Patient Engagement Efforts with the Clinical Trial Enterprise

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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 or Duke Clinical Research Institute.
Many of today’s patient groups serve as active partners in the clinical trial enterprise leveraging their skills, assets, and capabilities to de-risk research and reduce regulatory uncertainty.
CTTI

Public-Private Partnership co-founded by Duke University & FDA involves all stakeholders 90+ members

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

State Problem
- Issue Statement, Project Plan

Gather Evidence
- Literature Reviews, Multi-stakeholder Meetings, Surveys, Interviews

Find Solution
- Team Meetings, Multi-stakeholder Meetings

Refine Ideas
- Team Meetings, Multi-stakeholder Meetings

Action
- Workshops, Pilot Studies, Measure Impact

Identify Research Impediments

Identify Gaps/Barriers

Analyze and Interpret Findings

Develop Recommendations/Tools

Disseminate & Drive Adoption
Issues Around Engagement in 2014

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

There was a paucity of empirical evidence and no guidelines for best practices existed;

Actionable recommendations and metrics were needed.

Solution: CTTI project on best practices for effective engagement with patient groups around clinical trials; Patient Groups and Clinical Trials (PGCT)
Patient Groups and Clinical Trials Project Team

- Richard Klein (FDA)*
- David Leventhal (Pfizer)*
- Jaye Bea Smalley (Boehringer Ingelheim)*
- Sophia Smith (Duke)*
- Amy Abernathy (Duke)
- Ronald Bartek, (Friedreich's Ataxia Research Alliance)
- Joel Beetsch (Celgene)
- Patricia Cornet (Bristol-Myers Squibb)
- Jim Kremidas (ACRP)
- Paulo Moreira (EMD Serono)
- Steve Roberds (Tuberous Sclerosis Alliance)
- Jamie Roberts (Duke)
- Wendy Selig (WS Consultants)
- Jeff Sherman (DIA)
- James Valentine (Hyman, Phelps & McNamara, P.C.)
- Scott Weir (University of Kansas Medical Center)
- Joe DiMasi (Tufts)
- Eric Eisenstein (Duke)
- Ken Getz (Tufts)
- Michele Goldberg (J&J)
- Matthew Harker (Duke)
- Sharon Hesterlee (Bamboo Therapeutics)
- Bennett Levitan (J&J)
- Bray Patrick-Lake (Duke)
- Al Roy (Lupus Research Alliance)
- Amy Cornelia (CTTI Social Science Lead)
- Zachary Hallinan (CTTI Project Manager)

*Project team leader
Objective 1: Identify Best Practices for Engaging Patient Groups in Clinical Trials

1. Conduct a *literature review* and *survey* to assess types of relevant PGs, querying a representative sample across disease states to highlight distinctions in missions, reach, infrastructures, governance models and interest and engagement in clinical trials.

2. Identify current research sponsor and investigator *practices for engaging with PGs*, and practices used by patient groups to engage with research sponsors and investigators, around clinical trials.

3. Explore *successes and failures* to identify models of engagement with PGs that have led to more quality driven and efficient trials.

4. Formulate *recommendations* and opportunities for implementation of best practices with PGs, academia and industry that will lead to more efficient and successful clinical trials.
Objective 1 Methodologies

- **Joint CTTI/DIA Survey**
  - 2014
  - 244 respondents from diverse groups

- **32 Stakeholder Interviews**
  - 2014
  - Diverse stakeholders from industry, academia & patient groups

- **Multi-Stakeholder Expert Meeting**
  - 2015
  - 62 experts from diverse backgrounds

https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials
Timing of **Industry** Engagement with **PG**

Choose all that apply:

- 80% at Phase III
- 62% at Phase Ila
- 35% at Phase I/Proof of concept
- 15% at Discovery/Pre-clinical
CONFIRMED BARRIERS TO COLLABORATION

- Mismatched expectations between trial teams & PGs
- Providing PGs w/ only a token seat at the table, not making them full partners in the trials process
- Internal resistance, lack of buy-in
- Perceived difficulty of overcoming legal barriers to industry/patient collaboration
- Excluding PGs from early stages of trial planning & design
- Lack of sophistication of PGs
- Unsure of how to identify/engage w/ PGs
- Lack of engagement best practices & lack of infrastructure to support patient outreach operations
- Lack of demonstrated value
- Lack of funding
Patient Group Engagement Across the Clinical Trial Continuum

