Abstract citation ID: bvac150.1226

Pediatric Endocrinology

LBMON197

Predictors Of Response To Growth Hormone Treatment In Pediatric Growth Disorders: Analysis From The Answer Program And Nordinet® IOS

Judith L. Ross, MD[1], Moshe Fridman, PhD[2], Nicky Kelepouris, MD[2], Kristine Murray, PhD[2], Nils Peter Krone, MD FRCPCH[3], Michel Polak, MD, PhD[4], Navid Nedjatian, MD[5], Tilman Robert Rohrer, MD[6], Alberto Pietropoli, PhD[5], Neil Lawrence, MD[3], and Philippe Ferdinand Backeljauw, BSC, MD[7]

[1]Nemours duPont Hospital for Children, Thomas Jefferson University, Philadelphia, PA, USA[2]Novo Nordisk, Inc., Plainsboro, NJ, USA[3]University of Sheffield, Sheffield, United Kingdom[4]Hôpital Universitaire Necker Enfants Malades, Paris, France[5]Novo Nordisk Health Care AG, Zurich, Switzerland[6]University of Saarland, Homburg/Saar, Germany; [7]Cincinnati Children's Hospital Medical Center, Cincinnati, OH, USA

Background: The American Norditropin® Studies: Web-Enabled Research (ANSWER) Program and the NordiNet® International Outcome Study (NordiNet® IOS) were large-scale, non-interventional studies intended to gather long-term data on the effectiveness and safety of Norditropin® (somatropin) treatment in the real-world setting. Data were collected in the US and Europe from 2006 to 2016. **Objective:** To determine predictors of response to daily growth hormone (GH) for height outcomes using a longitudinal analysis across 5 years of real-world data from pediatric patients with growth hormone deficiency (GHD), Turner syndrome (TS), Noonan syndrome (NS), and Prader-Willi syndrome (PWS). **Methods:** Data were combined for ANSWER and NordiNet® IOS studies for GH-treatment-naïve pediatric patients on daily somatropin. Descriptive statistics for baseline demographics, outcomes, and GH (mg/kg/d) data were analyzed for each indication; statistics for outcome and GH data were analyzed over time for absolute values and change from baseline values at year 1 through year 5. A longitudinal statistical approach was used to identify factors, including age at treatment start and HSDS at baseline, significantly associated with change in height SDS from baseline (ΔHSDS) in a regression model. Repeated-measures regression analyses were performed on ΔHSDS, adjusting for covariates. Longitudinal growth responses over the follow-up years were evaluated. **Results:** Overall, 14,295 patients were included in the analyses: 3766 females and 8917 males with GHD, 1307 patients with TS, 55 females and 148 males with NS, and 52 females and 50 males with PWS. Mean (SD) GH dose for patients with GHD was 0. 037 (0. 012) mg/kg/d (females) and 0. 038 (0. 012) mg/kg/d (males), for those with TS was 0. 045 (0. 011) mg/kg/d, those with NS was 0. 042 (0. 012) mg/kg/d, and those with PWS was 0. 031 (0. 013) mg/kg/d. HSDS improved over time across all indications. Greater improvement in HSDS was observed for years 1 and 2 of follow-up, with a plateau in years 4 and 5; this plateau was most apparent in patients with TS. Age at start of GH therapy had a significant effect on ΔHSDS for all indications (P<0. 001 for all). Lower baseline HSDS on average resulted in lower HSDS improvement over time. In a regression model, the GH dose (mg/kg/d) from the previous year was positively associated with ΔHSDS, with a significant positive slope for GHD and TS (P<0. 0001 for both). A 0. 01 increase in GH mg/kg/d increased the average HSDS improvement by 0. 02 to 0. 03 units in GHD and 0. 03 units in TS. Slopes for NS (P=0.3534) and PWS (P=0.1485) were not significant. **Conclusions:** On average, GH treatment resulted in HSDS improvements over baseline across all indications; patients with GHD had the highest improvement, while patients with TS had the lowest improvement. Earlier treatment with GH led to better outcomes in HSDS. Increasing the dose of GH also led to better outcomes in HSDS.

Presentation: Monday, June 13, 2022 12:30 p.m. - 2:30 p.m.