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Vaso-occlusive Painful Events in Sickle Cell Disease: Impact on Child Well-Being

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Abstract

Background—This study describes how painful events affect the health related quality of life (HRQL) of children with sickle cell disease (SCD) and determines the responsiveness of a generic HRQL measure in SCD. Our hypotheses were twofold: 1) HRQL is significantly impaired at presentation to the emergency department for a painful event and 2) PedsQL 4.0 Acute Version Generic Core Scales is responsive to change in the evolution of a painful event.

Procedure—This prospective cohort study included 57 children with SCD. HRQL was measured with the Acute Version of the PedsQL 4.0 Generic Core Scales, completed by child (self-report) and caregiver (proxy-report) at presentation and seven days post-discharge. Independent comparisons of HRQL scores were made between children in the study cohort and a published reference sample of children with SCD in baseline health (historical SCD controls).

Results—Median PedsQL scores at presentation were significantly lower than historical SCD controls in all domains for child self-report and all domains except social and school functioning in parent-proxy. Clinically and statistically significant changes in HRQL between presentation and post-discharge resulted in similar HRQL scores at seven days post-discharge to historical SCD controls.

Conclusions—The PedsQL is responsive to change; thus a useful tool to measure the impact of interventions in future SCD clinical trials. Painful events significantly diminish all domains of HRQL and this improves seven days post-discharge.

Keywords

sickle cell disease; painful events; health related quality of life; child well-being; patient reported outcomes

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Conflict of Interest The authors have no conflicts of interest to declare.

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INTRODUCTION

Sickle cell disease (SCD), the most common inherited blood disorder in the United States, is characterized by recurrent vaso-occlusive painful events that manifest as pain, acute chest syndrome or priapism [1,2]. Pain in SCD is a complex problem with significant morbidity and associated mortality [3]. Vaso-occlusive painful events are the most common reason children with SCD seek medical attention, resulting in over 16,000 hospitalizations in 2003 [4]. Because of the significant morbidity experienced by children with SCD, it is important to characterize the impact of a single vaso-occlusive painful event on a child's health related quality of life (HRQL), both from the child's perspective and from that of their parent/caregiver.

HRQL has emerged as an important area of research to evaluate the impact of chronic diseases from the perspective of the child and parent. HRQL outcomes have been investigated within a number of chronic diseases, including asthma, inflammatory bowel disease, and cancer [5-7]. In addition to being predictive of clinical outcomes and health care utilization [8], HRQL provides a way to incorporate the patient's perspective in deciding between treatments with similar efficacy [9]. Previous HRQL research in SCD has relied upon cross-sectional designs focusing on children in their baseline state of health [10-12]. There is very little information on the HRQL of children with SCD either during or following a vaso-occlusive painful event. One prior study measured HRQL upon admission for a painful event; however, the purpose of their study was to measure the association between adherence and HRQL [13]. In addition, there is no research evaluating the ability of HRQL measures to detect change over time in children with SCD. The objectives of this study were to describe the HRQL of children at the time of presentation to medical care for an acute painful event and to determine the ability of a generic HRQL measure to detect change over time in children with SCD during the evolution of a painful event. Our hypotheses were twofold: 1) the HRQL of children with SCD is significantly impaired at presentation for an acute vaso-occlusive painful event, and 2) The PedsQL 4.0 Acute Version Generic Core Scales will be responsive to change over time during the evolution of a child's painful event.

METHODS

Study Setting and Subjects

This was a prospective cohort study conducted from June 2006 through May 2007. Children ages 2 to 18 years with SCD who presented with an acute vaso-occlusive painful event to the Medical College of Wisconsin's pediatric emergency department (ED) were eligible for enrollment. Exclusion criteria were children presenting with fever only, stroke, splenic sequestration, those on chronic transfusions, non-English speaking patients, those without access to a telephone, or previous enrollment in this study. The management of the child's pain while in the ED and the decision to admit or discharge the child was at the discretion of the treating physician. Inpatient pain management was also at the discretion of the treating inpatient physician and criteria for discharge from the inpatient unit included pain controlled on oral medications.

A comparison group for the cohort of children with acute painful events was identified and will be referred to as historical SCD controls. The HRQL scores for these historical SCD controls were collected from children with SCD in baseline health at routine clinic visits as part of a separate cross-sectional study evaluating the HRQL of children with SCD compared to a control population of healthy children without SCD. The methods of this study are described in detail elsewhere.[12] The study group and historical SCD controls were drawn from the same SCD center, thus a subset of children were common to both groups.

