Confronting convention

We take risks and challenge the status quo because disease must be fought with unexpected practice.

ScholarRock

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NASDAQ: SRRK

Our company

We eat, sleep, and breathe good science

Scholar Rock is a clinical-stage biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role.

Our most advanced investigational product candidate, apitegromab (SRK-015), a selective inhibitor of the activation of the growth factor myostatin in skeletal muscle, is in development for the treatment of spinal muscular atrophy, or SMA. Following positive results from the TOPAZ Phase 2 trial in patients with Type 2 and Type 3 SMA, we are planning to advance to a Phase 3 trial by year-end. In addition, we are conducting our DRAGON Phase 1 proof-of-concept trial for SRK-181, a selective inhibitor of latent TGFβ1 activation, in patients with locally advanced and metastatic solid tumors.

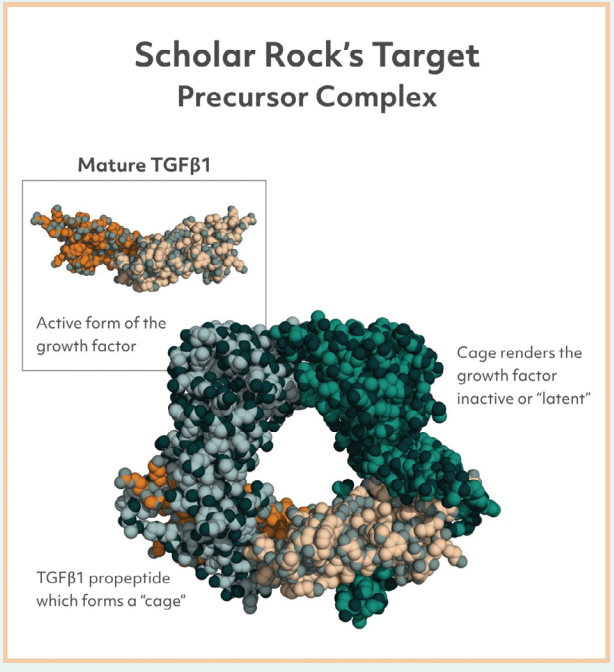
By combining our deep structural insights with antibody drug discovery, Scholar Rock is creating a pipeline of novel product candidates with the potential to transform the lives of patients suffering from a wide range of serious diseases, including neuromuscular disorders, cancer, and fibrosis.

Our platform

The foundation to see new possibilities

Our approach to the discovery and development of growth factor-targeted drugs is fundamentally new and different.

