Welcome!

Please be seated by 9:40 AM ET

The webinar will go live at 9:45 AM ET

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Prioritizing Comparative Effectiveness Research Questions for the *Management of Sickle Cell Disease*: A Stakeholder Workshop

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March 7th, 2016

Washington, DC

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Romana Hasnain-Wynia, PhD, MS

Director, *Addressing Disparities*, PCORI

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

PCORI Program Director

Romana Hasnain-Wynia, PhD, MS

Director, *Addressing Disparities*, PCORI

Chair

W. Keith Hoots, MD

Director, *Blood Diseases Branch, Division of Blood Diseases and Resources*, NHLBI

Housekeeping

- Today's meeting is open to the public and is being recorded.
  - Members of the public are invited to listen to the teleconference and view the webinar.
  - Meeting materials can be found on the PCORI website
  - Anyone may submit a comment through the webinar chat function, although no public comment period is scheduled.

- Visit www.pcori.org/events for more information.

Housekeeping (cont.)

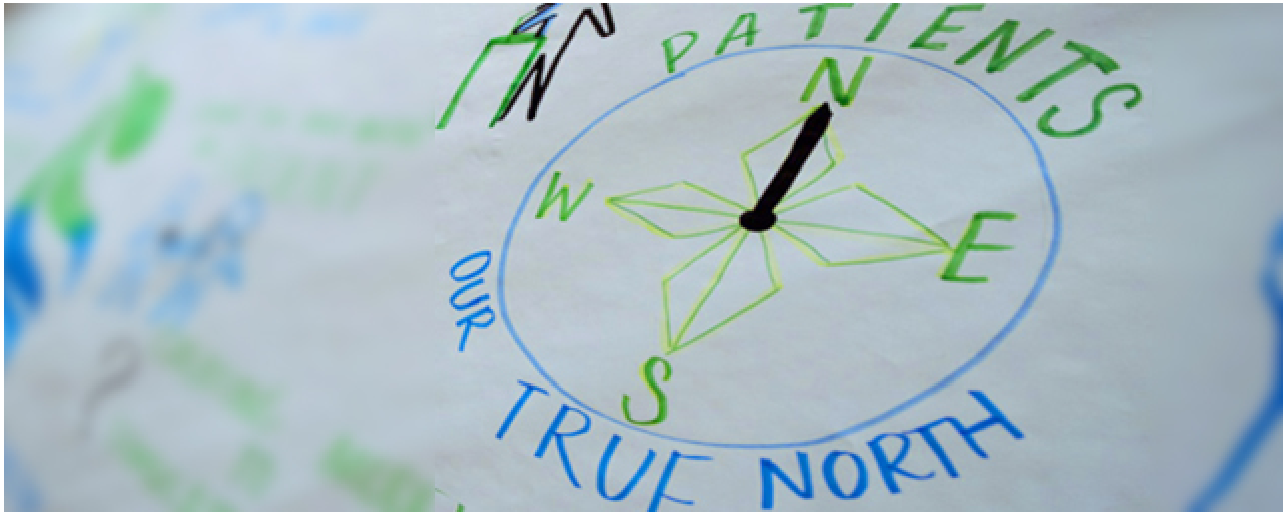
- We ask that workgroup members stand up their tent cards when they would like to speak and use the microphones.
- Please remember to state your name when you speak.
- Where possible, we encourage you to avoid acronyms in your discussion of these topics.

