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

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RESULTS

Demographic/Health Characteristics for Children of Parent Participants

Demographic characteristics for the children of parent participants are shown in Table 2.

- 30.6% of parent participants (n=11) had children aged 2 to <5 years with ACH, 36.4% of parents (n=12) had children aged 5 to <9 years, and 33.3% of parents (n=12) had children aged 9 to <12 years.
- Nineteen children (52.8%) were female and 17 (47.2%) were male.
- Twelve parents (33.3%) reported child’s health status as excellent, 7 (19.4%) reported as “good,” and 3 (8.3%) reported as “fair.”

Table 2. Demographic/health characteristics of children of parent participants

<table>
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<tr>
<th>Age Group</th>
<th>Number of Parents (%)</th>
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<tbody>
<tr>
<td>2 to &lt;5 yrs</td>
<td>11 (30.6%)</td>
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<td>Female</td>
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The newly developed APEM measure included 15 items in 4 conceptual domains:
- The APEM was designed as a patient-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 the 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 responsibilities (e.g., managing child’s medical care, helping child with self-care, assisting child, advocating for child, etc.).
  - Emotional impacts (e.g., worry about the future, worry about child’s physical health, safety concerns, feeling stressed/overwhelmed, worry about child’s social well-being, etc.).
  - Family strain (e.g., having less time); and
  - Missed work time to care for child.
- A future psychometric validation study of the APEM is needed to further assess the measure’s validity and reliability.
- As new treatments for pediatric ACH are being developed, it is critical for clinicians to understand and assess the impacts of having a child with ACH on parents’ lives, which may be lessened following children’s treatment.

Figure 4. Achondroplasia parent experience measure (APEM – Impact) conceptual framework

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

Figure 5. Achondroplasia parent experience measure (APEM – Impact) conceptual framework

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

Figure 6. Major impacts on parents’ work

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

Figure 7. 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, as well as the major impacts in each domain.

Table 1. Parent participant demographic characteristics

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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, as well as the major impacts in each domain.

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

Figure 3. Major impacts on family

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

Figure 2. Major impacts on parents’ emotional well-being

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

Figure 1. Major impacts on parents’ caretaking responsibilities

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

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/ caregiver 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.

Objective:

The purpose of the study was to conduct concept elicitation interviews with parents of children with achondroplasia to develop 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.

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 (PROs).

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.

Concept elicitation sample inclusion criteria:
- an adult aged 18 years or older
- able to read, write, and speak English (in the United States) or Spanish (in Spain)
- parent of a child ≤18 years of age diagnosed with ACH; and
- actively involved in the child’s care

Concept elicitation sample exclusion criteria:
- A cognitive impairment or other medical condition, including psychiatric conditions, that would affect a participant’s ability to participate in a telephone interview or focus group
- It should be noted that this study was part of a larger study of parents of children with ACH ≤18 years of age, and this study focused only on parents of children aged 2 to <12 years.

Individual telephone interviews and 1 parent focus group were conducted in the US and Spain with 36 parents of children aged 2 to <12 years with ACH.

Interview and focus group transcripts were analyzed for content and coded them using a qualitative analysis software program.

The qualitative analysis was used to develop a preliminary theoretical model of the impacts of having a child with ACH on parents and potential modifiers to inform the content and structure of the APEM measure.

Only impacts identified as major would be included in the measure.

Criteria for Identifying Major Impacts:
- Endorsement of at least 30% of parent participants in at least 2 of the 3 child age groups analyzed; or an endorsement of 25% to 29% of parent participants in at least 2 of the 3 age groups if conceptually important
- Endorsement percentages were considered across differing child age groups to ensure relevance to parents who have children of different ages.
- Would be relevant to child’s treatment
- Considered bothersome, limiting, or difficult
- Impacts must be proximal (rather than distal)