Development of a Parent Experience Measure for Parents of Children With Achondroplasia

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BACKGROUND

The clinical complications and medical impacts of achondroplasia (ACH) in children are well studied and frequently include recurrent ear infections, sleep apnea, hearing loss, teeth crowding, speech delay, and delayed developmental milestones, including gross motor and fine motor 

Little is known about how having a child with ACH impacts parents’ experiences and quality of life.

Research has shown that at the age of 7 years, many children with ACH continue to require minimal to moderate parent care, including assistance with self-care, and some children still require supervision in social settings. Evidence also suggests that parents may experience emotional and other impacts at the time of their child’s diagnosis.

METHODS

The qualitative research study design was based on an adapted grounded theory approach and followed FDA guidelines for the development of patient-reported outcome measures (PROMs). Based on a literature review and clinical expert interviews, a semi-structured interview guide was developed to elicit parents’ experiences related to having a child with ACH.

The qualitative analysis and the development of a preliminary theoretical model identified 4 conceptual domains for the impacts of having a child with ACH on parents, so only the major impacts in each domain are included.

OBJECTIVE

The purpose of the study was to conduct concept elicitation interviews with parents of children with ACH to provide qualitative evidence to support the development of the Achondroplasia Parent Experience Measure (APEM), which assesses the impacts of having a child with ACH aged 2 to <12 years on parents’ daily life and well-being.

RESULTS

The newly developed APEM measure included 15 items in 4 conceptual domains.

• The APEM was designed as a parent-reported outcome (PRO) measure to be completed by parents of children aged 2 to <12 years with ACH.

• Based on the cognitive debriefing interviews, minor edits to the measure were made to improve understanding and readability.

The conceptual framework for APEM is shown in Figure 5. The APEM is a validation-ready PRO designed to assess the impacts of having a child with ACH aged 2 to <12 years on parents’ general well-being, including caretaking responsibilities, emotional well-being, family, and work.

CONCLUSIONS

The study provides evidence to support the content validity for the validation-ready APEM parent PRO measure to assess the impacts of having a child with ACH aged 2 to <12 years on parents’ daily life and general well-being, including:

• Caring/feeding responsibilities (eg, helping child with meals and dressings, attending to children’s basic needs);

• Emotional impacts (eg, worry about the future, worry about child’s physical health, safety concerns, feeling stressed/overwhelmed, worried about child’s social well-being, etc.);

• Family strain (eg, wanting to have more time to care for another child);

• Work (eg, not having enough time to care for child, lost job)