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Former President of PWSAA

TO WHOM IT MAY CONCERN

It is extremely difficult for a small group of patients with a rare condition (EG: Prader-Willi Syndrome with 350) to have Drug Companies make a submission to the PBAC, Mainly because of what Profit the company would receive by having a drug listed on the PBS. With the proposal for Cost Recovery for submissions, these small groups would never have access to life saving drugs under the PBS, This is a major concern to all smaller groups. Many Companies with TGA approval (Indication) for drugs for smaller groups are not willing to make a submission due to the cost for a Submission and by having this Indication locks any other not so prominent drug company out who are willing to make a submission for these small group of patients.

Below you will find how difficult it is for small group of patients to force a drug company with an indication for a drug that would benefit children with a disability

I first started lobbying Government back in 1999 in May of 2000, Pharmacia Upjohn now Pfizer was granted the indication by the TGA for Growth Hormone for Prader-Willi Syndrome on Body Composition and Short Stature. In the mean time Growth Hormone treatment was approved in the United States by the FDA in August 2000 and England October 2001,and throughout Europe. That was as far as it went, until in December of 2002 I personally made a minor submission as a parent to the PBAC, with all the data required, trials and papers on treatment with Growth Hormone etc. (Which was accepted by the PBAC)
I received a letter back from the PBAC, It stated "Although the committee was sympathetic to this request, the submission was rejected

because the committee is required to examine the cost-effectiveness of proposed drugs for the requested purposes and this information was not provided in the submission."

In January of 2003 the PBAC requested APEG to submit a document on Prader-Willi Syndrome, It was completed in May of 2004. This letter was written by Dr. George Werther (Who is a prominent doctor in Australia on Prader-Willi Syndrome) and with the help of Dr. Martin Rizten from Sweden and Dr Phillip Lee from the USA, Both leading experts in the field of Prader-Willi Syndrome. The conclusion was as follows.

<u>Conclusions and APEG recommendations.</u>

- **There is now overwhelming evidence for multiple advantages of Growth Hormone therapy in children and adolescents with Prader-Willi Syndrome. In addition to it's effects on linear growth, GH has well-proven positive effects on body composition in children with PWS, including reduced fat mass, increased lean mass, reversal of lipid disturbances, and enhanced bone density. Furthermore, increased muscle strength and agility are now also well-established effects.**
- **The multiple serious morbidities, both physical and social, and very high mortality of Prader-Willi Syndrome are directly related to the morbid obesity and disordered body composition and fat and carbohydrate metabolism underlying PWS.**
- **Since GH therapy, in conjunction with ongoing supportive therapies, is clearly able to reverse many of these deleterious changes, which are all known to predispose to serious morbidities, the argument for its availability for young people with PWS is compelling. GH treatment in childhood is likely to contribute to prevention of these morbidities in adult life.**
- **The Australian Paediatric Endocrine Group recommends that Prader-Willi Syndrome be funded as a specific indication for GH therapy. A proven diagnosis of PWS should be the primary criterion for initiation of therapy, but detailed recommendations regarding dosage regimens monitoring, and response criteria remain to be determined.**

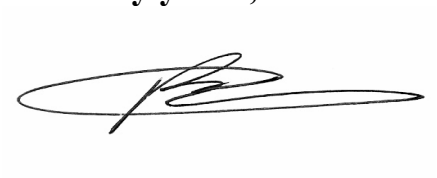
This was accepted by the PBAC in July of 2004. In the September of 2004 the PBAC invited Pfizer to make a submission to them.
It has taken over two years for Pfizer to make this submission, with the PBAC telling Pfizer that they would reject the submission unless they have data on cost-effectiveness. The Submission was made to the PBAC for body composition and with the recommendations from APEG.

Pfizer made a submission to the PBAC in November 2006, which was rejected, and with the request from the health minister at the time for Pfizer to work with the PBAC to finalise a new submission which would be accepted this was heard in March this year and accepted for listing under the PBS.

I believe that if it wasn't for the health minister request for a meeting with the PBAC we, as a small group of patient would not have this approval

I also believe that companies with indication for drugs for small group of patients should force to make a submission to the PBAC within two years of them receiving this from the TGA

Sincerely yours,



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