Scholar Rock Announces Collaboration with Johnson & Johnson Innovation to Discover and Develop Novel Biologics for the Treatment of Autoimmune Diseases and Cancer

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Company's first drug discovery partnership to target growth factor signaling in the immune system microenvironment with proprietary niche activators

CAMBRIDGE, Mass., Jan. 8, 2014 – Scholar Rock, Inc., a biotechnology company focused on discovering and developing novel therapeutics for targeting the disease microenvironment, announced today that it has signed a research collaboration with Johnson & Johnson Innovation and its affiliate Janssen Biotech, Inc. to discover and develop novel biologic therapeutics that regulate signaling in the immune system by targeting the growth factor, TGF-beta 1.

"We are delighted to collaborate with Johnson & Johnson Innovation and Janssen Biotech to discover and develop novel biologics that target growth factor signaling in the immune system microenvironment, with the potential to have therapeutic effects specifically at the source of disease," said Nagesh Mahanthappa, PhD, Chief Executive Officer of Scholar Rock. "Although TGF-beta 1 has been actively pursued as a drug target, most current approaches encounter challenges in triggering systemic effects. Our technology is designed to take a new approach by targeting TGF-beta 1 niche activators locally."

The research collaboration will focus on the discovery and development of novel biologics, called niche activators, to target TGF-beta 1 in the immune system microenvironment and offer a potential new therapeutic approach for autoimmune diseases and cancer immunotherapies. Immunologists from the Johnson & Johnson Innovation Center and Janssen Biotech will work with Scholar Rock to therapeutically target the TGF-beta 1 activation mechanism by applying Scholar Rock's proprietary technology platform. This platform builds upon the recent discoveries of Scholar Rock co-founder, Dr. Timothy A. Springer, that have elucidated molecular mechanisms of growth factor activation in tissue microenvironments.

"We envision development of niche activators as a broad class of therapeutics with novel mechanisms of action," said Timothy A. Springer, PhD, co-founder of Scholar Rock and Latham Family Professor at Harvard Medical School and Professor of Medicine at Children's Hospital Boston. "This collaboration exploits new understanding of growth factor biology in the microenvironment in order to create novel therapeutics with the potential to maximize action at the site of disease, and offer a new approach for debilitating diseases with limited treatment options."

Under the agreement, Janssen Biotech, Inc. obtains a worldwide exclusive option to license, develop and commercialize biologic therapeutics resulting from the alliance with Scholar Rock. Scholar Rock will receive research support, and is eligible to receive option payments, pre-clinical, clinical and regulatory milestone payments. Scholar Rock may also receive royalties and commercial milestones on product sales for each product advanced.

About Niche Activators of Growth Factors
Scholar Rock's therapeutics are designed to target niche activators of protein growth factors that are uniquely present in the microenvironment of specific types of cells and tissues. Niche activators are able to result in highly specific therapeutic effects for growth factor regulation at the site of disease, which is distinct from traditional approaches that systemically target a growth factor and shut down its harmful function in disease but can also cause side effects on normal functions that depend on the same growth factor. Protein growth factors have been widely recognized as disease targets because of their fundamental roles in regulating cell growth and differentiation, but recent discoveries deepen the understanding of activation mechanisms in the extracellular and cell surface microenvironments that enable improved therapeutic strategies.

About Scholar Rock
Scholar Rock, LLC, together with its wholly-owned operating subsidiary, Scholar Rock, Inc., is a biotechnology company discovering and developing a new class of biologic medicines, called niche activators, which target disease-causing proteins in the tissue microenvironment to achieve therapeutic effects fundamentally at the source of disease. The company's proprietary technology has a unique capability for discovering and developing niche activators to be highly selective in targeting specific protein growth factors and opens a new therapeutic approach to address challenging diseases. Scholar Rock's founders and scientific advisors are leaders in elucidating new insights related to molecular mechanisms of growth factor signaling. The company's niche activators have a wide range of disease applications including autoimmune diseases, fibrosis, and diseases of musculoskeletal systems.

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