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Safety and Efficacy of Treatment with Lonapegsomatropin in Children with Growth Hormone Deficiency at Week 130 in the enliGHten Trial

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Background: Once-weekly lonapegsomatropin (TransCon hGH) is a long-acting prodrug of somatropin, recently approved for the treatment of pediatric growth hormone deficiency (GHD) by the US FDA. In the pivotal 52-week phase 3 heiGHt trial and the 26-week fliGHt trial, lonapegsomatropin demonstrated safety and efficacy in both treatment-naïve and treatment-experienced children with GHD.

Methods: Results are reported from Week 130 (data snapshot date: September 01, 2021) from the open-label extension trial enliGHten. Upon entry into enliGHten, all participants received lonapegsomatropin via vial/syringe, with subsequent switch to TransCon hGH Auto-Injector when available.

Results: A total of 298 participants (98% of parent trials) continued from the heiGHt or fliGHt trials into enliGHten. At Week 130, 36 participants had completed the trial and 248 participants remain enrolled. The height standard deviation score (SDS) mean (standard deviation [SD]) at Week 130 was -0.64 (0.85) compared with baseline value of -1.56 (0.88) at the start of enliGHten. Participants continued to approach the average parental height SDS mean (SD) of -0.39 (0.77). The annualized height velocity mean (SD) at Week 130 was 9.32 cm/year (0.45). Average IGF-1 SDS mean (SD) at Week 130 was 1.46 (1.18) compared with 0.52 (1.58) at enliGHten baseline. For the 36 participants who had completed the trial, 14 had reached bone age of >14 years (girls) or >16 years (boys), and 24 had completed the study based on investigator judgement that treatment for growth hormone deficiency was no longer necessary. The difference between mean (SD) height SDS at last visit and average parental height SDS was -0.05 (0.75) for the 36 completers, and 61.1% of these participants had met or exceeded the average parental height SDS. The mean (SD) total duration of hGH treatment for participants who completed the trial (including daily treatment and lonapegsomatropin) was 3.53 (0.88) years, and the mean (SD) duration of treatment with lonapegsomatropin was 2.20 (0.66). With continued lonapegsomatropin treatment, the AE profile remained consistent with what was observed in the parent trials, with no new safety signals.

Conclusions: Children and adolescents treated with lonapegsomatropin showed continued improvement of height SDS through their 3rd year of therapy. Lonapegsomatropin continued to demonstrate a safety profile comparable to that of daily somatropin therapy.

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