



Developing the next generation of immuno-oncology therapeutics

BioAxone BioSciences Awarded \$1.1 Million Phase 2 SBIR Funding for Continued Development of sd-rxRNA Drug Candidate for the Treatment of Spinal Cord Injury

June 28, 2018

Award based on accomplishment of 1-year milestones; Collaborator RXI Pharmaceuticals to receive a portion of the allotted funding

CAMBRIDGE, Mass., & MARLBOROUGH, Mass.—(BUSINESS WIRE)—Jun. 28, 2018—BioAxone BioSciences, Inc. announced today that, as part of a collaborative effort with RXI Pharmaceuticals Corporation (NASDAQ: RXII), it is the recipient of a grant award from the National Institute of Neurological Disorders and Stroke (NINDS), part of the agency's SBIR Phase II funding program. This two-year grant provides funding for further development of BioAxone's preclinical candidate BA-434, a novel sd-rxRNA[®] compound that targets PTEN for the treatment of spinal cord injury (SCI).

Unlike other SCI drugs in clinical development that target growth inhibition in the central nervous system (CNS), BA-434 targets intrinsic barriers to axon regeneration. After neurotrauma, neurons in the central nervous system do not spontaneously regenerate their injured axons. Intrinsic barriers to regeneration refer to the diminished ability of adult CNS neurons to regenerate. PTEN is known to be an intrinsic barrier to regeneration and BA-434 uses RXI's self-delivering RNA interference therapeutic platform to block PTEN expression.

"This award reinforces the need for new therapeutics to address a critical unmet need for patients with spinal cord injuries," said Lisa McKerracher, PhD, CEO, BioAxone BioSciences. "We appreciate NINDS' recognition of the importance of our technology and the progress we have made to date."

In September 2017, BioAxone was awarded a total of \$1,794,895 to fund the collaborative project over 24 months. To date the project has received \$735,822 and today the remaining \$1,059,073 was awarded based on accomplishing drug development milestones. For their contribution, RXI received \$128,838 in the first year and is to receive an additional \$116,800.

Under this grant, entitled "Development of self-delivering RNAi targeted to PTEN for treatment of spinal cord injury," BA-434 will be further developed to silence PTEN, a protein known to be an intrinsic barrier to regeneration, thereby supporting regeneration in the adult central nervous system.

"We are very pleased with the advancements that BioAxone has achieved using our proprietary therapeutic platform in the development of potential therapeutics for spinal cord injuries," said Dr. Karen Bullock, Vice President of Research at RXI Pharmaceuticals. She further added, "Our team looks forward to continued collaboration with BioAxone in this important research supporting the development of BioAxone's preclinical candidate BA-434, a novel sd-rxRNA compound for the treatment of spinal cord injury."

The Small Business Innovation Research (SBIR) program was created by the U.S. Congress to strengthen the role of small, innovative companies in federally supported research and development. It is one of the largest sources of early-stage technology financing in the U.S. The National Institute of Neurological Disorders and Stroke (NINDS) is the nation's leading funder of research on the brain and nervous system and a component of the National Institutes of Health (NIH). Research reported in this publication was supported by the NINDS of the NIH under Award Number R44NS084489. The content is solely the responsibility of the authors and does not necessarily represent the official views of the National Institutes of Health.

About BioAxone BioSciences

BioAxone BioSciences is a clinical-stage biotechnology company based in Cambridge, MA, and is developing innovative drugs to restore neurological function for patients with spinal cord injuries and neurovascular disorders. The company also has preclinical programs in restoring epithelial barriers in gastrointestinal disease and in glaucoma. BioAxone is led by a team of scientists renowned for their work on axon regeneration, diseases involving RhoROCK signaling and cell barrier function. For more information, visit <http://www.bioaxonebio.com> and follow the company on Twitter at [@BioAxone](https://twitter.com/BioAxone).

About RXI Pharmaceuticals

RXI Pharmaceuticals Corporation (NASDAQ: RXII) is a biotechnology company developing the next generation of immuno-oncology therapeutics based on its self-delivering RNAi (sd-rxRNA[®]) therapeutic platform. The Company's discovery and research efforts are focused on developing sd-rxRNA therapeutic compounds to be used with an Adoptive Cell Transfer (ACT) approach. This process uses immune cells, such as T-lymphocytes that are isolated from the patient or retrieved from allogeneic immune cell banks, and then expanded and in some cases processed to express tumor-binding receptors. Our approach introduces a new and important step in ex-vivo processing of the immune cells where sd-rxRNA is used to eliminate the expression of immunosuppressive receptors or proteins from the therapeutic immune cells, making them less sensitive to tumor resistance mechanisms and thus improving their ability to destroy the tumor cells. Essentially, we aim to maximize the power of our sd-rxRNA therapeutic compounds by weaponizing therapeutic immune effector cells to attack cancer and ultimately provide patients battling terminal cancers with a powerful new treatment option that goes beyond current treatment modalities. For additional information, visit the Company's website, www.rxi-pharma.com and follow the company on Twitter at [@RXIPharma](https://twitter.com/RXIPharma).

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 expectation regarding closing of the offering, 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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