Welcome to CTTI’s
Engaging Stakeholders in Trial Design
Expert Meeting

- This meeting is being recorded for note taking purposes only
- Masks are recommended if you are experiencing cold-like symptoms
- Open discussion is encouraged and fostered by respect and collaboration
- Do you have a comment during the open discussion?
  - Please tip your name tent card and a microphone will be delivered

Here’s to a great discussion!
# Agenda

<table>
<thead>
<tr>
<th>Time (EST)</th>
<th>Content</th>
<th>Presenter</th>
</tr>
</thead>
<tbody>
<tr>
<td>8:30 AM</td>
<td>Welcome Remarks and Introduction to CTTI</td>
<td>Sally Okun (CTTI)</td>
</tr>
<tr>
<td>8:45 AM</td>
<td>Engaging Stakeholders Project Overview</td>
<td>Greg Vico (BMS)</td>
</tr>
<tr>
<td>9:00 AM</td>
<td>Model for Success (<em>Questions/Open Discussion and Break to Follow</em>)</td>
<td>Minetta Liu (Natera)</td>
</tr>
<tr>
<td></td>
<td><strong>Panel Discussion</strong></td>
<td>May Mo (Amgen)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Li Wang (AbbVie)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>James Donohue (Roche)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Daniel Millar (Janssen)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Stephanie Ann Christopher (Pfizer)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Moderator: Naziah Lasi-Tejani (Roche)</td>
</tr>
<tr>
<td>11:35 PM</td>
<td>Introduction to Break Out Groups (<em>Lunch to follow</em>)</td>
<td>Kelly Franzetti (CTTI)</td>
</tr>
<tr>
<td>12:45 PM</td>
<td>Break Out Session</td>
<td>All Attendees</td>
</tr>
<tr>
<td>2:15 PM</td>
<td>Summarize Breakout Session: Open Discussion</td>
<td>May Mo (Amgen)</td>
</tr>
<tr>
<td>2:55 PM</td>
<td>Closing Comments and Adjourn</td>
<td>Robyn Bent (FDA)</td>
</tr>
</tbody>
</table>
Introduction to CTTI

Sally Okun, CTTI Executive Director
Clinical Trials Transformation Initiative

MISSION
To develop and drive adoption of practices that will increase the quality and efficiency of clinical trials.

VISION
A high-quality clinical trial system that is patient-centered and efficient, enabling reliable and timely access to evidence-based therapeutic prevention and treatment options.

PUBLIC-PRIVATE PARTNERSHIP
- Co-founded in 2007 by FDA and Duke University
- Active collaboration with +500 individuals and groups
- Steering Committee with +80 member organizations

SCOPE
Focus on clinical trials of FDA-regulated medical products, recognizing that clinical trials are international and acting as a collaborative global citizen.
Multi-Stakeholder

Everyone must have an equal seat at the table

Patients as partners

- Patients, Caregivers & Patient Advocacy Groups
- Academia
- Trade & Professional Orgs
- Government & Regulators
- Industry
- Investigators & Sites

Includes pharma, bio, device, CRO, health data/IT
CTTI Products

Recommendations
Evidence-based and actionable results from a CTTI project that are approved by the CTTI Executive Committee

Tools
Supportive resources developed by a CTTI project team to assist with the implementation and adoption of project recommendations

Publications
Reports
Case Studies
Webinars
Presentations
TRANSFORMING TRIALS 2030

By 2030, clinical trials need to be:

- Patient-Centered & Easily Accessible
- Fully Integrated Into Health Processes
- Designed With A Quality Approach
- Maximally Leveraging All Available Data
- Improving Population Health

A critical part of the Evidence Generating System

https://ctti-clinicaltrials.org/who_we_are/strategic-vision/
Continuation of CTTI Quality by Design Work

Quality by Design (Qbd) - an approach that focuses resources on the errors that matter to decision making during a trial. Includes:

- **Create a culture** that values & rewards critical thinking & open dialogue about quality
- **Involve the broad range of stakeholders** in protocol development & discussions around study quality
- Prospectively identify & periodically review the **critical to quality factors**
- **Focus effort** on activities that are essential to the credibility of the study outcome

CTTI has developed a suite of QbD resources:
https://ctti-clinicaltrials.org/our-work/quality/quality-by-design/

---

Engaging All Stakeholders in Clinical Trial Design

Create resources that will enable clinical trial designers to efficiently & effectively engage all stakeholders across the trial design process

Drafting engagement roadmap

- Patients & Caregivers
- Investigators & Sites
- Regulators
- Institutional Review Boards
- Technology
- Payers
Engaging Stakeholders in Trial Design

Project Overview

Greg Vico, BMS
Engage all stakeholders early and often in trial design

Prospectively identify and prioritize essential activities

Better protocols with fewer ‘errors that matter’

**CTTI QbD Recommendations**: “Engaging all stakeholders with study development is an important feature of quality by design.”

