EMPOWERED PATIENT LEADERS WORKSHOP

FINAL LAY SUMMARY

BACKGROUND

Sick Cells hosted the hybrid **Empowered Patient Leaders Workshop** on June 26, 2025 in Washington, DC and online. This participatory event convened patient leaders, caregivers, providers, patient organizations, and other stakeholders to strengthen their capacity in developing patient-centered core impact sets. The workshop goals included:

- **Participants will understand** how Sick Cells adapted the Blueprint for Developing PC-CIS for the sickle cell disease context and what they learned in the process
- **Participants will be aware** of potential challenges in applying the Blueprint to their disease state and context
- **Participants will discuss** best practices on how to gather, understand, and share the priorities of the patient community
- **Participants will be familiar** with and know where to find the resources available to support the development of a PC-CIS for their disease state

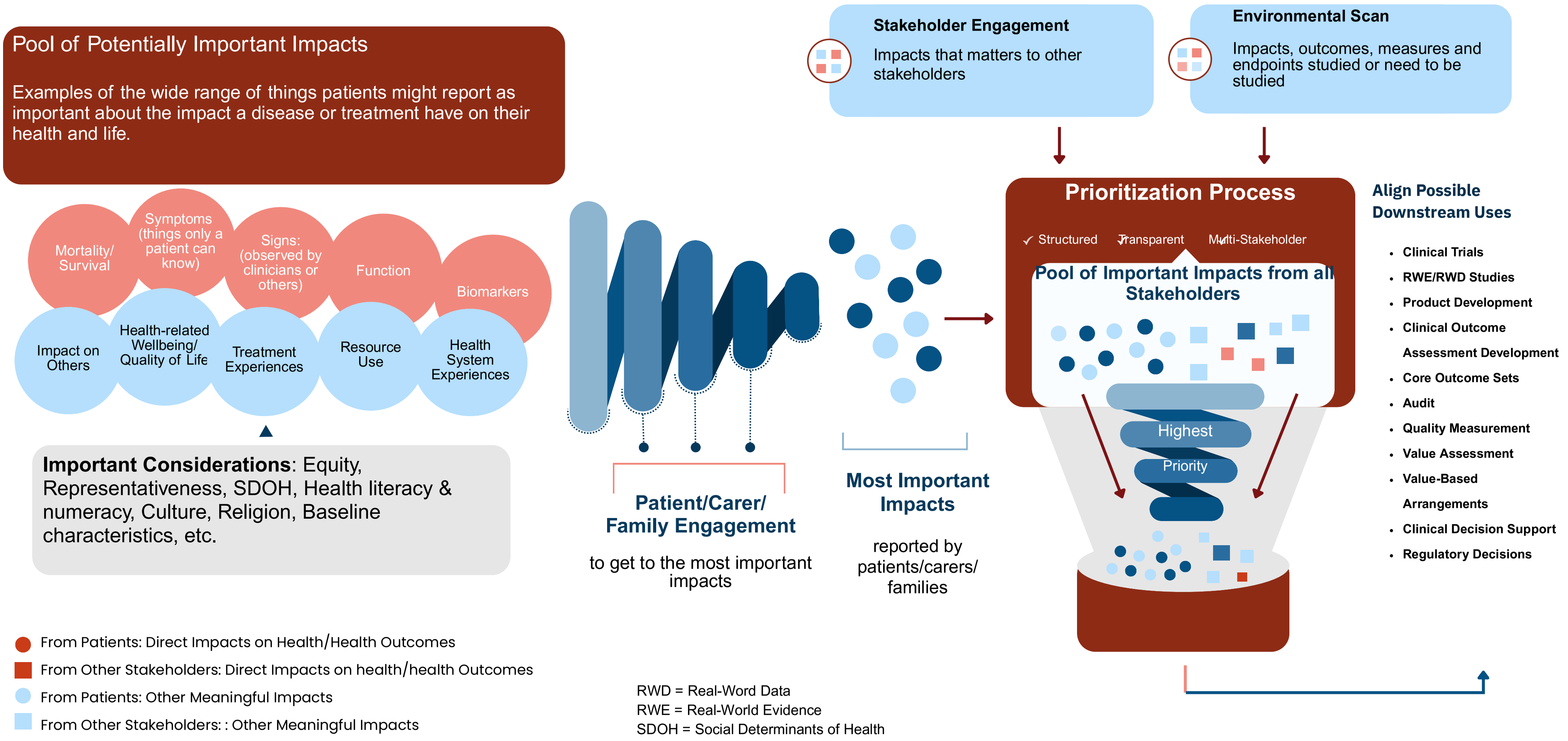
These objectives were developed and vetted by the Project Advisory Committee and the project team and drove the workshop agenda.

