

BURDEN AND IMPACTS OF DAILY RECOMBINANT HUMAN GROWTH HORMONE INJECTIONS IN GROWTH HORMONE DEFICIENT PEDIATRIC PATIENTS

Turner-Bowker DM¹, Yaworsky A¹, Palladino A², Pleil A³, Shields A¹, Kelly M¹, Lamoureux RE¹, Love E¹, Morrissey L¹, Loftus J⁴

¹Adelphi Values, Boston, MA, USA ²Pfizer, Inc., Collegeville, PA, USA ³Pfizer, Inc., San Diego, CA, USA ⁴Pfizer Ltd., Tadworth, United Kingdom

Topics: Treatment burden, growth hormone deficiency, human growth hormone, pediatric patients

Introduction and objectives

- Recombinant human growth hormone (r-hGH) replacement therapy has been safely and effectively used for over 30 years to treat growth hormone deficiency (GHD)
- Particularly for pediatric patients, r-hGH therapy is long term, and relies upon daily subcutaneous injections to achieve the goals of GHD treatment
- However, little information is available describing the burden and impacts experienced by pediatric patients related to daily r-hGH injections
- Objective:** To identify and describe the burden and impacts of a daily r-hGH injection regimen upon the lives of pediatric patients with GHD, based on input from patients and their caregivers that participated in four separate research studies

Method

- A retrospective evaluation of data drawn from four sources was conducted (please refer to Table 1 for details related to each data source)
- Data from all four sources were reviewed to identify and describe concepts related to the burden caused by daily r-hGH injections regimens
- Analyses were descriptive (rather than comparative) since data were drawn from different studies

Table 1: Details of data sources: Pediatric patients with GHD and their caregivers

Research activity	Location	Date Conducted	Total sample size N	Children (3-11 years) n (%)	Adolescents (12-17 years) n (%)
Qualitative interviews	EU*	Nov 2016—Apr 2017	17	9 (52.9%)	8 (47.1%)
Qualitative interviews	US	Oct – Dec 2016	15	11 (73.3%)	4 (26.7%)
Cross-sectional survey	US	Jan – May 2017	149	70 (47.0%)	79 (53.0%)
Advisory panel discussion	US	Sep 8 th , 2017	3	0 (0.0%)	3 (100.0%)

* Patient interviews were conducted in the following European countries: Czech Republic (n=6), Turkey (n=5), Spain, (n=3), and the United Kingdom (n=3)

Results

- Data across all four sources report responses from a total of 184 pediatric patients with GHD and their caregivers
 - 94 adolescents (ages 12 to 17 years)
 - 90 children (ages 3 to 11 years)
- Across all sources, pediatric patients reported that they had become acclimated to taking daily r-hGH injections despite reporting that the treatment regimen interfered with their life

Qualitative interviews with European pediatric patients with GHD (N=17)

- Participants reported emotional impacts, limitations in daily activities, social impacts, and impacts on familial relationships (see Table 2 for specific concepts)
- Participants expressed a preference for less frequent (e.g., weekly), as opposed to daily, r-hGH injections

Table 2: Impacts of daily injections reported by European pediatric patients (N=17)

Domain	Impact concepts
Emotional	Fear of injections
	Feeling anxious
	Being bothered by injections
Daily activities	Interference with overnight activities
	Interference with travel
	Having to incorporate injection routine into daily schedule
Social	Keeping use of injection secret from peers
	Fear or experience of judgment or bullying from peers
Family relationships	Negative reactions to taking injections by family members
	Interference with family activities

Qualitative interviews with US pediatric patients with GHD (N=15)

- Participants reported emotional impacts, limitations in daily activities, social impacts, and impacts on familial relationships (see Table 3)
- Caregivers of younger children also reported external manifestations of their child's displeasure with injection (e.g., crying, complaining, and/or having a tantrum)
- Participants expressed a preference for less frequent (e.g., weekly), as opposed to daily, r-hGH injections

Table 3: Impacts of daily injections reported by United States pediatric patients (N=15)

