RXi Pharmaceuticals Corporation

Jefferies 2013 Global Healthcare Conference

June 6, 2013

OTC: RXII
This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as “believes,” “anticipates,” “plans,” “expects,” “indicates,” “will,” “intends,” “potential,” “suggests” and similar expressions are intended to identify forward-looking statements. These statements are based on RXi Pharmaceuticals Corporation’s (the “Company”) current beliefs and expectations. Such statements include, but are not limited to, statements about the future development of the Company’s products (including timing of clinical trials and related matters associated therewith), the expected timing of certain developmental milestones, the reporting of unblinded data, potential partnership opportunities, the Company’s competition and market opportunity and pro forma estimates. The inclusion of forward-looking statements should not be regarded as a representation by the Company that any of its plans will be achieved. Actual results may differ from those set forth in this presentation due to risks and uncertainties in the Company’s business, including those identified under “Risk Factors” in the Company’s most recently filed Quarterly Report on Form 10-Q and in other filings the Company periodically makes with the U.S Securities and Exchange Commission. 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 presentation.
1. RXi has developed a powerful and proprietary platform of self-delivery RNAi-based therapeutics (sd-rxRNA®) that do not require a delivery vehicle to penetrate cells and tissues
   - Self-delivering technology possesses certain advantages in safety, potency & selectivity
   - Experienced scientific and development team

2. RXI-109, RXi’s lead product, is designed to silence a clinically validated target, Connective Tissue Growth Factor (CTGF), and circumvents many challenges facing other RNAi-based drugs
   - Reduction of CTGF with an antisense oligonucleotide correlated with improved scars in well-controlled clinical trials
   - Delivered locally; avoiding many delivery challenges of other RNA-based drugs
   - Large market opportunity in scar prevention/revision

3. RXi has broad development opportunities based on its RNAi platform
   - Ophthalmology, organ fibrosis, spinal cord injury
   - Platform potential enhanced by recent OPKO Health, Inc. asset purchase
RXi’s Unique RNAi Platform
**sd-rxRNA**
- “self-delivering” compounds can enter cells and tissues to silence, without the need for an additional delivery vehicle
- Has several properties key to the clinical development of RNAi-based drugs (potency, selectivity/specificity, half-life)

**rxRNAori**
- Similarities to classic siRNA, but slightly longer duplex length
- The compound is highly active at picomolar levels *in vitro*
- Modified to increase resistance to nucleases and to prevent off-target effects, including induction of an immune response
• Reduced duplex size of sd-rxRNAs (8 to 16 bp) results in enhanced silencing activity in the absence of a delivery vehicle
  • *in vitro* transfection
  • Length of passenger strand varied from <15 to 19 nucleotides
  • Length of guide strand kept constant

bp = base pairs
sd-rxRNA Combines Features of RNAi and Antisense Technologies

- Single compound designed to not require delivery vehicle
- Robust uptake & silencing in multiple preclinical models
- Structural diversity = novel intellectual property
- Combining many positives of RNAi & antisense, while avoiding many negatives
- Provides for broad pipeline of RNAi drugs for unmet medical needs

sd-rxRNA therapeutic compounds with drug-like properties
sd-rxRNA: Robust Cellular Uptake

in vitro and in vivo

Delivery and silencing demonstrated in many different cell types
Human, Primate, Rat, Mouse, Adherent, Non-adherent, Primary, Transformed

Efficient delivery of sd-rxRNA to multiple tissues in vivo upon local and systemic administration
Selection of Area of Focus: Dermal Scarring
Attractive Therapeutic Opportunity

- **Unmet need with limited competition for truly effective therapies**
  - No prescription drugs approved

- **Validating our pharmacological target**
  - Excaliard (EXC001) - antisense

- **Large market in scar prevention/revision**
  - 42.3 million surgical procedures per year in the U.S.*

