Blood Disorders
Basic and Translational Research
Voices of Sickle Cell Disease Stakeholders

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RESEARCH QUESTION

What are the attitudes and beliefs of different stakeholders within the Sickle Cell Disease (SCD) community *towards participation in future genome editing clinical trials*?

Persaud A, Desine S, Blizinsky K, and Bonham VL. A CRISPR Focus on Attitudes and Beliefs Towards Somatic Genome Editing from Stakeholders Within the Sickle Cell Disease Community. *(in press).*

STUDY DESIGN

Mixed-methods study design

- Comprehensive survey instrument with various measures including:
  - Validated Genetic Literacy Assessment
  - Brief Illness Perception
  - 2013 Pew Research Gene Editing Survey
  - Demographics
- 15 focus groups moderated, audio-recorded, and conducted across six cities in the mid-Atlantic and Southern regions of US

Pilot Testing (April 2017)

- Three pilot focus groups
- Improvements made to survey, video tool, focus group questions, and study design

Data Collection (June 2017- Dec 2017)

- Patients (N=46), parents (N=41), and physicians (N=23) are recruited, screened, and consented
- Participants completed pre-video survey
- Participants watched a 14- minute educational video
- Participants completed post-video survey
- 45-60 minute focus groups took place

Analysis

- NVivo 11, a qualitative analytical software, is used to identify and manage major themes across focus groups
- Statistical analysis of survey data to better contextualize focus group findings
- Results will be used to develop a large-scale international survey

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RESULTS

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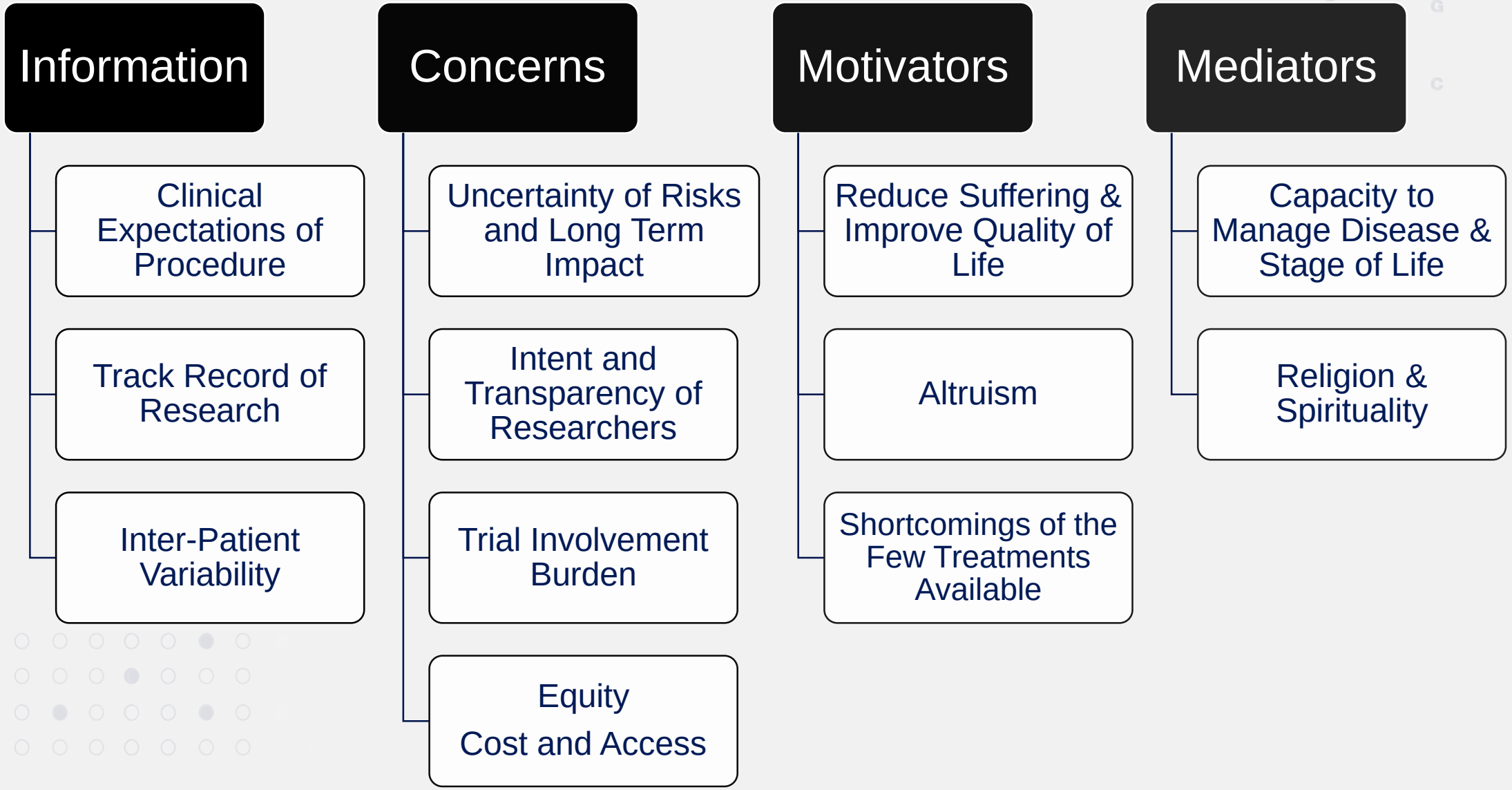
FOCUS GROUP RESULTS: OVERVIEW



