



Make today a breakthrough.

May 29, 2025

Dear Insurer,

Last year, Cure SMA provided an update on the state of spinal muscular atrophy (SMA). Our 2024 outreach highlighted the benefit of current SMA treatments and reported the significant unmet needs and healthcare barriers faced by children and adults with SMA. Since then, Cure SMA has collected direct experiences from the SMA community documenting treatment impact, healthcare barriers, and unmet needs. We are pleased to share our latest data and first-person perspectives to assist and inform you in best meeting the needs of individuals with SMA covered by your health plans.

SMA TREATMENT IMPACT ON SURVIVAL RATES AND HEALTH OUTCOMES

Strong SMA treatment utilization in the U.S. has resulted in significant improvements in the survival, health, and well-being of children and adults with SMA. More than 70 percent of all individuals with SMA in the U.S. are on a U.S. Food & Drug Administration (FDA) approved SMA treatment, up slightly from the previous year. SMA treatment access has contributed to an 80 percent drop in the mortality rate, from 1.84 in every 100 individuals with SMA in 2014 (before SMA treatments) to only 0.42 per 100 in 2024, according to our latest [State of SMA report](#).¹ *"SMA is a terrible diagnosis that if untreated would kill kids by the time they are two. Instead, our kids are alive and thriving thanks to therapies,"* said a **mother of two children with SMA** (ages 1 and 3). A **father of a child with SMA** said, *"My son is alive today due to the recent breakthrough therapies available to treat SMA."* SMA is no longer considered the leading genetic cause of infant death.

Current SMA treatments have resulted in decreased hospitalization, reduced reliance on specialized care and equipment (i.e., feeding tubes and breathing machines), and other improved healthcare measures. *"Before I started treatment I was often hospitalized for many reasons. Since starting treatment, I have not been unexpectedly hospitalized,"* said a **teenager with SMA**. A **parent of a school-aged child with SMA** said her daughter *"has no major issues with breathing, swallowing, or many other symptoms that go with spinal muscular atrophy."* Overall health and quality of life have improved among treated individuals with SMA of all ages and SMA types. A **parent of a young boy with 4 SMN2 copies** said, *"We continue to see him having great blood work results every week and also hitting all his milestones and continuing to grow and gain muscle/mass"* since accessing an SMA treatment. An **adult with SMA Type 3** said, *"A few years ago I began treatment for my SMA. It has been successful."* While a **35-year-old father with SMA** said SMA treatments have *"allowed me to continue the life that I am used to with my wife, kids, and career."*

SMA COMMUNITY EXPERIENCES WITH HEALTHCARE BARRIERS & DELAYS

Despite evidence to support full access to SMA treatments, the majority of individuals with SMA report frequent insurance denials for initial treatment authorization (44%), treatment renewal (33%), or both (17%). In addition, individuals with SMA also experience barriers in

¹ 2024 State of SMA Report, Cure SMA, https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf

accessing other health care, including durable medical equipment (51% report initial denials), physical and occupational therapy (44% of adults report no or limited coverage), and appointments with specialists (29% report scheduling barriers).

Following treatment denials, about two-thirds of individuals with SMA won their appeal. However, the denial and lengthy appeals process resulted in harmful and unnecessary treatment delays and gaps. A **mother of a child with SMA** said, *“We ended up running out of medication. Our son had an increase in falling, which then resulted in him relying on his wheelchair for mobility.”* A **43-year-old adult with SMA** had to *“stay in bed”* during the 2 to 3-week gap in treatment due to *“more progressive weakness and fatigue.”* Others described the impact on their mental health. A **35-year-old adult with SMA** said, *“It’s exhausting physically and mentally trying to navigate such an important treatment with insurance companies that don’t understand and just want to check boxes and never really take time to understand.”* A **parent of a child with SMA** said the treatment denial made her *“feel like a terrible parent that can’t help their child”* while a **22-year-old with SMA** said *“it’s had a drastic effect on my mental health, and potentially physical as well.”*

NEED TO RESTORE STRENGTH AND ADDRESS UNMET NEEDS

Current treatments effectively slow or stop further motor neuron loss and future disease progression, but they do not reverse past SMA-related damage. Since most individuals with SMA lost significant muscle strength and motor function before accessing SMA treatment, they seek new and add-on therapies to address their chronic health challenges related to muscle weakness, severe fatigue, motor function loss, and other SMA symptoms. A **young adult with SMA** who has not known a life without these devastating challenges said, *“It’s hard to say how having these needs more sufficiently met would impact me since I don’t have any other experience to compare my current way of living too.”*