Building a model to evaluate impact

<table>
<thead>
<tr>
<th>Pre-Discovery</th>
<th>Pre-Clinical</th>
<th>Phase 1</th>
<th>Phase 2/3</th>
<th>FDA review &amp; approval</th>
<th>PAS/Outcomes</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Interest of research question to patient community</td>
<td>• Network recruitment / outreach</td>
<td>• Direct funding and fund raising for trial operations support</td>
<td>• Direct funding and fund raising for trial operations support</td>
<td>• Serve on FDA advisory committees</td>
<td>• Serve on FDA advisory committees</td>
</tr>
<tr>
<td>• Provide data on unmet need and therapeutic burden</td>
<td>• Direct funding and fund raising for research or product development</td>
<td>• Network recruitment / outreach</td>
<td>• Network recruitment / outreach</td>
<td>• Provide testimony at FDA hearings</td>
<td>• Provide testimony at FDA hearings</td>
</tr>
<tr>
<td>• Direct funding and fund raising for research or product development</td>
<td>• Infrastructure support</td>
<td>• Provide input on study design (barriers to participation)</td>
<td>• Direct funding and fund raising for research or product development</td>
<td>• Feedback on meaningful clinical endpoints</td>
<td>• Feedback on meaningful clinical endpoints</td>
</tr>
<tr>
<td>• Understanding mechanisms of action relevant to disease and symptom burden</td>
<td>• Support trial awareness and recruitment</td>
<td>• Peer advocate during informed consent procedure</td>
<td>• Support trial awareness and recruitment</td>
<td>• Co-present results</td>
<td>• Co-present results</td>
</tr>
</tbody>
</table>

*Adapted from Parkinson’s Disease Foundation materials for CTTI’s Patient Groups & Clinical Trials Project*
Through active, continuous engagement in the development program, PGs can demonstrate a unique value to their industry partners.

This value has the effect of:

- Derisking early-stage development with funding and public-private partnerships for basic, translational, and early clinical research
- Reducing uncertainty in the regulatory process by working closely with the regulators throughout the entire R&D process
Active, continuous engagement in the development program – cont.

Helping to develop more effective, efficient trials with a greater chance of success through:

- better questions and study design
- efficient recruitment and improved retention
- fewer protocol amendments
- procedures that are better-suited to the patient
- clinical endpoints that are well-grounded in the natural history of the disease
- potential benefits that are most important to the patient
Engage the patient voice by establishing partnerships from the beginning of the research and development program to improve trial design and execution.

- Include the perspective of patients (i.e., the “patient voice”) in the early stages of product development

- Sponsors benefit by a clearer, more focused understanding of unmet need, therapeutic burden, subgroups of patients, perceptions of benefit-risk and opportunities for expanding indications

- Patients benefit by less burdensome study protocols and more meaningful and relevant endpoints & PROs
  - increases the likelihood they will participate in the trials or potentially help to develop a meaningful treatment for their disease
From the start, clearly define the expectations, roles, and responsibilities of all partners, including the resources being committed, data being shared, and objectives of the program.

- It is important to clearly delineate the roles of partnership and clarify the goals and objectives of the collaboration.

- Expectations about the role of PG consultation and input should be clarified at the start of the collaboration.

- PG input may be taken into account when determining the objectives of a clinical program or development of a protocol; research sponsors must balance that input with scientific understanding as well as business and regulatory needs.
Manage real or perceived conflicts of interest by establishing policies that require full disclosure, transparency, and accountability.

- There are no FDA laws, regulations, or guidelines explicitly prohibiting early engagement with PGs.
- It is important to clarify which kinds of interactions with PGs are permissible and which ones might violate FDA regulations or fraud, abuse, and other regulations.
- The bottom line is that research sponsors can engage with PGs in planning and conducting clinical trials.
- Each type of PG engagement will have its own contractual rules and parameters to mitigate risk.
Ensure that PGs are essential partners throughout the R&D process and not token voices.

- Research sponsors should recognize that the most successful partnerships with PGs are those in which both entities are full partners at the outset, working toward the same goals from different perspectives.

- The patients’ voice as communicated by PGs is key to understanding the day-to-day effects of the condition and the acceptable benefit-risk tradeoff of treatment.
Possible 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

*Above graphic is based on “Considerations of net present value in policy making regarding diagnostic and therapeutic technologies” by Califf et al.
**Conclusions**

- Partnerships with PGs around clinical trials are occurring with greater frequency.

- We now have evidence-based best practices and understanding of shared benefit to partnerships captured in CTTI’s recommendations.

- The needle is moving on patient engagement & medical device development and regulation!

- Read the full set of publications and recommendations at [https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials](https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials)

THANK YOU.

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