

Involving Patients in Research and Pragmatic Trials

Renee Leverty BSN, MA
PARTNERS PPRN,
Research Together Engagement Liaison
Duke Clinical Research Institute



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FROM THOUGHT LEADERSHIP
TO CLINICAL PRACTICE

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.



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. [...]

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.



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

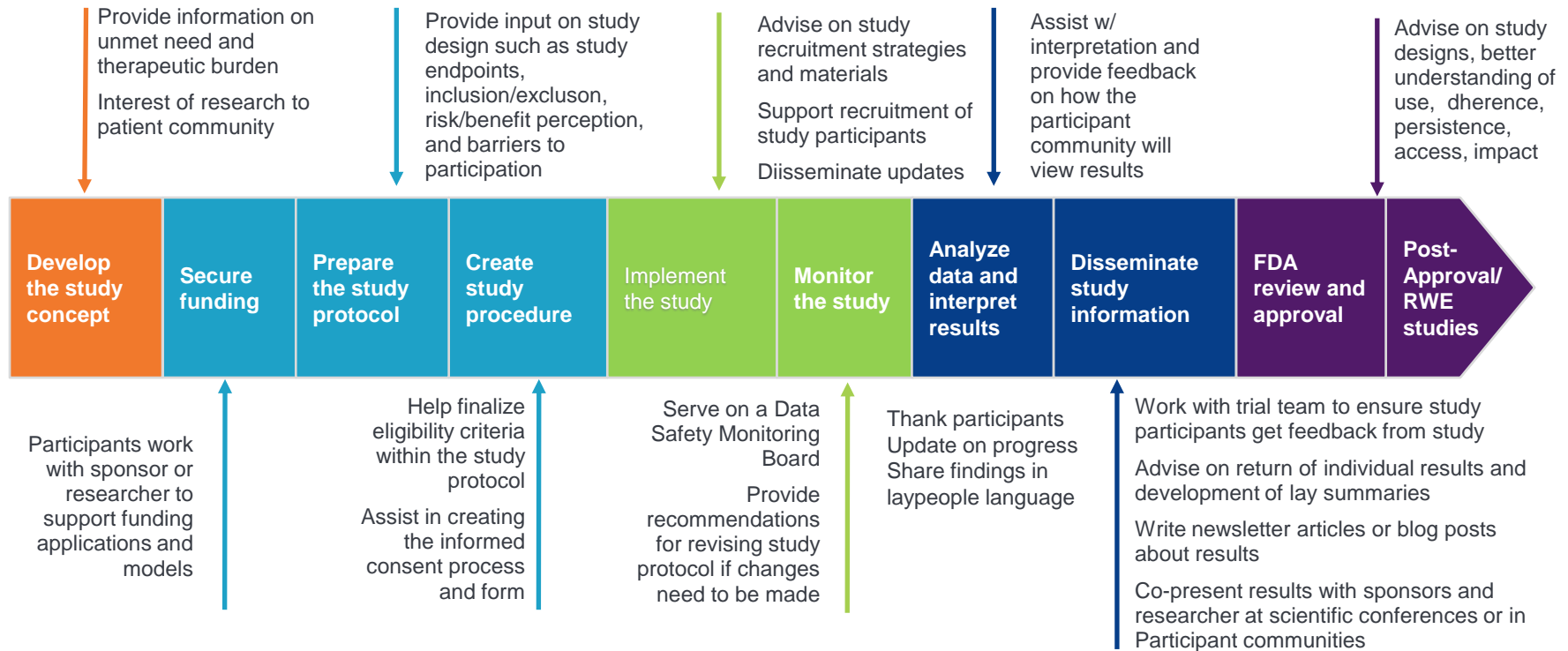


- **People** come first. Always.
- We recognize that people are embedded in **dynamic family and community frameworks** that we honor and respect across the continuum of life care.
- **People are our partners** in research; not our subjects. We believe in taking every opportunity to co-learn. We engage 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



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

*Getz K, et al,. Measuring the incidence, causes, and repercussions of protocol amendments. Drug Inform J. 2011;45:265-275.

Getz K, et. al. The impact of protocol amendments on clinical trial performance and cost. Ther Innov Regul Sci 2016; 50(4):436-441.



Why are we talking about it?

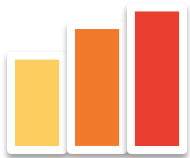
- Amendments cost an average of **\$500,000**
- **8 weeks** to implement each amendment
- Typical phase 3 study has **4 amendments**
- **40% of amendments** deemed “avoidable”



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

Source: Ken Getz, Tufts CSDD,
“The State of the Clinical Research Enterprise.”
NINDS INSPIRE Workshop, June 2013.





