

Growth Hormone Therapy

Probably the single-most important therapeutic intervention thus far for the treatment of Prader-Willi syndrome is Human Growth Hormone Therapy.

The release of growth hormone into the body does not work efficiently in almost all persons with PWS. In June of 2000, the U.S. Food and Drug Administration (FDA) approved an application from Pharmacia Corporation (now Pfizer) to market their recombinant human growth hormone product Genotropin, for the treatment of growth failure due to Prader-Willi syndrome. PWS is now one of the approved indications for growth hormone medication, with multiple pharmaceutical companies producing growth hormone, and children with PWS can now be considered for growth hormone treatment based solely on their diagnosis and growth pattern. A sleep study to rule out respiratory problems is generally required before treatment can begin.

There is no longer any question that growth hormone treatment can improve the health and quality of life of persons with PWS. Growth Hormone Therapy is now recognized as the standard of care and provides many potential and significant benefits to children with PWS including increased height and growth rate; increase of hand and foot sizes to normal proportions; decrease in body fat; decrease in body mass index; increase in muscle development; improved respiratory function; improved physical performance; increase in resting energy expenditure; improvement in cholesterol levels; increase in bone mineral density, and improved cognitive functioning and IQ. Although the FDA has approved growth hormone treatment specifically for children with PWS, endocrinologists will prescribe growth hormone for infants and adults who have PWS.

The most common side effects of growth hormone are minor: occasional bruising or slight bleeding at the injection site. Rarely do other side effects occur including headaches, pseudotumor cerebri, swelling in the feet and legs, increased levels of insulin, decreased levels of thyroid hormone, elongation of lower face, and progression of a pre-existing scoliosis.

Most insurance companies will cover growth hormone therapy for PWS, although it can require an appeal after an initial denial of the claim. Growth hormone medication is prescribed and monitored by an endocrinologist who is knowledgeable about PWS or is willing to consult with an endocrinologist who does specialize in PWS.

After receiving a prescription for growth hormone, a nurse will teach you how to administer the medication to your child, which consists of a subcutaneous (under the skin) shot using a short, thin needle, similar to that used by individuals who have diabetes. You will administer the growth hormone medicine every day, generally in the evening. Most endocrinologists recommend giving the shot at night time because growth hormone is naturally released in the body while we sleep, so administering growth hormone medication at bedtime simulates what the body would have naturally done on its own. Some families incorporate the shot into the nightly bedtime routine, while others wait until their child has fallen asleep before giving the shot. It is most advisable to treat the growth hormone shots matter-of-factly; the more calm and matter-of-fact about it parents are, the calmer the child is likely to be.

You will visit your endocrinologist every three to four months for follow up visits so that the physician can adjust the dosage according to your child's growth pattern. Some physicians monitor IGF-1 levels and dose according to these. After your child has reached his or her genetically determined linear height and the growth plates have closed, low dose growth hormone "maintenance" therapy will continue in order to

provide the ancillary benefits of this medication such as improved muscle tone, increased bone mineral density, increased energy, etc.

Resources

Growth Hormone Therapy Consultation Assistance: PWSA | USA (800) 926-4797

Pfizer Growth Hormone Information Website www.genotropin.com

Insurance Reimbursement Assistance: Pfizer Bridge Program (800) 645-1280
www.Pfizerbridgeprogram.com

SNAP – Special Needs Advocate for Parents. National information source providing assistance to parents in sorting out private medical insurance authorization or reimbursement problems.
www.snapinfo.org or (888) 310-9889

Assistance with Prescription Drug Costs and Co-pays: www.needymeds.org; www.rxassist.org;
www.phrma.org; www.Diplomat.is; www.Healthwellfoundation.org