HemaSphere

EHA EUROPEAN HEMATOLOGY ASSOCIATION

# P1448 CHARACTERISATION OF QUALITY OF LIFE-LINKED PATIENT-REPORTED OUTCOMES THROUGH A DIGITAL AND CONTINUOUS REMOTE MONITORING ECOSYSTEM IN SICKLE CELL DISEASE.

**Topic:** 26. Sickle cell disease

Kim Summers*[1], Orlando Agrippa[1], Kofi Anie[2], Paul Telfer[3], Sanne Lugthart[4]

*[1]Sanius Health, London, United Kingdom; [2]London North West University Healthcare Nhs Trust, London, United Kingdom; [3]Queen Mary University Of London, United Kingdom; [4]University Hospitals Bristol And Weston, United Kingdom*

**Background:**

As critical components in understanding patient health, disease state, complexity, and response to both existing and novel treatments in Sickle Cell Disease (SCD), there remains a need for deeper characterisation of patient-reported outcomes (PROs) and associated measures linked to patients' daily life. While snapshots are available through clinic visits or during follow-up points during clinical trials, these typically miss patients' day-to-day lived experiences.

**Aims:**

This work aimed to track patient PROs at a longitudinal level through a remote, digital monitoring ecosystem, in order to build a real-world characterisation of EQ-5D, pain, psychological (mood), and fatigue scores by patient and by SCD subgroups.

**Methods:**

Following informed consent for enrolment within a digital data capture ecosystem, participants gained access to a PRO portal via a mobile phone application, through which day-to-day EQ-5D-5L, EQ-5D health state, pain, psychological, and fatigue scores, as well as hydration levels, were self-reported. Data for a snapshot cohort of 310 patients with at least one available PRO were analysed, including testing for differences by age band, sex, and genotype. Mean scores were calculated at a patient level across their entire period of enrolment, with correlation analysis for links between these scores.

**Results:**

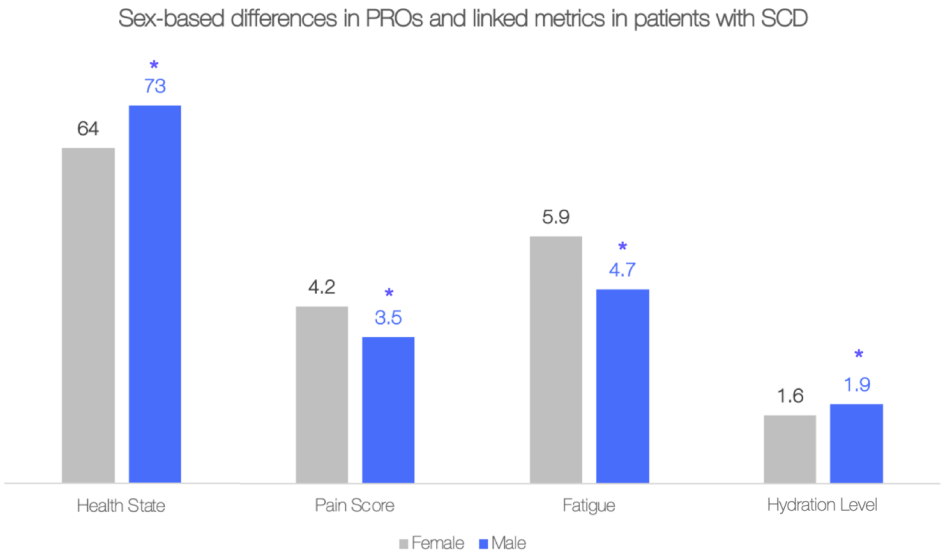
The mean age was 34 (SD 11) years, and most patients were female (73%). The HbSS genotype was most common (75%), followed by HbSC (14%), and HbS Beta + Thalassemia (5%). At a cohort level, the mean number of self-reported data points per patient for each metric was 43 (SD 79) over a mean follow-up period of 10 (SD 5) months.

Statistically significant correlations were identified between all metrics ($p < 0.001$), except for hydration levels. Higher hydration levels were found to correlate only with increasing EQ-5D health state ($p = 0.014$) and decreasing fatigue ($p = 0.035$). Increasing scores for both the EQ-5D-5L and the EQ-5D health state component were found to correlate with decreasing pain and fatigue scores, in parallel with increasing psychological scores.

Breakdown of patient means by subgroup found that EQ-5D health state scores were significantly higher in males than females (73 vs. 64, $p = 0.001$), with an associated lower mean pain score (3.5 vs. 4.2, $p = 0.020$), lower fatigue score (4.7 vs. 5.9, $p < 0.001$), and higher hydration level (1.9 vs. 1.6 L, $p = 0.006$).



**Abstract Book Citations:** Authors, Title, HemaSphere, 2023;7(S3):pages. The individual abstract DOIs can be found at https://journals.lww.com/hemasphere/pages/default.aspx.




Most identified differences were found between the 19-25 age band and other ages. Mean hydration levels were significantly lower in the 19-25 group, at 1.5 L, in comparison to 26-39-year-olds and 40-64-year-olds, at 1.8 L ($p = 0.003$). In contrast, EQ-5D-5L was higher for patients in the 19-25 age band compared to the 26-39 band, at 0.749 and 0.699, respectively ($p = 0.045$). Levels of fatigue were raised at 26-39 years (6.0), with statistical significance seen in comparison to the 40-64 group (5.0) ($p = 0.008$).

Significant differences between genotypes were seen regarding pain scores for HbS Beta + Thalassemia, at 5.5, in comparison to both HbSC ($p = 0.011$) and HbSS ($p = 0.016$) patients, who reported means of 3.8 and 4.0, respectively.

**Summary/Conclusion:** This work provides an important investigation of how patient-reported metrics link with daily factors in patients' lives and quality of life (QoL)-linked measures. With significant subgroup differences at baseline, including lower health state scores in females correlating with higher pain and fatigue, this may indicate a need for consideration during clinical monitoring and assessment of trial outcomes.

Future work will expand this analysis to create timelines of metric correlations longitudinally. This will help establish a detailed view of day-to-day PRO changes and correlations, and any variation between patient groups.