

December 10, 2025

RE: Request for Coverage of Itvisma (onasemnogene abeparvovec-brve) for Insurance Members with Spinal Muscular Atrophy (All Types, All Ages Above 2 Years Old)

Dear Insurer:

On behalf of individuals and families affected by spinal muscular atrophy (SMA), including those covered under your health plan, **Cure SMA urges you to provide immediate access to Itvisma (onasemnogene abeparvovec-brve) without restriction or delay.** We request that your coverage policy align fully with the U.S. Food and Drug Administration (FDA) - approved label for this gene replacement therapy, which is indicated for all adults and children with any type of SMA age 2 and older.

SMA is a progressive neurodegenerative disease that affects the motor nerve cells in the spinal cord and impacts the muscles used for activities such as breathing, eating, crawling, and walking. Cure SMA is the national organization that represents children and adults with SMA, including SMA community members represented through your health plan.

The FDA highlighted the “significant unmet need”ⁱ within the SMA community when it approved Itvisma (onasemnogene abeparvovec-brve) for all individuals with SMA age 2 and older. The agency found that Itvisma demonstrated “*substantial evidence of effectiveness*”ⁱⁱ and determined that the single-dose therapy “*restores SMN protein production and halts further disease progression.*”ⁱⁱⁱ The FDA noted “*adequate justification*”^{iv} for expanding the indication beyond the pivotal study population to include adult patients with SMA. Reflecting this broad label, the FDA stated in its November 24 approval announcement, “*Today’s approval shows the power of gene therapies and offers treatment to patients across the SMA disease spectrum, including patients at various ages, SMA symptoms, and motor functional levels.*”^v

The FDA’s approval of Itvisma provides individuals with SMA with a treatment option to address long-standing and persistent medical challenges. A **43-year-old adult with SMA** shared, “*I am afraid of losing respiratory function. Dying from pneumonia is a great fear of mine and losing respiratory function would allow fluid to build in my lungs, making this a great possibility.*” **Another adult with SMA, age 34,** said, “*As I’ve gotten older, I have noticed my condition worsening.*” You can help change the trajectory of this disease and further improve health, function, and quality of life of individuals with SMA by supporting timely access to this therapy—without harmful restrictions or delays due to administrative barriers.

As SMA treatment and care options continue to evolve and expand, it is essential that healthcare decisions remain in the hands of patients, families and healthcare providers, and not be dictated by arbitrary mandates, or bureaucratic restrictions. **You can demonstrate its commitment to the health and well-being of individuals with SMA by ensuring unrestricted coverage of Itvisma for all individuals with SMA age 2 and older who seek this treatment.**

Thank you for considering the urgent and ongoing healthcare needs of children, teens, and adults living with SMA. We appreciate your partnership in ensuring that all individuals with SMA can access the treatments, therapies, and medical devices necessary to live healthier, safer, and more independent lives. Please do not hesitate to contact Cure SMA with any questions or to discuss your plans to update your coverage policies regarding this newly approved 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 Press Release: <https://www.fda.gov/news-events/press-announcements/fda-approves-gene-therapy-treatment-spinal-muscular-atrophy>

ⁱⁱ FDA Prescribing Information: https://www.novartis.com/us-en/sites/novartis_us/files/ltvisma.pdf

ⁱⁱⁱ FDA Press Release: <https://www.fda.gov/news-events/press-announcements/fda-approves-gene-therapy-treatment-spinal-muscular-atrophy>

^{iv} FDA Press Release: <https://www.fda.gov/news-events/press-announcements/fda-approves-gene-therapy-treatment-spinal-muscular-atrophy>

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