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[1–5]
- 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[5]
- Evidence also suggests that parents may experience emotional and other impacts at the time of their child's diagnosis[6]

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.

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 [US]) 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 by themes using a qualitative analysis software program.

The qualitative analysis report 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 responsive to child's treatment
- Considered bothersome, limiting, or difficult
- Impacts must be proximal (rather than distal)

Once the APEM measure was developed, cognitive debriefing interviews were conducted with an additional 16 parents in the US to ensure that measure items were relevant and appropriate, and that instructions and items were easy to understand and complete.

RESULTS

Parent Participant Sample Description

Participant sample characteristics are shown in Table 1.

- Average age of parents was 41.5 years (SD, 6.6; range, 32-68)
- Thirty-one parents were mothers (86.1%), and 5 parents were fathers (13.9%)
- Most participants were married (80.6%, n=29), 8.3% were partnered (n=3), 5.6% were divorced (n=2), and 5.6% were single (n=2)
- Seven parents (19.4%), all residing in the US, also had a diagnosis of ACH

Table 1. Parent participant demographic characteristics

	Spain (n=11)	US (n=25)	Total (N=36)
Age, mean(SD)	40.4(3.1)	42.0(7.6)	41.5(6.6)
(range)	(35-43)	(32-68)	(32-68)
Relationship to child, n(%)			
mother	8(72.7)	23(92.0)	31(86.1)
father	3(27.3)	2(8.0)	5(13.9)
Marital status, n(%)			
single	2(18.2)	0	2(5.6)
married	6(54.5)	23(92.0)	29(80.6)
partnered	3(27.3)	0	3(8.3)
divorced	0	2(8.0)	2(5.6)
Education, n(%)			
less than high school	2(18.2)	1(4.0)	3(8.3)
high school or equivalent	4(36.4)	2(8.0)	6(16.7)
college degree	5(45.5)	12(48.0)	17(47.2)
post-graduate school	0	10(40.0)	10(27.8)
Work status, n(%)			
full-time	6(54.5)	10(40.0)	16(44.4)
part-time	3(27.3)	3(12.0)	6(16.7)
student	0	2(8.0)	2(5.6)
retired	0	1(4.0)	1(2.8)
not working (other)	2(18.2)	9(36.0)	11(30.6)
Parent has ACH			
n(%) yes	0	7(28.0)	7(19.4)

Percentages may not add to 100 due to rounding. ACH = achondroplasia; SD = standard deviation.

RESULTS

Demographic/Health Characteristics for Children of Parent Participants

Demographic/health 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, 36.1% of parents (n=13) 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,"14 (38.9%) reported as "very good,"7 (19.4%) reported as "good," and 3 (8.3%) reported as "fair"

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

	Spain (n=11)	US (n=25)	Total (N=36)
Child age, n(%)			
2 to <5 years	5(45.5)	6(24.0)	11(30.6)
5 to <9 years	4(36.4)	9(36.0)	13(36.1)
9 to <12 years	2(18.2)	10(40.0)	12(33.3)
Child gender, n(%)			
female	7(63.6)	12(48.0)	19(52.8)
male	4(36.4)	13(52.0)	17(47.2)
Health status (parent-reported), n(%)			
excellent	3(27.3)	9(36.0)	12(33.3)
very good	3(27.3)	11(44.0)	14(38.9)
good	3(27.3)	4(16.0)	7(19.4)
fair	2(18.2)	1(4.0)	3(8.3)
Age/time diagnosed with ACH, n(%)			
in utero	9(81.8)	12(48.0)	21(58.3)
at birth	1(9.1)	4(16.0)	5(13.9)
<2 months of age	1(9.1)	2(8.0)	3(8.3)
2-6 months of age	0	5(20.0)	5(13.9)
unknown (adopted)	0	2(8.0)	2(5.6)

Percentages may not add to 100 due to rounding. ACH = achondroplasia; SD = standard deviation.