<table>
<thead>
<tr>
<th>Type of Skin Surgery</th>
<th>Annual Number of Procedures</th>
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</thead>
<tbody>
<tr>
<td>Cosmetic</td>
<td>6.0 million</td>
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<tr>
<td>Reconstructive</td>
<td>3.9 million</td>
</tr>
<tr>
<td>Moles</td>
<td>3.4 million</td>
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<tr>
<td>Trauma</td>
<td>9.0 million</td>
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<tr>
<td>Elective (office)</td>
<td>9.6 million</td>
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<tr>
<td>Elective (hospital)</td>
<td>10.4 million</td>
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<tr>
<td><strong>TOTAL</strong></td>
<td><strong>42.3 million</strong></td>
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</tbody>
</table>

*US anti-scarring market – The Nemetz Group – December 2009
RXi’s Lead Clinical Product Candidate: RXI-109
Numerous studies implicate Connective Tissue Growth Factor (CTGF) overexpression in scarring and fibrotic diseases.

CTGF-targeting antisense oligo (Excaliard) demonstrated improved scarring (vs. placebo) in 3, well-controlled Phase 2 trials by another company.

RXI-109, an anti-CTGF sd-rxRNA compound, is delivered locally, avoiding delivery challenges of systemic RNA-based drugs.

Preclinical data demonstrate potent, selective, dose-dependent and long-lasting silencing of CTGF with RXI-109.

RXI-109 has a clear development path with early efficacy end points and entered clinical trials in mid-2012.
Connective Tissue Growth Factor
*A Central Factor in the Pathway to Fibrosis*

CTGF
- **Central player in the balance between healthy healing and excessive fibrosis**

**Cellular Effects**
- Collagen Deposition
- Adhesion
- Migration
- Proliferation
- Differentiation

**Pathologic Effects**
- Excessive Fibrosis

**Connective Tissue Growth Factor**

**RXI-109**

**Properties**
- Pulmonary Fibrosis
- Acute Spinal Injury
- Restenosis
- Ocular Scarring
- Liver Fibrosis

**Dermal Anti-Scarring**
RXI-109 Efficiently Silences CTGF in \textit{in vitro} and \textit{in vivo} Preclinical Experiments

**CTGF Silencing \textit{in vitro}**

- A549 cells were treated with RXI-109 and NTC
- Passive uptake 48 hours
- EC50 = 29.4 +/- 6.3 nM

**CTGF Silencing \textit{in vivo} in Rat Skin**

- mRNA levels were quantified by QPCR on Day 8, normalized to the housekeeping gene and set relative to PBS.
- **p=0.0015, ***0.0001 (relative to the dose-matched NTC)
- PBS = Phosphate Buffered Saline (Vehicle Control)
- NTC = Non-Targeting Control sd-rxRNA

---

Day

1

3

8

Excisional Wound

 harvesting
CTGF Silencing Does Not Delay, and May Enhance, Early Wound Healing in a Rodent Model

Silencing of CTGF mRNA

Wound Width

Wound Re-epithelialization
RXI-109 Phase 1 Clinical Trials

(1) Study 1201: Phase 1 single center, randomized, single-dose, double-blind, ascending dose, within-subject controlled study of RXI-109 for the treatment of incision scars

(2) Study 1202: Phase 1 single center, randomized, multi-dose double-blind, ascending dose, within-subject controlled study of RXI-109 for the treatment of incision scars

Parameters evaluated:
- Safety & side effect assessment versus vehicle
- Photographic comparison versus vehicle
- Histological comparison of the scar sites versus vehicle
- Pharmacokinetic parameters after local intradermal injection
• Four injections and incisions (2 cm in length) were made on the abdomen.
• The A and B ‘columns’ were at least 4 cm lateral to the midline of the abdomen.
• Rows were spaced at least 4 cm apart.
• Treatment at each incision site was made by intradermal injection according to a predetermined randomization pattern for each subject.
• Half of the sites were treated with RXI-109, half with placebo.
Monitoring for side effects, safety and toxicity in the 7 days post injection, and regularly afterwards for up to 3 months. Parameters monitored included:

- ECG
- Blood Biochemistry
- Blood Count
- Urinalysis
- Clinical Assessments of Incisions by Physician & Subjects
- RXI-109 in Systemic Circulation after Intradermal Injection

Conclusions:

