Pediatric Endocrinology

**RF26 | PSAT150**

**Growth Outcomes From the Phase 2 and Phase 3 Studies of Once Weekly Somatrogon vs Daily Genotropin in Pediatric Patients With Growth Hormone Deficiency: Comparisons With Published Literature and an International Growth Study Database**

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**Background:** Somatrogon is a long-acting recombinant human growth hormone (hGH) currently in development as a once-weekly injectable treatment for children with growth hormone deficiency (GHD). In a phase 2 (NCT01592500) and a phase 3 (NCT02968004) study, patients received either once-weekly somatrogon or once-daily Genotropin.

**Aims:** Compare the phase 2 and 3 study results with growth data from published literature and a database of children treated with once-daily Genotropin.

**Methods:** In the 12-month main portion of the phase 2 study (004), patients were randomized to 1 of 3 once-weekly somatrogon doses (0.25, 0.48, and 0.66 mg/kg/week) or once-daily Genotropin (0.24 mg/kg/week). After the main portion of 004, patients continued into the open-label extension (OLE), consisting of an additional 12 months at the original somatrogon dose (Genotropin recipients were randomized to 1 of the 3 somatrogon dose regimens), after which all patients received somatrogon at 0.66 mg/kg/week. In the 12-month main portion of the phase 3 study (006), patients were randomized to once-weekly somatrogon (0.66 mg/kg/week) or once-daily Genotropin (0.24 mg/kg/week). After the main portion of 006, all patients received somatrogon (0.66 mg/kg/week) in an OLE. Four year growth data from 004 and 006 were pooled and analyzed, then compared with growth data from matched subsets of hGH-treated patients as reported by Ranke and Lindberg1 (Genotropin dose: 0.22-0.31 mg/kg/week) and Bakker et al.2 (hGH dose: most patients received 0.3 mg/kg/week) and with data from a matched cohort from the Pfizer International Growth Study Database (KIGS), in which patients received once-daily Genotropin (0.20-0.28 mg/kg/week).

**Results:** The combined mean height velocity (HV) at the end of the 12-month main portions of study 004 and 006 was 10.37 cm/year for somatrogon-treated patients. The Year 1 mean HV reported by Ranke and Lindberg for children with a chronological average-centered age of 7.5 years was 9.4 cm/year and 8.3 cm/year for children with severe and moderate GHD, respectively. The Year 1 mean HV reported by Bakker et al for children with idiopathic GHD aged 7.5 years at the beginning of hGH therapy was ~10 cm/year (for males and females). The mean annual HVs during the OLE period were 9.37, 8.97, and 9.03 cm/year at OLE Year 1, 2 and 3 in somatrogon-treated patients, which were numerically greater than HVs of 7.09, 6.35, and 6.08 cm/year at the corresponding annual visit in the matched KIGS cohort (Genotropin dose: 0.20–0.28 mg/kg/week).

**Conclusions:** Comparisons with published literature and the KIGS database indicate that children treated with once-weekly somatrogon (0.66 mg/kg/week) showed good growth, compared with children treated with once-daily hGH, strengthening the expectation that somatrogon-treated children are likely to achieve a satisfactory final adult height.

**References:**

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