DEFINING VALUE AND SUPPORTING EQUITY IN SCD

LAY SUMMARY REPORT

2022-2023

SICK CELLS

INTRODUCTION

As more treatments for SCD become available, the need to measure the true value of treatments to patients becomes more important. Currently, there is insufficient data concerning what constitutes value to SCD patients and how to measure value in decision making.

Sick Cells worked in collaboration with the University of Southern California Hematology Utilization Group Studies (USC HUGS) and the Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute at University of Washington School of Pharmacy to identify and assess core elements of value to be incorporated into SCD research measures.

Project Goal

The **purpose of this project** was to establish action items for research and value assessments to capture cost and additional burdens that matter to patients living with sickle cell disease (SCD) and caregivers. **Project objectives included:**

- **Recruiting diverse** inputs from patient populations that are representative of the community
- **Prioritizing impacts and outcomes** to measure value for SCD across stakeholder groups
- **Evaluating current measures, methods, and data sources** that address patient-important elements of value for SCD
- **Establishing guidance** for research and value assessments to prioritize health equity for SCD

Project Timeline

June 1, 2022 to May 31, 2023

SCOPE & METHODS

This collaborative project was led by a steering committee and a community advisory board. Both bodies provide relevant expertise and insights into the project design and management.

- The **steering committee** was composed of diverse stakeholder representatives that acted as project advisors and played an active role in creating agenda items, and planning discussions. The steering committee met monthly.
- The **community advisory board (CAB)** was composed of non-profit leaders within the SCD community who served as community experts. The CAB met five times during the duration of the project.

Meet our CAB Members

Charles Carrington
Sickle Cell Association of Harford/Cecil Counties and Eastern Shore

Adrienne Shapiro
Axis Advocacy

Kevin Wake
Uriel Owens Sickle Cell Disease Association of the midwest

Dr. Carolyn Rowley, PHD
Cayenne Wellness Center

Carla Lewis
Kids Conquering Sickle Cell Disease

Cory Lewis
Sick Cells, Community Advisory Board (CAB) Liaison

Regina Hartfield
SCDAA, Inc.

Nilda Navedo
North Carolina Sickle Cell Syndrome Program

Teresa Ginger Davis
Sickle Cell Thalassemia Patients Network (SCTPN)

Dr. Wanda Whitten-Shurney, MD
SCDAA- Michigan Chapter

Zemoria Brandon BSW
SCDAA - Philadelphia/ Delaware Valley chapter

Talana Hughes, MPH
Sickle Cell Association of Illinois (SCDAI)

SCOPE & METHODS CONT.

This **project consisted of two roundtables** involving various stakeholders and experts in SCD. In preparation for the roundtables, the team fielded a questionnaire to identify patient-reported outcome (PROs) tools used by the steering committee members in their fields of work. Results from this questionnaire are provided below in Table 1.

Table 1: List of Elements of Value for SCD Gathered for Sick Cells' Roundtable 1

Disease Impact	Patient-Centric Description (Reported by patients and caregivers to describe disease impacts)	Patient-Reported Outcomes Measures (Reported by researchers to be used in current SCD research; align with disease impacts from this domain)
Bodily pain	pain crises, day-to-day chronic pain, joint pain, and how much pain interferes with daily activities	• Sickle Cell Pain Burden Interview (SCPBI) and Youth (SCPBI-Y) • PROMIS® Pain Intensity • ASCQ-Me® Pain Impact • Pain Coping Strategies Questionnaire for Sickle Cell Disease (CSQ) • Adult Responses to Child Symptoms (ARCS) • Pain Diaries • Annualized number of pain events and severe pain events
Fatigue	tiredness, weary, sleepy	• PROMIS® Fatigue 13a SF • PGIS Fatigue and Pain • PGIC Fatigue and Pain
Quality of life	standard indicator of individual's perception of their live	• Adult Sickle Cell Quality of Life Measurement Information System • PROMIS-25 v1.1 Pediatric Profile • PedsQL Sickle for QOL
Social functioning and support	ability to fulfill role in work, social activities, and other relationships	• ASCQ-Me® Social Functioning Impact • Multidimensional Scale of Perceived Social Support
Economic burden	financial burden, ability to work, out-of-pocket costs	• Work Productivity and Activity Impairment Questionnaire (WPAI:SNP) • Sickle Cell Disease Work Study Impact Questionnaire
Emotional well-being	emotions, stress, mood, anxiety, depression	• ASCQ-Me® Emotional Impact
Cognitive function	mental abilities, ability to think and focus, memory, mental fog	• Behavior Rating Inventory of Executive Function (BRIEF) – Abbreviated Parent
Stigma	experience of stigma and discrimination	• Measure of Sickle Cell Stigma (MoSCS) • Sickle Cell Disease Health-Related Stigma Scale (SCD-HRSS)
Self-efficacy	ability to reach personal goals, confidence in individual abilities	• Sickle Cell Self-Efficacy Scale (SCSES) • Jenerette Self-Care Assessment tool • Patient Activation Measure (PAM)
Sleep disruption	poor sleep, insomnia	• Adolescent Sleep Wake Scale
Caregiver impact	health outcomes and other impacts that personally affect the family and others	• Adult Responses to Child Symptoms (ARCS) • Bath Adolescent Pain Questionnaire – for parents (BAPQ-P)
Risk of other health complications	risk of organ damage, stroke, or other future health events	

