July 15, 2026

RE: The Current State of Spinal Muscular Atrophy: Thank You and Call to Action

Dear Insurer:

As the national organization representing individuals and families with spinal muscular atrophy (SMA), **Cure SMA is pleased to share our latest *State of SMA* report.** This report reflects a decade of transformation in SMA, driven by therapeutic innovation, universal newborn screening, and access. It also underscores the ongoing challenges individuals and families affected by this rare neuromuscular disease continue to face due to unmet needs and insurance-related barriers.

We share these findings as both a **thank you** and an urgent **call to action**. Together, we have helped transform SMA from the leading genetic cause of infant mortality into a treatable condition in which many individuals are living longer, healthier, and more independent lives. At the same time, the data makes clear that progress is not yet complete. Too many individuals still face delays, denials, and gaps in care that limit outcomes. We respectfully urge you to build on this progress by ensuring timely, consistent, and comprehensive access to the full spectrum of SMA care.

REMARKABLE CHANGES IN THE SMA COMMUNITY

The last decade has fundamentally reshaped the SMA landscape. Today, more individuals with SMA are being diagnosed earlier, accessing effective treatment, and living longer than ever before. Cure SMA estimates that approximately 9,500 individuals with SMA are living in the United States, and the majority (64%) are adults. The increased number of teens and adults in the last decade highlights the improvement in survival and health, driven by a nearly 60% reduction in mortality.

Earlier diagnosis has been a key driver of these improved outcomes. The median age at diagnosis has dropped from 1.2 years in 2017 to just 7 days in 2025, enabled by newborn screening in all 50 states. Just as critical, the average time from diagnosis to treatment has decreased from 196 days to just 28 days, allowing intervention during the most important window to preserve motor neurons and so function. Evidence shows that earlier treatment leads to better outcomes. For example, children treated earlier report improved swallowing and stronger vocal function, and prior findings demonstrated that children with two *SMN2* copies who were treated earlier achieved significantly better motor milestones than those treated later.

Today, approximately 77% of individuals with SMA have received an FDA-approved therapy, contributing to meaningful improvements in health and function. As one adult with SMA shared, *"I have benefited greatly from SMA research and treatments that have slowed the progression of this deadly disease, resulting in less intensive and fewer health-related medical visits."*

Many individuals are maintaining or gaining milestones once thought impossible. For example, 63% of children with SMA are ambulatory, and reliance on feeding tubes has declined significantly from 35% in 2019 to 17% in 2025. Even when traditional motor gains are not observed, clinicians report important benefits, including disease stabilization (72%), reduced need for respiratory support (55%), and fewer infections (55%). These findings highlight the importance of broader outcome measures in evaluating treatment effectiveness. Importantly,

improved health is also enabling greater participation in daily life. Together, the data demonstrates that access to treatment and specialized care has transformed SMA into a manageable, evolving condition. **You have played an important role in enabling this progress.**

CONSISTENT BARRIERS IN ACCESSING CARE

Despite these advancements, access to care remains inconsistent and too often delayed or denied. Approximately half of all children and adults with SMA report having received an insurance denial for a treatment prescribed by their provider. These denials have serious consequences. Twenty-two percent of newly diagnosed families experienced treatment delays of one to three months, most often due to insurance denials or extended review processes. For a progressive disease like SMA, even short delays can result in irreversible loss of motor neurons and long-term function. Initiating treatments within 14 days of birth rather than after 90 days was the difference between walking and not walking by age two for children with two *SMN2* copies. As one SMA parent shared, *"Waiting on insurance drastically changed [my child's] future and our lives."* These experiences reinforce what the data clearly shows: **timely access is not just important, it is critical**.

Barriers also extend to other essential healthcare supports and services. While 78% of individuals rely on durable medical equipment (DME), nearly 49% report insurance denials for equipment such as power wheelchairs, standers, and lifts. One parent with SMA shared, *"Currently, insurance only approves a wheelchair. My son is more fit for a motor scooter, which is not covered."* Similarly, access to supportive therapies remains limited. Among adults with SMA, 52% report not receiving physical therapy despite believing it would be beneficial. Key barriers include limited coverage (60%), denials (23%), and lack of provider expertise (55%). Financial and geographic challenges further compound these issues. Fifty-one percent of adults report concerns about treatment affordability or coverage, and many individuals, particularly children and those in rural areas, must travel long distances to access specialized SMA care.

These persistent barriers not only delay care but also create administrative burden for providers and emotional strain for families—ultimately undermining the full potential of treatment advances. **Addressing these challenges represents a critical opportunity for you to further improve outcomes.**

URGENT NEED TO ADDRESS UNMET NEED

Even with this significant progress, individuals with SMA continue to face substantial unmet needs. SMA remains a complex and progressive condition. Fifty-three percent of individuals report unmet medical needs that current treatments do not address, and more than 80% of treated individuals express interest in additional therapies, including muscle-enhancing or combination approaches. As one adult with SMA described, *"increasing weakness in right arm affecting ability to drive wheelchair,"* highlighting the need for additional treatment options and care. Another adult with SMA said, *"I began treatment a few years ago. It has been successful, but I still haven't regained the ability to walk."*

Respiratory health remains one of the most pressing concerns. Thirty-four percent of individuals with SMA require breathing support, and roughly one-third identify respiratory function as a top unmet need. One community member said, *"a cold could lead to a month of breathing problems."* Additionally, 54% of adults report concern about respiratory complications and safety during

anesthesia.

Beyond physical health, the broader impact of SMA is significant. Seventy-eight percent of adults report that SMA affects their mental and emotional well-being, driven by factors such as disease progression, dependence on caregivers, and long-term uncertainty. As one community member said, *"Every day, every action, every dream, every simple task is blurred by SMA."*

Gaps in healthcare access compound these challenges. More than half (54%) of adults report receiving incomplete or inadequate care, particularly in emergency medicine, preventative care, reproductive health, and rehabilitation services. Additionally, many individuals manage comorbid conditions, including chronic pain, osteoporosis, and hypertension, which require coordinated, multidisciplinary care that is not always accessible or sufficiently covered. These findings make clear that while treatment has changed the trajectory of SMA, it has not eliminated the need for comprehensive patient-centered care. **We respectfully urge you and insurers across the country to address these unmet needs by reducing barriers across the full continuum of SMA care.**

A FINAL THANK YOU AND CALL TO ACTION

Cure SMA is deeply grateful for the role you have played in advancing access to life-changing therapies, durable medical equipment, and specialized care. As the SMA landscape continues to evolve, we look forward to working together to eliminate remaining barriers and ensure that coverage policies reflect the urgency of early treatment and the lifelong needs of individuals living with SMA. Thank you for your continued partnership and commitment to improving outcomes for individuals living with SMA. Your team can contact Maynard Friesz, Vice President for Policy and Advocacy at Cure SMA, at 202.871.8004 or maynard.friesz@curesma.org.

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