

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