

Assessing the treatment burden and Quality of Life of children receiving daily recombinant Growth Hormone treatment in Greece – GHEA study

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Objectives

- To assess the health-related quality of life (HRQoL) of children and adolescents with growth hormone deficiency (GHD), receiving daily recombinant growth hormone (rhGH) treatment in Greece.
- To evaluate patients’ and caregivers’ perceptions on the burden associated with rhGH treatment in Greece.

Conclusion

- This analysis showed an overall good HRQoL and a moderate treatment burden for Greek patients receiving daily rhGH and their caregivers.
- Sub-optimal adherence rates were observed which may affect clinical outcomes.
- New treatment options could further improve HRQoL and treatment experience, as well as increase adherence rates which may lead to improved health-related outcomes.

Background

- Growth hormone deficiency (GHD) is a rare condition characterized by inadequate secretion of growth hormone (GH) and low serum concentration of insulin-like growth factor-1 (IGF-1). It is associated with growth attenuation/deceleration, short stature and metabolic defects.^{1,2}
- Recombinant human growth hormone (rhGH) is considered the standard of care treatment for GHD for over 30 years.^{3,4}
- Due to its short half-life, daily subcutaneous (SC) administrations are required, which may present a substantial burden for both patients and their caregivers.^{5,6}
- They may affect their QoL and may also result in poor treatment adherence, limiting the therapeutic effect.^{5,6,7}