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The institutional review board of the Children's Hospital of Wisconsin/Medical College of Wisconsin approved the study and informed consent was obtained in the ED from the parent or legal guardian and assent from the child when appropriate.

Measurements

Demographic and medical information was obtained through parental report and from review of the medical records. Upon enrollment, children were classified as having frequent or infrequent prior painful events. Children with 3 or more painful events requiring inpatient admission in the 3 years prior to study enrollment were classified as frequent and all others were classified as infrequent. Initial pain scores were obtained using age appropriate scales in ED triage to verify the child was experiencing a painful event [14-16]. All scales rate the child's pain from 0-10 with 10 representing the worst level of pain.

The primary outcome, HRQL, was measured by the Acute Version of the Pediatric Quality of Life Inventory (PedsQL 4.0) Generic Core Scales. This questionnaire differs from the standard PedsQL 4.0 Generic Core Scales in that it is designed to measure a 7 day recall period, whereas the standard version measures a one month recall period [5]. The Acute Version was used because it would better measure change in the relatively short period of time during which a painful event should improve. The acute version is a parallel report to the PedsQL 4.0 Generic Core Scales, thus allowing appropriate comparisons of the HRQL scores [17].

To date, there is no disease specific HRQL instrument for children with SCD, thus only a generic tool was used. The PedsQL 4.0 Generic Core Scales have been previously studied and validated in children with SCD in their baseline state of health.[12] The PedsQL 4.0 is comprised of parallel child self-report (ages 5 to 18 years) and parent proxy-report (ages 2 to 18 years) questionnaires. The instrument includes 23 items incorporating the following domains: physical functioning, emotional functioning, social functioning, and school functioning [18]. The tool utilizes a 5 point Likert scale (0-4) where child and parent rate how often each item has been a problem in the past 7 days (acute version) or past month (standard version) (0=never, 1=almost never, 2=sometimes, 3=often, 4=almost always). Individual item responses are reverse-scored and linearly transformed to a 0-100 scale (0=100, 1=75, 2=50, 3=25, 4=0) with higher scores indicating better HRQL. Scale scores are computed as the sum of the items divided by the number of items answered to account for missing data. A Total Score is calculated in addition to a Physical Health Summary Score and a Psychosocial Health Summary Score. The Physical Health Summary Score (8 items) is the same as the Physical Functioning Scale. To create the Psychosocial Health Summary Score (15 items), the mean is computed as the sum of the items divided by the number of the items in the Emotional, Social, and School Functioning Scales [19].

After obtaining informed consent, the PedsQL 4.0 was administered by a trained member of the research team to all eligible children and their primary caregivers in the ED. Seven days after discharge from the ED or inpatient unit, the PedsQL 4.0 was again administered via telephone interview to both the child when age appropriate and primary caregiver. The PedsQL 4.0 has been validated for administration via telephone interview [20].

DATA ANALYSIS

Descriptive statistics were calculated for all demographic variables using appropriate statistical methods. Demographic characteristics (age, gender, and frequency of prior painful events) were compared between the acute painful event cohort and the historical SCD controls using Chi-Square Tests. Due to skewed distributions for the PedsQL 4.0 summary and subscale scores, sample values are reported as medians and inter-quartile ranges.

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PedsQL 4.0 summary and subscale scores were compared using a Wilcoxon rank sum test between: 1) historical SCD controls and acute painful event cohort at presentation to the ED and 2) historical SCD controls and acute painful event cohort seven days post-discharge. Changes in HRQL between ED presentation and seven days post-discharge were assessed using Wilcoxon Signed Rank tests. We also assessed whether this change in HRQL scores met criteria for the minimal clinically important difference (MCID), the smallest change in a HRQL score that patients perceive to be clinically meaningful. The MCID has been defined for each subscale by the developer of the PedsQL 4.0 in a large feasibility, reliability, and validation study [17]. Subgroup analysis was also done using the one-sample Sign test for the 16 patients common to both the historical SCD controls and those in the study cohort to determine differences between HRQL scores in baseline health and seven days post-discharge.

All statistical analyses were conducted with SPSS version 14.0 for Windows (SPSS, Chicago, IL) and SAS v9.1.3 (SAS, Cary, NC). A p-value of ≤ 0.05 was considered statistically significant. The false discovery rate (FDR) was applied to control for false positives due to multiple testing with control set to 5% [21].

