

# Beneficial effects of long-term growth hormone treatment on adaptive functioning in infants with Prader-Willi syndrome

## Conclusions

- Marked delay in adaptive functioning in children with PWS
- An earlier age at start of growth hormone in infancy is associated with better adaptive functioning on the long-term

### Background

Prader-Willi syndrome (PWS) is a neurogenetic developmental disorder caused by the absence of paternal expression of genes in chromosome 15q11-q13. Its main characteristics are hypotonia, short stature, obesity, intellectual disability and a specific behavioral phenotype.

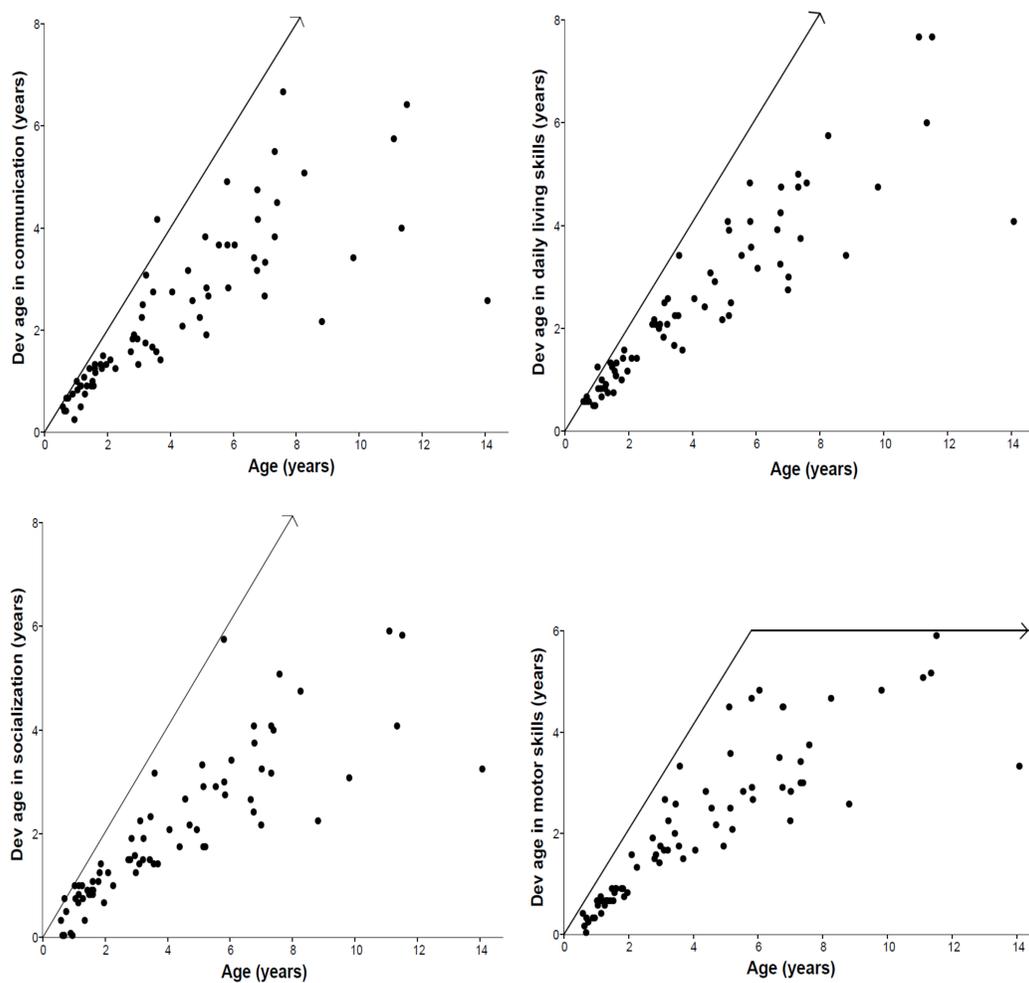
### Aim

To investigate the effect of growth hormone (GH) treatment on adaptive functioning in children with PWS.

### Methods

- RCT: 75 children
  - 42 infants for 1 year
  - 33 prepubertal children for 2 years
- Long-term study for 7 years: 53 children
  - 36 infants at start
  - 17 prepubertal children at start
- GH dose 1 mg/m<sup>2</sup>/day
- Adaptive functioning assessed using the Vineland Adaptive Behavior Scale (VABS) at start & at the end of RCT, and after 7 years of GH treatment

Figure 1. The relation between age and developmental age per domain of the VABS test at baseline



Dev age= developmental age. The arrow represents the maximal developmental age in normal development and is similar to age, except in motor skills in which the maximal developmental age is 6 years.

### Results

- Marked delay in adaptive functioning in children with PWS
- No effect of short-term GH treatment versus no treatment on adaptive functioning
- After 7 years of GH treatment, an earlier age at start of GH during infancy was associated with better skills in communication ( $\beta = -0.533$ ,  $p = 0.018$ ), daily living skills ( $\beta = -0.440$ ,  $p = 0.041$ ), socialization ( $\beta = -0.503$ ,  $p = 0.048$ ) and motor skills ( $\beta = -0.706$ ,  $p = 0.003$ ), after adjustment for change in IQ over time