Prioritizing Comparative Effectiveness Research Questions for Management of Sickle Cell Disease: Questions submitted for consideration by workshop participants

Prioritizing Comparative Effectiveness Research Questions: PCORI Stakeholder Workshops

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1. What are the comparative benefits and risks of a certified peer support specialist, social worker, and nurse navigator on the transition from childhood to adult care for sickle cell disease?
2. What are the comparative benefits and risks of a sickle cell ACA Medicaid Health Home model of care and a hospital-based sickle cell clinic in transitioning from adolescent to adult care for people with sickle cell disease? NOTE: Sickle Cell Disease was approved as a chronic condition for an ACA Health Home in Alabama, based on information indicating individuals with only this disease were at risk of a second condition.
3. Will transition programming that focuses on establishing a medical home (primary care driven) be more effective in reducing acute care utilization and access to disease modifying therapy than programming focused on specialty hematology care?
4. Upon discharge home from the emergency department, what is the comparative effectiveness of different referral strategies to improve quality of life indicators (ASCQ_Me measures), in persons with SCD?
5. Can community health workers improve continuity and care transitions for sickle cell, esp patients heavily affected by social determinants of health?
    a. People – Sickle cell patients who might develop pain
    b. Options –
        i. Optimize use of hydroxyurea therapy
        ii. Better oral pain meds at home
        iii. Usual care – no community health worker
        iv. Alternatives
            1. Telemedicine
            2. Strong partnership with PCP
    c. Outcomes –
        i. Good pain control that allows the pt to go home (avoid hospital admissions for pain management)
        ii. Reduce stress and conflict in obtaining pain treatment
6. Which method most frequently results is a “successful transition”* from pediatric- to adult-focused care in adolescents and young adults with sickle cell disease:
    a. A structured transition program initiated, at the latest, by 13 years of age* without the participation of the adult provider team and community-based organization
    b. A structured transition program initiated, at the latest, by 13 years of age with the participation of the adult provider team
    c. A structured transition program initiated, at the latest, by 13 years of age with the participation of a community-based organization
    d. A structured transition program initiated, at the latest, by 13 years of age with the participation of the adult provider team and community-based organization
    e. *A “successful transition” must include patient satisfaction components and quality of life measures as well as health management outcomes such as more than one outpatient visit to the new sickle cell specialist in a calendar year. In addition, one should consider including acute care visits such as ED or day hospital visits, one complication screening visit such as an annual ophthalmology visit and at least one vaccine
7. It has been previously identified that adult patients are transitioned to primary care providers who lack the specialized knowledge to provide adequate and patient centered care. What is the comparative effectiveness of

different educational mechanisms such as guidelines, multimedia presentations or publications that can be utilized to train primary care providers in pain management of sickle cell patients?

8. Will establishing regional clusters of medical neighborhoods for SCD adults that link a PCP with a hematologist with additional practice support versus traditional provider CME/written education material versus virtual peer mentoring via "Extension for Community Outcomes ECHO" for both primary care providers and adult hematologists be more effective in increasing access to adults with SCD and yield reduced acute care utilization and increased access to disease modifying therapies?
9. What is the comparative effectiveness of a standardized questionnaire or guideline to address transition from pediatric care to adult care? Should guidelines be developed to address patient, caregiver, and provider readiness, not limited to but including patient readiness, family dynamics and support, social issues including financial feasibility, and provider acceptance?
10. What is the comparative effectiveness of patient navigation versus mobile health coaching app interventions on successful engagement in care for individuals with sickle cell disease transitioning from pediatric to adult care?
11. Among young adults with sickle cell disease who will transition from pediatric to adult care, would peer navigators compared to text messaging/apps improve disease self-management (e.g. knowledge of disease, health related quality of life) and improve health promoting behaviors (e.g. attendance at outpatient visits, communication with providers, and medication adherence)?
12. Compare patient activation and engagement models on improving transition of care for patients living with sickle cell disease.
13. What is the comparative effectiveness of video games versus one-on-one health coaching on successful engagement in care for individuals with sickle cell disease transitioning from pediatric to adult care?
14. What are the comparative benefits and risks of patient navigators, technology-enhanced patient-centered programs, and patient-provider learning networks on self-management in children with sickle cell disease preparing to transition from pediatric to adult care?
15. Among young adults with sickle cell disease who will transition from pediatric to adult care, would group visits compared to individualized patient visits: 1) improve knowledge about hydroxyurea and management of acute symptoms of their disease; 2) improve medication adherence; and 3) decrease emergency department utilization?
16. What are the comparative benefits and risks for care continuity from utilizing electronic self-recorded pain diary records as a communication aid with the adolescent SCD population transitioning to adult care?
17. Among adolescents with sickle cell disease, would a disease specific transition readiness curriculum (e.g., Sickle Cell Disease Treatment Demonstration Program Transition Curriculum) compared to a general transition readiness curriculum (e.g., Got Transition) enhance self- efficacy to manage symptoms of their disease and improve health promoting behaviors (e.g. attendance at outpatient visits, communication with providers, and medication adherence)
18. In patients with sickle cell disease and avascular necrosis, does joint replacement surgery result in decreased pain and improved functioning compared to surgical conserving techniques?
19. What treatment options are available to patients living with Sickle Cell Disease to increase blood flow to bones to reduce the instances of avascular necrosis and eliminate the need for joint replacements?
20. What is the comparative effectiveness of interventions to reduce ischemia organ injury as sequela of acute (painful) vaso-occlusive episodes with SCD?

