Assessing Disease and Treatment Burden for Young Children with Growth Hormone Deficiency (GHD)

Background

• Children with GHD, in addition to short stature, may experience physiological symptoms6 as well as social and emotional problems.1
• Assessing these impacts is critical for understanding the extent of GHD disease and treatment burden and assessing treatment benefit.

Methods

• A methodology to collect objective, observer-reported information regarding GHD disease and treatment burden for young children was developed.

Results

• Limited, no adequately designed measures exist to assess these impacts in children who are unable to answer for themselves or complete assessments designed for adults.

• For young children, the assessment of outcomes in young children (approximately age 8-9 years), proxy measures are not acceptable as they are not objective and/or may reflect the bias of the person acting as proxy.1,4

• Thus, a scientifically valid parent observer-reported outcome (OBRQ) measure is needed, which can provide objective assessment of these impacts for young children.

Objectives

• Develop a simple, scientifically valid methodology to elicit objective, OBRQ information, which could be used when developing submeasures of treatment burden measures for children too young to complete a patient-reported outcome (PRO) measure.

• Determine if this methodology allows parents of young children with GHD to objectively assess GHD disease and treatment burden experienced by their child, and mirrors child reports of these burdens.

• Develop child appropriate PROs to assess GHD disease and treatment burden.

• Compare PRO versions of the measures, which can be objectively completed by a parent of a young child who is unable to report for themselves.

• A methodology to collect objective, observer-reported information regarding GHD disease and treatment burden for young children was developed.

• In this study, the qualitative assessment of the methodology, concept elicitation (CE) interviews, were conducted.

• Seventy CE interviews were conducted with GHD children (n=39) (Table 1a) and 31 parents of GHD children (n=39) (Table 1b). Child and parent interviews were conducted at different times.

• The CE interviews showed that parents were able to provide concrete examples of what they had seen or heard, which mirrored the children’s experiences of reported impacts (Tables 2a and 2b).

• Based on the qualitative analysis of these interviews, 2 PRO measures were developed, which underwent CD with 13 children ages 8 to 13 years and 13 parents of children with GHD ages 4 to 13 years.

• CD sessions were 60-minute face-to-face interviews to develop, elicit, evaluate, and refine newly created measures.

• The CD measures included 17-item burden measure named Treatment Related Impact Measure – Child Growth Hormone Deficiency (TRIM-GHD) and a 17-item burden measure named Treatment Burden Measure – Child Growth Hormone Deficiency (TB-M-GHD), each with a PRO and an OBRQ version.

• The TRIM-GHD has 4 domains: Symptoms, Physiological Functioning, Social Well-being, and Emotional Well-being.

• The TB-M-GHD has 3 child-related domains: Child Physical, Child Emotional, and Child Interference.

• CD results found that children aged 8 years had substantially more comprehension issues understanding the intended meaning of items, had more variability in how they interpreted the recalled period, and were less able to provide more-normative responses when completing the measures.

• This lead to the decision that a parent-reported OBRQ measure would be appropriate for children aged <9 years.

• PRO and OBRQ measures assess equivalent concepts, each with their own parent or child appropriate instructions, language, and formatting (Table 1).

• PRO versions contain brief, straightforward instructions; the items are brief, simple, and in language used by the children in the CE and CD interviews; instructions tell the child that he will not be ‘grilled’ on their responses and their answers will not be shared with others; and simple clip art – offering a visual cue to what the items in a section relate to – are also shown.

• PRO versions contain instructions emphasizing that they MUST have responses ONLY on what they have observed or heard about from their child or others about their child and NOT on what they think; an option for ‘Don’t Know’ is provided for each item in the event that the parent has no ‘evidence’ (observerable information) on which to base their answer.

Conclusions

• It is possible to elicit OBRQ data from parents of children with GHD and design scientifically valid:
  - GHD PRO disease and treatment burden outcome measures appropriate for children ages 9 to <15 years, which are understandable, relevant, and easily answered by the child without help from others.
  - OBRQ versions of these measures, which can be completed by parents of GHD ages 4 to <9 years.
  - Collection of both child PRO and parent OBRQ data from outcome measures developed using this methodology will allow:
    - For the inclusion of disease and treatment burden outcome data for young children in the ongoing developmental process.
    - Clinicians to assess treatment benefit for their GHD patients.
    - Researchers to design studies, which capture disease and treatment burden outcome data "for the child and his or her parents."