0.039 (0.012) mg/kg in the 4-6 yr group, and 3.5 (2.5) yr and 0.042 (0.012) mg/kg in the ≥6 yr group. Mean (SD) height SDS (HSDS) at GH start was −3.3 (1.2) in the <4 yr group, −3.1 (0.9) in the 4-6 yr group, and −2.8 (0.8) in the ≥6 yr group. After 4 and 8 yr of GH, mean (SD) ΔHSDS from baseline was 1.7 (0.7) and 2.5 (0.6) in the <4 yr group, 1.6 (0.7) and 2.2 (0.8) in the 4-6 yr group and 1.3 (0.7), and 1.7 (0.6) in the ≥6 yr group. Among patients who reached near-adult height in the study, mean (SD) HSDS was −1.9 (0.6) in the <4 yr group (n=3), −1.9 (0.8) in the 4-6 yr group (n=10), and −1.8 (1.0) in the ≥6 yr group (n=220). In the safety set (n=5643), the most commonly reported non-serious adverse reactions (AR) were headache (n=20) and arthralgia (n=5). The most common serious ARs were headache (n=3) and epiphysiolysis (n=4). ARs and serious ARs were distributed equally among groups.
Conclusions: This analysis of real-world data confirms the effectiveness and safety of GH in children born SGA, irrespective of patient age at treatment initiation.

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Efficacy and Safety of up to 2 Years of Treatment With TransCon hGH (Lonapegsomatropin) in Treatment-Naïve and Treatment-Experienced Children With Growth Hormone Deficiency

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Background: Once-weekly TransCon hGH (lonapegsomatropin) is an investigational long-acting prodrug of somatropin in development for GHD. In the pivotal 52-week phase 3 heiGHt trial, lonapegsomatropin demonstrated superior annualized height velocity (AHV) compared to the same weekly dose of daily somatropin in treatment-naïve children with GHD. In the 26-week fliGHt trial, switch from daily somatropin to lonapegsomatropin provided continued growth and maintained a good safety profile. **Methods:** Results are reported from heiGHt and fliGHt subjects who continued into the open-label long-term extension enliGHten trial (data cut: June 1st 2020). Subjects received either lonapegsomatropin (Group A; vial/syringe) or daily somatropin (Group B; pen device) in heiGHt, or lonapegsomatropin in fliGHt (Group C; vial/syringe). Upon entry into enliGHten, all subjects received lonapegsomatropin via vial/syringe, with subsequent switch to TransCon hGH Auto-Injector when available. Average IGF-1 was obtained on post-dose Day 5 (±1) in enliGHten. A by-visit ANCOVA model was used for numeric efficacy endpoints. **Results:** A total of 298 (98%) subjects continued into enliGHten. (A: n=103; B: n=55; C: n=140). The treatment difference in LS mean Δ height SDS (A vs B) at the end of heiGHt (Week 52, 1.10 vs 0.96, P=0.015) was sustained through Week 104 (1.61 vs 1.49, P=0.158). For Group C, height SDS improved from −1.42 at fliGHt baseline to −0.69 at Week 78. AHV was within the expected range for 2nd year therapy. Among children who switched (B), an attenuation in the expected 2nd year decline of AHV suggested that lonapegsomatropin had an improved treatment effect relative to the previous daily somatropin. Mean (SD) average IGF-1 SDS remained stable and generally within the expected range for all groups (Week 104, A: 0.95 [1.22], B: 1.04 [1.25]; Week 78, C:1.81 [1.08]). An improvement in injection site tolerability was observed after switching to the TransCon hGH Auto-Injector; subjects and parents also indicated overall ease-of-use of the device (assessed by the Device Usability Questionnaire). With continued lonapegsomatropin treatment, the AE profile remained consistent with what was observed in the parent trials, with no new safety signals. Throughout enliGHten and the parent trials, non-neutralizing low-titer anti-hGH binding antibodies were detected post-dose in a total of 15 subjects (5.0%). Lab parameters were stable and generally remained within the normal range throughout the trials. As of the data cut, 2 subjects have achieved near adult height (AHV <2 cm/year over the last 9 months or bone age >14 [females] or >16 [males]) and thus have completed the trial. **Conclusions:** Children treated with lonapegsomatropin showed continued improvement of height SDS through their 2nd year of therapy. Lonapegsomatropin continued to demonstrate a safety profile comparable to that of daily somatropin therapy.

