Scholar Rock Highlights 2025 Strategic Priorities

January 8, 2025

- BLA and MAA submission for apitegromab in SMA on track for 1Q 2025; if approved, U.S. launch expected in 4Q 2025

- Initiating Phase 2 OPAL clinical trial in SMA patients under two years old in mid-2025

- Data from Phase 2 EMBRAZE trial evaluating apitegromab in obesity expected in 2Q 2025

- Filing of IND application for SRK-439 on track for 3Q 2025

- Presenting at the 43rd Annual J.P. Morgan Healthcare Conference on Tuesday, January 14, 2025 at 9:45 a.m. PT (12:45 p.m. ET)

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 8, 2025-- 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 2025.

“We are excited about the potential for apitegromab to help those living with SMA as the first and only muscle-targeted therapy for the treatment of this condition and are making excellent progress in finalizing our regulatory applications and preparing for commercial launch,” said Jay Backstrom, M.D., MPH, President and Chief Executive Officer of Scholar Rock. “We remain on track for BLA and MAA submission in the first quarter of 2025 and are laser focused on bringing apitegromab to people with SMA as soon as possible. Building on the success of our leading, highly selective anti-myostatin approach in SMA, we plan to expand development of apitegromab into additional rare neuromuscular disorders to serve more people with unmet medical needs and as the foundation of a growing neuromuscular franchise.”

Dr. Backstrom continued, “Additionally, we are making excellent progress to deliver value across our pipeline, including the next wave of innovation in cardiometabolic disorders. We look forward to reporting topline data from our Phase 2 EMBRAZE proof-of-concept trial of apitegromab in combination with a GLP-1 receptor agonist in obesity and submitting an IND for SRK-439, our highly selective myostatin inhibitor specifically designed for obesity and cardiometabolic disorders.”

2025 Strategic Priorities and Upcoming Milestones:

Scholar Rock plans to focus on three strategic priorities in 2025 as it transitions into a commercial-stage biopharmaceutical company, creating sustainable long-term value opportunities:

- Commercialize apitegromab in SMA.
- Expand the potential benefit of apitegromab.
- Advance Scholar Rock’s anti-myostatin program in cardiometabolic disorders.

Commercialize apitegromab in SMA

- On track to submit a biologics license application (BLA) to the FDA and a marketing authorisation application (MAA) to the European Medicines Agency in 1Q 2025 for apitegromab in SMA.
- Preparing for U.S. commercial launch in 4Q 2025, and European launch to follow.
- Presenting detailed results from the pivotal Phase 3 SAPPHIRE trial at upcoming medical meetings in 2025.

Expand the potential benefit of apitegromab

- Initiating Phase 2 OPAL clinical trial in SMA patients under two years of age in mid-2025**.** The trial will evaluate apitegromab in patients under two years of age who have been or are continuing to be treated with any currently approved SMN therapy, including onasemnogene abeparvovec.
- Building upon the positive Phase 3 SAPPHIRE trial, the Company is exploring development of apitegromab in other rare neuromuscular disorders such as Duchenne muscular dystrophy, Becker muscular dystrophy, Facioscapulohumeral muscular dystrophy and amyotrophic lateral sclerosis to build a growing neuromuscular franchise.

Advance the anti-myostatin program in cardiometabolic disorders

- Topline data expected in the second quarter of 2025 from the Phase 2 EMBRAZE proof-of-concept trial, a randomized,

double-blind, placebo-controlled, multi-center study evaluating the safety and efficacy of apitegromab, a highly selective investigational myostatin inhibitor, to preserve muscle mass in overweight and obese adults who are taking a GLP-1 receptor agonist (GLP-1 RA). Trial outcomes will be used to guide clinical development of SRK-439.
- Plan to file an IND for SRK-439 for the treatment of obesity on the background of standard of care GLP-1 RAs in 3Q 2025.
- Scholar Rock disclosed new preclinical data which showed that SRK-439 protected against tirzepatide-induced muscle loss in DIO mice. Lean mass preservation was seen with doses of SRK-439 as low as 0.3 mg/kg and lean mass gain was observed at higher doses. The addition of SRK-439 with tirzepatide improved overall body composition with dose-dependent enhancement of fat mass loss.

Scholar Rock believes that its existing cash, cash equivalents and marketable securities will be sufficient to fund the Company's anticipated operating expenses and capital expenditure requirements into the fourth quarter of 2026.

"Scholar Rock is funded to scale up and drive forward our key priorities in 2025, including the commercialization of apitegromab, the EMBRAZE readout, the initiation of clinical development for SRK-439, and growing our pipeline," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. "We believe in the transformative potential of our highly selective muscle-targeted approach and will advance our programs as we prepare to transition into a commercial company."