SCOPE & METHODS CONT.

Description of Roundtables

ROUNDTABLE #1
NOV 16, 2022

- Roundtable One was assembled to facilitate a dialogue of important impacts and outcomes according to patients and stakeholders.
- The primary goal was to learn what data is available and what work has been done.
- There were a total of 50 participants, including 4 SCD advocates, 7 caregivers, 17 individuals from a government agency/industry, 9 researchers, and 13 SCD warriors.

ROUNDTABLE #2
MAR 30, 2023

- Roundtable Two focused on Prioritizing Outcomes and Measures for Sickle Cell Disease.
- Individuals were asked to rank disease impacts to determine their importance across the SCD community.
- More than 40 participants made up of patients, caregivers, and stakeholders participated in the second roundtable.

This project was built on the Patient-Centered Core Impact Set (PC-CIS) framework, which upholds the principles of patient leadership, equal voice and power, and emphasis on wider understanding of impacts that affect people living with sickle cell disease. A list of eight disease impacts that matter most to patients and caregivers was developed by the Community Advisory Board through the following initiatives:

- **Survey**. CAB and the project team gathered primary data related to the impact of disease and patient preference data to fill existing impact data gaps.
- **Community Meetings**. People living with SCD and community leaders informed about disease impacts through participation in listening sessions, interviews, and roundtable meetings.
- **Environmental Scans**. The project team reviewed existing data through a myriad forms, including published reports, peer-reviewed and gray literature, government reports, health technology reports, clinical practice guidelines and quality measures, and existing Core Outcome Sets (COS).
- **Patient Prioritization**. The CAB worked to prioritize the most important impacts before project convenings.

RESULTS

Reults of Roundtables

Figure 1: Stakeholder Participation in Ranking and Weighting Exercise

N = 42 Total Respondents

Patient and Caregiver	9
Community Organization	8
Industry	6
Physician	4
Researcher	4
Government	4
Payer	2
Other (did not report)	5

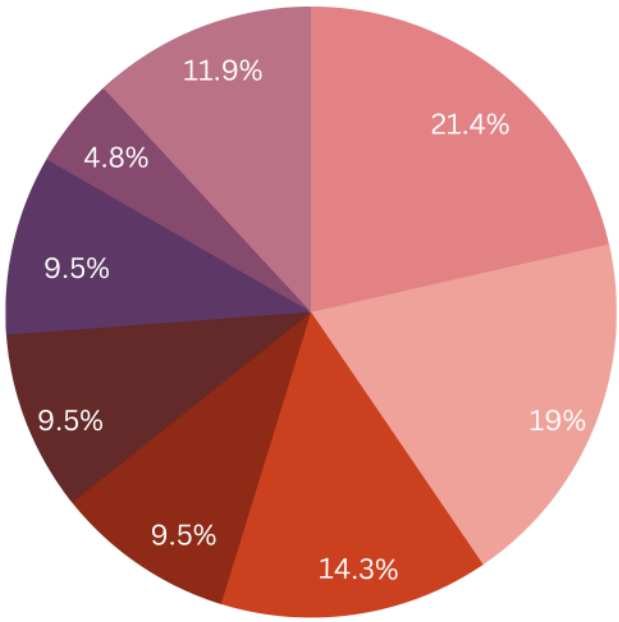


Table 2: Patient-centric Descriptions of Disease Impacts used for

Disease Impact	Description
Acute pain events	pain crises, joint pain, pain interfering with daily activities, pain resulting in emergency visits
Daily chronic pain	day-to-day pain, chronic pain, pain interferes with daily activities
Ability to Age	length of life, ability to live fulfilled life, risk of complications, risk of organ damage
Fatigue	not having energy, tiredness, weary, sleepy
Quality of life	individual's perception of their life
Social functioning and self-efficacy	ability to fulfill role in work, social activities, and other relationships, ability to reach personal goals, confidence in individual abilities
Economic and financial burden	financial burden, ability to work, out-of-pocket costs, home-related health costs, ability to go to school, education attainment, insurance coverage
Emotional well-being and mental health	emotions, stress, mood, anxiety, depression, caregiver well-being, reduction in trauma experience, fear of future complications
Cognitive health	mental abilities, ability to think and focus, memory, mental fog
Stigma	experience of stigma and discrimination, reduction in trauma experiences

RESULTS CONT.