CONFERENCE SUMMARY

The Empowered Patient Leaders Workshop introduced organizations to the PC-CIS development process, highlighted examples of its implementation, and explored the opportunities and challenges that may arise along the way. Designed to be participatory and action-focused, the workshop featured extensive small group discussions, peer sharing, and brainstorming sessions. More than 75% of participants were patients or representatives from patient groups. Many of them also played a key role in shaping the workshop itself, as advisory committee members, subject matter experts, and presenters. Their involvement ensured that the workshop remained focused on the needs and perspectives of patients, resulting in meaningful dialogue and a strong emphasis on centering the patient voice.

The workshop opened with an overview of the PC-CIS Blueprint developed by the National Health Council. This session introduced the blueprint's components and outlined what stakeholders can expect from both the PC-CIS process and its outcomes. See Figure 1. below.

Figure 1: A Framework for Developing Disease-Specific Patient-Centered Core Impact Sets (PC-CIS)*



Pool of Potentially Important Impacts

Examples of the wide range of things patients might report as important about the impact a disease or treatment have on their health and life.

- Mortality/ Survival
- Symptoms (things only a patient can know)
- Signs: (observed by clinicians or others)
- Function
- Biomarkers
- Impact on Others
- Health-related Wellbeing/ Quality of Life
- Treatment Experiences
- Resource Use
- Health System Experiences

Important Considerations: Equity, Representativeness, SDOH, Health literacy & numeracy, Culture, Religion, Baseline characteristics, etc.

● From Patients: Direct Impacts on Health/Health Outcomes
🟥 From Other Stakeholders: Direct Impacts on health/health Outcomes
● From Patients: Other Meaningful Impacts
▨ From Other Stakeholders: : Other Meaningful Impacts

Patient/Carer/ Family Engagement

to get to the most important impacts

Most Important Impacts

reported by patients/carers/ families

Stakeholder Engagement

Impacts that matters to other stakeholders

Environmental Scan

Impacts, outcomes, measures and endpoints studied or need to be studied

Prioritization Process

✓ Structured ✓ Transparent ✓ Multi-Stakeholder

Pool of Important Impacts from all Stakeholders

Highest Priority

Align Possible Downstream Uses

- Clinical Trials
- RWE/RWD Studies
- Product Development
- Clinical Outcome Assessment Development
- Core Outcome Sets
- Audit
- Quality Measurement
- Value Assessment
- Value-Based Arrangements
- Clinical Decision Support
- Regulatory Decisions

RWD = Real-Word Data
RWE = Real-World Evidence
SDOH = Social Determinants of Health

* Select colors may vary from original figure. Original is available on NIH website here: https://www.ncbi.nlm.nih.gov/core/lw/2.0/html/tileshop_pmc/tileshop_pmc_inline.html?title=Click%20on%20image%20to%20zoom&p=PMC3&id=9584872_40271_2022_583_Fig1_HTML.jpg

Following the introductory session, **the Sick Cells team presented the *Defining Value and Supporting Equity* project and outlined the process that led to developing a PC-CIS for sickle cell disease**. This included an overview of the project stakeholders as well as the brainstorming, ranking, and weighting sessions that shaped the development of the core impact set.


DEFINING VALUE AND SUPPORTING EQUITY IN SCD

LAY SUMMARY REPORT
2022-2023

SICK CELLS

Defining Value and Supporting Equity in SCD, report published in 2024

The COPD Foundation shared a **similar process and concept, called Patient-Inspired Validation of Outcome Tools (PIVOT)**. The presenter shared the key aspects of the PC-CIS philosophy that the research team applied to their work, discussed the evolution of the PIVOT initiative over time, and reflected on the lessons learned. They also shared how the PC-CIS Blueprint guiding principles are rooted in their work.

