



Developing the next generation of immuno-oncology therapeutics

RXI Pharmaceuticals Announces Publication in Molecular Therapy on Self-Delivering RNAi (sd-rxRNA®) Targeting PD-1 in Adoptive Cell Therapy for the Treatment of Malignant Melanoma

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- **Scientists demonstrate the potential of improving therapy with patient-derived tumor infiltrating lymphocytes (TILs) by applying RXI's novel sd-rxRNA compounds specific to PD-1. Targeting the PD-1/PD-L1 axis can enhance the ex vivo expansion rate and in vivo longevity and functionality of these T-cells and thereby have the potential to improve Adoptive Cell Therapy (ACT) outcomes in cancer patients.**
- **sd-rxRNA compounds are based on a proprietary therapeutic platform developed and owned by RXI Pharmaceuticals and are ideally suited to reprogram immune cells used in various forms of ACT.**

MARLBOROUGH, Mass., April 16, 2018 /PRNewswire/ -- RXI Pharmaceuticals Corporation (NASDAQ: RXII), a biotechnology company developing the next generation of immuno-oncology therapeutics based on its proprietary self-delivering RNAi (sd-rxRNA®) therapeutic platform, announced today that research conducted using its self-delivering RNAi platform in the field of immunotherapy to treat cancer was published in a leading peer-reviewed journal. *Molecular Therapy* published the paper today, "[Self-Delivering RNAi \(sd-rxRNA®\) Targeting PD-1 Using Adoptive Cell Therapy Approach for the Treatment of Malignant Melanoma](http://www.cell.com/molecular-therapy-family/molecular-therapy/abstract/S1525-0016(18)30172-2)."

Logo: http://mma.prnewswire.com/media/594749/RXI_Pharmaceuticals_Corporation_Logo.jpg

The paper describes research done to evaluate the potential of improving TIL therapy by applying RXI's novel sd-rxRNA compounds specific to PD-1. The results show that targeting the PD-1/PD-L1 axis can enhance the ex vivo expansion rate and in vivo longevity of patient derived TILs used for ACT in malignant melanoma patients. Transfection of these T-cells with the PD-1 targeting self-delivering sd-rxRNA compounds was easy to incorporate in an established, clinically relevant rapid expansion protocol and resulted in increased functionality of the transfected TILs against autologous tumors as compared to control TIL.

Rolf Kiessling, MD, PhD, Senior Professor in Experimental Oncology at the Karolinska Institutet, Senior Chief Physician at the Oncology clinic at the Karolinska University Hospital and member of RXI's Scientific Advisory Board stated: "TIL expanded in the presence of PD-1 specific sd-rxRNA performed with increased functionality against autologous tumors as compared to control TIL. This method of introducing RNAi into T cells to modify the expression of proteins could easily be adopted into any ACT protocol and will lead to the exploration of new combination therapies."

"These results are of direct clinical relevance to ACT for treatment of cancer and further support the broad applicability of the sd-rxRNA platform in multiple cell types," said Dr. Karen Bulock, Vice President of Research at RXI Pharmaceuticals. She further added, "We are continuing to expand our internal development efforts and external collaborations to explore our platform in various existing cell-based ACT approaches including TILs, CAR T, TCR, NK and engineered NK cells."

Immunotherapy of cancer has become increasingly important in clinical practice over the recent decade. By activating the patient's immune system, immunotherapy treatments have shown remarkable promise in extending the lifespan of previously untreatable cancer patients. ACT is an emerging immunotherapy approach which uses immune cells, such as T-lymphocytes or NK cells 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.

A new and important step in this ex-vivo processing of the immune cells is in development where self-delivering RNAi compounds (sd-rxRNA®) are 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 tumor cells. In this way, sd-rxRNA therapeutic compounds can be used to weaponize therapeutic immune cells to attack cancer and ultimately provide patients battling terminal cancers with a powerful new treatment option that goes beyond current treatment modalities.

"Self-Delivering RNAi (sd-rxRNA®) Targeting PD-1 using Adoptive Cell Therapy Approach for the Treatment of Malignant Melanoma" may be accessed on *Molecular Therapy's* website: [http://www.cell.com/molecular-therapy-family/molecular-therapy/abstract/S1525-0016\(18\)30172-2](http://www.cell.com/molecular-therapy-family/molecular-therapy/abstract/S1525-0016(18)30172-2)

About RXI Pharmaceuticals

RXI Pharmaceuticals Corporation (NASDAQ: RXII) is a biotechnology company developing immuno-oncology therapeutics based on its self-delivering RNAi (sd-rxRNA®) therapeutic platform. 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 sd-rxRNA compounds, that has the potential to highly selectively block the expression of any target in the genome, thus providing applicability to many therapeutic areas. 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.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 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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