Scholar Rock Provides Corporate Update and Highlights Priorities for 2024

January 4, 2024

- Advancing industry-leading antimyostatin pipeline, comprised of multiple, novel assets with unparalleled selectivity, to treat spinal muscular atrophy (SMA) and cardiometabolic disorders

- Completed enrollment for apitegromab pivotal Phase 3 SAPPHIRE trial in patients with SMA; topline data anticipated in 4Q 2024

- Apitegromab Phase 2 proof-of-concept trial in obesity expected to commence in mid-2024

- Presenting new preclinical data on SRK-439, a novel investigational myostatin inhibitor for the treatment of obesity, at Keystone Symposia in February

- Scholar Rock reports year-end cash and cash equivalents of approximately $280 million

- Presenting at the 42nd Annual J.P. Morgan Healthcare Conference on Tuesday, January 9, 2024 at 1:30 p.m. PT (4:30 p.m. ET)

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 4, 2024-- Scholar Rock (NASDAQ: SRRK), a late-stage biopharmaceutical company focused on advancing innovative treatments for spinal muscular atrophy (SMA), cardiometabolic disorders, and other serious diseases where protein growth factors play a fundamental role, today provided recent corporate updates and highlighted upcoming priorities for 2024.

“We were pleased with our progress in 2023 as we advanced our industry leading antimyostatin pipeline with apitegromab and SRK-439 and have great momentum heading into 2024. With enrollment completed for our Phase 3 SAPPHIRE trial for patients with spinal muscular atrophy, we expect topline data in Q4 this year,” said Jay Backstrom, M.D., MPH, President and Chief Executive Officer of Scholar Rock. “Additionally, we are thrilled with the planned initiation in mid-2024 of an apitegromab Phase 2 proof-of-concept trial in patients with obesity and on GLP-1 therapies that aims to establish the importance of selective, safe myostatin inhibition to preserve lean muscle mass as part of healthy weight loss.”

2024 Priorities:

SPINAL MUSCULAR ATROPHY

Apitegromab is an investigational, fully human monoclonal antibody that inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle and is being developed as a potential first muscle-targeted therapy for the treatment of SMA. Apitegromab is the only muscle-targeted therapy to show clinical proof-of-concept in SMA.

- **Planning to announce Phase 3 SAPPHIRE clinical trial topline data in 4Q 2024**. SAPPHIRE is a randomized, double-blind, placebo-controlled clinical trial evaluating apitegromab in patients with nonambulatory Types 2 and 3 SMA on either nusinersen or risdiplam. If the trial is successful and apitegromab is approved, the Company expects to initiate a commercial product launch in 2025.
- **Continue to progress the ONYX long-term extension study for patients from both the TOPAZ and SAPPHIRE studies.**

CARDIOMETABOLIC DISORDERS

SRK-439 is a novel, preclinical, investigational myostatin inhibitor that has high in vitro affinity for pro- and latent myostatin and maintains myostatin specificity (i.e., no GDF11 or Activin-A binding), and is initially being developed for the treatment of obesity.

- **Initiating a Phase 2 proof-of-concept trial with apitegromab in combination with a GLP-1 receptor agonist (GLP-1 RA) in obesity in mid-2024.** As part of the Company’s strategy to advance the development of SRK-439, it plans to initiate a Phase 2 proof-of-concept trial with apitegromab in combination with a GLP-1 RA, subject to IND clearance. Data from the clinical trial are expected in mid-2025 and will be used to guide clinical development of SRK-439. The Company plans to file an IND for SRK-439 for the treatment of obesity in 2025.
- **Presenting preclinical SRK-439 data in a poster presentation at Keystone Symposia** at the *Obesity: Causes and Consequences* meeting on February 5, 2024, in Vancouver, BC, Canada.