After hearing from these experts, participants reflected on how they might apply these approaches in their work, including exploring whether something similar already exists and what resources might be needed, as well as what supports are available to move this work forward. The conversation then shifted to lessons learned by both Sick Cells and the COPD Foundation, including challenges encountered and how they overcame them. Participants worked in small groups to anticipate challenges they might face in PC-CIS development and share ideas about how to overcome those challenges.

The workshop wrapped with a discussion of the future implications and the growing need for disease-specific PC-CIS, including their impact on clinical trials, value and health technology assessments, and medical decision-making. **Participants left energized and equipped to perform this important work within their patient communities.**

KEY FINDINGS

Through the discussion and participatory exercises, workshop participants crowdsourced a list of what is required to do this work successfully. The list below offers insights into the current research landscape and opportunities for improving the environment for PC-CIS work:

A network of qualitative researchers with expertise in this field to tap into

Financial support to expand PC-CIS work

Raising awareness with stakeholders and buy-in to the process

Support for dissemination, change management, and facilitating use of PC-CIS

Coordination among patient communities to come together and push this approach

Some key challenges the group identified include:

- **Overcoming the gender gap** - especially as it relates to participation of black males
- **Gaining buy-in from decision makers** to validate and use this research in drug development and disease treatment
- **Gaining organizational buy-in** to funnel limited resources toward this work and away from other important lines of research
- **Access to funding** for this work
- **Change management** and facilitating use of PC-CIS
- **Ensuring participation** of a heterogeneous patient panel that reflects the patient population of the disease in question

Feedback from workshop participants was overwhelmingly positive. Respondents to the evaluation rated the workshop an average of 4.86 out of 5 stars, and **100% of respondents reported feeling at least somewhat confident**, **if not confident or very confident**, in their ability to develop a PC-CIS for their disease state.

As the team considers offering this content again in the future, some participants recommended a stronger emphasis on practical tools, including the National Health Council's existing toolbox.

Some suggested breaking the content into a series of shorter modules, culminating in an in-person gathering once organizations have had time to absorb the foundational materials and reflect on how to apply them in their own context.

Another suggestion was to create a cohort of organizations going through the process together on a similar timeline. This will allow experienced practitioners to better support. Those who are newer to PC-CIS development and allow for more robust discussions and troubleshooting throughout the various stages of the process.

DISSEMINATION PLANS

This lay project summary will be posted to the Sick Cells website and distributed via newsletter. Project Advisory Committee members and workshop participants will also be encouraged to distribute the project summary to their membership.

Sick Cells is working with the National Health Council to include this lay workshop summary in the National Health Council's set of supplemental materials available for organizations wishing to develop a PC-CIS. The examples included in the workshop can support and inspire other organizations to pursue this important work.

The team is also discussing opportunities to hold follow up workshops with other organizations or develop the content into virtual modules, pending funding. A regionally-focused patient organization is currently working with Sick Cells to adapt the workshop materials for their context and group.

PROJECT FUNDING

This convening is funded through a Patient-Centered Outcomes Research Institute® (PCORI®) Eugene Washington PCORI® Engagement Award (#EASCS-32772).

APPENDIX A: CONFERENCE AGENDA

9:30 AM	Doors Open, In-person check in begins Coffee and continental breakfast will be available
9:55 AM	Virtual Room Opens
10:00 AM	**Session Begins**
12:05 PM	Welcome and Introductions Patient-Centered Core Impact Sets (PC-CIS) Blueprint Overview Sick Cells: Defining Value COPD Foundation: Patient-Inspired Validation of Outcome Tools (PIVOT)
12:35 PM	Lunch Break, Networking
3:00 PM	**Session Ends**

