

Prader-Willi Syndrome Association Pioneers use of Growth Hormone Treatment in PWS

Research + Advocacy + Funding + Education = Progress

The **Prader-Willi Syndrome Association (USA)** is the leading international organization for providing support to the medical and research communities and information and counseling to families. One of the greatest accomplishments of PWSA (USA) over its 25 history is the availability of growth hormone (GH) for people with PWS. For purposes of this abstract, it is assumed that the use of GH in people with PWS has a dramatic positive impact on their lives.

PWSA (USA) used its strategic theory that putting together over time the **People, Tools** and **Funding** for research will result in progress. As tracked below in a brief outline PWSA (USA) began a process in 1988 that culminated with major success in the U.S and internationally for the use of growth hormone treatment in PWS. At each step PWSA (USA) was a key participant in the process.

1988- \$2,500 seed grant to University of Wisconsin for an “early study” of the efficacy of GH in PWS.

Early 1990’s- Families begin use of GH, on a case by case –need established basis, with contested or limited insurance coverage.

Mid 1990’s With the interest piqued, drug companies sponsor major studies.

1998- Presentation at PWSA (USA) Scientific Conference of study in Sweden of impressive positive results.

June 2000- FDA approval for use of GH in PWS.

July 2000- International consensus statement signed by 21 prominent worldwide endocrinologists supporting the use of GH in PWS, without the requirement for individual testing.

Early 2000’s- Forced by FDA and consensus statement reimbursement as “a matter of course” begins from insurance companies. GH approved for use in adults with PWS.

2001- PWSA (USA) publishes a reference booklet, funded by Pharmacia, for families and care providers.

2004- In response to a presentation by the Executive Director of PWSA (USA) and the President of the International Prader-Willi Syndrome Organization, the Taiwan government approves the fully funded use of GH in PWS.

PWSA (USA) combined early seed money for research; with constant advocacy; with forums for scientific discussions and presentations; with parental education to move the state of the science on GH from concept to practical application and availability.