



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

904. OUTCOMES RESEARCH-NON-MALIGNANT CONDITIONS

Longitudinal Trends in Physical Functioning Patient-Reported Outcome Domains for Children with Sickle Cell DiseaseAshima Singh, PhD¹, Mahua Dasgupta, MS², Julie A. Panepinto, MD MSPH^{3,4}, Amanda M. Brandow, DO, MS⁵¹ Department of Pediatrics, Division of Hematology and Oncology, Medical College of Wisconsin, Pewaukee, WI² Medical College of Wisconsin, Milwaukee, WI³ NIH, Division of Blood Diseases and Resources, National Heart, Lung and Blood Institute, Bethesda, MD⁴ Dr. Panepinto contributed to this article as an employee of the Medical College of Wisconsin. The views expressed are her own and do not necessarily represent the views of the National Institutes of Health or the United States Government, Bethesda, DC⁵ Department of Pediatrics, Section of Hematology/Oncology/Bone Marrow Transplantation, Medical College of Wisconsin and Children's Research Institute of Children's Wisconsin, Milwaukee, WI

Background: Children with sickle cell disease (SCD) often experience pain and have impaired physical functioning. Patient Reported Outcomes Measurement Information System (PROMIS) tools have previously been shown to be valid and responsive to varied states of health for children with SCD. However, there is limited understanding of the trend of patient-reported outcomes (PROs) over time and the factors associated with this trend and time-dependent changes. The objective of this study was to determine the longitudinal trends in physical functioning domains in children with SCD over a three-year time period and identify factors associated with worse PROs.

Methods: Our study included a convenience sample of children with SCD recruited from a tertiary care facility during 2017 - 2020. Eligible children with SCD to self-report PROs were 8 years and older and cognitively capable. Validated PROMIS measures were used to assess fatigue, physical stress experience, strength impact and functioning/mobility over time. The PRO questionnaires were administered every 6 months (with time windows to facilitate completeness of data) for three years to obtain longitudinal data. In addition, we collected clinical data including disease management strategy (hydroxyurea use, chronic transfusion), pain medication, prior acute care use for pain (3 years at baseline and 6 months at follow-up) disease complications (overt stroke, acute chest syndrome, priapism) and other comorbidities. Generalized Estimating Equations with patients as clusters were used to determine changes in PROs over time. This model used compound symmetry covariance structure to account for correlations over time and was adjusted for age and other clinical data. Stepwise regression model was used to identify the significant variables associated with PROs.

Results: We recruited 170 children with SCD into our study. Of these, 145 (85.3%) had self-report PRO data for more than one time point. The mean age of the cohort was 10.6 (SD 3.3) years, 53.1% were female. Table 1 shows the demographic and clinical characteristics of our study population. The average T-score for fatigue, physical functioning/mobility, stress experience and strength impact were 47.9, 49.1, 57.2 and 41.5 respectively. Figure 1 shows the unadjusted T-scores over time for each of the listed domains. The regression models showed there were no significant changes in scores for fatigue and physical function mobility over time. However, the stress experience [β (time in months) = -0.13 (95% CI = -0.20 - -0.07), p-value <0.0001] and strength impact B estimate [β (time in months) = 0.13 (95% CI = 0.08 - 0.18), p-value <0.0001] improved over time. Age at enrollment was significantly associated with strength impact where older kids had a higher strength impact [β (age) = 0.49 (95% CI = 0.15 - 0.83), p = 0.005]. However, age was not associated with any of the other physical functioning domains. Disease severity at baseline, as determined by acute care use in the past three years, was significantly associated with stress experience [β (3 or more acute care visits for pain vs none) = 6.32 (95% CI = 1.71 - 10.93), p-value = .007] and fatigue [β (3 or more acute care visits for pain vs none) = 5.31 (95% CI = 0.95 - 9.66), p-value = .03]. The extent of acute care use within 6 months at follow-up time points was significantly associated with worse fatigue [β (acute care for pain within past 6 months) = 2.19 (95% CI = 0.19 - 4.20), p-value = 0.03] and physical functioning/mobility [β (acute care for pain within past 6 months) = -1.76 (95% CI = -3.24 - -0.28), p-value = 0.02].

Conclusions: Fatigue and functioning/mobility did not change over time in children with SCD. There were slight improvements in stress experience and strength impact, but the clinical significance of these improvements is unknown. Disease severity, as measured by acute care use at baseline, was significantly associated with worse physical health across all domains.

Disclosures No relevant conflicts of interest to declare.

Table 1: Demographic and clinical characteristics of study population

Total N	145
Age at enrollment, mean (sd)	10.6 (3.3)
Age at enrollment	
<8	10 (27.6%)
8 – 13	66 (45.5%)
Age >=13	39 (26.9%)
Gender, % females	77 (53.1%)
SCD type	
SS/Sβ ⁰ thal	90 (62.1%)
Others	55 (37.9%)
Hydroxyurea use at one or more time points(s)	105 (72.4%)
Chronic blood transfusion	40 (27.6%)
Overt Stroke	19 (13.1%)
Acute chest syndrome	80 (55.2%)
Priapism	12 (8.3%)
Acute care within past 3 years (collected at baseline)	
none	17 (11.7%)
<3	25 (17.2%)
>=3	74 (51.0%)
Acute care use within past 6 months - at one or more follow-up time point (yes, no)	107 (73.8%)
ADI	9 (7-10)
Medical comorbidities (yes, no)*	42 (29.0%)
Neurobehavioral comorbidities (yes, no)**	19 (13.1%)

*These included asthma, celiac disease, diabetes, obesity or thyroid disease

**These included anxiety, attention-deficit/hyperactivity disorder, behavioral problems, depression, development delay, headaches, learning disability, seizure disorder, sleep disturbances or stroke

Figure 1: Trend of physical functioning domains of fatigue, functioning/mobility, stress experience, strength impact over time in children with sickle cell disease. The figure shows T-scores for the respective domains at the specific time points (approximately 6 months apart) included in the study. TP = time points

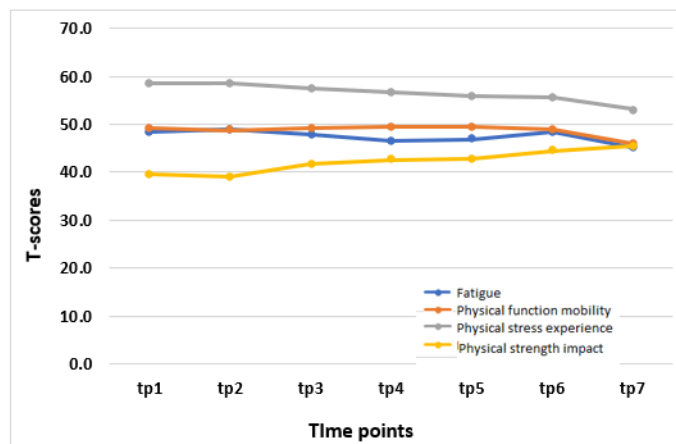


Figure 1

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