2023 Highlights:

- Completed enrollment of apitegromab pivotal Phase 3 SAPPHIRE trial.
- Presented TOPAZ 36-month extension trial data at the Cure SMA Research & Clinical Care Meeting in June, and at the 28th Annual Congress of the World Muscle Society in October, which showed long-term substantial and sustained

improvements in motor function and patient-reported outcome measures in patients with nonambulatory Types 2 and 3 SMA receiving survival motor neuron (SMN) therapy.
- Initiated the ONYX trial, a long-term extension study for patients from both the TOPAZ and SAPPHIRE studies, which remains ongoing.
- Announced plans to expand into cardiometabolic disorders with SRK-439, starting with a Phase 2 proof-of-concept trial evaluating apitegromab in obesity to inform development of SRK-439.
- Presented SRK-181 Phase 1 DRAGON trial clinical and biomarker data at the SITC 38th Annual Meeting, which showed favorable tolerability and promising anti-tumor activity in heavily pretreated patients with clear cell renal cell carcinoma (ccRCC) resistant to anti-PD-1. The Company believes these data support proof-of-concept and completed enrollment of the DRAGON trial in December. The Company will provide additional clinical data updates as they become available in 2024.
- Completed an equity financing of $98 million in October. As of December 31, 2023, Scholar Rock reported cash, cash equivalents, and marketable securities of approximately $280 million, which is projected to fund the Company's operations into the second half of 2025.

"We are excited to enter 2024 with several important near-term milestones ahead of us. As the leader in selective myostatin inhibition, we believe we are well positioned to deliver significant value to patients living with spinal muscular atrophy and the millions of people suffering from the wide array of health challenges stemming from cardiometabolic and obesity disorders. We are highly encouraged by our potential to advance the standard of care where muscle-targeted therapies can play a role in addressing unmet patient needs," said Ted Myles, Chief Operating Officer and Chief Financial Officer.

J.P. Morgan Healthcare Conference Presentation and Webcast

Scholar Rock management will highlight these updates in a corporate presentation at the 42nd Annual J.P. Morgan Healthcare Conference on Tuesday, January 9, 2024, at 1:30 p.m. PT (4:30 p.m. ET). A live webcast of the presentation may be accessed by visiting the Investors & Media section of the Scholar Rock website at http://investors.scholarrock.com. An archived replay of the webcast will be available on the Company's website for approximately 90 days following the presentation.

About Scholar Rock

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, Scholar Rock 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 our approach at ScholarRock.com and follow @ScholarRock and on LinkedIn.

Availability of Other Information About Scholar Rock

Investors and others should note that we communicate with our investors and the public using our company website www.scholarrock.com, including, but not limited to, company disclosures, investor presentations and FAQs, Securities and Exchange Commission filings, press releases, public conference call transcripts and webcast transcripts, as well as on Twitter and LinkedIn. The information that we post on our website or on Twitter or LinkedIn could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website or social media shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

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Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding Scholar Rock's future expectations, plans and prospects, including without limitation, Scholar Rock's expectations regarding its growth, strategy, progress, results and timing of its clinical trials for apitegromab and SRK-181 and its preclinical programs, including SRK-439, regulatory feedback including with respect to the IND submitted in connection with the planned Phase 2 trial of SRK-439 in combination with GLP-1 RAs in obesity, and indication selection and development timing, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, its expected cash and cash equivalents as of December 31, 2023 and cash runway, expectations regarding the achievement of important milestones, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and the potential of its product candidates and proprietary platform. The use of words such as "may," "might," "could," "will," "should,"

“expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify such forward-looking statements. All such forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, without limitation, that preclinical and clinical data, including the results from the Phase 2 clinical trial of apitegromab, or Part A or Part B of the Phase 1 clinical trial of SRK-181, are not predictive of, may be inconsistent with, or more favorable than, data generated from future or ongoing clinical trials of the same product candidates, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA or Part B of the Phase 1 clinical trial of SRK-181; Scholar Rock’s ability to provide the financial support, resources and expertise necessary to identify and develop product candidates on their expected timelines; the data generated from Scholar Rock’s nonclinical and preclinical studies and clinical trials; information provided or decisions made by regulatory authorities; competition from third parties that are developing products for similar uses; Scholar Rock’s ability to obtain, maintain and protect its intellectual property; Scholar Rock’s dependence on third parties for development and manufacture of product candidates including, without limitation, to supply any clinical trials; Scholar Rock’s ability to manage expenses and to obtain additional funding when needed to support its business activities and establish and maintain strategic business alliances and new business initiatives, and the impacts of public health pandemics such as COVID-19 on business operations and expectations, as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock’s Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, as well as discussions of potential risks, uncertainties, and other important factors in Scholar Rock’s subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Scholar Rock’s views only as of today and should not be relied upon as representing its views as of any subsequent date. All information in this press release is as of the date of the release, and Scholar Rock undertakes no duty to update this information unless required by law.

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