2024 Highlights and Accomplishments:

- Reported positive topline data from the pivotal SAPPHIRE trial in October 2024. The trial achieved its primary endpoint, demonstrating a statistically significant and clinically meaningful improvement for apitegromab versus placebo in motor function as measured by the Hammersmith Functional Motor Scale – Expanded (HFMSE) in SMA patients on chronic dosing of standard of care therapies (either nusinersen or risdiplam).
- Continued high participation in the ONYX open-label extension study evaluating the long-term safety and efficacy of apitegromab in patients who completed the TOPAZ or SAPPHIRE trials. More than 90 percent of patients on combination therapy in the TOPAZ trial have completed 4 years of apitegromab treatment and enrolled into ONYX. Following trial completion, 98 percent of SAPPHIRE patients (185/188) enrolled in the ongoing ONYX open-label expansion study.
- Initiated Phase 2 EMBRAZE proof-of-concept trial with apitegromab in combination with a GLP-1 receptor agonist (GLP-1 RA) in obesity in May and completed enrollment in September.
- Presented new SRK-439 preclinical data at multiple conferences: ObesityWeek, American Diabetes Association's 84th Scientific Sessions (ADA) and Keystone Symposia's *Obesity: Causes and Consequences* meeting.
- Successfully completed upsized $345 million public offering in October to fund planned commercial launch in SMA and continue advancement of priority programs.

J.P. Morgan Healthcare Conference Presentation and Webcast

Scholar Rock management will highlight these updates in a corporate presentation at the 43rd Annual J.P. Morgan Healthcare Conference on Tuesday, January 14, 2025, at 9:45 a.m. PT (12:45 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 Apitegromab

Apitegromab is an investigational fully human monoclonal antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin in the skeletal muscle. It is the first muscle-targeted treatment candidate to demonstrate clinical proof-of-concept in spinal muscular atrophy (SMA). Myostatin, a member of the TGFβ superfamily of growth factors, is expressed primarily by skeletal muscle cells, and the absence of its gene is associated with an increase in muscle mass and strength in multiple animal species, including humans. Scholar Rock believes that its highly selective targeting of pro- and latent forms of myostatin with apitegromab may lead to a clinically meaningful improvement in motor function in patients with SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track, Orphan Drug and Rare Pediatric Disease designations, and the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan Medicinal Product designations, to apitegromab for the treatment of SMA. Apitegromab has not been approved for any use by the FDA or any other regulatory agency.

About the Phase 3 SAPPHIRE Trial

SAPPHIRE was a randomized, double-blind, placebo-controlled Phase 3 clinical trial that evaluated the safety and efficacy of apitegromab in nonambulatory patients with Types 2 and 3 SMA who are receiving current standard of care (either nusinersen or risdiplam). SAPPHIRE enrolled 156 patients aged 2-12 years old in the main efficacy population. These patients were randomized 1:1:1 to receive for 12 months either apitegromab 10 mg/kg, apitegromab 20 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks. An exploratory population that enrolled 32 patients aged 13-21 years old was also evaluated. These patients were randomized 2:1 to receive either apitegromab 20 mg/kg or placebo.

About EMBRAZE

EMBRAZE is a randomized, double-blind, placebo-controlled, Phase 2 proof-of-concept trial evaluating the efficacy, safety and pharmacokinetics of apitegromab in adults with a body mass index (BMI) of >27 (overweight) or a BMI of >30 (obese) and taking a GLP-1 RA (tirzepatide or semaglutide). The target enrollment of EMBRAZE is 100 subjects aged 18-65 who are overweight or

obese without diabetes. As part of the study design, the treatment period is 24 weeks, and all subjects will receive a GLP-1 RA. In addition, all subjects will be randomized 1:1 to receive either apitegromab or placebo by intravenous (IV) infusion every four weeks during the 24-week treatment period. The primary endpoint is change from baseline at Week 24 in lean mass assessed by dual-energy X-ray absorptiometry. Secondary endpoints include additional weight loss measures, safety and tolerability, and pharmacokinetic outcomes. Exploratory endpoints at Weeks 24 and 32 include cardiometabolic parameters (e.g. HbA1c), body composition, and physical function.

About SRK-439

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 cardiometabolic disorders, including obesity. Based on preclinical data, SRK-439 has the potential to support healthier weight management by preserving lean mass during weight loss. The efficacy and safety of SRK-439 have not been established and SRK-439 has not been approved for any use by the FDA or any other regulatory agency.

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 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.

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.

The efficacy and safety of apitegromab, SRK-181, and SRK-439 have not been established and apitegromab, SRK-181, and SRK-439 have not been approved for any use by the FDA or any other regulatory agency.

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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.

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, its product candidate selection and development timing, including timing for the initiation of and reporting results from its preclinical studies and clinical trials for apitegromab, SRK-439, linavonkibart and other product candidates and indication selection and development timing, its cash runway, 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," "could," "might," "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 for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. 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 3 trial of apitegromab or Part A or Part B of the Phase 1 trial of linavonkibart, are not predictive of, may be inconsistent with, or more favorable than, data generated from future or ongoing clinical trials of the same product candidate; Scholar Rock's ability to provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline; the data generated from Scholar Rock's nonclinical and preclinical studies and clinical trials, including from the EMBRAZE clinical trial; 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; the success of Scholar Rock's current and potential future collaborations; 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; its ability to establish or maintain strategic business alliances; its ability to receive priority or expedited regulatory review or to obtain regulatory approval of apitegromab; its ability to expand globally and the anticipated commercial launch in the United States of apitegromab in the fourth quarter of 2025; as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Form 10-K for the year ended December 31, 2023, and Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, 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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