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

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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 – 2:00 pm 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
Please remember to submit your list of 3 challenges/barriers limiting increased adoption of LST designs and strategies to overcome them

6:00 – 8:00 pm Reception

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*