September 23, 2025

Dear SMA Community,

Ahead of the U.S. Food and Drug Administration's (FDA) target action date for apitegromab, our team has been working urgently to prepare for the potential launch of the first muscle-targeted treatment for SMA. During this time, we've appreciated hearing directly from you about the difference that gains in motor function could bring to the community, and we've committed to keeping you updated on the regulatory process.

We're writing with news that the FDA has issued a Complete Response Letter (CRL) for our Biologics License Application (BLA) to approve apitegromab in SMA. **This response is solely related to manufacturing observations identified at a third-party facility,** Catalent Indiana LLC (Novo Nordisk's Indiana site). **Importantly, it did not cite any other approvability concerns, including apitegromab's efficacy and safety data.** A CRL is the FDA's way of informing a company that their application cannot be approved without changes. We wanted to notify you as soon as possible to assure you that we are working closely with the FDA to move forward.

Catalent Indiana is a third-party fill-finish facility which was acquired by Novo Nordisk A/S in December 2024. Novo Nordisk's Catalent Indiana site submitted a comprehensive response in early August 2025 to address the manufacturing observations noted by the FDA. Following that submission, Novo Nordisk's Catalent Indiana site has continued to work rapidly to take corrective action and has kept the FDA apprised of that progress.

We are continuing to work closely with Novo Nordisk's Catalent Indiana site on the FDA's manufacturing observations so that we can resubmit our apitegromab BLA as soon as possible. We believe that the FDA will be able to act expeditiously on our application once the third party manufacturing site issues have been resolved.

We know that improving motor function is a key unmet need for the SMA community, and we recognize the unfortunate impact of this delay. For more than a decade, we've been working towards a muscle-targeted treatment for SMA, and our commitment to that goal remains as strong as ever.

In addition to working with Novo Nordisk's Catalent Indiana site to resolve the manufacturing issues for FDA approval, we are pursuing approval by the European Medicines Agency (EMA) as we look to bring apitegromab to those living outside the United States, with European launch projected in 2026, upon receipt of marketing authorisation.

We also wanted to share Scholar Rock's press release on the news: https://investors.scholarrock.com/news-releases/news-release-details/fda-issues-complete-response-letter-crl-apitegromab-treatment

We will continue to keep you updated on next steps. For any questions, you can contact us at mstewarthart@scholarrock.com. Please do not hesitate to reach out — we are here to help.

Your Scholar Rock Patient Advocacy Team

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