

VIEWPOINT

AMERICAN PEDIATRIC SOCIETY

Advocacy for Research That Benefits Children

An Obligation of Pediatricians and Pediatric Investigators

Scott C. Denne, MD
Department of
Pediatrics, Indiana
University School of
Medicine, Indianapolis.

William W. Hay, Jr, MD
University of Colorado
School of Medicine,
Aurora.

Pediatricians have a long and distinguished history of advocating for children. From its founding in 1888, the American Pediatric Society has publicly advocated for the development of new knowledge for the care of infants and children including a special emphasis on preventive medicine.¹ In 1921, pediatrician members of the American Medical Association supported federal legislation (the Sheppard-Towner Act) that was designed to develop prenatal care services for poor women and health services for poor children. Because the American Medical Association strongly opposed this federal program, the pediatricians decided to form their own organization dedicated to the health of all children—the American Academy of Pediatrics.²

An important component of advocacy by pediatricians and pediatric organizations has been to champion research that benefits children including the proper inclusion of children in clinical trials. Robert Cooke, MD, chair of pediatrics at Johns Hopkins University, in 1960 strongly urged the director of the National Institutes of Health (NIH) to make clinical research centers available to children; his request was denied on the basis that results from adult studies could be used to extrapolate to children. The perseverance of Cooke and pediatric organizations eventually resulted in the NIH recognizing the value of clinical trials in children. These efforts also led the NIH and President John F. Kennedy to establish the National Institute of Child Health and Development (now the Eunice Kennedy Shriver National Institute of Child Health and Development). As a result, clinical trials involving childhood diseases were developed. Perhaps the best example of such early clinical research success is in the area of pediatric cancer, supported by long-standing funding from the NIH. This success has been greatly aided by high clinical trial participation rates of children and young adults with cancer; older adults enroll in trials at much lower rates. These sustained efforts have transformed the outcome of childhood cancer from an essentially incurable disease to one with a combined 5-year survival rate of 80%.

Despite this remarkable success in pediatric cancer, continued advocacy by pediatricians and the public has been needed to ensure wider inclusion of children in clinical trials. Children are a vulnerable population and thus properly receive special considerations and protections from the risks of participating in clinical trials. However, beginning in the late 1980s and catalyzed by the AIDS crisis, the public began to appreciate not only the risks but also the benefits of medical research. Im-

proving access to research participation was viewed as important, leading to changes in federal policy in 1993 that required the inclusion of women and minorities in clinical trials. Strong advocacy by pediatric organizations resulted in 1998 in the NIH instituting a similar rule requiring the inclusion of children in NIH-supported clinical trials.³

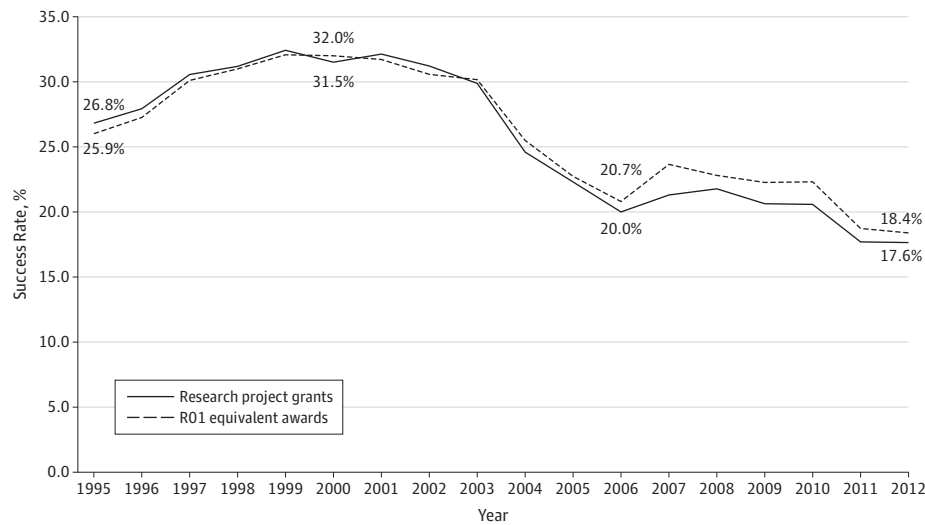
At the same time, pediatricians were pushing to better ensure the safe and effective use of medications in children. Clinical trials of most drugs did not include children, leading to extensive off-label drug use with limited information extrapolated from adult studies. An important body of legislation, beginning with the 1997 US Food and Drug Administration Modernization Act, provided an incentive for pharmaceutical companies and other sponsors to carry out drug trials in children.⁴ These laws have been remarkably successful, resulting in more than 425 drug labels being revised to include new pediatric information. Reauthorization of this legislation was initially required every 5 years, but concerted advocacy efforts by pediatric organizations resulted in the legislation being made permanent at the end of 2012.

These examples of persistent, determined advocacy efforts by the pediatric community have successfully advanced the opportunities for children to benefit from research. Now, we are positioned to enable many more children to reap the benefits of new and improved therapies. Furthermore, there is greater recognition throughout the scientific community that the understanding and prevention of important public health problems in adulthood—such as obesity, cardiovascular disease, and mental illness—require that investigations begin early in the lifespan including preconception and fetal research.⁵ Parents and the public appear to better appreciate the necessity and benefits of pediatric research, reflected by active participation in clinical trials. More than 120 000 children have enrolled in more than 300 pediatric interventional studies completed in the last 5 years (<http://www.clinicaltrials.gov>).

