

Case studies in precision drug development

- ▣ Creative trial design
 - Sophisticated recruitment: diagnostic tests performed by Dx companies, trial sponsor, clinical sites
 - “Just in time” site establishment with central IRB
- ▣ Collaboration
 - Multiple academic centers and multiple companies
 - How best to centralize data (most likely outside pharma sponsors)
- ▣ Complexity even in Mendelian diseases
 - Patient heterogeneity
 - Locus heterogeneity – multiple targeted therapies? Or identify common intervention points?
- ▣ Advantages of pre-symptomatic treatment – how to go beyond pediatric diseases