Agenda

Agenda Item	Group	Time	
Introductions and Setting Stage	All Panel	9:45 – 10:45 AM	60 minutes
Break into Groups	Pain Management and Care Transitions	10:45 – 11:00 AM	15 minutes
Discussion of Question Fit	Pain Management and Care Transitions	11:00 AM – 12:15 PM	75 minutes
Break for Lunch and Initial Prioritization	All Panel	12:15 – 1:00 PM	45 minutes
Refinement of Top 2-3 Questions	Pain Management and Care Transitions	1:00 – 2:30 PM	90 minutes
Break and Reconvene	All Panel	2:30 – 2:45 PM	15 minutes
Consensus		2:45 – 4:00 PM	75 minutes
Closing Remarks		4:00 – 4:15 PM	15 minutes

Introductions

- Please quickly state the following:
  - Name
  - Stakeholder group you represent
  - Position title and organization



Purpose of the Workshop

- **Romana Hasnain-Wynia, PhD, MS**

PCORI’s Mission and Vision

Mission

The Patient-Centered Outcomes Research Institute (PCORI) helps people make informed healthcare decisions, and improves healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community.

Vision

Patients and the public have the information they need to make decisions that reflect their desired health outcomes.

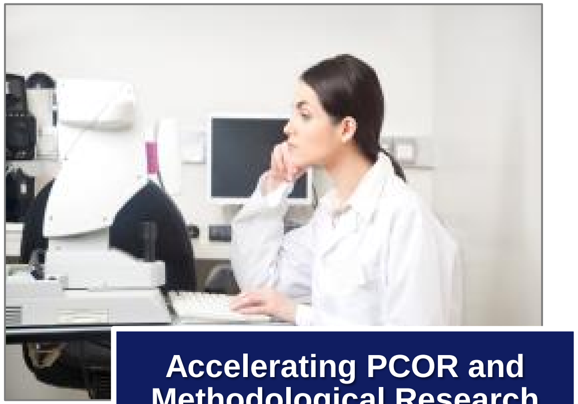
Our National Priorities for Research

Assessment of Prevention, Diagnosis, and Treatment Options

Improving Healthcare Systems

Communication & Dissemination Research

Addressing Disparities



Accelerating PCOR and Methodological Research

Addressing Disparities Mission Statement

PCORI’s
Vision, Mission, Strategic Plan

Program’s Mission Statement

To **reduce disparities** in healthcare outcomes and **advance equity** in health and healthcare

Program’s Guiding Principle

To support comparative effectiveness research that will identify best options for reducing and **eliminating disparities**

Addressing Disparities (AD): Program Goals

Identify Research Questions

- **Identify** high-priority **research questions** relevant to reducing and eliminating disparities in healthcare outcomes

Fund Research

- **Fund** comparative effectiveness **research** with the highest potential to reduce and eliminate healthcare disparities

Disseminate Promising/Best Practices

- **Disseminate** and facilitate the adoption of **promising/best practices** to reduce and eliminate healthcare disparities

Improving Healthcare Systems (IHS) Program: Mission and Goals

- IHS supported studies aim to optimize the quality, patient-centered outcomes, and/or efficiency of patient care and that have the greatest potential for sustained impact and replication within and across healthcare systems.

- Healthcare Systems patient-centered outcomes research (PCOR) compares healthcare system interventions that may include, but are not limited to:
  - Innovative Technologies
  - Personnel Structures
  - Organizational models and policies within and across healthcare systems
  - Patient and provider incentives
    - *only non-financial provider incentives are of interest*

Communication and Dissemination Research (CDR): Mission and Goals

Producing information is not enough.

- Clear communication approaches and active dissemination of findings to all audiences, in easy to understand formats, are critical to increasing the awareness, consideration, adoption, and use of research by patients, caregivers, and healthcare providers
- In other words, information itself is of little use unless:
    - It reaches those who need it
    - It is clear and comprehensible
- Focus on **CER** in the following three key areas:

1. **Communication strategies** to promote the use of health and healthcare CER evidence by patients and clinicians
2. **Dissemination strategies** to promote the use of health and healthcare CER evidence by patients and clinicians
3. **Explaining uncertain health and healthcare CER evidence** to patients and clinicians

Comparative Effectiveness Research (CER): Mission and Goals

- **Mission for CER:**
  - To develop evidence and inform clinical decision-making about diagnosis, prevention, or treatment through funding high quality studies that compare the clinical effectiveness, benefits, and harms of different options.

