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January 24, 2013

The Honorable Fred Upton
Chairman
Committee on Energy and Commerce
United States Congress
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Henry Waxman
Ranking Member
Committee on Energy and Commerce
United States Congress
2125 Rayburn House Office Building
Washington, D.C. 20515

Dear Chairman Upton and Ranking Member Waxman,

FightSMA is pleased to offer its enthusiastic endorsement of the National Pediatric Research Network Act (H.R. 225). We thank you for advancing this important bill quickly through the Energy and Commerce committee and deeply appreciate your commitment to press for its consideration on the House floor as soon as possible. FightSMA is a non-profit organization of families across the nation working to find a treatment or cure for spinal muscular atrophy (SMA), the leading genetic killer of children under the age of two.

We have been grateful for Congress' longstanding support for SMA research, including House passage of the NPRNA last year and the annual Labor-HHS appropriations report language Congress has included to encourage the National Institutes of Health (NIH) to expand its support for translational and clinical research. Privately funded research has produced a number of promising drug therapies for SMA that are now at the door of the clinic, and the development of an effective and accessible clinical trials infrastructure is our next challenge and our greatest opportunity.

FightSMA has been proud to work with Reps. Lois Capps (D-CA) and Cathy McMorris Rodgers (R-WA) in the development of the National Pediatric Research Network Act (NPRNA). It would strengthen the federal investment in pediatric research by authorizing adequate funding for infrastructure and core services needed to support translational and clinical research, including for rare diseases like SMA. The NPRNA would authorize NIH to establish up to 20 pediatric research consortia throughout the nation. Each consortium would be investigator-initiated and competitively selected through a rigorous review process and consist of

Spinal Muscular Atrophy (SMA) kills more babies than any other genetic disease.

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multiple institutions in a "hub and spoke" arrangement, and consortia would be required to communicate regularly with one another. Awards would provide resources to create the infrastructure necessary to conduct leading-edge pediatric research today and well into the future, including support for promising young investigators beginning their careers in pediatrics. FightSMA recently launched an initiative to attract a new generation of researchers into the SMA field, joining with the Gwendolyn Strong Foundation to fund the Emerging Investigator Awards program. Having NIH as a partner in the effort to recruit young, talented researchers will improve scientific research and leverage additional private resources in the quest to find treatments for SMA and many other pediatric disorders.

Recognizing the significant budget challenges the federal government faces today, we firmly believe that the NPRNA will strengthen the nation's commitment to pediatric medical research in a cost-effective manner, allowing us to promote the well-being of our nation's children through a collaborative approach that makes the most of every federal dollar.

Thank you very much for your consideration. We look forward to working with you to secure enactment of the National Pediatric Research Network Act as soon as possible.

Sincerely,

Daniel Hayden
Executive Director
FightSMA

Michael Calise
Chairman
FightSMA