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

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**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/m²/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

**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