# RXi Pharmaceuticals Announces the Initiation of a Phase 1/2 Trial in Ophthalmology with RXI-109 for Retinal Scarring

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MARLBOROUGH, Mass., Nov. 5, 2015 /PRNewswire/ -- RXi Pharmaceuticals Corporation (NASDAQ: RXII) a biotechnology company focused on discovering and developing innovative therapies primarily in the areas of dermatology and ophthalmology today announced the initiation of a Phase 1/2 clinical trial in ophthalmology. The clinical study RXI-109-1501 will evaluate the safety and clinical activity of RXI-109 to prevent the progression of retinal scarring, a harmful component of numerous retinal diseases.

"We are very pleased to have initiated our first clinical trial in the area of ophthalmology. Currently, there is no effective way to prevent the formation or progression of retinal scars that may occur as a consequence of several devastating ocular diseases. In advanced neovascular or 'wet' age-related macular degeneration, our first area of study, retinal scarring can result in continued vision loss," said Dr. Pamela Pavco, Chief Development Officer of RXi Pharmaceuticals. She further added, "RXI-109 has the potential to fill this unmet medical need by reducing this continuing damage to the retina and, in doing so, helping to preserve vision in these individuals for a longer period of time."

RXI-109-1501 is a multi-center, multi-dose, dose escalation trial conducted in subjects with advanced neovascular or 'wet' age-related macular degeneration (AMD). In this Phase 1/2 trial, each subject will receive a total of four doses of RXI-109 at one month intervals. RXI-109 will be administered by intravitreal injection in one eye only. The dosing period (3 months) will be followed by a four month observation period. The safety and tolerability of RXI-109, as well as the potential for clinical activity, will be evaluated over the course of the study using numerous assessments to monitor ocular health and visual acuity. Several dose levels will be evaluated in a small number of subjects in this first trial in order to establish safety information and to help determine the dosing regimen for continued study.

RXI-109 is a self-delivering RNAi (sd-rxRNA®) compound developed to target connective tissue growth factor (CTGF), a key regulator of scar formation in the skin and known to be involved in retinal scarring as well. Wet AMD is currently treated with anti-VEGF therapies to block vascular endothelial growth factor (VEGF) from causing blood vessel leakiness and the consequential damage to the retina. However, as the disease progresses, many advanced patients also experience retinal scarring which leads to further vision loss. Our ultimate goal is to reduce the scarring that is secondary to advanced wet AMD and in doing so, preserve vision for a longer period time.

Currently, there are no approved therapeutics in the U.S. for the treatment and prevention of subretinal fibrosis. Such a therapy could benefit patients with the advanced wet AMD as well as those with other ocular indications with a scarring component such as proliferative vitreoretinopathy (PVR) and proliferative diabetic retinopathy (PDR).

**About RXI-109: Novel Self-delivering RNAi (sd-rxRNA®) Compound with Built-In Drug-Like Properties**

Building on the pioneering work of RXi's Scientific Advisory Board Chairman and Nobel Laureate Dr. Craig Mello, scientists at RXi developed novel sd-rxRNA compounds where drug-like properties are built into the RNAi compound itself. sd-rxRNA compounds are novel RNAi compounds with enhanced properties for therapeutic use including: efficient spontaneous cellular uptake, stability *in vivo*, reduced potential for immune stimulation, and potent, long-lasting intracellular activity. All cell types tested (primary, neuronal and non-adherent) internalize sd-rxRNA compounds uniformly and efficiently, resulting in potent and long lasting silencing. Efficient cellular uptake is observed both *in vitro* and *in vivo*, including tissues like skin, retina, lung, spinal cord and liver.

RXI-109, an sd-rxRNA compound, is the Company's first clinical development candidate. RXI-109 silences connective tissue growth factor (CTGF), which plays a key role in tissue regeneration and repair and is initially being developed to reduce or inhibit scar formation in the skin and in the eye.

In addition to the Phase 1/2 trial in ophthalmology described here, RXI-109 is also currently being evaluated in Phase 2a clinical trials in dermatology. Approximately 100 subjects have been treated with RXI-109 by intradermal injection in all trials to date. Multiple intradermal injections were well tolerated at all dose levels. RXI-109 was shown to cause a dose-dependent silencing of CTGF messenger RNA and protein levels in the treated areas of the skin compared to placebo and preliminary results from Phase 2a studies indicate a clinical effect on scar appearance.

**About RXi Pharmaceuticals Corporation**

RXi Pharmaceuticals Corporation (NASDAQ: RXII) is a biotechnology company focused on discovering and developing innovative therapeutics primarily in the areas of dermatology and ophthalmology that address high-unmet medical needs. Our discovery and clinical development programs are based on siRNA technology as well as immunotherapy agents. These compounds include, but are not limited to, our proprietary, self-delivering RNAi (sd-rxRNA®) compounds for the treatment of dermal and ocular scarring. It also includes an immunomodulator, Samcyprone™, a proprietary topical formulation of diphenylcyclopropenone (DPCP), for the treatment of disorders such as warts, alopecia areata, non-malignant skin tumors and cutaneous metastases of melanoma.

RXi's robust pipeline, coupled with an extensive patent portfolio, provides for product and business development opportunities across a broad spectrum of therapeutic areas. We are committed to being a partner of choice for academia, small companies, and large multinationals. We welcome ideas and proposals for strategic alliances, including in- and out-licensing

opportunities, to advance and further develop strategic areas of interest. Additional information may be found on the Company's website, www.rxipharma.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™ 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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