October 29, 2025

Dear SMA Community,

We are writing to share two updates on our Biologics License Application (BLA) for apitegromab in SMA. As shared on September 23, 2025, Scholar Rock received a Complete Response Letter (CRL) from the FDA for the apitegromab BLA in SMA. As a reminder, the CRL was solely related to manufacturing observations identified at a third-party vialing facility, Catalent Indiana, LLC (owned by Novo Nordisk). Importantly, the CRL did not cite any other approvability concerns, including apitegromab's efficacy and safety data.

Although the FDA observations at the vialing facility were not specifically related to apitegromab, we proactively completed our own product-specific impact assessment to confirm that all apitegromab product vialed at Novo Nordisk's Catalent Indiana facility have met the product quality, sterility and safety requirements. Manufacturing of apitegromab for pre-approval use, including the Early Access Program (EAP) and ongoing clinical trials, continues today with robust controls in place to ensure that our product meets all applicable quality standards and regulatory requirements.

Below are the latest updates related to the apitegromab BLA:

Update 1:

We learned that the site inspection of Novo Nordisk's Catalent Indiana facility that led to our CRL was classified by FDA as "Official Action Indicated" (OAI). OAI classification typically means that FDA will require a satisfactory follow-up inspection of the facility before allowing any applications for new medicines manufactured at the facility to be approved for commercial use. During this time, commercially approved medicines can continue to be vialed at the facility and used by patients.

Although we know the SMA community shares our hope that this is not the case, there can often be extended timelines between FDA reinspection and the initial inspection. Novo Nordisk reports good progress addressing the observations noted by FDA, including completing the majority of the Corrective and Preventative Actions (CAPAs) in the remediation plan. We will continue to keep the SMA community informed as we work to expedite the process with the FDA.

Update 2: As is standard process for BLA applicants following receipt of a CRL, Scholar Rock has requested and is planning for what is called a Type A meeting with the FDA to discuss next steps for BLA resubmission.

We look forward to having more information to share with the community in November.

Our focus to urgently drive toward the BLA resubmission on behalf of SMA patients remains unchanged. For any questions, you can contact us at mstewarthart@scholarrock.com.

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