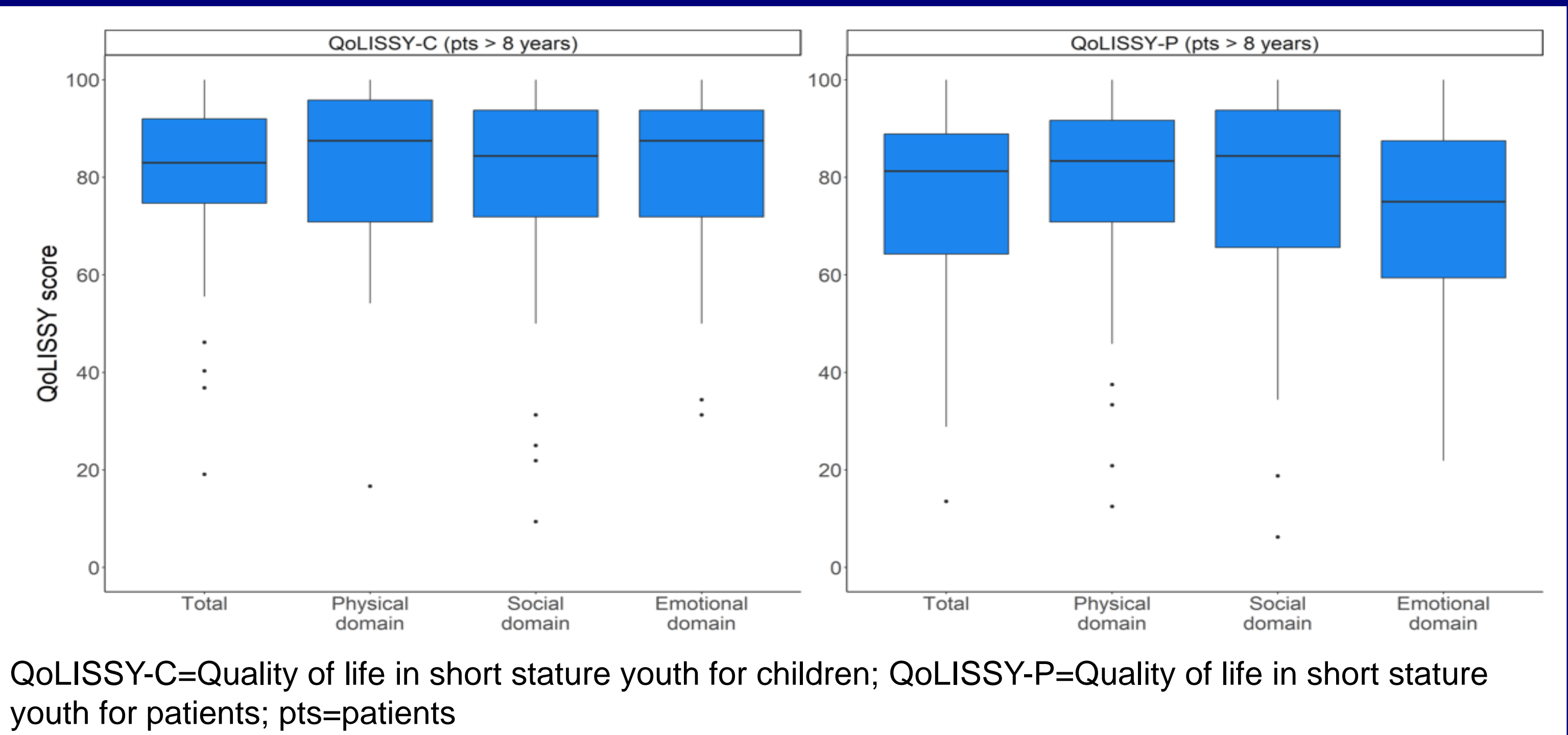
Methods

- GHEA is an ongoing cross-sectional study enrolling patients aged 3-17 years diagnosed with idiopathic GHD receiving daily rhGH for at least 12 months.
- Two questionnaires were administered to patients and their caregivers: The Quality of Life in Short Stature Youth (QoLISSY)⁸ and the Life Interference Questionnaire for Growth Hormone Deficiency (LIQ-GHD)⁹.
- QoLISSY assessed patients’ health-related quality of life (HRQoL) from both patients’ and caregivers’ perspective (higher score denoting better HRQoL).
- LIQ-GHD, to be completed as a Dyad pair (child and caregiver together), evaluated patients’ and caregivers’ treatment burden (higher score denoting greater life interference).

Table 1. Patient Demographic Characteristics	
Age, mean (SD), years	12.2 (3)
Gender, n (%)	
Male	54 (64.3%)
Female	30 (35.7%)
Height, mean (SD), cm	144.5 (18.4)
Weight, mean (SD), kg	40.8 (14.6)
BMI, mean (SD), kg/m ²	18.8 (3.3)

SD=standard deviation

Figure 1. Box plots of QoLISSY total, Physical, Social, and Emotional domains scores, as reported by children (QoLISSY-C) and their parents (QoLISSY-P)



References

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Disclosures

Ioannis Skiadas, Oresteia Zisimopoulou, Apostolia Poimenidou, Manfred Windisch, Nikoletta Sofiaki, and Evangelia Baxevanidi are Pfizer employees



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Results

- Between July 2022 and January 2023, 84 patients from 5 pediatric endocrinology outpatient clinics (77 aged >8 years) were enrolled and included in this interim analysis; mean age (SD) was around 12 (3) years and 64.3% were male (Table 1).
- The mean QoLISSY scores reported from children (>8 years old) and their caregivers were 81.2 and 74.9, respectively (Figure 1).
- Both children and caregivers reported medium to high QoL levels in the physical, social and belief domains. However, caregivers reported a more compromised emotional domain, whereas both coping and treatment domains appeared to be more challenging (Figures 1 and 2).
- The LIQ-GHD results revealed a mean (SD) overall patient life interference (LI) score of 22.4 (19.4) (Figure 3), similar to what has been reported in the literature⁹. Ease of injection schedule (EoI), willingness to continue (WtC), and to a lesser degree, LI appear to drive the reported burden (Figure 3).
- Adolescents (>12 years) demonstrated lower adherence than patients 8-12 and <8 years of age. There was a significant - at the level of 5% - association between age categories and the number of missed injections (Table 2).

