Scholar Rock Presents Preclinical Data for First Demonstration of Novel Pharmacological Approach to Targeting the TGFβ1 Latent Complex

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Innovative approach demonstrates potential for therapeutic modulation of growth factor activation across the TGFβ superfamily

Presentation at FASEB conference on TGFβ superfamily developments

CAMBRIDGE, Mass., July 14, 2015 – Scholar Rock, a biotechnology company focused on discovering and developing niche modulators that selectively target growth factor activation in the disease microenvironment, today announced the presentation of preclinical data demonstrating the effective targeting of the latent complex of transforming growth factor β1 (TGFβ1). The presentation, entitled "Pharmacological activation of TGFβ1 by targeting the latent complex" was presented at the Federation of American Societies for Experimental Biology (FASEB) conference on the TGF-β Superfamily Signaling in Development and Disease held in Snowmass, Colorado on July 12-17, 2015.

"We believe that the data Scholar Rock reported at this meeting represent the first demonstration of targeting and modulating the latent form of TGFβ1. This opens the door to a whole new approach to addressing a wide variety of diseases by modulating the latent forms of TGFβ1 and other drug targets in this superfamily of growth factors," said Nagesh Mahanthappa, Ph.D., President and Chief Executive Officer of Scholar Rock. "Scholar Rock is rapidly expanding our niche modulator discovery and development platform across a number of different TGFβ superfamily members, and the positive preclinical results on these targets are leading us toward selection of our first development candidate that we expect to announce in the coming months."

In the preclinical studies, Scholar Rock reported for the first time that TGFβ1's primary form, an inactive latent complex, can be targeted and modulated as a therapeutic approach, which is distinct from the traditional drug development approach of solely inhibiting the active form of TGFβ1. In particular it was demonstrated that a high-affinity biologic, known as a "niche modulator," can cause activation of latent TGFβ1 and promote signaling. Furthermore, preclinical studies showed that co-culture of regulatory T cells (Tregs), a cell type that plays a central role in autoimmune diseases and cancer, with naïve T cells in the presence of the niche modulator increased the immunosuppressive properties of the Tregs.

TGFβ1 is a key growth factor in a wide number of physiological processes, and dysregulation of its activity underlies a number of diseases. The ability to modulate the latent form of TGFβ1 offers an important new way to treat autoimmune diseases, cancer, and fibrosis, and may be generalizable to modulating activation of members of the TGFβ superfamily and other growth factor families for therapeutic applications.

About Niche Modulators
Scholar Rock's therapeutics are designed to the target the activation mechanism of protein growth factors selectively in the microenvironment of specific types of cells and tissues. By doing so, these niche modulators are able to achieve highly localized therapeutic effects at the site of disease while avoiding undesirable side effects that can result from systemic modulation of such growth factors.

About Scholar Rock
Scholar Rock is a biotechnology company focused on discovering and developing niche modulators, a novel class of biologic therapies that selectively modulate the activation of growth factors in the disease microenvironment. The Company's initial proprietary and partnered drug discovery programs target specific growth factors, including members of the TGFβ superfamily, which are present in the microenvironments of significant diseases such as fibrosis, diseases of the musculoskeletal systems and autoimmune diseases. Scholar Rock was founded based on discoveries made by its scientific founders, Professors Timothy Springer, PhD, and Leonard Zon, MD, of Boston Children's Hospital and Harvard Medical School, related to the molecular mechanisms of growth factor activation. The company is backed by leading life sciences investors, including Polaris Partners, ARCH Venture Partners, Timothy Springer, EcoR1 Capital and The Kraft Group.

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