Scholar Rock Presents First Data for Niche Modulator Inhibiting Myostatin Activation and Announces SRK-015 as Lead Drug Program

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Preclinical data presented at EMBO Molecular Mechanisms of Muscle Growth and Wasting demonstrate highly selective targeting of latent forms of myostatin, preventing muscle atrophy in disease

CAMBRIDGE, Mass., September 23, 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 that SRK-015 is the company's lead drug development candidate, a niche modulator inhibiting activation of the latent myostatin precursor. The announcement of this program coincides with the presentation of preclinical data for SRK-015, which demonstrated for the first time that therapeutic targeting of the latent complex of myostatin is an important mechanism for enhancing muscle growth in disease. The data were presented at the European Molecular Biology Organization's Workshop on Molecular Mechanisms of Muscle Growth and Wasting in Health and Disease taking place on September 20-25 in Ascona, Switzerland.

"We are excited to announce SRK-015 as the first development candidate from our niche modulator platform. The data we presented demonstrate the exceptional selectivity that is achieved by targeting the latent, inactive forms of myostatin compared to the traditional approach of targeting the mature, active form of myostatin or the myostatin receptor," said Nagesh Mahanthappa, Ph.D., President and Chief Executive Officer of Scholar Rock. "We look forward to advancing SRK-015 for the treatment of primary myopathies and believe it will be the first of many novel niche modulators that target protein growth factors with unprecedented specificity and localization of effect."

The preclinical data demonstrated that SRK-015 can selectively block the activation of myostatin in vitro and enhance muscle growth in vivo. These data suggest that SRK-015 may be useful in treatment of muscle wasting disorders while avoiding the undesirable, systemic side effects that can result from directly targeting myostatin or its receptor with insufficient selectivity. Myostatin is a member of the TGFβ superfamily of growth factors that is expressed primarily in skeletal muscle cells to inhibit muscle growth. Myostatin has been implicated in a range of muscle diseases, and Scholar Rock plans to pursue initial development of SRK-015 in primary myopathies, disease states in which restoration of normal muscle function can significantly improve patients' lives.

In further details in the poster presentation entitled, "Blockade of Myostatin activation specifically and potently enhances muscle growth in vivo," Scholar Rock reported for the first time that:

- SRK-015 binds selectively with nanomolar affinity to the inactive forms of myostatin with no binding observed to other members of the TGFβ superfamily.
- By binding to the inactive forms of myostatin, SRK-015 inhibits its activation without modifying activation of other closely-related members of the TGFβ superfamily.
- SRK-015 demonstrates robust effect on lean mass and muscle weight in healthy mice and preserved muscle mass in mouse models of muscle atrophy.

About Niche Modulators
Scholar Rock's therapeutics are designed to 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 specific and 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, and have a near-term focus on the treatment of muscle disease, fibrosis and immuno-oncology. 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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