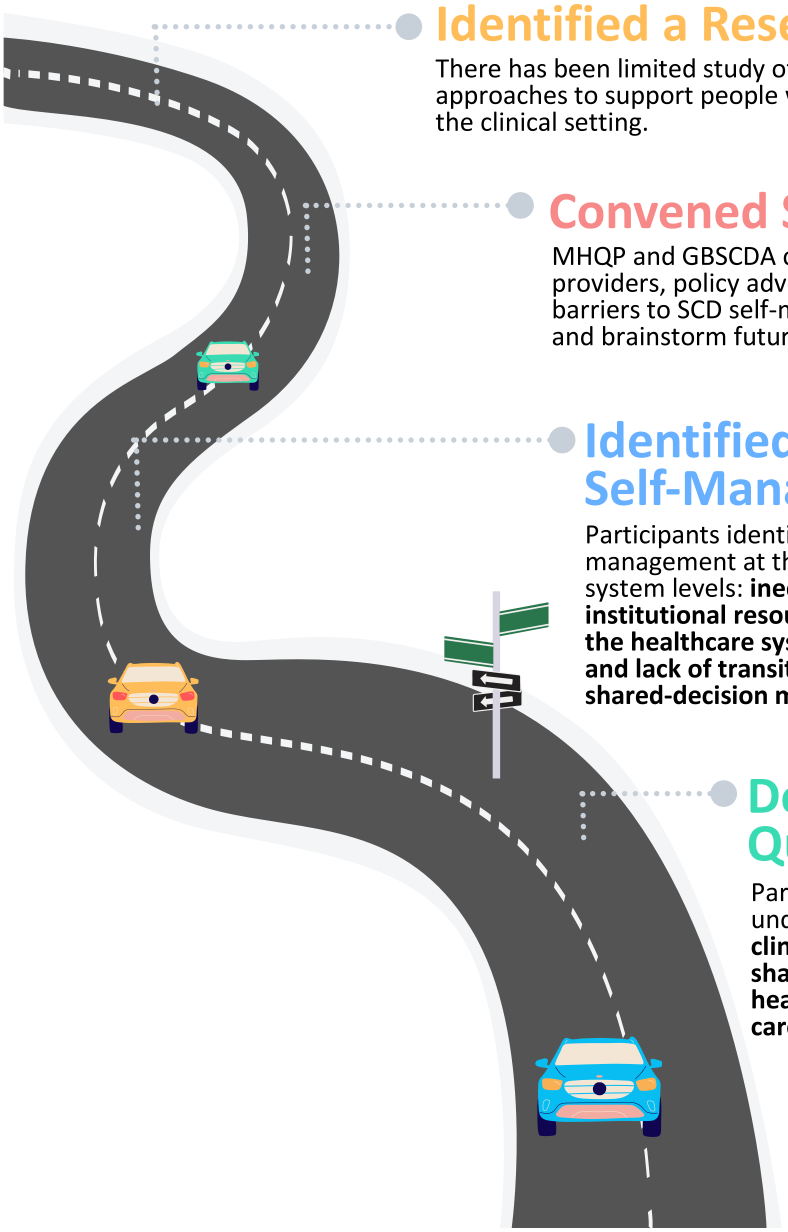
mhqp Massachusetts Health Quality Partners

Greater Boston Sickle Cell Disease Association

Roadmap for Future Research Directions into Sickle Cell Disease Pain Self-Management

Massachusetts Health Quality Partners (MHQP) and the Greater Boston Sickle Cell Disease Association (GBSCDA) came together to develop this roadmap for future patient-centered outcomes research and comparative effectiveness research (PCOR/CER). This roadmap offers stakeholder-identified directions and insights to guide potential research into sickle cell disease (SCD) pain self-management. It is an important first step in addressing this understudied topic through a patient-centered lens. The graphic below is a summary of the process used to identify these research priorities.



Identified a Research Gap

There has been limited study of the most effective, patient-centered approaches to support people with SCD manage their pain outside of the clinical setting.

Convened SCD Stakeholders

MHQP and GBSCDA convened patients with SCD, caregivers, providers, policy advocates, and researchers to discuss barriers to SCD self-management, identify research priorities, and brainstorm future PCOR/CER questions.