RESULTS

Study Population

A total of 95 eligible children presented to the ED during the study period. Of those eligible, 74 were approached, 61 were enrolled and 13 refused to participate. Reasons children were not approached included legal guardian not present or missed opportunities. Reasons for refusal included “already in research study” (other SCD research studies at our site), “don’t like studies” and “just not interested”. Figure 1 depicts the flow diagram for the final study population. There were 6 caregivers and 9 children who did not complete the initial PedsQL 4.0 for reasons not stated by the participant. There were 23 parents and 16 children common to both the study group and historical SCD controls. The demographics for the study cohort are shown in Table I. There were no significant differences in age, gender, or frequency of prior painful events between the historical SCD controls and those in the study cohort (data not shown).

Children’s HRQL at the time of an acute painful event is significantly impaired (Table II)

The initial median pain score in ED triage was 8 (IQR 5-9) on a 10 point scale. Table II compares the median PedsQL 4.0 scores for both the child-self and parent-proxy reports between the historical SCD controls and the acute painful event cohort at presentation. For the child-self reports, median HRQL scores were significantly lower upon presentation to the ED across all summary and subscale scores. The parent-proxy reports exhibited a similar pattern, although there were not significant differences for social and school functioning, and the median scores upon presentation to the emergency department were higher than those self-reported by the children.

The PedsQL 4.0 Acute Version Generic Cores Scales is responsive to change during the evolution of a painful event (Table III)

There was a significant increase in the median HRQL scores between the two time points for all subscales except the parent proxy-report for school functioning (Table III). In addition to a statistically significant change in the HRQL scores, all child-report subscale scores met criteria for the minimal clinically important difference (MCID) [17].

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Children's HRQL is comparable to baseline state of health in historical SCD controls 7 days after discharge (Table II)

There were no significant differences in scores obtained 7 days post-discharge for an acute painful event and those from historical SCD controls, suggesting that children return to baseline HRQL. As previously stated, there were some patients common to both the study population and historical controls, and the prior analyses do not account for the potential correlations induced by these individuals. This has the potential to underestimate parameter standard errors and thus overstate statistical significance. However, we conducted a subgroup analysis of those individuals with complete data at all three time points (baseline health, acute presentation, seven days post discharge). The results displayed a virtually identical pattern to all data presented above. In particular, the median differences [IQR] for the HRQL scores between seven days post-discharge and baseline were as follows: total score 1.1 [−10.0,13.3], p-value= 1.0; physical functioning score 9.4 [−11.7,21.9], p-value=0.45; psychosocial functioning score −0.8 [−13.3, 10.0], p-value = 1.0 (Figure 2).

DISCUSSION

To our knowledge, this is the first study to assess the HRQL of children with SCD during an acute vaso-occlusive painful event and to show the responsiveness of a generic HRQL measure in children with SCD. The HRQL of children upon presentation for an acute painful event is significantly impaired compared to children with SCD in baseline state of health. Additionally, our study is the first to demonstrate a significant improvement in HRQL over time for children with SCD supporting the responsiveness of the HRQL measure. The change in HRQL scores between ED presentation and seven days post-discharge was not only statistically significant, but also met criteria for the minimal clinically important difference (MCID) [17]. The changes in the child self-report were on the order of 3 to 8 times the published MCID for each subscale score, suggesting a substantial shift in HRQL from the child's perspective. Since most children return to baseline HRQL seven days after discharge, we believe that this could serve as an appropriate endpoint to use in clinical trials studying the outcome of painful events. Furthermore, the statistically significant and clinically meaningful change in HRQL between these two time points demonstrate the PedsQL 4.0 Generic Core Scales is responsive to change in children with SCD, thus showing its usefulness to evaluate the effect of treatment interventions on children's HRQL in future clinical trials [22].

Previous studies of HRQL in children with SCD in baseline state of health have shown HRQL is significantly impaired in the physical functioning domain compared to healthy controls [10-12,22,23]. Our study demonstrates acute painful events significantly impact a child's physical functioning and also significantly impact a child's social, emotional and school functioning. Although children in the study cohort had comparable HRQL scores at seven days post-discharge to historical SCD controls, it is vital to acknowledge that these baseline scores are still significantly impaired when compared to healthy children without SCD.

