Quality of Life in Duchenne Muscular Dystrophy

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What's to come....

- Boys with Duchenne muscular dystrophy need to experience more challenge in their daily life

- "Just right" challenge may provide a useful framework to improve quality of life for boys with DMD
Background

- DMD is an X-linked fatal muscle disease with an incidence of 1 in 3,500 males

- Considerable research into curative therapies for DMD

- Understanding the impact of the disease on daily life is important
What is quality of life?

Defined by many, the WHOQOL group:

“individuals perceptions 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”

Quality of Life in Children

- We are currently expanding measurement of QoL in children

- Interestingly, the degree of physical disability of is not always directly related to an individual's QoL

- Self-report is accepted as the gold standard for measuring QoL

- Issues with proxy reporting (parents and health professionals)
Why QoL?

- Understanding QoL in children can assist in making informed judgements about the impact of treatment and disease progression.

- Quality of life is an important concept to explore in youth with disabilities as they typically face obstacles and restrictions to their daily life experiences that inhibit participation.
Quality of Life in DMD

- 35 parents and their sons participated from NSW, QLD and VIC

- Parents underestimated their sons’ quality of life

- What effect does such underestimation have on parents’ decision-making, particularly with regard to major or life threatening/sustaining interventions
Quality of life in different disease groups measured on the PedsQL™

- Physical Functioning
- Emotional Functioning
- Social Functioning
- School Functioning

Healthy
Cancer
Cerebral Palsy
DMD
Experience Sampling Method

- The ESM is a time-sampling of self reports developed to study the dynamic process of person-environment interactions

(Csikszentmihalyi & Larson, 1984; Delespaul, 1995).
The ESM is a useful tool in occupational therapy research as it facilitates in-depth exploration of daily occupations and subjective accounts of experiences, which are not available with other research methods (Farnworth, Mostert, Harrison, & Worrell 1996).
Experience Sampling Method

- Occurs in natural setting, and samples individuals throughout the day using a PDA or beeper

- Does not rely on retrospective recall as it occurs in ‘real time’

- Can provide detailed data on daily life experiences

- The ESM is a well established and effective means of obtaining self-reported real-time data
Where were they?
Is the activity interesting?

- Not at all: 24%
- A Little: 19%
- Somewhat: 23%
- Very much: 34%
What the boys' were doing when they were beeped

- School work: 12%
- Home work: 6%
- Playing games: 24%
- Nothing: 5%
- Travelling: 1%
- Sleeping: 6%
- Watching TV: 14%
- Eating: 3%
- Toilet or shower: 2%
- Talking: 12%
- Chores: 1%
Were others expecting a lot from you?

- Not at all: 58%
- A little: 17%
- Somewhat: 13%
- Very much: 13%
At School

![Bar chart showing the mean values of difficulty, skill, and feelings about self in response to different others' expectations.](chart.png)
Discussion

- Understanding quality of life from the boys’ viewpoint can assist parents and health professionals in making informed judgements about future therapies.

- Self-report should be included in studies of QoL in this population.
Future Directions

- Participation is defined by the World Health Organisation (WHO) [33] as “involvement in a particular life situation” or “the lived experience’ of people in the actual context” (p.123)

- When defined as involvement or experience, the link between QoL and Participation is apparent
Future directions

- Challenge is to understand relationship between participation and QoL

- Exploration of measurement that explore this construct further
Boys with Duchenne muscular dystrophy need to experience more challenge in their daily life.

“Just right” challenge may provide a useful framework to improve quality of life for boys with DMD.
Thank-you

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