



Make today a breakthrough.

February 11, 2018

The Honorable Alex Azar  
Secretary of Health and Human Services  
U.S. Department of Health and Human Services  
200 Independence Avenue, SW  
Washington, D.C. 20201

Dear Secretary Azar:

On February 8, the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) recommended that spinal muscular atrophy (SMA) be added to the Recommended Uniform Screening Panel (RUSP). The final decision on this recommendation now rests in your hands.

We join Senators Edward Markey and Orrin Hatch, and Representatives Greg Walden and GK Butterfield along with their colleagues, in urging you to approve the recommendation without delay. A timely approval will ensure that states begin implementation as quickly as possible for this devastating disease. Approximately one baby affected with SMA is born each day in the U.S. Each day that passes represents a missed opportunity for early detection and life-saving treatment.

Spinal muscular atrophy (SMA) is the most common genetic cause of death for infants today. It affects approximately 1 in 11,000 babies. The natural history of SMA type 1, the most severe and most common form, results in death or permanent ventilation before two years of age.

On December 23, 2016, the U.S. Food and Drug Administration (FDA) approved the first-ever treatment for spinal muscular atrophy, SPINRAZA™. In clinical trials of SPINRAZA, infants treated before showing symptoms are walking, talking, and in many cases reaching age-appropriate developmental milestones. To date, none of the infants treated before showing symptoms have died.

On behalf of the entire SMA community, we hope you will respond quickly to this matter and approve SMA for inclusion on the RUSP. If you have any questions or if we can provide you with any additional information as you review this request, please contact Kenneth Hobby, President of Cure SMA, at 847 709-6324 or via email at [kenneth@curesma.org](mailto:kenneth@curesma.org).

Sincerely,

Kenneth Hobby  
President

Jill Jarecki, PhD  
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