

Abstract citation ID: bvad114.1522

Pediatric Endocrinology**OR21-04****Long-term Efficacy And Safety Of Once-weekly Somatrogen In Pediatric Subjects With Growth Hormone Deficiency: Results From Up To 8 Years Of Somatrogen Treatment**

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Disclosure: **Z. Zadik:** None. **N. Zelinska:** Consulting Fee; Self; Novo Nordisk, Berlin-Chemie, Medtronic, Sanofi-Aventis. Research Investigator; Self; MacroGenics, Novo Nordisk, Pfizer, Inc., Merck, OPKO Health, Ferring Pharmaceuticals, Teva Pharmaceutical Industries Ltd., Parexel, Genexine. Speaker; Self; Medtronic, Berlin-Chemie, ACINO, Novo Nordisk, Pfizer, Inc., Sanofi-Aventis, Johnson & Johnson, Wörwag Pharma. **V. Iotova:** Grant Recipient; Self; Pfizer, Inc. Speaker; Self; Novo Nordisk, Pfizer, Inc., Swixx, Sandoz, Berlin-Chemie. **Y. Skorodok:** None. **O.A. Malievskiy:** None. **N. Mauras:** Consulting Fee; Self; Agios. Grant Recipient; Self; Novo Nordisk, Abbvie. Research Investigator; Self; OPKO Health, Abbvie, Beta Bionics. **S.R. Valluri:** Employee; Self; Pfizer, Inc. Stock Owner; Self; Pfizer, Inc. **A. Pastrak:** Employee; Self; OPKO Health. Stock Owner; Self; OPKO Health. **R.G. Rosenfeld:** Advisory Board Member; Self; Lumos, DNARx, BioMarin. Consulting Fee; Self; OPKO Health. **C.T. Taylor:** Employee; Self; Pfizer, Inc. Stock Owner; Self; Pfizer, Inc. **M. Nijher:** Employee; Self; Pfizer, Inc. Stock Owner; Self; Pfizer, Inc. **C. Roland:** Employee; Self; Pfizer, Inc. Stock Owner; Self; Pfizer, Inc. **R. Wang:** Employee; Self; Pfizer, Inc. Stock Owner; Self; Pfizer, Inc. **M.P. Wajnrajch:** Employee; Self; Pfizer, Inc. Stock Owner; Self; Pfizer, Inc. **J.F. Cara:** Employee; Self; Pfizer, Inc. Stock Owner; Self; Pfizer, Inc.

Introduction: Somatrogen, a long-acting recombinant human growth hormone, is approved in multiple countries as a once-weekly treatment for pediatric growth hormone deficiency (pGHD). This abstract describes results from up to 8 y of study (main study + open label extension [OLE]) evaluating the efficacy and safety of somatrogen in pGHD. **Methods:** This randomized, open-label, dose-finding Phase 2 study comprised 5 treatment periods. Subjects were randomized 1:1:1:1 to once-weekly somatrogen (titrated up to 0.25, 0.48, or 0.66 mg/kg/wk) or once-daily Genotropin (0.24 mg/kg/wk) for 12 mo of the main study (periods I and II), after which they were eligible to enroll in the OLE (periods III-V). In period III, somatrogen recipients continued at their previous dose; Genotropin recipients were re-randomized to 1 of the 3 somatrogen doses. In period IV (OLE Year (Y)2–4), all subjects switched to somatrogen 0.66mg/kg/wk; in period V (OLE Y5 onward), all subjects switched from single-use somatrogen vials to prefilled pen devices (somatrogen 0.66 mg/kg/wk). **Results:** Of 53 subjects who completed the main study, 48 entered the OLE. At OLE entry, 66.7% were male, all but 1 were pubertal Tanner stage 1, and mean (SD) age was 7.7 (2.1) y. At OLE Y6 end, there were 30 subjects with a mean±SD height velocity (HV) of 6.47±2.07 cm/y and a mean±SD height SD score (HT SDS) of 0.43±0.94. At OLE Y7 end, 26 subjects had a mean±SD HV of 5.31±1.68 cm/y and a mean HT SDS of 0.44±0.96. Four subjects achieved final height (defined as HV <1 cm/y). Their HVs in the last 6-mo interval and last assessed heights were: subject 1: 0.54 cm/y, 174.5 cm; subject 2: 0.7 cm/y, 173.2 cm; subject 3: 0.56 cm/y, 179.5 cm; and subject 4: 0.19 cm/y, 168.7 cm. Somatrogen efficacy was not affected by positive test results for antidrug antibodies (ADA). HV and cumulative ΔHT SDS (cΔHT SDS) were similar between subjects who tested ADA+ vs ADA-: at OLE Y6 end, 15 who tested ADA+ had HV 6.47±1.69 cm/y and cΔHT SDS 3.09±1.04; and 15 who tested ADA- had HV 6.48±2.46 cm/y and

cΔHT SDS 3.62 ± 1.40 . At OLE Y7 end, 14 subjects who tested ADA+ had HV 5.22 ± 1.52 cm/y and cΔHT SDS 3.06 ± 0.98 ; 12 who tested ADA- had HV 5.42 ± 1.91 cm/y and cΔHT SDS 3.75 ± 1.39 . In OLE Y7, TEAEs were reported in 35.5% subjects, which was lower than the main study (69.0%) and OLE Y1–6 (range: 41.9–57.5%). No serious TEAEs or TEAEs leading to study drug withdrawal occurred in OLE Y7. Study drug-related TEAEs occurred in 2 subjects: keeled chest acquired and scoliosis in 1 and arthralgia in 1. No deaths were reported throughout the OLE.

Conclusion: Following up to 8 y of somatrogen treatment, including 7 y of the OLE, subjects demonstrated continued growth, 4 achieved final height, and somatrogen maintained a favorable safety profile. Testing ADA+ did not appear to affect subject growth. **Clinicaltrials.gov:** NCT01592500. **Acknowledgements:** The authors wish to thank all the investigators involved in this study.

Presentation: Saturday, June 17, 2023