Identified Barriers to SCD Pain Self-Management

Participants identified several barriers to SCD pain self-management at the patient, provider/care team, and system levels: **inequities and biases in care, inadequate institutional resources and patient education, mistrust in the healthcare system, patient bandwidth limitations, and lack of transitional care, provider training, and shared-decision making.**

Developed PCOR/CER Questions & Measures

Participants created research questions under the following prioritized topic areas: **clinical care decision-making, care plan, shared decision-making, behavioral health, nutrition, equity, and transitional care.**

Participants also brainstormed patient-, caregiver-, and provider-reported, and clinical outcomes that could be used to measure the effectiveness of health options and interventions that are studied.

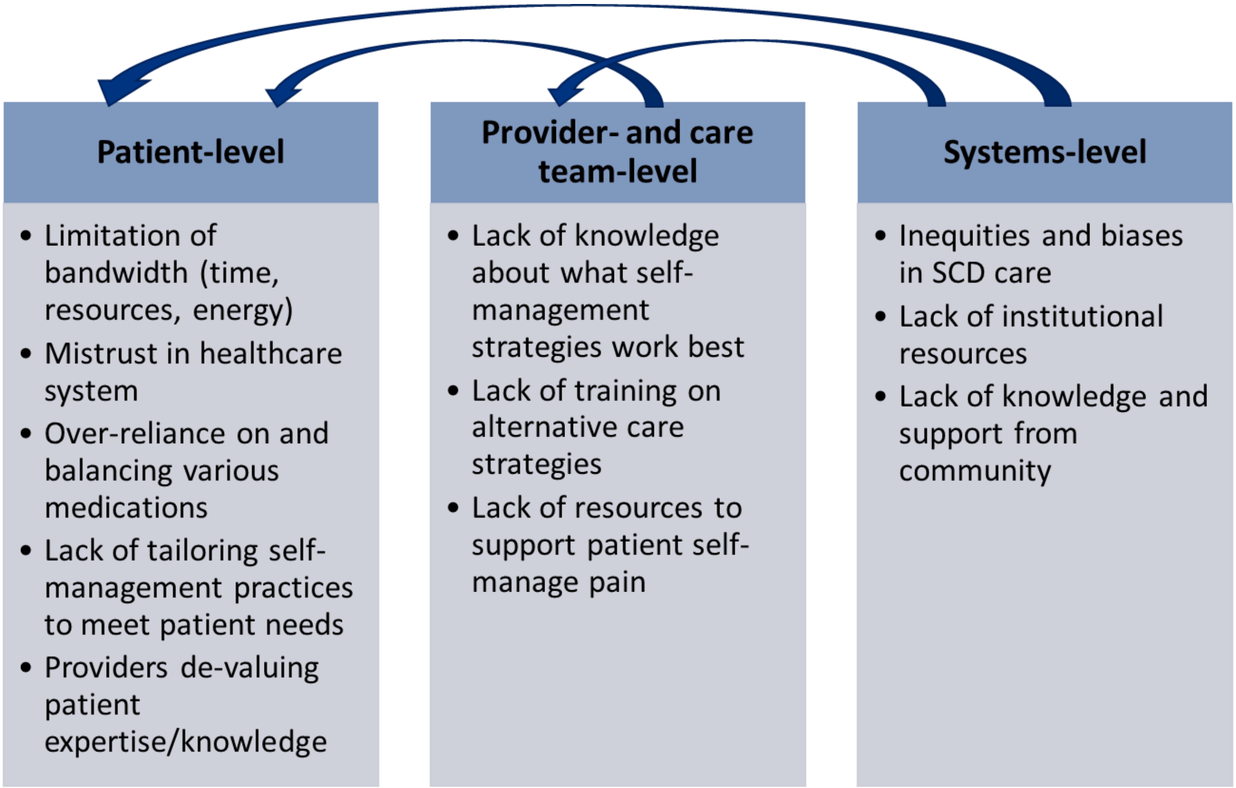
BACKGROUND

Massachusetts Health Quality Partners (MHQP) and the Greater Boston Sickle Cell Disease Association (GBSCDA) held two convenings in April and June of 2022 focused on setting future research directions in SCD pain self-management. The meetings were facilitated by Dr. Lanetta Bronté-Hall, President and CEO of the Foundation for Sickle Cell Disease Research. Eighteen stakeholders – patients, caregivers, providers, researchers, and policy advocates from different parts of the country joined these meetings to set future PCOR/CER directions on the topic.

