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SOMATROGON GROWTH HORMONE IN THE TREATMENT OF PEDIATRIC GROWTH HORMONE DEFICIENCY: RESULTS OF THE PIVOTAL PEDIATRIC PHASE 3 CLINICAL TRIAL.

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Introduction: Somatrogon (hGH-CTP) is a long acting re-combinant human growth hormone (rhGH; somatropin) in development for once-weekly treatment of pediatric patients with growth hormone deficiency (pGHD). A 12-month phase 2 trial of somatrogon administered once weekly vs Genotropin administered once daily in patients with pGHD showed that somatrogon (0.66 mg/kg/wk) had a similar safety and efficacy profile to Genotropin (0.24 mg/kg/wk). This report summarizes top-line results from a global phase 3 pediatric trial (NCT02968004).
Objectives: The trial's objective was to test non-inferiority of somatrogon administered once weekly compared to Genotropin administered once daily in hGH-naïve, prepubertal children with pGHD.
Methods: 224 subjects were enrolled and randomized 1:1 to receive either once-weekly somatrogon (0.66 mg/kg/wk) or once-daily Genotropin (0.24 mg/kg/wk) for 12 months. Randomization was stratified by geographic region, peak GH level and age. The primary endpoint was annualized height velocity (HV) at month 12; secondary endpoints included HV at month 6, change in height SDS at months 6 and 12, IGF-1 and IGF-I SDS, immunogenicity, and safety.
Results: At baseline, mean (SD) age and height SDS of the somatrogon (N=109, 75.2% male) and Genotropin (N=115, 68.7% male) groups were 7.83 (2.66) and -2.94 (1.29), and 7.61 (2.37) and -2.78 (1.27), respec-tively. One subject in each group discontinued the study, and 95% of completers continued into an open-label extension study. At month 12, HV was 10.10 cm/yr in the somatrogon group and 9.78 cm/yr in the Genotropin group; the treatment difference of 0.33 cm/year favored somatrogon and the lower bound of the two-sided 95% confidence interval of the treatment difference (-0.24) was higher than the pre-established non-inferiority margin, demonstrating non-inferiority of once-weekly somatrogon vs daily Genotropin. HV at month 6 (10.59 cm/yr vs 10.04 cm/yr), and change in height SDS at months 6 (0.54 vs 0.48) and 12 (0.92 vs 0.87) were numerically higher in the somatrogon vs Genotropin-treated groups, respectively. The majority of adverse events were mild to moderate in severity (somatrogon: 78.9%, Genotropin: 79.1%) and, overall, weekly somatrogon was generally well-tolerated and comparable to daily Genotropin.
Conclusion: This pivotal phase 3 trial demonstrates that somatrogon once weekly is non-inferior to Genotropin once daily and that once-weekly somatrogon has a safety profile similar to daily Genotropin treatment.

Disclosure of Interest: C. Deal Conflict with: OPKO, Pfizer Inc, A. Pastrak Conflict with: OPKO Health, L. Silverman Conflict with: Novo Nordisk, Conflict with: OPKO, Pfizer Inc, S. R. Valluri Conflict with: Pfizer Inc, Conflict with: Pfizer Inc, M. Wajnrajch Conflict with: Pfizer Inc, Conflict with: Pfizer Inc, J. Cara Conflict with: Pfizer Inc, Conflict with: Pfizer Inc