Lonepegsomatropin (TransCon™ hGH) in Children with Growth Hormone Deficiency: Efficacy and Safety of up to 2 Years of Treatment

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Abstract

Objectives: Lonepegsomatropin (TransCon™ hGH) is an investigational prodrug for growth hormone deficiency (GHD) that consists of a recombinant somatropin core covalently attached to a liver-specific telomeric repeat that gradually degrades to intact hGH. The current study assessed the long-term efficacy and safety of lonepegsomatropin in children with GHD.

Methods: Children aged 3-11 years old with GHD who switched from daily somatropin to lonepegsomatropin continued to grow well and maintained a good safety profile through 52 weeks, data cut: June 1, 2020. The total duration of participation for each subject in the trial will therefore depend on the conditions listed above and may be extended up to 52 weeks.

Results: Of 104 study participants who entered the extension trial (OLE), 5 subjects (4.8%) met or exceeded their target height and 15 subjects (14.4%) had a BMI > 95th percentile. Among subjects who switched from daily somatropin to lonepegsomatropin, a consistent positive treatment effect of lonepegsomatropin relative to the previous daily treatment was observed, with differences in all key efficacy parameters maintained through continued lonapegsomatropin treatment at week 104.

Conclusions: Long-term lonapegsomatropin exposure was well-tolerated and maintained efficacy, providing a convenient and efficacious long-term treatment option for children with GHD.

Keywords: lonepegsomatropin, TransCon™ hGH, GHD, growth hormone deficiency, height, safety, tolerability, BMI, IGF-1 SDS, AEs, SAEs, TEAEs, PK, PD, Bioanalytical, Pharmacokinetics, Pharmacodynamics, PK/PD, PK/PD Model, Pharmacokinetics/Pharmacodynamics, Clinical Trial, Single-arm Study, Open-label Study, Randomized Feasibility Study, Protocol, Study Design, Study Population, Study Methods, Study Results, Study Conclusion.