- Encourages clinical comparative effectiveness studies that are done in typical clinical settings and patient populations (vs. highly selective and specialized research conditions) and are readily applicable and generalizable to daily clinical practices and decision-making.

- Interventions that may be compared for diagnosis, treatment, or palliation include:
  - Surgical treatments
  - Medications
  - Medical devices
  - Behavioral interventions
  - Clinical management strategies

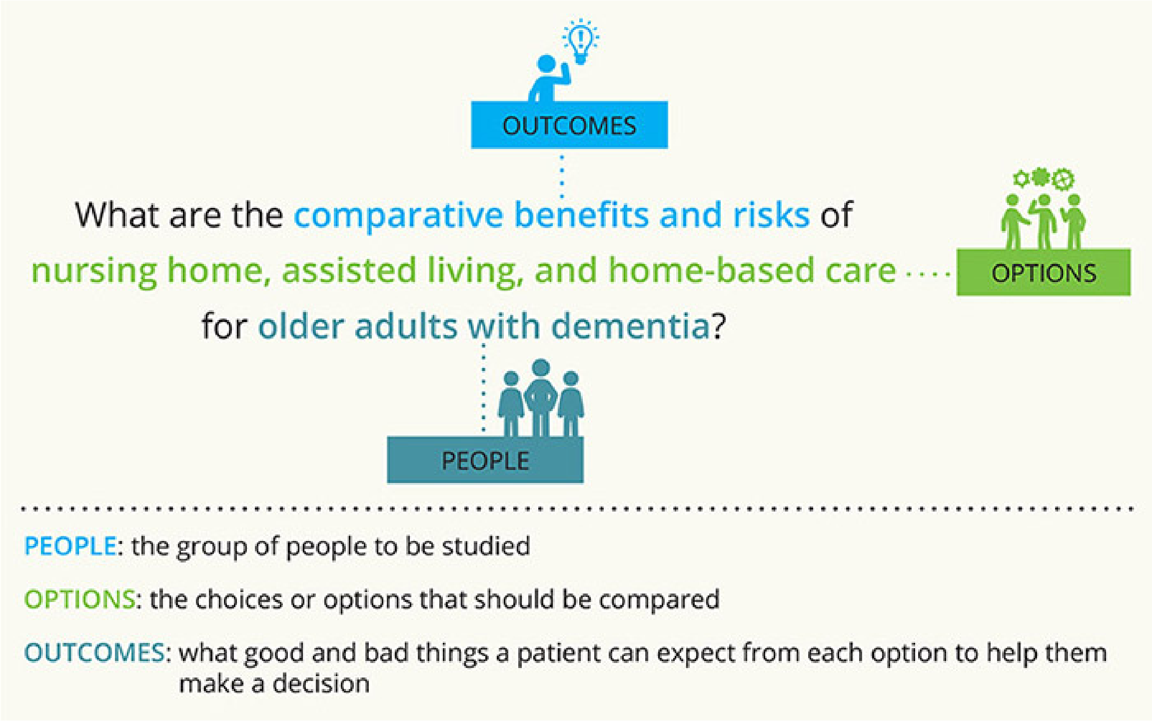
PCORnet: Mission and Goals

- **Vision for PCORnet:**
  - PCORnet will enable research that can be conducted with greater speed, accuracy and relevance within real-world care delivery systems and improve patient outcomes.

- **Overall Objectives of PCORnet: Achieving a Functional Research Network**
  - **Create** a secure national research framework that will enable teams of health researchers, patients, and their partners to work together on researching questions of shared interest.
  - **Utilize** multiple rich data sources to support research, such as electronic health records, insurance claims data, and data reported directly by patients.
  - **Engage** patients, clinicians and health system leaders throughout the research cycle from idea generation to implementation.
  - **Support** observational and interventional research studies that compare how well different treatment options work for different people.
  - **Enable** external partners to collaborate with PCORI-funded networks.
  - **Sustain** PCORnet resources for a range of research activities supported by PCORI and other sponsors.

