Motivators and Barriers for People with Sickle Cell Disease Participating in Clinical Trials: Global Findings from the LISTEN Survey

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BACKGROUND & AIMS

- The success of clinical trials of new therapies for sickle cell disease (SCD) will depend on the recruitment and retention of a large and diverse group of people with SCD.
- Qualitative studies have reported barriers to participation in clinical trials, including the potential impact on health, unmanageable study demands, limited knowledge of trials, and lack of trust in the healthcare system.[1,2]
- The **L**earnings and **I**nsights into **S**ickle Cell **T**rial **E**xperie**n**ces (**LISTEN**) Survey was developed to provide a robust and comprehensive understanding of the global barriers and motivators to participation in clinical trials for people with SCD, carers and healthcare professionals (HCPs).
- Here we present the LISTEN Survey results for people with SCD and HCPs.

METHODS

- Between October 6, 2022 and August 22, 2023, adults (≥18 years) with SCD and HCPs involved in the treatment and/or clinical research of SCD in 17 countries completed quantitative surveys (online, by telephone or face-to-face).
- People with SCD **rated** on a 7-point scale (from not at all to extremely important) and **ranked** (from most to least) the importance of specified factors when deciding whether to participate in a clinical trial for SCD. In the HCP survey, HCPs provided their perspectives on the importance of these factors to people with SCD.
- Questions were grouped into five categories: impact on daily life, treatment impact, wider clinical trial outcomes, trial information, and further considerations.
- Results presented here include the total proportion of respondents who rated factors as extremely or very important, and the proportion who ranked factors first or second.

RESULTS (1)

Respondent characteristics

- Overall, 1,506 people completed the survey comprising 1,145 people with SCD (58% female; median age [interquartile range] 30 years [24–38]) and 361 HCPs (67% hematologists and/or SCD specialists) from 17 countries (**Figure 1**).

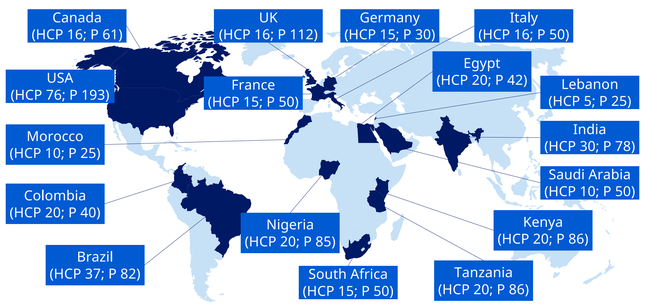

Figure 1. Respondents of the quantitative LISTEN Survey (n=1,506). HCP, healthcare professional; P, people with sickle cell disease.

Clinical trial experience

- In total, 33% of people with SCD had previously been invited to participate in a clinical trial for SCD.
- In those people who had participated (n=256/1,145; 22%), 70% reported that their experience was the same or better than they expected (**Figure 2**).

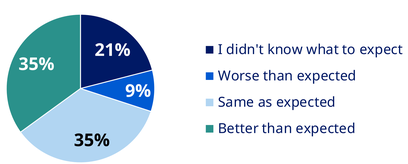

Figure 2. Experience of people with SCD participating in clinical trials (n=256) compared with their expectations. SCD, sickle cell disease.

Barriers and motivators to participation in clinical trials

- An important barrier for people with SCD was that the treatment might have different side effects than they currently experience (51%). Important motivators included the potential to better manage their symptoms (50%), the opportunity to try a new treatment that might work better (50%), and to increase their knowledge of SCD (50%; **Figure 3**).
- **Trial information:** people with SCD ranked (first or second of five) safety measures (56%) and how the treatment works (48%) as the most important factors; these were significantly more important than who is leading (29%) or funding (25%) the trial; p<0.001 for all combinations.
- **Further considerations:** people with SCD ranked (first or second of five) speaking to other people with SCD involved in the trial (49%) and experts running the trial (50%) as the most important factors.

