FOR IMMEDIATE RELEASE: Children Underrepresented in Drug Studies

Durham, North Carolina - October 1, 2012 - The number of clinical trials enrolling children is far lower than for adults, and the scope of research is also narrower, according to an analysis of public-access data conducted by researchers at Duke University.

The findings, reported online October 1, 2012, in the journal Pediatrics, quantify an imbalance that has been observed in recent years and highlights an issue that has generated concern among health leaders and policymakers alike. (http://pediatrics.aappublications.org/content/early/2012/09/26/peds.2011-3565)

"Although children comprise one-quarter of the population in the United States, they are greatly underrepresented in the clinical trial process that is designed to lead to new and better therapies, determine appropriate drug dosages and establish standards of practice," said Sara K. Pasquali, M.D., MHS, first author of the study.

The analysis was launched as part of the Clinical Trials Transformation Initiative (CTTI), a public-private partnership founded by the U.S. Food and Drug Administration (FDA) and Duke University to identify practices that will improve the quality and efficiency of clinical trials.

Pasquali, who is now co-director of the Michigan Congenital Heart Outcomes Research and Discovery Program at the University of Michigan, said the study looked at more than 60,000 research trials from 2005 to 2010 using data entered into the ClinicalTrials.gov registry. The on-line registry, mandated by Congress in 1997, was intended to improve patient access to clinical trials and provide greater transparency of trial results and data.

The researchers found that just over 5,000 of those trials were specifically designed to enroll children under the age of 18. Pasquali said the low number of clinical trials enrolling children is likely the result of several factors, including the rarity and diversity of many pediatric diseases, which makes them difficult to study; lack of pediatric research infrastructure; ethical issues associated with testing on children; and difficulty in establishing which endpoints or outcomes of the investigational therapies should be evaluated.

"Many pediatric diseases are relatively rare, as opposed to something like adult coronary artery disease. As a result, it can take much more time to build a research infrastructure, often involving multiple hospitals, to enroll enough patients in a study," Pasquali said. "But with fewer studies to guide therapeutic decisions, treatments and outcomes for young patients often vary widely from center to center."

The researchers also found that enrollments tended to be small in the studies that were conducted with children, making it difficult to obtain clinically meaningful information that could be generalized across larger populations. Study authors suggested that resources might be better spent on larger trials aimed at answering the most pressing questions, rather than on numerous small trials.

The most common areas of study for the pediatric trials included infectious diseases/vaccine studies (23 percent) and psychiatric/mental health studies (13 percent).

"For the vast majority of therapies used on children every day in United States and around the world, clinicians lack basic data to support decisions about the correct dosage, the best type of medication to use, and the appropriate situations to provide treatment," Pasquali said. "Without that information, it really puts physicians and the children we're treating at a significant disadvantage."

Jennifer Li, M.D., MHS, senior author of the study and a member of the Duke Clinical Research Institute, said the analysis provides clarity on an issue that researchers, funders and policymakers have been working to resolve in recent years. Several initiatives have already been passed to help ease the problem, including the FDA Modernization Act in 1997, the Best Pharmaceuticals for Children Act in 2002, the Pediatric Research Equity Act in 2003 and the FDA Amendments Act in 2007.

"Conducting clinical trials with children is a complex issue – they are not volunteers, they are dissimilar in terms of size and disease condition, and the number of patients is a lot less than what we would find among adults," Li said. "This analysis provides one snapshot in time, and it's good to know what the research landscape is so that we can address where we should focus our efforts."

In addition to Pasquali and Li, study authors include Wendy K. Lam, PhD, of the Duke Translational Medicine Institute; and Karen Chiswell, PhD, of the Duke Clinical Research Institute (DCRI); and Alex R. Kemper, M.D., MPH, MS of Duke's Department of Pediatrics and the DCRI.

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