



# CLINICAL TRIALS TRANSFORMATION INITIATIVE

## Patient Group and Clinical Trials Expert Meeting

Agenda of the Meeting/Workshop held January 21-22, 2015

Fairmont Hotel  
2401 M Street Northwest, Washington, DC 20037

**CTTI MISSION:** To identify and promote practices that will increase the quality and efficiency of clinical trials

### MEETING OBJECTIVES:

- ▶ Define consensus principles of Patient Group engagement from the 3-way (Industry, Patient Group, and Academia) stakeholder perspectives using case studies of success and barriers.
- ▶ Share evidence and key findings from a 3-way stakeholder assessment of Patient Group engagement in the Clinical Trial process
- ▶ Gain understanding of the FDA Rules of Engagement and various Conflict of Interest (COI) issues
- ▶ Solicit feedback on evidence to inform recommendations that can further enhance the value to industry, academia and patient groups of mutually beneficial partnerships that engage the patient groups as fully as possible in the complete spectrum of the clinical research enterprise
- ▶ Solicit feedback on benchmarking, metrics, and value of patient engagement in clinical trials for research sponsors

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For more information, contact the CTTI Patient Groups & Clinical Trials project manager, Bray Patrick-Lake, at [bray.patrick-lake@duke.edu](mailto:bray.patrick-lake@duke.edu), or visit <http://www.ctti-clinicaltrials.org>.

## DAY 1: Wednesday, January 21, 2015

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**8:00 am**      **BREAKFAST AVAILABLE**

**8:30 am**      **Welcoming Remarks and Overview**

*Dr. Pamela Tenaerts (CTTI)*

*Bray Patrick-Lake (CTTI)*

### **SESSION I: Background/Landscape**

*Moderator: Ron Bartek (Friedreich's Ataxia Research Alliance)*

Objectives:

- ▶ Learn about the FDA's perspective on the roles of patient advocacy in the approval process
- ▶ Review key findings from the CTTI project on Best Practices for Effective Engagement with Patient Groups around Clinical Trials
- ▶ Review and discuss an example of best practices of patient engagement in clinical trial design and operations

**8:45 am**      **Roadmap to Advocacy – FDA Perspective**

*Presenter: Dr. Janet Woodcock (Food and Drug Administration, CDER)*

**9:30 am**      **Success from Start to Finish: The COMFORT Trial**

*Presenter: William Tunno (Boehringer-Ingelheim, formerly InCyte)*

**10:00 am**      **Key Findings from CTTI's Project on Best Practices for Effective Engagement with Patient Groups around Clinical Trials**

*Presenter: Wendy Selig (Melanoma Research Alliance, CTTI Project Team Lead)*

**10:30 am**      **Interactive Discussion**

*All attendees*

**10:45 am**      **BREAK**

### **SESSION II: Identifying Partners for Clinical Trials**

*Moderator: Dr. Scott Weir (University of Kansas)*

Objectives:

- ▶ Understand how sponsors identify which patient groups to partner with

- ▶ Understand how patient groups make disease area more attractive to sponsors through unification when multiple groups work in the space
- ▶ Understand how patient groups identify which sponsors to engage with when multiple sponsors are working in the space

**11:00 am Identifying Patient Group Partners – Capability and Asset Assessment to define What is my Currency?**  
*Presenter: Margaret Heim (Bristol-Myers Squibb)*

**11:30 am Building Partnerships for Clinical Trials: A Multistakeholder Model**  
*Presenters: Dr. Scott Weir (University of Kansas)  
 Dr. G. Sitta Sittampalam (NIH, NCATS)  
 Jun Xu (Leukemia & Lymphoma Society)  
 Ron Bartek (Friedreich’s Ataxia Alliance)  
 Joel Beetsch (Celgene)*

**12:15 pm Interactive Discussion**  
*All attendees*

**12:45 pm LUNCH (PROVIDED)**

**SESSION III: RULES OF ENGAGEMENT**  
*Moderator: James Valentine (Hyman, Phelps, & McNamara, P.C.)*

- Objectives:**
- ▶ Understand key regulatory considerations for sponsor-patient group engagement
  - ▶ Become attuned to conflict-of-interest and appearance issues for patient groups when engaging with academia and industry

**1:15 pm Deciphering Regulatory Restrictions on Promotion of Investigational Products**  
*Presenter: Carla Cartwright (Johnson & Johnson)*

**1:30 pm Understanding Clinical Trial Recruitment Rules: Debunking Myths**  
*Presenter: Richard Klein (Food and Drug Administration, OCHA)*

**1:45 pm Conflicts-of-Interest: Real Conflicts and Appearance Issues**  
*Presenter: Ron Bartek (Friedreich’s Ataxia Alliance)*

**2:00 pm** **Navigating the Rules of Engagement – In-house counsel perspective**

*Presenter: Rebecca Prince, Bristol-Myers Squibb*

**2:15 pm** **Q&A and Interactive Discussion**

*All attendees*

**2:30 pm** **BREAK**

**2:45 pm** **SESSION IV: Breakout Groups**

**Breakout 1: Assessing Partnership Potential with Research Sponsors: What Sponsors Bring to the Table**

*Facilitators: Dr. Sharon Hesterlee, Joel Beetsch, and Paulo Moreira*  
Objectives:

- ▶ Identify the top 10 things patient groups should ask research sponsors about partnerships around clinical trials
- ▶ Identify Do's and Don'ts of effective partnerships
- ▶ Discuss strategies for managing partnerships when there are multiple sponsors working in a space

**Breakout 2: Identifying and Leveraging Patient Group Assets and Capabilities: What Patient Groups Bring to the Table**

*Facilitators: Ron Bartek, Jamie Roberts*

Objectives:

- ▶ Identify assets that Patient Groups have and can/should leverage
- ▶ Identify mechanisms for leveraging those assets appropriately

**Breakout 3: Value and Metrics: Patient Group Effects on Clinical Trial Variables**

*Facilitators: Dr. Steve Roberds, Matthew Harker*

Objectives:

- ▶ Understand perception of the “value” of a successful clinical trial from all stakeholders perspectives
- ▶ Document the metrics used to quantify “value” at the conclusion of a clinical trial from both industry and academic perspectives
- ▶ Understand patient group effects on variables in clinical trial continuum that impact the metrics defined above

**3:45 pm**     **Rotate to 2nd Breakout Group**

**4:45 pm**     **Day 1 Wrap Up**

*Richard Klein (Food and Drug Administration, OCHA)*

**6:00 pm**     **RECEPTION**

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**DAY 2: Thursday, JANUARY 22, 2015**

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**7:30 am**     **BREAKFAST AVAILABLE**

**8:15 am**     **Welcoming Remarks**

*David Leventhal (Pfizer)*

**SESSION V: Value and Metrics**

*Moderator: Dr. Eric Eisenstein (Duke)*

Objectives:

- ▶ Learn about cost associated trial delays and models for predicting probability of success
- ▶ Consider methods for evaluating the success and value of partnerships with patient groups around clinical trials in various settings and time points in the development lifecycle.
- ▶ Review and discuss findings from Day1 Breakout sessions

**8:30 am**     **Cost of Trial Delays**

*Presenter: Ken Getz (Tufts Center for the Study of Drug Development)*

**8:55 am**     **What Industry is Measuring Around Patient Group Partnerships**

*Presenter: David Leventhal (Pfizer)*

**9:20 am**     **Impact of Patient Engagement on Project Valuation**

*Presenter: Dr. Bennett Levitan (Johnson & Johnson)*

**9:50 am**     **Breakout Session Findings on Value and Metrics**

*Presenters: Matthew Harker (Duke)*

*Dr. Steve Roberds (Tuberous Sclerosis Alliance)*

**10:10 am**    **Q&A and Interactive Discussion**

- 10:45 am    BREAK**
- 11:00 am    Breakout Session Findings from Assessing Partnership Potential with Research Sponsors: What Sponsors Bring to the Table**  
*Presenters: Joel Beetsch (Celgene)  
Sharon Hesterlee (Parent Project Muscular Dystrophy)  
Paulo Moreira (EMD Serono)*
- 11:20 am    Breakout Session Findings from Identifying and Leveraging Patient Group Assets and Capabilities**  
*Presenters: Ron Bartek (Friedreich's Ataxia Research Alliance)  
Jamie Roberts (formerly NIH, now Duke)*
- 11:40 am    Interactive Discussion**
- 12:00 pm    PICK UP BOXED LUNCH (PROVIDED)**
- 12:15 pm    Progress Through Partnership: Integrating Patient Groups into the NCATS Clinical Research Process**  
*Presenter: Dr. Petra Kaufmann (NIH, NCATS)*
- 1:00 pm    Adjourn**

## Appendix A. Meeting/Workshop Background

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### Background

The patient community has been taking a more active role in the clinical trials enterprise (CTE) and is rapidly evolving in its areas of focus, sophistication about the research process, levels of expertise, reach and desired outcomes when pursuing their research agendas. Thus, patient groups, defined here as patient advocacy organizations, voluntary health agencies, public health organizations and their affiliated members, have been increasingly recognized as equal partners in the research enterprise, especially in the important field of clinical trials.

### Issue

Key sectors of the research community have identified a gap in knowledge and understanding about how and when to best interact with patient groups regarding clinical trials. This knowledge gap has the potential to delay the start of meaningful clinical trials or lead to the conduct of less efficient trials by not tapping into the patient resource. Complex legal, ethical and regulatory issues and ill-defined expectations can lead to unproductive relationships and disparate or unanticipated outcomes. While key stakeholders have declared commitment to create a more effective model for engagement between research sponsors, investigators and patient groups leading to better clinical trials, no guidelines for best practices currently exist. Thus, actionable recommendations are being developed through CTTI's Patient Groups and Clinical Trials project.



## Appendix B. Meeting/Workshop Participants

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### CTTI Project Team Leads

- ▶ Matthew Harker (Duke)
- ▶ Sharon Hesterlee (Parent Project Muscular Dystrophy)
- ▶ Richard Klein (Food and Drug Administration, OCHA)
- ▶ David Leventhal (Pfizer)
- ▶ Jamie Roberts (formerly National Institutes of Health, now Duke)
- ▶ Wendy Selig (Melanoma Research Alliance)
- ▶ Sophia Smith (Duke)

### CTTI Project Team Members

- ▶ Ron Bartek (Friedreich's Ataxia Research Alliance)
- ▶ Joel Beetsch (Celgene)
- ▶ Patricia Cornet (Bristol-Myers Squibb)
- ▶ Paulo Moreira (EMD Serono)
- ▶ Steve Roberds (Tuberous Sclerosis Alliance)
- ▶ Jeff Sherman (Drug Information Association)
- ▶ James Valentine (Hyman, Phelps & McNamara, P.C.)
- ▶ Scott Weir (University of Kansas)

### MEETING/WORKSHOP ATTENDEES

Our workshop participants include 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 in dialogue both days. Please see workshop participant list to be distributed upon onsite registration.

### STAFF

Bray Patrick-Lake (CTTI)  
Rene Hamilton (CTTI)

Marsha Marquess (Duke)  
Lauren Binanay (CTTI)