Disclaimer

The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative.

The presenters are Employees of Duke University. Salary support comes from pooled membership fees of the Clinical Trials Transformation Initiative and from FDA Cooperative agreement.
Clinical trials in crisis
Addressing This Need

To identify and promote practices that will increase the quality and efficiency of clinical trials

Public-Private Partnership involving all stakeholders
60+ members
Collaboration Towards Solutions

Better Streamlined Fit for purpose

Clinical Trials

Government and regulatory agencies

Industry: pharma bio device CRO

IRBs

Clinical investigators

Patients / Patient advocacy groups

Academia

Industry trade / Professional organizations
Better, Streamlined, Fit for Purpose Clinical Trials

- Target problem areas in clinical trials
- Identify solutions
- Formulate recommendations
- Gather evidence
- Build consensus
- Change
## Portfolio of CTTI Projects

<table>
<thead>
<tr>
<th></th>
<th>Investigational plan</th>
<th>Study start-up</th>
<th>Study conduct</th>
<th>Analysis and dissemination</th>
<th>Specialty areas</th>
</tr>
</thead>
</table>
| **Completed projects**        | • Large simple trials
• Uses of electronic data                                                                                | • Central IRB
• Site metrics                                               | • Adverse event reporting
• IND safety
• Monitoring                                           |                                         | • Long-term opioid data                                    |
| **Current projects**          | **Patient groups and clinical trials**                                              | • Central IRB advancement
• GCP training
• Informed consent
• Investigator turnover
• Recruitment and retention | • Safety case studies
• IND safety advancement                                       | • State of clinical trials
• DMCs                                                           | • Streamlining HABP/VABP trials
• Pediatric Antibiotic trials
• Unmet need in Antibiotic development
• HABP/VABP pilot study                                       |
PGCT Project Team Members

**Team Leaders**
- Matthew Harker (Duke)
- Sharon Hesterlee (Formerly Parent Project Muscular Dystrophy, now Myotonic Dystrophy Foundation)
- Richard Klein (FDA)
- David Leventhal (Pfizer)
- Jamie Roberts (formerly NIH, now Duke)
- Wendy Selig (Melanoma Research Alliance)
- Sophia Smith (Duke)

**Project Manager**
- Bray Patrick-Lake (CTTI)

**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 (DIA)
- James Valentine (Hyman, Phelps & McNamara)
- Scott Weir (University of Kansas)
Many of today’s patient groups serve as active partners in the clinical trial enterprise and invest private funding in milestone driven research with focus on leveraging their assets to de-risk research and increase return on investment.
Patient Group Engagement Across the Clinical Trial Continuum

Building a model to evaluate impact

**Pre-Discovery**
- Interest of research question to patient community
- Provide data on unmet need and therapeutic burden
- Direct funding and fund raising for research or product development
- Understanding mechanisms of action relevant to disease and symptom burden

**Pre-Clinical**
- Network recruitment / outreach
- Direct funding and fund raising for research or product development
- Infrastructure support
- Provide input on study design (barriers to participation)
- Support trial awareness and recruitment
- Peer advocate during informed consent procedure

**Phase 1**
- Direct funding and fund raising for trial operations support
- Network recruitment / outreach
- Serve on a Data Safety Monitoring Board
- Report on patient feedback regarding sites, investigators, and study participant experience

**Phase 2/3**
- Serve on FDA advisory committees
- Provide testimony at FDA hearings
- Feedback on meaningful clinical endpoints
- Natural history database / registry support
- Provide feedback on how the patient community views results
- Help return study results to participants
- Write newsletter articles or blog about results
- Co-present results
- Serve on post-market surveillance initiatives

**FDA review & approval**

**PAS/Outcomes**

*Adapted from Parkinson’s Disease Foundation materials for CTTI’s Patient Groups & Clinical Trials Project*
## Continuum of Patient Advocacy Organizations

### Examples of Advocacy Outreach & Linkage

<table>
<thead>
<tr>
<th>Patient Support:</th>
<th>Research:</th>
</tr>
</thead>
</table>
| Provide medical, psychosocial support to patients & families | Patient Decision Support  
• Caregiver Support  
• Care Navigation |

