Poster POSA351

Dyadic Evaluation of Patient and Caregiver Treatment Burden With Once Weekly Growth Hormone (GH) Injection Schedule (somatrogon) vs Once Daily GH Injection Schedule (somatropin) by Baseline Burden Subgroup in Paediatric Patients with Growth Hormone Deficiency

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Background

- Growth hormone deficiency (GHD) is characterized by inadequate secretion of growth hormone (GH); treatment with daily GH as a subcutaneous (SC) injection is standard of care.[1]
- Somatrogon is a long-acting rhGH comprised of the amino acid sequence of hGH and 3 copies of the carboxy-terminal peptide (CTP) from human chorionic gonadotropin.[2] Phase 3 trial results in children with GHD demonstrated somatrogon to be well tolerated and demonstrated non-inferiority to Genotropin.[3]
- A separate phase 3 cross-over trial, comparing treatment burden of the once weekly regimen vs once daily Genotropin in pediatric GHD, demonstrated that patients had less treatment burden and had overall a more preferred and better injection experience with the weekly schedule compared to daily [4].
- This post-hoc analysis focuses on the treatment burden associated with weekly somatrogon compared to daily somatropin injection in subgroups identified as 'Not Burdened' or 'Burdened' at baseline (BL).

Methods

- This was a phase 3, 24-week, randomized, open-label, crossover design multicenter study (NCT03831880) in non-naïve pediatric patients with GHD aged ≥3 to <18 years, treated with GH ≥3 months
- Patients were randomized 1:1 to Sequence #1 or Sequence #2 (**Fig 1**).
- Pts were categorised at baseline based on how often they were bothered by injections in the past 4 weeks: Burdened (Sometimes, Often, Always) or Not Burdened (Never, Rarely). Analysis on primary & secondary endpoints were performed on these sub-groups.

Results

- Between Feb 2019 and Aug 2020, 87 patients from Bulgaria, Czech Republic, Slovakia, the UK, and the US were randomized and treated with ≥1 dose of study drug (**Figure 1**). 85 completed.
- At BL, 51 pts were 'Not Burdened' and 31 were 'Burdened'. Baseline characteristics for both sub-groups groups were similar.
- For the primary endpoint 'Life Interference' (LI) (Total score = **Figure 2,** individual item scores = **Figure 3**) showed both sub-groups had significantly lower (better) scores for weekly compared to daily GH.
- The majority of the other aspects of treatment experience (e.g., ease of use, willingness to continue, caregiver life interference) were favourable for somatrogon (**Figure 4)** for both sub-groups.
- After experiencing both treatments, once weekly somatrogon was associated with improvements in other aspects of treatment experience: more patients/caregivers preferred once-weekly somatrogon (**Figure 5),** a higher proportion indicated it was more convenient **(Figure 6)** and a higher proportion indicated a greater intent to comply with treatment **(Figure 7)**

Objective

- To evaluate patient and caregiver perceptions of the treatment burden associated with weekly somatrogon compared to daily somatropin injection in subgroups identified as 'Not Burdened' or 'Burdened' by GH injection at baseline.

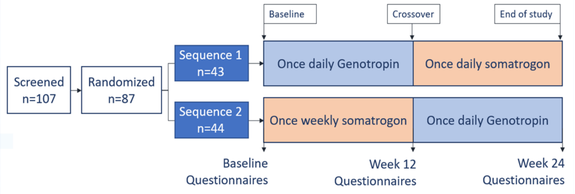
Conclusion

- In paediatric patients with GHD, compared with daily somatropin, weekly somatrogon had a lower treatment burden as shown by less LI, regardless if patients were categorized as 'Not Burdened' or 'Burdened' at baseline.
- These results highlight that the introduction of a weekly treatment could benefit all patients who currently use daily GH.

Limitation

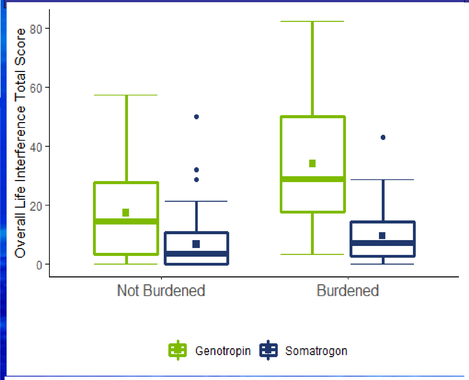
- The slight imbalance in patient numbers between the 'Burdened' (N=31) and the 'Not Burdened' (N=51) groups.

