



May 3, 2016

RXi Pharmaceuticals and Thera Neuropharma Enter into an Exclusive License Agreement for RXi's Self-Delivering RNAi (sd-rxRNA®) Platform Targeting SOD1 to Develop Therapeutics for Neurodegenerative Diseases, such as ALS (Lou Gehrig's Disease)

MARLBOROUGH, Mass., May 3, 2016 /PRNewswire/ -- RXi Pharmaceuticals Corporation (RXi) (NASDAQ: RXII) and privately-held Thera Neuropharma, Inc. (Thera) announced today that they have entered into an exclusive license agreement for RXi's novel and proprietary sd-rxRNA® platform to develop therapeutics for neurodegenerative diseases.

Under the terms of the agreement, Thera will be responsible for all research, development, manufacturing, regulatory and commercialization activities for the licensed products. The initial focus of the agreement will be on sd-rxRNA compounds targeting superoxide dismutase 1 (SOD1) for use in developing innovative treatments for amyotrophic lateral sclerosis (ALS), commonly known as Lou Gehrig's disease. Furthermore, there are potential synergies between RXi's SOD1 sd-rxRNA compounds and Thera's small molecule regenerative therapeutics (SMRT), to target multiple factors involved in the pathogenesis of ALS. During the past several months, the companies have jointly filed provisional patents on the mechanism of action of the combined compounds for the management of neurodegenerative diseases associated with SOD1.

"We are pleased to announce this licensing deal with Thera for our SOD1 targeting sd-rxRNA compounds," said Dr. Geert Cauwenbergh, President and CEO of RXi Pharmaceuticals. He added that, "Building on the early work that was done at RXi in collaboration with Dr. Robert Brown, Chair of the Department of Neurology at the University of Massachusetts Medical School, we are happy to provide Thera with access to our robust self-delivering RNAi technology platform to develop therapeutics targeting SOD1. Thera has a management team and Scientific Advisory Board with a deep knowledge of ALS in particular, and neurodegenerative diseases in general, that well positions the company for success."

Dr. Antonella Favit-VanPelt, President and CEO of Thera Neuropharma echoes Dr. Cauwenbergh's remarks highlighting the breakthrough nature of the deal. "We are excited about the opportunity to leverage RXi's and Thera's technologies and strengthen significantly

our development efforts in CNS. Tackling ALS and other neurodegenerative diseases with RNA-based and small molecule therapeutic approaches,” added Dr. Favit-VanPelt, “dramatically increase the possibility to bring tangible and decisive therapeutic interventions to ALS patients and others with incurable neurodegenerative disorders.”

RXI’s novel and proprietary self-delivering RNAi (sd-rxRNA®) compounds are designed with unique features that result in robust cellular uptake and target-specific mRNA reduction. RXI’s most advanced sd-rxRNA compound, RXI-109, is currently in Phase 2 human clinical trials for dermal scarring and Phase 1/2 human clinical trials for subretinal fibrosis associated with late stage age-related macular degeneration.

RXI’s SOD1-targeting sd-rxRNA compounds share similar cellular uptake features with RXI-109 and have been found to successfully enter the cells of the spinal cord and brain when administered by intrathecal injection in rodent. SOD1 protein misfolding is one of the key contributing factors associated with ALS progression and reduction of SOD1 levels by sd-rxRNA treatment could provide therapeutic benefits. Thera Neuropharma’s broad-spectrum neuroprotective agents are small molecule compounds shown to be well tolerated *in vivo* and designed to up-regulate critical cell functioning pathways. Thera’s clinical candidates have a direct effect on protecting motor neurons against protein misfolding and oxidative toxicity. Their neuroprotective and regenerative effects are achieved by inducing axonal regeneration, thus restoring the viability of the neuronal network and activating modulatory/compensatory mechanisms that delay progression of ALS symptoms.

