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Determinants of quality of life in children with colorectal diseases☆

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ABSTRACT

Background: Health related Quality of Life (HRQoL) is an important outcome in medical care. The aim of our study was to identify characteristics associated with lower HRQoL scores in children with anorectal malformation (ARM) and Hirschsprung disease (HD).
Methods: Patients younger than 18 years, with HD or ARM, who were evaluated at our center from April 2014 to August 2015, were identified. The results of comprehensive questionnaires regarding diagnosis, symptoms, comorbidities and previous medical/surgical history, and validated tools to assess urinary status, stooling status and HRQoL were evaluated.
Results: In children aged 0–4 years, vomiting and abdominal distension were found to be associated with a significant reduction in total HRQoL scores. In children >4 years of age, vomiting, nausea, abdominal distension, and abdominal pain were also associated with a significantly lower HRQoL. The strongest predictor of lower HRQoL scores on regression tree analysis, in all age groups, was the presence of a psychological, behavioral or developmental comorbidity.
Conclusion: Patients with either HD or an ARM who have a psychological, behavioral or developmental problem experience significantly lower HRQoL than children without such problems, suggesting that provision of behavioral/developmental support as part of the multidisciplinary care of these children may have a substantial impact on their HRQoL.

Health related Quality of Life (HRQoL) has been established as an important patient reported outcome in medical care, especially for patients with chronic diseases where complete recovery may never be achieved [1,2]. Patients with anorectal malformations (ARMs), Hirschsprung Disease (HD) or other complex colorectal diagnoses are a particular focus in the pediatric surgical literature as they are known to encounter diverse problems affecting HRQoL. These problems include fecal incontinence, constipation, problems with toilet training, increased stool frequency, abdominal pain, and gynecological and sexual difficulties, all of which may lead to marked limitations in social functioning and emotional wellbeing in addition to the development of behavioral problems [2,3]. The surgical outcomes of HD and ARM patients have improved with modern surgical techniques, and there is increased understanding of the anatomic and physiologic derangements. Despite this however, many patients have impaired fecal and urological control, which may negatively impact their health related quality of life [4].

Information on HRQoL in children with ARMs and HD is limited owing to the rarity and heterogeneous nature of these diseases [2,5,6]. Past studies have used a variety of HRQoL assessment tools, including nonvalidated tools, and tools that do not measure at least three domains of HRQoL (e.g. physical, mental, and social functioning) [2]. Some of these issues can now be addressed because of the availability of numerous validated tools to measure HRQoL in children. Currently, the most widely used tool to measure HRQoL in children is the PedsQL™ Generic Core Scales developed in the 1990s by James Varni [7]. In addition to these HRQoL scores, validated tools have been developed to assess bowel and urinary function [8,9]. The aim of our study was to identify characteristics associated with lower HRQoL scores in children with ARM and HD, thereby facilitating an understanding of which interventions might improve HRQoL in these patients.

1. Methods

1.1. Study population

The Center for Colorectal and Pelvic Reconstruction at our institution is a quaternary referral center for children with complex colorectal

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diagnoses including HD, ARM and functional constipation. The center has a comprehensive prospective clinical registry into which patients are enrolled that interfaces with the hospital electronic health record to allow systematic reporting of patient data during routine patient care interactions. Prior to their initial appointment, patients and their families are asked to complete an extensive questionnaire providing information on their underlying diagnosis, current symptoms, comorbidities and previous medical and surgical history. They also complete a series of validated tools to assess functional urinary and stooling status and HRQoL (parent-reported and self-reported surveys). Only parent-proxy surveys were examined in this study owing to the young age of most patients and concerns about the quality of the self-reported survey data as most surveys were completed online, so it could not be verified that the patient rather than the parent truly completed the self-report survey.

All patients with the diagnosis of HD and ARM who were younger than 18 years and seen in the outpatient clinic between April 2014 and August 2015 were included. All patients were consented at the time of enrollment into the Patient Registry using an opt-out consent process. This study was approved by the Institutional Review Board at our institution.

1.2. Quality of life measures

Parent-reported HRQoL scores were assessed using the PedsQL™ Pediatric Quality of Life inventory [7,10]. The PedsQL is a modular instrument designed to measure HRQoL in healthy and acutely or chronically ill children and adolescents, aged 0–18 years. These PedsQL Generic Score Scales are age specific and include parent reported versions for all age groups: PedsQL Infant Scales (Infant 1–12 months, Infants 13–24 months) and PedsQL Generic Score Scales (Toddlers 2–4 years, Young children 5–7 years, Children 8–12 years and Teens 13–17 years). Therefore for children aged 0–2 years, the PedsQL Infant Scales were used which measure 5 domains: physical, emotional, social, cognitive functioning, and physical symptoms. For children aged 2–17 years, the PedsQL 4.0 Generic Core Scales-Child and Parent Report module was used which measures four domains: physical, emotional, social and school functioning. For the purpose of this analysis, total parent-reported HRQoL scores as well as physical (physical function) and psychosocial (sum of emotional, social and school functioning) HRQoL scores were examined.