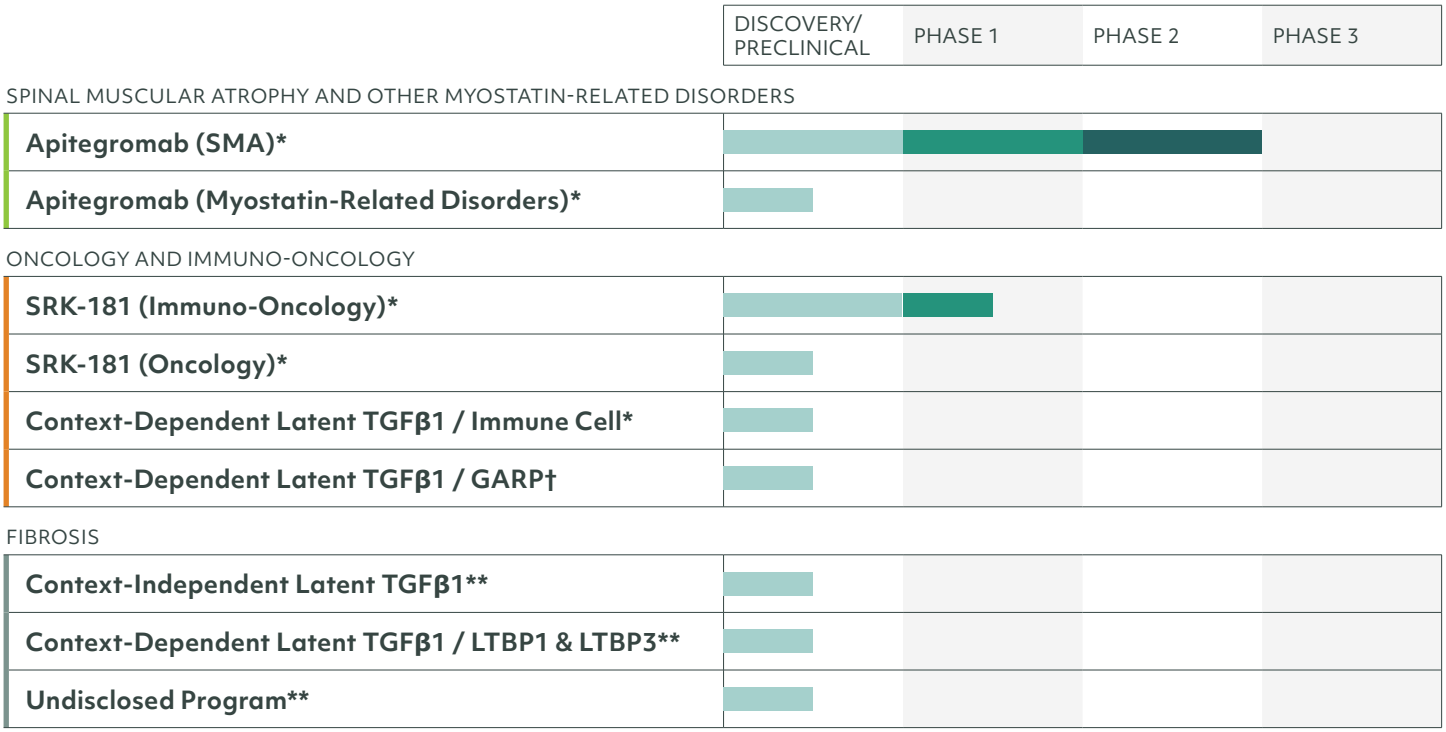
By exploiting greater structural differences amongst the inactive forms of growth factors, our uniquely designed antibodies selectively target the precursor, or latent, forms of growth factors in the microenvironment of cells and tissues. By doing so, we hope to overcome the toxicity challenges that have plagued traditional approaches.



Our pipeline

A path toward novel solutions

The precursor structure coupled with an understanding of the activation mechanism allowed us to establish a differentiated pipeline of product candidates that could provide revolutionary approaches to treating serious diseases.



* Internal Proprietary Program ** In Partnership with Gilead † In Partnership with Janssen

Apitegromab for Spinal Muscular Atrophy (SMA)

TOPAZ

Apitegromab selectively inhibits myostatin activation in the muscle.

The TOPAZ Phase 2 proof-of-concept trial investigated apitegromab in patients with Type 2 and Type 3 SMA over a 12-month treatment period. A pre-planned interim analysis was conducted following a six-month treatment period and positive interim proof-of-concept data was announced in October 2020. Positive 12-month top-line results were announced in April 2021, further demonstrating apitegromab's therapeutic potential. Our aim is to establish apitegromab as the first potential muscle-directed therapy intended to be used in conjunction with available SMN upregulator therapies to help improve motor function for individuals with SMA. Based on these results, we plan to initiate a randomized, double-blind, placebo-controlled Phase 3 trial in patients with non-ambulatory Type 2 and 3 SMA by year-end 2021.

Learn more about the TOPAZ clinical trial >

SRK-181 for Immuno-Oncology

DRAGON

In preclinical studies, SRK-181 demonstrated that targeting the precursor form of the TGFβ1 growth factor can render tumors vulnerable to anti-PD-1 therapy and drive tumor regression through combination therapy.

We are investigating SRK-181, a selective inhibitor of latent TGFβ1 activation, in the DRAGON Phase 1 clinical trial in patients with locally advanced or metastatic solid tumors. Part A dose escalation is evaluating SRK-181 as a single-agent and in combination with anti-PD-(L)1 therapy and Part B dose expansion will consist of multiple cohorts that will enroll patients who have urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, and other solid tumors. An update on dose escalation and initial clinical data from Part A are anticipated by the end of 2021.

Learn more about the DRAGON clinical trial >

ScholarRock™