September 2, 2026

Dear FSHD Community,

We are pleased to share an important update with you: Scholar Rock has begun a Phase 2 clinical trial evaluating our investigational muscle-targeted treatment, apitegromab, in adults living with facioscapulohumeral muscular dystrophy (FSHD).

Called FORGE, this study is now open, enrolling, and participant dosing has begun. FORGE is a first step to assess the potential for apitegromab for people living with FSHD. FORGE builds on the clinically validated mechanism of apitegromab, the first and only muscle-targeted therapy to show statistically significant and clinically meaningful improvement in motor function in people living with spinal muscular atrophy (SMA) in a Phase 3 study.

We are also pleased to share that the U.S. Food and Drug Administration (FDA) granted apitegromab Fast Track designation for FSHD, a regulatory pathway intended to expedite the review of eligible investigational drugs that treat serious conditions and fill an unmet medical need. The FDA also granted apitegromab Orphan Drug designation for FSHD, a regulatory classification intended to support development and evaluation of new treatments for rare diseases.

While this milestone marks the beginning of a new clinical study, it also marks the start of a deeper relationship with the FSHD community. As we take this step forward, we wanted to share more about our company and who we are, why we are pursuing this research, and our commitment to the FSHD community to honor your experiences and voices.

Who We Are

For those who are unfamiliar with Scholar Rock, we have been working for over a decade to advance muscle-targeted treatment for rare neuromuscular diseases. Our company is named for the visual resemblance of naturally occurring rocks, called “scholar rocks,” to protein structures. We are the first company to complete a successful Phase 3 study with a myostatin inhibitor, apitegromab, in spinal muscular atrophy (SMA).

What is FORGE

FORGE is a Phase 2 trial designed to evaluate apitegromab, an investigational treatment that selectively inhibits myostatin, a protein that helps regulate muscle growth. We believe that apitegromab may hold promise for people living with neuromuscular diseases characterized by progressive muscle weakness, including FSHD. By inhibiting myostatin, which acts like a stop sign for muscle growth, apitegromab aims to promote muscle growth and improve motor function.

The purpose of this study, like any clinical research, is to answer important scientific and medical questions. We know that progress in rare disease research is only possible because of the contributions of people living with rare diseases, family members, caregivers, clinicians, scientists, and advocacy organizations. We are grateful for your time, expertise, and willingness to share your experiences as we work to advance innovation in FSHD.

Our Commitment

We are committed to deepening our engagement with the FSHD community by listening and learning from people living with FSHD, and we also understand that trust is earned over time. We look forward to keeping the FSHD community informed and working closely with trial enrollees and the advocate community.

We are also grateful to this FSHD community for everything it has done to advance awareness, education, support, and research. We are especially appreciative of the advocates and many community leaders who have helped create opportunities for patients, families, researchers, and industry to work together in pursuit of new treatment options for FSHD.

Please stay tuned for additional updates as FORGE progresses.

You can view our press release announcing the news **linked here.**
You can find the FORGE trial website here: **scholarrockforge.com**.

With gratitude,

Your Scholar Rock Patient Advocacy Team

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Frequently Asked Questions

What is FORGE?

- FORGE is a Phase 2 randomized, double-blind, placebo-controlled, multi-center clinical trial evaluating Scholar Rock's investigational therapy, apitegromab, in adults living with facioscapulohumeral muscular dystrophy (FSHD).
- The study is designed to help researchers better understand the efficacy, safety, and treatment effects of apitegromab in people living with FSHD.

What is a Phase 2 clinical trial?

- In a Phase 2 clinical trial, researchers evaluate how a treatment affects the body, gather safety data, and monitor for side effects. The treatment is often compared to a placebo.
- The FORGE Phase 2 clinical trial will help researchers better understand the potential effects of apitegromab in FSHD.
- FORGE has been informed by preclinical research demonstrating that myostatin inhibition can preserve and promote muscle health in FSHD disease models.

