

September 11, 2026

Dear SMA Community:

Today, we 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. This approval brings the first and only FDA-approved treatment for those living with SMA to directly target the muscle.

We want to express deep appreciation from all of us at Scholar Rock for the many insights shared with us by the community as we sought to develop and deliver a treatment that can positively impact the lives of people with SMA. Following an unexpected delay, Cure SMA and the community responded with trust, patience, and unwavering commitment, for which we are thankful.

From Scholar Rock's founding over a decade ago, your voices have guided us: in the design of our clinical programs, in our understanding of motor function, in what gains are meaningful to you, and in the standards to which we hold ourselves. You made clear that motor function gains are not just points on a scale – they represent daily realities in your lived experiences with SMA. We have focused on these realities with every decision as we worked toward this milestone.

With FDA approval, we now are highly focused on continuing our work so that individuals living with SMA who may benefit from ISEMBYLD have a clear and prompt path to access.

- ISEMBYLD 10 mg/kg is administered once every 4 weeks as an intravenous (IV) infusion by a healthcare professional at an infusion center, hospital, or at home. ISEMBYLD will be available to ship in the coming days.
- Scholar Rock is working closely with top commercial and government payers to accelerate reliable, broad access to ISEMBYLD for appropriate patients.
- Scholar Rock has worked closely with the SMA community to build Scholar Rock Supports™, which is now available to assist patients who have been prescribed ISEMBYLD. Scholar Rock Supports offers personalized assistance at every step:
 - Help understanding and navigating insurance coverage
 - Financial assistance for eligible patients
 - Education regarding the ISEMBYLD treatment process and SMA
- Scholar Rock Supports will also help patients and families navigate their site of care options, as well as ongoing infusion logistics. Based on eligibility, infusions can be given at convenient locations including hospital, home, or infusion center.
- Patients and caregivers can learn more about Scholar Rock's patient support services by visiting www.ScholarRockSupports.com or calling 833-777-5444 (833-SRRK-444).
- For more information about ISEMBYLD and U.S. Prescribing Information, visit www.ISEMBYLD.com.
- For any questions related to accessing ISEMBYLD, the best place for an individual to start is by reaching out to their doctor.

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](#). **Cure SMA will be hosting an informational webinar for the community on Wednesday, September 30 at 12:00pm EST.** Please look for further communications and registration from Cure SMA.

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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