



# AMERICAN ACADEMY OF PEDIATRICS AND THE PUBLIC POLICY COUNCIL

## An Overview/Outline Of Pediatric Research Issues and Interests Important To The Pediatric Community In The Obama - Biden Administration

### 1. Importance of including children in clinical research

- Effectiveness/return on investment
  - There is significant scientific importance in performing research along the life cycle. Many diseases usually associated with adulthood begin in childhood.
  - Studying children and antecedents of adult disease has great implications for cost effective diagnosis, prevention and therapy
- Equity
  - Children as a group are underrepresented in clinical research. This has had the consequence of inadequate and sometimes dangerous therapy, and in a slower rate of medical advances. The pediatric community has played a leadership role in the development of and strong support for the Best Pharmaceutical for Children's Act, the Pediatric Research Equity Act and the Pediatric Medical Device Safety and Improvement Act, which have increased studies in children. However, the opportunities for children to participate in clinical research remain limited.

### 2. Current policy for inclusion of children in clinical research

- It is of vital importance that children be permitted to serve as participants in clinical research so that they may gain from both the personal benefits of participation (such as that afforded by access to new therapeutic agents only available through clinical trials, or through access to clinical trials that are associated with heightened clinical monitoring that leads to improved clinical outcome) as well as the benefits that accrue to all children as a group (i.e., so that new therapeutics and diagnostics can be developed and evaluated that will benefit children).
- We commend the NIH for its 1998 guidelines to promote the participation of children as subjects in clinical research.
- These guidelines are an excellent initial step. The guidelines reflect an important partnership and the commitment of the research community to work with the NIH in the development of proposals that will increase clinical research participation for children without mandating it.
  - However, these guidelines have not been entirely effective.
    - Not statutory (unlike inclusion of women)
    - 18-21 year old loophole (children are defined as <21 years of age)
    - Cursory study section review/no tracking
    - Unique requirements to perform research in children



- Including women in research can largely be accomplished by the same investigators, research infrastructure, resources, and with minor changes to the protocol.
- Including children requires different investigators with special expertise, additional research infrastructure, extra resources, and substantial alterations to protocol design.

### 3. Enhanced commitment by NIH to include children in research

- Initial review and tracking
  - Serious consideration by study section
  - Tracking of children in reporting
  - Consider redefinition of children (<18 yrs)
- Research infrastructure
  - Clinical and Translational Science Awards (CTSA)
    - Maintain and enhance capacity for children
  - National Children's Study
    - Rigorous science
    - Sustained and continued appropriations for the NCS at the NIH in collaboration with the CDC and the EPA.
  - Pediatric clinical research networks
    - Promote interaction and collaboration
- Resources
  - Additional funding to include children across all institutes and offices of the NIH
  - Pediatric Research Initiative (PRI) - authorized in the Children's Health Act of 2000 - designed to make pediatric research a priority at the highest level of NIH. Provides the NIH director with additional *new* funds to stimulate new pediatric research throughout NIH including fostering collaboration among institutes and with private research organizations seeking partners, as well as to focus more resources in targeted areas of research. Intent is to preserve scientific judgment on the best opportunities to support research that will benefit children now and adults in the future.
  - Health Services Research in Children – Additional funding for quality of care and health care delivery research supported by the Agency for Healthcare Research and Quality (AHRQ).
  - Explore the development of new regional consortia of pediatric centers for research
- Maintaining and expanding pediatric investigator expertise
  - Pediatric loan repayment program
  - Institutional training grants in Pediatrics (T32)
  - Career development and mentoring awards in Pediatrics

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