21. In adolescents and adults with sickle cell disease, do individualized pain plans for acute pain crisis jointly developed by patient and provider compared to standard order sets/algorithms implemented in the emergency department, lead to more timely administration of pain medication, improvement in pain (e.g. decrease in pain score), and decreased hospital admissions due to acute pain crisis.
22. In people with sickle cell disease, do individualized home pain plans compared to cognitive behavior therapy including mindfulness interventions lead to improved coping, decreased missed days from school or work and decreased emergency department visits for acute pain crisis?
23. What are the impacts on pain control and vaco-occlusive crises resolution from utilization of smart phone (tracking and communication) application(s) for children experiencing painful episodes with SCD?
24. What are the comparative benefits and risks of chronic use of short-acting opioids, and combinations of slow-release opioids in the ambulatory management of pain in children and adults with SCD?
25. What are the comparative benefits and risks of transdermal buprenorphine (an opioid partial agonist) and full opioid agonists in the treatment of individuals with acute painful sickle cell crisis?
26. Is intravenous fluid superior than oral fluid for improving patient outcomes (decreased pain, decreased need for opioids, decreased need for admission) for patients with sickle cell disease in an acute pain crisis?
27. What is the comparative effectiveness of opioid versus non-opioid intravenous analgesic in the treatment of acute sickle cell pain crisis?
28. What quality of care and drug safety parameters should be assessed to identify the benefits of opioid versus non-opioid pain medications in this setting?
29. What is the comparative effectiveness of a variety of pharmacologic and non-pharmacologic interventions for the treatment of vaso-occlusive crisis in the emergency department setting for patients with SCD?
30. Does the implementation of non-pharmacologic measures such as Art Therapy for patients with Sickle Cell Disease admitted with acute VOC increase satisfaction with pain management and time to acceptable analgesia as measured by pain scales and decrease the quantity of opioids used throughout the hospitalization as well as length of stay compared to management with opioid therapy alone?
31. What is the comparative effectiveness of cognitive behavioral therapy and non-opioid pain management versus chronic opioid therapy on pain experience for adults with sickle cell disease?
32. Do acute pain management protocols including non-pharmacologic techniques such as heat, water therapy, acupressure and biofeedback result in improved patient satisfaction or lower opioid analgesic equivalent usage when compared to protocols that do not include non-pharmacologic approaches to pain control?
33. In children with sickle cell disease, what is the impact of hydroxyurea therapy compared to no therapy, on pain, physical functioning, and fatigue?
34. What are the comparative benefits and risks of hydroxyurea and other treatments in adults and children with hemoglobin SC disease?
35. What treatment options are currently available which may be compared to Hydroxyurea to decrease anemia, organ damage, and increase quality of life in patients with Sickle Cell Disease
36. What are the comparative benefits and risks of shared-decision making, personalized patient decision analysis, and patient education on hydroxyurea use in patients with sickle cell disease?
37. What is the comparative effectiveness of high vs. low intensity emergency department initiatives to improve the timeliness of pain management for the treatment of SCD patients with VOC in the ED?
38. Compare standard pain care clinical practice protocols to individual patient pain care plans for treatment in emergency department settings for patients living with sickle cell disease.

