Patient-Centered Approaches to Research Pathways

Sharron Close, PhD, MS, CPNP-PC, FAAN
Emory University
School of Nursing and Department of Human Genetics
sharron.m.close@emory.edu
Why Care about Patient-Centered Research?

• Outcomes that patients can use sooner rather than later
• Comparative effectiveness of interventions most likely to help patients
• Research that informs clinical decision-making in reasonable time frames.
• Patients and families often offer solutions (interventions) that can be studied
Lessons Learned: The PCORI Perspective

• Thought I understood going in:

• What I learned in the process of writing a grant

• What I learned about patient-centeredness in the process
Bidirectional Translation Engagement Project

The Problem
• Gaps in Knowledge
• Gaps in Research
• Lack of Treatment or Interventions that are Useful to Patients or Families
• Inadequate Communication with Clinicians & Scientists
• Powerlessness to Influence Symptom Management
• Scientists study problems that have limited interest or utility to patients and families

Inputs

The Investment
• AXYS
• Emory University
• PCORI
• Staff
• Volunteers
• Time
• Money
• Research
• Technology

Brainstorm Mtg.
AXYS/Emory Stakeholder Selection
Online Stakeholder Planning Mtg
Family Conference 2015
Scientific Conference 2016

Activities How & What We do

Brainstorm Mtg.
AXYS/Emory Stakeholder Selection
Online Stakeholder Planning Mtg
Family Conference 2015
Scientific Conference 2016

Outputs

Participants Who We Reach
• Patients
• Families
• Clinicians
• Scientists
• Payors
• Pharma
• Clinical Administration
• AXYS
• Emory University
• PCORI

The Investment

Participants Who We Reach

Outcomes/Impact

Short Term Results
• AXYS
• Emory University
• PCORI
• Staff
• Volunteers
• Time
• Money
• Research
• Technology

Medium Term Results
• AXYS
• Emory University
• PCORI
• Staff
• Volunteers
• Time
• Money
• Research
• Technology

Long Term Results
• AXYS
• Emory University
• PCORI
• Staff
• Volunteers
• Time
• Money
• Research
• Technology

Assumptions: Families Informing Science  Science Informing Families

External Factors: Time, Location, Individual Commitment, Conflicts, Unexpected Events
Monthly Engagement Process

• PI met every month with genetic signature representatives (they wished to be called “ambassadors” to conduct a stepwise exploration of personal and clinical needs

• Systematic collaboration ensued toward “Bidirectionality” of communication

• Goal-driven plans initiated to initiate meaningful communication between patients, families, clinicians and scientists

• Building toward the ultimate goal of changing the way “science” considers X & Y participants.
Ambassador Engagement Call
September 3, 2015

Sharron Close, PhD, MS, CPNP-PC
Ambassador Engagement Call
October 5, 2015

Sharron Close, PhD, MS, CPNP-PC
Kristy Martyn, PhD, CPNP-PC FAAN
Ambassador Engagement Call
December 10, 2015

Sharron Close, PhD, MS, CPNP-PC
Kristy Martyn, PhD, CPNP-PC FAAN
Jim Moore, BS
Ambassador Engagement Call
April, 2016

Sharron Close, PhD, MS, CPNP-PC
Kristy Martyn, PhD, CPNP-PC FAAN
Jim Moore, BS
Ambassador Engagement Call
June 28, 2016

Sharron Close, PhD, MS, CPNP-PC
Kristy Martyn, PhD, CPNP-PC FAAN
Jim Moore, BS
Arriving at Outcomes Year 1

• 2 Focus Groups: Patients, Family Members, Clinicians and Scientists (Conference)
• Using **Qualitative Methodology: Interpretive Description**
• Results
  • Mindfulness of Person
  • Clinical Confusion
  • You Can’t Study That
  • Invisibility
  • Systems Letdown

Framing the Structure of Future Research
Over 24 months of Engagement

- Probing clinical and all X & Y Chromosome-related needs
- Teaching patients and families about how research is done
- Taking the outcomes from Year 2 and turning them into research questions that matter to them
- Plan to re-engage patients, families, clinicians and scientists (Conference) to form
  - New research questions
  - Determine best methodologies
  - Form new collaborations

Framing the PATHWAY of Future Research
Productivity-Driven Outcomes Year 2

- Research Project Outcomes (Emory)
  - Trisomy X (Health and Reproductive Concerns of Women with Tri X)
  - XXYY (Voices of Patients and Caregivers with XXYY)
  - NIPT Study (Accessible Genetic Counseling with NIPT)
  - Management of puberty in adolescents
    - Autism
    - X & Y Variations
- Healthcare Transitions
  - Autism
  - X & Y Variations
Unexpected Dividends Year 2

- Creation of Southeast Regional Support Group
- Attention of Emory for eXtraordinarY Clinic
- State legislative Awareness
- Documentary Film

Patient-Derived Research Questions

“I want to know what to do when he comes home at 3 o’clock”

• What’s behind this statement?
• Executive function and behavior
• Forming a research question:
  • In boys with 47 XXY does a systematic program within a technology-based companion improve executive function tasks and undesirable behavior compared to boys with 47 XXY not receiving the intervention?
    • Homework, same-day task completion
    • Advanced readiness for next day
    • Self-efficacy
    • Self-esteem
    • Risk for depression
    • QOL child and parent
Initiating Collaborations

• Known Centers & People with Expertise
• Academic Sources
  • Universities
  • Clinics
  • NIH/PCORI
• Specific Areas of Specialty
• Creation of Networks
• Co-PI’ing and subcontracts to co-promote productivity
Thinking about Comparisons Across Groups

- Several diagnostic groups sharing disability concerns
- X & Y Variations with Autism, Fragile X, other groups
- X & Y Variations across national and international geographic locations
Needed: Transparency of Effort and Co-potentiation of research

- Who wants to play in the sandbox?
- Different approach to outcomes
- Patient-centeredness focuses on useable outcomes
- Finessing the current funding structures to feature patient outcomes and not academic centers or academicians
- Reconciling IMPACT over CLAIMED OWNERSHIP; Different and shifting approach.
Questions?