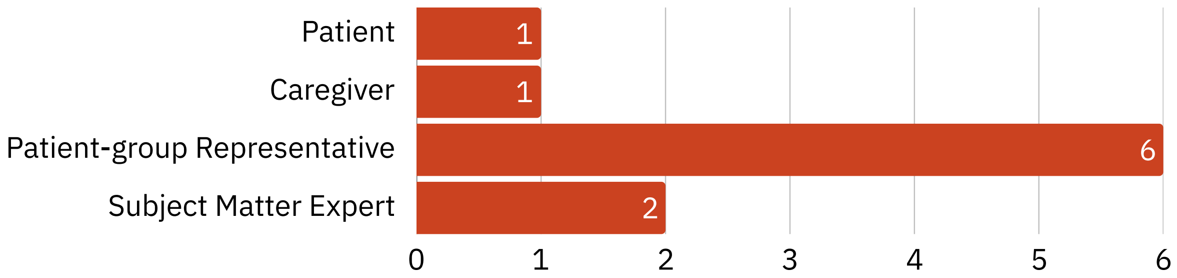
APPENDIX B: ATTENDEE LIST

- Elisabeth Oehrlein, *Applied Patient Experience, LLC*
- Anna Hyde, *Arthritis Foundation*
- Hannah Jaffee, *Asthma and Allergy Foundation of America*
- Adrienne Shapiro, *Axis Advocates*
- Casey Gibson, *Axis Advocates*
- Ushma Patel, *Center for Innovation & Value Research*
- Alan Hamilton, *COPD Foundation*
- Bruce Miller, *COPD Foundation*
- Misty A. Green, *COPD Foundation*
- Shadwanda Rainey, *COPD Foundation*
- Olivia Dieni, *Cystic Fibrosis Foundation*
- Ruth Tal-Singer, *Global Allergy & Airways Patient Platform*
- Phyllis Foxworth, *Huntington's Disease Society of America*
- Jackie Hinckley, *National Aphasia Association*
- Doreen Méndez-Sierra, *National Aphasia Association*
- Kim Irby, *National Aphasia Association*
- Brenda Martin, *Patient Educator*
- Ivey Wohlfeld, *PCORI*
- Daniella Mak, *Self*
- Mariah Scott, *Sick Cells*
- Ashley Valentine, *Sick Cells*
- Simone Williams, *Sick Cells*
- Maria DiFuccia, *Sick Cells*
- Melissa Bevins, *Sick Cells*
- Abby Tainter, *Sick Cells*
- Sarita Edwards, *The E.WE Foundation*
- Eleanor Perfetto, *University of Maryland*
- Dorothy Winningham, *Winn2BHealthy LLC*

APPENDIX C: POST-WORKSHOP SURVEY

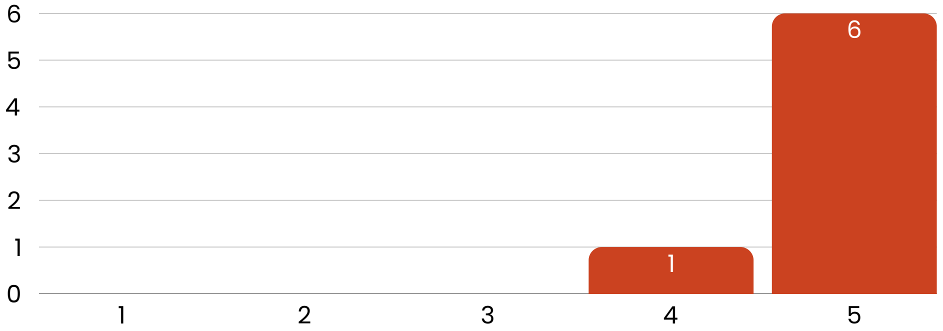
Question 1: What best describes you? (select all that apply)

N=7 responses



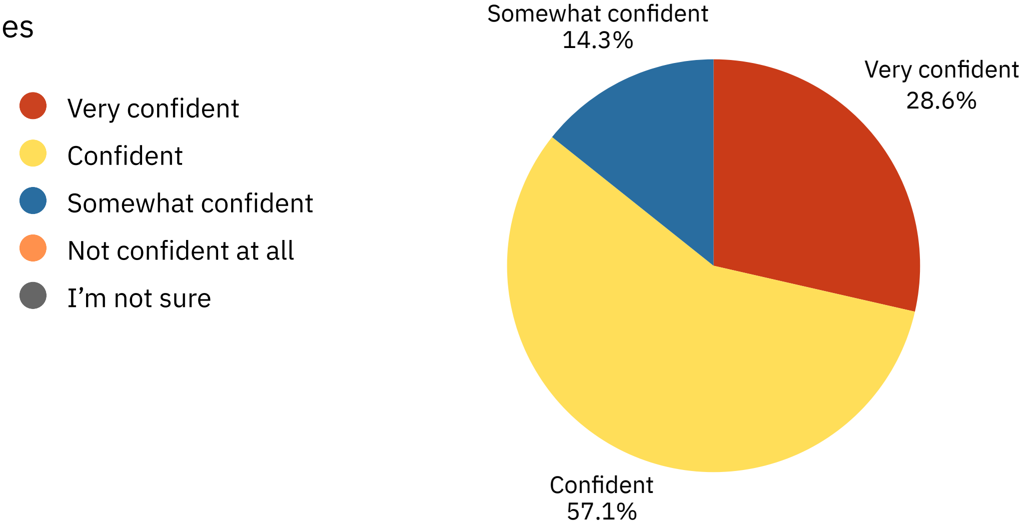
Question 2: Overall, how satisfied are you with the Empowered Patient Leaders Workshop? (Scale 1-5, 5 is highly satisfied)