Figure 2. Box plots of QoLISSY Coping, Beliefs, and Treatment domains scores, as reported by children (QoLISSY-C) and their parents (QoLISSY-P)

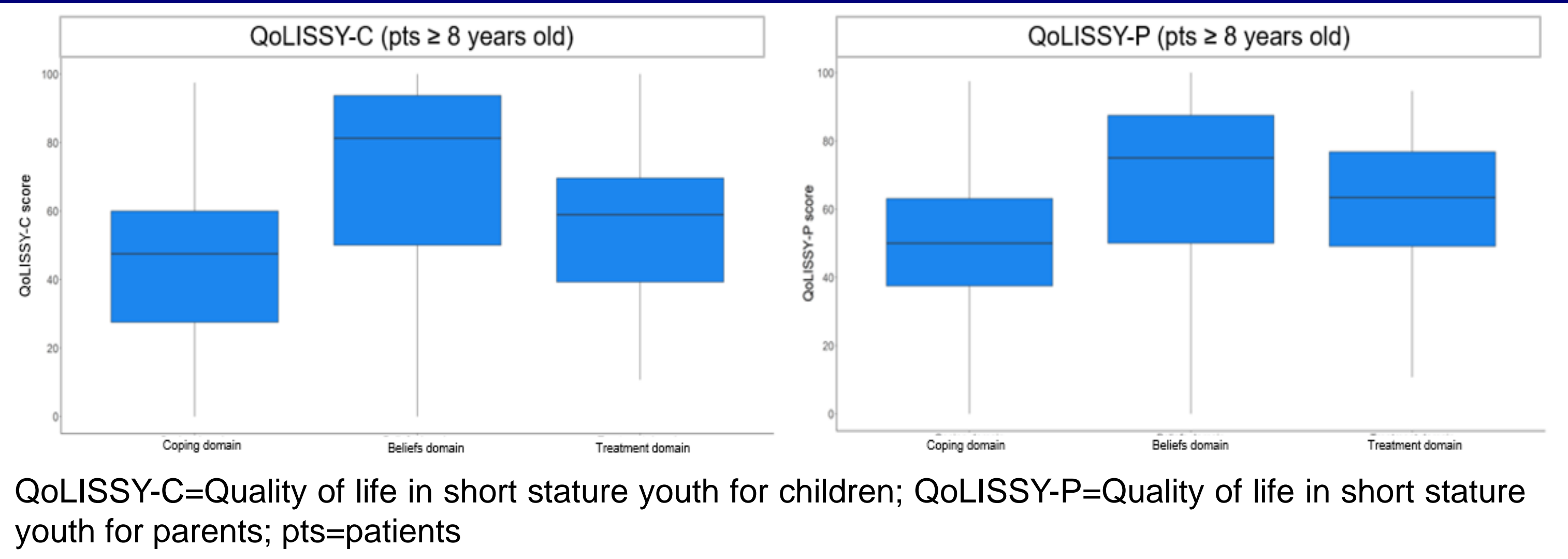


Figure 3. Box plots of LIQ-GHD Treatment Burden (PEoU, EoIS, LI, WtC, SS, CS domain scores) amongst all patients, as reported by patients and their caregivers