1.3. Bowel and urinary function scales

The Vancouver dysfunctional elimination syndrome/non-neurogenic lower urinary tract dysfunction (NLUTD/DES) questionnaire was administered to assess urinary functional outcomes, including urinary accidents, nocturnal enuresis, dysuria and hesitancy [8]. The Vancouver NLUTD/DES questionnaire is a 14 item survey designed to be self-administered by children 9 years and older, and self-administered by parents of children less than 9 years of age. The questionnaire uses a 5-point Likert scale. Scores range from 0 to 52 with lower scores indicating better urinary function. The developers of the questionnaire suggest a total score of 11 as the optimal cutoff score to diagnose NLUTD/DES [8].

The Baylor Continence Scale (BCS) was used to assess bowel function because it is a validated tool that is able to differentiate between normal children and those with various underlying conditions, including anorectal malformations and idiopathic constipation/enuresis [9]. This scale is a self-administered 23-item parent reported survey which measures social continence in children with ARM after surgical correction. It uses a 4-point Likert response scale. Scores range from 2 to 92 with lower scores indicating better social continence. In the report describing the development and validation of this survey, normal children without anatomic or functional continence problems were found to have an average BCS score of 11.5 whereas those with an anorectal malformation had an average BCS of 28.

1.4. Other patient characteristics evaluated

Patient characteristics evaluated in this study included age, gender, adoption status and the diagnosis of HD or ARM. In addition, parents were asked whether their child currently had specific gastrointestinal symptoms including nausea, vomiting, abdominal pain and distension, constipation and fecal impaction, stool and urinary accidents, early satiety and urinary tract infection. The presence of a variety of comorbidities was also evaluated and these are categorized in Table 1.

1.5. Statistical analysis

In order to account for the fact that many normal children younger than 4 years are not yet toilet trained, all analyses were stratified by age. Separate analyses were performed in patients aged 0–4 years (infant and toddler) and patients 5–17 years (young child, child and teen). Parent-reported HRQoL scores were described using means and standard deviations (SDs) for the overall patient population and across groups defined by the various patient demographic and clinical characteristics evaluated. In order to evaluate whether HRQoL differed across groups defined by patient demographic and clinical characteristics, HRQoL scores were compared across patient characteristics using t-tests or analysis of variance (ANOVA). In order to identify the most important predictors of QOL among all patient characteristics evaluated, regression tree analysis was performed in lieu of traditional multivariable regression modeling, owing to expected collinearity and interactions among the evaluated variables. A regression tree involves recursive binary partitioning such that, at each split, all predictors and all possible cut point values for each of the predictors are considered, then the predictor and cut point that minimize the residual sum of squares (i.e. explain the largest amount of remaining variability) of the resulting tree are chosen. This is done repeatedly until each "leaf" in the tree reaches a minimum number of observations. Finally, 10-fold cross validation is used to reduce the size of the tree in order to achieve an optimal model without overfitting the data [11]. Since this was an exploratory study, no adjustments were made for multiple comparisons and a p-value of 0.05 was considered to be statistically significant. SAS version 9.3 (SAS Institute Inc., Cary, NC) and R version 2.15.1 (R Foundations for Statistical Computing, Vienna Austria) were used for analyses.

2. Results

2.1. Cohort description

A total of 549 patients younger than 18 years were enrolled in the patient registry during the study period, 353 of which had a known diagnosis of HD or ARM. Of these, 325 (92.1%) had parent-reported HRQoL data and were thus included in this study. The overall mean (SD) of parent reported total HRQoL in this cohort was 74.2 (17.5). 244/325 (75.1%) were in the 0–7 year age range.

2.2. Patients aged 0–4 years

Average total HRQoL scores across groups based on key demographic and clinical characteristics in the 185 patients between age 0 and 4 years are shown in Table 2. Only demographic information, and symptoms and comorbidities for which significant differences emerged in total HRQoL are shown in Table 1. The overall mean (SD) of parent reported total HRQoL in this entire cohort was 80.2 (14.4). Total HRQoL scores differed significantly by age with children aged 2–4 having higher parent-reported QOL than children aged 0–24 months, but no differences were seen in total HRQoL scores by gender, adoption status or HD vs. ARM diagnosis.

Significant differences emerged in the total HRQoL score in children with vomiting and abdominal distension; however, no significant