Animal Models for Drug, Biologic, Device Discovery for Child Adolescent Disorders

Facilitators: Doug Feltner, Marc Walton

Experts: Greg Holmes, Karen Gale

Summary

- Model tells us whether the drug effects the general biology of an living organism, rather than a signal of efficacy
- Varying opinions on whether animal models are useful as models of "efficacy".
- Models of disease depend on our degree of understanding of human disease which is often, though not always, limited.
  - Models of disease pathophysiology, including disease progression, are thus will often mislead us in looking for therapeutic candidates
  - Models of symptoms may have greater utility as therapeutic efficacy models (MES for seizures as an example)
- Human disorders are often syndromes with multiple underlying pathophysiologies with a common final set of symptomatic manifestations

Summary

- Nonclinical safety models
  - For children/adolescents, rat and other animals have a very accelerated rate of brain development, making it difficult to model human brain developmental toxicities
    - Should non-human primates have a greater role in assessing developmental brain toxicities of drugs?
- Phsyiological role of target may differ in developing vs adult brain and so one must consider carefully how drug is impacting wider physiology with respect to both safety and efficacy.

- Animal model data to support efficacy is a part of a larger dataset (in vitro, human, etc data)
- How much weight to give the animal model data varies, depending on indication, model, drug mechanism and most importantly by individual perspective