

# *Pediatric Study Decision Tree*

**Reasonable to assume (pediatrics vs adults)**

- ✓ similar disease progression?
- ✓ similar response to intervention?

**NO**

**YES TO BOTH**

- Conduct PK studies
- Conduct safety/efficacy trials\*

**NO**

**NO**

**Is there a PD measurement\*\*  
that can be used to predict  
efficacy?**

**YES**

- Conduct PK/PD studies to get C-R for PD measurement
- Conduct PK studies to achieve target concentrations based on C-R

**Reasonable to assume similar  
concentration-response (C-R)  
in pediatrics and adults?**

**YES**

- Conduct PK studies to achieve levels similar to adults
- Conduct safety trials

- Conduct safety trials