Duke | MARGOLIS CENTER for Health Policy

Measuring Clinical Benefit in Neonatal Randomized Clinical Trials: Challenges and Opportunities

March 23, 2023 • 9:00 AM – 4:30 PM

Hybrid Public Workshop: National Press Club, Washington D.C. and Virtual

Meeting Agenda

Objective: This meeting aims to promote discussion and collaboration between researchers, clinicians, industry, and regulators on efficacy endpoint considerations for neonatal randomized clinical trials, and to provide a forum for patients and families to share what clinical benefits they find important.

09:00 a.m. **Welcome and Introduction**
Duke-Margolis Center for Health Policy

09:10 a.m. **Opening Remarks**
FDA

09:30 a.m. **Session 1: Current Approaches to Measuring Efficacy in Neonatal Randomized Control Trials**

This session will provide an overview of currently utilized approaches to measuring clinical benefit in neonatal randomized controlled trials (RCTs), highlighting differences between efficacy measurement to support regulatory approval versus clinical practice change. Participants will discuss strategies and considerations related to endpoint selection and clinical outcome measurement in neonatal RCTs.

Presentations

Q and A

10:30 a.m. **Session 2: Challenges in Measuring Efficacy for Neonatal Conditions with Unmet Clinical Needs**

This session will focus on the challenges and considerations for developing core outcome sets for neonatal research and choosing appropriate primary endpoints for regulated trials. During this session, participants will review potential efficacy endpoints related to key neonatal conditions, such as bronchopulmonary dysplasia (BPD), neonatal seizures, neonatal opioid withdrawal syndrome (NOWS), and pain. Participants will discuss best practices and key solutions for generating high-quality evidence for these conditions with high unmet clinical needs.

Presentations

Open Discussion

12:00 p.m. **Break for Lunch**

1:00 p.m. **Session 3: Key Considerations for Endpoint Selection for Neonatal Conditions**

In this session, participants will discuss endpoint types and key aspects of selection for neonatal conditions, including the timing of outcome measurement and the interpretability, reliability, and validity of measured endpoints. In addition, participants will consider how feasibility with respect to timing, costs, and other burdens may impact endpoint selection. Participants in this session will consider the clinical importance of endpoints to various stakeholders, including patients and families.

Panelist Remarks

Open Discussion

2:30 p.m. **Session 4: Novel Approaches to Measure Clinical Benefit in Neonatal Clinical Trials**

This forward-looking session will focus on new approaches to measuring clinical benefit in neonatal RCTs, such as defining a global rank score (GRS), EHR/technology-based clinical outcome assessment tools, and data-driven surrogate or intermediate endpoints. In addition, participants will focus on considerations related to balancing efficacy with potential or known safety concerns and challenges with using new approaches to neonatal trial conduct.

Presentations

Panel Discussion

4:00 p.m. **Fireside Chat**

Session moderators will summarize the key takeaways from their sessions and discuss next steps and how to improve clinical benefit measurement in neonatal randomized clinical trials in the future.

4:25 p.m. **Closing Remarks and Meeting Adjournment**
Duke-Margolis Center for Health Policy

4:30 p.m. **Adjourn**

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