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FOCUS GROUP RESULTS

Motivators Improve Quality of Life

"It's exciting... it's what I've been waiting for" (Patient)

"I'm very optimistic…it's another possible option for sickle cell patients and unfortunately we don't have many." (Patient)

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FOCUS GROUP RESULTS

"Show me every animal that died and why." (Patient)

"There's a saying, there's proof in the pudding. I want you to show me evidence and your findings and your results, whether it's 25 percent, 50 percent." (Parent)

"Chances of success, chances of failure, chance of death, chance of irreversible complications, known possible things that could go wrong." (Physician)

Information Track Record of Research

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FOCUS GROUP RESULTS

“Scientists and researchers should want to do this to help patients, to help people, to see them as people and not as science experiments...keep the patient first.” (Parent)

“We need a seat at the table. When these clinical trials are going on and you’ve got the researchers setting up protocols, setting up how it is going to work ... people that have sickle cell need to be involved in every aspect of the trial.” (Patient)

Recommendations to the Research Community on Meaningful Engagement

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FOCUS GROUP RESULTS

“To have the sickle cell population move this forward and then not have this available for them equally would be extremely traumatic to the community.” (Physician)

Concerns
EQUITY

Persaud A, Desine S, Blizinsky K, and Bonham VL. A CRISPR Focus on Attitudes and Beliefs Towards Somatic Genome Editing from Stakeholders Within the Sickle Cell Disease Community. *(in press).*

FOCUS GROUP RESULTS

"If this treatment becomes available to the public, will it be available to everyone equally? I am not rich, but I qualify. I have sickle cell. I struggle with it daily... I don't want the reason why I can't get it done is because, oh, your insurance or you don't have the money." (Patient)

Concerns
EQUITY

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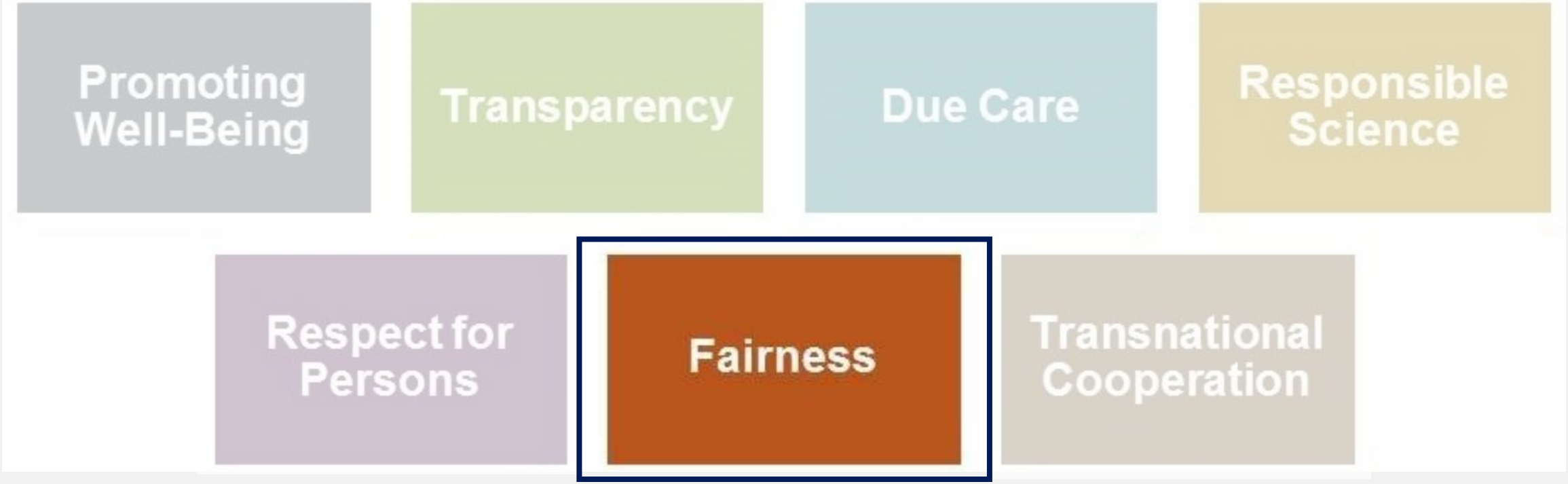
Seven Governance Principles for Human Genome Editing