This project was funded through a Patient-Centered Outcomes Research Institute® (PCORI) Eugene Washington PCORI Engagement Award (EASCS-23286), a program that aims to bring more patients, caregivers, clinicians, and other healthcare stakeholders into the research process. The statements presented in this roadmap are solely the responsibility of the authors and do not necessarily represent the views of PCORI, its Board of Governors, or Methodology Committee.

STAKEHOLDER-IDENTIFIED BARRIERS TO SICKLE CELL DISEASE PAIN SELF-MANAGEMENT

The participants identified several barriers to SCD pain self-management at the patient, provider and care team, and system levels (see Figure 1 below). The arrows in Figure 1 denote that these barriers are interrelated.



Patient-level

- Limitation of bandwidth (time, resources, energy)
- Mistrust in healthcare system
- Over-reliance on and balancing various medications
- Lack of tailoring self-management practices to meet patient needs
- Providers de-valuing patient expertise/knowledge

Provider- and care team-level

- Lack of knowledge about what self-management strategies work best
- Lack of training on alternative care strategies
- Lack of resources to support patient self-manage pain

Systems-level

- Inequities and biases in SCD care
- Lack of institutional resources
- Lack of knowledge and support from community

Figure 1. Stakeholder-Identified Barriers to Sickle Cell Disease Pain Self-Management

POTENTIAL THEMES FOR FUTURE RESEARCH

The participants identified several future research themes on the topic of SCD pain self-management at the patient, care team, and system levels. A summary of the discussion with accompanying quotes from the participants can be found below.

Patient-level

Identifying pain triggers: There are psychological, physical, and environmental pain triggers, and it is important for patients to be able to identify them. It is also important for providers to understand that triggers are not always present prior to a pain crisis.

> *"For my son, he learned it just wasn't worth going into the pool after a while… He learned not to be in the snow for more than 10 or 15 minutes… [But, sometimes] you can't point to say something, you know, this is what I was doing. Sometimes you can, but sometimes you can't. And I think that's when I talk about cultural sensitivity and competency… so that the providers understand that this is not somebody's fault. You know, sometimes maybe, yeah, maybe my son shouldn't have been in the pool for an hour, right?... But sometimes he wasn't doing anything." [caregiver]*

> *"I believe that pain self-management really starts at home, and I think a lot of pain is self-managed way before patients even get to the ER and then just being able to identify the things that… cause or trigger your pain [is important]." [caregiver]*

Identifying when to seek clinical care: Self-care is a key strategy for people with SCD to manage the disease and avoid or mitigate pain episodes; though, it is important for patients to recognize when they should seek clinical care. Patients with SCD often attempt to self-manage their pain to avoid clinical care, because clinical care experiences are often negative. However, waiting too long to seek medical care in certain circumstances can be dangerous from a medical perspective.

> *"I'm just going to go to my ER or I'm going to go to my day hospital because I know I can get something, and I'll be home in a couple of hours. But that's not the case. It's if I go, it's going to be a nightmare. So let me try the Motrin, let me try the Tylenol, let me try the heating pad. None of it works. And you end up where you end up six hours into the pain crisis and it just is worse. So maybe looking at the question of why are we doing this self-management is probably something I think could be discussed as well." [caregiver]*

> *"In terms of patients not wanting to go to the ER, we saw with the pandemic the delay in care. People just did not want to go seek care, and what did we see more of? Strokes, you know, heart attacks where people just weren't being treated timely. But this has actually been going on with sickle cell for some time. So, when patients delay their care, they're also [potentially] increasing the complications. So, we learned so much from the pandemic that I think we can actually utilize when we think about… why are we trying to encourage patients to be better with pain self-management? So, not only do they have to be better with that, they actually have to identify when they're having some real medical, you know, challenges and need to be seen sooner. We don't want them to wait." [provider]*

Provider- and care team-level

Taking a person-centered approach to pain care and shared decision-making: It is important for providers to engage with patients in the decision-making process and understand why patients make certain pain management and care decisions.

