M-CERSI Pediatric Workshop
Pediatric Formulation Development: Challenges of Today and Strategies for Tomorrow

Breakout Session 2:

Acceptability of Pediatric Formulations

Framing Statements

- Assuring the acceptability of a drug product for use in the target pediatric population continues to be an area of concern for sponsors, regulators and patients. The goal of this breakout discussion is to collaborate on identifying approaches to advance the understanding of what constitutes an acceptable product and how to demonstrate that acceptability.
- In order to help organize and facilitate discussion, the following topics and seed questions are being provided to participants for their consideration prior to the workshop
- The topics here are not intended to constrain the discussion but rather to provide some insight and opportunity for participants to consider these or similar questions related to the topic of Acceptability. Participants are encouraged to share any comments, perspectives or potential solutions in their respective breakout discussions.
- The discussions will be led by a designated facilitator. Key discussion points will be captured for synthesis and readout post workshop and shared with registered participants. These notes may also be used by the co-chairs in creating a post workshop communication strategy.

Request to Participants

- Please come prepared to share your ideas and opinions on the acceptability assessment for drug products in pediatric patients. In addition to our discussions, we plan to provide a mechanism for you to contribute your written thoughts during the workshop as well.
- Please refer to the publications below. They are provided to form a baseline level of understanding and are not meant to be comprehensive on the topic.
- Ruiz F. Standardized method to assess medicines' acceptability: focus on paediatric population
  - https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5396311/
- Kozarewicz P. Regulatory perspectives on acceptability testing of dosage forms in children. Int J Pharm2014; 2: 245–248.
  - https://www.sciencedirect.com/science/article/pii/S0378517314002129?via%3Dihub

Acceptability Breakout Discussion Topics

- **Methodology for Assessing Acceptability**
  - **Standardization of Methodology**
    - **Is standardization the goal?**
    - **Is this achievable? If so, How?**
    - **Advantages and Disadvantages of Standardization**
  - **Metadata to Collect**
    - **Is there valuable information to collect beyond the "response variable"?**
    - **What is this information?**
  - **Criteria for Demonstrating Acceptability**
    - **Can this be established a priori?**
      - **For some attributes or all attributes?**
      - **Is risk based criteria a better approach?**
    - **Acceptance versus Preference**

Acceptability Breakout Discussion Topics

- **Data and Information Sharing**
  - **Ability and Willingness to Share Data**
    - **By Industry**
    - **By Regulators**
    - **By Academia**
  - **Potential Platforms for Data Sharing**
    - **Databases**
      - **Consortia sponsored**
      - **Regulatory sponsored**
    - **Publication**
  - **Opportunities for Using Shared Data**
    - **What could be done with shared data sets**
      - **Predictive tools or relationships?**
      - **In-silico determination of acceptability?**

Acceptability Breakout Discussion Topics

- **Timing and Risk Associated with Assessing Acceptability**
  - **Which attributes can be assessed outside the clinic only?**
  - **Are there situations or attributes that can only be verified as acceptable in patients in the clinic?**
    - **If so, what are those and how do we de-risk this for sponsors?**
    - **What happens if a sponsor fails to hit that criteria?**
  - **Are their attributes that should have both pre-clinical/development data and then supported by clinical data?**
    - **Fear of conflicting data –how to reconcile**
  - **Regulators as active partners, Sponsors typically have 1 opportunity in the clinic.**
  - **Will the fear of not passing acceptability prohibit innovation in the space?**
    - **Has a regulatory body NOT approved a product due to the formulation?**