Comparison of responses from HCPs versus people with SCD

- HCPs understated the importance of several wider clinical trial outcomes for people with SCD compared with the responses of people with SCD, including supporting treatment developments for them in the future (41% vs 49%; p=0.008) or for others with SCD (38% vs 48%; p<0.001; **Figure 4**).
- HCPs overstated the importance of impact on daily life for people with SCD compared with the responses of people with SCD, including missing school/work (51% vs 41%; p<0.001), additional effort to start trial treatment (45% vs 33%; p<0.001), and travel requirements (53% vs 37%; p<0.001).

RESULTS CONT. (2)

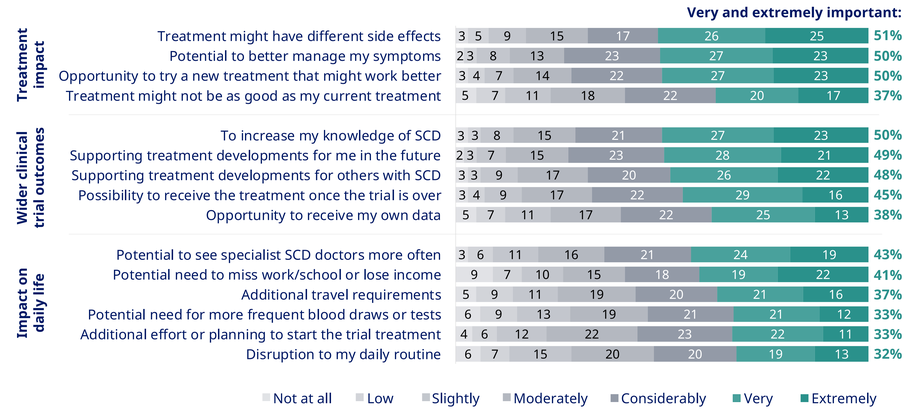

Figure 3. Proportions (%) of people with SCD (n=1,145) who rated the importance of factors in the treatment impact, wider clinical trial outcomes, and impact on daily life categories when considering whether to participate in a clinical trial for SCD. Statements have been simplified for inclusion in the graph; percentages have been rounded. SCD, sickle cell disease.

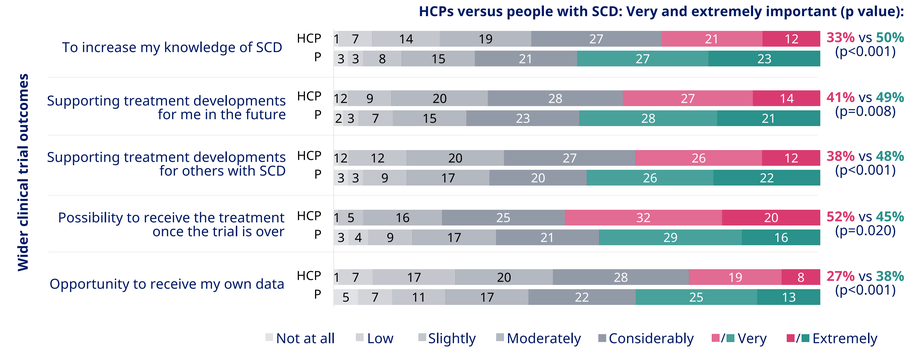

Figure 4. Proportions (%) of people with SCD (n=1,145) and HCPs (n=361) who rated the importance of factors in the wider clinical trial outcomes category when considering whether to participate in a clinical trial for SCD. Statements have been simplified for inclusion in the graph; percentages have been rounded. HCP, healthcare professional; P, people with sickle cell disease; SCD, sickle cell disease.

CONCLUSIONS

- Improving access and recruitment into clinical trials in SCD will require clear communication of the potential benefits to individuals and the wider SCD community, as well as potential safety and side effects.
- Findings from the quantitative LISTEN Survey highlight that those directly involved in the clinical trial should deliver these messages, including other people with SCD who are involved in the trial.
- Given the disconnect between people with SCD and HCPs in rating the importance of barriers and motivators to trial participation, shared decision-making may also improve understanding and increase participation in clinical trials.
- Further analyses of the survey results will be important to identify the differences in responses between regions, age groups, and other subpopulations.

REFERENCES

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