Confronting convention

We are committed to addressing an unmet need and making a meaningful difference in the daily lives of those living with serious diseases.

ScholarRock

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NASDAQ: SRRK

Our company

We work every day to create new possibilities for patients.

Scholar Rock is a biopharmaceutical company that discovers, develops, and delivers life-changing therapies for people with serious diseases that have high unmet need. As a global leader in the biology of the transforming growth factor beta (TGFβ) superfamily of cell proteins and named for the visual resemblance of a scholar rock to protein structures, the clinical-stage company is focused on advancing innovative treatments where protein growth factors are fundamental. Over the past decade, the company has created a pipeline with the potential to advance the standard of care for neuromuscular disease, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted drugs can play a transformational role.

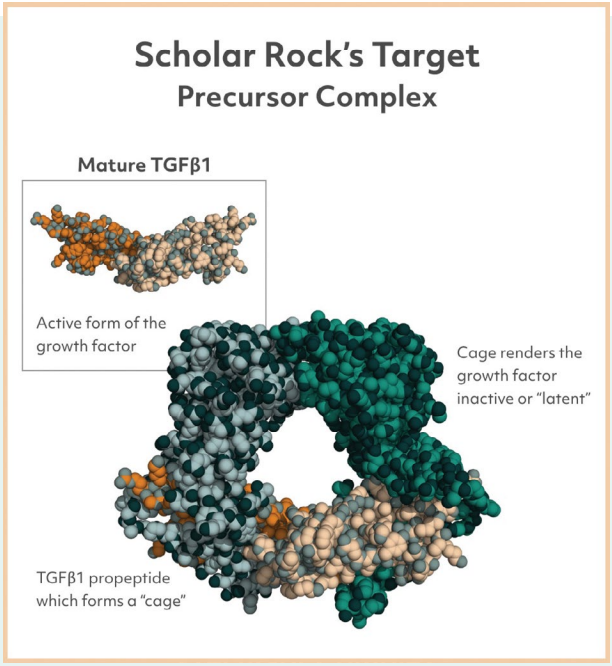
Scholar Rock is the only company to show clinical proof of concept for a muscle-targeted treatment in spinal muscular atrophy (SMA). This commitment to unlocking fundamentally different therapeutic approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. By harnessing cutting-edge science in disease spaces that are historically under-addressed through traditional therapies, Scholar Rock works every day to create new possibilities for patients. Learn more about the company's approach at ScholarRock.com and follow @ScholarRock and on LinkedIn.

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.

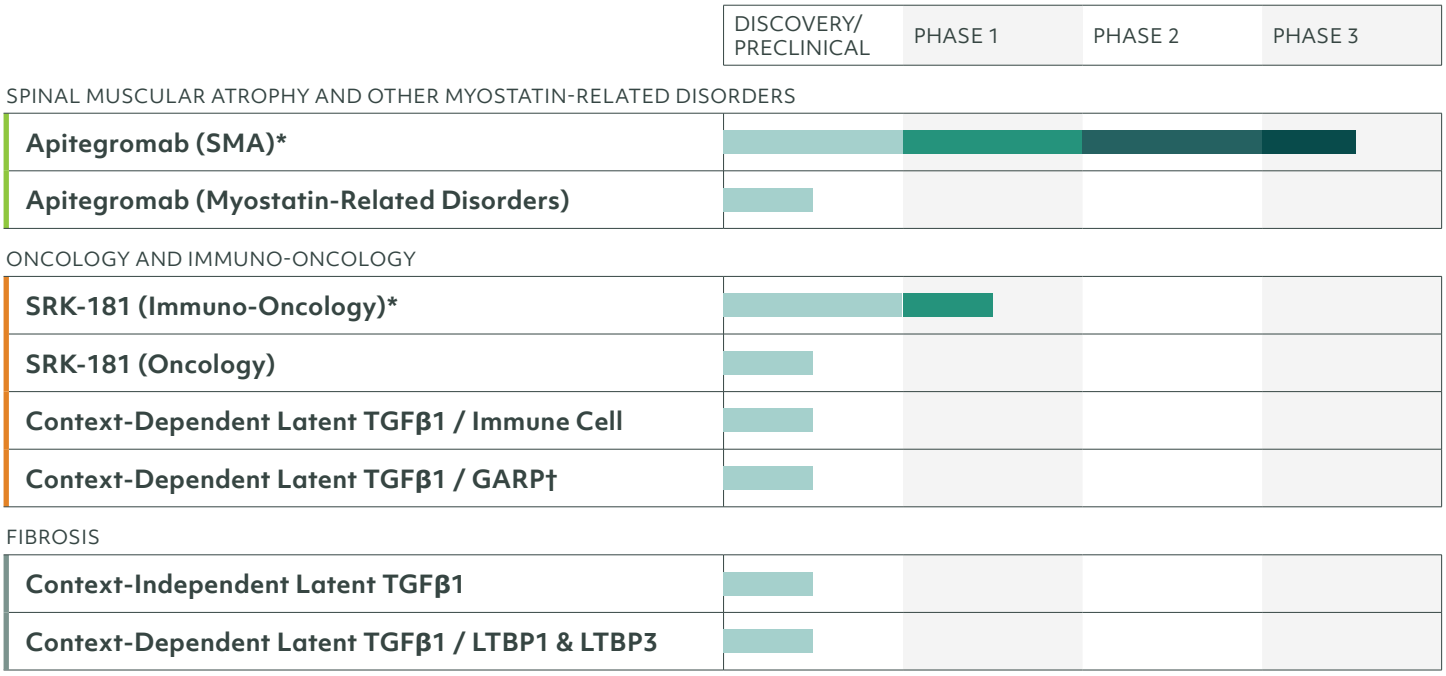
By exploiting greater structural differences amongst the inactive forms of growth factors, our uniquely designed antibodies selectively target the precursor, or latent, forms of growth factors in the microenvironment of cells and tissues. By doing so, we hope to overcome the toxicity challenges that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



† In Partnership with Janssen

Apitegromab for Spinal Muscular Atrophy (SMA)

TOPAZ

SAPPHIRE

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 are now advancing apitegromab development through SAPPHIRE, a randomized, double-blind, placebo-controlled, phase 3 clinical trial in people with non-ambulatory Type 2 and 3 SMA.

Learn more about the TOPAZ clinical trial ›

Learn more about the SAPPHIRE 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.The trial is anticipated to advance to Part B (dose expansion) mid-year 2021 and initial clinical response and safety data from Part A are anticipated by the end of 2021.

Learn more about the DRAGON clinical trial ›

ScholarRock™