PCR29

Patient And Caregiver Perspectives Regarding Once-Weekly Injection Devices In Growth Hormone Deficiency: Qualitative Interviews To Inform A Patient Preference Study

Chen JV[1]; Yuen KJC[2]; Kelepouris N[3]; Shah S[4]; Sharp R[5]; Jamal H[5]; Miller BS[6]

Aim

- The overall objective of this study is to quantify the preferences of patients with growth hormone deficiency (GHD) and caregivers (CGs) for long-acting growth hormone analogs (LA-GHAs).
- This poster presents findings from in-depth, qualitative interviews conducted with patients with GHD and CGs, which will be used to inform final selection of attributes and levels to include in an upcoming discrete choice experiment (DCE).

Introduction

- GHD is a rare disease estimated to affect approximately 1 in 3,500 to 10,000 children[1,2].
- Treatments have typically involved once-daily injections; however, the treatment landscape has evolved with the introduction of once-weekly LA-GHAs[3-6].
- This study explored the perspectives of patients with GHD and their CGs-regarding once-weekly LA-GHA injection devices to inform the development of a quantitative patient preference survey.
- Here, we describe Phase 2 results of a three-phase study investigating patient and CG preference for GHD treatments.

Methods

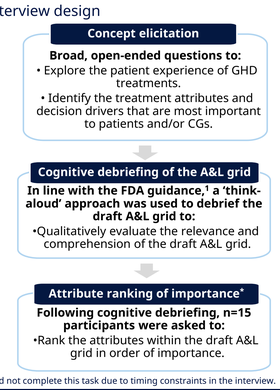
- The three-phases for this observational (non-interventional) study are outlined below:
  - **Phase 1:** A targeted literature review was conducted to identify key attributes and levels relevant to patients with GHD and CGs. This informed the development of a draft attributes and levels (A&L) grid.
  - **Phase 2:** In-depth qualitative concept elicitation (CE) and cognitive debriefing (CD) interviews were conducted with patients with GHD and CGs to finalize the A&L grid.
  - **Phase 3:** The finalized A&L grid will be used in the conduct of cognitive pilot interviews and an online survey (including a discrete choice experiment) with a targeted minimum of N=120 participants (consisting of the same sub-populations as Phase 2).

Methods cont.

Interview procedure and qualitative analysis:

- N=20 United States (US)-based participants took part in a telephone interview conducted by Adelphi Values. The number of participants by sub-population is summarized below:
  - Adult patients with GHD (≥18 years), n=6
  - Dyads of adolescent patients with GHD (12–17 years) and their CGs, n=8
  - CGs of pediatric patients with GHD (4–11 years), n=6
- **Figure 1** outlines the interview design. Each interview was conducted in alignment with a semi-structured interview guide designed to allow for open-ended concept elicitation (CE) focusing on treatment experience and perspectives, and targeted cognitive debriefing (CD)[7] of the draft A&L grid.

Figure 1: Interview design



*n=5 participants did not complete this task due to timing constraints in the interview.

- Each interview was audio-recorded and transcribed verbatim for analysis.
- CE data = iterative thematic analysis[8,9] was used to identify key themes in the data, using inductive-deductive approaches[10] to identify themes directly from the data while applying prior knowledge.
- CD data = a dichotomous coding scheme was applied to indicate 'yes/no' categories, using a framework analysis approach.

Results

Table 1: Patient sociodemographic and clinical characteristics

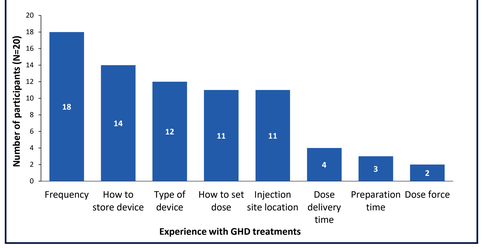
Characteristics	Full sample (N=20)	Adults (n=6)	Adolescents (n=8)	Pediatrics (n=6)
Mean age (range)	20.55 (4–52)	41.17 (24–52)	14.63 (12–17)	7.83 (4–10)
Female, N (%)	6 (30%)	5 (83.3%)	1 (12.5%)	0 (0%)
Race	**19 White** 1 Other	**5 White** 1 Other	**8 White** 0 other	**6 White** **0 other**
Years since Dx	**0-5 years: 10** 6-10 years: 7 11+ years: 3	**0-5 years: 4** 6-10 years: 0 11+ years: 2	0-5 years: 2 **6-10 years: 5** 11+ years: 1	**0-5 years: 4** 6-10 years: 2 11+ years: 0
Highest level of education	7 less than high school 5 some high school **8 some college or higher**	2 some college **3 Bachelor's** 1 Master's	2 less than high school **5 some high school** 1 some college*	**5 less than high school** 1 missing data*

*These data relate to patients' education. All adult CG's of adolescent patients (n=8) or pediatric patients with GHD (n=6) were well-educated (college/degree or above).

