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May 8, 2018

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

Dear Secretary Azar,

We write regarding the recent recommendation by the Health Resources and Services Administration (HRSA) federal Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) to add spinal muscular atrophy (SMA) to the Recommended Uniform Screening Panel (RUSP) for newborn screening. We urge you to swiftly review the recommendation.

Each year, approximately four million newborns in the United States are screened for many serious and debilitating conditions that are not clearly apparent at birth. Newborn screening ensures that such disorders are identified early, leading to prompt initiation of treatment in hope of achieving the best possible health outcomes for the affected child. Delayed diagnosis of conditions until symptoms are overtly present can be disastrous and potentially fatal.

One such condition, spinal muscular atrophy (SMA), is a leading genetic cause of infant death. SMA affects the nerve cells in the spinal cord that control motor function, robbing many patients of the ability to walk, eat, or breathe. It can affect any race or gender. The most severe type of SMA, Type 1, is also the most common type. Based on symptom onset alone, affected babies are typically diagnosed within their first six months of life and rarely survive beyond two years without intensive and invasive therapies, including permanent mechanical ventilation.

In December 2016, the Food and Drug Administration (FDA) approved the first drug treatment for SMA. Clinical study data for this treatment appears to support initiation of treatment in the pre-symptomatic phase of the disease. Screening newborns for SMA will shorten diagnosis time and provide greater likelihood that treatment will start before symptom onset, thereby improving the prospect that a child will not experience serious developmental delays or premature death.

The Department of Health and Human Services (HHS) maintains a list of conditions that it recommends states include in their newborn screening programs, the "Recommended Uniform Screening Panel" (RUSP). The ACHDNC advises the HHS Secretary on the most appropriate application of universal newborn screening tests, including recommendations for adding conditions to the RUSP. Many states work quickly to update their state's screening panel to include a new condition once it has been approved by the Secretary for addition to the RUSP.

On February 8, 2018, the ACHDNC recommended to the Secretary that SMA screening be added to the RUSP. Following ACHDNC review and recommendation, the Secretary has up to 120 days to accept or reject the recommendation. However, time is of the essence. To delay the recommendation further than absolutely necessary for thorough review of the ACHDNC's findings would be a tragedy for children born in the interim who may benefit from screening because they will miss the window for receiving treatment when it is most effective. As any postponement could delay diagnosis and access to time-critical treatment for babies suffering from this terrible and often-fatal disease, we urge you to act expeditiously in reviewing the ACHDNC recommendation.

Thank you for your consideration of this matter.

Sincerely,

Greg Walden
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Member of Congress

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