



RXi Pharmaceuticals Announces Remarkable and Positive Results with its Novel Self-Delivering RNAi Platform: Potent Reduction of Long Non-coding RNAs Demonstrated in Collaboration with Biogazelle

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MARLBOROUGH, Mass., Oct. 20, 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 positive results from a collaboration with Biogazelle NV. Biogazelle is a company with a longstanding track record and internationally recognized expertise in RNA gene expression analysis, with a particular focus on non-coding RNA, such as microRNAs and long non-coding RNAs (lncRNA).

Specifically-designed sd-rxRNAs based on RXi's proprietary, self-delivering RNAi platform demonstrated robust and potent reduction of the levels of lncRNAs in a target specific manner. These findings expand the number of potential genes RXi is capable of targeting by approximately four fold.

Dr. Craig Mello, Chairman of RXi Pharmaceuticals' Scientific Advisory Board and co-recipient of the 2006 Nobel Prize in Medicine for RNAi, said that, "This discovery is a significant advancement for RXi's proprietary self-delivering RNA platform. lncRNAs represent a diverse class of promising therapeutic targets. Currently, researchers must use multiple oligonucleotide platforms to reduce expression of lncRNAs depending on the subcellular localization of the target. Biogazelle's findings suggest that RXi's technology can efficiently silence lncRNAs regardless of their subcellular localization."

lncRNAs are a diverse class of non-protein coding RNA molecules that are greater than 200 nucleotides in length. lncRNAs are involved in crucial cellular processes, including the regulation of gene expression, and have been implicated in many diseases and disorders, including cancer, cardiovascular diseases, neurological disorders, diabetes, and HIV.

"When we began the collaboration with RXi, we were intrigued by their self-delivering RNAi technology, which offers a clear advantage over conventional siRNAs. Many lncRNAs are believed to be primarily located in the nucleus and considering siRNAs are not expected to reduce the expression of nuclear lncRNAs, we did not believe the platform would be competitive with other nucleic acid-based technologies, such as antisense oligonucleotides," said Professor Jo

Vandesompele, CSO of Biogazelle. He further added that, "Remarkably, the sd-rxRNA platform demonstrated potent and target-specific silencing of multiple lncRNAs, including lncRNAs that are strictly localized in the nucleus, such as MALAT1. We're considering RXi's self-delivering platform for therapeutic applications in the future. "

"We greatly appreciate the thorough work that Biogazelle has done in screening our sd-rxRNA compounds against lncRNA targets and we look forward to working with Biogazelle in the future," said Dr. Geert Cauwenbergh, President and CEO of RXi Pharmaceuticals. He added that, "These results constitute a major boost to the value of our self-delivering platform by significantly expanding the breadth of therapeutic targets we can now pursue. This opens up additional business development and partnering opportunities with our sd-rxRNA platform, further supporting RXi's initiatives to build shareholder value."

About lncRNA

Following the sequencing of the mammalian genome, ~20,000 protein-coding genes were identified. At that time 99% of the genome was thought to contain non-functional and repetitive sequences. More recently, researchers utilizing transcriptome profiling approaches have discovered that ~60,000 of these non-functional sequences of the genome are transcribed into long non-coding RNAs (lncRNAs), many of which are functional. Long non-coding RNAs, containing >200 nucleotides, were found to function in various biological processes, such as cell proliferation, differentiation and the regulation of gene expression. Importantly, dysregulation of lncRNA expression has been shown to be associated with the progression of many diseases including cancer, cardiovascular diseases, neurological disorders, diabetes and HIV. Therefore, targeting specific lncRNAs may result in therapeutic benefit.

About sd-rxRNA®: Novel RNAi Compounds with Built-In Drug-Like Properties

Scientists at RXi have used an alternative approach to delivery, where drug-like as well as delivery 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 effective silencing. Efficient cellular uptake is observed *in vitro* and, following direct administration *in vivo*, into tissues such as skin, retina, lung, and spinal cord.

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 scar formation. 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.

Building on the pioneering work of RXi's Scientific Advisory Board Chairman and Nobel Laureate Dr. Craig Mello, the Company's first RNAi product candidate, 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. RXI-109 is currently being evaluated in Phase 2a clinical trials in dermatology and a Phase 1/2 trial is planned to initiate this year in ophthalmology.

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.

About Biogazelle

Biogazelle is a Ghent University spin-off founded in 2007 by Professor Jo Vandesompele and Dr. Jan Hellemans. It is focused on the development of RNA-targeted therapeutics and has proprietary knowledge to prioritize candidate therapeutic RNA targets in specific cancer types, building on years of expertise in exploring the human transcriptome. Therapeutic inhibition of candidate lncRNA targets are evaluated in high-throughput in vitro RNA perturbations and in preclinical animal models.

Biogazelle also offers expert laboratory services (with ISO 17025 accreditation) in the area of RNA, biomarker discovery and diagnostics.

Being at the forefront of the genomics revolution, Biogazelle is convinced that unraveling the coding and non-coding regions of the genome will lead to RNA-targeted therapeutics and biomarkers for better healthcare.

More information can be found on their website at www.biogazelle.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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