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

Trial	Days to 1 st 1,000 Patients Enrolled	Number of Enrolling Sites
EUCLID	192	217
ODYSSEY	621	262
ADAPTABLE	281	17

*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

Partnering to improve cardiac care

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



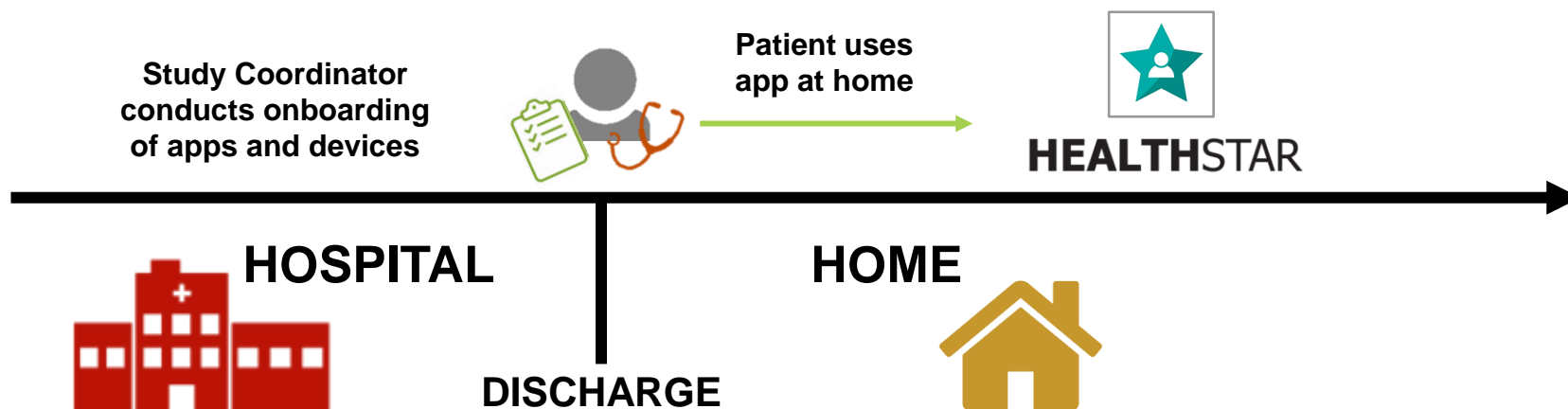
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Cardi-Yacks: Patient Advisors



Digital Substudy Designed in Partnership with Patients

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



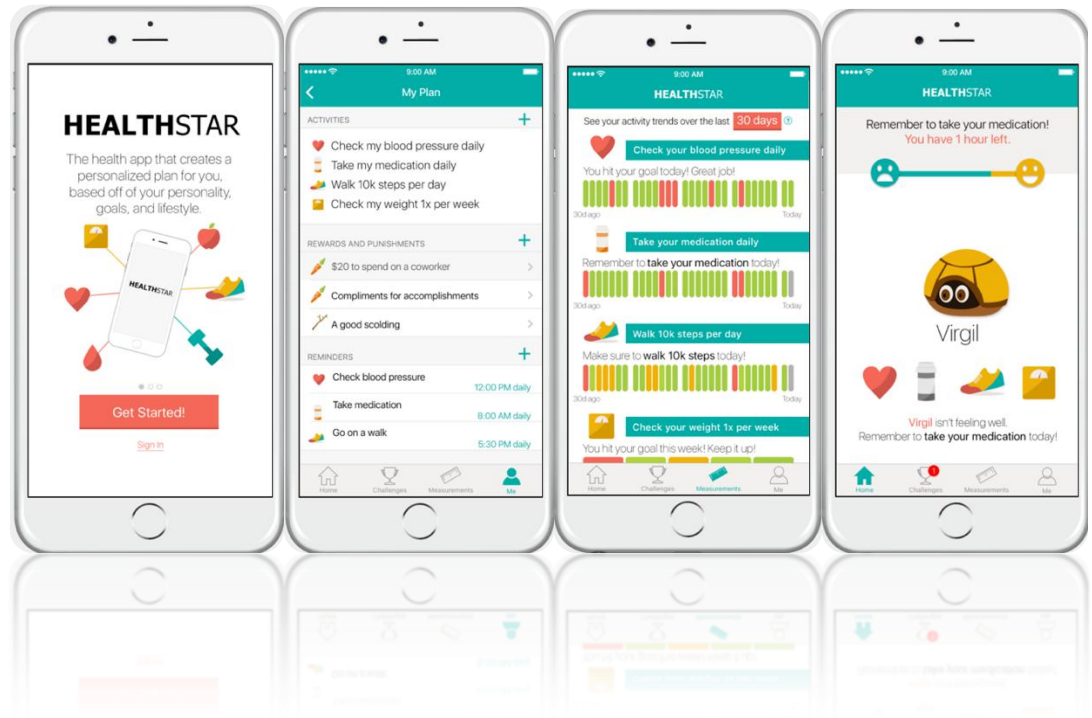


CONNECT-HF

Partnering to improve cardiac care

HealthStar

- Utilizes the principle of loss aversion on:
 - Medication adherence
 - Activity
 - Diet
 - Weight measurements



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CONNECT-HF

Partnering to improve cardiac care

IN DEPTH

STATE OF THE ART

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

Leslie L. Chang, BS
Adam D. DeVore, MD,
MHS
Bradi B. Granger, PhD,
MSN, RN
Zubin J. Eapen, MD, MHS
Dan Ariely, PhD
Adrian F. Hernandez, MD,
MHS



<http://www.connectheartfailure.org/>

Website designed
to support Heart
Failure Patients



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Thank you!

DCRI-ResearchTogether@Duke.edu



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