> *"For my daughter, as far as her pain being managed, [what works] is when the providers actually listened to what she says works best for her." [caregiver]*

> *"He's working basically from like 5:00 PM to 1:00 or 2:00 in the morning, and then he'll come in for treatment. The doctors kept asking him, you know, what about your pain medications? He was filling not one prescription of the pain medications. His biomarkers are all in the red zone. He is married and has four children. He's just trying to make it work. And he doesn't have time to go to the hospital and be there for days. And he doesn't have time to take the pain medications because they make him too drowsy – he will not be able to work. So, his method was to let me go do my job, and then I'll go to the ER, you know, every day pretty much." [provider]*

Utilizing effective alternative care strategies: Patients and caregivers reported that there is a bias against self-management and alternative care strategies. In the past and in more traditional care models, patients were given pain medications to take at home to remove the need for clinical care; however, this repeated use of opioids has led to many complications, including hyperalgesia and allodynia. Providers noted that they have not been trained to provide alternative/complementary care, nor do they have the resources to direct patients to alternative care options.

> *"[There is] bias against self-management… When you're trying to [get] care from some of the providers, they're turning to… the more traditional approaches. So, [have] you taken [the] Motrin? Or have you taken this [or] that? And I think that puts you in a mindset of going away from some of the other strategies that you could use… [For example,] taking supplements at the time of pain – we don't hear about that. We don't really hear about nutrition. We don't hear about those strategies. And that could help prevent some of the pain issues. The focus on the tradition is one of the barriers to [effective] self-management." [caregiver]*

> *"Providers are not open to alternative [care methods]." [patient]*

> *"When I went to medical school, nobody taught us about some of these things… We need… much more provider education from people who are experts in alternative pain management techniques so we know how to best utilize them. I know some centers have… that but most of us don't have that at our fingertips." [provider]*

Incorporating nutritionists into the care team: There was interest in researching and promoting healthy dietary habits for individuals living with SCD and incorporating a nutritionist as part of the care team.

> *"We don't hear about [nutritional] strategies, and that could help prevent some of the pain issues... I really think we need to look at having a nutritionist as part of the team. I know funding is always an issue… And we come, I mean the majority of folks in America with sickle cell are from Africa, with traditions, that's a big part of our diet." [caregiver]*

> *"In today's fast-moving world… very few people [have] the time to really prepare a meal… And we know… the dangers of continually eating fast food… And also the economics part of it… the frequency of crisis and hospitalization is way less in the… more affluent neighborhoods and it goes back again to the economic aspect of it." [patient]*

Incorporating behavioral health providers into the care team: Patients often suffer from psychological distress due to SCD pain and complication. Ideally, behavioral health should be integrated into care models. It is important to explain to patients how the psychological and physical factors of pain influence one another.

> *"I think the whole psychological component kind of gets lost because that conversation is really not had with the patients. And when we try to have it, they get defensive many times because they do think we're saying they're, you know, drug seekers, not really in pain." [provider]*

> *"One thing that we have tried to do in our multidisciplinary approach to our patients is to bring onto our team a psychologist and a dedicated social worker because of the psychosocial issues that all of us have, but you know, when you add the burden of pain, there are psychosocial stressful triggers to the pain. And this is sort of an adjunct to help into pain management." [provider]*

Creating Care Plans: Providers voiced the importance of creating a Care Plan for patients, to ensure that patients' pain is adequately treated.

> *"Oftentimes [care plans are] the best way to make sure that your pain is adequately treated in the ED and knowing when to kind of escalate into like admission versus other types of medications that the patient might need." [provider]*

> *"Pain is what the patient says it is… You get residents that rotate through… They want to see a biological marker or a lab or something that's going to show them and so we do a lot of education around that… We have care plans that we try to put ranges in there, and those ranges are there for a reason, but we don't frequently even see our ED folks, sometimes, escalating dosages to be able to capture pain better." [provider]*

Implementing programs that support transitional care: Most patients with SCD experience an increase in pain and in disease complications and the need for comprehensive health care as they age, particularly, though not exclusively during the time of transition to adult care. Some institutions have developed Transition Programs to systematically enhance this process, as there is a need for strong programs that not only improve patient education and self-management of SCD, but also treat patients like human-beings.