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 impact(s) in each domain:

- **Caretaking responsibilities**:
  - 6 major impacts (Figure 1)
- **Emotional well-being:**
  - 6 major impacts (Figure 2)
- **Family:**
  - 2 major impacts (Figure 3)
- **Work:**
  - 1 major impact (Figure 4)

Preliminary theoretical model for Experience of Parents of children with achondroplasia (children ages 2 to <12 years)

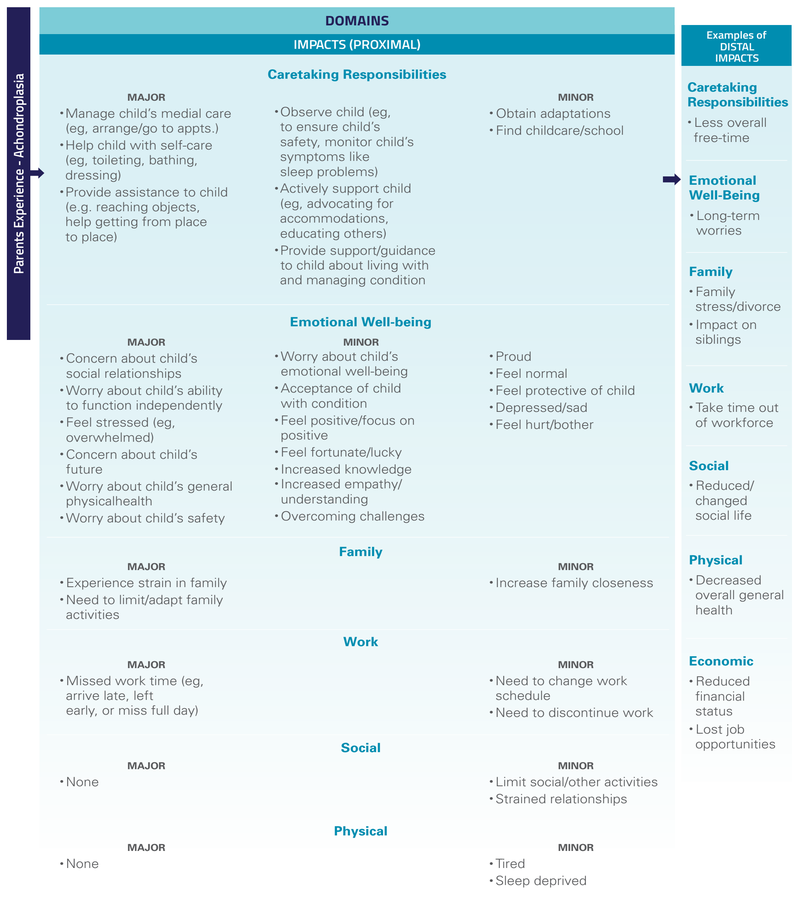
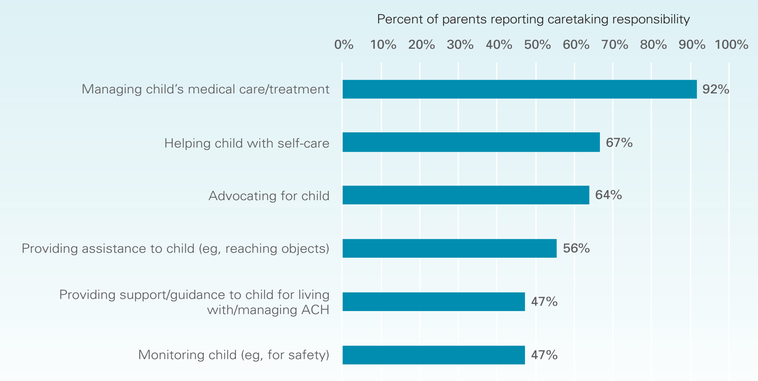


