

Question 1: Do you support criterion one?

Yes.

Increasing the threshold to 5/10000 would bring Australia in line with the EMA this would mean that diseases such as Narcolepsy (with and without cataplexy) would meet the new criteria. Currently there is an effective treatment (Xyrem) available but only via the TGA Special Access Scheme and at a very high financial cost of \$1200 - \$1800 per calendar month. This treatment is considered the first line treatment in the USA and Europe. A change in the orphan drug program would mean that the pharmaceutical company that distributes the drug within Australia could apply for designation and potentially register the drug as a prescription medication within Australia.

Question 2: Do you support criterion two?

Yes.

Current treatments in Australia for Narcolepsy treat the symptoms only, however Xyrem (only available through the SAS) treats the cause which in turn has a greater success rate with sufferers and improves their quality of life and potential productivity.

Question 3: Do you support criteria three and four?

Yes

Question 4: Do you support the proposed consideration of paediatric indications?

Yes

Question 5: Do you support the proposed changes to the designation process and the timing of automatic lapsing?

No

I would think that it would be more suitable for the designation to lapse after 12 months if no registration application is received. 3-6 months seems to be to a very short length of time to submit a registration application.

I do agree with retaining the 100% waiver of fees

Question 6: Are there any other key issues that should be considered in developing the changes to the orphan drug program?

Narcolepsy in Australia has an incidence of 3 in 10000 people within Australia, this neurological disorder dramatically affects all aspects of the sufferer's lives. If people diagnosed with Narcolepsy could access this life altering medication more easily in Australia it will make living with this disorder a little easier and give them better outcomes. The proposed changes to the Orphan Drug Program could make this happen.

My son was diagnosed with Narcolepsy in September 2011 and with a huge amount of financial support from my family we are able to give him access to Xyrem, however if something doesn't change we will not be able to continue to do so.

The drug Xyrem has dramatically improved his life he has been able to return to University, get a casual job, participate in sport and pass a test in order to obtain his learners permit. Prior to commencing Xyrem he was unable to stay awake throughout the day and was sleeping approx. 18 hours per day (despite taking the prescribed medication) and was unable to function during the other 6 hours a day. He also put on approx. 20 kg due to the amphetamines he was taking to treat his narcolepsy, but the change to Xyrem has seen him lose 10kg so far. He has gone from barely functioning to becoming an active member of society