What is a Master Protocol and Why Are We Talking About It?

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WHY?
EXPLORING THE POSSIBILITIES
PDUFA VI promises to encourage future efforts by advancing Model-Informed Drug Development (MIDD) and the use of complex innovative and adaptive clinical trial designs. To facilitate the advancement and use of complex adaptive, Bayesian, and other novel clinical trial designs during PDUFA VI, FDA proposes to convene a public workshop on complex innovative trial designs, publish guidance on complex innovative trial designs, and incorporate guidelines on evaluating complex innovative trial designs.

**Master Protocols to Study Multiple Therapies, Multiple Diseases, or Both**

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High-quality evidence is what we use to guide medical practice. The standard approach to generating this evidence — a series of clinical trials, each investigating one or two interventions in a single disease — has become ever more expensive and challenging to execute. As a result, important clinical questions go unanswered. The conduct of "precision medicine" trials to evaluate targeted therapies creates challenges in recruiting patients with rare genetic subtypes of a disease. There is also increasing interest in performing mechanism-based trials in which eligibility is based on criteria other than traditional disease definitions. The common denominator is a need to answer questions that go beyond the single intervention, single disease model.
WHERE ARE WE HEADED?
Thank you!