N=7 responses



Question 3: After attending this workshop, how confident do you feel in your ability to develop a PC-CIS for your disease state?

N=7 responses

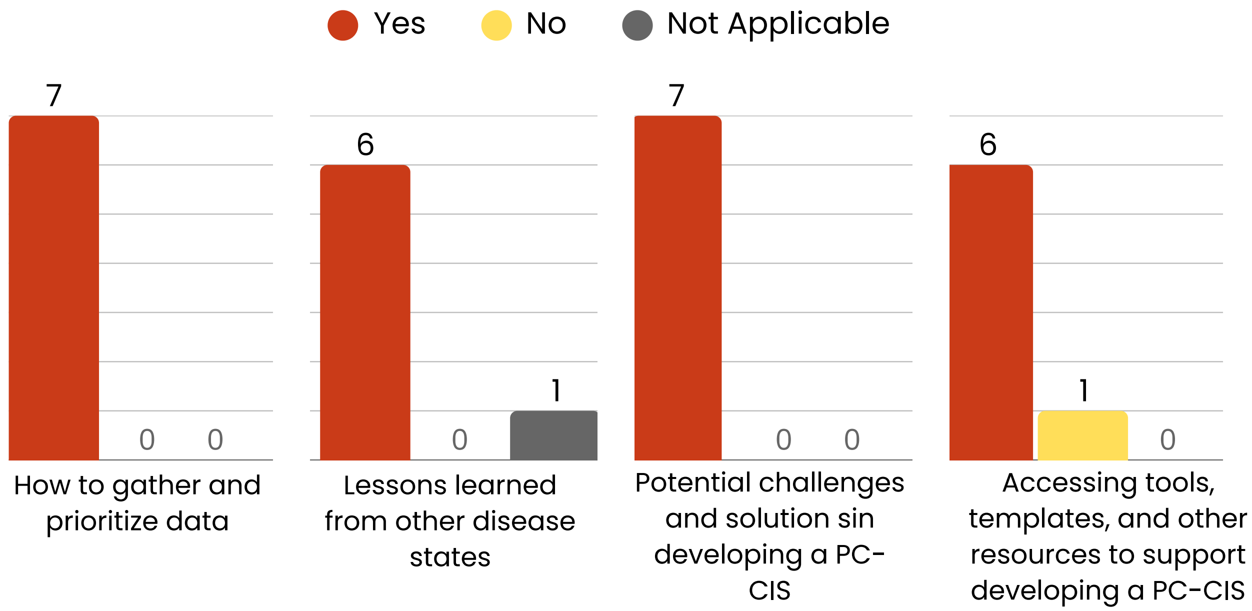


Question 4: What are your key takeaways from attending the Empowering Patient Leaders Workshop?

