September 14, 2026

Dear SMA Europe:

Last Friday, we were able to share a moment that reflects not only scientific achievement, but the strength of a community that has never stopped advocating for more.

The U.S. Food and Drug Administration (FDA) has approved ISEMBYLD™ (apitegromab-mstn) for the treatment of spinal muscular atrophy (SMA) in adults and children two years of age and older who are currently receiving a survival motor neuron 2 (SMN2)-targeted treatment.

We want to express deep appreciation for the many insights shared with us by the community as we sought to develop a treatment that can positively impact the lives of people with SMA. Following the unexpected delay associated with the inspection results at a third-party manufacturing site, the community responded with trust, patience, and unwavering commitment, for which we are thankful.

With U.S. FDA approval, we now are highly focused on continuing our work, partnering with relevant regulatory agencies so that individuals living with SMA may have a clear path to access.

As we shared previously in August, following Official Action Indicated (OAI) inspection classification by the U.S. FDA at our third-party fill-finish facility, in alignment with European Medicines Agency (EMA), Scholar Rock has withdrawn the apitegromab Marketing Authorisation Application (MAA) and will resubmit with an alternate fill-finish facility to facilitate the most expeditious path to Committee for Medicinal Products for Human Use (CHMP) opinion.

We remain encouraged by the collaborative engagement with the EMA and our ability to advance the apitegromab MAA through the regulatory review process. We will provide more information as it becomes available.

On behalf of our entire team at Scholar Rock, thank you for your relentless determination and your insight. To those who participated in our clinical trials, who opened their lives to research – thank you. To the advocates who have elevated awareness of the daily motor

function challenges in SMA – thank you. To the individuals and families who have shared their hopes, their fears, and their wisdom – thank you.

You can find our press release announcing FDA approval here.

We sincerely appreciate your partnership and look forward to continuing important work for the SMA community together.

With respect and gratitude,

Your Scholar Rock Patient Advocacy Team

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