Purpose of the Workshop

- Identify, refine, and prioritize 2-3 clinical comparative effectiveness research questions on the **Management of Sickle Cell Disease** whose findings could improve patient-centered outcomes.



What are the comparative benefits and risks of nursing home, assisted living, and home-based care for older adults with dementia?

PEOPLE: the group of people to be studied

OPTIONS: the choices or options that should be compared

OUTCOMES: what good and bad things a patient can expect from each option to help them make a decision

PCORI’s Process for Identifying Research Topics and Gaps

Topics come from multiple sources

- Board topics
- Workgroups, roundtables
- 1:1 interactions with stakeholders
- Guidelines development, evidence syntheses
- Website, staff, Advisory Panel suggestions

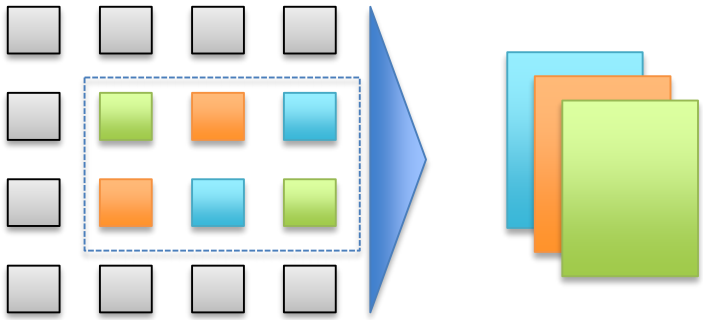
Gap confirmation

(PCORI staff in collaboration with stakeholders)

- Eliminating non-comparative questions
- Aggregating similar questions
- Assessing research gaps
- Preparing topic briefs

Priority topics/ questions

(Multi-stakeholder Advisory Panels and Workgroups)



Topics/Questions proposed for further consideration

Building on PCORI's Investment in Sickle Cell Disease

- **Three** CDRNs in PCORnet that are developing a rare disease cohort specific to sickle cell disease (SCD)
- PCORI has invested over **$8.1 million**, across **5 projects**, in SCD related research
  - **Three** Broad awards
    - 2 in Assessment of Prevention, Diagnosis and Treatment Options
    - 1 in Improving Healthcare Systems
  - **One** Pipeline to Proposal
  - **One** Engagement award

- Due to the cross-cutting nature of the topic, this presents an opportunity for collaboration across ***all*** PCORI programs.