Figure 1. Study design and patient flow



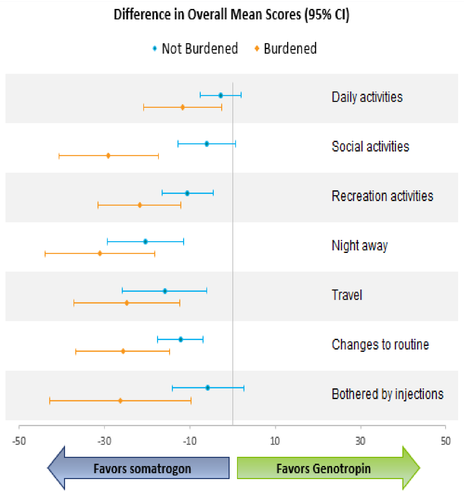
Results : **Primary Endpoint Life Interference**

Figure 2. Overall Life Interference Total Score for Not Burdened and Burdened Sub-Groups



Mean overall LI total score was significantly lower for the somatrogon group (Not Burdened' group: somatrogon (6.77) vs somatropin (17.31) mean difference: -10.54; two-sided 95% CI: [-14.59, -6.49]; P<0.0001 ; 'Burdened' group : somatrogon (9.80) vs somatropin (34.06) mean difference: -24.27; two-sided 95% CI: [-33.02, -15.51]; P<0.0001).

Figure 3. LI: Individual Items from Primary EP: Not Burdened and Burdened

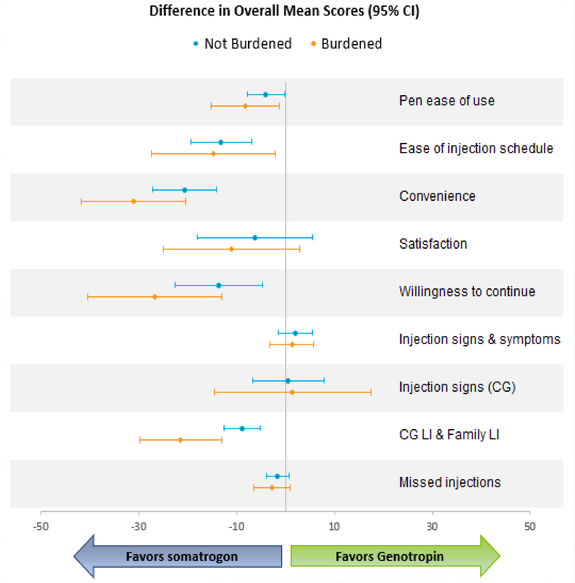


Assessments

- A recently developed, validated Dyad Clinical Outcome Assessment (DCOA) questionnaire [5] was administered electronically to the Dyad pair (child and caregiver together).
- The DCOA questionnaire is comprised of a comprehensive list of questions to determine the treatment burden and consists of 2 parts (DCOA 1 and 2).
- At baseline and after each 12-week treatment period DCOA 1 (rating treatment experience) was completed. After experiencing both treatment schedules, DCOA 2 was administered ; patients/caregivers selected their preference for daily or weekly injections

Results: **Secondary Endpoint From DCOA 1**

Figure 4. Genotropin and Somatrogon for Not Burdened and Burdened Sub-Groups



Results: **Secondary Endpoint Treatment Experience DCOA 2**

Figure 5. Which Injection schedule Do You Prefer Overall? for Not Burdened and Burdened Sub-Groups

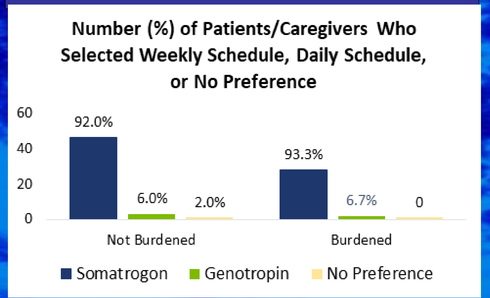


Figure 6. Which Injection Schedule Was More Convenient Overall? for Not Burdened and Burdened Sub-Groups

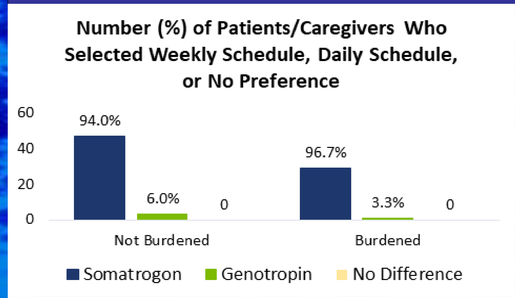
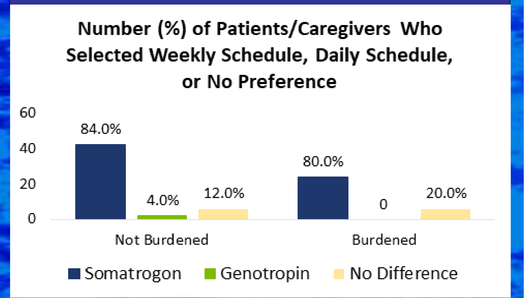


Figure 7. Which Schedule Would Be Better Able to Follow for a Longer Time? for Not Burdened and Burdened Sub-Groups



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