Agenda: Expert Meeting on Large Simple Trials (LSTs)

Clinical Trials Transformation Initiative

Purpose: “To develop recommendations to facilitate and promote the adoption of LST designs for regulatory submissions or other purposes.”

May 13 and 14, 2013

Hilton Washington DC/Rockville Hotel and Executive Center
1750 Rockville Pike
Rockville, MD, 20852

Plaza I Ballroom

Key Objectives
- Discuss findings from a survey of practices
- Discuss strategies that companies are using to implement LSTs
- Discuss the challenges to LSTs

Participants
- Representatives from a broad cross-section of the clinical trial enterprise including regulators, government sponsors of clinical research, academia, industry, patient advocates, clinical investigators, and other interested parties
- Participants are expected to be actively engaged and dialogue both days

Day 1 - Monday, May 13, 2013 (9:00 am to 5:00 pm)

By the end of Day 1, all participants should submit a list of up to 3 barriers for conducting LSTs. In addition, participants are encouraged to submit a list of potential solutions for these barriers. The submitted items will provide the basis for our Day 2 discussions.

Meeting Co-chairs: Patrick Archdeacon (FDA)
Frank Cerasoli (Orexigen)
David Gordon (NIH)
Christopher Granger (Duke University)
Gail Pearson (NIH)

9:00 am WELCOMING REMARKS

9:00 – 9:05 am Welcome
Leanne Madre, Clinical Trials Transformation Initiative

9:05 – 9:10 am Opening remarks
Christopher Granger, Duke University
9:10 am  
**SESSION I: Landscape of LST trials**

*Session Facilitator: David Gordon*

9:10 – 9:25 am  
Perspectives on LST Trials  
*Michael Lauer, NHLBI*

9:25 – 9:30 am  
Q&A

9:30 – 9:45 am  
Patient Perspective on LST Trials  
*Carolyn Petersen*

9:45 – 9:50 am  
Q&A

9:50 – 10:05 am  
Opportunities for LSTs - Industry  
*Reshma Kewalramani, Amgen*

10:05 – 10:10 am  
Q&A

10:10 – 10:25 am  
Opportunities for LSTs – Regulatory  
*Sandra Kweder, Deputy Director, Office of New Drugs, CDER/FDA*

10:25 – 10:30 am  
Q&A

10:30 – 10:50 am  
Summary of Survey Results  
*Patrick Archdeacon, Office of Medical Policy, CDER/FDA*

**10:50 – 11:05 am  
Break**

11:05 – 11:30 am

*Session Moderator: Christopher Granger*

Panel Discussion on Survey Results

*Panel: Michael Lauer, Sandra Kweder, Robert Temple,*  
*Reshma Kewalramani, Frank Cerasoli, Colin Baigent, Carolyn Petersen*

**11:30 am  
SESSION II: Models of Approaches to LSTs**

*Session Facilitator: Gail Pearson*

11:30 – 11:50 am  
Models of Industry Trials for Regulatory Purpose (Efficacy/Effectiveness)  
*Christopher Granger, Duke University*

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11:50 – 11:55 am  Q&A
11:55 – 12:15 pm  Models of Industry Trial for Regulatory Purposes (Safety)
  Frank Cerasoli, Orexigen
12:15 – 12:20 pm  Q&A
12:20 – 1:10 pm  Lunch (Provided)  Regency
1:10 – 1:30 pm  Pragmatic Trials using EHR Platforms
  Ryan Ferguson, Veterans Administration
1:30 – 1:35 pm  Q&A
1:35 – 1:55 pm  Population-based trials with high cost-efficiency (VITAL)
  JoAnn Manson, Harvard University
1:55 – 2:00pm  Q&A
2:00 pm  Session III:  Case Studies
  Session Facilitator:  Patrick Archdeacon
  What has worked well and what lessons have been learned?
2:00 – 2:20 pm  CHAMPION Program
  Meredith Todd, Clive Meanwell (The Medicines Company)
2:20 – 2:30 pm  CHAMPION Program – FDA perspective
  Stephen Grant, Office of New Drugs, FDA
2:30 – 2:50 pm  Q&A
2:50 – 3:10 pm  Break
3:10 – 3:30 pm  SHARP
  Colin Baigent (Clinical Trial Service Unit, Oxford)
3:30 – 3:50 pm  JUPITER
  Johannes Hulthe (AstraZeneca)
3:50 – 4:05 pm  SHARP and JUPITER – FDA perspective
  James Smith, Office of New Drugs, FDA
4:05 – 4:25 pm  Q&A

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Please remember to submit your list of 3 challenges/barriers limiting increased adoption of LST designs and strategies to overcome them

Day 2 – May 14 (8:30 am – 12:00 pm)

8:30 – 8:40 am Summary of Day 1, including review of challenges to increased adoption of LST trial designs and potential solutions

*Christopher Granger, Duke University*

8:40 – 9:00 am Q&A

9:00 – 9:15 am Reflections on Challenges and Potential Solutions

*Robert Temple, Deputy Center Director, CDER/FDA*

9:15 – 10:00 am Q&A, Facilitated Discussion

*Moderator: Patrick Archdeacon*

*Panel: Robert Temple, Stephen Grant, James Smith, Michael Lauer, Reshma Kewalramani, Clive Meanwell, Carolyn Petersen*

10:00 – 10:15 am Break

10:15 – 11:30 am Break-out sessions to Identify Challenges and Potential Solutions for Adoption of Large Simple Trial designs

11:30 – 12:00 pm Break-out Group Report Out of top 3 Challenges and Solutions

12:00 – 12:30 pm Q&A

12:30 – 12:45 pm Closing remarks: (Boxed lunch available)

*Presenter: Christopher Granger, Patrick Archdeacon*