

# 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 for which protein growth factors may play a fundamental role.

Our most advanced product candidate, apitegromab (SRK-015), a selective inhibitor of the activation of latent myostatin in skeletal muscle, is currently in development for the treatment of spinal muscular atrophy, or SMA. In addition, we are conducting our DRAGON Phase 1 proof-of-concept trial for SRK-181, a highly specific inhibitor of latent TGF $\beta$ 1 activation, in patients with locally advanced and metastatic solid tumors exhibiting primary resistance to anti-PD-(L)1 therapy.

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 diseases, including neuromuscular disorders, cancer, and fibrosis.

## Our platform

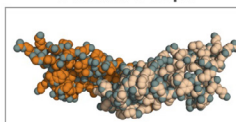
### The foundation to see new possibilities

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

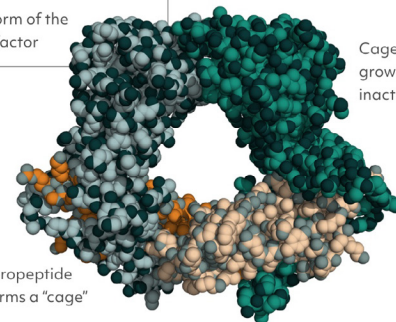
Mature TGF $\beta$ 1



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>	██████████			
<b>Undisclosed Program**</b>	██████████			

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

## Apitegromab for Spinal Muscular Atrophy (SMA)



Apitegromab selectively targets the precursor form of myostatin to block its activation in the muscle.

The TOPAZ Phase 2 proof-of-concept trial is investigating apitegromab in patients with Type 2 and Type 3 SMA over a 12-month treatment period. Positive proof-of-concept data from the six-month interim analysis were announced in October 2020, showing the potential therapeutic benefits of inhibiting the activation of latent myostatin with apitegromab in patients with SMA.

[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 potent and highly selective inhibitor of latent TGFβ1 activation, in the DRAGON Phase 1 clinical trial in patients with locally advanced or metastatic solid tumors exhibiting primary resistance to anti-PD-(L)1 therapy.

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