

# Growth hormone therapy for children with PWS



## Introduction

Use of GH treatment for children with PWS was approved in Europe in 2001. There have now been many research trials, mostly done on a small scale, which have produced data showing clear benefits for children with PWS. These include:

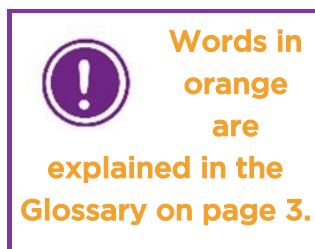
- Increased growth (height)
- Improved tolerance for exercise
- Improved bone health
- Improved motor development, muscle strength and tone and better body composition (i.e. more muscle mass and less fat mass)
- Improved respiratory function

## Should every child with PWS have GH treatment?

GH treatment should be considered for every child with PWS, and the NHS will provide treatment for a child with PWS if their doctor recommends it and has confirmed it is safe to go ahead. GH treatment is not compulsory and some families decide not to have GH for their child.

## There are some medical reasons why treatment with GH cannot be started:

- A significant illness in addition to the PWS
- Severe obesity
- Severe breathing problems
- Untreated **central and/or obstructive sleep apnoea**
- Uncontrolled diabetes



## Approval by NICE

In the UK, GH treatment for children with PWS has been approved by the National Institute for health and care Excellence (NICE).

<https://www.nice.org.uk/guidance/TA188/informationforpublic>

To find out how to access growth treatment for your child through the NHS, please see the article - Growth Hormone therapy in Prader-Willi Syndrome - UK practice

<https://www.pwsa.co.uk/assets/files/GHT-UK-Practice.pdf>



## Side effects of growth hormone treatment

Growth hormone treatment has been used for children with growth problems since the 1960s and recombinant (laboratory manufactured GH) has been available since 1985. GH has been used to treat PWS since the 1990s.

Follow up of children who have been treated with GH for any reason has shown that there have been very few reported side effects.

### For anyone treated with GH for any reason:

- There is a side effect called benign intracranial hypertension which causes very severe headaches. This is rare and goes away if GH is stopped.
- Follow up of people who have been treated with GH in the past does not suggest that there is an increased risk of other illnesses later on, but these studies are continuing.

### For children with PWS treated with GH:

- When the first studies of GH treatment in PWS were started there were reports of a few children who died in the time around starting GH. A clear link with the GH treatment was not proved, but it has been shown that GH changes the airway for some children with PWS and could potentially make obstructive apnoea worse. This is the reason for the assessments that are done before starting GH and why they are now closely monitored whilst on GH, especially when it is newly started. The risk is greater for children who are obese.
- Diabetes can become more difficult to control if GH is started.
- 30-40% of children with PWS develop scoliosis (which can vary from very minor to severe). Studies have not shown that there is an increased risk of this happening with GH treatment and may be a reason why GH is not started.

## At what age should growth hormone treatment start?

The first studies of GH in children with PWS looked at older children, but more recent research has shown benefit in younger children, and there have been studies looking at GH in infants. There is good evidence to support starting GH early in life, before 2 years of age, unless there are medical reasons why this would not be advised.

## Assessment and tests before starting GH

Because of the concern that GH may have respiratory effects, it is recommended that a sleep study should be performed before GH therapy is started. This test might suggest that treatment is needed before GH can start, (for example removal of tonsils or adenoids, starting overnight respiratory support).

Some specialists check for growth hormone deficiency before starting GH (either with IGF 1 testing or a stimulation test), but you do not have to confirm GH deficiency to start GH treatment in PWS.

Before starting GH, your child should have a blood test to check for underactive thyroid (hypothyroidism) as this should be treated before GH can be started. Your specialist will also check your child's growth level IGF-1 to allow monitoring of GH treatment.

### Monitoring treatment

Whilst receiving GH treatment, your child should be monitored every 3-6 months to ensure that the treatment is both safe and effective.

They will have regular blood tests and medical assessment which can include:

- Height and weight measurement and assessment of body composition.
- Blood tests about once a year, including thyroid function (looking for **hypothyroidism**) and **IGF 1** (which is a marker of GH levels in the body and a way of monitoring the dose of GH) and markers for diabetes.
- Assessment of breathing and sleeping patterns. A repeat sleep study should be considered for all children with PWS within the first 3-6 months after starting on GH and at regular intervals thereafter.
- Checks for scoliosis.
- Assessment for any other side effects of GH treatment.

### Glossary

**IGF-1** - a hormone produced by the body in response to GH. Blood levels of IGF1 are used as a way of monitoring the dose of GH.

**Hypothyroidism** - Hypothyroidism (underactive thyroid gland) is the term used to describe a condition in which there is a reduced level of thyroid hormone (thyroxine) in the body. This can cause various symptoms, the most common being: tiredness, weight gain, constipation, aches, dry skin, lifeless hair and feeling cold. Treatment is usually easy by taking a tablet each day to replace the missing thyroxine. Treatment usually works well and symptoms usually go. Around a third of children with PWS have this condition, but it is less common in adults with PWS.

**Obstructive sleep apnoea** - occurs when the brain sends the signal to the muscles and the muscles make an effort to take a breath, but they are unsuccessful because the airway becomes obstructed and prevents an adequate flow of air.

### Weight management during GH treatment

**GH treatment does not change the problems of increased appetite in PWS, and is not a solution to weight gain. During the first few years of life it is important to get your child into good habits with their diet and set firm boundaries around their eating habits. Children with PWS need less calories, approximately 60–80% compared to children without PWS. Regular exercise is helpful and GH treatment can help by improving muscle strength**

### Length of time for treatment

**Unless there are unexpected complications or reasons why GH is no longer appropriate, GH treatment should continue until your child has stopped growing. Some adults with PWS are being offered GH treatment, although at the moment there is less research evidence for this and no NHS guidance for treatment.**

## Further reading

“Growth Hormone Research Society Workshop Summary: Consensus Guidelines for Recombinant Human Growth Hormone Therapy in Prader-Willi Syndrome” – J Clin Endocrin Metab 2013.

<http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3789886/>

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