N=7 responses

- It was well presented even the slides along with the explanation was meaningful.
- Overall framework for PC-CIS
- Importance of protecting the voice, how impacts differ from endpoints, importance of understanding from patients "what to measure" and then using that information to determine "how to measure" including determining if existing outcome measures are adequate or if new outcome measured need to be developed and validated, importance of demonstrating "fit for purpose"
- Thanks for providing the sick cell process. This is so helpful
- Not everything has to be done all at once, patient data we're already collecting can serve as building blocks for PC-CIS development
- Strong degree of alignment between the PC-CIS, PFDD roadmap to COA development and PIVOT patient-inspired health concepts
- There are ways any organization can collect meaningful PED, but resources for many groups remain a challenge and a need.
- Cross talk between disease areas is important - day to day we tend to stay in our silos but engaging with experts in other disease areas is very helpful.
- I really enjoyed hearing from both NHC, who developed the framework, and patient groups who put it to use. Really nice to hear from multiple perspectives

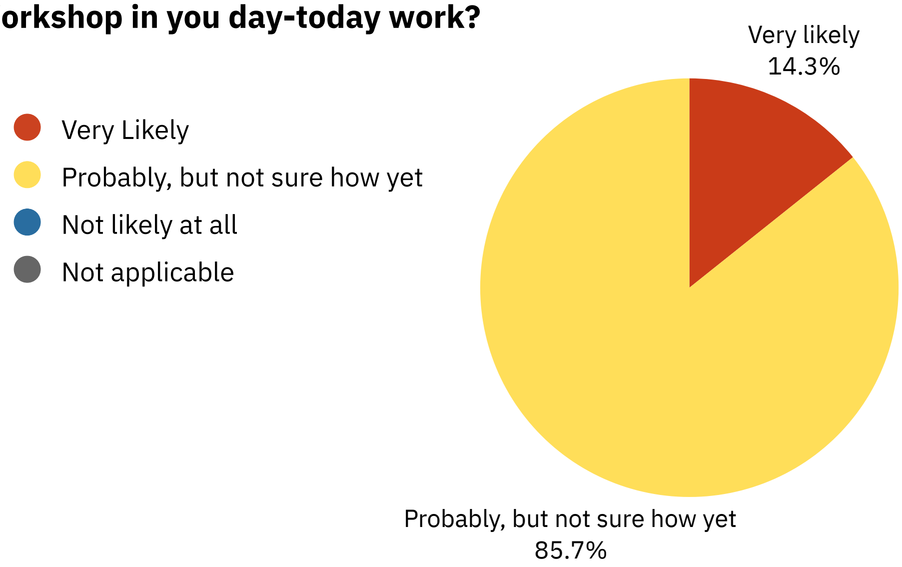
Question 5: Did the Workshop meet your expectations in the following areas?



Question 6: If there were any parts of the workshop that did not meet your expectations, please explain how we can do better next time.

- I'd love to see a round 2 workshop once you all have determined next steps; getting things like this implemented is a hurdle we are currently thinking a lot about and I'd love the opportunity to solve for that in concert with other patient groups.

