FACT SHEET: Data on the Inclusion of Children in NIH-Sponsored Clinical Studies

Issue Summary. While the National Institutes of Health (NIH) formally encourages the inclusion of children as research participants in the clinical research it supports, the NIH does not collect sufficient data to determine if children are actually being appropriately included in clinical studies. The NIH systematically collects study enrollment data by race and gender, but not age.

Background. Beginning in 1977, the American Academy of Pediatrics (AAP) began raising concerns about the inequities between adults and children in clinical research. The AAP argued that children were often needlessly excluded from NIH-sponsored clinical research, as well as industry-sponsored drug trials regulated by the Food and Drug Administration (FDA). In the 1990s, the AAP began working extensively with the Administration and with Congress to change NIH and FDA policy. This led to the creation of the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA), two enormously successful laws that incentivize and require the pharmaceutical industry to include children in FDA-regulated drug approval research programs.

In 1993, Congress had required the inclusion of women and minorities in NIH research (Pub. L. 103-43, 42 U.S.C. 289a-2), but no such provision existed for children. After advocacy by the AAP and directives from the House and Senate in FY1996 appropriations reports, the NIH published a formal policy requiring the inclusion of children in research: “It is the policy of NIH that children (i.e., individuals under the age of 21) must be included in all human subjects research, conducted or supported by the NIH, unless there are scientific and ethical reasons not to include them.”

Data Collection. Despite this policy, the only systemic data that the NIH collects on the inclusion of children in studies comes at the application stage, before a research grant is awarded and a study is even conducted. Therefore, this data shows only the intent to include children and not whether they actually are included. Furthermore, this pre-award classification of child inclusion only specifies whether or not any individuals under age 21 are intended to be included, and gives no information on the numbers of children or the age groups into which they fall—meaning that a study that intends to include only some 19- and 20-year-old participants would be categorized as including children. Further, while the NIH requires reporting and collects data on numbers of women and minorities actually enrolled in research, there is no such reporting and data collection on children.

Pre-Award Data. The NIH child inclusion policy requires investigators to detail their intent to include children (or the reasons not to) in their research plans when applying for funding. Scientific review groups at the NIH are required to judge the pediatric plan as “acceptable” or “unacceptable” when judging grant applications. While defining children as those under the age of 21 is consistent with the AAP’s definition of pediatrics, it allows a research plan being evaluated by a reviewer to be considered as including both “children and adults” so long as there would be only some representation under age 21 and even if there was no serious intent to include any minors (under the age of 18). This binary approach to defining pediatric inclusion fails to provide meaningful pre-award data on the extent of planned pediatric inclusion.

Post-Award Data. Researchers are required to fill out an annual “Inclusion Enrollment Report” on the numbers of study participants enrolled in studies, broken down by race, ethnicity, and sex/gender. This enrollment report does not require any information on the ages of the participants or ask whether any children have actually been enrolled. Therefore, a study classified as including both “children and
adults” in the pre-award context and which opens enrollment to any individuals 18 or older, could never actually enroll any individuals under the age of 21 and still be classified as including children.

RCDC. The NIH does categorize its research portfolio into currently 235 research/disease areas through a text data mining process called the Research, Condition, and Disease Categorization (RCDC) system. One of these areas is indeed “pediatric” and as such RCDC estimates the total NIH spending on “pediatric” research per fiscal year. These estimates, however, are imprecise are not designed to be able to answer questions about clinical study enrollment. RCDC is especially ill-equipped to evaluate whether otherwise adult-focused research appropriately includes children if relevant to the pediatric population.

Why Change is Needed. The inclusion of children in human subjects research is essential to ensure that children benefit from important scientific advances. Further, the study of children’s health at successive stages of development is important for understanding how diseases develop and persist later into adulthood, and can serve as a basis for developing early treatments for chronic, lifelong illnesses. If no further action is taken to revise NIH data collection practices, a lack of data concerning different age groups—such as neonates, infants, and children—will persist. In the absence of better data, the potential overrepresentation of older adolescents (i.e. ages 18-21) and underrepresentation of younger children could continue without opportunity for public comment or oversight.

Policy Change Requested. The NIH should collect data and report on the actual numbers of children enrolled in its clinical studies. This data should be broken down by pediatric subgroup (e.g. neonates, infants, children, adolescents, etc.). In addition, the pre-award evaluation of research proposals should include a more detailed classification of the pediatric subgroups that are intended to be included in studies.