



Thursday May 7, 2026

Dear SMA Community:

We are pleased to share that the U.S. Food and Drug Administration (FDA) has accepted to review Scholar Rock's Biologics License Application (BLA) for our investigational treatment, apitegromab, in spinal muscular atrophy (SMA) with a September 30, 2026 Prescription Drug User Fee Act (PDUFA) action date.

What BLA Acceptance Means

As part of the standard regulatory process, when the FDA accepts a BLA, it has determined that the application is ready to begin a full review. We expect a review period of up to six months and will make apitegromab available immediately upon FDA approval. It is important to note that a BLA acceptance does not guarantee approval by the FDA.

Additional Background

As we [shared on March 31, 2026](#), our BLA resubmission included Catalent Indiana (part of Novo Nordisk) and a second U.S. based fill-finish manufacturing facility, in alignment with FDA guidance during a positive meeting between Scholar Rock and FDA.

Next Steps

As we await the FDA's decision, our team is ready to launch apitegromab at any point following approval. We thank the SMA community for your continued support, and we look forward to providing information as it becomes available.

You can find an announcement of the news [linked here](#).

With gratitude,

Your Scholar Rock Patient Advocacy Team

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