CE results – patient experience of current GHD treatment devices

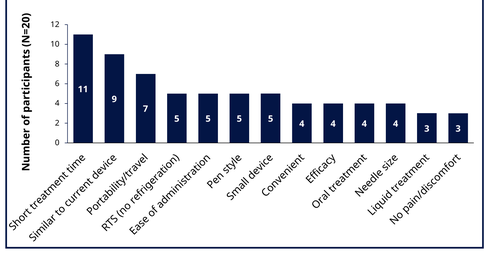
- Participants were asked to describe their experience with the device or injection they currently use for GHD.
- Concepts discussed spontaneously are summarized in **Figure 2**.
- Frequently reported concepts included **frequency of administration**, **device storage**, **dose setting**, **injection site location**, and **type of device**.

Figure 2: Experience of current GHD treatment devices



- A **short treatment time** was the most reported feature of an ideal treatment, followed by **device similarity** and **portability** (**Figure 3**).

Figure 3: Features of an 'ideal treatment' (treatment drivers)



- For adolescent/caregiver dyads, the most frequently reported feature of an ideal treatment was a **short treatment time** (n=6/11), which was predominantly adolescent-reported.

CD of the A&L grid

- An overall summary of attribute-level findings associated with participants' interpretation of the A&L grid is summarized in **Figure 4** (key result).
- All participants asked interpreted all attributes correctly.
- Most participants perceived **type of injection device** and **device preparation time** as important attributes.
- Most participants perceived **type of injection device** and **injection-site pain** as influential attributes to treatment decision-making.
- **Needle visibility** was deemed less important and influential, with no perceived meaningful difference between levels.
- Following the CD section of the interviews, n=15 participants were asked to rank the 7 attributes in order of importance (**Table 2**).
- **Needle visibility** and **dose delivery time** were ranked as less important.
- **Type of injection device** was ranked within the top 3 most important attributes (n=8) and for 7 patients this was ranked as less important.
- Heterogeneity in reported rankings were apparent, independent of reported characteristics.

Table 2: Attribute ranking of importance

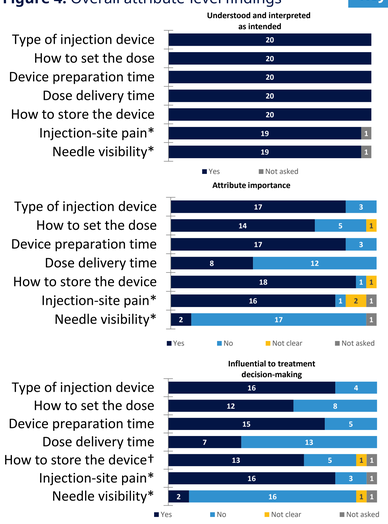
Attribute	Most important → Least important						
	Rank 1	Rank 2	Rank 3	Rank 4	Rank 5	Rank 6	Rank 7
Type of injection device	**n=4**	n=1	n=3		n=1	n=6	
How to store the device	n=2	**n=6**	**n=5**	n=2			
Pain experienced after the injection	**n=5**	n=2	**n=4**	**n=3**	n=1		
Device preparation time	**n=3**	**n=3**	n=2	**n=5**	n=2		
How to set the dose	n=1	**n=3**		n=2	n=6	n=1	n=2
Dose delivery time			n=1	n=3	n=3	n=8	
Needle visibility					n=2		n=13

- Indicative quotes from the qualitative CE findings relating to key attributes are summarized in **Figure 5.**