Human Genome Editing Science, Ethics and Governance, National Academies of Sciences, 2017

HEALTH EQUITY

“Health equity means social justice in health (i.e., no one is denied the possibility to be healthy for belonging to a group that has historically been economically/socially disadvantaged).”

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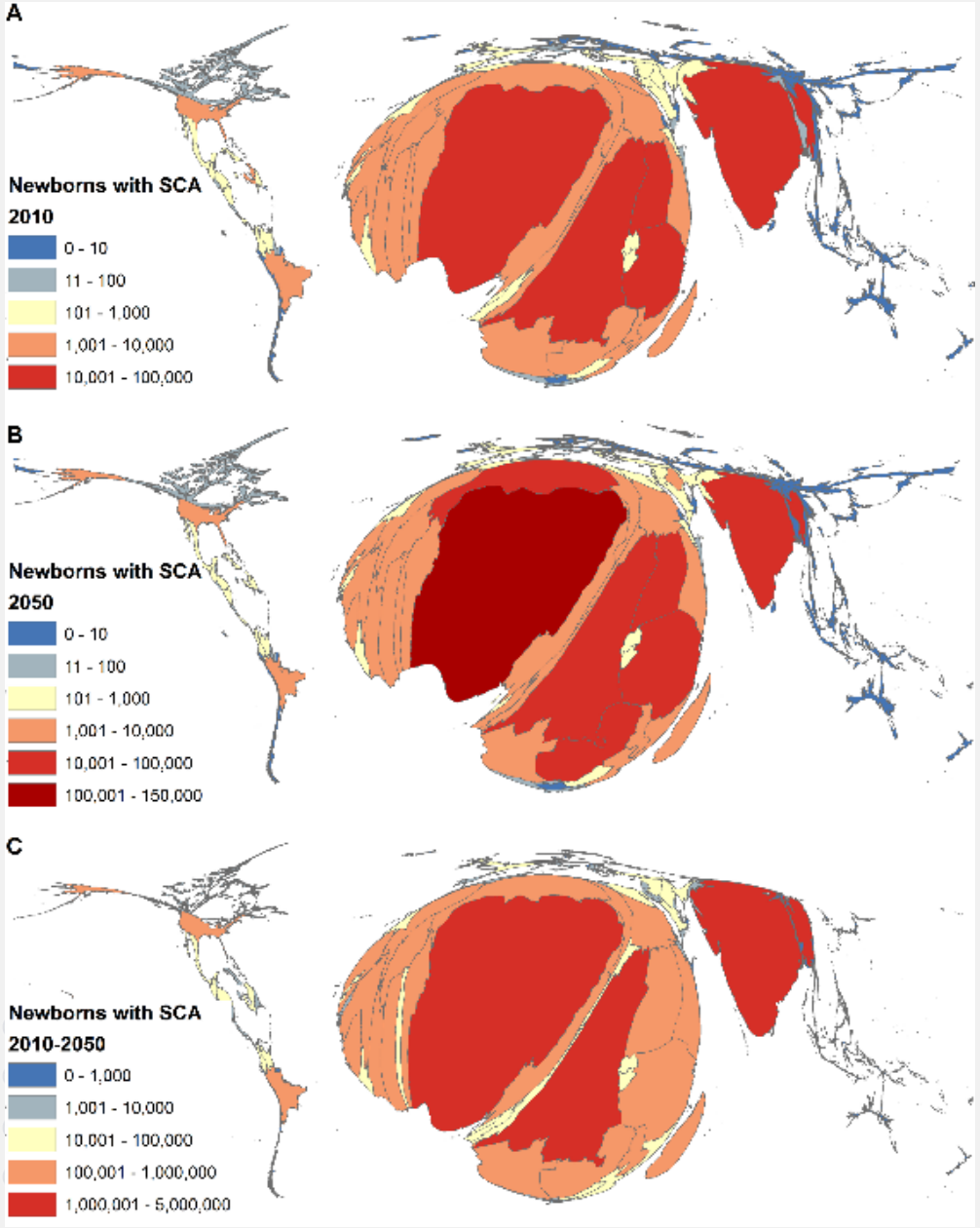
Paula Braverman, MD, MPH, What are Health disparities and Health Equity? We Need to be Clear. Public Health Rep. 2014 129 (Suppl2) 5-8.



