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

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Children’s Mercy-Kansas City
Professor of Pediatrics
University of Missouri-Kansas City School of Medicine

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