

Innovative Approaches to Study Planning and Design: Power, Simulation and Adaptation

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Welcome!



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- Introduction (Bryan Luce)
 - Background
 - PCORI considerations
 - Concept
- Technical presentation (Roger Lewis)
 - Trial Design trial design simulation
 - Adaptive designs



Background

- Pragmatic Clinical Studies (PCS) may themselves be unfamiliar to applicants.
- PCSs are often complex in that they...
 - Study real world applications of interventions.
 - Address “what works for whom under what circumstances?” which may mean....
 - Multiple choices (thus, multiple arms may be appropriate)
 - Practice patterns and new innovations may change during the trial
 - May be complex and difficult to calculate power reliably
- Applicants may be unfamiliar with certain innovative trial design features, e.g. trial design simulation; Adaptive techniques. However...
- These (or other) innovative designs may be appropriate to consider



Thus, PCORI is Considering to...

- Encourage applicants to propose innovative design approaches (as appropriate)
- Recruit a cadre of experts in specialized trial design issues (e.g. design simulation, adaptive designs)
- Evaluate high scoring applications (prior to funding) for possible trial design consultation
- For selected applications, offer (at no cost to applicant) expert design consultation and possibly additional funding and time with aim to improve:
 - Power analysis
 - Trial flexibility (e.g. multiple arms, adaptation)
 - Trial efficiency
 - Scientific rigor



Questions?

- We will now have an in-depth discussion, during which you can provide feedback.
- Roger Lewis will then drill down into the science of these trial design issues.



Thank You!

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