Although the child-self report and parent-proxy report scores decreased in parallel, our results show differential perceptions of HRQL based on the child and parent during the acute presentation for a painful event. There are several explanations posited in the literature for these differences, including the age of the child, the particular HRQL domain investigated, parental HRQL and well-being, and whether child pain was involved [24,25]. Specifically, for HRQL assessments involving pain domains, a prior study demonstrated parents tend to rate their child's HRQL higher than their child rated their own HRQL. This disagreement in terms of pain-related HRQL also appeared to worsen as the severity of the

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pain increased [26]. Given that pain was the predominant clinical event assessed in this study, our results appear consistent with these previous reports.

Limitations

Due to the convenience sample obtained upon presentation to the ED, the study population may have selected for a more severe cohort of patients, thus biasing the sample. However, all patients in our center utilize the ED for treatment of an acute painful event. Some children may have received pain medication before completing the PedsQL, however since the PedsQL was self-completed, the ability to read the questionnaire is implied. In addition, other studies in advanced end-stage cancer have assessed HRQL while patients are receiving pain medications [27,28] and there was a parallel decrease in HRQL scores in the child-self report and parent-proxy report suggesting that the child-self report scores are valid. Another limitation is that the comparison to historical controls was not patient specific paired data. Therefore, we were not able to control for all patient specific variables, however we did not find a difference between these groups with regards to age, gender, and frequency of prior painful events. In contrast, data collected at the time of an acute painful event and seven days post-discharge was patient specific paired data, allowing us to truly detect change between these two time points, with other patient related variables well controlled. Some missing data were missing especially in the school functioning domain since some data collection occurred during the summer months when children were not enrolled in school. This may have affected our ability to find a difference in this domain of the HRQL assessment in the parent proxy-report scores. The sample was not large enough to analyze based on different age groups and a larger sample would allow for this analysis. Finally, since the data was obtained from a single institution its generalizability could be limited.

Conclusions

Vaso-occlusive painful events significantly impact the physical and psychosocial functioning of children with SCD. HRQL significantly improves and returns to baseline low levels by seven days post-discharge. The PedsQL 4.0 Generic Core Scales is responsive to change in children with SCD, thus this HRQL tool is useful to measure the effects of treatment interventions in future clinical trials. Ultimately, prevention of these painful events and the physical and psychosocial impact they have on children's lives should improve children's overall health-related quality of life.

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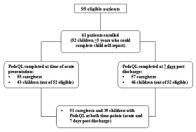

Figure 1.
Flow diagram depicting study population

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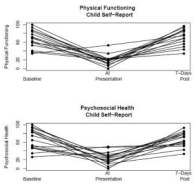

Figure 2.

Change in HRQL scores for patients common to both historical SCD controls and study cohort

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TABLE I

Demographics and Disease Characteristics of Study Cohort (n=57)

Variable	Result
Mean Age (years)	10.7 (± 4.8)
Gender	
Female	30 (52.6%)
Genotype	
HgbSS	38 (65.7%)
HgbSC	13 (22.8%)
HgbSβ+thal	4 (7.0%)
Other	2 (3.5%)
Frequency of Prior Painful Events[#]	
Infrequent	31 (54.4%)
Frequent	23 (41.8%)
Unreported	3 (5.5%)
Initial Median Pain Score in ED	8.0 (IQR 5-9)
ED Discharges	14 (24.6%)
Inpatient Discharges	43 (75.4%)
Mean Length of Stay (days)	2.0 (±1.7)

[#]Infrequent = <3 pain events requiring inpatient hospitalization in 3 years prior to study entry

Frequent = ≥3 pain events requiring inpatient hospitalization in 3 years prior to study entry

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TABLE II

Comparison of Median HRQL Scores at Time of Presentation for Acute Vaso-Occlusive Painful Event and 7 Days Post-Discharge to Historical SCD Controls Based on PedsQL4.0 Parent-Proxy Report and Child Self-Report

