Management of Care Transitions for Emerging Adults with Sickle Cell Disease

Town Hall Webinar
Washington, DC
July 21, 2016 at 2:00pm ET
Agenda

Welcome

About PCORI

Topic Background

PFA Overview

Resources and Q&A

Submit questions via the chat function in Meeting Bridge.

Ask a question via phone (an operator will stand by to take your questions).
Welcome

Romana Hasnain-Wynia, MS, PhD
Program Director
Addressing Disparities

Steven Clauser, PhD, MPA
Program Director
Improving Healthcare Systems
Today’s Presenters

Parag Aggarwal, PhD
Senior Program Officer
*Addressing Disparities*

Gyasi Moscou-Jackson, PhD,
MHS, RN
Program Officer
*Improving Healthcare Systems*
About PCORI
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.

Our Strategic Goals:

- Increase quantity, quality, and timeliness of useful, trustworthy research information available to support health decisions
- Speed the implementation and use of patient-centered outcomes research evidence
- Influence research funded by others to be more patient-centered
Our Focus

Comparative Clinical Effectiveness Research

- Patient-centered
  - Engages patients and key stakeholders throughout the research process
  - Answers questions that matter to patients and other clinical decision makers
  - Compares outcomes that matter to patients
  - Compares two or more evidence-based interventions
Topic Background
Overview: Sickle Cell Disease (SCD)

- SCD is a chronic genetic disorder affecting the body’s red blood cells

- Between 70,000-100,000 Americans, predominantly African Americans, have SCD
  - Early onset disease (5-6 months of age)
  - Average lifespan ranges between 36 and 56 years
  - The emerging adult population (ages 16-25) is particularly vulnerable to worsened health outcomes during the time of transition from pediatric to adult care

- By age 45, SCD patients average ~150 hospital visits, and will have accrued almost $1 million in medical expenses
Emerging Adults (16-25 Years) with SCD

• High rates of **mortality** (7-fold increase relative to other SCD age groups)

• High rates of **comorbid conditions** (e.g., asthma, restrictive lung disease, cardiac dysfunction and renal dysfunction)

• High rates of **hospitalizations and re-hospitalizations**
  – 3.61 hospitalizations per year vs. 1.93 in other SCD age groups
  – 41% re-hospitalized within 30 days vs. 23% in other SCD age groups

• High prevalence of **depression**
  – 26% vs. 9.5% in the general population

• Experience high rates of **school/work absences**
  – Average of 38.4 missed school days/year
Care Transitions in Emerging Adults

• For emerging adults with SCD, transition in care is a life-changing and continuous process
  • Very different from traditional transition models (from hospital to home)
• Quality of care decreases from pediatrics to adult care
  • Challenges with access to specialists (e.g., hematologists)
    • ~60% on Medicaid; limits access to specialists
  • Adult care clinicians report dissatisfaction with the quality of care they can provide
  • Patients report dissatisfaction with quality of care they receive
• Emerging adults become disengaged from the healthcare system
  • Loss of usual source of care
    • Decrease in routine preventative and screening visits (for chronic blood transfusions, hydroxyurea treatments, vaccines)
    • More likely to seek care for acute medical events in emergency department (ED)
      • 5.0 ED visits per year vs. 3.3 in other SCD age groups
Evidence Gaps: Sickle Cell Disease

- Current guidelines are based on weak evidence and/or consensus-based opinion.

- SCD-related complications are highest among emerging adults, but there is a lack of evidence about how to improve the care transition process and outcomes.

- Further research is needed to help to fill gaps to improve care processes and outcomes for individuals with SCD.
  - There are no current CER trials for care transitions for individuals with SCD.
  - Necessary to improve healthcare and health outcomes for vulnerable population when evidence base is weak.
• Stakeholders submitted 59 questions prior to workgroup meeting

• Staff refined and consolidated the questions into two topic areas: *Care Transitions* and *Pain Management*

• By consensus, each breakout group (care transitions and pain management) identified three potential comparative effectiveness questions, for a total of six potential questions

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PFA Overview
Targeted PFA Goal

The goal of the proposed targeted PFA is to generate evidence to:

- Support care transitions from pediatric to adult health care providers for emerging adults with sickle cell disease (SCD)
Proposed Research Question & Study Details

• **Research Question:** What is the comparative effectiveness of established transition coordination models for emerging adults with SCD transitioning from pediatric to adult care?

