Introduction

Children with growth hormone deficiency (GHD) may have to deal with practical, emotional, and functional difficulties. To date, there is no condition-specific measure of the impact of GHD for these children. The Growth Hormone Deficiency – Child Impact Measure (GHD-CIM) was developed under the guidance of the European Society for Paediatric Endocrinology and Endocrinology/European Medicine Agency guidelines to address this gap.1

Psychometric testing was conducted for the GHD-CIM to determine measurement properties, reliability, validity, and interpretability of the measure. – The preliminary GHD-CIM had two versions: a child self-report (PRO) for ages 4 to less than 13 years and an observer-report (ObRO) for parents/guardians of children aged 4 to less than 9 years. However, preliminary analyses raised concerns about floor and ceiling effects; therefore, the preliminary 20-item version (validation data shown in abstract) was further refined to an 11-item version which reduced these effects. This study presents the psychometric validation results of the refined GHD-CIM 11-item version.

Methods

Study design

A non-interventional, multi-clinic-based validation study was conducted in the USA and the United Kingdom.

Two populations of participants were recruited:

Pre-intervention children aged 9 to less than 13 years with a diagnosis of GHD and parents/guardians of younger children with GHD aged 4 to less than 9 years.

Each population was further divided into a Treatment-Maintenance group (Group A or Group C) or Maintenance group (Group B or Group D) for a total of four subgroups and no control group (Figure 1).

All groups completed a baseline assessment battery in clinic, with in-person follow-up for the Treatment-Maintenance groups (Figure 1).

Participants were treated with commercially available products according to routine clinical practice at the discretion of their clinician.

Statistical analysis plan

Exploratory factor analysis procedures on the correlation matrices derived from the items comprising the GHD-CIM measures and confirmatory factor analysis to verify the final factor structure derived were performed.

Items were considered for deletion for reasons of high correlation with other items or total score, floor or ceiling effects, poor fit or construct relevance considerations.

Cronbach’s alpha was used to assess internal consistency reliability. A minimum correlation of 0.70 was expected.

Test-retest reliability was assessed using the intraclass correlation coefficient (ICC) in a subsample from the Maintenance group who indicated experiencing no change in treatment since their last assessment.

Convergent construct validity was assessed with Pearson’s correlation between measurement scores and other items or instruments measuring similar concepts, and supported when the scores were substantially correlated (≥0.40).

Known-groups validity was also tested for hypotheses using a two-tailed test at a p≤0.05 level and was supported when at minimum one hypothesis per subdomain was significant.

Results

• The analytic data set was 243 subjects (145 children and 98 parents/guardians); children’s average age was 9.2 years, 72% male, and average age at diagnosis was 6.9 years. Parent’s average age was 41.6 years, predominantly mothers (80.7%), married (88.1%), and worked full-time (51.0%).

• Factor analysis identified 3 domains: Physical, Functional, Social Well-being, and Emotional Well-being (Figure 2).

• Item reduction resulted in an 11-item measure (Figure 3).

• Ceiling effects above 66% for items with responses of “Not at all” or “Never” (where respondents could not get any better) were seen in 3 items.

• Internal consistency reliability was acceptable for all domains and Overall score (Cronbach’s alpha >0.7).

• At least one of the convergent validity hypotheses for each domain and construct was proven correct (p<0.30).

• For known groups validity, Emotional Well-being and Social Well-being scores were able to significantly discriminate between levels of coping. There were trends that younger children had greater disease impact, and children who experienced a larger increase in growth (at 12 weeks) reported higher (better) scores in Physical Functioning scores.

• Associated effect sizes ranged from −0.40 to −0.58, indicating that the GHD-CIM is sensitive to change.

• After reviewing the concordance between child PRO and parent ObRO versions, it was decided due to inconsistencies in the validation data between them, to only have an ObRO version.

• It is recommended to have an ObRO version used for parents of children aged 4 to less than 13 years.

Conclusions

• PRO development is an iterative process and measures are refined by the results.

• This validation study resulted in the GHD-CIM ObsRO version for parents/guardians of children aged 4 to less than 13 years.

• The GHD-CIM ObsRO version was found to be reliable and valid and is considered ready for inclusion in clinical trials and clinical practice.

• Accurate and reliable assessment of disease burden can help researchers and clinicians better assess and address impacts of disease, factors that may affect treatment compliance and improve doctor–patient communications.

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References