Dear SMA Community,

Scholar Rock is excited to announce that the SAPPHIRE study has opened clinical trial sites in Europe. SAPPHIRE is a global Phase 3 study assessing the efficacy and safety of apitegromab (SRK-015) in individuals with non-ambulatory (unable to walk independently) Type 2 or Type 3 SMA on SPINRAZA® (nusinersen) or EVRYSDI® (risdiplam).

Scholar Rock has begun enrolling patients in Europe, with our first clinical trial sites up and running in Spain. The team is working hard to open additional sites as soon as possible. We will also update the SAPPHIRE page on ClinicalTrials.gov (NCT05156320) with the new trial sites on a regular basis.

The SAPPHIRE study intends to enroll approximately 204 individuals with SMA across Europe and the USA. We plan to have European trial sites in Belgium, France, Germany, Italy, Netherlands, Poland, Spain, and the United Kingdom.

We would like to thank SMA Europe and the European community for their engagement as we continue to develop apitegromab. We will periodically provide updates of our progress.

Sincerely,
The Scholar Rock Team

About apitegromab

Apitegromab selectively inhibits the activation of myostatin (a protein found in skeletal muscles that restricts muscle growth and strength), which may improve muscle and motor function in people living with SMA.

Apitegromab is an investigational product candidate for the treatment of those living with SMA and has not been approved by the European Medicines Agency (EMA) or any other health authority.

About SAPPHIRE

SAPPHIRE is a Phase 3, placebo-controlled study assessing the efficacy and safety of apitegromab (SRK-015) in individuals aged 2-21 years with non-ambulatory (unable to walk independently) Type 2 or Type

3 SMA on SPINRAZA (nusinersen) or EVRYSDI (risdiplam). The study will have 2 groups of participants with the following estimated number of participants:

- 156 individuals aged 2-12 years.
- 48 individuals aged 13-21 years.

In SAPPHIRE, study participants will be randomly assigned to either apitegromab or placebo while continuing their existing treatment with either SPINRAZA or EVRYSDI under the supervision of their healthcare provider. The treatment period (when participants will receive apitegromab or placebo) in the study is planned to be 12 months long. After completing the 12 months in the study, participants will have the option to enroll in an open-label extension study of apitegromab or they will be followed for an additional 20 weeks after their last dose in the treatment period. Participants who enroll in the open-label extension study will receive apitegromab for the duration of the extension study. Participants in the 20-week follow-up period will no longer receive apitegromab or placebo.

Please visit ClinicalTrials.gov (NCT05156320) or mysapphirestudy.com for more information about the SAPPHIRE study.

Questions & Answers:

Who is eligible to participate in the SAPPHIRE study?

Individuals who meet the following criteria may be eligible to enroll in the study:

- Aged 2-21 years at the time of screening.
- Living with non-ambulatory (unable to walk independently) Type 2 or Type 3 SMA. A confirmed, documented diagnosis of 5q SMA will be needed. There are no restrictions on number of SMN2 copies.
- Able to sit up straight with their head erect for $\geq$ 10 seconds without using their arms.
- Comfortable receiving medication via intravenous infusion (directly into a vein over a period of time, in this case approximately 1-2 hours).
- Currently on SPINRAZA or EVRYSDI. Participants need to have completed at least 10 months of treatment with SPINRAZA or at least 6 months of treatment with EVRYSDI at the time of screening. Patients who have taken ZOLGENSMA® (onasemnogene abeparvovec) are not eligible for this study.

This is not a comprehensive list of all enrollment criteria in the SAPPHIRE study. For a full list please visit ClinicalTrials.gov (NCT05156320).

How can I enroll in the SAPPHIRE study?

A list of currently active study sites and their contact information can be found on ClinicalTrials.gov (NCT05156320). If you are interested in participating in the SAPPHIRE study, please speak with your healthcare provider and contact a study site. The decision to participate in a clinical trial should be made in consultation with your healthcare provider.