

QUESTIONABLE CAPACITY IN US PEDIATRIC DRUG TRIALS: EXPERT VIEWS

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Background: Recently renewed Best Pharmaceuticals for Children and Pediatric Research Equity Acts (BPCA/PREA) have increased industry incentives and opportunities for pediatric drug trials (PDTs). Knowledge of the current capacity for PDTs is, however, severely limited.

Objective: To deepen understanding of the capacity for US PDTs.

Design/Method: Experts in pediatric clinical research participated in semi-structured interviews on US pediatric research capacity (Feb-July 2007). An informant list was developed to maximize cognitive diversity and refined to explore emerging themes. A physician/researcher (PR) led each phone interview; health researchers (HRs) took notes and recorded calls. HRs produced detailed summaries, which were verified by the PR and informants. Qualitative analysis, using grounded theory, consisted of two main activities: post-interview debriefing, and coding/theme compilation using Atlas.ti to organize data. Two HRs independently coded each summary for themes. Coding variations were resolved by PR/HR discussion. Consensus was achieved on themes.

Results: The 33 informants' primary or secondary areas of expertise included: academician (n=21); federal official (5); industry medical officer (8); pediatric research network leader (10); pediatric specialist leader (8); pharmacologist (5); practitioner/research site director (9).

While most experts noted an increase in PDTs since BPCA/PREA, a dominant theme of insufficient US PDT capacity emerged. Subthemes included: 1) lack of systems for finding, incentivizing, maintaining trial sites; 2) complexity/demands of conducting PDTs in clinical settings; 3) inadequate numbers of qualified pediatric pharmacologists and clinician investigators trained in FDA Good Clinical Practice; 4) poorly designed PDT protocols that discourage pediatricians and parents from participating.

Potential solutions for insufficient capacity included: 1) consensus-building among stakeholders to create PDT systems; 2) initiatives to train more pediatric pharmacologists and educate clinicians in Good Clinical Practice; 3) advocacy for high quality PDT protocols designed by scientists sensitive to pediatric issues; 4) pediatric and public education on the importance of PDTs.

Conclusion: Insufficient US PDT capacity hinders development of new drugs for children and studies on the safety and efficacy of existing pediatric drugs. Further public policy initiatives are needed to achieve the full promise of BPCA/PREA.