



Small Group Breakout Session

Discussion Questions



Speakers

When you speak or make a comment:

- **Give your name**
- **Affiliation**
- **Disclosures**



Meeting Ground rules:

- **Respect the speaker and the listener**
- **One person speaks at a time**
- **Equal participation**
- **Timeliness (come back on time)**
- **Headlines (just the facts)**
- **E-manners (turn all electronics off)**
- **Stay on Track**
- **Honest Disclosure**
- **Scope: Exchange of information**



Discussion Questions

1. **What are the 5 most important unmet research needs in each specific disease/anatomic category? (e.g. cardiovascular, orthopedic)**
 - **Although there are obvious barriers to clinical trials including liability and concerns about the effects on child development, there are significant needs in both rare and common diseases or disorders that have not been met with modifications of adult devices. To start, FDA would like to focus on the most significant needs in each device area. This may mean starting with less common health concerns that impact pediatric care in a more global arena. We should start by asking simple questions such as:**
 - **What are the most important unmet device needs in each category?**
 - **What are the scientific or clinical barriers or potential barriers to developing devices to meet those needs?**



Discussion Questions

2. What are some clinical trial designs that encourage enrollment of pediatric patients while providing adequate high quality data to support safety and effectiveness of devices? It may be that the current thinking behind clinical trials involving adults needs to be drastically changed to even contemplate designing clinical trials involving pediatric patients. We need to understand:
 - What are appropriate controls to use in pediatric trials to satisfy the legal regulatory definitions of valid scientific evidence as described in 21CFR 860.7?
 - How can follow up be maximized?
 - What time frames are needed given the age of patients and the expected lifetime of the device/disease being treated?
 - How do we understand the long term effect on development and growth in a short clinical trial?



Discussion Questions

3. Preclinical and Animal Studies

- Although there are examples of immature and fetal animal studies that are well established for pharmaceuticals, how do we translate those concepts for devices?
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- What types of endpoints and timeframes translate into outcomes in the human population? How do we know that?
- How do we set a standard to judge subsequent trial outcomes as acceptable and safe?
- What animal models exist or are appropriate for studying each of the diseases or disorders we identify as significant unmet needs?



Discussion Questions

4. How do we measure safety and effectiveness in a pediatric population?

The pediatric population cannot always describe symptoms or functional problems in the same way that adults can. It therefore follows that the same assessment tools and surrogate endpoints will not apply to a pediatric population.

Therefore we are striving to understand:

- - What validated assessments are needed or exist for the pediatric population being treated?
 - What surrogate markers or endpoints are needed for each disease?
 - What surrogates are needed or are available to determine long-term outcomes?
 - How do we validate surrogate endpoints?



Discussion Questions

5. How do we know that the study and the treatment are successful?
 - Assessment and judgment of patient outcomes varies considerably for a pediatric population. The needs of the patient and his caregiver or parent must be considered. The longevity of and durability of devices captures a new meaning when the lifespan is 50-60 years; remaining lifespan in adults is very different. We are soliciting feedback on:
 - **What constitutes successful or unsuccessful treatment outcomes?**
 - **What criteria should be used to determine successful or unsuccessful treatment outcomes?**
 - **What human factors in each case need to be considered?**
 - **What patient factors unique to the pediatric population have to be considered?**
 - **What criteria are required to acknowledge that successful treatment for a patient has been achieved?**
 - **What constitutes a successful clinical trial?**
 - **How long should a device or treatment last to be considered effective?**