

United States Senate

WASHINGTON, DC 20510

January 21, 2016

Dr. Francis Collins
Director, National Institutes of Health
9000 Rockville Pike
Bethesda, MD 20892

Dr. Catherine Y. Spong
Acting Director, National Institute of Child Health and Human Development
21 Center Dr. Room 2A03, MSC 2425
Bethesda, MD 20892

Dear Drs. Collins and Spong:

On behalf of children across the nation living with devastating pediatric diseases, we urge you to fully and promptly implement the National Pediatric Research Network Act (NPRNA), Public Law 113-55, in a meaningful and timely manner. As Directors of the National Institutes of Health (NIH) and the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) – the entities responsible for executing this law – we respectfully request that you provide an update on the steps your offices have taken to comply with the law’s intent, particularly related to the establishment of new pediatric research consortia.

As you know, the NPRNA was passed by Congress and signed into law by President Obama in November 2013. The NPRNA was designed to expand the geographic scope of research, increase access to research programs for children across the country, and improve the variety and number of institutions participating in this critical research. In order to achieve these goals, the law recognizes the need for new, pediatric research consortia to conduct or coordinate multi-site clinical trials for pediatric rare diseases or conditions, such as spinal muscular atrophy and Duchenne muscular dystrophy. It has now been over two years since the bill’s enactment, and the NIH has yet to create a national network of research consortia focusing on rare pediatric diseases. This is what Congress intended.

We recognize and support your ongoing effort to conduct an analysis of existing research networks and centers supported by NIH and improve communication and data sharing across these existing programs. However, we strongly urge you to work with stakeholders, investigators, clinicians, and patient organizations to create new pediatric research consortia with the capacity to address the many unmet needs that exist in the current pediatric research environment. Highly-successful research networks, such as the National Cancer Institute’s Children’s Oncology Group or the Pediatric Heart Network, offer models that can be applied to pediatrics more broadly as new research networks are selected by NIH through a competitive review process.

Specifically, we would like clarification in the following areas:

- When Congress passed the NPRNA, the bill's authors and advocates made it clear that the purpose of the legislation was to create *new* consortia to form a nationwide network of pediatric researchers. While the law permits the use of existing consortia, centers, or networks in developing a national pediatric research network, the bulk of the statute is dedicated to the organization and goals of the pediatric research consortia receiving awards under paragraph (d)(2)(A). Please describe the specific steps that NIH and NICHD have taken and plan to take to ensure that congressional intent is followed as the NPRNA is implemented.
- What is the timeline for implementation? When will a final program plan be in place, and when do you anticipate that a solicitation or solicitations will be developed and announced?
- How do your plans for implementation address rare diseases or conditions, including through multi-site clinical trials?
- How do your plans address the need to expand the variety and number of institutions participating in research for rare, pediatric diseases?
- How do you plan to address the law's provisions regarding training researchers in pediatric research techniques?

We were encouraged to hear Dr. Kathy Hudson, NIH's Deputy Director for Science, Outreach and Policy, testify before the House Energy And Commerce Committee on April 30, 2015, when asked about the implementation of NPRNA, that "we look forward to building this new network" while ensuring it is complementary to and not duplicative with existing research networks. Now is the time to take full advantage of the opportunity offered by the NPRNA to build on the strong body of pediatric research that NIH and NICHD currently conduct and support.

We look forward to your response to our questions and concerns. Timely and robust implementation of the law is of the utmost importance to Congress and to the thousands of families waiting for a treatment for the diseases and disorders affecting their children. Thank you for your continued dedication to improving the health of our nation.

Sincerely,



Sherrod Brown
U.S. Senator



Roger F. Wicker
U.S. Senator



Sheldon Whitehouse
U.S. Senator



Mark Kirk
U.S. Senator

Bill Nelson

Bill Nelson
U.S. Senator

Rob Portman

Rob Portman
U.S. Senator

Bob Casey, Jr.

Robert P. Casey, Jr.
U.S. Senator

Chris Coons

Christopher A. Coons
U.S. Senator

Jack Reed

Jack Reed
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J. A. B.

Cory A. Booker
U.S. Senator

Robert Menendez

Robert Menendez
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Richard Blumenthal

Richard Blumenthal
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Tom Carper

Thomas R. Carper
U.S. Senator