

# Confronting **convention**

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



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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 now advancing apitegromab development through SAPPHIRE, a randomized, double-blind, placebo-controlled, phase 3 clinical trial. In addition, we are conducting our DRAGON Phase 1 proof-of-concept trial for SRK-181, a selective inhibitor of latent TGF $\beta$ 1 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

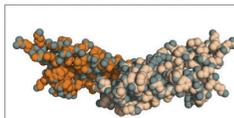
**The foundation to see new possibilities**

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.

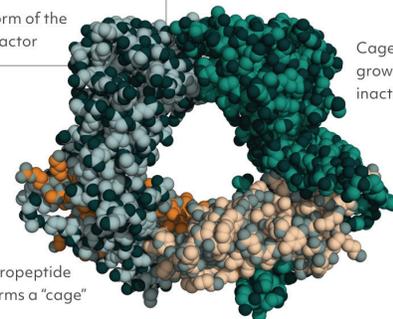
### Scholar Rock's Target Precursor Complex

Mature TGF $\beta$ 1



Active form of the  
growth factor

Cage renders the  
growth factor  
inactive or "latent"



TGF $\beta$ 1 propeptide  
which forms a "cage"

# 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.

	DISCOVERY/ PRECLINICAL	PHASE 1	PHASE 2	PHASE 3
<b>SPINAL MUSCULAR ATROPHY AND OTHER MYOSTATIN-RELATED DISORDERS</b>				
<b>Apitegromab (SMA)</b>	██████████	██████████	██████████	
<b>Apitegromab (Myostatin-Related Disorders)</b>	██████████			
<b>ONCOLOGY AND IMMUNO-ONCOLOGY</b>				
<b>SRK-181 (Immuno-Oncology)</b>	██████████	██████████		
<b>SRK-181 (Oncology)</b>	██████████			
<b>Context-Dependent Latent TGFβ1 / Immune Cell</b>	██████████			
<b>Context-Dependent Latent TGFβ1 / GARP†</b>	██████████			
<b>FIBROSIS</b>				
<b>Context-Independent Latent TGFβ1</b>	██████████			
<b>Context-Dependent Latent TGFβ1 / LTBP1 &amp; LTBP3</b>	██████████			

† In Partnership with Janssen

## Apitegromab for Spinal Muscular Atrophy (SMA)



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.



**SAPPHIRE**

[Learn more about the TOPAZ clinical trial >](#)

[Learn more about the SAPPHIRE clinical trial >](#)

## SRK-181 for Immuno-Oncology



**DRAGON**

In preclinical studies of SRK-181, a selective inhibitor of latent TGFβ1 activation, data suggests that targeting the precursor form of the TGFβ1 growth factor can render tumors vulnerable to anti-PD-(L)1 therapy and drive tumor regression (in human tumors) through combination therapy with an anti-PD-(L)1 therapy.

We are investigating SRK-181 in the DRAGON Phase 1 clinical trial in patients with locally advanced or metastatic solid tumors. Initial clinical data from Part A of the trial, which was designed to evaluate the safety and tolerability of SRK-181 alone or in combination with anti-PD-(L)1 checkpoint inhibitor therapy and to determine the recommended dose for the Part B dose expansion phase, was shared in November 2021. Based on the safety and pharmacokinetic data from Part A, we have initiated Part B to assess SRK-181 in combination with an approved anti-PD-(L)1 therapy across multiple solid tumor cohorts, including in patients with urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, and clear cell renal cell carcinoma.

[Learn more about the DRAGON clinical trial >](#)