

## **Abstract: P1482**

### **Title: FACTORS DRIVING PARTICIPATION IN CLINICAL TRIALS AMONG PEOPLE WITH SICKLE CELL DISEASE DIFFER IN IMPORTANCE ACROSS GEOGRAPHIC REGIONS: FINDINGS FROM THE GLOBAL LISTEN SURVEY**

**Abstract Type: Poster Presentation**

**Topic: Sickle cell disease**

#### **Background:**

Recruiting and retaining a large and diverse group of participants is crucial to the success of global clinical trials (CTs) of new therapies for sickle cell disease (SCD). Trial populations may under-represent some regions of the world where SCD is prevalent, for reasons that are not clearly understood—limiting the generalizability of findings. The Learnings and Insights into Sickle Cell Trial Experiences (LISTEN) Survey was a global survey that identified a need to communicate the potential benefits of CT participation for individuals and the wider SCD community, as well as the anticipated safety profile, to improve access to and recruitment into CTs (James J et al. *Blood* 2023;142[Suppl 1]:2498).

#### **Aims:**

To investigate potential geographic differences in the importance of factors affecting the ability and willingness of people with SCD (PwSCD) to participate in CTs, as identified in the LISTEN Survey.

#### **Methods:**

Between October 2022 and June 2023, PwSCD ( $\geq 18$  years old) in 17 countries completed a quantitative survey assessing factors that drive the decision to participate in a CT across five categories. Respondents rated the importance of factors on a 7-point scale (from not at all to extremely important) or ranked them from most to least important. Responses are reported by region for Middle East and North Africa (MENA), Sub-Saharan Africa (SSA), South America (SA), North America (NA), Europe (EUR), and India (IN). Differences between the mean of each region and the mean of the other regions were assessed using two-proportion Z-tests.

#### **Results:**

Overall, 1,145 PwSCD (142 MENA, 307 SSA, 122 SA, 254 NA, 242 EUR, 78 IN) completed the survey; 58% were female and the median age was 30 years (interquartile range 24–38). The proportion of respondents not aware of CTs was large in IN (88%), MENA (39%), and SSA (39%), compared with 12% in SA, 5% in EUR, and 5% in NA. Prior participation in an SCD CT was reported by 37% in NA, 33% in SA, 25% in EUR, 16% in SSA, 11% in MENA, and 0% in IN. For questions about the potential impact of participation on daily life, responses were similar or had minor differences across regions. Among factors regarding impact of receiving trial treatment, the potential to better manage symptoms was rated extremely/very important by a greater proportion of respondents in SA and by a smaller proportion in IN than in other regions (Figure 1A). Most respondents in SSA placed less importance on the potential for different side effects and the treatment not being as good as their current treatment than in other regions. When considering wider trial impact, more respondents in SA and fewer respondents in SSA and IN rated supporting new treatment developments for the benefit of other PwSCD extremely/very important than in other regions. Most respondents in SA rated increasing their knowledge about SCD as extremely/very important. Among factors related to CT information, understanding planned safety measures was most often ranked first or second in importance across regions, except in SA where how the trial treatment works and in IN where who is leading the trial were ranked in the top two more often than in the other regions (Figure 1B).

#### **Summary/Conclusion:**

Awareness of and participation in CTs for SCD was low in IN, MENA, and SSA. Geographic differences in the

importance of factors motivating or discouraging PwSCD to participate in CTs may reflect differences in education needs, local culture, or confidence in the healthcare system. These geographical differences should be considered when designing trial protocols to enhance recruitment and diversity in global CTs.

**Figure 1: Proportion of PwSCD (N=1,145) within each region who (A) rated factors associated with treatment impact and wider trial impact as extremely or very important, and (B) ranked factors associated with clinical trial information as one of the two most important, when considering whether to participate in a clinical trial**

<b>(A) Treatment impact and wider trial impact (% rating as extremely/very important)</b>	<b>MENA (n=142)</b>	<b>SSA (n=307)</b>	<b>SA (n=122)</b>	<b>NA (n=254)</b>	<b>EUR (n=242)</b>	<b>IN (n=78)</b>
The potential to better manage my symptoms by taking the new treatment	46	42	74*	56	52	28*
The trial treatment might have different side effects than I currently experience	55	39*	57	58	55	53
The trial treatment might not be as good as my current treatment	45	27*	43	41	38	38
Feeling that I am supporting new treatment developments for the benefit of other PwSCD	47	31*	70*	59	53	32*
To increase my knowledge of SCD	46	44	74*	59	43	38

  

<b>(B) Clinical trial information (% ranking as one of the two most important factors)</b>	<b>MENA (n=142)</b>	<b>SSA (n=307)</b>	<b>SA (n=122)</b>	<b>NA (n=254)</b>	<b>EUR (n=242)</b>	<b>IN (n=78)</b>
What research led to the development of the trial treatment	28*	45	33	47	46	50
How the trial treatment works	39	41	68*	53	54	24*
Who is leading the trial	41*	31	25	21*	21*	56*
Which organizations are funding the trial	27	36*	19	17*	16*	37*
What safety measures will be in place	65	48	56	62	63	32*

EUR, Europe; IN, India; MENA, Middle East and North Africa; NA, North America; SA, South America; SSA, Sub-Saharan Africa; SCD, sickle cell disease; PwSCD, people with SCD  
 Darker colours indicate greater proportions; \*p<0.05 vs average of other regions

**Keywords:** Patient reported outcomes, Real world data, Sickle cell patient, Epidemiology