November 14, 2025

Dear SMA Community,

We're writing with two updates on our Biologics License Application (BLA) for apitegromab in SMA.

1. In our previous community statement on October 29, we shared with you that Scholar Rock requested a Type A meeting with the FDA, as is standard practice following the receipt of a Complete Response Letter (CRL). Of the meeting types that can follow a CRL, Type A meetings are treated with the strongest sense of urgency. **The FDA granted us the meeting and on November 12, 2025, Scholar Rock met with the FDA in-person to discuss next steps for BLA resubmission**.
    - The meeting was constructive and collaborative, and it remains clear that the CRL we received from FDA in September is based solely on the status of the third-party Novo Nordisk fill-finish facility.
    - Catalent Indiana, LLC (part of Novo Nordisk), joined Scholar Rock, alongside Cure SMA, at the meeting and presented the progress in executing the remediation plan at the site and affirmed that it expects the facility to be ready for reinspection by the end of this year.

2. **In addition to continuing to work closely with Novo Nordisk, Scholar Rock has accelerated the process to bring a second fill-finish facility online for apitegromab.**
    - While we had always planned to onboard a second fill-finish facility, we have now accelerated our timelines. This second facility is a commercially approved fill-finish facility based in the U.S. that has a proven track record of successful site inspections.
    - The process of technology transfer is underway, and commercial capacity has been reserved beginning in the first quarter of 2026. Technology transfer, often referred to as "tech transfer," is the process of transferring knowledge, skills, and expertise from one facility to another.

Next steps: We remain in close coordination with Novo Nordisk as we await meeting minutes (the official record of the meeting) from the FDA. Resubmission of the BLA and U.S. launch, pending FDA approval, of apitegromab for children and adults with SMA is anticipated in 2026.

As we previously shared with you, apitegromab product being manufactured for pre-approval use, including the Early Access Program (EAP) and ongoing clinical trials, continues to meet all product quality, sterility, and safety requirements and ongoing product monitoring continues.

The regulatory challenges we face today are temporary, and we continue to believe that it is a not matter of *if* — but *when* — apitegromab will be approved and available for children and adults living with SMA in the U.S. The robust clinical and safety data we shared with FDA, and the absence of any clinical efficacy and safety data approvability concerns in our CRL, have only reinforced our conviction in the opportunity to make a meaningful difference for the SMA community.

For any questions, you can contact us at mstewarthart@scholarrock.com.

You can also find our latest press release on these updates here.

Your Scholar Rock Patient Advocacy Team

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