Make today a breakthrough.

March 7, 2025

Dear Insurer,

Earlier this month, the U.S. Food and Drug Administration (FDA) approved a new tablet version of Evrysdi, an oral treatment option for children and adults with spinal muscular atrophy (SMA). The FDA's action addresses treatment goals identified by individuals with SMA and their families related to easier treatment administration and year-round adherence. **Cure SMA respectfully asks you to provide individuals with SMA who seek the Evrysdi tablet formulation with full access to the treatment option without unnecessary barriers.**

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.

Evrysdi was first approved in 2020 as an oral liquid drug to treat SMA in children and adults. The treatment's at-home administration and efficacy have made Evrysdi an effective choice for many. Previous FDA prescribing information required that the liquid treatment bottle be stored "in a refrigerator at 2°C to 8°C (36°F to 46°F)." SMA community members have raised adherence concerns while traveling or during emergency situations. During the 2024 Cure SMA National Conference, individuals with SMA and their families could only attend the event if their rooms had refrigerators. *"I take the medication Evrysdi every day and it needs to be refrigerated,"* shared one conference attendee with SMA. However, neither of the conference hotels included in-room refrigerators. This new tablet version does not require any refrigeration.

The FDA's approval of a new tablet version of Evrysdi will provide greater flexibility and less impact on activities such as traveling and work, which will improve adherence. Many individuals with SMA have also noted access challenges related to the current 12-day bottle supply of the liquid version. The tablet's 30-day supply will ease these concerns and could also reduce administrative costs for providers and others. Finally, the FDA updated the Evrysdi label to reflect that now both the tablet and liquid version can be taken "with or without food."

Cure SMA asks that you update your policy to reflect the recent FDA approval and label of the Evrysdi tablet. Because the FDA concluded that the tablet "demonstrated comparable bioavailability" to the oral solution, and the safety profiles are consistent, we respectfully ask that you simplify the process for individuals with SMA seeking to switch between liquid and tablet formulations by not requiring new prior authorizations, clinical assessments, or other unnecessary steps that will interfere with patient access and choice.

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As you update your policy, please also ensure that your policy addresses any other access barriers, such as any restrictions based on age, ambulation or ventilation status, that do not correspond with the FDA label and fail to reflect the latest real-world data, particularly related to improvements for older adults with SMA. Cure SMA advocates for children and adults with SMA to have full access to the treatment most appropriate to their clinical needs and goals, as recommended by their healthcare provider. Burdensome steps or policy restrictions that lead to delays or gaps in treatments are harmful and will result in lost motor neurons and impacts on muscles and function.

Thank you for considering SMA community views and needs and for your coverage of healthcare services, including SMA treatments, that improve the health and well-being of individuals with SMA covered through your health plan. Please do not hesitate to contact Cure SMA if you have questions or need additional information. Cure SMA can be reached through Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at maynard.friesz@curesma.org or 202-871-8004.

Sincerely,

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