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21st Century Clinical Trials: Rethinking Best Practices Challenges and Opportunities

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PEDIATRIC ACADEMIC SOCIETIES 2019 ANNUAL MEETING

PANEL DISCUSSION

Moderator

William E. Truog, MD
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Professor of Pediatrics
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Disclosure

I have no conflicts of interest to disclose.

Challenges for Traditional Trials

- **Non-reproducibility of larger studies not confirming results from smaller studies (in pediatrics, mostly small studies)**
- **Changing baseline/loss of equipoise**
- **Take too long**
- **Difficult endpoints (composite of non-equivalent outcomes)**
- **Results reflect the aggregate characteristics of the population enrolled and cannot distinguish distinctive features of individual patients**

- **These and other challenges result in wariness about incorporating results of RCT's into everyday clinical practice.**

Today's Panelists

William E. Truog, MD	**Introduction and Overview**
Claudia Pedroza, PhD McGovern Medical School at the University of Texas Health Science Center	Clinicians and Statisticians: Using Bayesian Methods So We Answer the Same Question
Jon Tyson, MD, MS McGovern Medical School at the University of Texas Health Science Center	When Trial Results Aren't Definitive: Do Bayesian Analyses Help?
Lisa Askie, PhD National Health and Medical Research Council Clinical Trials Centre, University of Sydney, Australia	The Challenge of Meta Analyses Created from Summary Data and From Individual Patient Data: Importance of Cooperation Among Investigators
Lynne Yao, MD Director, Division of Pediatric and Maternal Health; Center for Drug Evaluation and Research at the United States Food and Drug Administration	The FDA Pediatric Trials and Innovation to Speed Outcomes to the Bedside
Audience	Questions/Answers/Discussion

Some of the Panel Discussion Topics

- **Study design using frequentist analysis versus the potential advantages and challenges of Bayesian based design.**
- **Interpretation of study results by point analysis; need to move towards using and interpreting confidence intervals**
- **The challenges and limitations of large pragmatic trials versus trials utilizing endotyping to allow smaller, quicker to perform, and less expensive studies.**
- **External acceptance of the results and alteration of clinical practice or the confirmation of current clinical practice.**

Some Outcomes of This Panel Discussion

- **Improve understanding of different methods of analysis.**
- **Improve understanding of innovative study designs.**

Clinical trials are an essential path to progress; they are the brightest torches we have to light our way to better outcomes.*

****slightly adapted from the cancer advertising campaign***