



Deep Insights, Impactful Medicines

NASDAQ: SRRK

MANAGEMENT

Tony Kingsley, MBA
CEO & President

Ted Myles, MBA
CFO & Head of Business Operations

Yung H. Chyung, MD
Chief Medical Officer

Gregory J. Carven, PhD
Head of Research

Scholar Rock's Proprietary Solutions to Traditional Challenges

Traditional approaches focused on inhibiting growth factors have been limited by structural similarities, overlapping receptors and diverse physiological roles. Scholar Rock's proprietary solutions to overcome these challenges include:

- Unique mechanism of growth factor regulation
- Exploits Scholar Rock's structural biology insights
- Offers localization of effect and high selectivity
- Well-established modality (monoclonal antibodies)
- Broad IP portfolio covering compositions and methods

620 Memorial Drive, 2nd Floor
Cambridge, MA 02139
857.259.3860

info@scholarrock.com
www.scholarrock.com



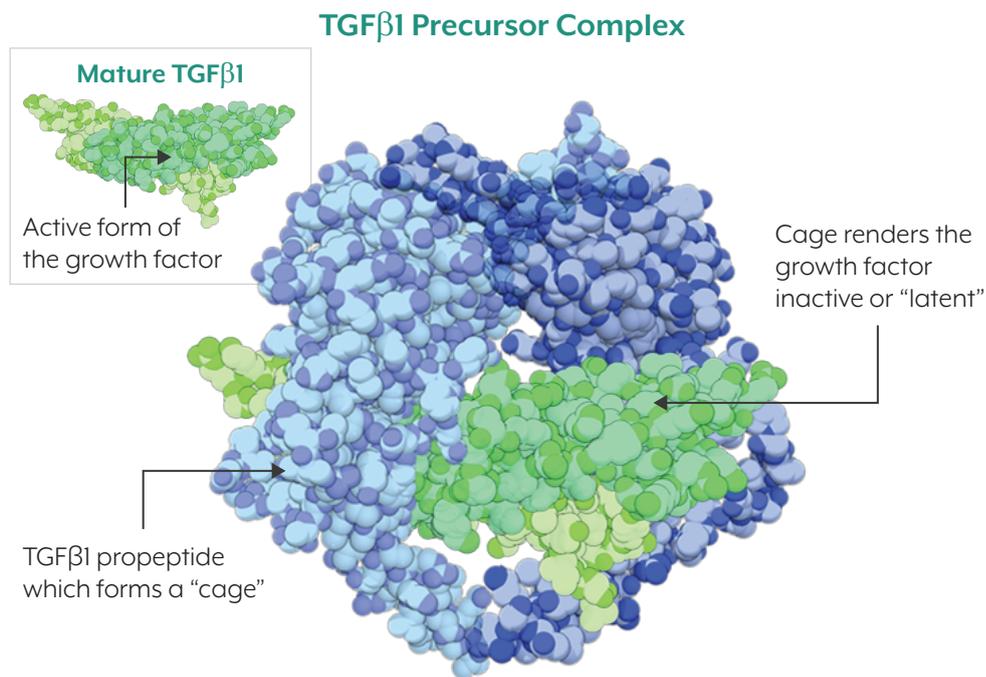
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We Look Deeper into the Biology of Growth Factors and Disease to Discover New Treatments for Patients

Scholar Rock is a clinical-stage biopharmaceutical company focused on the discovery and development of new medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. 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, fibrosis and anemia. Scholar Rock's two lead programs include SRK-015 in clinical development as a muscle-targeted therapy for spinal muscular atrophy (SMA) and SRK-181 in clinical development to expand anti-tumor responses by overcoming primary resistance to checkpoint inhibitor therapies.

By exploiting structural differences in the precursor, or latent form of growth factors, Scholar Rock intends to avoid the historical dose-limiting safety challenges associated with inhibiting the mature form of growth factors for therapeutic effect. The Company's two lead antibodies target biologically validated growth factors, in the precursor form, to achieve an unprecedented level of selectivity.

Our Drug Discovery Platform as Product Engine Nature's Growth Factor Activation Machinery



TGFβ Superfamily: More than 30 Related Growth Factors that Mediate Diverse Biological Processes

Scholar Rock's newly elucidated understanding of the molecular mechanisms of growth factor activation enabled it to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target the activation of these signaling protein.

