



ORAL PRESENTATION

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# Response to low dose growth hormone treatment in infants and toddlers with Prader-Willi Syndrome

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## Aim

We aimed to assess the benefits and safety of low dose growth hormone treatment (GHT, 4.5 mg/m<sup>2</sup>/week) in young children with genetically confirmed Prader-Willi Syndrome (PWS).

## Methods

Data of 20 infants (2-12 months) and 24 toddlers (13-24 months) were collected from the PWS-OZGROW database. The two groups were evaluated for standard deviation scores (SDS) of height (length under age 2 years), weight and BMI using the World Health Organization standards (SDS<sub>WHO</sub>) and PWS specific BMI (SDS<sub>PWS</sub>), bone age (BA), insulin-like growth factor-1 (IGF-1) levels, hypotonia, developmental delay, spinal curvature, sleep studies and adverse events over 2 years of GHT.

## Results

At commencement of GHT infants had a reduced BMI SDS<sub>WHO</sub> (P=0.003), while toddlers had a reduced height SDS<sub>WHO</sub> (P=0.014). The height/length SDS<sub>WHO</sub> of infants increased from -1.09±1.15 at baseline to -0.26±0.89 after one year and -0.02±0.80 after two years GHT (GLM repeated measures; P < 0.0001), and in toddlers increased from -2.11±1.45 to -1.11±1.11 and -0.87±0.94 (P < 0.0001). BMI SDS<sub>WHO</sub> increased in both groups (data not shown), while BMI SDS<sub>PWS</sub> decreased (P < 0.0001, age groups P > 0.05) from 0.40±0.84 to -0.07±0.67 at Year 1 and -0.31±0.95 at Year 2 (both age groups combined). Pre-term and full term children did not differ significantly in response to GHT, nor did children with deletion (14) and

uniparental disomy (16). All children had low to very low serum IGF-1 at baseline which increased to within the normal reference range for the majority of children (61%) with the remainder modestly increased during the first 2 years of treatment. An improvement in tone, spinal curvature and developmental delay was noted in those who were more severely affected at baseline. Two children developed scoliosis. Three children ceased GHT temporarily to adjust positive airway pressure settings or for tonsillectomy following onset or worsening of obstructive and/or central sleep apnoea. Bone age was not advanced and no other serious adverse events were reported during the two year GHT.

## Conclusion

Treating young children (<2 yrs) with PWS with 4.5 mg/m<sup>2</sup>/week of GH normalises height and achieves IGF-1 levels in the normal range in the majority of patients. The risk of respiratory adverse events can be minimised by regular monitoring. The dose was sufficient to keep height and most IGF-1 values in the normal range and PWS specific BMI SDS in a negative range and may lower potential risks of long-term treatment of very young children with PWS.

On behalf of the PWS and OZGROW collaboration

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