Involving Patients in Research and Pragmatic Trials

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Shift in Research and Regulatory Engagement

There has been a significant shift in how sponsors engage with patients, moving away from the traditional approach of keeping patients at arm’s length as passive subjects in clinical trials, toward one of recognizing them as active partners across the research and development continuum.

As patient organizations, industry leaders, and regulatory officials work to achieve patient centricity and precision medicine, and develop mechanisms for ensuring the patient voice is incorporated into all aspects of therapy development, evidence of the positive impact of patient engagement is mounting.
Patients and researchers agree

"Patients, caregivers, and others with an interest in improving clinical research can provide a unique voice to inform researchers and help identify knowledge gaps by sharing their experiences with the daily burden of disease conditions, their thoughts on opportunities for treatment, and the types of research questions they value the most."

As coinvestigators for PROSPER, we are working alongside doctors and scientists to answer key questions about quality of life, depression, and fatigue that have often been neglected in clinical trials.

Circulation

Certain Uncertainty
Life After Stroke From the Patient’s Perspective
Deidre Hannah, MSN, RN; Brianna Lindholm, BA; Lesley Maisch, BA

As patients, we must accept the responsibility of being advocates for our own healthcare, and we cannot do so if we are not empowered by a fundamental understanding of medical research. […]
Value of Patient Engagement

- Goal of driving high-quality, efficient, patient-centered research by:
  - Developing study that is feasible and minimally burdensome for participants
  - Increasing accrual rates by proactively addressing barriers to recruitment and improving study materials
  - Drive higher rates of retention and compliance through enhanced value and improved participant experience
  - Improving data quality through minimizing patient dropout and enhancing participant adherence to protocol

- Drive more rapid research innovation cycles fueled by continuous patient input.

- Answering questions that matter to patients.
DCRI Guiding Principles for Engagement

- **People** come first. Always.

- We recognize that people are embedded in dynamic family and community frameworks that we honor and respect across the continuum of life care.

- **People are our partners** in research; not our subjects. We believe in taking every opportunity to co-learn. We engage participants, families, and community members in our research design, conduct, oversight, and dissemination activities.

- We are transparent and trustworthy. We communicate to research participants how valuable their contributions are to science and medicine. **We take the time to thank research participants, update them on progress, and share our findings in language understandable to everyone.**

- We create value. **We work to return results in a responsible and meaningful manner and maximize what can be learned by sharing data with other researchers.** We give back.

- We are not transactional in our approach. We encourage and incentivize collaborations with people and communities that look past the end of a project or last study visit. We create opportunities to continue co-learning and working in partnership with Participants, families, and community members to improve health outcomes.
When to Engage Patients:
Early, often, always from bench to bedside and back

- Provide information on unmet need and therapeutic burden
- Interest of research to patient community

- Provide input on study design such as study endpoints, inclusion/exclusion, risk/benefit perception, and barriers to participation

- Advise on study recruitment strategies and materials
- Support recruitment of study participants
- Disseminate updates

- Assist w/ interpretation and provide feedback on how the participant community will view results

- Advise on study designs, better understanding of use, adherence, persistence, access, impact

Participants work with sponsor or researcher to support funding applications and models

- Help finalize eligibility criteria within the study protocol
- Assist in creating the informed consent process and form

- Serve on a Data Safety Monitoring Board
- Provide recommendations for revising study protocol if changes need to be made

- Thank participants
- Update on progress
- Share findings in laypeople language

- Work with trial team to ensure study participants get feedback from study
- Advise on return of individual results and development of lay summaries
- Write newsletter articles or blog posts about results
- Co-present results with sponsors and researcher at scientific conferences or in Participant communities

Develop the study concept
Secure funding
Prepare the study protocol
Create study procedure
Implement the study
Monitor the study
Analyze data and interpret results
Disseminate study information
FDA review and approval
Post-Approval/RWE studies

Duke Clinical Research Institute
Active, continuous engagement in pragmatic clinical trials

Helps 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
- Outcomes and potential benefits that are most important to the patient
What can happen when patients provide input on study design?

