Health-related Quality of Life in Children With Prune-belly Syndrome and Their Caregivers

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OBJECTIVE To compare health-related quality of life (HRQoL) in children with prune-belly syndrome (PBS) and their caregivers to healthy controls, as children and adolescents with PBS face numerous potential physical and psychosocial challenges.

MATERIALS AND METHODS Study participants completed the Pediatric Quality of Life Inventory Generic Core Scales (PedsQL) 4.0 generic core scales (children) or Quality of Life Enjoyment and Satisfaction Questionnaire Short Form (Q-LES-Q-SF) (caregivers) in an online, anonymous format. The PedsQL 4.0 is a 23-item, age-adjusted, validated questionnaire that assesses physical, emotional, social, and school functioning in pediatric patients. The Q-LES-Q-SF is a validated, self-report measure that assesses various areas of daily functioning in adults.

RESULTS PedsQL 4.0 was completed by 32 children with PBS. Individual physical (66.3 ± 20 vs 84.4 ± 17.3; $P < .0001$), emotional (68.4 ± 23.4 vs 80.9 ± 19.6; $P < .01$), social (63.1 ± 21.3 vs 87.4 ± 17.2; $P < .0001$), and school (53 ± 21.7 vs 78.6 ± 20.5; $P < .0001$) functioning scales were all significantly lower than in healthy children. Nineteen caregivers completed the Q-LES-Q-SF. Caregivers had a mean raw score of 54.8 ± 9.6, which was significantly lower ($P = .02$) than the comparative healthy adult cohort (59.8 ± 11.3).

CONCLUSION PBS profoundly impacts HRQoL in children, negatively affecting physical, emotional, social, and school functioning. Caregivers of PBS patients also report an overall lower quality of life, highlighting the challenges that families with chronically ill children often face. UROLOGY 87: 224–227, 2016. © 2015 Elsevier Inc.

Prune-belly syndrome (PBS) is characterized by a deficiency of abdominal wall musculature, bilateral intra-abdominal testes, and urinary tract abnormalities including megacystis, hydroureteronephrosis, and renal dysplasia. There is wide variability in disease severity and patients may also have concomitant cardiopulmonary, gastrointestinal, and/or musculoskeletal anomalies.[1-3] Our experience in a large contemporary single center series of children with PBS emphasized the continued need for numerous surgical interventions to correct associated urologic anomalies and address comorbidities with testicular, bladder, and ureteral surgeries.[4] The current study was designed to address the concomitant stress this has upon the patient and their caregivers.

Children with chronic disease often experience physical and/or cognitive limitations, increasing their dependence on family-provided healthcare.[5] Chronic health conditions are often associated with fatigue, reduced activity level, and emotional distress, which may affect a child's well-being.[6] Health-related quality of life (HRQoL) of pediatric patients is increasingly recognized as a salient outcome measure complementing traditional health indicators such as survival.[7] The complex, multisystem nature of PBS, coupled with the potential for renal failure and the need for reconstructive surgery, places affected children and their caregivers at significant risk. In the current study, we compared HRQoL in children with PBS and their caregivers to healthy controls, and hypothesized that PBS would negatively impact self-reported quality of life.

MATERIALS AND METHODS

Institutional review board approval was obtained. Patients were recruited via the PBS Network (http://www.prunebelly.org) from May 1 to August 31, 2014. Study participants completed the PedsQL 4.0 generic core scales (children) or the Quality of Life Enjoyment and Satisfaction Questionnaire Short Form (Q-LES-Q-SF) (caregivers) in an online, anonymous format. Pediatric Quality of Life Inventory Generic Core Scales (PedsQL) 4.0 is a 23-item, age-adjusted (children aged 2-18 years), validated

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questionnaire that assesses physical, emotional, social, and school functioning with parallel child self-report and parent-proxy report. Scoring ranges from 0 to 100 in each of the 4 scales, with higher values indicating a better quality of life. Comparative healthy children sample was derived from the PedsQL 4.0 database.[8]

The Q-LES-Q-SF is a validated, self-report measure that assesses satisfaction with general activities and/or daily functioning in adults, where normal quality of life is defined as within 10% of the recognized community norm (raw score 59.8; 81.8% maximum score).[9,10] Scoring of the Q-LES-Q-SF involves summing 14 items rated on a 5-point scale to yield a raw total score, which ranges from 14 to 70. The raw total scores were then transformed into a percentage maximum possible score using the following formula: [(raw total score—minimum score)/(maximum possible raw score—minimum score)] to facilitate comparisons across areas of functioning.[9] Mean caregiver Q-LES-Q-SF scores were compared to a historical healthy adult cohort.[10]

Comparison between PBS patients and/or caregivers and healthy control cohorts was completed using an independent two-sample, two-sided *t*-test. Statistical analysis was performed using SAS (SAS Institute, Cary, NC) 9.3, with $P < .05$ representing statistical significance.

