

March 31, 2026

Dear SMA Community:

We are writing to share an important update that Scholar Rock has resubmitted our Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for our investigational treatment, apitegromab, in spinal muscular atrophy (SMA).

This resubmission marks a meaningful step in the regulatory process, and we remain thankful for your encouragement and continued support as we have worked toward this milestone.

What Happens Next

Next, the FDA will now assess whether the apitegromab application is ready for review. We expect FDA to determine its acceptance of our resubmission within 30 days. If the BLA is accepted, the FDA will begin a full review of the apitegromab application. We anticipate up to a six-month review period from the date of resubmission. It is important to note that a resubmission does not guarantee BLA acceptance or approval by the FDA.

What Led to BLA Resubmission

As we previously [shared](#), Scholar Rock received a Complete Response Letter (CRL) from the FDA solely related to observations identified during a routine inspection of a third-party fill-finish manufacturing facility, operated by Catalent Indiana, LLC (part of Novo Nordisk). More specifically:

- The observations were not specific to apitegromab
- The FDA did not raise concerns regarding efficacy or safety data
- The observations were not related to the drug substance manufacturer

Since that CRL, there have been several collaborative interactions:

- Scholar Rock and Cure SMA participated in a [Type A meeting](#) with the FDA
- FDA and Catalent Indiana met in the first quarter of 2026 to discuss progress in remediating the manufacturing facility, with no additional corrective actions requested by FDA
- These interactions resulted in alignment with the FDA to resubmit the apitegromab BLA prior to FDA reinspection of the Catalent Indiana manufacturing facility

What Else to Know

The apitegromab BLA resubmission now includes a second U.S.-based fill-finish manufacturing facility to strengthen our supply chain and support future demand. This update follows a positive Type C meeting between Scholar Rock and the FDA, which was held on March 3, 2026. The Type C meeting offered an opportunity for us to discuss important aspects of our drug development program, outside of the more structured Type A and B meeting formats. Based on Scholar Rock's ability to secure accelerated fill-finish timelines for anticipated apitegromab commercial supply, the company aligned with the FDA to include the second facility in the BLA resubmission.

On behalf of the entire team at Scholar Rock, we appreciate your support and the opportunity to provide updates as we work as quickly as possible to bring apitegromab to the SMA community. As a next step, Scholar Rock will update the community as information becomes available.

You can find our press release announcing our BLA resubmission here:

<https://investors.scholarrock.com/news-releases/news-release-details/scholar-rock-resubmits-biologics-license-application-bla-fda>.

With gratitude as we continue forward,

Your Scholar Rock Patient Advocacy Team
Marjorie Stewart-Hart
Vice President of Patient Advocacy
Scholar Rock

Leah Dzintars
Director of Patient Advocacy
Scholar Rock