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Once-weekly Somapacitan Is Effective And Well Tolerated In Children With GH Deficiency: A Randomized Phase 3 Trial

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Growth hormone (GH) replacement therapy usually requires daily subcutaneous (s.c.) injections that can be burdensome for patients and their caregivers. Somapacitan, a long-acting reversible albumin-binding GH derivative, is in development for once-weekly s.c. administration in children with GH deficiency (GHD). REAL4 is a randomised, multi-national, open labelled, and active-controlled parallel group phase 3 trial, comprising a 52-week main phase and three-year extension period (NCT03811535). Two-hundred GH-treatment-naïve, prepubertal children with GHD (74.5% male) were randomly assigned in a 2: 1 ratio to receive 0.16 mg/kg/week s.c. somapacitan (n=132) or daily s.c. GH (0.034 mg/kg/day Norditropin®; n=68). The 52-week main trial results are presented here. The primary endpoint was annualized height velocity (HV) after 52 weeks of treatment. At week 52, the estimated mean HV was 11.2 cm/year for somapacitan compared to 11.7 cm/year for daily GH. The estimated treatment difference was -0.5 [95% CI -1.1 to 0.2] cm/year, confirming non-inferiority (non-inferiority threshold: -1.8 cm/year). Secondary height-related endpoints supported the primary endpoint. Insulin-like growth factor-I standard deviation score (IGF-I SDS) showed consistent increases for both somapacitan and daily GH over the 52 weeks, with change differences from baseline not statistically significant between treatment groups. At week 52, mean IGF-I SDS levels were similar between somapacitan (+0.28) and daily GH (+0.10) and within normal range (-2 to +2). Somapacitan was well tolerated, with no safety or local tolerability issues identified. There were no clinically relevant findings with respect to changes in glucose metabolism, no neutralizing anti-somapacitan or anti-GH antibodies were detected, and a low number of patients reported injection-site reactions, with similar proportions for somapacitan (5.3%) and daily GH (5.9%). In both treatment groups, 1.5% of patients reported injection site pain. Adherence was high for both treatments. The mean and median adherence for somapacitan treatment were 95.8% and 100%, respectively. The mean and median adherence for the daily GH group were 88.3% and 96.9%, respectively. In conclusion, once-weekly somapacitan has a similar efficacy and safety profile as daily GH with similar mean IGF-I levels in treatment-naïve children with GHD.

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