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# Phase 3 Study Evaluating Once Weekly Somatrogon Compared to Daily Genotropin in Japanese Patients With Pediatric Growth Hormone Deficiency (pGHD)

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**STEP 6: ABSTRACT BODY:**

**Objectives** Somatrogon is a long-acting recombinant human growth hormone consisting of the amino acid sequence of human growth hormone and three copies of the carboxy-terminal peptide of human chorionic gonadotropin. Somatrogon is being developed as a once weekly treatment for children with pGHD. A Phase 3 trial was designed to compare the efficacy and safety of somatrogon administered once weekly with Genotropin administered once daily in Japanese patients with pGHD (ClinicalTrials.gov: NCT03874013).
**Methods** 44 Japanese pGHD patients (age 3-11 years) were randomized in a 1:1 ratio to receive either once weekly somatrogon (0.66 mg/kg/week) or once daily Genotropin (0.025 mg/kg/day) subcutaneously for 12 months. Somatrogon-treated patients had a pharmacokinetic assessment in the first 6 weeks with dose escalation occurring in 3 steps, at 0.25, 0.48, and 0.66 mg/kg/week, for 2 weeks at each dose. For the remaining 46 weeks, patients in the somatrogon treatment group continued to receive somatrogon at a dose of 0.66 mg/kg/week. The primary endpoint of the study was annualized height velocity (HV) at 12 months.
**Results** Baseline characteristics were balanced and comparable between the two treatment groups. The least square means of HV at month 12 were 9.65 cm/year in the somatrogon group (n=22) and 7.87 cm/year in the Genotropin group (n=22), with a point estimate treatment difference of 1.79 cm/year (95% confidence interval: 0.97, 2.60) in favour of somatrogon. The point estimate was greater than the pre-established mean treatment difference of -1.8 cm/year, which was the non-inferiority margin met in the global Phase 3 study (n=224) with somatrogon (0.66 mg/kg/week) and Genotropin (0.034mg/kg/day) (ClinicalTrials.gov: NCT02968004). Most of the adverse events were mild to moderate in severity and somatrogon was generally well-tolerated with no notable difference in safety between the two treatment groups. Injection site pain was more common in the somatrogon group (somatrogon: 72.7%, Genotropin: 13.6%).
**Conclusions** The Japanese Phase 3 trial in patients with pGHD demonstrated that once weekly somatrogon was comparable to daily Genotropin. The annual HV after 12 months of treatment was higher in the somatrogon group than the Genotropin group. Somatrogon administration was generally well tolerated in patients with pGHD. The results of this Japanese Phase 3 study are consistent with the results previously reported from the global Phase 3 study that met its primary endpoint of noninferiority to daily Genotropin.
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Author Disclosure Information:

**R. Horikawa:** Advisory Board Member; Self; Novo Nordisk, Pfizer, Inc., Lumos Pharma, OPKO. Consulting Fee; Self; Novo Nordisk, Pfizer, Inc., Lumos Pharma, Sandoz, Ascendis Pharma. Grant Recipient; Self; Novo Nordisk, Sandoz. Speaker; Self; Novo Nordisk, Pfizer, Inc., Eli Lilly & Company, Sandoz. **T. Tanaka:** Advisory Board Member; Self; OPKO. **Y. Hasegawa:** None. **T. Yorifuji:** Advisory Board Member; Self; Pfizer, Inc.. Consulting Fee; Self; Pfizer, Inc.. **D. Ng:** None. **R.G. Rosenfeld:** Advisory Board Member; Self; Lumos, DNARx, BioMarin. Consulting Fee; Self; OPKO. **Y. Hoshino:** Employee; Self; Pfizer, Inc. **A. Okayama:** Employee; Self; Pfizer, Inc. **D. Shima:** Employee; Self; Pfizer, Inc. **R. Gomez:** Employee; Self; Pfizer, Inc.. Stock Owner; Self; Pfizer, Inc. **A. Pastrak:** Employee; Self; OPKO. Stock Owner; Self; OPKO. **O. Castellanos:** Employee; Self; OPKO.

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