



WELCOME !!

Pediatric Clinical Trials Workshop: Unmet Needs, Trial Designs and Clinically Meaningful Safety and Effectiveness Outcomes

**October 29-30, 2009
College Park, MD**



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)
 - 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?
 - 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?
- 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?
 - 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?
 - 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?