pwsa
prader-willi syndrome australia

POLICY ON GROWTH HORMONE TREATMENT IN THE PRADER-WILLI SYNDROME POPULATION

POSITION SUMMARY

The use of recombinant Growth Hormone (GH) under medical supervision is recommended for the treatment of Prader-Willi syndrome (PWS) in individuals of any age or height.

BACKGROUND

Prader-Willi syndrome is a severely disabling, complex genetic condition with serious physical and social implications. The high mortality is directly related to the morbid obesity with the disordered body composition and metabolism underlying PWS. A large number of scientific studies have investigated the effect of GH therapy in PWS. The Association and medical community have long recognised that patients with PWS respond well to GH in terms of increased linear growth, growth velocity and reversal of the disordered body composition.

The Therapeutic Goods Administration (TGA) registered recombinant growth hormone "Genotropin" as an orphan status drug in Australia dd 11/08/03 "for improvement of body composition and treatment of short stature in patients with Prader-Willi syndrome" (http://www.tga.gov.au/docs/html/orphand2.htm).

Research on the use of growth hormone therapy in PWS has been carried out worldwide over the last decade with overwhelming evidence for multiple benefits. In the USA, western Europe, Scandinavia, and the UK, GH therapy is used commonly to treat PWS in recognition of the benefits in weight management and physical performance independent of height improvements in this population.

In May 2004, the Australasian Paediatric Endocrine Group (APEG) stated its position to the Pharmaceutical Benefit Advisory Committee (PBAC) in support of GH therapy in patients with PWS with its submission of "PRADER-WILLI SYNDROME – THE CASE FOR GROWTH HORMONE THERAPY IN AFFECTED CHILDREN AND ADOLESCENTS".

RATIONALE

Existing scientific literature reports that benefits of GH therapy in PWS include:

1. Tendency towards normalisation of lean body mass to fat ratio, with an associated decrease in the Body Mass Index, i.e. definite improvements in the following
   - ♦ Increase in muscle development (tone, size, strength)
   - ♦ Increase in bone mineral density and therefore a decrease in the incidence of osteoporosis and fractures
   - ♦ Decrease in body fat
2. Tendency towards normalisation of growth parameters such as final height in relation to genetic potential:
   - ♦ Tendency to increase hand and foot size in relation to body size to normal proportions
   - ♦ Some increase in small jaw size, potentially, lessening dental intervention
3. Improved physical performance and agility
4. Improved respiratory function
5. Increase in resting energy expenditure due to an increased fat free mass
6. Improvement in cholesterol levels
7. Improved insulin action

Whilst:

a) People with PWS may or may not have classical growth hormone deficiency, the level is not a determinant of the effectiveness of the GH therapy in Prader-Willi syndrome

b) Artificial stimulation tests do not give an accurate result of the functional growth hormone deficiency in people with PWS

c) There are risks associated with the use of GH, these are minimal if patients adhere to recommendations of a sleep and respiratory study and test negative for potential underlying infections prior to the onset of GH therapy with regular bi-annual or quarterly medical check-ups as requested by the pediatrician or endocrinologist.

The consequent behavioural advantages of using GH include:

- Better weight management, that is, less restriction on food intake and also less obesity and associated health problems, reducing hospitalisation and medical intervention • Better gross motor abilities, fewer accidents and greater ability to exercise
- Better fine motor skills, including speech articulation
- Greater alertness and stamina
- Better social integration

It is evident that the behavioural advantages will mean fewer costs to the community in managing the usual health, educational and social problems associated with PWS.

CONCLUSION

One of the objectives of the Australian Government's National Medicines Policy 2000 is "*timely access to the medicines that Australians need, at a cost individuals and the community can afford"*, and that policy states, in part, "*that cost should not constitute a substantial barrier to people's access to medicines they need"*.

Presently GH therapy is only available on the Pharmaceutical Benefit Scheme (PBS) for children below a critical height or proven classical GH deficiency.

PWSAA and their Professional Advisory Board is firmly of the view that for individuals where:

- the treating physician has made a recommendation and
- a decision is taken by the parents or legal guardian,

GH should be available to all people with PWS as a pharmaceutical benefit, pre and post puberty, and should be available to them for the reasons noted above, with height considerations not the only indicator of need. This is consistent with the AGNMP 2000 guidelines.

Prepared by the Professional Advisory Board in association with the Prader-Willi Syndrome Association of Australia, Inc.

As at 22 July 2005

Chairman of the Professional Advisory Board
President of the Prader-Willi Syndrome Association of Australia Inc.