- No significant side effects nor toxicity were observed.
- Incisions did not show slower healing on the active or the placebo side, i.e. no negative effect on wound healing/closure.
- Blood level of RXI-109 are only 5% of intradermally administered dose.
All Sites: Scatterplots of Averages Per Site

Day 84

Average Wound Area (mm$^2$) Per Site

- RXI-109: 30% (9/30)
- Placebo: 70% (21/30)
- p = 0.22

Average % CTGF Positive Staining Per Site

- RXI-109: 30% (9/30)
- Placebo: 70% (21/30)
- p = 0.02
CTGF Protein Levels of RXI-109 Dosed Sites as a Percent of Placebo Dosed Sites

Day 84

All Sites

Bottom Sites

Paired t-test p-values

<table>
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<tr>
<th>Cohort</th>
<th>All Sites</th>
<th>Bottom Sites</th>
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<tbody>
<tr>
<td>Cohort 1</td>
<td>102%</td>
<td>105%</td>
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<tr>
<td>Cohort 2</td>
<td>97%</td>
<td>100%</td>
</tr>
<tr>
<td>Cohort 3</td>
<td>87%</td>
<td>83%</td>
</tr>
<tr>
<td>Cohort 4</td>
<td>83%</td>
<td>81%</td>
</tr>
<tr>
<td>Cohort 5</td>
<td>100%</td>
<td>80%</td>
</tr>
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Paired t-test p-values

<table>
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<tr>
<th>Cohort</th>
<th>All Sites</th>
<th>Bottom Sites</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cohort 1</td>
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<td>0.54</td>
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<tr>
<td>Cohort 2</td>
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<td>Cohort 3</td>
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<tr>
<td>Cohort 4</td>
<td>0.08</td>
<td>0.13</td>
</tr>
<tr>
<td>Cohort 5</td>
<td>0.05</td>
<td>0.14</td>
</tr>
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</table>
Conclusions Unblinded Phase 1 study (single dose in normal healthy volunteers)

- RXI-109 locally well tolerated & no systemic toxicity
- No negative effect on healing with injection pre-incision
- Minimal uptake in blood after intradermal injection.
- Statistically significant lowering of CTGF protein concentration in RXI-109 treated incisions compared to placebo control \textit{3 months after a single dose} (i.e. potent and long lasting)
- Trend for dose dependent silencing with more pronounced effect at the 2 highest doses.
RXI-109-1202: Still Blinded Abdominal Incision Layout

- 8 injections and incisions (2 cm in length) were made on the abdomen – each incision received 3 injections over a given time period.
- The A and B 'columns' were at least 4 cm lateral to the midline of the abdomen.
- Rows were spaced at least 4 cm apart.
- Treatment at each incision site was made by intradermal injection according to a predetermined randomization pattern for each subject.
- Half of the sites were treated with RXI-109, half with placebo.

<table>
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<tr>
<th>Wound Addresses</th>
<th>Column A (Right)</th>
<th>Column B (Left)</th>
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<tr>
<td>Row 1</td>
<td>1A</td>
<td>1B</td>
</tr>
<tr>
<td>Row 2</td>
<td>2A</td>
<td>2B</td>
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</tbody>
</table>
R and L are on the right and left side of the abdomen, and are treated with either RXI-109 or Placebo - Blinded Data – Code has not been broken
RXI-109-1202: Clinical Pictures and CTGF mRNA Levels of Subject in Cohort 1 (3 days after 3rd and last dose)

Right side and left side are treated with either RXI-109 or Placebo – Blinded Data – Code has not been broken

Right Side

Top Sites

Left Side

Bottom Sites

% CTGF mRNA relative to normal
Two or three randomized, double-blind, within-subject controlled studies

Potential indications include:
- Breast augmentation scar revision
- Hysterectomy scar revision
- Cesarean section scar revision
- Bilateral keloid scar revision

Study objectives include:
- Determination of optimal dose and schedule
- Demonstration of safety and efficacy

Planned initiation in H2 2013
Advancing the Product Pipeline

<table>
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<tr>
<th>Program</th>
<th>Discovery</th>
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<th>Clinical</th>
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<tr>
<td></td>
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<td>Phase 1</td>
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<tr>
<td>Anti-Scarring (RXI-109)</td>
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<tr>
<td>Ophthalmology (PVR)</td>
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<td>Ophthalmology (Macular Degeneration)</td>
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<td>Ophthalmology (Retinoblastoma)</td>
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<tr>
<td>Ophthalmology (Bevasiranib)</td>
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<tr>
<td>Liver disease / Liver fibrosis (RXI-209)</td>
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<tr>
<td>CNS (ALS)</td>
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- **Core focus**
- **Strategic interest**
- **Ophthalmology franchise**
- **Projected next steps**
<table>
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<tr>
<th></th>
<th>sd-rxRNA</th>
<th>Conventional siRNA</th>
<th>Placebo</th>
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<tr>
<td><strong>Mouse</strong></td>
<td></td>
<td></td>
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<tr>
<td>immediately post-dose</td>
<td><img src="image" alt="" /></td>
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<td>Mouse at 24 hours post-dose</td>
<td><img src="image" alt="" /></td>
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<tr>
<td>Mouse at 24 hours post-dose</td>
<td><img src="image" alt="" /></td>
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<tr>
<td>Rabbit at 24 hours post-dose</td>
<td><img src="image" alt="" /></td>
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- Dosing by intravitreal injection to mouse or rabbit eye with Dy547-labeled sd-rxRNA, conventional siRNA or placebo

**sd-rxRNA chemistry is required for robust uptake to the cells of the eye**

**No overt toxicity observed after sd-rxRNA treatment to the eye**
sd-rxRNA: Extended Silencing in vivo in the Rodent Eye

- 3 μg PPIB or NTC administered by intravitreal injection (in 1 μl) to mouse eyes
- mRNA levels were quantified by Quantitative PCR (QPCR) and normalized to β-actin
- Data assembled from 5 different studies to enable sufficient ‘n’ for each data point (n=5-8); graphed +/- SD relative to PBS in each study; * p ≤ 0.05, ** p ≤ 0.01
- PBS = Phosphate Buffered Saline (Placebo)
- NTC = Non-Targeting Control sd-rxRNA
- PPIB = Anti-cyclophilin B sd-rxRNA
Proliferative vitreoretinopathy (PVR) is caused by scar tissue formation within the eye.

Most common complication of a retinal detachment (RD)
- Occurs in approximately 8-10% of patients who develop an RD

Preclinical data strongly support CTGF involvement

A potential Orphan indication with unmet need

Collaboration with Steven Fisher, Ph.D. and Geoff Lewis, Ph.D. (UCSB) to demonstrate efficacy in the rat retina scar model

Potential to file an IND based on the RXI-109 anti-scarring IND with minimal ocular toxicity studies
In Vivo Silencing of CTGF and Uptake in Model of Retinal Detachment

RXI-209 silences CTGF in a normal rat retina 48 hours post intravitreal administration.

fl-RXI-109 (red) is detectable in all retinal layers 24 hours post injection both in the presence and absence of a detachment and at 14 days in a detached retina. Specifically, RXI-109 is taken up by Müller cells and photoreceptors.
VEGF and CTGF sd-rxRNA Combination for Treatment of Ocular Neovascularization/Fibrotic Disorders

- VEGF-targeting sd-rxRNA
  - Dose dependent mRNA silencing of up to 90% with a duration of action through 14 days *in vivo* in rat retina

- Potential treatment either alone or in combination with a CTGF-targeting sd-rxRNA for retinopathy disorders
  - Therapeutics relying on suppression of VEGFA address the neovascularization component of these disorders but not the subsequent retinal scarring that often occurs*
  - Potential indications include proliferative diabetic retinopathy (PDR), wet AMD and others

Retinoblastoma

- Retinoblastoma is a cancer that originates in the retina and primarily affects young children.
- Accounts for about 3% of cancers occurring in children younger than 15 years of age.
- The heritable form is driven by mutations in the retinoblastoma (RB1) gene.
- Expression of genes involved in the “normal” signaling circuitry of retinal cells are also involved.
- The goal is to develop compounds against novel retinoblastoma therapeutic targets.
- Dose-dependent mRNA silencing in retinoblastoma cell lines.
- Uptake by human retinoblastoma cells in vivo in a mouse model.
- Funded by NIH/NCI SBIR grant.
Uptake of sd-rxRNA *in vivo* in Mouse Retina and Tumor Cells 24 hr Post Injection

Mouse eyes were seeded subretinally with Y79 retinoblastoma cells.

10 µg of DY547-labeled sd-rxRNA (red) was administered by intravitreal injection (1µl) 3 weeks after seeding.

Twenty-four hours post injection

a) sd-rxRNA (red) co-localized with tumor cells (green) in the subretinal space

b) sd-rxRNA co-localized with tumor cells in the vitreous

c) sd-rxRNA is visible in the retina
Conversion of Bevasiranib to a “Self Delivering” RNAi Compound

Phase III Clinical Trial of Bevasiranib was initiated in 2007
- NDA not filed with FDA
- Conversion of Bevasiranib into a sd-rxRNA
  - Reduce length of Passenger strand (<15 bp duplex)
  - Identify optimal “self delivering” chemical modification pattern
  - Add hydrophobic conjugate to Passenger strand to enhance cellular penetration

Example sd-rxRNA Bevasiranib Configuration

Passenger Strand
Guide Strand

Key
- ○ = standard ribonucleotide bases
- □ = 2’ deoxyribose bases
- ∧ ∨ = phosphodiester linkages
- = standard and modified bases
- = various hydrophobic moieties
- ∧ ∨ = phosphodiester linkages
- ∧ ∨ = phosphorothioate linkages
- ∧ ∨ = other hydrophobic linkages
Management Team

Geert Cauwenbergh, Dr. Med. Sc. – 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.
- Doctoral degree in Medicine from K.U., Leuven, Belgium

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
- Ph.D. in Biochemistry from Virginia Commonwealth University and post-doctoral work at Duke University

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
- M.D. from University of Massachusetts

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.
- Ph.D. in Pharmacology from Yale University
Governance & Scientific Leadership

- **Robert Bitterman - Board of Directors: Interim Chairman, Chairman of the Compensation Committee and Chairman of the Nominating Committee**
  - President and 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.

- **Keith Brownlie - Board of Directors: Chairman of the Audit Committee and Chairman of Corporate Governance**
  - Distinguished career with Ernst & Young that spanned 36 years where he held the position of Metro New York Area Life Sciences Industry Leader at Ernst & Young.

- **H. Paul Dorman - Board of Directors**
  - Chairman and CEO of DFB Pharmaceuticals, a Fort Worth, TX based holding company that, over the last 20 years, has successfully invested in and operated multiple pharmaceutical businesses.

- **Curtis Lockshin, Ph.D. - Board of Directors**
  - Independent Pharmaceutical & Life Sciences Consultant for OPKO Health, Inc. Prior to this role, Dr. Lockshin served as Vice President, Corporate R&D Initiatives for OPKO Health, Inc., with operational responsibilities inside several of OPKO's R&D units.

- **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 and the 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 and the 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 and an 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.
<table>
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<tr>
<th>Milestone</th>
<th>Goal</th>
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<td>Topline results from RXI-109 single-dose Phase 1 (Study 1201)</td>
<td>Q2 2013</td>
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<tr>
<td>Topline results from RXI-109 multi-dose Phase 1 (Study 1202)</td>
<td>Mid-2013</td>
</tr>
<tr>
<td>Start Phase 2 studies with RXI-109 in scar remodeling</td>
<td>H2 2013</td>
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<td>Partnering activities</td>
<td>2013</td>
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<td>Financial Overview</td>
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<tr>
<td>Cash</td>
<td>~$19.1 million</td>
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<tr>
<td>Burn rate</td>
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<td>Shares outstanding</td>
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<td>a/o 4/30/13</td>
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<tr>
<td>Market Cap</td>
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<tr>
<td>a/o 4/30/13 (excluding preferred shares)</td>
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