



THE SECRETARY OF HEALTH AND HUMAN SERVICES
WASHINGTON, D.C. 20201

JUL 02 2018

Joseph A. Bocchini, Jr., M.D.
Committee Chairperson
Advisory Committee on Heritable
Disorders in Newborns and Children
5600 Fishers Lane
Room 18W68
Rockville, MD 20857

Dear Dr. Bocchini:

Thank you for your recent letter, on behalf of the Advisory Committee on Heritable Disorders in Newborns and Children (Committee), regarding the Committee's recommendation to add Spinal Muscular Atrophy (SMA) due to homozygous deletion of exon 7 in *SMN1* to the Recommended Uniform Screening Panel (RUSP).

I reviewed the Committee's evidence review report, which provides support that early screening and treatment can lead to decreased mortality for individuals with SMA and improved motor milestones. Additionally, I have taken into consideration the utility of current screening technologies, treatment for SMA, and the impact on public health systems, and accept the Committee's recommendation to expand the RUSP to include the addition of SMA.

I want to clarify that the addition of SMA to the RUSP does not constitute a requirement for states to implement screening and is only a recommendation. In addition, I ask the Committee to provide a report to me within 2 years describing the status of implementing newborn screening for SMA and clinical outcomes of early treatment, including any potential harms, for infants diagnosed with SMA.

Please accept my personal thanks to you and the members on the Committee for all of your dedicated work to improve the health of our nation's infants and children.

Sincerely,

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Alex M. Azar II