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April 15, 2026

Emma Sandoe
Medicaid Director
State of Oregon, Oregon Health Authority
500 Summer Street, NE E49
Salem, OR 97301

RE: Request Coverage of High Dose Spinraza (nusinersen) for Individuals with Spinal Muscular Atrophy

Dear Director Sandoe,

On behalf of individuals and families with spinal muscular atrophy (SMA) who are covered under your health plan, **Cure SMA respectfully requests that Oregon Medicaid provide timely and unrestricted access to High Dose Spinraza (nusinersen) for individuals with SMA who seek this treatment.** This request follows the Food and Drug Administration's (FDA) March 30, 2026 approval of the high dose regimen of Spinraza for the treatment of SMA across all ages and disease severities.[1]

SMA is a progressive, neurodegenerative disease that causes irreversible motor neuron loss, severe muscle weakness, and significant functional decline. Historically, the most severely affected individuals did not survive past their second birthday. Those who did often required permanent ventilation, feeding support, and 24/7 care from a multidisciplinary medical team.

The FDA's 2016 approval of Spinraza (nusinersen) at the original dosing, now referred to as the low dose regimen, marked a transformative moment in SMA care, which led to meaningful reductions in mortality, hospitalizations, and dependence on supportive medical equipment.[2] As one **grandparent of a child with SMA said,** *"My granddaughter was born with SMA and has lived beyond the original prognosis because of the clinical trial she was in and the subsequent approval of Spinraza."*

Despite these advances, substantial unmet needs remain, particularly for children and adults with SMA who experienced debilitating disease symptoms prior to initiating treatment. Many individuals with SMA continue to seek new options that may improve strength, reduce fatigue, and maintain or enhance motor function.[3] As one **adult with SMA** explained, *"When it is nearly my next dose, I find myself getting tired more easily. Getting less fatigued would help me do more."*

Spinraza (nusinersen) is an antisense oligonucleotide (ASO) administered intrathecally into the cerebrospinal fluid to increase production of survival motor neuron (SMN) protein, which is deficient in individuals with SMA. Building on the established clinical efficacy and real-world benefits of the low dose regimen, including improvements in motor milestones and reductions in *"the risk of death or*

[1] Cure SMA Announces FDA Approval of High Dose Spinraza, 2026, https://www.curesma.org/biogen-receives-fda-approval-of-high-dose-spinraza-for-the-treatment-of-sma/
[2] *Cure SMA State of SMA Report, 2024, https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb-4.pdf*
[3] *Cure SMA State of SMA Report, 2023, https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf#page=34*

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permanent ventilation," the FDA has also determined that High Dose Spinraza was safe and effective for both *"pediatric and adult patients."*[4]

Clinical data reviewed by the FDA demonstrated *"statistically significant improvement"* in motor function, based on the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND),[5] and found a *"statistically significantly greater percentage of patients"* with SMA who achieved treatment response on the Hammersmith Infant Neurological Examination (HINE-2).[6] **The FDA noted in the prescribing information that:** *"The overall findings from these trials support the effectiveness of SPINRAZA administered with either the Low Dose Regimen or the High Dose Regimen* ***across the range of ages and severities in patients with SMA****, and support the early initiation of treatment with SPINRAZA."*[7]

Given this, Cure SMA strongly urges Oregon Medicaid to provide full coverage of High Dose Spinraza, without restrictions or delays, for all individuals with SMA who choose the treatment, in accordance with the FDA label.

We respectfully emphasize that treatment decisions must remain in the hands of patients, families, and their healthcare providers, and not constrained by administrative barriers, restrictive sequencing requirements, or policies that delay access until irreversible decline has occurred. SMA is a genetic, degenerative condition in which motor neuron loss cannot be reversed. Delays in treatment can result in permanent loss of function. As treatment approaches evolve, it is essential that your coverage policies reflect the need for individualized, patient-centered care based on clinical judgment.

Thank you for your continued commitment to individuals with SMA and their families. Please feel free to contact Cure SMA with any questions or to share your plans for updating your coverage policies related to this new treatment option. 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

Jacqueline Glascock, PhD
Chief Scientific Officer

Mary Curry
Vice President, Clinical Research and Care

Maynard Friesz
Vice President, Policy and Advocacy

[4] FDA Prescribing Information for High Dose Spinraza, 2026, https://www.spinraza.com/content/dam/commercial/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf
[5] FDA Prescribing Information for High Dose Spinraza, 2026, https://www.spinraza.com/content/dam/commercial/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf#page=17
[6] FDA Prescribing Information for High Dose Spinraza, 2026, https://www.spinraza.com/content/dam/commercial/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf#page=18
[7] FDA Prescribing Information for High Dose Spinraza, 2026, https://www.spinraza.com/content/dam/commercial/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf#page=14