Progress Through Partnership: Actively Engaging Patient Groups in the Clinical Research Process

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Background

• Translation is a team sport. No single player can bring new treatments all the way to the finish line.
• Relevant communities and stakeholders should be included as active partners, not “subjects” or “consumers.”
• Early engagement of affected or interested parties is critical to success.
• Patient groups, NIH, industry and others increasingly recognize the need for partnership.
Partnership Across the Spectrum of the Clinical Research Enterprise

• Make sure that the research questions matter to patients.
• Ensure feasible protocols with acceptable burden.
• Promote stakeholder input into consent language.
• Include patients in implementation and safety oversight.
• Improve dissemination through communication with relevant communities.
NEURONEXT - CLINICAL TRIALS NETWORK FOR NEUROLOGICAL DISORDERS

EXAMPLE OF PATIENT ENGAGEMENT ACROSS THE PROJECT LIFESPAN
NeuroNEXT
Clinical trial network for neurological disorders

NINDS
- Exploratory trials (Phase 2)
- Biomarker studies intended to inform trial design
- The Network also accepts proposals for adaptive, seamless Phase II/III designs for diseases with prevalence of <5,000 in US

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Leverage partnerships
Working with Patients and Communities

- All NeuroNEXT Protocol Working Groups include a patient representative to provide input on the protocol as well as informed consent from the beginning.
- The Steering Committee for each study includes a patient representative.
- The Data and Safety Monitoring Board has a patient representative.
Example: NeuroNEXT Study NN101

- Spinal Muscular Atrophy (SMA) Biomarkers in the Immediate Postnatal Period of Development
  - PI: Stephen Kolb MD PhD
  - Ohio State University
- This study was done in collaboration with Families of SMA (providing travel funding).
- Families and stakeholders were included from the onset.
Lessons Learned

• We need best practices in this somewhat uncharted territory.
• Establish transparent way of inviting patient representatives.
• No need for scientific expertise for patient representative, but need enough information to be an equal partner.
• Consider all issues related to patient representative participation (time, cost, work, travel, needs).
THE CLINICAL AND TRANSLATIONAL SCIENCE AWARDS (CTSA) PROGRAM
Clinical and Translational Science Awards (CTSA) Program Sites (n=62)

- National consortium of medical research sites
- Work together to improve the way clinical translational research is conducted nationwide
- Provide training for clinical translational researchers
Standard Model

Basic Laboratory Research

Clinical Research

Translational Research

Population Research

Improved Public Health
Community Engagement Studios

- Structured process of eliciting project-specific input
- May be used in any phase of translational research
- Stakeholders selected based on researchers’ needs
- An experienced core team identifies stakeholders and prepares them for engagement; reduces burden to researcher

Clinical Trial Recruitment Before and After Community Engagement Studio

Before: No participants enrolled after 3 months of active recruitment

After: Targeted enrollment reached ahead of schedule; 100% retention in randomized, blinded, placebo controlled trial with 10 study visits
RARE DISEASES CLINICAL RESEARCH NETWORK (RDCRN)

A Model for Collaborative Rare Diseases with patient advocacy groups as partners.
About RDCRN

- Each consortium conducts at least two multi-site clinical studies (including one longitudinal study), has a training program, pilot project program
- Collectively, the RDCRN is studying 200 rare diseases in natural history and clinical trials at 240 clinical sites located in the US and in 14 countries.
- There are more than 90 active protocols.
- 29,000 patients have enrolled in clinical studies.
- There have been 174 trainees.
- There are 2,290 collaborative consortium members.
- There are 98 PAGs as research partners, collectively formed a Coalition (CPAG). [http://rarediseasesnetwork.epi.usf.edu/](http://rarediseasesnetwork.epi.usf.edu/)
Each consortium has direct involvement of patient advocacy groups (PAGs)

- Advise on operations, activities and strategy

Collectively, CPAG represent all PAGs associated with the RDCRN

- Meets via monthly call
- Influences the direction of the RDCRN as a whole
Take-Home Messages

• Our understanding of what causes diseases creates unprecedented opportunities for translation
• The process currently takes too long
• Partnership among the stakeholders in the clinical research enterprise is critical to accelerate progress
• We need to develop, demonstrate and disseminate effective models of partnership, considering
  ➢ How to identify and involve patient groups at the earliest stages of research
  ➢ How to change the research culture to engage patients
  ➢ How to achieve bidirectional communications between researchers and patients
  ➢ How to measure outcome, not just process