

## Scholar 'Rock on' \$36M series B shows faith in growth factor activation

By Marie Powers, News Editor

Scholar Rock Inc., the 2-year-old company pursuing drugs that target supracellular activation of growth factors, jump-started its New Year with a \$36 million series B financing. Fidelity Management and Research Co. led the round, with participation from additional new investor Cormorant Asset Management and from Scholar Rock's existing investors: Polaris Partners, Timothy Springer, Arch Venture Partners, Ecor1 Capital and the Kraft Group.

The financing will allow the Cambridge, Mass.-based company to move its lead candidate, SRK-015, into a human proof-of-concept study in muscle disease, to advance a second program toward the clinic and to expand its early stage pipeline.

Discussions about the series B started several months after Scholar Rock raised its \$20 million A round in September 2014, according to Nagesh Mahanthappa, the company's president and CEO. The process of "investigating the interest" in the company's work began in earnest in mid-2015, and the syndicate came together quickly.

"Despite some of the uncertainties in the financial markets more recently, there's clearly a big appetite for fundamental innovation," Mahanthappa told *BioWorld Today*, characterizing that interest as a craving for platforms and technologies that will lead to "meaningful new products."

Citing competition in the space, the CEO declined to divulge details about the exact timing or design of the first-in-human trial of SRK-015, a selective and local inhibitor of latent myostatin activation in development to treat primary myopathies.

Because several growth factors are closely related to myostatin – a member of the transforming growth factor beta, or TGFβ, superfamily of growth factors – Scholar Rock seeks to avoid cross-talk across the TGFβ superfamily by targeting myostatin activation rather than mature myostatin.

By targeting the latent form of myostatin – also known as growth differentiation factor 8, or GDF-8 – and inhibiting its activation in muscle, SRK-015 has shown reproducible increases in lean mass and muscle weight in preclinical muscle disease models. Company execs said the candidate has the potential to preserve or restore normal muscle function across a wide range of muscle diseases.

More than 40 agents are in development to treat muscle disease and wasting, with half of them still in discovery, according to Cortellis Competitive Intelligence. Eli Lilly and Co., of Indianapolis, has another GDF-8 candidate, landogrozumab, in development.

But SRK-015 "is only one standard bearer" for Scholar Rock's progress over the past year, Mahanthappa emphasized. In January 2014, the company inked a research collaboration with Janssen Biotech Inc., a unit of Johnson & Johnson, of New Brunswick, N.J., to discover and develop therapeutics designed to regulate the immune system by targeting TGFβ 1, with applications both in autoimmune disease and immuno-oncology. (See *BioWorld Today*, Feb. 28, 2014.)

So far, that work "has confirmed our ability to modulate TGFβ 1 in a fashion which appears to be largely specific to the immune system," Mahanthappa said.

In parallel, Scholar Rock is advancing on its own with another aspect of TGFβ 1 biology focused on fibrosis. In that regard, the company named Scott Friedman, professor of medicine, dean for therapeutic discovery and chief of the division of liver diseases at the Icahn School of Medicine at Mount Sinai Hospital, to its scientific advisory board.

Friedman's accolades for pioneering research into the underlying causes of fibrosis associated with chronic liver disease include the International Achievement Prize of the European Association for the Study of Liver Diseases in 2012.

"A key element of the supracellular activation approach is that it allows us to attack growth factors that play roles in a wide variety of physiologic and pathophysiologic processes," Mahanthappa explained. The company's platform "de-convolutes" the pharmacology to produce selective effects in cells and tissues of interest.

"The Scholar Rock story is larger than just the myostatin program," he added. "We've only talked about TGFβ 1 and myostatin publicly, but we're continuing to make, I think,

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very good progress on additional growth factors.”

The series B will fund ongoing buildout of the discovery platform while helping Scholar Rock to expand its development capability. Mahanthappa wouldn't say just how far the series B will take the pipeline, but Scholar Rock plans to stay flexible as it looks to the future.

“A company at our stage, ideally, is seeking to build a pipeline

comprised of both proprietary programs and partnered programs,” he observed. “There are a variety of strategies around partnership, which could span broad arrangements across multiple targets or specific types of collaborations focused on particular programs and ways of sharing value on those programs. A combination of partnerships and additional financing are both in the future of Scholar Rock.” //