About Amyotrophic Lateral Sclerosis (ALS)

ALS, sometimes called Lou Gehrig's disease, is a rapidly progressive, invariably fatal neurological disease that attacks the nerve cells (neurons) responsible for controlling voluntary muscles (muscle action we are able to control, such as those in the arms, legs, and face). The disease belongs to a group of disorders known as motor neuron diseases, which are characterized by the gradual degeneration and death of motor neurons. More than 12,000 people in the U.S. have a definite diagnosis of ALS, for a prevalence of 3.9 cases per 100,000 persons in the U.S. general population, according to a report on data from the [National ALS Registry](#). ALS is more common among white males, non-Hispanics, and persons aged 60–69 years, but younger and older people also can develop the disease. Men are affected more often than women. ALS causes weakness with a wide range of disabilities. Eventually, all muscles under voluntary control are affected, and individuals lose their strength and the ability to move their arms, legs, and body. When muscles in the diaphragm and chest wall fail, people lose the ability to breathe without ventilatory support. Most people with ALS die from respiratory failure, usually within 3 to 5 years from the onset of symptoms. However, about 10 percent of those with ALS survive for 10 or more years.

About Thera Neuropharma

Thera Neuropharma is a wholly owned affiliate of Synaerion Therapeutics, a privately held biotechnology company founded by Dr. Antonella Favit-VanPelt (President & CEO) and Mr. Guy

Maestre (COO). Thera is developing a new class of disease-modifying small molecules for the treatment of neurologic disorders, initially focusing on Amyotrophic Lateral Sclerosis (ALS) and eventually expanding to traumatic brain injury (TBI), Alzheimer's disease (AD) and multiple sclerosis (MS). Our technology, licensed from Southern Research in Birmingham, AL, is the first and only small molecule regenerative therapy (SMRT) technology that uncovers the therapeutic potential of a dual-target approach that leverages the direct activation of the nuclear factor - κ B p65 (NF- κ B p65) subunit and the increase of manganese superoxide dismutase (MnSOD) expression. Thera's small molecules appear to be effective through the simultaneous mitigation of a) oxidative toxicity, b) cell dysfunction, and c) neurotransmission deficit. The multifactorial effect of our compounds translated in the animal model through symptomatic improvement (over 50% reduction in weight loss and over 65% improved neurologic scores), delayed disease progression (higher than 60% permanence at milder disease stages), and increased survival rate: (over 50% increase survival in SOD1 G93A animals treated at disease onset). Thera's broad-spectrum neuroprotective and regenerative compounds exert their potential disease modification effects by protecting neurons against cell toxicity, inducing brain network regeneration, and maintaining functionality by delaying the progression of symptoms of ALS and TBI. Thera has established a number of partnerships to advance our discovery and research program. Thera is also implementing a strong discovery program and we have established important collaborations with Southern Research, Alabama, and the Vlaams Instituut voor Biotechnologie, Belgium, to advance research on our technology platform and small molecule portfolio. Thera offers a unique investment opportunity and we welcome parties interested in investing, strategic partnerships, and collaborations. For more information on Thera Neuropharma, visit our website at www.theraneuro.com

About RXi's Proprietary Self-delivering RNAi (sd-rxRNA[®]) Platform

Building on the pioneering work of Dr. Craig Mello, Nobel Laureate and RXi's Scientific Advisory Board Chairman, scientists at RXi developed novel sd-rxRNA compounds where drug-like properties are built into the RNAi compound itself. These proprietary compounds are novel RNAi compounds with enhanced properties for therapeutic use including: efficient spontaneous cellular uptake, stability, 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 into tissues such as skin and retina following local administration, and liver following systemic delivery. RXI-109, an sd-rxRNA compound is designed to silence connective tissue growth factor (CTGF), which plays a key role in tissue regeneration and repair. RXI-109 is currently being evaluated in two clinical trials. A Phase 1/2 clinical study, RXI-109-1501, is underway to evaluate the safety and clinical activity of RXI-109 to prevent the progression of retinal scarring, a harmful component of numerous retinal diseases. In addition, a Phase 2 clinical trial, RXI-109-1402, is currently evaluating RXI-109 as a treatment to reduce the recurrence of hypertrophic scars following scar revision surgery. Over 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 clinical-stage RNAi company developing innovative therapeutics in dermatology and ophthalmology that address significant unmet medical needs. Building on the pioneering work of RXi's Scientific Advisory Board Chairman and Nobel Laureate Dr. Craig Mello, our discovery and clinical development programs are based on our proprietary RNAi (sd-rxRNA) platform and Samcyprone™, a topical immunomodulator. Our clinical development programs include RXI-109, an sd-rxRNA, for the treatment of dermal and ocular scarring, and Samcyprone™ for the treatment of such disorders 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 multiple 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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