**ICH E8(R1) Draft**: “Clinical study design is best informed by input from a broad range of stakeholders…”

**CDRH/CBER Draft Guidance**: “FDA believes medical device clinical investigations prospectively designed with input from patient advisors may help to address common challenges…”
Engaging Stakeholders in Trial Design Project Overview

**Purpose:**
Provide details and logistics for engaging all stakeholders in the design of clinical trials

**Objectives:**
- Identify specific opportunities and high value approaches for study designers to engage with stakeholders
- Identify situation-specific considerations for ensuring engagement is appropriately equitable, effective, and feasible
- Assess the extent to which draft products address stakeholder needs and incorporate

**Anticipated Impact:**
- Increase meaningful engagement
- Produce high quality efficient clinical trials
Engage all stakeholders to:

- Identify critical to quality aspects of trial design and potential challenges
- Tailor design to avoid errors that could undermine evaluability or safety
- Streamline trial where feasible
- Verify proposed design consistent with scientific question
- Highlight and evaluate residual risks
Issues To Be Addressed

- Lack of comprehensive resources for engaging all stakeholders in trial design

- Most approaches assume patients and other stakeholders will be engaged separately, rather than having all stakeholders ‘at the table,’ as emphasized by Quality by Design

- The clinical trials enterprise lacks a unifying framework, especially one tied to regulatory guidance, delineating how the various existing engagement methods can be used collectively and coherently across the trial design process
Stakeholders to Engage

- Clinical / Medical
- Biostatistics
- Medical Writing
- Clinical Operations
- Clinical Data Management
- Regulatory Affairs
- Safety / Pharmacovigilance
- IRB
- Payers
- Clinical Quality Management
- Investigative Site Staff
- CRO and technology
- Patients, Caregivers & Patient Groups
- Regulatory Bodies
Initial Project Outputs

- Engagement Roadmap identifying detailed, ‘nuts and bolts’ considerations for who and how to engage at each stage of trial planning
  - Covers all phases of development – though may not address all in equal depth or detail
  - Identifies all major stakeholders, though may not address engagement approaches for all stakeholders in equal depth or detail
  - Serves as a ‘one-stop shop,’ linking to existing tools/resources
  - Visual and easy to follow, likely structured as a timeline or flowchart
  - Flexible, helping to identify the best engagement approach given particular situation
  - Outlines engagement approach that is effective and efficient (e.g., with respect to time, cost, minimizing repetition)
## Multi-Stakeholder Project Team

### Team Leads
- Lorri Wiggins (FDA)
- Naziah Lasi-Tejani (Roche)
- Durga Borkar (VeranaHealth)
- Karlin Schroeder (Parkinson’s Foundation)*
- Whitney Bondurant (University of Mississippi)*
- Michael Howland (Genentech)*

### Team Members
- Robyn Bent (FDA)
- David Borasky (WCG IRB)
- Suanna Bruinooge (American Society of Clinical Oncology)
- Patrick Frey (Amgen)
- Courtney Granville (Drug Information Association)
- Heather Kim (WCG IRB)
- David Leventhal (Pfizer)
- Renee Leverty (Duke)
- May Mo (Amgen)
- Xinyi Ng (FDA)
- Nuru Noor (Patient/Caregiver)
- Gregory Pennock (EMD Serano)
- Jamil Rivers (Patient/Caregiver)
- Jeanine Salamone (American Society of Clinical Oncology)
- Theresa Strong (Foundation for Prader-Willi Research)
- Pujita Vaidaya (Amgen)*
- Greg Vico (Bristol-Myers Squibb)
- Glendon Zinser (Susan G. Komen)

### Writer
- Alexis McCloskey (Duke)

### Communications Lead
- Rae Holliday (CTTI)

### Project Manager
- Kelly Franzetti (CTTI)

### Event Planner
- Susan Morris (CTTI)

*former team lead or member
Today’s Meeting Objectives

- Review two clinical trial ‘models’ where stakeholder engagement was well-executed
- Discuss and explore opportunities, barriers, and best practices for study designers to engage all stakeholders in trial design
- Identify situation-specific considerations for ensuring engagement is appropriately equitable, effective, and feasible
Model for Success