"It's almost as though we've created a system of orphans… They just feel abandoned and that no one really loves them or listens to them. And so, [we] approach it from, [how] do we really reach the patients and reach their needs because their needs are just so great. And it's almost basic psychological needs related to just the lack of belief in their pain, the lack that they were born with a disease that obviously gets worse over time, not better over time, but the providers, from the patient's perspective, seems to think that somehow miraculously, they should be getting better, should not be on pain medication. And so their behavior… is often very like orphans who have just been neglected and bounced around from home to home." [provider]

"When they left pediatrics, they would [go] to the adult side… They'd go from a very nurturing place where the nurses would come in and say, 'hi, how are you doing? Did your sister have the baby? How is your grandmother doing?' type thing, rubbing their backs… [to] sharing a room with an older individual and the nurse coming in every four hours." [provider]

System-level

Addressing inequities and biases in SCD care: Although SCD chronic and acute pain can be severe, providers sometimes do not understand patients' pain or believe that they need medications to manage their pain. In addition, in the context of the opioid crisis and the regulations that followed, many providers became more hesitant to prescribe pain medications. Pain is subjective, so it is susceptible to bias, which leads many patients with SCD to be labelled as "drug-seeking."

"I've heard if providers see lab work that looks like your hemoglobin is at a certain level, then there will be some resistance to treating pain or believing that there is pain… The opioid crisis in general has made it harder for sickle cell patients over these past four or so years. [patient]

"If someone's frequently in the emergency department, they're like, oh, why are you always here, they must not be in pain they're drug seeking… And then also from like a patient perspective, going to a place where you can normally get care, because if they see that you're like, oh, always going to Brigham, but now you're going to another hospital, people are going to start saying like, oh, maybe… they are trying to work the system, get more pain meds... And then also like emergency physicians as much as they're trying to work on it… they're not as diverse as our patient population… because of that, you also have issues with cultural competency… Labeling people before you even see them as a drug-seeker [further] delays their care." [provider]

Addressing access barriers driven by the high cost of care and lack of institutional resources: The cost of care is a major barrier to receiving high quality and timely care. In addition, institutions often lack the resources to support their patients fully.

"One barrier… for going to get pain managed at the hospital is the cost… It's like, okay, if I go to the emergency room, I have to pay a hundred dollar deductible right off the bat. And then I have to deal with the bill that's going to come, and I do private insurance through my employer and so it's like, okay, I'm going to be stuck with that 20%, and ER visits are not cheap." [patient]

"The resources are lacking at the institutions… I'm in the Baltimore area—I remember talking to a patient and they were raving about, you know, [this] infusion center, but it's not 24/7. So, I asked, so, what do you do? And she, you know, jokingly said, well, I just decided I'm not going to get sick on the weekends, right?" [caregiver]

CONSIDERATIONS FOR PCOR/CER ON SICKLE CELL DISEASE PAIN SELF-MANAGEMENT

To further inform PCOR/CER, participants recommended that qualitative interviews with patients with SCD, their caregivers, and providers be conducted to answer the following questions:

- *How do patients with SCD manage their pain?*
- *What pain outcomes are important to patients and other stakeholders?*
- *How do patients define patient-centered care in SCD?*

FUTURE PCOR/CER QUESTIONS

The participants brainstormed several PCOR/CER questions on the topic of SCD pain self-management (see Table 1). Note that, while some research questions are specific to pain self-management, others address facets of care related to the management of SCD, and therefore, have a role in pain management. In addition, PCOR/CER questions were not created for all potential research themes identified by convening participants.