What does it mean that FORGE has been initiated?

- Trial initiation means that Scholar Rock has begun the process of conducting the study, including activating clinical trial sites and beginning participant enrollment and dosing.
- The first participant in FORGE has received their first infusion, meaning they have received either apitegromab or a placebo. This marks an important milestone as the study moves from planning into active execution.

What is apitegromab?

- Apitegromab is an investigational monoclonal antibody designed to selectively inhibit the activation of myostatin, a naturally occurring protein that helps regulate muscle growth.
- Myostatin acts like a stop sign for muscle growth and is expressed primarily in skeletal muscle, which are muscles that connect to bones and tendons (as opposed to other muscles like the heart). By targeting myostatin, researchers hope to better understand whether this approach may help address muscle weakness and functional limitations in certain neuromuscular diseases, including FSHD.
- Apitegromab does not address the DUX4 gene, which is the genetic cause of FSHD.

Has apitegromab been studied before?

- Yes. Before beginning the FORGE study in FSHD, apitegromab was evaluated extensively in a clinical development program for spinal muscular atrophy (SMA).
- Apitegromab has been studied for more than four years in children and adults living with SMA, representing more than 916 patient-years of clinical experience. In the Phase 3 SAPPHIRE trial, 99% of participants completed the study, and no participants discontinued treatment because of adverse events.
- Apitegromab for the treatment of SMA is currently under review by the U.S. Food and Drug Administration (FDA).

Why is Scholar Rock now studying apitegromab in FSHD?

- Scholar Rock's research focuses on muscle biology and rare neuromuscular diseases. Through our work studying apitegromab and the role of myostatin in muscle growth and function, we are advancing our understanding of whether this approach may have potential in additional diseases characterized by progressive muscle weakness, including FSHD. We understand that FSHD is characterized by progressive muscle weakness and loss of function, and there are currently no approved therapies for the disease.
- FORGE was designed based on insights from FSHD disease biology, natural history research, studies in mouse models, and Scholar Rock's prior experience studying apitegromab in neuromuscular disease.
- The study is intended to help researchers better understand whether apitegromab may have potential in adults living with FSHD.

Who is eligible to participate in FORGE?

- FORGE is enrolling ambulatory adults with genetically confirmed FSHD Type 1 or FSHD Type 2 who are 18 to 60 years of age and who meet other enrollment criteria, which can be found at the study website (scholarrockforge.com). FORGE is expected to enroll approximately 60 participants.

Do I need to have a genetic test to be included? If so, will this be provided?

- Yes, participants must have a genetic test.
- We recommend participants work with their doctor to start the process of getting a genetic test before trying to join this clinical trial. Genetic testing can take many weeks to complete, so starting the process early may be relevant in the enrollment process. Learn more about genetic testing.

How will the study team determine whether I can take part in the study?

- The first step is usually a pre-screening phone call to talk through the eligibility criteria. A coordinator at the study site will call participants. After this initial call, the coordinator may have additional pre-screening steps for participants to complete. If participants meet the initial requirements, the coordinator will schedule an in-person screening visit.
- The screening visit will take place at a study site. At this visit, participants will learn more about the study and provide their consent to proceed. Then, the study team will perform all the tests needed to determine if participants can take part in the study. This could take several hours, or participants may need multiple visits. After all test results come back, the study team will tell participants whether they can take part in the study. This screening process can take about 4 weeks.

Why is the study only enrolling adults?

- Conducting clinical studies in adults is a standard practice in the development of most new drugs and, since this is the first study of apitegromab in FSHD, the trial is only enrolling adults. FORGE data will help inform the enrollment criteria for future studies.

How was ambulation status for trial inclusion criteria determined?

- FORGE is intended to robustly test the impact apitegromab may have in adult individuals living with FSHD, including functional measures that require ambulation (the ability to walk or move from place to place) to complete. FORGE will help inform the ideal participants for future studies.