Setting the Stage

Parag Aggarwal, PhD
Senior Program Officer, Addressing Disparities

W. Keith Hoots, MD
Director, *Blood Diseases Branch, Division of Blood Diseases and Resources*, NHLBI

Topic Overview

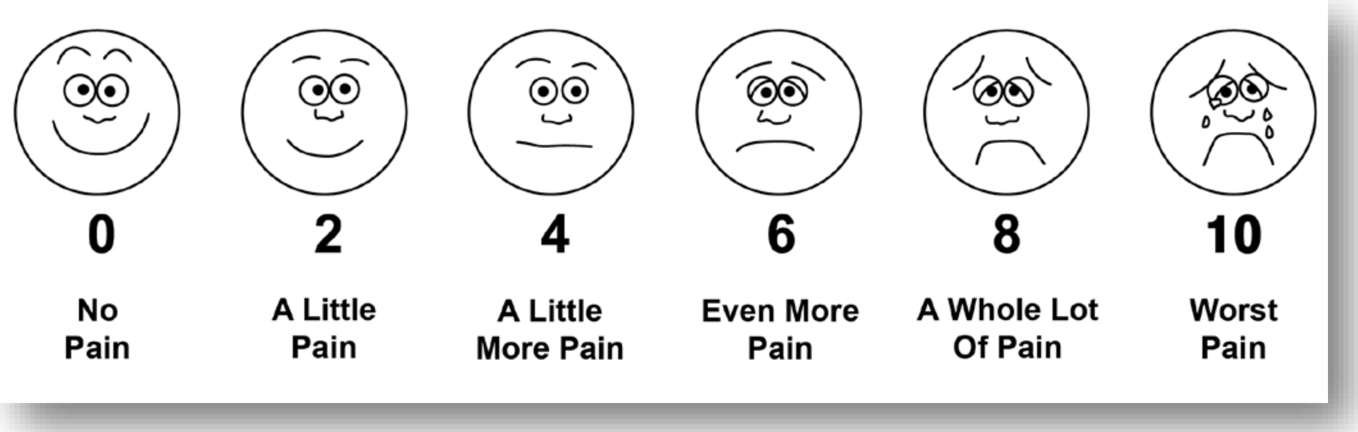
- **Parag Aggarwal, PhD**

Topic Overview

- Sickle cell disease (SCD) is a chronic genetic disorder affecting the body’s red blood cells.
- It is estimated that between 70,000 and 100,000 Americans, predominately African Americans, have SCD.
- This disorder induces a series of disease-related complications, such as acute chest syndrome, ***pain crises***, and stroke.
- These patients are also prone to lack of care coordination and difficulties when ***transitioning from childhood to adulthood***.
- Currently, practices for the treatment of SCD are being used with limited evidence, leaving health care professionals and patients with little information to make informed health care decisions regarding treatment.

Pain Management and SCD

- Nearly all individuals with SCD will suffer from an acute pain crisis in their lifetime.
- The management of pain is central to the care of SCD; however, it is **inadequately addressed** across all types of healthcare settings
- Lack of reliable guidelines and stigma associated with pain medications have left both patients and physicians dissatisfied with the quality of pain management.