Figure 2: Final Group Ranking of Disease Impacts, Aggregation from Three Ranking Votes
N = 40 Total Respondents

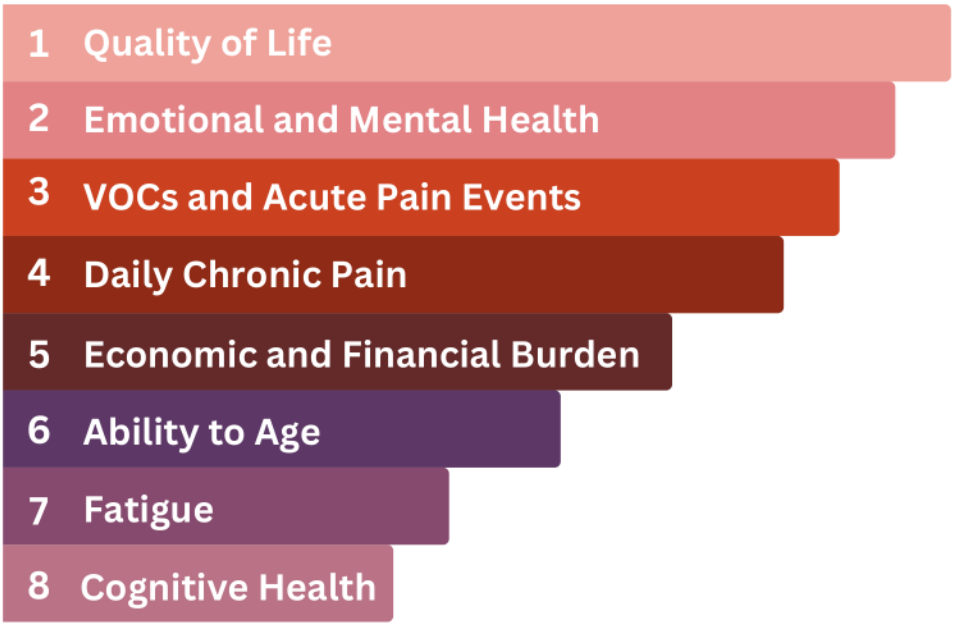


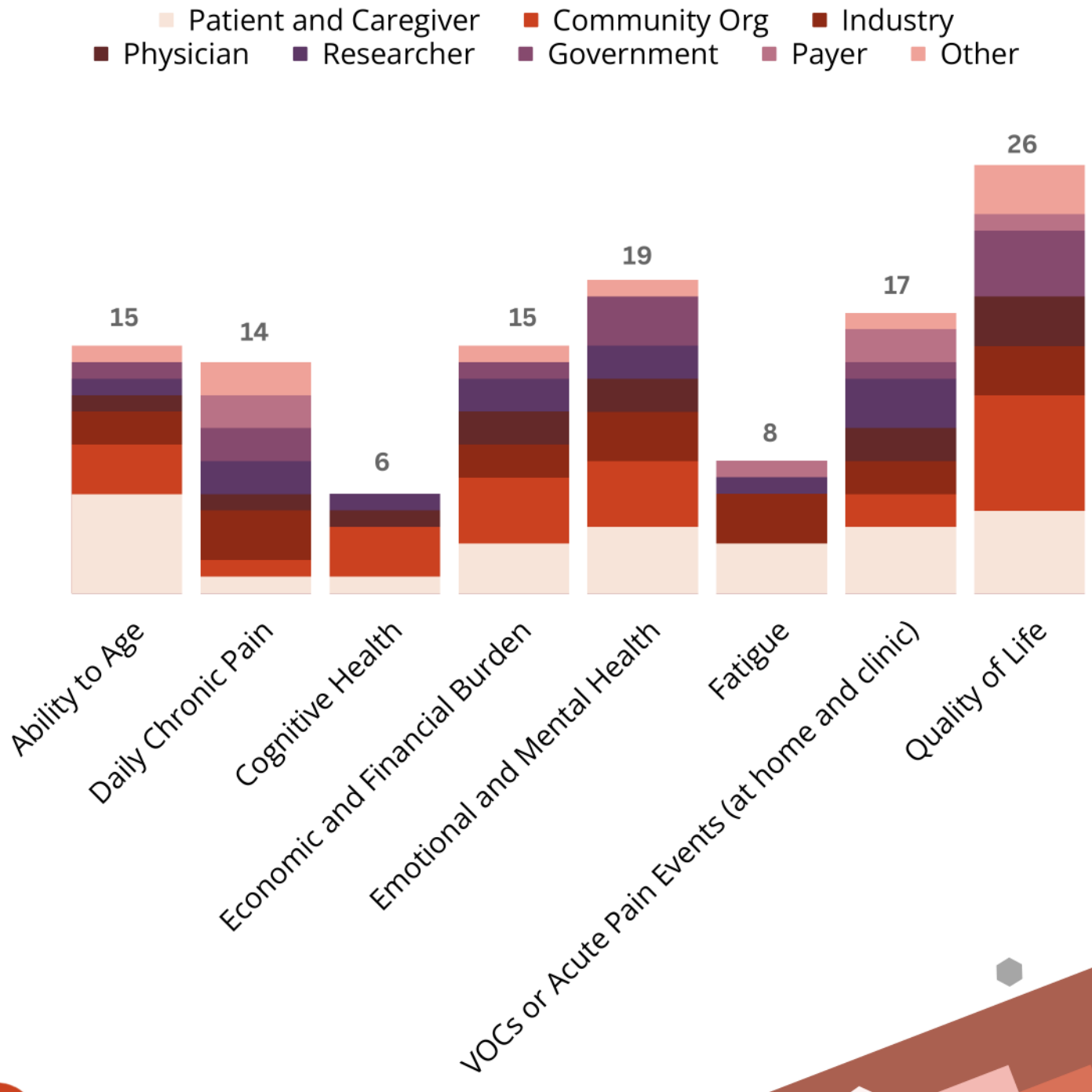
Figure 3: Calculated Group Weighting of Disease Impacts
Relative importance scores between disease impacts converted to weights (swing weighting)
Total = 100%

Quality of Life	17%
Emotional and Mental Health	16%
VOCs and Acute Pain Events	15%
Daily Chronic Pain	14%
Economic and Financial Burden	12%
Ability to Age	10%
Fatigue	8%
Cognitive Health	7%

RESULTS CONT.

Figure 4: Top Three Disease Impacts of Individual Respondents, Segmented by Stakeholder Group

N = 40 Respondents



IMPACT OF WORK

Sick Cells identified several areas of learning from this initiative, including:

Direct interaction and communication of lived experience and the complexities of a disease across age, race and ethnicity, severity of condition, and caregiver status is important for clinician and research communities, as well as payers to understand the health journey and the varying health and economic impacts of SCD.

Multi-level structure to community involvement is important, including both steering committee and patient-focused bodies, with a liaison role that has responsibility and empowerment to educate, identify gaps in methods and participation, and facilitate interaction. It is vital to use creative and multi-layered methods and outreach to expand inclusivity of patient voices – in this case identifying underrepresented communities.

Utilizing mixed methods approaches is important, and creating steps in the project to use structured research methods will engage research contributors who desire structured approaches, and create patient-focused results.

Subgroup engagement and analysis is important to really addressing equity. Sick Cells learned that significant time was needed to improve representation from identified subgroups and that such outreach ideally would occur before the project launch. As a result, the organization is pursuing extension research efforts to engage subgroups in similar discussions and ranking activities, focusing initially on elders and Latinos living with SCD.

Budget more time at the front end of a project to explore underrepresented groups and undertake outreach to bring those voices to the table. Consider methodologies and tools that will engage and gain input from all stakeholder, even while you're prioritizing patient and lived experience input.

IMPACT OF WORK CONT.

Identification of patient-important impacts has influenced structural choices in existing projects, including considering pain events occurring outside clinical settings, and including data on fatigue in economic modeling. Sick Cells is using this information to influence clinical research, including patient-centered comparative clinical effectiveness research, to influence definition of quality standards and measures, and to converse with regulators and policymakers about how patient-prioritized impacts can drive improved data collection and evaluation of value. This project is a precursor and can inform many downstream decisions and uses, including:

- Clinical Trials
- Value Assessment
- Regulatory Decisions
- Value-Based Arrangements
- Quality Measurement
- Clinical Practical Guidelines
- Coverage Decisions
- Research Collaborations
- Policy Decision-makers
- Best Practices in Other Disease States

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