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