Session I Objectives:

- Explore trials in which stakeholders were well engaged
- Identify key milestones/interactions that supported this success
Models for Success

Minetta Liu, Natera
INSERT MINETTA’S SLIDES
Q & A
BREAK

Return at 10:30 am
Challenges and Opportunities
Panel Discussion

Naziah Lasi-Tejani, Roche
Session Objectives

- Discuss and explore opportunities, barriers, and best practices for engaging all stakeholders in trial design.
- Identify specific opportunities and approaches for study designers to engage with internal and external stakeholders across the clinical trial design and planning process.
Topics Overview

- Building the Value Proposition of Engaging Stakeholders in Trial Design
- Timing of Stakeholder Engagement
- Operationalizing Stakeholder Feedback
- Simplifying the Process
- Measuring the Impact
Panel: Challenges and Opportunities to Engagement

Moderator: Naziah Lasi-Tejani, Roche

Stephanie Ann Christopher, Pfizer
James Donohue, Roche
Daniel Millar, Janssen
May Mo, Amgen
Li Wang, AbbVie
Breakout Topics Overview

- Timing of Stakeholder Engagement
- Operationalizing Stakeholder Feedback
- Simplifying the Process
- Measuring the Impact
Breakout Group Instructions

- Check badge for dot color
- Duration: 90 minutes
- Refreshments available in foyer throughout the session
- Please enjoy lunch and then go directly to your breakout session location

<table>
<thead>
<tr>
<th>Group</th>
<th>Dot Color</th>
<th>Location</th>
<th>Moderator</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Green</td>
<td>Lincoln</td>
<td>Glendon Zinser</td>
</tr>
<tr>
<td>2</td>
<td>Yellow</td>
<td>Declaration A</td>
<td>Renee Leverty</td>
</tr>
<tr>
<td>3</td>
<td>Red</td>
<td>Banneker</td>
<td>Suanna Bruinooge</td>
</tr>
<tr>
<td>4</td>
<td>Blue</td>
<td>Declaration B</td>
<td>Courtney Granville</td>
</tr>
</tbody>
</table>
Go to Breakout Session at 12:45 pm
Breakout Sessions

Return to Main Session at 2:15 pm
Breakout Summary
with Discussion

May Mo, Amgen
Welcome Back!

Session III Objectives:

- Session moderators share highlights of each breakout
- Q&A/Discussion
Breakout Session Summaries

**Breakout Moderators:**
- Group 1: Glendon Zinser, Susan G. Komen Foundation
- Group 2: Renee Leverty, Duke Clinical Research Institute

**Timing of Stakeholder Engagement**
- Who is engaged during the discovery and early research phases?
- What topics are most relevant for study teams to get feedback on from external stakeholders?
- Are specific topics better suited for specific stakeholders?
- Soliciting structured feedback from each stakeholder group
- How can the stages or timing of stakeholder engagement be classified?

**Measuring the Impact**
- How can the impact of stakeholder engagement be measured in drug development efforts?
- What role can organizations like CTTI play in the development of measurable KPIs for stakeholder engagement in the design of clinical trials?
Breakout Session Summaries

**Breakout Moderators:**
- Group 3: Suanna Bruinooge, American Society of Clinical Oncology
- Group 4: Courtney Granville, Drug Information Association

**Simplifying the Process**
- Challenges when planning for and executing stakeholder engagement
- When and how to engage with stakeholders
- What role will technology play and how will it complement or augment stakeholder engagement
- Soliciting structured feedback from each stakeholder group
- How can the stages or timing of stakeholder engagement be classified

**Measuring the Impact**
- How can Sponsors measure the impact of stakeholder engagement in their drug development efforts?
- What role can organizations like CTTI play in the development of measurable KPIs for stakeholder engagement in the design of clinical trials?
Closing Comments
Robyn Bent, FDA

April 4, 2023
Next Steps & Potential Timeline

April 2023
- Expert Meeting Summary
  - Key themes from meeting will be posted on CTTI Website

Q2 2023
- Post Expert Meeting
  - Expand engagement roadmap
  - Develop recommendations and resources

Q2-Q3 2023
- Second Virtual Expert Meeting
  - Gain feedback on engagement roadmap
  - Troubleshoot remaining gaps

Q1-Q2 2024
- Launch Recommendations
  - CTTI convenes a Recommendations Advisory Committee to refine recommendations
  - CTTI hosts public webinar to launch recommendations and supporting tools

CTTI