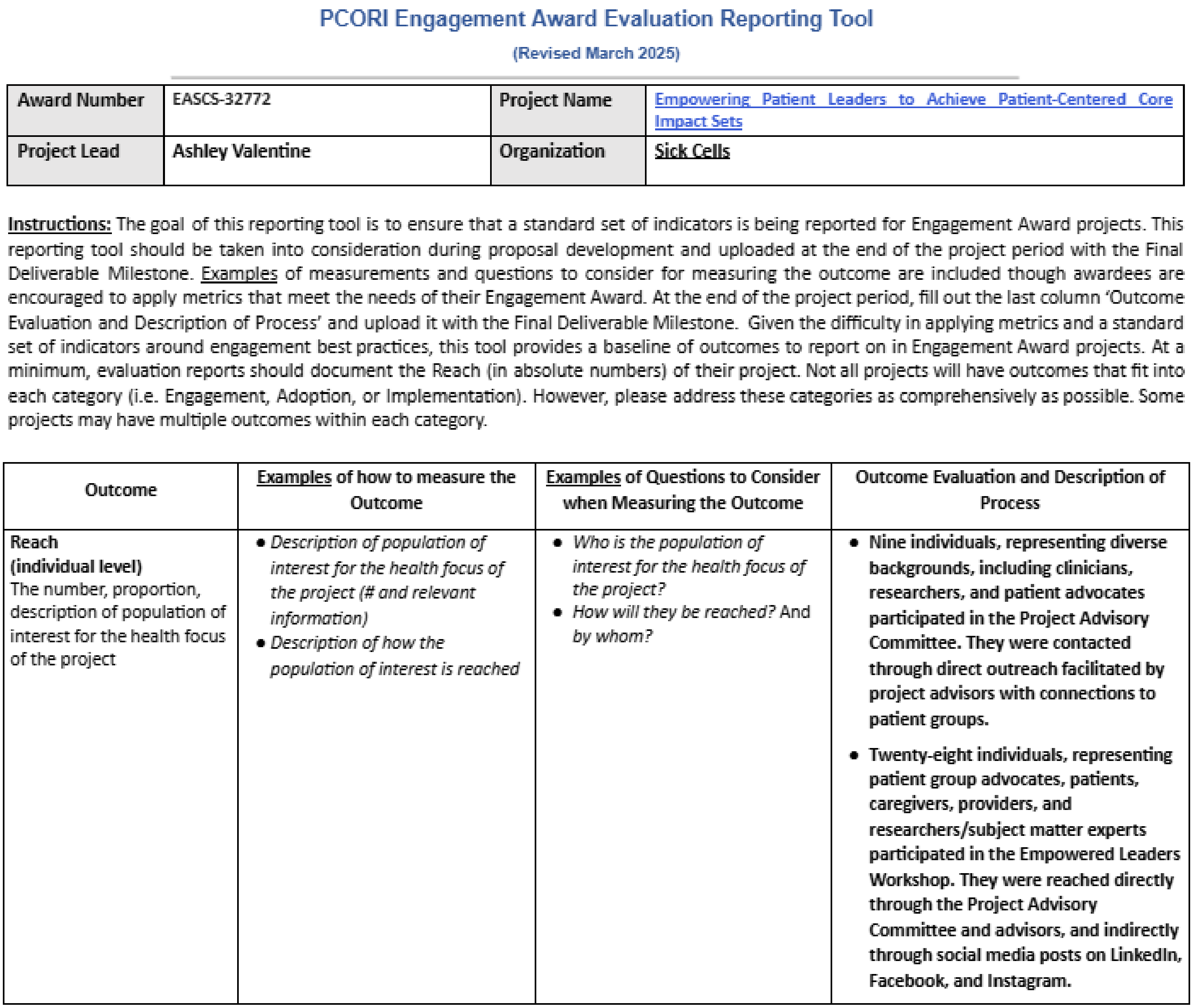
Question 7a: How likely are you to apply what you learned in the Empowered Patient Leaders Workshop in you day-today work?



Question 7b: If very likely, how do you plan on applying what you learned? If probably or not likely, please explain why.

- It is better to get consensus from those you are promising to serve to see what's important to them.
- The content of the workshop is very relevant to a number of aspects of our daily work.
- We are in phase 2 of our PC cis initiative. I am much more confident about moving forward using what I learned.
- I plan to do an environmental scan of what's already out there, and develop a plan for what we would need to do this work as a first step
- Further probe the connection between PC-CIS and patient-inspired health concepts (PIVOT) and explore ways to communicate that patient-inspired health concepts within PIVOT (with a measurement focus) are downstream of a PC-CIS in COPD
- I always love referencing examples like this in conversations with other stakeholders, even if it’s outside my therapeutic area; and it will provide a good concrete example for us as we continue our patient engagement work.

APPENDIX D: PCORI ENGAGEMENT AWARD EVALUATION REPORTING TOOL



PCORI Engagement Award Evaluation Reporting Tool

(Revised March 2025)

Award Number	EASCS-32772	**Project Name**	Empowering Patient Leaders to Achieve Patient-Centered Core Impact Sets
Project Lead	Ashley Valentine	**Organization**	Sick Cells

Instructions: The goal of this reporting tool is to ensure that a standard set of indicators is being reported for Engagement Award projects. This reporting tool should be taken into consideration during proposal development and uploaded at the end of the project period with the Final Deliverable Milestone. Examples of measurements and questions to consider for measuring the outcome are included though awardees are encouraged to apply metrics that meet the needs of their Engagement Award. At the end of the project period, fill out the last column 'Outcome Evaluation and Description of Process' and upload it with the Final Deliverable Milestone. Given the difficulty in applying metrics and a standard set of indicators around engagement best practices, this tool provides a baseline of outcomes to report on in Engagement Award projects. At a minimum, evaluation reports should document the Reach (in absolute numbers) of their project. Not all projects will have outcomes that fit into each category (i.e. Engagement, Adoption, or Implementation). However, please address these categories as comprehensively as possible. Some projects may have multiple outcomes within each category.

