



GROWTH HORMONE

FDA Approval On Growth Hormone Therapy

The following is an excerpt from a letter written by Janalee Heinemann, MSW Executive Director, PWSA (USA). FDA approved growth hormone for PWS in 2000.

For years there has been no medication specifically approved for individuals with Prader-Willi syndrome (PWS). Now, finally, we have good news. We are pleased to inform you that the U.S. Food & Drug Administration (FDA) has recently determined that PWS is an "indication" (eligible condition) for treatment with Genotropin TM (somatropin rDNA for injection), which is a form of growth hormone manufactured by Pharmacia Corporation (now Pfizer). Previously approved to treat "growth hormone deficiency" in children and adults, Genotropin is now a treatment approved specifically for "growth failure in children with PWS."

This does not mean that there is a problem if a child is on another brand of growth hormone. In general, growth hormone therapy has been approved for some time--but now, Genotropin specifically has been approved for treating PWS. Genotropin's approval for PWS was issued by the FDA under THE Orphan Drug A . (This designation is only given to treatments for which the potential patient population is under 200,000. *Orphan Drug status* entitles Pharmacia [*Ed.Note: now Pfizer*] exclusivity in marketing the drug for this purpose for the next seven years.) FDA approval should make it easier for families to appeal to insurance companies for coverage and should help with Medicaid coverage. Also, under the FDA ruling, growth hormone deficiency testing (muscle biopsy) will no longer be required for children with PWS and growth failure who are being considered for GH treatment.

Results from the studies submitted to the FDA reveal that growth hormone treatment improves growth and body composition in children with PWS, including stimulating skeletal growth, decreasing the amount of body fat and increasing lean body mass (muscle). Given the many issues faced by families affected by PWS, we believe the increased availability of growth hormone will be of benefit to many members of our community by helping to reduce some of the major medical problems often inherent in this syndrome. Please note that you should consult with your physician as to whether growth hormone therapy is appropriate in your particular case, since it may not be beneficial for every child with PWS.

Further information may be found in the publication Growth Hormone and Prader-Willi Syndrome A Reference for Families and Care Providers by Linda Keder. This excellent 52-page booklet [revised in 2011] is described by PWSA(USA) Scientific Advisory Board member and endocrinologist Dr. Phillip Lee as "by far the best monograph on growth hormone for any condition I have ever seen."

In addition, more information may be found on the PWSA USA website www.pwsausa.org or by calling National Headquarters 1-800-926-4797.