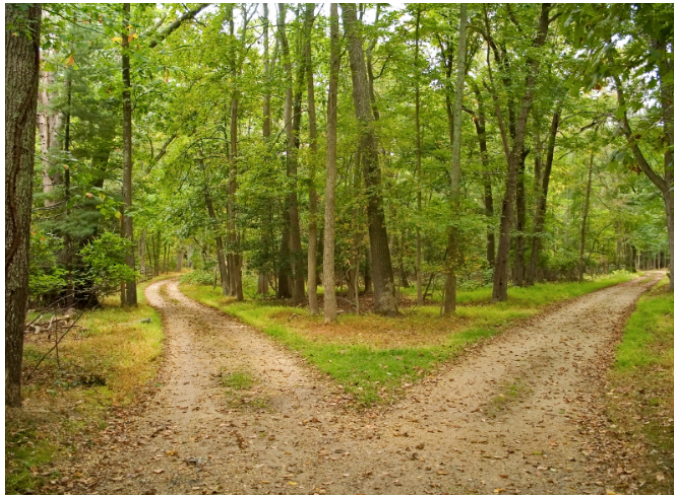
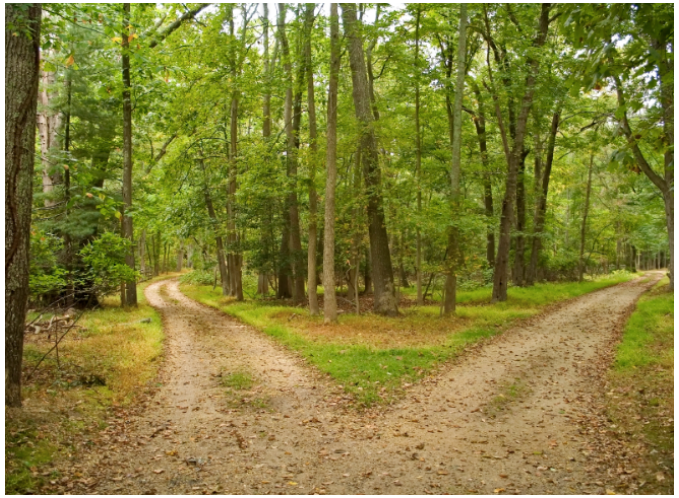
Patient-Centered Approaches to Research Pathways





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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 → **Outputs** → **Outcomes/Impact**

The Investment

- AXYS
- Emory University
- PCORI
- Staff
- Volunteers
- Time
- Money
- Research
- Technology

Activities How & What We do

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

Participants Who We Reach

- Patients
- Families
- Clinicians
- Scientists
- Payers
- Pharma
- Clinical Administration
- AXYS
- Emory University
- PCORI

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 scientitsts
- 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

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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

PCORI Blog Post: http://bit.ly/2sklmOV

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?