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

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 – 1:10pm	**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

4:25 – 4:30 pm	Closing
4:30 pm	Adjourn

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*