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

Breakout Session 1:

Excipients in Pediatric Formulations

Framing Statements

- Assuring the safety of excipients in drug product formulations for use in pediatric populations continues to be an area of concern for sponsors, regulators and patients. One of the goals of this breakout discussion is to collaborate on identifying approaches to advance the development of novel excipients and to identify gaps and challenges in the current paradigms used to assess excipient safety. Another goal is to identify opportunities to leverage existing data or to establish novel frameworks for excipient development.
- 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 Excipients. 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 assessment of excipients 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.

Excipients
Breakout Discussion Topics

"Novel" Excipients

- Excipients with no prior human experience (adult or pediatric)
  - Areas with a critical need for development of novel excipients in pediatric formulations?
  - Is there resistance in the pharmaceutical industry to developing novel excipients?
    - If yes, is this due to real or perceived regulatory hurdles to development?
    - What are the real or perceived roadblocks to development of novel excipients?
- Excipients with prior human experience
  - Under what circumstances is adult clinical experience enough to qualify an excipient for pediatric use?
  - Under what circumstances is adult clinical experience unlikely to be enough to qualify an excipient for pediatric use?
- Opportunities to qualify excipients during the drug development program?
  - What are the limitations/hurdles to developing a pediatric clinical formulation with a novel excipient during adult clinical development?
  - Is it feasible to build an arm into juvenile animal studies when these are required for development of the active pharmaceutical ingredient?
  - When would a step down approach where safety is assessed in the clinic while moving progressively lower in pediatric age be reasonable?

Excipients
Breakout Discussion Topics

Excipients with Pediatric Liabilities

- Are there opportunities to identify these excipients a priori?
  - Common features to classes already identified as having a liability?
- Are there opportunities to reduce/eliminate excipients of concern?

- Can current use in the clinical setting be utilized to establish safety or thresholds of toxicological concern?
  - Is there a way to leverage clinical samples taken for other purposes?
    - Blood spots, blood draws, etc.

Excipients
Breakout Discussion Topics

Opportunities to Improve Excipient Development for Pediatric Formulations

- Are there ways to better utilize current databases
  - STEP database
  - FDA IID
- Are there opportunities to better refine/expand current databases?
  - Expanding the FDA IID to include pediatric use?
- Are there opportunities to promote data sharing in industry/academia?
  - What role might regulatory agencies play in the promotion of data sharing?
- Independent avenues for excipient development
  - Opportunities to uncouple excipient development from drug product registration?