Table 1. PCOR/CER Research Questions

Topic	*Research Questions*
Clinical Care Decision-Making	• *What does a better job of helping patients with SCD determine when to seek clinical care for their pain, a decision-making aid handout, verbal instruction, an automated hotline, communicating with a hematologist on call, or communicating with a hematologist via chat?* • *Does the use of a device application (e.g., Living Well with Sickle Cell or Sickle Cell Disease Warrior) or the usual standard of care, work better in helping patients determine when to seek clinical care for their pain?* • *Does a system-driven algorithm or patient education, work better in helping patients determine when to seek clinical care for their pain?*
Care Plan	• *Does having a care plan, versus not having a care plan, help people with SCD who present with pain in an inpatient setting manage pain?* • *Does receiving a care plan during a clinic visit, versus during a clinic visit plus at a 6-month follow-up clinic visit, help patients with SCD manage their pain?*
Shared Decision-Making	• *What does a better job of supporting shared decision-making about medications during healthcare visits, using a shared decision-making aid plus provider-facing training materials or a patient-facing video plus question prompt card?* ○ *Medication examples: opioids, Adakevo, Oxbryta, Endari, Hydroxyurea* • *Does a shared decision-making aid or pain education do a better job of helping patients with SCD manage their pain?*
Behavioral Health	• *Does having a social worker embedded in the care team, versus not having a social worker embedded in the care team, help patients with SCD reduce depression and anxiety?* • *Does completing a behavioral health assessment help patients with sickle cell reduce depression and anxiety?*

Topic	*Research Questions*
Nutrition	- *Does having a nutritionist on the care team, versus not having a nutritionist on the care team, help individuals with SCD eat healthier?* - *What does a better job of helping individuals with SCD eat healthier, being part of a virtual nutrition support group or having a nutritionist on the care team?*
Equity	- *Does a restorative justice response to negative healthcare experiences, versus not having a restorative justice response, improve the care experience for patients, caregivers, and providers?*
Transitional Care	- *Do integrated pediatric/adult facilities or non-integrated facilities do a better job helping individuals with SCD manage their pain?* - *Does partnering with a community-based organization (triad partnership: patient, clinician, and community-based organization), compared with usual source of care, help individuals with SCD transition from pediatric to adult care?*

OUTCOMES TO MEASURE

The group identified several outcomes to measure to quantify the effectiveness of the health interventions and options proposed in Table 1 (see Table 2 below).

Table 2. Outcomes to Measure

Outcome Type	*Outcomes*
Patient-reported	- Pain levels: day-to-day pain, how much pain interferes with daily activities, pain intensity - Physical health/status: physical function, fatigue, sleep disturbance, nutrition - Medication-specific: time to wait for pain medicine, how likely patients were to have their pain re-checked after receiving medicine, medication adherence, knowledge of medication effects (use, risks, benefits), medication use outside of the clinical setting, satisfaction with medication decisions - Treatment decisions: knowledge of treatment options, decisional conflict, decisional preparedness, decisional regret - Patient experience: satisfaction, communication with provider, feelings of safety with care/support with care, trust in provider/healthcare system, quality of life, healthcare self-efficacy, acceptability of treatment, ability to make decisions, internalizing of guidance - Mental health: anxiety, depression
Clinical	- Admissions: healthcare utilization (e.g., rates of hospital/ER readmissions/admissions), length of admissions (e.g., time in ER), discharged or admitted to HemOnc - Procedures and test results: echocardiogram, 6-minute walk, biomarkers - Follow up: number of patients lost to follow-up
Caregiver-reported	- Caregiver experience: satisfaction, feelings of support with care
Provider-reported	- Provider experience: provider satisfaction, provider understanding of patient and patient needs, provider feels that they have helped the patient managed their pain effectively and safely

CLOSING REMARKS FROM THE RESEARCH TEAM

Figure 2. "Ten Redefined" by Hertz Nazaire

In Figure 2 (left), artist Hertz Nazaire depicts SCD pain. An advocate for the community, he used his art to help people better understand and empathize with those who have SCD.

Despite the success of this work, the project team recognizes that there is still much work to be done to even begin to touch the surface of improving SCD care. However, we are confident that the research roadmap will be utilized by researchers and others in the SCD space to begin to address the understudied topic of pain self-management through a patient-centered lens. We also hope that this project will serve as a springboard for further collaboration between patients, caregivers, providers, researchers, and policy advocates.

If you have any questions about this research roadmap, please contact Natalya Martins at nmartins@mhqp.org or Jackie Haley at jacqueline.haley@gbscda.org.