Challenges to Excellence in Child Health Research

Call for Papers

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THE UNIQUE NEEDS OF CHILDREN, ALONG WITH THE DISeases that are particular to the pediatric age group, have been responsible for the development of pediatrics as a specialty and for the creation of children's hospitals as distinct from general hospitals for the care of children. These same factors have also driven the creation of the child health research enterprise.

At its best, child health research stands as a model for the advancement of knowledge to improve health and health care. More than 200 hospitals in North America, Europe, Australia, and New Zealand participate in the National Cancer Institute–supported Children's Oncology Group,[1] and more than 90% of children diagnosed with cancer in the United States are treated at one of the participating hospitals. Discoveries from this research network have transformed the 5-year survival rate for all childhood cancers combined from virtually 0% to 80% and have led to far more rapid advancement in treatment of childhood cancer than adult cancer. The National Institute of Child Health and Human Development supports the Neonatal Research Network, a national multicenter collaborative that has provided important advances in neonatal care, with current survival of infants as small as 24 weeks' gestation.[2] Research by the Pediatric Emergency Care Applied Research Network (PECARN) has placed pediatric emergency care on a firmer evidence base.[3]

At least one large prospective life-history study has been funded and data collection begun. The National Children's Study, examining the effects of the environment and genetics on the growth, development, and health of children, is the largest long-term study of child health ever conducted. It will involve more than 100 000 children who will be followed up from conception to age 21 years.[4]

However, child health research has not always met the challenges to excellence. The quantity and quality of child health research are limited in many areas, leaving physicians with no choice but to extrapolate from adult studies, using interventions that may not have been properly evaluated or medications that may be potentially harmful.[5]

Fewer research studies involve children than adults. Two analyses of randomized controlled trials published over 20 years found a greater increase in those conducted among adults than children. In 174 specialty journals, the number of trials involving adults increased by 91 per year compared with only 17 trials per year involving children.[6] In 5 high-impact general medical journals, adult trials increased by 4.71 per year while those in children increased by 0.4 per year.[7]

In addition, child health research is often of lower quality than that involving adults.[8] Most child health research studies are cross-sectional[8] and often single-center studies with small sample sizes. Other problems include reliance on surrogate rather than hard clinical end points and outcome assessment instruments that have not been validated in children. Drug studies may rely on off-label or unlicensed medications as the standard, dosing regimens that are not empirically based, or inappropriate formulations.[9]

A number of unique challenges may affect the child health research enterprise.[9] In the developed world, the majority of children are healthy, limiting the pool of potential participants in research studies and underscoring the need for multicenter research networks. The types of diseases in children, including many behavioral and emotional conditions, may be challenging to study. Research in pediatrics needs to consider a child's age and developmental stage, which may require a larger study population and a longer time frame.[10] Unlike studies in adults, studies in children are complicated by the necessary participation of the parent or caregiver. Children depend on adults to recognize their illness, take them for care, and make decisions about treatment, adding another layer of complexity in studying child health. In addition, parents or caregivers often provide information to researchers, rather than the data being obtained directly from the child-participant. In attention-deficit hyperactivity disorder, for example, ratings by parents and teachers are required.[11] Parents must provide informed consent for their child's participation in a study, which can lower the participation rate.[10] For example, parents may be reluctant to agree to research participation if a potentially painful procedure is involved. In addition, ethi-

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cal considerations loom large when the research participant is a child. The amount of acceptable risk[5] and whether financial incentives should be provided to parents of minors[7] are 2 such issues. Finally, outcomes may be less immediate or harder to measure. For instance, reducing cardiovascular disease in adulthood by preventing obesity in a child is more difficult to measure than the rapid weight loss following gastric bypass surgery. How to translate short-term data from childhood into long-term outcomes over the lifespan is challenging.[10]

Funding and workforce issues also influence the quantity and quality of child health research. A preliminary inventory of federally funded comparative effectiveness research found 728 studies, only 79 of which were conducted in pediatric populations.[11] In terms of drug development, Caldwell and colleagues stated, "Given the smaller pool of patients available for trials in children, the higher fixed and marginal costs are a major disincentive for the pharmaceutical industry to fund trials in children, particularly when the market size at the end of an expensive research and development programme is often small."[5] In addition, the pediatric research workforce "remains small and is not growing substantially."[12]

In an effort to improve child health research, the Standards for Research in (StaR) Child Health was established in 2009 and involves an international group of researchers, methodologists, practitioners, regulators, and journal editors with a mission "to improve the design, conduct, and reporting of pediatric research through the development and dissemination of evidence-based standards."[9] Priority topics have recently been published, and strategies to support the recommendations for research design, conduct, and reporting and to adapt the Consolidated Standards of Reporting Trials statement to include items specific to children are future goals.

JAMA is committed to encouraging excellence in child health research. In demonstration of this commitment, in May 2013, preceding the Pediatric Academic Societies annual meeting, we will publish a third theme issue on child health, following our previous theme issues in 2007 and 2009. We are particularly interested in studies with rigorous designs: randomized controlled trials, multicenter studies, and longitudinal prospective cohort studies. Submission of systematic reviews, particularly meta-analyses, and scholarly Viewpoint articles on child health topics of current interest is also encouraged. Studies that are accepted for presentation at the Pediatric Academic Societies meeting will be highlighted. Articles submitted to *JAMA* but not determined to have sufficiently high priority for *JAMA* publication may be referred to another journal in The JAMA Network. Articles received by December 1, 2012, will have the best chance of consideration for the theme issue. Authors should consult the Instructions for Authors[13] for guidelines on manuscript preparation and submission.

Conflict of Interest Disclosures: All authors have completed and submitted the ICMJE Form for Disclosure of Potential Conflicts of Interest and none were reported.

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