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Factors Driving Patient Preferences for Growth Hormone Treatment in Japanese Children With Growth Hormone Deficiency

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Background: There are no any clear evidence to date has evaluating patients and caregiver preferences for r-hGH (recombinant-human growth hormone) injection in children in Japan. This study quantitatively evaluated the factors driving preferences for daily r-hGH injection among Japanese children with Growth Hormone Deficiency (GHD) or their respective caregivers to determine the relative importance of treatment delivery attributes. This study was performed amongst Japanese children with GHD or their caregivers who visited a specialized clinic in Japan as part of their routine care between June and July 2020. **Methods:** The participants were asked to complete a web-based discrete choice experiment (DCE) questionnaire using a handheld tablet device during a routine clinical

visit. DCE is a quantitative method widely used in healthcare to elicit preferences from participants in the absence of revealed preference data. Choice-based conjoint analysis was used to evaluate the relative importance of attributes as choice predictors and determine utilities for each attribute. Of the 47 respondents who participated in this study, 41 were caregivers who responded on behalf of the patients, and the remaining 6 were patients who completed the DCE themselves. All participants were screened by a clinician to ensure they met all eligibility criteria. **Results:** The injection schedule was found to be the most important attribute for both patients and caregivers (Relative importance: 43.6%); a once-weekly injection schedule was preferred over a daily injection schedule. For maintenance of injection devices, patients had a stronger preference for reusable pens which can be used by replacing cartridges, while caregivers preferred disposable pen devices. The storage and preparation attribute was deemed more important to patients than it was to caregivers, with patients preferring storage in room temperature even if it needed an additional mixing(reconstitution) step. Both patients and caregivers showed a clear preference for devices that offered a dose setting memory. **Conclusion:** The results of this study showed that patients prefer a once-weekly injection schedule over a daily injection schedule. A less frequent injection schedule should enhance adherence and compliance to r-hGH treatment over the long term and will also improve QoL in children with GHD. The benefits of a less frequent injection schedule can be further explored using real-world studies.

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Growth Hormone Stimulation Testing Patterns Contribute to Gender Disparities in Growth Hormone Treatment

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Introduction: Growth hormone (GH) registries demonstrate that males outnumber females 2:1 for all indications combined and 3:1 for the idiopathic short stature indication. The aim of this study was to determine if gender disparities in GH treatment are due to differences in rates of stimulation testing and/or GH prescribing. **Methods:** Retrospective chart review was performed including children aged 2-16 years seen for short stature or poor growth in 2012-2019 at a large tertiary referral center. Children previously diagnosed with GHD were excluded. Continuous variables, reported as medians [IQR], were compared by Wilcoxon rank sum test and categorical variables by Chi-squared test. A two-tailed p-value <0.05 defined statistical significance. **Results:** Of 10,125 children seen for evaluation of short stature or poor growth (35% [3542] females [F], 65% [6583] males [M]), 1,245 underwent GH stimulation testing (30% [379] F, 70% [866] M). A larger proportion of males than females were tested (M 13.2%, F 10.7%; p <0.001). Amongst the entire study population, females had lower height Z-scores than males (F -1.98 [-2.46, -1.44], M -1.80 [-2.24, -1.31]; p<0.001). This difference persisted in those who proceeded to GH stimulation testing (F -2.52 [-3.00, -2.04], M -2.18 [-2.6, -1.81]; p<0.001) and GH treatment (F -2.62 [-3.11, -2.07], M -2.19 [-2.60, -1.81; p<0.001). Mean difference between height Z-score and mid-parental height (MPH) Z-score for the entire population did not differ by sex (F -1.52 [-2.17, -0.87], M -1.52 [-2.04, -0.97]; p=0.76), but the difference was greater in females among those who underwent GH stimulation testing (F -1.95 [-2.57, -1.40], M -1.79 [-2.32, -1.32]; p=0.009) and started GH treatment (F -1.93 [-2.58, -1.48], M -1.80 [-2.30, -1.32]; p=0.016). Peak stimulated GH levels were similar for males and females (F 9.6 [6.0, 13.6] ng/mL, M 9.4 [6.1, 13.2] ng/mL, p=0.62). The proportion of children prescribed GH after stimulation testing did not differ by gender (F 55% [208], M 56% [488]; p=0.63). This finding did not change upon sub-analysis by peak stimulated GH concentration groups (peak GH concentrations <7 ng/mL, 7-10 ng/mL, and >10 ng/mL). **Conclusion:** The male predominance among children seen for subspecialist evaluation of short stature was compounded by a greater proportion of those males subsequently undergoing GH stimulation testing despite less severe short stature. Although females who underwent GH stimulation testing had greater height deficit from their genetic potential than tested males, peak stimulated GH concentrations and GH prescription rates were similar by sex. Thus, gender disparities in GH treatment occur at the subspecialist referral and stimulation testing, but not GH prescription, steps. Further, GH stimulation test results failed to account for the more severe shortness among tested females, yet another limitation identified with such testing.

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Growth Outcome and Metabolic Profile of PWS Patients Treated With GH and Differences Between AGA and SGA Group

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Objective: Prader-Willi syndrome (PWS) is a complex genetic disease associated with growth impairment, severe obesity and metabolic dysfunctions. High proportion of PWS patients are born small for gestational age (SGA), which also increase the risk of growth impairment and metabolic dysfunction. The aim of this study was to describe growth outcome and and metabolic profiles in GH treated PWS patients. We also investigated the differences in clinical outcomes between AGA and SGA group **Methods:** Data of 55 children and adults with genetically verified PWS aged more than 2 years old (32 male and 23 female, age 2-18.8 years) from single center were studied. Only patients who were treated with GH were included. The clinical characteristics and laboratory findings were reviewed