Novel Clinical Trial Designs & Supporting Innovation: An FDA Center for Drug Evaluation & Research (CDER) Perspective

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Main Points

- FDA has a well-defined, ongoing role in encouraging innovation and improving regulatory science, including use of innovative trials designs:
  - Protecting the patients through sound science before & after the products are on the market
  - Promoting the development of important novel therapies through clear guidance and flexibility
FDA’s Role in Stimulating Future Innovation In Medical Therapy: Strengthening Regulatory Science and Innovation
Comment: Regulatory Science and Regulators

- “(H)ow do we make sure that we fully translate the potential and promise of that research into real-world products and programs that really matter?”

- “A big part of the answer is by strengthening regulatory science. This is a component of the overall scientific enterprise that is really essential, and yet has been underappreciated and underdeveloped.”

  Commissioner Hamburg (AAAS, June 2010)
FDA Role in Supporting Innovation

- Essential Regulator’s roles
  - Provide clarity on the rules in operation and how they’ll be interpreted
    - Guidances, Rules
  - Ensure level playing field
    - Process transparency, equity, timeliness
    - Peter Barton Hutt: ‘Consistent and dependable rules that are equally applicable to everyone’
  - Thoughtful and informed regulation that does not stifle innovation
    - Standard-setting with outside partners
FDA’s Additional Role in Supporting Innovation

- Supporting appropriate collaboration among government, academia, industry and patient groups
- Building opportunities to share existing knowledge & databases
- Developing enabling standards
  - Infrastructure and “toolkit” development, not product development
- Building support for academic science bases in relevant disciplines
Targeted FDA Activities
Supporting Innovation

Improving Clinical Trials Through Guidance
CDER Guidance to Improve Clinical Trials Conduct

- Draft Guidances on use of trial enrichment designs and on the use of meta-analytic analyses (imminent)
General Comments About Adaptive Design Trials in CDER

- Discussed with sponsors frequently
- Used less frequently......reasons are complex

- We use some adaptive approaches reasonably frequently: trials run based on # of events not # of patients, adapting trials based on safety data
- Guidance is out for comment....please!
Other CDER Activities to Speed Innovation

Consortia, Collaborations, and Partnerships
Improving Clinical Trials: CTTI

- FDA and Duke University - founding members of a public-private partnership focused on improving clinical trials
- > 60 members from government, industry, academia, patient & consumer representatives, clinical investigators, professional societies, & research organizations
CTTI and Clinical Trials Monitoring

- Need to move away from one-size fits-all approach
  - Expensive, time-consuming, not always needed
- Build quality into the scientific and operational design and conduct of clinical trials
  - Focus on what matters, avoid duplication/waste
  - Improve training and procedures
  - Similar to Quality by Design principles used in manufacturing of complex drugs and drug delivery systems (e.g., transdermal patches)
- Ongoing pilot project with Pfizer and FDA for a phase III trial
Summary: Supporting Innovation

- FDA takes the efficient development and appropriate use of medical treatments seriously.
- FDA role is more than simply reviewing applications: Guidance and Collaboration.
  - Willingness to question previous assumptions about product development.
  - Be open to change when assumptions no longer hold or there are more efficient answers.