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

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

PEDIATRIC ACADEMIC SOCIETIES 2019 ANNUAL MEETING

PANEL DISCUSSION
Moderator

William E. Truog, MD
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Children’s Mercy-Kansas City
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

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