Figure 5: Contextualisation of key attributes

- **Type of injection device:**
  > ***"The electronic one takes a while and then if you forget then they may not have the medication that night because your kid may already be asleep. [...] It just seems like a lot of mistakes could happen if you had the electronic one compared to the prefilled one."***
- **How to set the dose:**
  > ***"...reading the dose selector, if I don't happen to have my glasses, I have to go find glasses before I would do that. Um, whereas just inserting a premeasured dose cartridge seems like it would be easier for me. I wouldn't have to worry about, um, getting a selector onto the proper number."***
- **Device preparation time:**
  > ***"Um, well, if it was once a week, I don't see that as big, as-as big of a deal. If it's like every single day, then that is a little bit more burdensome but once a week isn't as big of a deal."***
- **Needle visibility:**
  > ***"...comparing the two, like it makes no difference to me. Like they're just two different methods of-of, um, administering. Uh, it doesn't really matter to me whether I can see the needle or not."***
- **How to store the device:**
  > ***"Um, we currently have one that has to be refrigerated at all times and sometimes that can get tricky for, you know, any kind of sleepovers or traveling, um, to make sure that it stays cold and doesn't get frozen"***

Figure 4: Overall attribute-level findings — **Key result**



*'Injection-site pain' and 'needle visibility' were not debriefed with 1 participant due to time constraints during this interview.

†One participant was not specifically asked about the influence of 'how to store the device' due to time constraints during their interview.

Discussion

- **Type of injection device**, **how to set the dose**, **preparation time**, **storage**, and **injection-site pain**, were perceived as important attributes and reported by more than two-thirds of participants to be influential to treatment decision-making, supporting their inclusion in the A&L grid.
- Findings convey a preference for devices with simple preparation steps, minimal injection-site pain, and favorable storage conditions.
- **Device size** was discussed as an important driver of treatment, therefore is to be included in the next version of the A&L grid.
- **Dose delivery time** and **needle visibility** were perceived as less important and impactful relative to other attributes, which led to exclusion from the A&L grid.
- The observed heterogeneity in reported rankings warrants further exploration in the quantitative survey.

Conclusion

- Patients with GHD and CGs value a once-weekly LA-GHA device that is convenient to use with minimal injection-site pain.
- Findings from this qualitative study and upcoming pilot interviews will inform a larger preference study that will quantify the relative importance of attributes corresponding to LA-GHA devices and explore the trade-offs that patients and CGs are willing to make.

[1]Genesis Research Group, Hoboken, USA; [2]Department of Neuroendocrinology, Barrow Pituitary Center, University of Arizona College of Medicine, and Creighton School of Medicine, Phoenix, USA; [3]Novo Nordisk Inc., Plainsboro, NJ, USA; [4]Jazz Pharmaceuticals Inc., Philadelphia, USA; [5]Adelphi Values Patient-Centered Outcomes, Bollington, UK; [6]Division of Pediatric Endocrinology, Department of Pediatrics, University of Minnesota Medical School, M Health Fairview Masonic Children's Hospital, Minneapolis, MN, USA.

Acknowledgements: We would like to thank Brad Mason (Adelphi Values) for providing oversight and review as Adelphi project lead, and Adam Gater (Adelphi Values) for providing senior strategic oversight.

This study was sponsored by Novo Nordisk. The authors acknowledge the medical writing assistance of OPEN Health.

Presented at the ISPOR 2024 conference, May 5–8 2024, Atlanta, GA, USA.

References :

(**1**) Stanley et al. Current Opinion in Endocrinology, Diabetes, and Obesity 2012:19(1);47; (**2**) Tornese et al. Archives of Disease in Childhood 2023:108;176–177 (**3**) Graham et al. Patient Preference and Adherence 2020:14;1889–1899;

(**4**) FDA. https://www.fda.gov/drugs/news-events-human-drugs/fda-approves-weekly-therapy-adult-growth-hormone-deficiency. 2023; (**5**) Ascendis Pharma. https://investors.ascendispharma.com/news-releases/news-release-details/ascendis-pharma-announces-us-food-and-drug-administration 2021; (**6**) Pfizer. https://www.pfizer.com/news/press-release/press-release-detail/fda-approves-pfizers-ngenlatm-long-acting-once-weekly 2023; (**7**) Willis GB. Cognitive interviewing: a tool for improving questionnaire design. Thousand Oaks: SAGE Publications, Inc; 2004; (**8**) Braun et al. Qualitative Research in Psychology 2006:3(2);77-101; (**9**) Hsieh et al. Qualitative Health Research 2005:15(9);1277-1288; (**10**) Fereday et al. International Journal of Qualitative Methods 2006:5(1);80–92.