

October 5, 2026

RE: Request for Coverage of ISEMBYLD (apitegromab-mstn) for Health Plan Members with Spinal Muscular Atrophy

Dear Insurer,

On behalf of individuals with spinal muscular atrophy (SMA), including those covered by your health plan, **Cure SMA respectfully requests that you ensure immediate access to ISEMBYLD (apitegromab-mstn)**, a muscle-targeted therapy used in combination with an *SMN2*-targeted treatment. We urge you to ensure coverage of ISEMBYLD without restriction or delay for all adults and children with SMA 2 years of age and older who seek the treatment in accordance with the U.S. Food and Drug Administration (FDA)-approved label.

On September 11, 2026, the FDA approved ISEMBYLD (apitegromab-mstn) for use alongside an *SMN2*-targeted therapy for the treatment of SMA, a progressive neurodegenerative disease that causes severe muscle weakness and loss of motor function.ⁱ ISEMBYLD is a monoclonal antibody that inhibits activation of myostatin, a protein that limits muscle growth, and is intended to improve motor function in patients also receiving an *SMN2*-targeted therapy.

The FDA's approval was based on results from a randomized, double-blind, placebo-controlled Phase 3 study (SAPPHIRE), which demonstrated that ISEMBYLD (apitegromab-mstn) was safe and produced a statistically significant, clinically meaningful improvement in motor function for patients with SMA who were already receiving an *SMN2*-targeted treatment. The FDA noted, *"Those in the treatment arm were more than twice as likely than patients in the placebo arm to demonstrate a clinically meaningful improvement (34.2% vs. 13.5%)."*ⁱⁱ

Individuals with SMA and their caregivers consistently identify improving motor function and muscle strength as top goals to improve their health and independence, given most experienced significant muscle and motor function loss before accessing a genetic targeted treatment. The 2025 State of SMA report highlighted wide interest in a muscle-focused therapy to address these unmet needs.ⁱⁱⁱ As one **adult with SMA** shared, *"Just being stronger will lead to better outcomes from illnesses, decrease likelihood of hospital stays, and improve my mental health."*

Cure SMA urges you to cover ISEMBYLD (apitegromab-mstn) for all adults and children with SMA age 2 and older—regardless of *SMN2* copy number or ambulatory status—who are currently receiving a *SMN2*-targeted treatment. Your leadership in ensuring timely access to this therapy, consistent with the FDA label, can meaningfully change health outcomes across the SMA community.

Finally, **we stress the importance of patient choice and timely access to care.** Medical decisions should rest with patients, families, and healthcare providers — not be dictated by arbitrary mandates, bureaucratic review, or restrictive sequencing criteria for previous treatment and care choices. This is especially important as SMA treatment approaches evolve to include a dual-modality strategy. Delaying access until documented decline has occurred is harmful, since motor neuron loss and SMA-related damage are irreversible.

Thank you for your continued commitment to serving individuals with SMA and their families. Please contact Cure SMA with any questions or to share your plans for updating coverage policy on this treatment. Your team can contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at 202.871.8004 or maynard.friesz@curesma.org.

Sincerely,



Kenneth Hobby
President
Cure SMA



Jacqueline Glascock, PhD
Chief Scientific Officer
Cure SMA



Mary Curry
Vice President, Clinical Research and
Care, Cure SMA

ⁱ FDA Prescribing Information for ISEMBYLD, September 11, 2026, https://scholarrock.com/documents/label/us/ISEMBYLD_PI.pdf

ⁱⁱ FDA Approves First Therapy to Target Muscle Loss in SMA, September 11, 2026, <https://www.fda.gov/drugs/news-events-human-drugs/fda-approves-first-therapy-target-muscle-loss-spinal-muscular-atrophy>

ⁱⁱⁱ 2025 Cure SMA State of SMA Report, https://www.curesma.org/wp-content/uploads/2026/05/2025_State-of_SMA_Report_vWeb.pdf