francisco, CA

**Ticker**
NASDAQ: RXII

**Initial Trading**
May 2012

**Investor Relations**
Tamara McGrillen
508-929-3646
ir@rxipharma.com

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Next Generation in RNAi®

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