The National Pediatric Research Consortia Establishment Act

FAQ Sheet

1) What is the purpose of the legislation?

The National Pediatric Research Consortia will accelerate the development of new treatments and therapies to combat devastating diseases that affect children through a greater investment in basic, translational, and clinical research. The NPRC will empower outstanding peer-reviewed researchers and research institutions by providing state-of-the-art technologies and developing interdisciplinary collaborations and multi-institutional networks. This approach will lead to new understanding of the biological and genetic basis of major disorders that afflict children, and translate this knowledge into prevention and intervention strategies and improved outcomes that have immediate and lifelong impacts. Many adult diseases (e.g. diabetes, coronary heart disease, obesity, hypertension) have their beginnings in childhood and are influenced by genetics or a lifestyles. The NPRC will support research across the lifespan to address these and other diseases, improving quality of life and lessening the economic burden.

2) What is the difference between basic research and translational research?

Both basic and translational research are vital to improving child health. Basic research focuses on very early stage initiatives and is exploratory and investigative in nature. The fruits of basic research provide the foundation on which further study and work are built upon. Translational research describes the more advanced stages of the research process whereby scientific discoveries are moved, typically at a more rapid pace, from the laboratory to the patient. This legislation recognizes the need for expanded pediatric research in both the basic and translational spheres.

3) Why do we need Pediatric Research Consortia?

Despite the fact that about 20 percent of our nation's population is in the pediatric care range, only about 12 percent of NIH funding is directed to children, with an even smaller amount directed to pediatric departments and Children's Hospitals. In fact, a recent study that tracked NIH funding of pediatric research over the past 15 years that concluded that over this span, the proportion of the budget devoted to the pediatric research portfolio declined overall. This inadequate investment hinders the pursuit of new discoveries and therapies and discourages young researchers from focusing on pediatric research. Another problem is insufficient networking of existing pediatric research programs. Infrastructure to develop such networks is greatly needed if we are to pursue translational research and other pediatric initiatives.

4) What does the legislation mean by infrastructure?

Cutting edge research in the post-genomics era requires access to large-scale infrastructure that includes intellectual, technological, training, and patient resources. Given this requirement, the legislation will support resources including gene banks or other repositories of patient data that could be shared by multiple sites, training programs for new researchers just beginning their careers in pediatrics, and even multi-center clinical trial sites to bring together an adequate pool of patients with a rare condition.

5) Doesn't NIH already provide awards for pediatric research and infrastructure? Why is it necessary to create the National Pediatric Research Consortia?

No. While NIH does support research into childhood diseases and conditions, this legislation will intensify this commitment and direct additional resources to consortium participants chosen from among the leading research institutions in the country. NIH currently has no program dedicated to supporting infrastructure investments increasingly needed to facilitate these types of intensified research programs, especially those focusing on multi-disciplinary and translational state-of-the-art approaches that emphasize multi-institutional networks.

6) What is the difference between the CTSA and the proposed National Pediatric Research Consortia?

There are three important fundamental differences between Clinical and Translational Science Awards (CTSAs) and the proposed National Pediatric Research Consortia. First, as their name implies, the CTSAs are focused exclusively on translational research and supporting clinical trials. The NPRCs, however, will focus on both translational and basic research. The CTSAs are also focused on clinical research across the breadth of academic institutions and specifically defining new relationships with community clinical research studies. Children's hospitals cannot compete for CTSAs alone and are required by NIH regulations to partner with degree-granting institutions. The practical result of this requirement is that pediatrics is forced to compete with more prevalent adult research initiatives for resources. Finally, while many smaller institutions are simply unable to compete for a CTSA, the NPRCs would be comprised of pediatric institutions of various sizes working together to provide important settings for research in childhood diseases. So while the CTSAs are important initiatives, they are not designed to focus on pediatrics the way the NPRC will.

7) How will the consortia work?

The National Pediatric Research Consortia would be modeled after the very successful National Cancer Institute (NCI) Comprehensive Cancer Centers. These comprehensive centers have been the impetus for developing many new therapies in cancer patients. In addition, the NCI concept is further improved in this legislation by employing a consortia approach in pediatrics. Each consortium will be formed from a collaboration of cooperating institutions in a "hub and spoke" model where a lead institution coordinates activities involving various satellite centers. This means that while the number of consortia would be capped at 20, a larger number of pediatric research institutions and children's hospitals would

be able to participate in this program by coming together to form a consortium, likely at a regional level. Each center would provide enabling infrastructure to support translational research by providing state-of-the-art technologies and developing interdisciplinary collaborations and multi-institutional networks.

8) How will NIH select each Consortium? Will there be a competitive process?

A rigorous NIH review process will determine consortium awards. Private or public nonprofit agencies or institutions will all be eligible to apply.

9) How much will this cost?

The legislation authorizes annual investments in each consortium up to $2.5 million. Grant periods will be for 5 years and may be extended in additional 5-year increments by the NIH Director after a successful peer review. Consortia will likely be rolled out to reach the total of 20 over the course of 3 to 5 years.

10) How will patients benefit from this legislation?

By marshalling federal research dollars into a targeted pediatric research program, NIH will substantially increase the infrastructure needed to advance and accelerate the process of finding treatments and cures for childhood diseases and disorders, as well as adult onset disorders rooted in the pediatric years. In particular, enhanced coordination of research around the innovation and development of therapies used in children (such as pharmaceuticals, biologics, and devices) will expedite evaluation and dissemination of the effects of such therapies in pediatric populations. This bill also will help fund essential training programs to ensure we lay adequate groundwork to attract, nurture, and retain the next generation of researchers. In addition, non-drug treatments (such as biological agents) are often developed in the setting of academic medical institutions. Thus, these centers will focus on developing therapies for genetic-based conditions in fetal life, infancy, and childhood that could have major implications across the lifespan.

11) How will taxpayers realize a return on this investment?

If enacted, this legislation will result in a targeted infusion of federal resources into a number of our nation's leading pediatric research institutions. The benefits will include an accelerated pace of discovery leading to a healthier population, as well as a better-trained corps of next generation pediatric investigators. The consortia will empower outstanding peer-reviewed researchers and research projects by providing state-of-the-art technologies and developing interdisciplinary collaborations and multi-institutional networks. This approach will lead to new understandings of the biological and genetic basis of major disorders that afflict children, as well as into disorders rooted in childhood that have lifelong implications. The investment will also have a key economic development impact by spurring life science innovation and new business opportunities through the discovery of new drugs and treatments.