Today, the pediatric research community has enormous potential to build on its past successes and accelerate advances in child health and wellness. There are new therapeutic possibilities for childhood diseases, scientific opportunities for lifespan and life course research to understand adult diseases, and growing public support for pediatric research. But there are significant threats to realizing this potential. Resources to support the NIH are under intense pressure. President Barack Obama's budget request for the NIH for fiscal year 2013

Corresponding Author: Scott C. Denne, MD, Indiana University School of Medicine, Department of Pediatrics, 699 Riley Hospital Dr, RR 208, Indianapolis, IN 46202 (sdenne@iu.edu).

Figure. Success Rates for National Institutes of Health Grants



The success rates exclude the American Recovery and Reinvestment Act of 2009. Adapted with permission from the Federation of American Societies for Experimental Biology.⁶

is the lowest since 2001 after taking inflation into account. The number of research project grants funded by the NIH has consistently declined during the last 8 years. Success rates for grant applications are at the lowest level in 20 years and have fallen nearly 50% in the last 12 years (Figure).⁶ Child health research funding at the NIH has been reduced more than overall NIH research funding, putting children at even greater disadvantage.⁵

Biological scientists, including pediatric researchers, are often reluctant public advocates for their work. Many are concerned about the appearance of self-promotion and the difficulty of explaining the complicated nature of their investigations. However, pediatric researchers and pediatricians have an individual obligation to children to build on decades-long successful advocacy efforts, especially at this time of opportunity and challenge.⁷ Education of both the public and lawmakers is needed to make the results of pediatric research projects understandable and clearly related to improving the lives of children. It also is an opportune time to remind everyone that solving health problems early in life helps establish preventive measures that can reduce later-life diseases, promoting health and, at the same time, economic prosperity. This is a population issue with potential benefit for the entire country. At this time of financial uncertainty and reduced federal research spending, it is important to take a long-term view of advocacy and remember that all previous advocacy successes required years of perseverance by many individuals and pediatric organizations.

There are many ways that pediatricians and pediatric researchers can engage in effective advocacy including meeting with members of Congress to personally testify about the opportunities and successes of research in children and the need for resources to support these efforts. The American Pediatric Society and the American Academy of Pediatrics, along with several other pediatric organizations, provide researchers with advocacy training sessions, relevant written materials, and help with setting up the visits with members of Congress and their staff.

While personal engagement of researchers with legislators can help to influence public policy, there is an even simpler way that pediatricians and pediatric investigators can participate in advocacy:

help to build a library of pediatric success stories. One of the most effective tools of advocacy is to identify and relate specific, living examples of how child health research has benefitted the lives of children. Both NIH officials (including Director Francis Collins) and legislators have identified success stories as a critical element to building support for research funding. These stories also can serve to make the activities of pediatric researchers more accessible to the public at large. They may also be useful additions to the new NIH Director's Blog that serves to highlight important discoveries in biology and medicine.

The American Pediatric Society advocacy committee is requesting assistance from all pediatricians and pediatric investigators in collecting research success stories that demonstrate to legislators the marked value of research in children and the tremendous return on investment such research support has produced in improved health and in economic contributions of the healthier children throughout their lifespan. This is particularly important for child health research because it has fared less well over the years, yet offers the greatest opportunity for long-term improved health and economic benefit. Examples would include the benefits of research that have led to vaccines for childhood infectious diseases, prenatal steroids and surfactant for reducing death and morbidity from neonatal respiratory distress syndrome, cancer therapies, back to sleep positioning to reduce sudden infant death syndrome, child abuse prevention, and many more. The collected examples will be made available to the Public Policy Council of the Pediatric Academic Societies to assist in engaging the support of members of Congress, to Collins and the NIH, and to any organization advocating for pediatric research support.

Specific examples of research that have benefitted children's well-being or health can be submitted to William Hay, MD, chair of the Advocacy Committee of the American Pediatric Society, at bill.hay@ucdenver.edu or through the American Pediatric Society website (<http://www.aps-spr.org/about/aps/committees/academic-advocacy.asp>). Real-life stories of patients whose lives have been improved by research are welcome. Active participation in establishing this library will help to ensure that the children of to-

day and tomorrow will continue to benefit from research. We fully expect that pediatricians and pediatric researchers will embrace their role as advocates and continue the long and distinguished history of advocacy for child health by the pediatric community.

ARTICLE INFORMATION

Published Online: July 8, 2013.
doi:10.1001/jamapediatrics.2013.2769.

Conflict of Interest Disclosures: None reported.

REFERENCES

1. Stokes J Jr. History of the American Pediatric Society 1887-1965. *Pediatrics*. 1967;39:150.
2. Hughes JG. Conception and creation of the American Academy of Pediatrics. *Pediatrics*. 1993;92(3):469-470.
3. National Institutes of Health. NIH policy and guidelines on the inclusion of children as participants in research involving human subjects. <http://grants.nih.gov/grants/guide/notice-files/not98-024.html>. Accessed February 5, 2013.
4. Shaddy RE, Denne SC; Committee on Drugs and Committee on Pediatric Research. Clinical report: guidelines for the ethical conduct of studies to evaluate drugs in pediatric populations. *Pediatrics*. 2010;125(4):850-860.
5. Hay WW Jr, Gitterman DP, Williams DA, Dover GJ, Sectish TC, Schleiss MR. Child health research funding and policy: imperatives and investments for a healthier world. *Pediatrics*. 2010;125(6):1259-1265.
6. Federation of American Societies for Experimental Biology. NIH research funding trends: FY1995-2013. <http://www.faseb.org/Policy-and-Government-Affairs/Data-Compilations/NIH-Research-Funding-Trends.aspx>. Accessed February 5, 2013.
7. Pollard TD. The obligation for biologists to commit to political advocacy. *Cell*. 2012;151(2):239-243.