Differentiated Pipeline

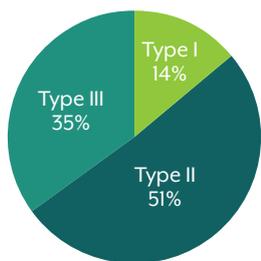
TGFβ superfamily is a key driver of disease across multiple therapeutic areas including SMA/myostatin-related disorders, oncology/immunology, fibrosis, and anemia.



SRK-015 for Spinal Muscular Atrophy (SMA)

SRK-015 is a local and highly selective inhibitor of the activation of latent myostatin. Based on existing research on the mechanism of myostatin in muscle growth and strength, Scholar Rock believes SRK-015 may promote a clinically beneficial increase in muscle strength. SRK-015 is currently being evaluated in the TOPAZ Phase 2 trial as a potential treatment to improve muscle strength and motor function in patients with SMA.

SMA Prevalence of 30,000-35,000 in U.S. and Europe*



Relative Prevalence Among Patients Living with SMA



Type I:

- Infant-onset; often fatal

Type II and non-ambulatory Type III:

- Later-onset but still early childhood
- Severe deficits in motor function

Ambulatory Type III:

- Limited mobility and substantial morbidity

Type IV:

- Population not well-characterized

Potential to use SRK-015 in conjunction with SMN upregulators

Potential to use SRK-015 as monotherapy or in conjunction with SMN upregulators



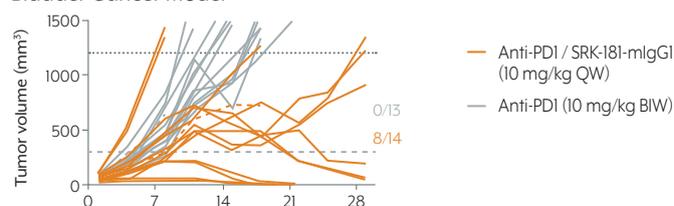
*Lally et al. Orphanet Journal of Rare Diseases, 2017

SRK-181 for Immuno-oncology

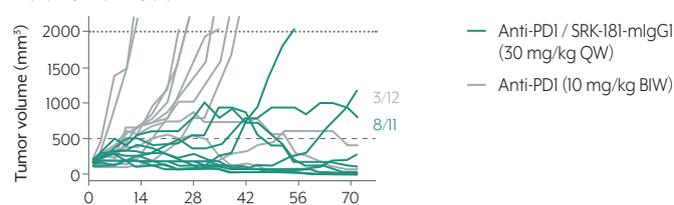
A significant proportion of patients fail to respond to checkpoint inhibition therapy because their cancers have pre-existing resistance to immunotherapy. SRK-181 has the potential to render resistant solid tumors vulnerable to immune checkpoint inhibitors such as anti-PD-L1 therapies and drive tumor regression through combination therapy. SRK-181 is currently being evaluated in a Phase I proof-of-concept trial in patients with locally advanced or metastatic solid tumors.

SRK-181-mIgG1* Rendered TGFβ1- and TGFβ1/3-Expressing Preclinical Tumor Models Susceptible to Anti-PD1 Therapy

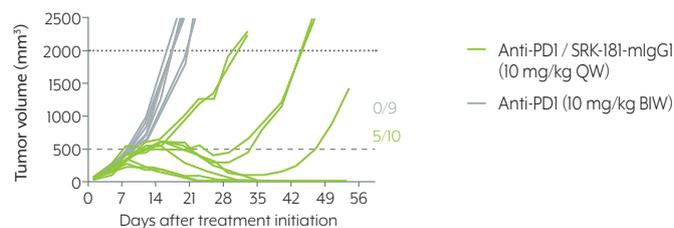
Bladder Cancer Model



Melanoma Model



Breast Cancer Model



*SRK-181-mIgG1 is the murine version of SRK-181. Preclinical data shown above as presented at American Association of Cancer Research (AACR) Annual Meeting (April 2019) www.scholarrock.com/wp-content/uploads/2019/04/2019-AACR-Defeating-primary-checkpoint-resistance-SRK-181.pdf