How is the study designed?

- FORGE is a randomized, double-blind, placebo-controlled, multi-center Phase 2 clinical trial. This type of study design helps researchers evaluate the effects of an investigational treatment as objectively as possible.
- Participants will be randomly assigned to receive either apitegromab or a placebo. Double-blind means that neither participants nor study investigators will know which participants are receiving apitegromab during the study.

Do inclusion/exclusion criteria have long-term implications for later access?

- FORGE includes various study measures and endpoints that together will inform the breadth of those who could potentially benefit from apitegromab.
- Our priority is understanding which people living with FSHD may benefit from apitegromab through a thoughtful clinical development program. It is too early to speculate on the totality of that development program and implications for access.

How can I learn more about participating?

- Individuals interested in learning more about FORGE should contact participating study sites or speak directly with their healthcare provider.
- Check the study website (scholarrockforge.com) or your local clinical trial registry, such as clinicaltrials.gov or eudract.ema.europa.eu, for the location of participating sites and eligibility criteria.
- Although all study locations are not yet operational, there are expected to be approximately 20 sites across North America and Europe.

Will some participants be given a placebo instead of the medicine?

- Yes, this is a placebo-controlled study. Half of the participants will receive a placebo and not apitegromab. Placebo is administered via intravenous (IV) infusion at the same treatment schedule as apitegromab but does not contain any active ingredient. Neither participants nor the study team will know who receives apitegromab and who receives the placebo.

Why does the study include a placebo group?

- Placebo-controlled studies are a standard part of clinical research and help researchers determine whether observed changes may be related to the investigational treatment.
- Comparing outcomes between study participants who receive apitegromab and those who receive placebo helps ensure that study results can be interpreted accurately.

How will the investigational drug be given?

- Apitegromab and the placebo will be given as an intravenous (IV) infusion. The infusion is given through a needle in a blood vein, usually in a participant's arm. Each infusion will take 1-2 hours. Participants will receive 13 infusions over 52 weeks.
- In addition to receiving the IV infusion, participants will also have tests and assessments done to monitor health and response to the IV infusion. Most study visits last 3 to 5 hours, but visits that include MRIs may take longer. The clinical trial study team will provide a schedule so that participants know what to expect at each visit.

What will researchers be measuring in FORGE?

- FORGE will evaluate changes in muscle-related measures as well as safety and tolerability.
- The study includes assessments of total lean muscle volume measured by an MRI scan (not through a biopsy), along with additional exploratory measures of muscle and motor function to help better understand the potential effects of apitegromab in FSHD.

How long will participation in FORGE last?

- The FORGE study will last up to 76 weeks (about a year and a half), which includes:
    - A screening period of about 4 weeks,
    - A 52-week study period with 13 infusions, and
    - A 20-week follow-up period.
- Study teams can provide more information about the trial schedule and participation requirements.

What activities will participants be asked to do?

- Participants will need to:
    - Undergo MRI scans and functional assessments
    - Perform a series of tests to assess muscle strength and function
    - Answer questionnaires about symptoms related to FSHD and how they are feeling
    - Use highly effective contraception. If participants have the ability to get pregnant, they will take regular pregnancy tests to confirm that they are not pregnant.

How many study visits are there? Are they in-person or virtual?

- If enrolled, participants will attend a study visit every 4 weeks to receive the IV infusion. In addition to receiving the IV infusion, participants will also have tests and assessments done to monitor their health and response to the IV infusion. Most study visits last 3 to 5 hours, but visits that include MRIs may take longer. The study team will give participants a schedule so that participants know what to expect at each visit.

Will I have to stay overnight for any visits?

- Some visits may require overnight stays, and Scholar Rock will reimburse hotel stays and expenses for up to 1 night.

Will apitegromab be offered to participants once the clinical trial is over?

