Pediatric Endocrinology

PEDIATRIC GROWTH AND ADRENAL DISORDERS

24-Month Efficacy and Safety of Once Weekly and Every Other Week Administration of GX-H9, Hybrid FC-Fused Long-Acting Human Growth Hormone: A Phase 2 Study in Children With Growth Hormone Deficiency

Oleg Malievskiy, MD, PhD[1], Aryaev Mykola, MD[2], Nataliya Zelinska, MD, PhD[3], Elena Bolshova, MD, PhD[4], Ganna Senatorova, MD[5], György Oroszlán, MD, PhD[6], Julia Skorodok, MD[7], Valentina Peterkova, MD, PhD[8], Nataliya Chorna, MD[9], Tamila Sorokman, MD, PhD[10], Seung Yang, MD,PhD[11], Ji-Eun Lee, MD, PhD[12], Agota Muzsnai, MD, PhD[13], Jin Soon Hwang, MD,PhD[14], Sang-Yoon Lee, MD[15], HyunJoo Son, MS[16], SuJin Heo, MS[16], Minkyu Heo, PhD[17], Yun Jung Choi, PhD[17], Young Chul Sung, PhD[17].

[1]Bashkir State Medical University, Ufa, Russian Federation, [2]Odessa National Medical University, Odessa, Ukraine, [3]Ukrainian Scientifically Practical Center of Endocrine Surgery and Transplantation of Endocrine Organs and Tissues, Kyiv, Ukraine, [4]Institute of Endocrinology and Metabolism named after Komisarenko NAMS of Ukraine, Kyev, Ukraine, [5]Kharkiv National Medical University, Kharkiv, Ukraine, [6]Markusovszky University Hospital, Szombathely, Hungary, [7]St. Petersburg State Pediatric Medical University, St. Petersburg, Russian Federation, [8]Institute of Pediatric Endocrinology, Moscow, Russian Federation, [9]Regional Clinical Children's Hospital, Ivano-Frankivsk, Ukraine, [10]Bukoinian State Medical University, Chernivtsi, Ukraine, [11]Kangdong Sacred Heart Hospital, Seoul, Korea, Republic of, [12]INHA University Hospital, Inchon, Korea, Republic of, [13]St. John's Hospital and United Hospitals of Northern Buda, Budapest, Hungary, [14]Ajou University School of Medical, Suwon City, Korea, Republic of, [15]Handok Inc., Seoul, Korea, Republic of, [16]Handok, Seoul, Korea, Republic of, [17]Genexine Inc., Seongnam, Korea, Republic of.

SAT-LB15

Objectives GX-H9 is a long-acting form of recombinant human GH under clinical development for both adults and children with GHD. In this report, 24-month efficacy and safety of once weekly and every other week (EOW) administration of GX-H9 were evaluated, in addition to Genotropin® switch-ability to GX-H9 after 12-month of treatment. **Methods** Subjects were randomly assigned to receive either one of three doses of GX-H9 (0.8 mg/kg/week, 1.2 mg/kg/week or 2.4 mg/kg every other week) or 0.03 mg/kg/day of Genotropin®. Treatment duration is 24-month for all patients in GX-H9 arms while patients in Genotropin® arm were re-randomized to one of three doses of GX-H9 at the completion of the first 12-month of treatment. Doses of GX-H9 were adjusted throughout the treatment period whenever necessary, based on IGF-1 levels. **Results** Out of 56 randomized, 54 received either GX-H9 or Genotropin®. Fifty subjects completed the 12-month treatment period. Of 50, 45 subjects completed the next 12-month, comprising 33 patients from GX-H9 and 12 patients who switched from Genotropin®. First year/second year mean±SD annualized height velocity (aHV) for 0.8 mg/kg/week, 1.2 mg/kg/week or 2.4 mg/kg every other week of GX-H9 were 10.50±2.54/9.14±1.96, 11.76±1.96/9.88±1.92 and 11.03±2.92/9.72±1.90 cm/year, respectively. First year mean±SD aHV for Genotropin® was 9.14±3.09 cm/year. Patients switched to one of the three doses of GX-H9 in the second year showed comparable aHV in the second year (8.73±2.69/7.60±0.90/9.13±1.07 cm/year for 0.8 mg/kg/week, 1.2 mg/kg/week and 2.4 mg/kg/EOW GX-H9, respectively). No significant slow-down of the growth was observed in the second year from patients who received GX-H9 throughout and patients who switched from Genotropin®. Mean change in height SDS after 12 months/24 months of GX-H9 treatment throughout from baseline treatment improved continuously (+1.10/+1.61 and +1.31/+1.89 and +1.15/+1.69 for 0.8 mg/kg/week, 1.2 mg/kg/week and 2.4 mg/kg EOW GX-H9, respectively). First year mean change in height SDS for Genotropin® was +0.92 SDS, and showed comparable improvement in height SDS after switching to GX-H9 weekly arms (+0.76 and +0.79 SDS for 0.8 mg/kg/week and 1.2 mg/kg/week, respectively). Most treatment-emergent adverse events were evaluated as unrelated to the study drug and were mild or moderate in severity. No new safety concerns were observed throughout 24 months of long-term GX-H9 treatment or after switching to GX-H9 from Genotropin®.**Conclusions** Growth response and safety profile of GX-H9 in children with GHD is comparable to those of daily GH, achieving robust growth rates after 24-month treatment. Subjects switched from Genotropin® in the second year, also showed substantial catch-up growth indicated by improvement in height SDS. GX-H9 has a unique potential to be a convenient long-term GH providing not only weekly but also twice-monthly treatment.

Neuroendocrinology and Pituitary

PITUITARY TUMORS II

An International Simulated Use Study (PRESTO) to Evaluate Nurse Preferences Between the Lanreotide Autogel New Syringe and Octreotide Long-Acting Release Syringe

Daphne T. Adelman, RN[1], Xuan-Mai Truong-Thanh, MD[2], Marion Feuilly, PharmD[2], Aude Houchard, MPH[2], David Cella, PhD[1].

[1]Northwestern University, Chicago, IL, USA, [2]Ipsen Pharma, Boulogne-Billancourt, France.

MON-LB47

Background: A new lanreotide autogel/depot (LAN) syringe was developed based on feedback from a human factors study to improve user experience.
Methods: PRESTO was a multinational, simulated-use study in nurses with ≥2 years' experience injecting LAN or octreotide long-acting release (OCT LAR) in patients with acromegaly and/or neuroendocrine tumors, which aimed to assess injector preference between the LAN new syringe and the current OCT LAR syringe. Participating nurses were invited to test both the LAN new syringe (120 mg) and the current OCT LAR syringe (20 mg or 30 mg), using injection pads. The sponsor was not involved in these sessions. In an anonymous web-based questionnaire, nurses reported overall preference ('strong' or 'slight'; primary endpoint), and rated and ranked the importance of nine attributes for each syringe (1 [not at all] to 5 [very much]).
Results: In total, 90 nurses attended injection sessions and completed valid questionnaires. Overall, 97.8% of nurses expressed a preference (85.6% 'strong', 12.2% 'slight')