Outcome	Examples of how to measure the Outcome	Examples of Questions to Consider when Measuring the Outcome	Outcome Evaluation and Description of Process
Reach (individual level) The number, proportion, description of population of interest for the health focus of the project	• *Description of population of interest for the health focus of the project (# and relevant information)* • *Description of how the population of interest is reached*	• *Who is the population of interest for the health focus of the project?* • *How will they be reached? And by whom?*	• **Nine individuals, representing diverse backgrounds, including clinicians, researchers, and patient advocates participated in the Project Advisory Committee. They were contacted through direct outreach facilitated by project advisors with connections to patient groups.** • **Twenty-eight individuals, representing patient group advocates, patients, caregivers, providers, and researchers/subject matter experts participated in the Empowered Leaders Workshop. They were reached directly through the Project Advisory Committee and advisors, and indirectly through social media posts on LinkedIn, Facebook, and Instagram.**

Engagement (researchers, stakeholders, patients, communities) Involvement of partners in the planning, implementation, and follow-up of proposed activities	• *# of opportunities for patient and stakeholder partners to engage* • *# of jointly identified alternatives/solutions discussed and adopted*	• *Which partners have been involved throughout the planning and implementation process?* • *How will their engagement be sustained?*	• The Project Advisory Committee, representing nine stakeholder partners, were engaged over a four month period to inform planning and implementation of the Empowered Patient Leaders Workshop, providing guidance and feedback on topics including: defining value and centering patients; data collection and prioritization, and common impacts. • In the post-workshop survey, 86% of participants said that they were very likely to apply what they learned in the Empowering Patient Leaders Workshop in their day-to-day work, and the remaining 14% said that they will probably apply what they learn, although not sure how yet. • While there is no formal plan for the Project Advisory Committee or the Workshop Participants to engage post-award, many participants expressed an eagerness to create a PC-CIS for their disease state, and will utilize the resources shared and the expertise of the subject matter experts for support. • One participant noted in the post-survey that "cross talk between disease areas is important - day to day we tend to stay in our silos but engaging with experts in other disease areas is very helpful." Another noted the value in hearing from both the framework developers and patient groups who have put it to practical use.

Adoption (system/setting level) The number, proportion, and description of those delivering the activity or resource/tool (i.e., staff, clinics, systems)	• *Methods of how the engagement effort (tool/resource/conference) will be delivered* • *For projects* ***adapting*** *a resource/tool: description of how the tool will be delivered similarly or differently compared to the initial tool*	• *How will this resource/tool be delivered in the desired context?* • *Who Is delivering the tool in the desired context?*	• **The Empowered Patient Leaders Workshop was delivered in a hybrid setting to allow both in-person and virtual participation in an interactive format.** • **The workshop presenters included: 1. Dr. Eleanor M. Perfetto, who led the effort to develop the NHC PC-CIS Blueprint; 2. Mariah Scott, MS, MPH and a sickle cell warrior who supported Sick Cells in using the PC-CIS framework; and 3. Dr. Alan Hamilton who led the COPD Foundation's Patient-Inspired Validation of Outcome Tools. All three advisors served an integral role in developing, refining, and disseminating the training program materials.**
Implementation Appropriate delivery of Engagement Award project	• *Descriptions of safeguards in place to ensure that the resource/tool/conference will be delivered appropriately to the population of interest for the health focus of this project and context*	• *What safeguards are in place to determine that the resource/tool/conference will be delivered appropriately?* • *How are steps in the engagement process being documented and evaluated?* • *How will recipients of this award be prepared to participate in patient-centered CER?*	• **To determine that the workshop and related resources were delivered appropriately, we relied on expert project advisors from NHC, patient advocates and patient-centered CER subject matter experts.** • **For the Project Advisory Committee, engagement was documented through member's active participation. For the Empowered Patient Leaders Workshop, engagement was documented through pre- and post-surveys.** • **In the post-workshop survey, 86% of participants said that they were confident in their ability to develop a PC-CIS for their disease state after attending the Empowering Patient Leaders Workshop, and the remaining 14% said that they were somewhat confident in their ability.**