- Yes, after 52 weeks of treatments, participants will have the option to take part in an open-label extension. This is an additional period where all clinical trial participants receive the investigational drug, even if they were previously on the placebo. Trial participants can decide whether they want to enroll in the open-label extension or not.

Who is paying for the clinical trial?

- Scholar Rock is paying for the study.

Will I have to pay for any medical expenses? Will my insurance be billed for any costs?

- No, all costs are paid for by Scholar Rock.

Will I be reimbursed for travel costs?

- Yes, Scholar Rock will provide travel and reimbursement for expenditures related to travel to study sites.

How many volunteers are needed?

- The study needs 60 participants. However, many more volunteers are needed because not everyone who volunteers will be eligible and able to take part.

Where are the study sites that are conducting the clinical trial?

- Check the FORGE study website (scholarrockforge.com) or your local clinical trial registry, such as clinicaltrials.gov or eudract.ema.europa.eu, for the location of participating sites and eligibility criteria.
- Although all study locations are not yet operational, there are expected to be approximately 20 sites across North America and Europe.

How do I contact a study site to learn more and sign up?

- If a study site is marked as "Recruiting," there should be an email and/or phone number listed for contact. Sometimes there is also contact information listed for sites that are "Not Yet Recruiting." Interested individuals should email and call these contacts and let them know that they are interested in the FORGE study.

What will happen after I contact the study site?

- It can take several weeks or months to hear back. Sites could still be in the process of getting ready for the study and may not be able to contact anyone just yet. Sometimes, sites are overloaded with more interested patients than they can enroll in the study.

Will participating in FORGE guarantee access to apitegromab?

- Participation in a clinical trial does not guarantee access to an investigational treatment outside the parameters of the study.

Does trial initiation mean apitegromab has been proven to work in FSHD?

- No. Apitegromab is investigational in FSHD and has not been approved by regulatory authorities for the treatment of FSHD. The purpose of FORGE is to help researchers better understand whether apitegromab has potential in people living with FSHD.

What happens next?

- Scholar Rock will continue enrolling trial participants across clinical trial sites. We look forward to providing updates to the community as the study progresses.
- Individuals interested in learning more about FORGE should speak with their healthcare provider and review information about the study.

When will results from FORGE be available?

- Clinical trials take time to complete, and researchers must carefully analyze the data.
- We look forward to sharing updates with the FSHD community and will provide information regarding study progress and future milestones when available.

Why is Scholar Rock studying apitegromab in FSHD when other investigational treatments have not been successful?

- Researchers have studied a variety of approaches targeting muscle biology over the years, including other approaches with an entirely different mechanism of action. Each investigational therapy has unique characteristics and study designs.
- Apitegromab is designed to selectively inhibit myostatin, a protein that helps regulate muscle growth, and is distinct from other investigational approaches that have been studied in FSHD.

What makes apitegromab different from other myostatin-targeting therapies that have been studied in the past?

- Apitegromab has been evaluated across multiple successful clinical studies, contributing to a growing body of clinical experience demonstrating its differentiated mechanism and safety profile. Through FORGE, researchers hope to better understand whether this approach may have potential for people living with FSHD.

- Apitegromab is administered through intravenous infusion and is designed to selectively inhibit myostatin activation throughout the body. The FORGE study is designed to help researchers better understand the effects of this approach for people living with FSHD.

What happens if new FSHD treatments become available while FORGE is ongoing?

- The FSHD research landscape continues to evolve, and we will continue to monitor new scientific and clinical developments, including other research programs underway.
- FORGE is designed to evaluate apitegromab as a standalone treatment. If important developments occur during the study, we will evaluate any potential implications, as appropriate.
- Participants should continue to discuss any questions about treatment options with their healthcare provider.

How will Scholar Rock engage with the FSHD community?

- As we advance our clinical program in FSHD, we are committed to building long-term relationships with people living with FSHD, caregivers, healthcare professionals, researchers, and advocacy organizations.
- We look forward to listening, learning, and sharing updates as our work in FSHD progresses.