	Acute painful event cohort at presentation		Historical SCD controls[a]		
Scale	**n**	**Median (IQR)**	**n**	**Median (IQR)**	***P*-value[b]**
Parent-proxy report					
Total score	55	53.3 (45.7, 67.9)	104	67.4 (50.0, 83.5)	0.005
Psychosocial health[c]	55	56.7 (46.7, 73.3)	104	68.1 (52.5, 82.5)	0.011
Physical health[c]	54	51.6(40.6. 68.8)	104	68.8 (50.0, 87.5)	0.005
Emotional functioning	55	60.0 (45.0, 80.0)	104	72.5 (60.0. 90.0)	0.004
Social functioning	55	65.0 (45.0, 80.0)	104	75.0 (55.0. 90.0)	0.100
School functioning	53	45.0 (35.0, 58.3)	97	55.0 (40.0, 70.0)	0.106
Child self-report					
Total score	43	28.3 (19.6, 37.0)	78	68.3 (53.3, 79.3)	<0.001
Psychosocial health[c]	43	28.3 (19.6, 40.0)	78	65.8 (50.0, 83.3)	<0.001
Physical health[c]	43	21.9 (16.7, 34.4)	78	68.8 (56.3, 81.3)	<0.001
Emotional functioning	43	30.0 (15.0, 50.0)	78	65.8 (55.0, 85.0)	<0.001
Social functioning	43	20.0 (0.0, 35.0)	77	80.0 (55.0, 95.0)	<0.001
School functioning	42	40.0 (25.0, 45.0)	77	56.3 (40.0, 75.0)	<0.001

	Acute painful event cohort 7 days post-discharge		Historical SCD controls[a]		
Scale	**n**	**Median (IQR)**	**n**	**Median (IQR)**	***P*-value[b]**
Parent-proxy report					
Total score	57	69.6 (57.1, 81.5)	104	67.4 (50.0, 83.5)	0.521
Psychosocial health	57	70.0 (58.3, 80.8)	104	68.1 (52.5, 82.5)	0.670
Physical health	57	71.9 (57.1. 84.4)	104	68.8 (50.0, 87.5)	0.448
Emotional functioning	57	70.0 (60.0, 85.0)	104	72.5 (60.0, 90.0)	0.852
Social functioning	57	75.0 (58.3, 100.0)	104	75.0 (55.0, 90.0)	0.268
School functioning	48	55.0 (40.0, 70.0)	97	55.0 (40.0, 70.0)	0.975
Child self-report					
Total score	46	69.6 (57.6, 77.2)	78	68.3 (53.3, 79.3)	0.928

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	Acute painful event cohort 7 days post-discharge		Historical SCD controls[a]		
Scale	**n**	**Median (IQR)**	**n**	**Median (IQR)**	***P*-value[b]**
Psychosocial health[c]	46	70.0 (55.0, 78.3)	78	65.8 (50.0, 83.3)	0.875
Physical health[c]	46	68.8 (56.3, 81.3)	78	68.8 (56.3, 81.3)	0.891
Emotional functioning	46	72.5 (60.0, 85.0)	78	65.8 (55.0, 85.0)	0.535
Social functioning	46	75.0 (60.0, 90.0)	77	80.0 (55.0, 95.0)	0.933
School functioning	40	55.0 (45.0, 67.5)	77	56.3 (40.0, 75.0)	0.833

IQR, interquartile range.

[a]Historical SCD controls from Panepinto et al. [12];

[b]False discovery rate adjusted*P*-values based on Wilcoxon two-sample test;

[c]Psychosocial Health—Psychosocial Health Summary Score, Physical Health—Physical Health Summary Score, HRQL—Health-Related Quality of Life.

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TABLE III

Change in Child's HRQL following Vaso-occlusive Painful Event based on PedsQL 4.0 Parent Proxy-Report and Child Self-Report

	Change in HRQL 7 Days Post-discharge		
Scale	**n**	**Median (IQR)**	**p†**
PARENT PROXY-REPORT			
Total Score	51	8.7 (2.2, 20.2)	**<0.001**
Psychosocial Health	51	5.0 (1.7, 16.7)	**<0.001**
Physical Health	50	15.6 (0.0, 28.1)	**<0.001**
Emotional Functioning	51	5.0 (0.0, 20.0)	**<0.001**
Social Functioning	51	5.0 (0.0, 15.0)	**<0.001**
School Functioning	44	0.0 (0.0, 9.2)	0.097
CHILD SELF-REPORT			
Total Score	39	43.5 (19.6, 54.3)	**<0.001**
Psychosocial Health	39	41.7 (23.3, 55.0)	**<0.001**
Physical Health	39	37.5 (21.8, 68.8)	**<0.001**
Emotional Functioning	39	40.0 (15.0, 60.0)	**<0.001**
Social Functioning	39	60.0 (20.0, 90.0)	**<0.001**
School Functioning	35	15.0 (0.0, 35.0)	**<0.001**

† False Discovery Rate adjusted p-values from Wilcoxon rank-sum test; QR – Interquartile Range