**Question 1: Do you support criterion one?**

Yes.

By increasing the rare disease threshold to 5/10000 it would bring Narcolepsy into the new threshold. My daughter has been diagnosed with Narcolepsy (with Cataplexy) when she was eight years old, we have tried numerous drugs to help her manage this condition. We are currently accessing Xyrem through the TGA Special Access Scheme (SAS) and The Canberra Hospital (TCH)has offered it to us for trial, due to its prohibitive cost ($500+ for 180mls). Xyrem is a firstline treatment for Narcolepsy in Europe and the USA, this should also be the case in Australia. By changing the threshold of the orphan drug program it would mean that the pharmaceutical company that distributes Xyrem in Australia would be able to apply to register the drug as a prescribed medicine. My understanding is that, currently the company has no plans to register Xyrem as there are not enough people who suffer from Narcolepsy here in Australia to make it financially viable for them to do so.

**Question 2: Do you support criterion two?**

Yes.

Existing treatment here in Australia for Narcolepsy generally treat the symptoms. Xyrem however assists in treating the cause, being able to sleep at night, being one of them. It has certainly improved our daughter's quality of life and has increased her concentration and productivity at school.

**Question 3: Do you support criterion three & 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 think it would more beneficial for all to have the designation lapse after 6-12 months is no registration as received.

I agree in 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?**

We are fortunate that we have a great medical team (Paediatrician and Sleep specialist) that look after my daughter, to deliver, the best of life outcomes for her in dealing with Narcolepsy. We have put submissions for access to Xyrem for some time and last December were granted access through the TGA SAS. Due to its cost Xyrem would essentially be unaffordable for us, the TCH has agreed to fund a trial for my daughter, where we pay the PBS amount, this has been a huge relief for us. Since the trial began we have seen a dramatic improvement in her confidence and alertness during the day, she's also been able lose weight. Whereas her previous treatment her in school activities were monitored and she would need constant sleep breaks throughout the year. With Xyrem these sleep break have virtually ceased and her cataplexy attacks have also dramatically reduced.

I feel that Xyrem has made a drastic change to our daughter life and I feel that these changes to the Orphan Drug Program will help make access to the drug easier and more affordable in the future.