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Results From an Open-label Extension of the Phase 2 Dose Finding Study of Once Weekly Somatrogon vs Daily Genotropin in Pediatric Patients With Growth Hormone Deficiency (GHD)

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STEP 6: ABSTRACT BODY:

Background Somatrogon, a long-acting recombinant human growth hormone, is being developed as a once weekly treatment for pediatric patients (pts) with GHD. A phase 2, 12 month study (NCT01592500) in pts with GHD showed that weekly somatrogon at 0.66 mg/kg/week had similar efficacy and safety to daily Genotropin. Pts who completed 12 months of treatment could be enrolled into an open-label extension (OLE).

Aims Evaluate the safety and efficacy of long-term exposure to somatrogon in pediatric pts with GHD who continued in the OLE for up to an additional 5 years.

Methods Methods for the main phase 2 study were published previously (Zelinska et al, 2017), in which 53 pts were randomized to 1 of 3 weekly somatrogon dose cohorts (0.25, 0.48, and 0.66 mg/kg/week) or the daily Genotropin cohort (0.24 mg/kg/week) for 12 months. After the main study (Periods I/II), 48 pts who consented to participate continued in the OLE, consisting of 3 periods: Period III=12 additional months at original somatrogon dose (Genotropin recipients randomized to 1 of the 3 somatrogon dose regimens); Period IV=subsequent years 2-4 with all pts receiving somatrogon at 0.66 mg/kg/week; Period V=ongoing, with pts transitioned from the vial to a pre-filled pen device at the same somatrogon dose (0.66 mg/kg/week). Data up to 1 year of Period V are reported.

Results Overall subject retention in different periods of this long-term study ranged from 87.5% to 97.7%. 39 pts (81.3%) reported at least one treatment-emergent adverse event (TEAE). Most TEAEs were mild or moderate in intensity and most were classified as unrelated to study treatment. 3 pts (6.3%) reported at least 1 serious adverse event (SAE); most SAEs were considered unrelated to study treatment, except for 1 instance of scoliosis. At the end of Period III, the mean annual height velocity (HV) was similar for the 0.25 and 0.48 mg/kg/week dose cohorts (7.73±1.89 and 7.54±1.28 cm/year, respectively) but was higher in the 0.66 mg/kg/week dose cohort (8.81±1.12 cm/year), consistent with the results of the main study. The HV at Periods IV and V showed sustained growth response. Height SDS showed consistent improvement and near normalization of height for age and gender after up to 6 years on somatrogon, irrespective of initial cohort assignment; height SDS at baseline of the main study was -3.98±1.22 and was well within the normal range at -0.69±0.87 at the end of Year 1 in Period V. IGF-1 SDS values remained above baseline and were maintained within the therapeutic target range with weekly somatrogon treatment at all time points in all OLE periods. Anti-drug antibodies (ADAs) were reported in 18 pts, of which 10 pts had ADAs in the main study. The presence of ADAs did not impact efficacy or safety.

Conclusions Somatrogon administered once weekly for up to 5 years after the main study was generally well tolerated and participants showed sustained improvement in annual HV, height SDS, and delta height SDS.

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Author Disclosure Information:

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, Ferring Pharmaceuticals, Teva Pharmaceutical Industries Ltd., Paraxel, Genexine. Speaker; Self; Medtronic, Berlin-Chemie, ACINO, Novo Nordisk, Pfizer, Inc., Sanofi-Aventis, Johnson &Johnson, Wörwag Pharma. **V. Iotova:** Advisory Board Member; Self; Pfizer, Inc., Sandoz, Sanofi, Medtronic. Advisory Board Member; Spouse; Pfizer, Inc., Novartis, AstraZeneca, Boehringer, Bayer, Inc., Servier, Menarini Berlin-Chemie, Amgen Inc. Grant Recipient; Self; Pfizer, Inc.. Research Investigator; Self; OPKO, Pfizer, Inc., Ascendis Pharma, Merck, Novo Nordisk, Sanofi, Resolute, Novartis. Research Investigator; Spouse; Novartis, AstraZeneca, Boehringer, Bayer, Inc., Menarini Berlin-Chemie, Amgen Inc, Teva Pharmaceutical Industries Ltd., Novo Nordisk, Polpharma, Sanofi, MSD, Servier. Speaker; Self; Pfizer, Inc., Sandoz, Novo Nordisk, Sanofi, Berlin-Chemie, Eli Lilly & Company, Medtronic, Shire. Speaker; Spouse; Pfizer, Inc., Novartis, AstraZeneca, Boehringer, Bayer, Inc., Servier, Menarini Berlin-Chemie, Amgen Inc, Teva Pharmaceutical Industries Ltd., Sandoz, Actavis, Gedeon Rihter, Polpharma, Krka, Sanofi, Shire, MSD, Merck. **Y. Skorodok:** Speaker; Self; Berlin-Chemie, Sanofi. **O.A. Malievskiy:** None. **N. Mauras:** Grant Recipient; Self; Novo Nordisk, Abbvie. Research Investigator; Self; OPKO. Speaker; Self; Novo Nordisk. **S.R. Valluri:** Employee; Self; Pfizer, Inc.. Stock Owner; Self; Pfizer, Inc. **A. Pastrak:** Employee; Self; OPKO. Stock Owner; Self; OPKO. **R.G. Rosenfeld:** Advisory Board Member; Self; Lumos, DNARx, BioMarin. Consulting Fee; Self; OPKO.

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