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June 24, 2024

Re: New Cure SMA Data on Treatment Impact and Harmful Access Barriers

Dear Insurer,

As the leading national organization dedicated to individuals and families impacted by spinal muscular atrophy (SMA), **Cure SMA is pleased to share some updates and results from our newest *State of SMA* report**. While the state of SMA is greatly improving, our data also points to troubling signs related to barriers faced by the SMA community in accessing clinically recommended healthcare. We ask that you consider this information to improve access to treatments, therapies, and equipment for all individuals with SMA enrolled through your health plan.

SMA is a progressive neurodegenerative disease that robs people of physical strength by affecting the motor nerve cells in the spinal cord, impeding their ability to walk, swallow, and—in the most severe cases—to breathe. Historically, individuals with SMA required aggressive care from a multi-disciplinary team of health professionals to survive. The most severely affected patients needed permanent ventilation, feeding tubes, and costly, intensive, around the clock medical care. Before treatments, babies born with SMA Type 1, the most common form of the disease, often died before reaching their second birthday.

Today, SMA is much different than it was just a decade ago. SMA is no longer considered the leading genetic cause of infant death. The SMA mortality rate has decreased by one-third, from 2.36 in every 100 individuals in 2013 to only 0.75 per 100 in 2023. Hospitalizations and reliance on specialized care and equipment are also down significantly. Over the past three years, annual hospitalizations decreased by nearly one-third for both adult and pediatric patients, BiPAP breathing utilization declined from over 30 percent to 25 percent, and feeding tube use fell from 35 percent to 20 percent.

The prognosis of SMA has significantly improved thanks to early diagnosis, powerful treatments, and specialized healthcare equipment and supports. Because of universal newborn screening—which was achieved in all 50 states in January 2024, the median age of SMA diagnosis declined from well over a year (497 days) to less than two weeks (12 days). Early diagnosis has led to earlier access to treatment. The median age at first treatment for infants diagnosed through newborn screening is now under one month, regardless of SMN2 copy number. The combination of newborn screening and early access to treatments is leading to unprecedented developmental achievement. Our newest data shows that 100 percent of newborns with 2 SMN2 copies who received treatment within two weeks are walking at age two. Conversely, only 50 percent of babies with 2 SMN2 copies reported walking at age two when first treated between 30 and 90 days, and 0 percent of babies with 2 SMN2 copies walked when treatment initiation occurred three months or later. Previous data found that untreated babies with SMA Type 1 lose 90 percent of their motor neurons by age 6 months. This real-world evidence demonstrates the importance of early access to an SMA treatment for all babies, regardless of their SMN2 copy number.

Timely access to treatment is critical to all individuals with SMA, regardless of age, given each day without treatment represents lost motor neurons and developmental opportunities. New SMA community data points to troubling signs related to access barriers for needed treatments, equipment, and therapies. The cause is not a lack of insurance coverage, as 98 percent of individuals with SMA are enrolled in public and private insurance plans. Access barriers are caused by utilization protocols or other harmful access barriers included in the patient's health plan. **More than half (55 percent) of all individuals treated with a SMA treatment reported receiving an insurance coverage denial for the treatment.** Of these individuals, two-thirds reported that they eventually won their appeal and received the SMA treatment. The motor neurons lost during this bureaucratic delay cannot not be regenerated and result in permanent loss of motor function. **Insurance barriers also hampered access to other essential healthcare, including durable medical equipment (DME) and physical and occupational therapies (PT/OT).** A significant number of individuals with SMA (50 percent of all adults and 32 percent of all children) reported receiving an insurance denial related to DME. In addition, 33 percent of children with SMA and 38 percent of adults with SMA said insurance does not cover their PT and OT needs. Significant access barriers identified through this new data are jeopardizing SMA community advancements and, unless addressed, could lead to greater reliance on more costly care.

You have contributed toward the progress and current state of SMA. We appreciate and rely on your partnership to ensure that all children and adults with SMA receive full and immediate access—without harmful and irreversible delays—to the treatment and other healthcare services that are most clinically appropriate to them, based on their choice and specific needs and the recommendation of their healthcare provider. We encourage you to review your policies and procedures to ensure that they do not create unintended barriers to accessing healthcare, including treatments, medical equipment, therapies, and other health services.

The *State of SMA* report validates the effectiveness and importance of SMA treatments, medical equipment, and care, which are greatly improving lives and reducing reliance on costly, specialized care. This is especially the case for individuals with SMA who received early access to treatments before symptom onset. However, the largest segment of the SMA population consists of older individuals who were born years or decades before the approval of SMA treatments and implementation of newborn screening. **Because SMA is not cured, significant unmet needs remain in the SMA community, particularly for adults and older children**. Congress recognized the unmet needs of the SMA community in federal legislation approved earlier this year. New treatments are required to achieve top SMA community goals, which include gaining muscle strength, achieving new motor function, reducing fatigue, improving respiratory function, decreasing dependency on assistive devices (i.e. wheelchairs, cough assist machines), improving swallowing, and improving voice strength. Cure SMA and the SMA community are encouraged by the new therapy candidates in various clinical trial stages aimed at accomplishing those key goals.

Thank you for considering Cure SMA's data related to the SMA community and its ability to access healthcare. 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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