Health-related Quality of Life in Boys with Barth Syndrome: Using both Child Self-reports and Parent Proxy-reports

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BACKGROUND

❖ Quality of life (QoL) is defined by the World Health Organization (WHO) as, “an individual's perception of their position in life in the context of the culture and value systems in which they live and in relation to their goals, expectations, standards and concerns” [1]. As a distinct component of QoL, health-related quality of life (HRQoL) focuses on the impact of disease and treatment on disability and daily functioning [2]. As the survival rates of life-threatening conditions have increased, the focus of medical services has shifted to not only evaluating treatment outcomes, but also HRQoL [3].

❖ The HRQoL of children with disabilities can be measured through both child self-reports and parent proxy-reports. Parent proxy-reports can supplement HRQoL child self-reports [4], especially when a child is too young or sick to complete the assessment. Research indicates that parents can assume the values and preferences of their child in parent proxy-reports [5]. However, many studies have reported discrepancies between these reports [4], as parents of children with disabilities tend to report lower HRQoL for their children than the children do for themselves [6]. One of the reasons for the inconsistencies between child self-reports and parent proxy-reports is a lack of well-established statistical methods.

PURPOSE

❖ Currently, most research on boys with Barth syndrome (Barth) has focused on their physical symptoms, and only limited studies have been performed regarding health-related quality of life.

❖ The purpose of this cross-sectional study is to **1) examine HRQoL in boys with Barth using both child self-reports and parent proxy-reports and 2) investigate the level of agreement in HRQoL of boys with Barth syndrome between child self-reports and parent proxy-reports.**

METHODS

❖ Participants
- Thirty boys with Barth syndrome and their parents.
- Age of boys: 4-17 years old (Mean: 10.6, SD: 3.8).

❖ Instrument
- The Pediatric Quality of Life Inventory Version 4.0 (PedsQL™) [7].
  - A 23-item questionnaire that assesses HRQoL.
  - Consists of a child self-report and a parent proxy-report.
    - Physical Functioning, Emotional Functioning, Social Functioning, & School Functioning.
  - 5-point scale (never=100; almost never=75; sometimes=50; often=25; almost always=0).
  - Scores range from 0 to 100, with higher scores indicating better HRQoL.

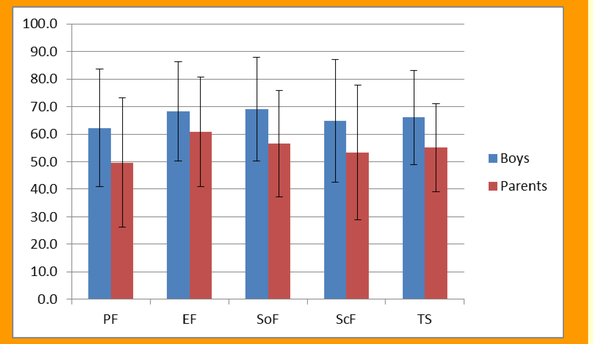
❖ Data analysis
- Intraclass correlation coefficient (ICC).
- Paired t-test (paired *t*).
- Pearson correlation coefficient (Pearson *r*).

This study was approved by the University of Florida’s Institutional Review Board. Written informed consent was obtained from the parent(s), and written assent was obtained from the children.

RESULTS

HRQoL mean scores and the level of agreement between children and parents

	Boys with Barth syndrome		Parents		Pearson *r*	ICC+
	Mean	SD	Mean	SD		
Physical Functioning*	62.2	21.3	49.7	23.4	0.625*	0.705
Emotional Functioning*	68.3	18.1	60.8	19.8	0.668*	0.768
Social Functioning*	69.0	18.8	56.6	19.3	0.479*	0.572
School Functioning*	64.8	22.2	53.3	24.5	0.564*	0.673
Total Composite Score*	66.1	17.1	55.1	16.1	0.722*	0.747



* $p < 0.05$
+ ICC of ≤ 0.40 = poor-to-fair agreement, 0.41 to 0.60 = moderate agreement, 0.61 to 0.80 = good agreement, and 0.81 to 1.00 = excellent agreement [8]

❖ The mean scores of boys with Barth syndrome and parents fell well below the clinically meaningful cutoff scores for all domains and overall HRQoL [9].

❖ The level of agreement of the ICC was good between children and parents, except for Social Functioning.

❖ Both the mean difference and the Pearson *r* were significant for all domains and overall HRQoL. These results indicated that the ratings of children and parents were consistent in terms of rank order; however, child self-reports and parent proxy-reports did not obtain the same score.

DISCUSSION & CONCLUSION

❖ The findings indicate that parents reported lower HRQoL for their children than the children did for themselves, especially in Social Functioning. These results suggest that parents may anticipate a more negative effect from the disability than their child actually experiences.

❖ The findings suggest the need to assess both a child self-report and a parent proxy-report when measuring a child’s HRQoL in both clinical and research applications.

❖ The results may provide further information for health professionals when planning therapy goals and family-centered services.

❖ Additional research is needed to better understand functional and clinical implications to observe discrepancies between child and parent reports.

❖ Additional research is needed to examine how child and parent factors, such as the child’s age and parents’ HRQoL, may affect the level of agreement between child self-reports and parent proxy-reports.

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