
PRADER-WILLI SYNDROME & GROWTH HORMONE USE

It can be overwhelming if your child has just been diagnosed with Prader-Willi syndrome (PWS). One of the things that you may have heard about is growth hormone (GH) treatment for children with PWS. This hand-out will give you information about the treatment, and it will describe the process of getting your child started safely on it.

What are the benefits of growth hormone in PWS?

In the short term, GH has a positive effect on motor development in infants and children with PWS. It is thought that GH may improve brain development by increasing a hormone called IGF-1.³ In the long term, GH has been shown to help growth (height), distribution of muscle and fat, physical strength, lung function and day-to-day functioning in people with PWS.

What are the risks of growth hormone with PWS?

GH may increase the pressure of the liquid surrounding the brain (benign intracranial hypertension). There is also a risk of higher blood sugar (diabetes). GH may also cause problems with the hip joint (slipped capital femoral epiphysis). While studies have not shown this, there is a theoretical risk that for children with curvature of the spine (scoliosis), GH may worsen it.

Sudden death has been described in children with PWS who start GH. The exact cause of the sudden deaths has not been determined.¹ Deaths have often occurred in children who have a history of breathing obstruction, lung infection or severe obesity. Deaths have generally occurred soon after starting GH therapy.

What are your BC Children's Hospital (BCCH) medical team's goals for you and your child?

Our goals of treatment include: (1) safe prescribing and monitoring of therapy, (2) following the medical evidence, (3) starting therapy in as timely a manner as possible, and (4) striving for equitable access. Should you be interested in starting GH for your child, we want to support you in doing so.

We've decided to start... what's next?

There are a few things that need to be done when starting GH:

- 1) In BC, kids with PWS who want to start on GH will be checked for growth hormone deficiency (GHD). This may involve special tests. See *Growth Hormone Stimulation Test*: <http://www.bcchildrens.ca/endocrinology-diabetes-site/documents/ghstimtest.pdf>.
- 2) If your child has GHD, the GH will be eligible for coverage under BC Fair PharmaCare. Please ensure you are registered with Fair Pharmacare:

<https://www2.gov.bc.ca/gov/content/health/health-drug-coverage/pharmacare-for-bc-residents/who-we-cover/fair-pharmacare-plan/register-for-fair-pharmacare>.

- 3) If your child does not have GHD, you may be covered under your extended health. If it is not covered under extended health, your doctor and nurse will help you look at other options. The cost of GH is based on price-per-milligram of GH, and it will increase as the dose increases. An average price range would be approximately \$3,000 to \$22,000 a year.
- 4) All children who would potentially benefit from GH are presented before the BCCH Growth Hormone Advisory Committee to seek approval for use. Once your child is approved, your Endocrinologist will contact you to let you know the result. Then, one of the Endocrine Nurses will contact you to help with the practical steps in starting GH. See *BCCH Growth Hormone Therapy* for the process of starting GH:
<http://www.bcchildrens.ca/endocrinology-diabetes-site/documents/ghtherapy.pdf>.
- 5) Because of the risk of sudden death, clinical guidelines recommend a sleep study before starting GH in people with PWS. This may also be need to be repeated after starting GH. If the sleep study shows sleep apnea (pauses in breathing), this should be treated before starting GH. Timing of the sleep study can vary depending on wait times. Every effort will be made to get your child in as quickly as possible.

How long will my child stay on GH treatment?

Generally, your child will be treated on GH until they reach adult height. If there are issues with GH treatment, it can be stopped sooner. You will have a conversation with the doctor once your child reaches his or her adult height to see whether your child continues or stops.

References

¹Krystal A. Irizarry, MD et al. Prader Willi syndrome: genetics, metabolomics, hormonal function, and new approaches to therapy. *Adv Pediatr* 2016;63(1):47-77.

²Renske J. Kuppens, et al. Beneficial effects of GH in young adults with Prader-Willi syndrome: a 2-year crossover trial. *J Clin Endocrinol Metab* 2016;101(11):4110-4116.

³Stephany H. Donze, et al. Improved mental and motor development during 3 years of GH treatment in very young children with Prader-Willi syndrome. *J Clin Endocrinol Metab* 2018;103(10):3714-3719.

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