<table>
<thead>
<tr>
<th>Education &amp; Information:</th>
<th>Public Influence:</th>
</tr>
</thead>
</table>
| Inform & Educate about risks, screening, disease & treatment & quality of life issues | Trial Matching  
• Trial Education |

<table>
<thead>
<tr>
<th>Research:</th>
</tr>
</thead>
<tbody>
<tr>
<td>Involved in shaping the research agenda, oversight of the research process, &amp; starting new initiatives</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Political Activity:</th>
</tr>
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<tbody>
<tr>
<td>Influence elected/regulatory bodies about reimbursement, research funding, patient needs/access, legislative issues</td>
</tr>
</tbody>
</table>

- Influence Policy on Covered Expenses for Patients in Clinical Trials  
- Legislation Development
How do you know which patient group will be a suitable partner for your clinical trial activities?

“A patient advocacy group is not a patient advocacy group. Some are very knowledgeable about the mechanics of their population and have databases to help recruit our trials. Some even help fund research. Others are nothing more than a Google group of five people.”

CTTI PGCT Interview Respondent 2014
Issues Around Engagement

Key sectors of the research community have identified a gap in knowledge and understanding about how and when to best interact with patient groups (PG) around clinical trials;

There is a paucity of empirical evidence and no guidelines for best practices currently exist;

Actionable recommendations and metrics are needed.

Solution: CTTI project on best practices for effective engagement with patient groups around clinical trials; Patient Groups and Clinical Trials (PGCT)
I believe that we would learn a lot from patient insight if we worked with patient advocacy groups. But the data [are] not rich enough to claim that conclusively. It is difficult to answer questions like, ‘If we focus our attention on the patient, does that translate into shorter timelines?’
Impact of Engaging PG’s Earlier on NPV

Increasing PG engagement reference points could Decrease
- Launch time
- Cost of CTE

Leverage assets ➔ De-risk investment

Pre-Discovery Pre-Clinical Phase 1 Phase 2/3 FDA review & approval

PAS/Outcomes
Better, Streamlined, Fit for Purpose Clinical Trials

- Change
- Build consensus
- Gather evidence
- Formulate recommendations
- Identify solutions
- Target problem areas in clinical trials
Key Term: Patient Groups (PGs)

- Patient advocacy organizations
- Disease advocacy organizations
- Voluntary health agencies
- Non-profit research foundations
- Public health organizations
Key Term: Clinical Trial Enterprise (CTE)

CLINICAL TRIAL ECOSYSTEM & ITS STAKEHOLDERS

- CLINICAL RESEARCH ORGANIZATIONS & SERVICES
- INSTITUTIONAL REVIEW BOARDS
- Payers
- Health Systems
- Sites
- Institutional Review Boards
- Payers
- Health Systems
- Sites
- Patient Groups & Advocates
- Research Participants
- Clinicians
- Regulators
- Academia
- Industry
- Government
CTTI Patient Groups and Clinical Trials Project
Expert Meeting Logistics

Day 1 Meals: Lunch 12:45-1:15 PM; Dinner 6:00 PM
Day 2 Meals: Breakfast 7:30-8:15 AM; Working Lunch 12:00

Wireless internet: Meeting ID DCRI, Meeting Password DCRI

Microphones Use: Push to talk/Push for off. Red is on.

Restrooms: Outside the Executive Forum to the right.

Parking: Please see Rene or Lauren at CTTI registration desk

Recording: This session is being recorded. The recording will not be made public, but used for archival purposes only.

Folder Contents and Breakout Session Logistics

Breakout 1- Executive Forum, Breakout 2 – 3rd floor Lindens, Breakout 3 – 3rd floor Potomac
Session I
Moderator: Ron Bartek (Freidreich’s Ataxia Research Alliance)

- Roadmap to Advocacy – FDA Perspective
  - Dr. Janet Woodcock (Food and Drug Administration, CDER)

- Success from Start to Finish: The COMFORT Trial
  - Presenter: William Tunno (Boehringer-Ingelheim, formerly InCyte)

- Key Findings from CTTI’s Project on Best Practices for Effective Engagement with Patient Groups around Clinical Trials
  - Wendy Selig (Melanoma Research Alliance)
Thank you.