Consider two major mechanisms that can impact on project value:

1. **May avoid a protocol amendment**
   - ~70% of phase 2 and 3 trials have at least one amendment*
   - ~22% of amendments are due to recruitment difficulty or feedback from sites or investigators*

2. **Improving the patient experience**
   - Making the informed consent easier to understand
   - Simplify the eligibility criteria
   - Making the trial less demanding for patients
   - Providing in-trial feedback to patients


Why are we talking about it?

- Amendments cost an average of $500,000
- 8 weeks to implement each amendment
- Typical phase 3 study has 4 amendments
- 40% of amendments deemed “avoidable”

Many amendments occur before the first patient is enrolled and could have been prevented with better planning and engagement of the patient community.

Measures

- Participant experience
- Recruitment rates
- Retention rates
- Adherence rates
- Comparison of number of protocol amendments to similar trials in sponsor portfolio
- ROI on engagement via CTTI conceptual model for ENPV
Case example: Meet the Adaptors

The ADAPTABLE patient partners, Adaptors, work alongside researchers in all aspects of the trial.

- Involved with the trial from the beginning
- Helped design protocol, consent form, study portal, and study materials
- Played an integral role in disseminating study updates and raising awareness
- Participated in investigator meetings and trial committees
- Stayed active in Facebook Live programs, discussions, and social media activities
- Served as patient representatives on the Executive and Steering Committee. *Additional patient reps on DSMB*
Pragmatic Design + Patient Engagement = Fewer Sites + More Rapid Recruitment

### ADAPTABLE vs Traditional Trials

<table>
<thead>
<tr>
<th>Trial</th>
<th>Days to 1st 1,000 Patients Enrolled</th>
<th>Number of Enrolling Sites</th>
</tr>
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<tr>
<td>EUCLID</td>
<td>192</td>
<td>217</td>
</tr>
<tr>
<td>ODYSSEY</td>
<td>621</td>
<td>262</td>
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<tr>
<td>ADAPTABLE</td>
<td>281</td>
<td>17</td>
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</table>

*US Sites Only
CONNECT-HF: Care Optimization through Patient and hospital Engagement Clinical Trial for Heart Failure

A large-scale, pragmatic, cluster-randomized clinical trial to evaluate the effect of a customized, multifaceted, health system-level quality-improvement program compared with usual care on heart failure outcomes and quality-of-care metrics

Scientific rationale: Change care improve outcomes
CONNECT-HF

Site Study Teams and QI Leaders Academy

Patient Representatives Cardi-Yacks

Steering Committee

Coordinating Center (Duke Clinical Research Institute)

Center for Advanced Hindsight
Cardi-Yacks: Patient Advisors
Digital Substudy Designed in Partnership with Patients

Optimizing chronic disease management and secondary prevention efforts requires sustainable and durable change in patient behavior.

Study Coordinator conducts onboarding of apps and devices.

Patient uses 1 of 2 apps at home.

HOSPITAL

HEALTHSTAR

HOME

DISCHARGE
HealthStar

- Utilizes the principle of loss aversion on:
  - Medication adherence
  - Activity
  - Diet
  - Weight measurements
Leveraging Behavioral Economics to Improve Heart Failure Care and Outcomes

**ABSTRACT:** Behavioral challenges are often present in human illness, so behavioral economics is increasingly being applied in healthcare settings to better understand why patients choose healthy or unhealthy behaviors. The application of behavioral economics to healthcare settings parallels recent shifts in policy and reimbursement structures that hold providers accountable for outcomes that are dependent on patient behaviors. Numerous studies have examined the application of behavioral economics principles to policy making and health behaviors, but there are limited data on applying these concepts to the management of chronic conditions.
Website designed to support Heart Failure Patients
References


Thank you!

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