• **Population:** Emerging adults (e.g., 16-25 years of age) with SCD
  • SCD patients typically transition from pediatric to adult care between 16-18 years of age (timing varies based on needs and readiness)
  • Pediatricians may continue to see patients through college
  • By 26 years of age, emerging adults are no longer covered by their parents’ insurance
    • Interest in older age (up to 30 years of age) range to assess issues related to insurance transitions for emerging adults
Interventions and Comparators:

- Interventions must incorporate patients, care givers, and clinicians
- Interventions should be patient-facing, with robust patient engagement
- Direct comparisons of efficacious or commonly used transition coordination interventions
  - Examples could include (but are not limited to):
    - Co-located pediatric and adult care providers;
    - Clinic-based transition coordinator;
    - Virtual consultation (telehealth) with provider or specialist;
    - Use of mHealth (e.g., mobile apps, text messaging)
  - An appropriate comparator may be usual care or standard of care
- Evidence of efficacy in other diseases (e.g., diabetes, cystic fibrosis, congenital heart disease) and transition models may be used
Proposed Research Question & Study Details (cont.)

- **Outcomes:**
  - Health related quality of life (e.g. depression, physical and mental health)
  - Patient activation/self-management
  - Patient satisfaction and experiences of care
  - Social functioning (e.g., missed days from work and school)
  - Number of hospitalizations and number of days hospitalized due to complications (e.g., pain crises, strokes, comorbid conditions)
  - Measures of emergency department use
Proposed Research Question & Study Details (cont.)

- **Study Design**: Cluster RCT with sufficient sample size and/or clusters to power study

- **Setting(s)**: Outpatient settings including primary care practices, patient-centered medical homes, specialty SCD clinics

- **Timing**: Maximum 5 year study

- **Proposed Research Commitment**: Up to 3 studies, $25M (total costs)
Proposed Research Question & Study Details (cont.)

- Estimated number of individuals with SCD, by state
- 15-20% of all SCD patients are 16-25 years of age (15,000-20,000 total)
- Applicants are encouraged to leverage existing SCD networks, cohorts, consortia, (e.g. NHBLI, PCORnet) to the extent possible
Research Activities Not Supported

• The Management of Care Transitions for Emerging Adults with Sickle Cell Disease PFA will **NOT** support the following types of studies:
  • Pilot studies
  • Efficacy trials
  • Cost-effectiveness analyses
  • Direct comparisons of the costs of care between two or more alternative approaches
  • Development of clinical prediction or prognostication tools
  • Evaluation of new or existing decision-support tools
  • Studies of the natural history of disease, instrument development, pharmacodynamics, and fundamental science of biological mechanisms
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Resources and Q&A
Tips for Success – Administrative

• Adhere to the PFA and Application Guidelines for the **funding cycle** you are applying to (Cycle 3, 2016)
• Talk to a Program Officer if you have questions
• Start and submit early
• Download [PCORI’s Online User Manual for Submitting an Online Application](#)
• Ensure that all team members can see the application in the system (check during the LOI stage)
• Inform your AO of your intent to submit
• Submit the completed application before the due date or on it by **5:00 PM ET**
Tips for Success – Programmatic

• Propose a comparative effectiveness study
• Clearly describe comparators for the study
• Document evidence of efficacy/effectiveness for the intervention and comparator(s) and/or demonstrate that they are in widespread use
• Clearly demonstrate the feasibility of the study
  – Show that you have the team to do this and are the right team
  – Define and support your recruitment and retention plan
  – Document that sites are already committed to participating
  – Include realistic timelines for site start-up, IRB approval, and recruitment
• Clearly describe how the study will address sustainability and scalability of practice change for successful trials
Tips for Success – Programmatic (cont.)

• Consider how your project applies to PCORI’s unique merit review criteria around Patient-centeredness and Patient and Stakeholder Engagement
  • What we mean by engagement: http://www.pcori.org/funding-opportunities/what-we-mean-engagement

• Recognize that you must submit a Letter of Intent (LOI)
  • You must be invited, on the basis of the LOI, to submit an application

• PCORI does not fund:
  • Research aimed at developing clinical practice guidelines
  • Cost-effectiveness analysis measuring dollar-cost per quality-adjusted life-year
Applicant Resources

Sickle Cell Disease Pre-announcement:


Stakeholder workshop meeting materials:


May 23, Board of Governors Meeting Slides:

• http://www.pcori.org/sites/default/files/PCORI-Board-Meeting-Presentation-Slides-052316.pdf#page=90

PCORI Topic Brief: Management of Sickle Cell Disease:

Applicant Resources: Where Can I Find Help

- **Visit pcori.org/apply**
  - Application Guidelines
  - FAQs
  - PCORI Online User Manuals
  - Sample Engagement Plans

- **Schedule a Call with a Program Officer**
  - Submit a request at pcori.org/content/research-inquiry
  - Call 202-627-1884 (programmatic inquiries)
  - E-mail sciencequestions@pcori.org

- **Contact our Helpdesk**
  - E-mail pfa@pcori.org
  - Call 202-627-1885 (administrative and technical inquiries)
Please use this time to ask any programmatic and administrative questions you may have about the PFA or LOI submission process.

Ask a question via the Q & A function in Meeting Bridge.

Ask a question via phone (an operator will standby to take your questions).

If we are unable to address your question during this time, e-mail the Helpdesk at pfa@pcori.org.
Thank You!

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