RESULTS

The PedsQL 4.0 was completed by 32 parent-proxy or children with PBS (age range 2 to 18 years). Surveys were completed for 10 children aged 2-4 years, 7 children aged 5-7 years, 8 patients aged 8-12 years, and 7 teens aged 13-18 years. All surveys for children 5 years and older were self-report, whereas surveys for children 2-4 years were completed by a parent-proxy. Total mean HRQoL score for children with PBS was 61.4 ± 18.2, significantly lower than reported values for healthy controls (83 ± 14.8; $P < .0001$). Of the 32 PBS children, 27 (84.4%) had a total score less than 75.6 (half a standard deviation below the control mean). Individual physical, emotional, social, and school functioning scales were all significantly lower than in healthy children (Table 1). There was a trend toward higher scores in surveys completed by a parent-proxy (n = 10; 66.3 ± 11.6) compared to those completed by the patient (n = 22; 58.3 ± 19.3), though it did not reach statistical significance ($P = .15$).

Nineteen primary caregivers completed the Q-LES-Q-SF. Caregivers had a mean raw score of 54.8 ± 9.6, significantly lower ($P = .02$) than the healthy adult cohort (community norm total = 59.8 ± 11.3; 81.8% maximum score). Nine PBS caregivers (47.3%) reported QoL scores significantly below the expected normal range for adults.

Table 1. PedsQL 4.0 scores for children with PBS and healthy controls

Domain	Patient (n = 32)	Control (n = 401)	*P* Value
Physical function	66.3 ± 20	84.4 ± 17.3	<.0001
Emotion function	68.4 ± 23.4	80.9 ± 19.6	<.01
Social function	63.1 ± 21.3	87.4 ± 17.2	<.0001
School function	53 ± 21.7	78.6 ± 20.5	<.0001

PBS, prune-belly syndrome; PedsQL, Pediatric Quality of Life Inventory Generic Core Scales.

Comment

Quality of life is defined as an individual's perspective on satisfaction across many realms, and HRQoL includes aspects of overall quality of life that are directly related to physical or mental health.[6] Medical and surgical advances have resulted in dramatic improvement in the health care of numerous pediatric chronic conditions, and with improved survival HRQoL has emerged as an important outcome measure in clinical trials, practice improvement strategies, and research involving children.[7,11] Comparisons between healthy children and those with chronic health conditions are useful in understanding the relative clinical impact of different conditions on HRQoL. Pediatric patients with diabetes, gastrointestinal conditions, cardiac conditions, asthma, obesity, end-stage renal disease, psychiatric disorders, cancer, rheumatologic conditions, and cerebral palsy have all self-reported impaired overall HRQoL compared to healthy children; patients with cerebral palsy reported the lowest scores (66.9 ± 16.7).[7] PBS patients reported total mean scores comparable to those of children with cerebral palsy, perhaps reflecting the multisystem nature and variability of the disease process.

Optimal management of children with PBS remains challenging, owing to the rarity and complex nature of the disease, and the initial course is often dictated by the severity of comorbidities.[1,2,12,13] Perinatal mortality is largely attributed to the degree of prematurity and pulmonary hypospasia.[1,14] Children with PBS require multiple procedures to correct associated urologic anomalies and address comorbidities.[4,15] Not surprisingly, our results indicate that children with PBS have lower overall HRQoL scores than their healthy peers. It has been shown the threshold of discrimination for changes in HRQoL for chronic diseases appears to be approximately half a standard deviation.[16] The difference we observed appears to be clinically significant, as 84.4% of PBS children scored below half a standard deviation below the mean for healthy controls. Furthermore, individual physical, emotional, social, and school functioning scores were all significantly lower than in healthy children. As more patients survive the neonatal period, the timing of urinary tract reconstruction and abdominoplasty in these children remains a source of debate; however, the issue of quality of life is yet to be thoroughly addressed.

Abdominal wall reconstruction plays a significant role in treatment, and elevated self-esteem with improved abdominal contour approaching normal is well established.[17,18] However, abdominoplasty in the PBS population cannot be solely classified as "aesthetic surgery" as there are well-recognized functional and physiological implications of the procedure. Early simultaneous correction of PBS anomalies with individualized urinary tract reconstruction, orchidopexy, and abdominoplasty also has been described.[19]

In addition to potential decreased quality of life associated with physical deformity, children with PBS are also at risk for chronic kidney disease. Renal dysplasia is common, and approximately 40% to 50% of patients will ultimately require renal replacement therapy.[20] The impact