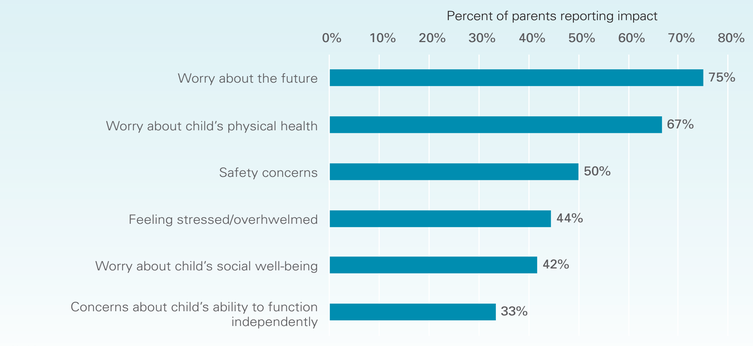
Figure 1. Major impacts on parents' caretaking responsibilities



N=36.

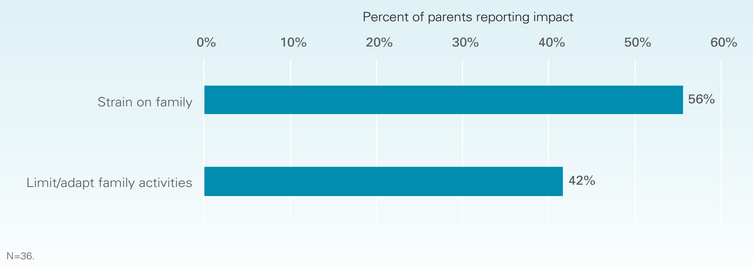
RESULTS

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



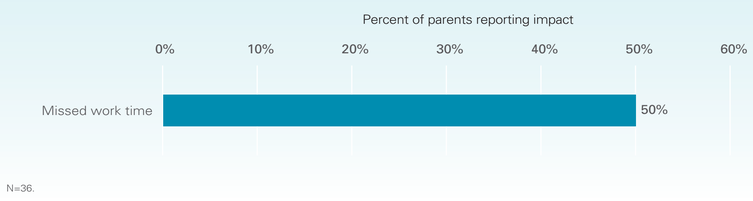
N=36.

Figure 3. Major impacts on family



N=36.

Figure 4. Major impacts on parents' work



N=36.

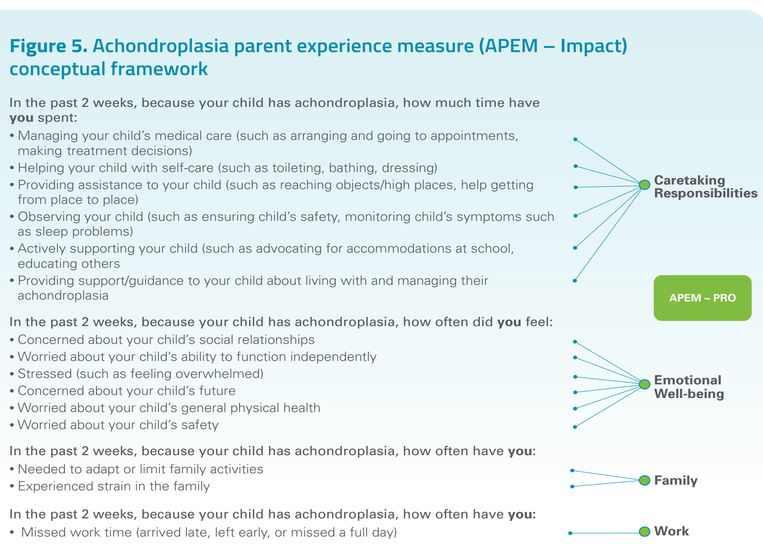
The newly developed APEM measure included 15 items in 4 conceptual domains.

- 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 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.

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



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:
  - Caretaking responsibilities (eg, managing child's medical care, helping child with self-care, assisting child, advocating for child, etc.);
  - Emotional impacts (eg, 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 (eg, 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

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Growth and syndromes (to include Turner syndrome)

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PosterSessionOnline.com