

# Effects of eight years growth hormone treatment on the onset and progression of scoliosis in children with Prader-Willi syndrome

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## Conclusion

**Preliminary data show reassuring results: no difference in the severity of scoliosis after eight years of GH-treatment compared to untreated children with PWS.**

### Background

Most children with Prader-Willi syndrome (PWS) develop scoliosis. Our previous study found no difference in the onset of scoliosis and curve progression between growth hormone (GH) treated and untreated children. There are, however, no longer-term results about the effects of GH treatment on the onset and progression of scoliosis with PWS.

### Aim

To investigate the effects of eight years of GH on the onset and progression of scoliosis in children with PWS and to assess whether there are correlations with trunk lean body mass.

### Participants & Method

Prospective cohort study in 34 children (preliminary data) with PWS. All patients were naïve to GH treatment at time of enrolment and received GH at a dose of 1 mg/m<sup>2</sup>/d (≈ 0.035 mg/kg/d).

Main outcome measures were onset of scoliosis (determined as a Cobb angle of 10° or higher) and the progression of the scoliotic curve (expressed as the change in the Cobb angle) during 8 years of GH. After 8 years of GH the outcomes were compared with a group of 15 children with PWS who did not receive GH.

### Prevalence and severity of scoliosis after 8 years of GH compared to controls

	After 8 years of GH	GH-untreated children
Age (years)	9.4 (9.0 to 10.1)	11.9 (11.5 to 14.8) <sup>a</sup>
Height SDS	0.7 (-0.3 to 1.6)	-2.2 (-3.8 to -1.8) <sup>a</sup>
Weight SDS	1.0 (0.3 to 2.1)	2.2 (7.8 to 8.4) <sup>a</sup>
TrunkLBM:BSA	8.5 (7.7 to 9.2)	8.1 (7.8 to 8.4) <sup>b</sup>
Cobb Angle (°)	15 (10 to 23.5)	35 (19.0 to 41.5) <sup>a</sup>
IS (%)	78%	100%
Brace (%)	6.1%	13%
Surgery (%)	6.1%	33%

<sup>a</sup> p < 0.001, <sup>b</sup> P < 0.05

IS: Idiopathic scoliosis

GH-treated children had a significantly smaller Cobb angle than the untreated children.

The group treated with GH for 8 years had a significantly higher TrunkLBM:BSA compared to untreated children with PWS.

### Baseline characteristics (N=34)

Age at start GH (years)	1.3 (0.9; 2.3)
Gender (♂ / ♀)	20 / 14
Genetic subtype	
- Deletion	20
- mUPD	13
- ICD	1
Height SDS	-1.8 (-2.7; -0.6)
Weight SDS	-1.5 (-2.4; -0.5)
BMI SDS	-0.7 (-1.2; 0.6)
TrunkLBM:BSA	6.9 (6.6; 7.5)
IGF-1 SDS	-2.7 (-4.3; -1.1)
Cobb Angle (°)	10.5 (5.4;17)

Data expressed as median (IQR).

TrunkLBM:BSA: ratio of truncal lean body mass divided by body surface area

### Onset of idiopathic scoliosis in GH-treated children with PWS

- Median (IQR) age of onset of idiopathic scoliosis was 3.7 (2.1 to 5.9) years.
- Brace therapy was started at a median (IQR) age of 8.0 (5.1 to 12.5) years. The mean (SD) Cobb angle at start brace therapy was 38.5° (7.5).



### Idiopathic scoliosis

Example of an idiopathic scoliosis (IS). In older children with PWS, IS is the most common type of scoliosis.