Care Transitions and SCD

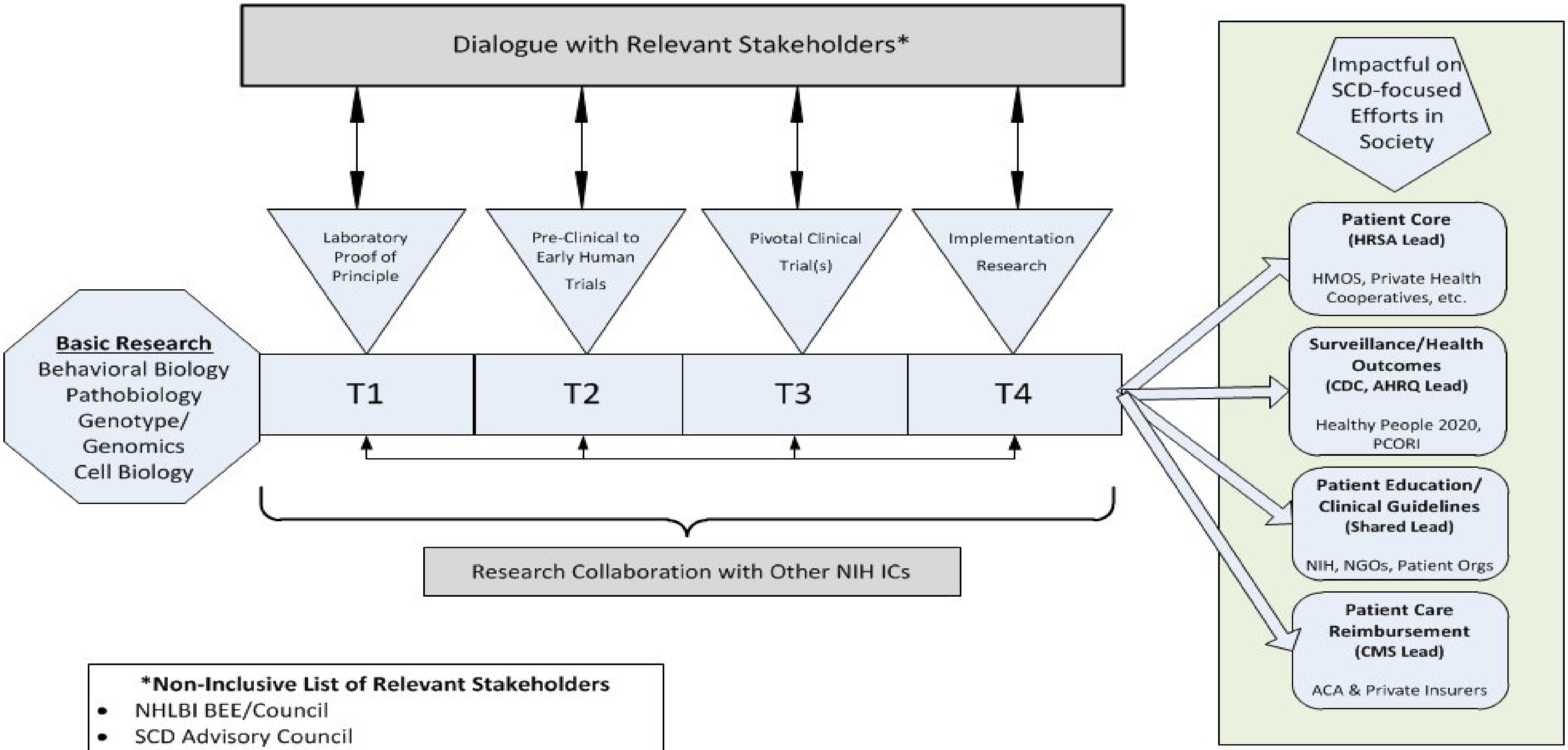
- SCD-related mortality rates are highest among young adults transitioning from pediatric to adult care.
- The guidelines available for facilitating this transition are based on **weak evidence** and/or **consensus-based opinion**.
- These gaps need to be addressed, as clinicians and patients are seeking guidance about treatment options to inform decision-making to improve outcomes.

Stakeholder Investment in SCD –
National Heart, Lung, and Blood Institute (NHLBI)

- W. Keith Hoots, MD

Expanded Model to Guide NHLBI Approach

NHLBI Research Framework to Improve the Lives of People with SCD



Dialogue with Relevant Stakeholders*

Laboratory Proof of Principle

Pre-Clinical to Early Human Trials

Pivotal Clinical Trial(s)

Implementation Research

Basic Research
Behavioral Biology
Pathobiology
Genotype/
Genomics
Cell Biology

T1

T2

T3

T4

Research Collaboration with Other NIH ICs

Impactful on SCD-focused Efforts in Society

Patient Core
(HRSA Lead)
HMOS, Private Health Cooperatives, etc.

Surveillance/Health Outcomes
(CDC, AHRQ Lead)
Healthy People 2020, PCORI

Patient Education/ Clinical Guidelines
(Shared Lead)
NIH, NGOs, Patient Orgs

Patient Care Reimbursement
(CMS Lead)
ACA & Private Insurers

***Non-Inclusive List of Relevant Stakeholders**

- NHLBI BEE/Council
- SCD Advisory Council
- Trans-NIH Collaborations
- NGOs (e.g., ASH, AHA, ATS, etc.)
- Patient Constituency Groups (e.g., SCDAA)
- Government (Federal, State, Local) Partners

T4 Research – What Is It?