GAINING MUSCLE STRENGTH

About 90% of individuals with SMA said gaining muscle strength was a top goal for new therapies taken in combination with existing treatments. A **24-year-old with SMA** said, *“If I had more upper mobility, I would be able to perform some of the medical procedures that I rely on caregivers for.”* A **51-year-old with SMA** said, *“If I got some muscle strength back, I could stand for more than a few seconds without being in pain. I could prepare my own meals.”* A **47-year-old adult with SMA** said, *“If I had more strength, I would be able to do more things by myself and maintain my independence. It would allow me to not have to rely on caregivers as much. I would be able to work again. I could eat and drink whatever and whenever I wanted to. Life-changing stuff!”*

ADDRESSING SEVERE FATIGUE

About 70% of individuals with SMA said severe fatigue was a top challenge. A **37-year-old with SMA** said, *“Being less tired will enable me to work longer, earn more money, and live a better life. It would also allow me to eat better because I could afford better food.”* A **43-year-old with SMA** said that she is *“too tired to do anything”* after 3 p.m. New treatments to reduce severe fatigue would also greatly improve the health, well-being, and safety of children with SMA. The **parent of a 6-year-old with SMA** said a fatigue-reducing treatment

“would help as our son would be able to keep up with class activities and work. And be able to stay awake for the bus ride home, which is unsafe in the event of an accident.”

GAINING NEW MOTOR FUNCTION & IMPROVING FINE MOTOR SKILLS

Individuals with SMA also seek greater motor function and fine motor skills. A **31-year-old with SMA** said, *“Gaining new motor functions would allow me to regain the strength of my youth, giving me more independence in daily living... such as feeding myself again.”* Gaining new motor functions could also reduce hospitalizations and related healthcare costs due to fewer falls, bladder infections, and other health complications. A **35-year-old with SMA** who required stitches due to a recent fall said, *“Improved muscle strength would remove the fall risk, make me safer, and make me less terrified I’ll fall & not be able to get back up.”* An **adult with SMA** said, *“If I was less afraid of falling, I could move more physically, probably be more likely to lose weight and be healthier.”*

ADDRESSING OTHER UNMET NEEDS THROUGH HEALTHCARE, INCLUDING NEW TREATMENTS:

Individuals with SMA and their families seek access to new combination treatments and other healthcare supports to decrease dependency on assistive devices, improve breathing, increase mental health, improve swallowing, strengthen communication, and address other challenges.

- **Mobility Devices:** *“Gaining strength could possibly improve the things I could do for myself and give me more stamina to walk more easily with my walker or not depend on a wheelchair when out of the house.” (49-year-old with SMA)*
- **Breathing:** *“Stronger lungs would allow me to feel less tired and short of breath by the end of a workday. Moreover, I will get less winded when talking in meetings at work.” (31-year-old with SMA)*
- **Mental Health:** *“New treatments would definitely help feeling depressed or sad most times and give me a somewhat normal life.” (47-year-old with SMA)*
- **Swallowing:** *“If her swallow were to improve, we could try to give her soft foods like apple sauce.” (Parent of a 7-year-old with SMA)*
- **Communicating:** *“I could speak louder with better lung function, cheer on my step kids at events, be less fatigued, be able to have medical procedures requiring anesthesia, and be less afraid of pregnancy.” (34-year-old with SMA)*
- **Eating:** *“New therapies to help me gain strength and achieve new function that I’ve either lost or have never before had ... like having the energy to eat entirely on my own.” (33-year-old with SMA)*
- **Grooming:** *“Being able to raise her arms above her eyebrows would allow her the ability to brush and wash her own hair.” (Parent of a 16-year-old with SMA)*
- **Typing:** *“If I type for extreme amounts of time my arms will just become two fleshy pool noodles and become barely usable. With increased energy I could do more.” (19-year-old with SMA)*
- **Holding Small Objects:** *“I long for being able to once again hold a pencil and sign documents, journal, artwork, writing letters, and working on my memoir.” (63-year-old with SMA)*

The SMA community is willing to accept some side effects (i.e., nausea, vomiting, dizziness) if add-on therapies that work in combination with existing treatments can restore function and strength, according to a Cure SMA Risk/Benefit Survey. An **adult male with SMA** said, *"I am patiently waiting for the day I can add an additional therapy to my treatment and see even more gains."*

CONCLUSION

Thank you for being a key partner in helping to change the SMA prognosis and improve the health of individuals with SMA. We also recognize though that far too many children and adults with SMA still face unnecessary barriers to accessing lifesaving healthcare and suffer from debilitating SMA symptoms that negatively impact their health and independence. Cure SMA respectfully asks that you review your policies and procedures to eliminate current delays and gaps in healthcare treatments, services, and to support the future healthcare needs of individuals with SMA covered through your health plans. Thank you for considering the views and perspectives of individuals with SMA and their caregivers. Please contact Cure SMA at maynard.friesz@curesma.org or 202-871-8004 if you have any questions.

Sincerely,



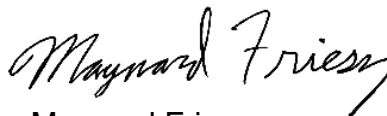
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