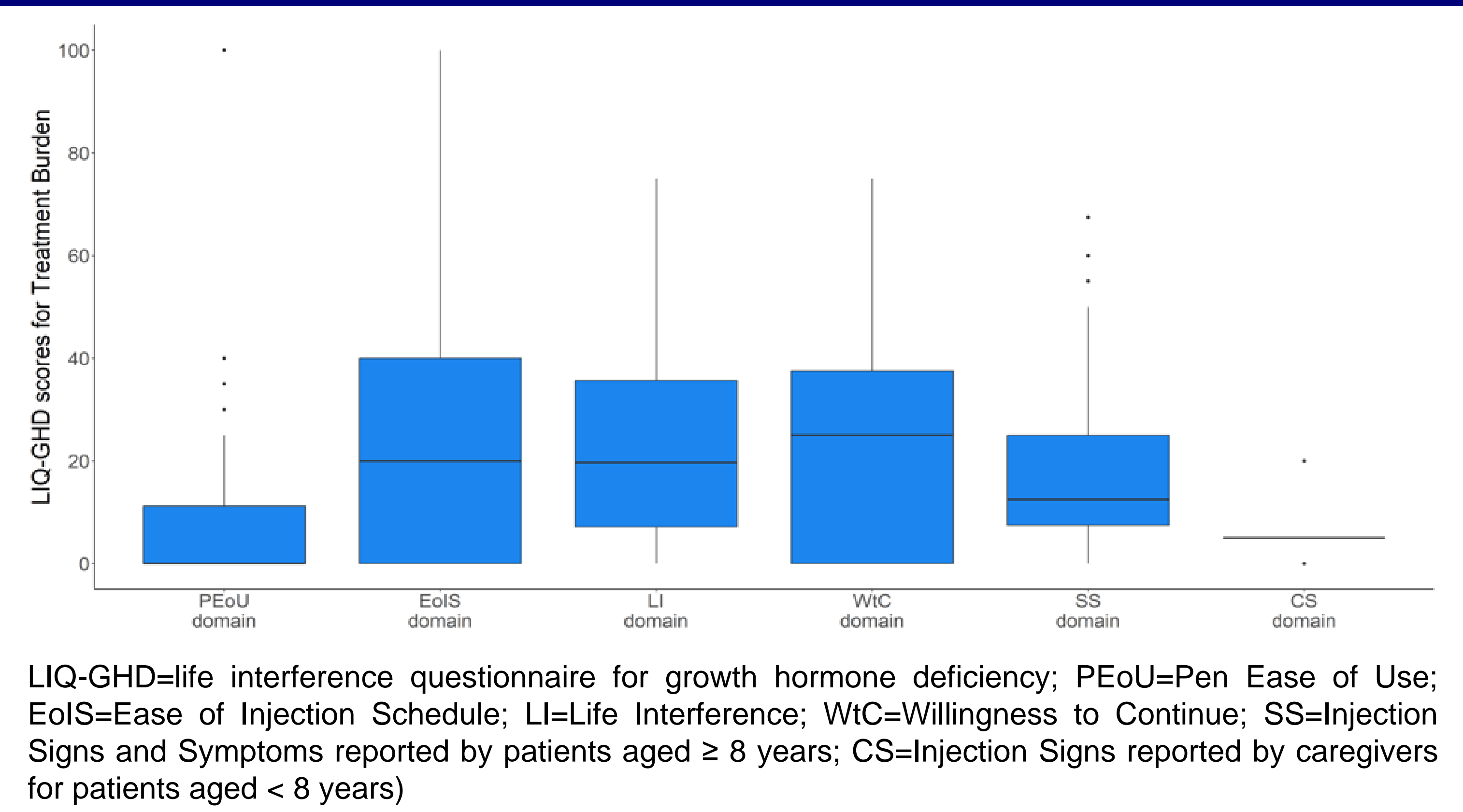


Table 2. Distribution of LIQ-GHD number of missed injections per month by age categories, as reported by patients and caregivers

	Overall (n=84)	< 8 years (n=7)	8-12 years (n=35)	> 12 years (n=42)
Missed Injections, n (%)				
0	45 (53.6%)	4 (57.1%)	21 (60.0%)	20 (47.6%)
1	23 (27.4%)	0 (0.0%)	12 (34.3%)	11 (26.2%)
2	10 (11.9%)	3 (42.9%)	1 (2.9%)	6 (14.3%)
≥3	6 (7.1%)	0 (0.0%)	1 (2.9%)	5 (11.9%)

p-value=0.038