



## Developing the next generation of immuno-oncology therapeutics

### RXI Pharmaceuticals Announces Business Strategy to Focus on Immuno-Oncology Programs to Accelerate Growth

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- Company to capitalize on proprietary self-delivering RNAi platform (sd-rxRNA®) that has now been proven to be safe and effective in clinical trials
- Partner/Out-license robust dermatology and ophthalmology franchises to strengthen the Company's balance sheet with non-dilutive funding, as such supporting accelerated growth in immuno-oncology focus area
- Prioritize financial and R&D resources to advance the development of immuno-oncology therapeutics for high unmet medical needs with multi-billion dollar market potential

MARLBOROUGH, Mass., Jan. 16, 2018 (PRNewswire) – RXI Pharmaceuticals Corporation (NASDAQ: RXI) a clinical-stage company developing a new class of RNAi-based therapeutics outlined its 2018 business strategy during a webcast presentation at the Biotech Showcase conference which brought together over 3,500 life science decision makers and investors from over 50 countries to collaborate and discuss industry trends.

Logo - [http://mma.prnewswire.com/media/584748/RXI\\_Pharmaceuticals\\_Corporation\\_Logo.jpg](http://mma.prnewswire.com/media/584748/RXI_Pharmaceuticals_Corporation_Logo.jpg)

"After a thorough review of our business operations, development programs and financial resources, a decision was made to focus our efforts on RXI's expanding and promising immuno-oncology programs to accelerate growth and in turn support a return on investment for our shareholders," said Dr. Geert Cauwenbergh, President and CEO of RXI Pharmaceuticals. He stated that: "The positive clinical results recently announced from our Phase 2 dermatology study validate the safety and efficacy of our sd-rxRNA therapeutics in humans. These outcomes coupled with robust preclinical results in our immuno-oncology program, further affirm the Company's focused development path. With a much-improved regulatory landscape for the development of cancer therapeutics, we believe that there is great potential for our sd-rxRNA compounds to provide major advances that could change the paradigm in cancer treatments. Furthermore, the feedback that we have received, through relationships with leading cancer centers, potential business partners as well as institutional investors, in the context of last week's Biotech Showcase meeting, endorses our sharpened strategy. It is our goal to develop novel immuno-oncology treatments to benefit patients as well as our long-term shareholders and business partners."

#### 2018 Business Strategy

RXI will focus development initiatives on novel immuno-oncology therapeutics using its proprietary sd-rxRNA platform. Focusing the development portfolio on Immuno-oncology, with a near term focus on Adoptive Cell Transfer (ACT), will streamline and reduce our quarterly burn-rate for 2018. The Company is aiming to enter the clinic with at least one of our compounds in the next 18 months, targeting a large multi-billion dollar market. This will be achieved by:

- Accelerating the development of RXI-762 and RXI-804, sd-rxRNA compounds targeting PD-1 and TIGIT respectively, for use in the treatment of solid tumors in the context of ACT. Finalize cGMP manufacturing of lead compound.
- Expanding internal development efforts and external collaborations with various existing cell-based approaches, evaluating hematopoietic stem cells, NK cells, TILs, CAR T, TCR and engineered NK cells.
- Developing immuno-oncology targets beyond checkpoint inhibition, i.e. cell differentiation.
- Expanding our research efforts to further demonstrate that our technology is ideally suited to be combined with existing immune effector cell expansion/manufacturing.

#### Monetize Existing Dermatology and Ophthalmology Franchises:

Relating to the operational review, RXI intends to partner/out-license both its Dermatology and Ophthalmology Franchises. Successfully completing these transactions should provide non-dilutive funding for the Company's focused development path in Immuno-oncology.

Each of these Franchises is comprised of preclinical and clinical-stage assets broadly covered by a robust intellectual property estate. These assets include:

##### Dermatology

- RXI-109: Phase 2 asset for dermal hypertrophic scarring with positive and statistically significant results addressing a USD 1-3B market.
- RXI-231: Topical cosmetic ingredient in a proprietary formulation with positive results reducing a change of skin tone (pigmentation) triggered by UV (p < 0.04) addressing an estimated USD multi-billion dollar market.
- RXI-185: Cosmetic ingredient that reduces collagenase in *in vitro* models. May be developed as a cosmetic to improve the appearance of aging skin.
- Samcyprone™ Phase 2 small molecule topical immuno-therapy, with proven efficacy in cutaneous warts with an estimated market size USD 2-4B.
- Access to the self-delivering platform for human therapeutic and diagnostic use in the field of dermatology, providing access to new compounds targeting proteins of relevance for the treatment of skin diseases.

The intellectual property estate for RXI's Dermatology Franchise is comprised of 14 patent families covering: RXI-109, RXI-231, RXI-185 and Samcyprone™. Importantly, this estate includes 18 granted patents broadly covering the sd-rxRNA platform, including RXI-109 in the US, Europe, Japan and China and Samcyprone™ in the US.

##### Ophthalmology

- RXI-109: Phase 1/2 asset with proven safety, using intra-ocular injections, for wet AMD with estimated market size USD 3-5B. Readouts expected Q1 2018.
- Preclinical data package supporting development of RXI-109 by intravitreal injection or topical application to the eye for retinal or corneal scarring indications.
- Patented set of sequences against a variety of relevant proteins for targeting ocular disorders. Also, sd-rxRNA compounds against targets which may be involved in retinoblastoma or retinal or corneal scarring.
- Preclinical intra-ocular asset (sd-rxRNA targeting VEGF) with dose-dependent effects and tolerability demonstrated in a rodent model.
- Access to the self-delivering platform for human therapeutic and diagnostic use in the field of ophthalmology, providing ready access to new compounds targeting proteins of relevance for the treatment of ophthalmic diseases.

RXI's Ophthalmology intellectual property estate includes 21 patent families. This estate includes 73 patents covering the composition and methods of use the sd-rxRNA platform and targets and sequences from the OPKO assets, including the use of RXI-109 for the treatment of ocular scarring in the US, Japan and China.

The webcast presentation took place on January 8, 2018, and the relating slides and audio may be found on the Company's website, [www.rxpharma.com](http://www.rxpharma.com).

#### About RXI's self-delivering RNAi (sd-rxRNA) technology platform

sd-rxRNA, RXI's proprietary self-delivering RNAi platform, is a single chemically modified compound with delivery and therapeutic properties built directly into the compound itself. The compound is asymmetrical with a phosphorothioate backbone and contains chemical modifications that provide for efficient cellular uptake and gene silencing. These compounds are potent, stable and specific, and demonstrated to be safe and active in a clinical setting.

RXI's novel sd-rxRNA technology differs from natural and most synthetic RNA interference (RNAi) molecules in that they are chemically modified to allow for efficient internalization of the compounds by cells and silencing of the targeted genes. Importantly, unlike other naked siRNA compounds, delivery of sd-rxRNAs are not limited to a specific cell type. For local delivery and ex vivo cell-based therapeutic applications, our compounds do not require delivery vehicles. This is a significant advantage since delivery vehicles can have related toxicity that affects cell viability. sd-rxRNA has demonstrated nearly 100 percent transfection efficiency with high cell viability in numerous cell types.

#### About RXI Pharmaceuticals

RXI Pharmaceuticals Corporation (NASDAQ: RXI) is a clinical-stage company developing innovative therapeutics that address significant unmet medical needs. Building on the pioneering discovery of RNAi, scientists at RXI have harnessed the naturally occurring RNAi process which can be used to "silence" or down-regulate the expression of a specific gene that may be overexpressed in a disease condition. RXI developed a robust RNAi therapeutic platform, including self-delivering RNA (sd-rxRNA®) compounds, that have the ability to highly selectively block the expression of any target in the genome, thus providing applicability to many therapeutic areas. Our current programs include dermatology, ophthalmology, and cell-based immunotherapy. RXI's extensive patent portfolio provides for multiple product and business development opportunities across a broad spectrum of therapeutic areas, and we actively pursue research collaborations, partnering and out-licensing opportunities with academia and pharmaceutical companies. For additional information, visit the Company's website, [www.rxpharma.com](http://www.rxpharma.com).

#### Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements about: our ability to successfully develop RXI-109, Samcyprone™, RXI-762, RXI-804 and our other product candidates (collectively "our product candidates"); the future success of our clinical trials with our product candidates; the timing for the commencement and completion of clinical trials; our ability to enter into strategic partnerships and the future success of these strategic partnerships; and our ability to deploy our sd-rxRNA® technology through partnerships, as well as the prospects of these partnerships to provide positive returns. Forward-looking statements about expectations and development plans of RXI's product candidates and partnerships involve significant risks and uncertainties, including the following: risks that we may not be able to successfully develop and commercialize our product candidates; risks that product development and clinical studies may be delayed, not proceed as planned and/or be subject to significant cost over-runs; risks related to the development and commercialization of products by competitors; risks related to our ability to control the timing and terms of collaborations with third parties; and risks that other companies or organizations may assert patent rights preventing us from developing or commercializing our product candidates. Additional risks are detailed in our most recent Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q under the caption "Risk Factors." Readers are urged to review these risk factors and to not act in reliance on any forward-looking statements, as actual results may differ from those contemplated by our forward-looking statements. RXI does not undertake to update forward-looking statements to reflect a change in its views, events or circumstances that occur after the date of this release.

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