National MPS Society
Support for Families. Research for a Cure.

Advisory Committee on Heritable Disorders in Newborns and Children Votes to Approve MPS II for Recommended Uniform Screening Panel

Durham, NC

February 11, 2022 - The National MPS Society announces that the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) has voted to recommend mucopolysaccharidosis type II (MPS II, Hunter Syndrome) to the Recommended Uniform Screening Panel (RUSP) for newborn screening. This will be provided to the Secretary of the U.S. Department of Health and Human Services. Conditions listed on the RUSP are provided to individual states for recommendation for adoption for newborn screening.

This nomination, led by National MPS Society President/CEO Terri Klein and Chief Scientific Officer Matthew Ellinwood, was the product of years of work and collaboration, publications, and a community with a purpose. Klein shares:

> *This is a momentous occasion for the MPS II Patient Community. Our expert committee worked for three years to produce a successful nomination. Screening newborns for MPS II will provide equitable access to immediate, life-saving therapies. Since ERT has been available for our boys, we have witnessed increased quality of life and an opportunity for young men to thrive in the world.*
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> *Now, early access will erase many of the debilitating manifestations of this disease and newborns treated with ERT will have access to eventual therapies that address cognitive decline.*
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> *We are grateful to the ACHDNC committee for their acknowledgement of the solid evidence provided to recommend screening babies across the country. A new day for MPS II has just begun.*

We would like to extend thanks and gratitude to the EveryLife Foundation for Rare Diseases, MPS Superhero Foundation, and Project Alive for their oral commentary. We also recognize and appreciate the families and individuals with MPS II who shared their stories and experiences, and the thousands of signatories who contributed to the letter of support provided to the ACHDNC.

Our efforts to support newborn screening for all diseases remain at the forefront of our work. With MPS I and MPS II now on the RUSP, the Society will submit a request for MPS VII, Sly Syndrome, in the coming months. We are investigating paths forward to present requests for MPS III, IVA, and VI and continue to prioritize this work, recognizing this as an effort that supports preservation of physical and cognitive functioning for babies diagnosed and directly saves lives.