SUPPORTING PEOPLE WITH PRADER-WILLI SYNDROME AND THEIR FAMILIES

Prader-Willi Syndrome Association (NZ) Incorporated

September 27th 2016

Proposed Amendment to the Special Authority Criteria SA1451

Somatropin Treatment for Prader-Willi Syndrome – New Information and Further Comment

We seek an amendment to SA 1451 as set out in our detailed submission forwarded to Pharmac in December 2015. We note that this submission was not included in the information considered by the Endocrinology subcommittee when they met in June 2016, due to an error in the submissions process at Pharmac.

Growth hormone treatment for people with Prader-willi Syndrome has long term benefits for the individual and their families and these benefits do not cease when skeletal maturity is reached. We attach two very strong well designed clinical studies that have been published since our December submission that support this.

- Kuppens et al 2016 "Beneficial effects of growth hormone in young adults with Prader-Willi syndrome: a 2-year cross-over trial."
- Dykens et al 2016 "Cognitive and adaptive advantages of growth hormone treatment in children with Prader-Willi syndrome"

Prader-willi syndrome is a rare disorder characterised by a unique constellation of clinical issues - abnormal body composition in the presence of hypothalamic dysfunction causing excessive appetite, accompanied by severe behavioural difficulties and intellectual disability. While the improvement of body composition is clearly the primary benefit, it is also in the context of the other clinical issues that we seek funded treatment.

A Parliamentary Select Committee considered the PWSANZ petition 2004/161 and recommended that a separate category be created for Prader-willi syndrome related to changes in body composition rather than height.

We would also like to highlight again the Medsafe indication for Omnitrope:

> **"Growth disturbance due to insufficient secretion of growth hormone and growth disturbance associated with Turner syndrome or chronic renal insufficiency. Prader-Willi syndrome, for improvement of growth and body composition."**

We would like to respond to statements in the minutes of the Endocrinology Subcommittee.

- "The Subcommittee noted that parents' main reason for wanting to start growth hormone early appeared to be the possibility that it might improve their child's strength and body mass index. The Subcommittee considered that the benefits of this would be to improve motor milestones, improve the ability to play with other children, and to improve long-term lean body mass (although the latter benefit is unproven)."

  Strength, BMI and body composition benefits are shown in many clinical studies . Refer PWSANZ December 2015 Submission References: 4, 5,6,7,10,11,12,13,14

- "The Subcommittee considered that the evidence for initiating treatment with growth hormone in the first 18-24 months following birth was weak."

  There is good evidence for the benefits for early GH treatment for PWS. Refer PWSANZ December 2015 Submission References: 4, 6, 14,15,16,17,18,19,21

- "The Subcommittee noted that the published literature in adults with PWS does not support a reduction in body mass index (BMI) from starting growth hormone, and there is no evidence that stopping growth hormone in adults with PWS increases BMI. The Subcommittee noted that there was one small published study that suggested that stopping growth hormone increased body mass but not BMI (Butler et al. Growth Horm IGF Res 2013;23:81-7)."

  There is not "no evidence" for reduction in BMI. Refer PWSANZ December 2015 Submission References: 4, 23, 43

  The Butler study mentioned (reference 43), looked at 11 adults and supports the continuation of treatment after diminished benefits seen after cessation of treatment. The study measures lots of things but it does not measure BMI.

  Otto et al, 2014 (reference 23) shows significant increase in BMI when treatment ends

  Bakker, 2013 (reference 4) shows improvement in BMI with GHT

- "The Subcommittee considered that non-adherence to treatment was an ongoing issue for at least 30% of patients with PWS in New Zealand."

We were very surprised by this statement as it does not correlate to our experience of working with families and we had not come across any data that supports this statistic. We asked Pharmac to release the data the figure came from as part of an OIA request.

Pharmac advised:

"The reference to this statistic in the June 2016 minute was a record of the Subcommittee's opinion based on its members' professional experience; it is not information PHARMAC holds or that we provided to the members for consideration. There is no other information to provide for this part of your request."

We work closely with families and we know how much they value GH treatment. It is the opinion of PWSANZ that our member's adherence to GH treatment for their children would be close to 100%.

We requested figures from Pharmac in early September 2016 regarding growth hormone treatment in adolescents and adults. These are attached below.

Patients dispensed treatment for 2016 FYR

Defined as patients who received at at least one dispensing in the period 01-07-2015 to 30-06-2016

Row Labels	Count of ID
Adolescents	**122**
adults and adolescents	<10
growth hormone deficiency in children	50
Prader-Willi syndrome	<10
short stature due to chronic renal insufficiency	<10
short stature without growth hormone deficiency	32
Turner syndrome	26
Adult	**235**
adults and adolescents	223
growth hormone deficiency in children	<10
short stature without growth hormone deficiency	<10
Turner syndrome	<10
Grand Total	**357**

Note: Adolescent ages 12-17, Adult 18+

While this data does not include information about bone age, we include it to point out that there are 122 adolescents and 235 patients over 18 who are currently receiving funded growth hormone treatment.

We note that dietary and behavioural management take a huge toll on families impacted by Prader-willi Syndrome, and that the teenage years are particularly difficult.

We ask that as with the Endocrinology subcommittee, that PTAC consider the case for removing the requirement for slow growth and lowering the commencement age to 6 months separately from the case to extend treatment to at least 18 years of age.

We note that these two changes would create an estimated total increase in the growth hormone budget of approximately $51,000 per annum. Given the total spend on GH for 2013/14 year was $3,724,160 we note the estimated cost of implementing our proposed changes will be a global increase of less than 1.5%. This increase is completely offset by the reduction in price of GH with the move to Omnitrope.

Sarah McLarin

CEO

Prader-willi Syndrome Association New Zealand Inc.