Endocrine (2018) 62:1–2
https://doi.org/10.1007/s12020-018-1654-9

EDITORIAL

Clinical outcomes of childhood craniopharyngioma: can we do better?

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Received: 19 April 2018 / Accepted: 12 June 2018 / Published online: 22 June 2018

Patient-oriented instruments like questionnaires to evaluate a patient's health-related quality of life (QoL) have become progressively important in recent times, both from a clinical and regulatory points of view [1–3]. For patients, improving their subjective every day-to-day living is of primordial importance, that is, that they can assume their personal, social and family aspirations, as well as their schooling or working responsibilities and enjoy their leisure time with hobbies or sports activities. If a disease determines impairments or problems like chronic pain, extreme fatigability, loss of vision, difficulties in concentrating or the need for frequent medical check-ups or complex therapeutic regimens, this will mean that the affected individuals will have to change their activities, because they cannot do them anymore due to the physical or psychological issues they are experiencing. Some adapt to their limitations more easily than others, take up new hobbies or accept that their personal or professional aims require a change, and usually will experience a better QoL than those who do not adapt to this new situation. In this latter case, depression often occurs, further negatively impacting on QoL.

Craniopharyngioma is a histologically benign condition, which, however, is associated with higher morbidity and mortality rates compared to both the general population and with other causes of hypopituitarism. Hypothalamic involvement with ensuing obesity and impaired growth leading to an abnormal body image, as well as loss of vision and impaired pituitary function all tend to affect QoL in a negative way. Growth hormone (GH) deficiency, if present, usually impairs QoL further, whatever the underlying disease [4–7]; in contrast, if QoL is impaired, starting GH substitution therapy leads to improvement after several months, which persists for years [8, 9].

In this new article from the German KRANIOPHARYNGEOM 2007 study [10], which includes nearly 200 patients diagnosed with a childhood-onset craniopharyngioma followed prospectively, 47 were selected; they had been diagnosed of a craniopharyngioma above the age of 5 years and after baseline assessment (within 3 months of diagnosis), had provided QoL evaluation at both 1 and 3 years after diagnosis and surgery. Postoperative endocrine assessment revealed GH deficiency, subsequently treated with GH substitution therapy by the treating physician in 28 patients, while in the other 19 patients, substitution therapy was not prescribed. Both groups (treated or not with GH) were comparable for gender, age at diagnosis, last known functional capacity, additional endocrine deficits, body mass index (BMI) and height standard deviation score (SDS) at baseline and throughout the study period.

QoL was assessed both in the patients and their parents using the Paediatric Quality of Life (PEDQOL) questionnaire, that defines seven health-related QoL domains (self-esteem, emotional functionality, body image, cognition, physical function, social functionality in family/among friends); a high score is indicative of worse QoL assessment [11]. Furthermore, functional capacity or daily life abilities and its influence on QoL was evaluated with the German Fertigkeitenskala Münster-Heidelberg (FMH) questionnaire which evaluates the patients capacity for routine actions with 56 items, and has reference ranges with age-dependent percentiles from the normal population [12].

On the whole, parents considered the QoL of their children worse than the patients themselves. This was especially so for emotional stability (significantly worse at 3 months, 1 and 3 years after diagnosis), socialisation among friends (worse at 1 and 3 years), and body image and physical function (different at 1 year). Both patients and parents perceived autonomy, cognition and social issues related to the family similarly. When the patients' self-assessment was compared between those patients who

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finally received GH substitution therapy and those who did not, at the 1-year timepoint, the subsequently GH-treated patients referred more problems in the domains of autonomy, cognition, physical function and socialisation with friends, but not before or at the end of the 3-year period.

Why is this so? First, it is infrequent that soon after surgery GH therapy is offered to operated craniopharyngioma patients, since a minimum follow-up of several months is considered a safety precaution, to rule out possible postoperative tumour rests, relapses or recurrences. In fact, 3 months after surgery, no patient was receiving GH substitution therapy, while at 1 year, 15 out of the 28 finally treated, had begun GH. Additionally, it is probable that those patients who after diagnosis and surgery perceived more problems were offered GH-substitution therapy more often, than those who did not. This would explain why at the 1-year timepoint those who were finally treated with GH scored worse on the QoL questionnaires, despite no differences in height SDS or BMI. After GH therapy, their QoL was maintained. In the non-GH-treated patients, the domains of autonomy, cognition and physical function progressively worsened (as illustrated by higher PEDQOL scores), so that at 3 years they were significantly more impaired than at 1 year after diagnosis. Thus, at the 3-year timepoint, no differences were evident anymore, between the GH-treated and non-treated patients, on any of the domains of the PEDQOL questionnaire, since the non-GH-treated patients showed a progressive decline in QoL throughout the study period, while the GH-substituted patients who experienced worse QoL at the 1-year timepoint did not worsen anymore over time. Thus, GH therapy stabilised QoL.

The parental evaluation, however, did not differ at 3 months, 1 and 3 years after diagnosis in the GH-treated and non-treated groups. When graphically comparing the box plots in Figs. 2 (which depicts patients self-assessment) and 3 (showing parental assessment) [10], median scores tend to be higher in the parental assessment (indicating worse QoL), than in the patients self-assessment, thus evidencing that parents rate the QoL worse than the children themselves.

These findings illustrate how self-perceived QoL by affected patients is not always correctly inferred by other people, be it the family or the health care providers. Parents, presumably foreseeing the long-term consequences, express an indirect and less optimistic opinion on their child's current status, while the patients themselves are more concerned on the immediate consequences of their health problem. Thus, contemplating these patient-reported outcome tools in the global care of an individual patient is complementary to other biochemical or imaging parameters generally used. They will highlight those problems perceived as important for the affected individual, and in some cases, may subsequently be approached and improved. If irreversible (for example, blindness), it may also be approached with measures directed to promote more autonomy, like learning the Braille alphabet or techniques for walking with a stick, which in the long term, after adaptation, may lead to QoL improvement, something which is always desirable, whatever the underlying cause.

Compliance with ethical standardsC

Conflict of interest The author declare that he has no conflict of interest

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