39. Can individualized patient care plans accessible to both primary providers and emergency providers improve the management of patients with sickle cell disease, including decreased emergency department utilization and decreased episodes of acute pain crisis?
40. What are the comparative benefits and risks of virtual human technologies provider training, face-to-face provider training, and telementoring provider training on the treatment and management of acute pain crises in patients with sickle cell disease?
41. What are the comparative risks and benefits of pain treatment in emergency departments and day hospital facilities in the management of acute pain in adults and children with SCD?
42. What is the comparative effectiveness of a Sickle Cell Day Unit and inpatient or observation hospital treatment for people with an acute painful sickle cell crisis?
43. What is the best way to manage pain in an ED that does not know the patient? (sickle cell, generalizable to other diseases with severe acute pain)
    a. People - sickle cell patients with pain
    b. Options –
        i. Personalized pain mgt passport in ED
        ii. Usual care - ED - pain management ad hoc or wt-based, care is delayed when crowding occurs
        iii. ED withholds opioids due to concern from drug abuse
        iv. Alternative sites
            1. Specialty unit for sickle cell pain management
            2. Observation unit for extended management
            3. Home care IV treatment
    c. Outcomes
        i. Good pain control that allows the pt to go home (avoid hospital admissions for pain management)
        ii. Reduce stress and conflict in obtaining pain treatment
44. How can pain be managed with a minimum of hospital readmissions?
    a. People – Sickle cell patients hospitalized for pain
    b. Options –
        i. Blood transfusion as part of the mgt of uncomplicated sickle cell pain
        ii. Community health worker care coordinator
        iii. Usual care – very minimal use of transfusions, no community health worker
        iv. Alternatives
            1. Maximal use of hydroxyurea
            2. Day hospital alternative to hospital
    c. Outcomes
        i. Good pain control that allows the pt to go home (avoid hospital admissions for pain management)
        ii. Reduce stress and conflict in obtaining pain treatment
45. Will early simple blood transfusion or Community health workers improve pain control, as measured by hospital readmissions for pain?
    a. People – sickle cell patients hospitalized for pain
    b. Options –
        i. Blood transfusion as part of the mgt of uncomplicated pain
        ii. Community health worker care coordinator

iii. Usual care – very minimal use of transfusions, no CHW
iv. Alternatives
1. Maximal use of hydroxyurea
2. Day hospital alternative to hospital

c. Outcomes –
i. Good pain control that allows pt to go home (avoid hospital admission)
ii. Reduce stress and conflict in obtaining pain treatment

46. Does a health care model provided by a multidisciplinary sickle cell disease provider team result in decreased re-hospitalizations, fewer acute care visits, and improved health-related quality of life for patients with sickle cell disease compared to a model of care provided by non-sickle cell disease providers?
47. What are viable treatment options to increase immunity in patients living with Sickle Cell disease to increase quality of life and decrease the number of life threatening infections? Are vaccines currently available or can they be developed to eliminate the viruses linked to Acute Chest Syndrome for patients with Sickle Cell Disease?
48. Does adoption of a patient centered medical home with co-management by a hematologist appropriately increase access to specialty care and reduce both acute and chronic SCD related complications?
49. For patients with acute chest syndrome, is the use of fluoroquinolone (e.g., levofloxacin) as effective as the current recommendation of ceftriaxone + azithromycin for decreasing morbidity and length of hospital stay?
50. What is the effectiveness of a provider checklist (based on recent clinical practice guidelines) to improve patient outcomes in the outpatient sickle cell population? I have done a pilot of sorts on this one here at Tulane and had promising results.
51. What is the effectiveness of a patient-specific Emergency Department treatment guideline on increasing patient satisfaction, decreasing time spent in the ED, decreasing inpatient admissions?
52. Will transition programming that focuses on establishing a medical home (primary care driven) be more effective in reducing acute care utilization and access to disease modifying therapy than programming focused on specialty hematology care?
53. What is the comparative effectiveness of short door to initiation of pain medication times in all adults with sickle cell disease in academic and community hospital settings?
54. What is the comparative effectiveness of levels of education and employment have for adolescents or young adults with SCD, with particular emphasis on self-efficacy, self-management and patient-centered outcomes?
55. Would addition of home health nurse visits improve outcomes (decrease hospitalizations and Emergency Room visits) in young adults (18 -25 years) with sickle cell who have recently transitioned to adult care?
56. Does age of transition (18 vs 21 years) of sickle cell patients to adult care lead to better outcomes as measured by decreased ED visits and hospitalizations?
57. What are the comparative risks and benefits of global functional pain management models as an alternative to the classic "chronic/ acute pain" models for adults and children with sickle cell disease?
58. What are the comparative benefits and risks of Patient Navigators and combined Pediatric/Adult provider visits for adolescents and young adults with sickle cell disease who are transitioning from pediatric to adult care settings?
59. What is the comparative effectiveness of the FDA approved iron chelation products used by patients with sickle cell disease who have iron overload? Outcome measures can include standard endpoints (safety and efficacy) for iron chelation as well as compliance.