- (T1,2) T3 tests WHAT interventions work

- T4 tests HOW to deliver them in real world setting

NHLBI Research Effort Funding Announcement

- NHLBI released a funding announcement entitled “**Using Implementation Science to Optimize Care of Adolescents and Adults with Sickle Cell Disease**” in July 2015
- The goal of this initiative is to focus on improving the quality of care for individuals with SCD
- Awards to seven geographically diverse sites will be made in the first quarter of 2016

NIH
National Heart, Lung, and Blood Institute

Creating a Consortium from a “Neighborhood” of SCD Patients and Providers:

A Requisite for this Implementation Research Initiative

- A Consortium will consist of a team of all providers (community,-based, acute care, and academic centers) that are responsible for the care of adolescents/adults with SCD in a geographic area defined by the investigative team and their collaborators

- Institutions comprising a consortium must collectively enroll at least 300 adolescents/adults (age 15-45)with SCD for prospective longitudinal follow up and enrollment in an implementation research project developed by the team in collaboration with NIH

Question Refinement Process

- **W. Keith Hoots, MD**

Categorization of Submitted Questions

- 59 Questions Submitted
- Duplicates Combined
- Non-Workshop Specific Questions Removed
- Questions Consolidated into Themes
- 10 Themes to be Discussed

Question Refinement Process

- **Step 1: Discuss the questions submitted by the group**
  - Identification of populations, interventions, comparators, outcomes, timing and settings
  - PCORI Criteria
- **Step 2: Rank the themes in order of priority**
- **Step 3: Refine the top 2-3 research questions/themes**
  - Expanded discussion of specific populations of interest, health decisions, and treatments
  - Consideration of study design, challenges to conducting research on specific question, and ongoing work in the field
- **Step 4: Consensus**

Breakout Groups

Topic	Moderator	Room
Pain Management	Dr. Harvey Luksenburg	Victory
Care Transitions	Dr. W. Keith Hoots	Valor

Please listen in to one of our breakout sessions

Pain Management**:** Dial-in number: 1-866-640-4044
Participant Code: 783315

Care Transitions: Dial-in number: 1-866-640-4044
Participant Code: 134255

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Step 4: Consensus

- **Top 3 Refined Questions from *Pain Management* Breakout**

1. For adolescents or adults with SCD, what self-efficacy and/or care models would result in outcomes related to improved functionality, patient satisfaction, school/work attendance, reduction in admission to ER/hospitals, and reduced pain, outside of the health care setting?
2. For adolescents or adults with SCD, what are the comparative effectiveness benefits and risks of various standardized vs. individual pain plans, related to outcomes to improve pain relief, patient satisfaction, reduce stress and conflict, etc., inside the healthcare setting?
3. For adolescents or adults with SCD, what are the comparative effectiveness benefits and risks of various provider education plans and speed of care, related to outcomes to improve pain relief, patient satisfaction, reduce stress and conflict, etc., inside the healthcare setting?

Step 4: Consensus

Top 3 Refined Questions from *Care Transitions* Breakout

1. What is the comparative effectiveness of a transition model that links a multidisciplinary SCD expert team and primary care clinician (e.g. Project ECHO) vs. other transition model/usual/standard of care on satisfaction with care (provider and patient), hospitalization, reliance ratio (ED and ambulatory care) among pediatric patients with SCD?

Step 4: Consensus

2. What is the comparative effectiveness of virtual consultation vs. in-person basic decision support (e.g. specialty consultation vs. EHR decision support or co-located peds/adult care) on increased QOL, increased provider self-efficacy, decrease utilization (ER utilization, hospitalization, ambulatory reliance), missed days from work/school in adolescents with SCD that will transition from pediatric to adult care?

Step 4: Consensus

3. What is the comparative effectiveness of two patient activation models (at least one including a navigator) on patient-reported outcomes and other outcomes of interest to patients in adolescents who transition from peds to adult care?

Closing Remarks

Romana Hasnain-Wynia, PhD, MS

W. Keith Hoots, MD

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Wrap Up and Next Steps

- Meeting summary will be distributed in a few weeks
- Prioritized questions and deliberations from workshop will be shared with PCORI leadership
- PCORI governance will determine next steps

Adjourn

Thank you for your participation!

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