EFFECTS OF GROWTH HORMONE TREATMENT IN CHILDREN WITH PRADER-WILLI SYNDROME

Recent reports from a number of research groups around the world have now confirmed what was suspected a decade earlier: Growth hormone treatment offers many potential benefits to children with PWS. In addition, there seems to be no significant increased risk for side effects of GH treatment in people with PWS. Studies are just beginning on the use of GH in infants and adults with PWS, but researchers expect to find benefits for those age groups as well.

Following is a summary of what has been learned about the benefits and side effects of GH treatment in children with PWS. Results from the major recent studies are outlined in more detail in Appendix E.

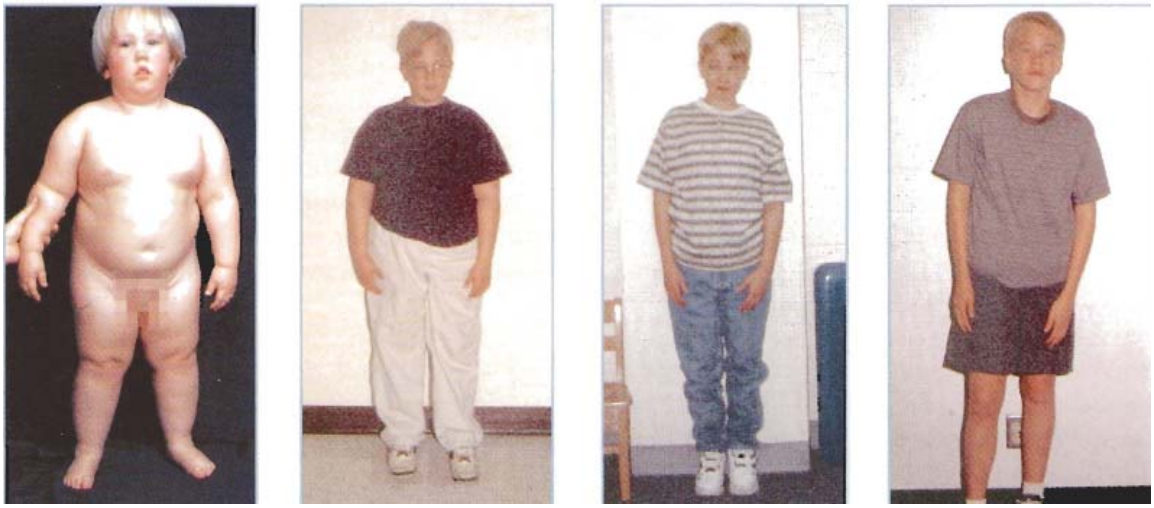
BENEFITS OF GH TREATMENT

Measured Improvements

The following physical changes were documented in various research studies, and the most dramatic results were reported in the first year of GH treatment. Studies in Sweden and the United States Followed children beyond one year of treatment and reported some additional improvements.

- **Increased height and growth rate** — Treated children grow in height at double or more their rate before treatment. For example, some study participants grew five or more inches during the first year of treatment, compared with two inches a year or less prior to GH treatment, A child treated with GH measures higher on the normal growth curves than before treatment and continues to grow along that higher curve as long as GH therapy is continued. Depending on the starting age of treatment, a child's final height can be closer to that of others in the family.
- **Increase of hand and foot sizes to normal proportions** — GH treatment enables hand and foot sizes to catch up with height growth in just one year. Without GH treatment, people with PWS typically have small hands and feet for their body size, which can affect motor skills.
- **Decrease in body fat** — The excess fat that is characteristic of PWS was reduced in all the studies where diet was controlled. One study reported a 25 percent decrease in fat mass, while others reported a slightly lower decrease. Despite the improvements with GH treatment, fat levels seem to remain somewhat higher than normal in the children with PWS. It is not yet known whether GH treatment from infancy can reduce fat levels further.
- **Decrease in body mass index (BMI)** — BMI, which is a measurement of obesity based on weight and height, declines with GH treatment and increases when treatment is stopped.
- **Increase in muscle development**— Improvements have been shown in measured size of muscles and in muscle as a present of body weight. As with the changes in fat, muscle growth does not quite reach normal levels, although it is significantly improved. Young, underweight children in one study gained weight because of the increased muscle.
- **Improved respiratory function** — GH-treated children can breathe better, due to stronger respiratory muscles and improved response to build-up of carbon dioxide (CO_2).
- **Improved physical performance** — Two studies documented improvements in physical performance with GH treatment due to increased muscle strength and respiratory function. In one study, children were able to run faster, jump farther, lift more weight and do more sit-ups than those who were not treated with GH.

- **Increase in resting energy expenditure (REE)** — One study recorded an improvement in REE after two years of treatment. REE is the level of calories a body burns while at rest, which is most of the day's calorie usage, or the individual's basic rate of metabolism. REE is raised by adding muscle and increasing physical activity.
- **Improvement in cholesterol levels** — One study reported that total cholesterol decreased in treated children, while their HDL. (high-density lipoprotein, or so-called "good cholesterol") levels rose.
- **Increase in bone mineral density (BMD)** — One study found that BMD increased at a faster rate in children who were treated with GH for one year than in those who were not treated. Continued increases after two years of treatment suggest that GH therapy may help avoid osteoporosis (thinning of the bones), which is a concern for adults with PWS.



This young man clearly illustrates the consequences of the growth disorder in Prader-Willi syndrome (two photos on left) and the correction that is possible with GH treatment (two photos on right). As shown on his growth chart on Page 15, this boy was able to achieve the 50th percentile for both height and weight after just two years of GH therapy.

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This article is Chapter 3 of the booklet, *Growth Hormone and Prader-Willi Syndrome: A Reference For Families And Care Providers*, by Linda S. Keder. This booklet can be purchased from PWSA (USA) by calling (800) 926-4797 or email info@pwsausa.org