Long-term effects of GH therapy in adult patients with Prader- Willi syndrome: a longitudinal study.

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Introduction: Prader-Willi syndrome (PWS) is a complex disorder resulting from the failure of expression of paternal alleles in the PWS region of chromosome 15. The PWS phenotype resembles that observed in the classic non-PWS GH deficiency (GHD), including short stature, excessive fat mass, and reduced muscle mass. To date, a small number of studies on the long-term effects of GH treatment are available in adult subjects with PWS.

Methods: In this longitudinal study, 12 obese subjects with PWS (GHD/non-GHD 6/6) were treated for a median of 17 years, with a median GH dose of 0.35 mg/ day. The median age was 27.1 years. Anthropometric, body composition, hormonal, biochemical, and blood pressure variables were analyzed in all subjects.

Results: Waist circumference was significantly lower at the end of the treatment period (p-value=0.0449), while body mass index (BMI) did not differ significantly. Compared to the baseline, a highly significant reduction of Fat Mass % (FM%) was observed (*p*-value=0.0005). IGF-I SDS values significantly increased during GH therapy (*p*-value=0.0005). A slight impairment of glucose homeostasis was observed after GH therapy, with an increase in the median fasting glucose

levels, while insulin, HOMA-IR, and HbA1c values remained unchanged. Considering GH secretory status, both subjects with and without GHD showed a significant increase in IGF-I SDS and a reduction of FM% after GH therapy (*p*-value= 0.0313 for all).

Discussion: Our results indicate that long-term GH treatment has beneficial effects on body composition and body fat distribution in adults with PWS associated with obesity. However, the increase in glucose values during GH therapy should be considered, and continuous surveillance of glucose metabolism is mandatory during long-term GH therapy, especially in subjects with obesity.

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