SCD occurs in approximately 300,000 births annually.

By 2050, the number of people with SCD is expected to increase by ~ 30% globally.

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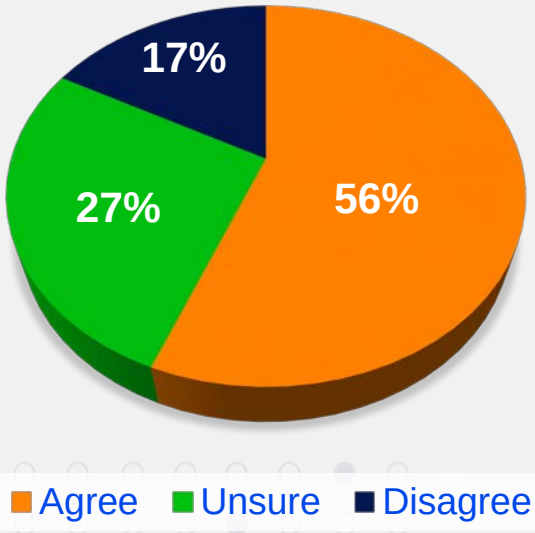
Piel FB, Hay SI, Gupta S, Weatherall DJ, Williams TN (2013) Global Burden of Sickle Cell Anaemia in Children under Five, 2010–2050: Modelling Based on Demographics, Excess Mortality, and Interventions. PLoS Med 10(7): e1001484. https://doi.org/10.1371/journal.pmed.1001484

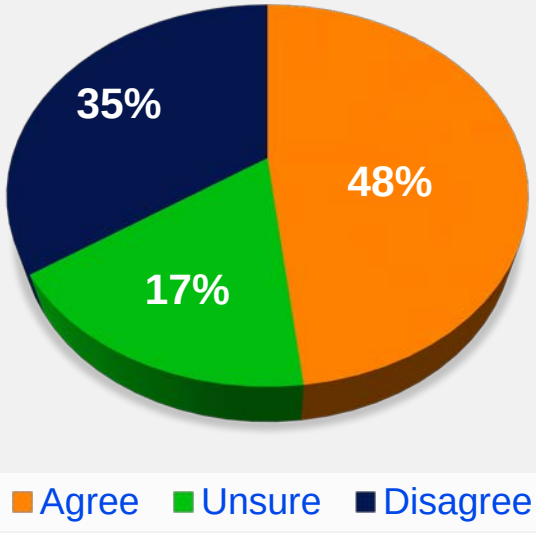
SURVEY RESULTS

If equal access to human gene editing cannot be provided to all, it should not be approved for clinical medical practice.

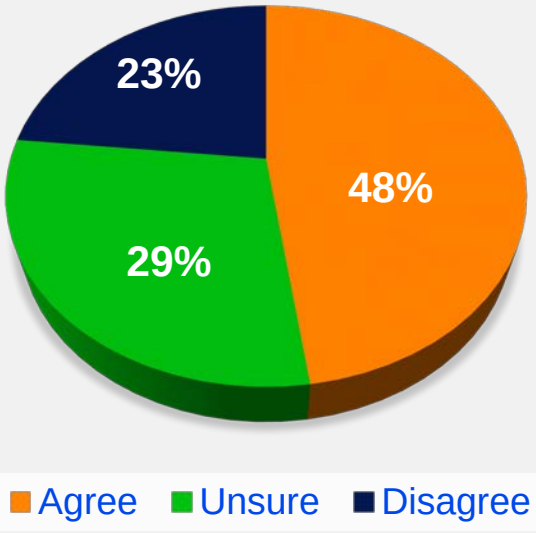
PATIENTS



PARENTS



PHYSICIANS



*1-5 Likert scale used (1=Strongly Disagree → 5=Strongly Agree)
*Scores 1 or 2 denoted as “Disagree”, scores of 3 denoted as “In the Middle”, and scores 4 or 5 denoted as “Agree”

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TAKE HOME POINTS

- **Optimism towards a gene editing curative treatment was expressed across all stakeholders due to debilitating nature of SCD and lack of existing treatments**
- **Reservations were expressed related to uncertain nature of risks, long term impact, and trial burden**
- **Apprehension related to equitable access to future clinical treatment and trustworthiness of research enterprise**
- **Stakeholders can inform the development of models of engagement whereby both the patient and research community benefit**

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Study Team

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