Domain	Impact
Emotional	Fear of injections
	Feeling annoyed by injections
	Feeling anxious
	Feeling worried about injections
Daily activities	Interference with overnight activities
	Interference with travel
	Interference with recreational activities
	Going to bed later due to need to inject
Social	Keeping use of injection secret from peers
	Fear or experience of judgment or bullying from peers
Family relationships	Negative reactions to taking injections by family members

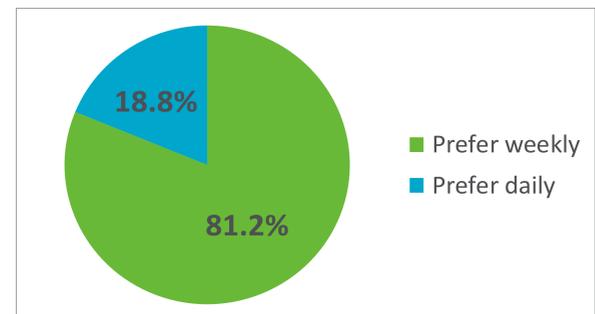
Responses to an online questionnaire submitted by US pediatric patients with GHD (N=149)

- Respondents reported limitations in daily activities, emotional, and social activities (see Table 4)
- Respondents indicated that they consider injection schedule to be the most important attribute of their GHD treatment (44.0% of children, 54.0% of adolescents)
- Respondents indicated that they would prefer a hypothetical once-weekly injection regimen over their current daily injections (78.6% of children, 83.5% of adolescents – see Figure 1)

Table 4: Impacts of daily injections reported by US pediatric patients completing online questionnaire

Domain	Impact	Children (n=70) n (%)	Adolescents (n=79) n (%)	Total (N=149) n (%)
Emotional	Bothered by injections	45 (64.3%)	56 (70.9%)	101 (67.8%)
Daily activities	Changes to daily routine to accommodate injections	45 (64.3%)	58 (73.4%)	103 (69.1%)
	Interference with overnight activities	46 (65.7%)	54 (68.4%)	100 (67.1%)
	Interference with travel	41 (58.6%)	52 (65.8%)	93 (62.4%)
	Interference with recreational activities	17 (24.3%)	27 (34.2%)	44 (29.5%)
	Interference with usual daily activities	6 (8.5%)	17 (21.5%)	23 (15.4%)
Social	Interference with social activities	18 (25.7%)	39 (49.4%)	57 (38.3%)

Figure 1. US pediatric and adolescent patient preference for weekly over daily injections (N=149)



Advisory panel discussion with US pediatric patients with GHD (N=3)

- Participants reported limitations in daily activities, and impacts on family relationships (see Table 5)
- Participants expressed a preference for less frequent (e.g., weekly), as opposed to daily, r-hGH injections

Table 5: Impacts of daily injections reported by US adolescent patients in advisory board (N=3)

Domain	Impact
Daily activities	Interference with travel
Family relationships	Strain on family relationships due to injections

Discussion

- Although pediatric patients with GHD can acclimate to daily r-hGH injections, they continue to experience the burden and impacts associated with a daily injection schedule
- Pediatric patients most frequently reported emotional impacts, limitations in daily activities, social impacts, and impacts on familial relationships
- Impacts on emotional functioning (primarily associated with fear anxiety or bother linked with injections), may be decreased with a less frequent injection schedule
- The limitations in daily activities that were reported largely centered around time spent away from home, such as travel
- The impact on social functioning and familial relationships relate largely to the way the patient feels they are perceived due to their injections
- These data add to growing evidence^{1,2} of the burden that daily r-hGH injections impose on pediatric patients with GHD
- Evidence further suggests that the burden of daily injections has a negative effect on treatment adherence, and thus on effectiveness³⁻⁵
- Across all four sources, pediatric patients reported a preference for less frequent (e.g., weekly) r-hGH injections, rather than daily injections

Conclusions

- Pediatric patients with GHD experience burden and impacts related to daily r-hGH injections
- A less-frequent injection regimen may help improve adherence and thus treatment outcomes

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ADELPHI VALUES



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254--P2

Growth and syndromes (to include Turner syndrome)

Jane Loftus

Poster presented at:

