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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GROWTH AND GROWTH HORMONE

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