Scholar Rock Submits Biologics License Application (BLA) to the U.S. FDA for Apitegromab as a Treatment for Patients with Spinal Muscular Atrophy (SMA)

January 29, 2025

- Scholar Rock's BLA submission is based on the Phase 3 SAPPHIRE trial that demonstrated a statistically significant improvement in motor function for patients receiving apitegromab compared to placebo, as measured by the Hammersmith Functional Motor Scale-Expanded at week 52
- The FDA has granted apitegromab Fast Track, Orphan Drug and Rare Pediatric Disease Designations in SMA
- Scholar Rock remains on track to submit the apitegromab Marketing Authorisation Application (MAA) to the European Medicines Agency in 1Q 2025, with PRIME and Orphan Medicinal Product Designations

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 29, 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 announced the submission of a Biologics License Application to the U.S. Food and Drug Administration (FDA) for apitegromab, a muscle-targeted therapy designed and developed to provide clinically meaningful improvement in motor function for people living with SMA who are receiving SMN-targeted treatments. The Company remains on track to file a Marketing Authorisation Application to the European Medicines Agency in 1Q 2025.

"We are gratified that in patients already on a SMN-targeted treatment, the SAPPHIRE trial met its primary endpoint for the main efficacy population showing a statistically significant 1.8-point improvement for patients receiving apitegromab compared to placebo, as measured by the Hammersmith Functional Motor Scale-Expanded at week 52," said Jing Marantz, M.D., Ph.D., Chief Medical Officer of Scholar Rock. "With the strength of our Phase 3 data as the foundation of our submission, we look forward to continuing to work closely with the FDA through the review of our BLA on behalf of patients and families living with SMA."

The review of the apitegromab BLA submission will be conducted by the Division of Neurology Products in FDA's Center for Drug Evaluation and Research. The submission is supported by data from the Phase 3 SAPPHIRE trial and the Phase 2 TOPAZ trial. The Company shared positive topline data from the SAPPHIRE trial in 2024, and additional data will be presented at the 2025 MDA Clinical & Scientific Conference being held March 16-19 in Dallas, Texas.

Scholar Rock has requested Priority Review which, if granted, would shorten the FDA's review time to six months from the date of filing acceptance. The 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.

Scholar Rock plans to also study apitegromab in SMA patients under two years of age in the Phase 2 OPAL trial, with a planned initiation in mid-2025. The trial will evaluate apitegromab in patients who have been or are continuing to be treated with any currently approved SMN therapy (nusinersen, risdiplam and onasemnogene abeparvovec).

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 clinically meaningful and statistically significant motor function improvement in a pivotal Phase 3 trial 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. 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 were 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 either apitegromab 10 mg/kg, apitegromab 20 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks for 12 months. An exploratory population including 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 every 4 weeks for 12 months.

The SAPPHIRE trial met its primary endpoint for the main efficacy population with a statistically significant 1.8-point improvement for all patients receiving apitegromab 10 mg/kg and 20 mg/kg (with an SMN-targeted treatment) compared to placebo (SMN-targeted treatments), as measured by the Hammersmith Functional Motor Scale-Expanded at week 52.

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.

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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 progress and plans for apitegromab. 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, 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; 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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