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Development Of A Measure Of The Quality Of Life Of Parents Caring For A Child With A Lysosomal Storage Disorder (LSD).

Abstract

Aim: This presentation discusses the development of a new instrument to measure the quality of life (QoL) of parents caring for a child with a lysosomal storage disorder (LSD).

Background: LSDs are a group of more than 40 inherited disorders with a prevalence of 1 in 5,000 live births. LSDs are characterised by the lack of a specific enzyme in the lysosome of the cell. In a normal cell, the lysosome recycles waste products. When this enzyme is missing, waste products build up and retard the functioning of the cell and the organ to which it belongs.

Most LSD patients present with clinical symptoms and are diagnosed in their first five to ten years of life. Clinical problems are progressive and may include changes in facial appearance, bone deformities and joint stiffness, loss of skills, behaviour problems and mental retardation, respiratory infections and heart disease, and enlarged liver and spleen. Some patients may survive into adulthood, but patients who are more severely affected die in their mid-teens or earlier following a period of severe disability during which they need 24-hour care.

The diagnosis of LSDs by doctors from test results provide only limited information about how these disorders impact on the broader day-to-day lives of parents. The lack of instruments available also impedes the development of interventions designed to improve the QoL of these children and their families. Without a questionnaire, it is difficult to evaluate how new interventions prolonging the lives of these children may impact on parents.

Methodology: Following a thorough literature review, likely QoL domains were derived and a series of questions relevant to these domains were used to generate transcripts recorded during focus groups with parents caring for a child with an LSD in Adelaide and Sydney. A content analysis of the transcripts was performed and an initial pool of items was generated and formulated into a pilot questionnaire. The pilot questionnaire allowed parents to rate whether the issue mentioned in the item had occurred (frequency rating), and how much of a 'bother' it was (bother rating). This questionnaire was then administered to parents participating in the focus groups and an additional group of parents in Melbourne (total n=50) in order to reduce the item pool and determine face validity of items.

Results: Utilising frequency and bother ratings generated for each item, as well as item construct correlations, the item pool was reduced and formulated into a 50-item and a 100-item final version of the questionnaire entitled 'The Inventory of Quality of Life for Parents Caring for a Child with a Chronic Illness' (the PARQoL). These final versions are currently being employed in a study of parents whose children have LSDs in the United Kingdom.

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