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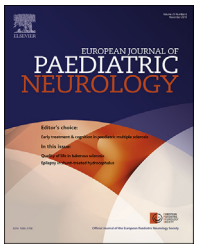

Editorial

Quality of life for children and families affected by neurological disorders

Dramatic advances in the diagnosis of neurological disorders – not least of which within radiology, genomics and proteomics, has allowed the development of "precision" therapies. Although some of these e.g. gene therapy may be almost completely curative, in most cases they will alleviate the disorder resulting in a lower level of disability-which may or may not progress.

Modern targeted therapies for both common and rarer disorders have significant financial implications, either due to the high cost of single treatments or the cumulative numbers involved. Balancing their cost with the benefit to specific populations and hence society at large, can be challenging. Funders are keen to receive data on cost versus efficacy and the lack of or inadequate data can preclude prescription.[1,2] Even when there is robust data, then improvements in function considered worthwhile by clinicians and families, may be considered inadequate to merit the cost.[3]

However, in our post truth world- what is efficacy?

Within its assessment, the effect on the quality of life (QOL) of children and their families is often cited. However, there are many different tools that can be used. Most are standardised on a relatively narrow age and/or intellectual ability range-which may not detect the changes in children with neurodisabilities that families consider worthwhile. Equally, simple questions/Likert scales are vulnerable to placebo bias.

In this issue - Amin et Al. report the significant impact of a relatively common neurological disorder – tuberous sclerosis- on quality of life.[4] Their paper has a number of strengths.

Firstly, they reviewed the appropriateness of testing and which tools to use in QOL for their large and diverse population. They further stratified to consider factors that may independently influence it. In particular, they noted the significant impact of the disorder- independent to age and intellectual disability, although both of those variables have separate and further impact.

They reported the challenges in the assessment, including whether the study population represents the wider patient group, selection of tool to assess quality of life and in particular the difficulty hearing the voice of children/adults with moderate or severe intellectual disability.

Lastly, they controlled their findings noting the significantly worse QOL for children/their families with TS when compared against those affected by asthma, diabetes, cancer and inflammatory bowel disease.

Assessing the effect of neurological diseases on QOL will become more important as precision therapies become available, and will help us justify the resources needed. We need to carefully consider which tools need to be developed and their use in which families.

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