August 7, 2026

Dear SMA Community,

We are writing to update you on the regulatory review of apitegromab, our investigational treatment for spinal muscular atrophy (SMA).

The FDA previously accepted our Biologics License Application (BLA) submission with two fill-finish facilities included: Catalent Indiana LLC (part of Novo Nordisk) and a second, U.S.-based facility. We have been notified that FDA has classified its latest site inspection of the Catalent Indiana LLC fill-finish facility as Official Action Indicated (OAI). OAI typically means that FDA will require a satisfactory follow-up inspection of the facility before allowing any applications for new medicines that go through the facility to be approved for commercial use. Under the Agency's guidance, we will remove the Catalent Indiana facility from the apitegromab BLA and FDA's review of our application will continue with our second U.S.-based fill-finish facility.

Importantly, the apitegromab BLA remains on track for potential FDA approval at any time through the September 30, 2026 Prescription Drug User Free Act (PDUFA) action date.

As we've shared previously, we worked proactively with FDA earlier this year to establish two independent paths to approval:

- The apitegromab BLA was accepted by the FDA with two fill-finish facilities included, in alignment with FDA guidance.
- The second fill-finish facility is a U.S.-based facility producing numerous commercial products with a strong track record, including recent successful inspections.

Following notification of the facility-level classification on August 7, 2026, Scholar Rock will remove the Catalent Indiana facility from the BLA and FDA review will progress solely with the second fill-finish facility.

Scholar Rock remains prepared to launch apitegromab in the United States immediately upon approval. We have a robust supply of apitegromab from the second facility available, awaiting packaging and labeling, and ready for potential commercialization following FDA approval.

In Europe, we are engaging with the European Medicines Agency (EMA) on next steps to include our second fill-finish facility in the apitegromab Marketing Authorizations Application. We will provide updated timelines upon alignment with the EMA.

We recognize that the SMA community is following this process. Thank you for the opportunity to share this update as we continue our work to bring apitegromab to the SMA community as quickly as possible. You can read more in Scholar Rock's announcement **here**.

With gratitude,

Your Scholar Rock Patient Advocacy Team

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