Question 1: Do you support criterion one?

Yes.

Increasing the incidence threshold would bring Australia in line with the EMA and is more appropriate for our population. There is many people affected by rare diseases that do not meet the current criteria of less than 2000 total that would greatly benefit from this change. Many of these are currently disadvantaged due to the extremely inadequate current rate

Question 2: Do you support criterion two?

Yes.

Rare diseases affect many people making their quality of life very poor and leave them severely incapacitated, despite their diseases being rare they do not meet the current guidelines.

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 6 months if no registration application is received. I believe the 3-6 month timeframe may be unrealistic for some applicants.

I 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?

I currently pay out $3600 quarterly in order for my grandson to take medication for his rare disease. This drug is not registered as a prescription medication in Australia and the pharmaceutical company do not see it as financially viable to register due to the limited number of people affected by the disease.

We pay for the drug as it has truly changed his life and made him able to function as any 20 year old man should, without it he would be left incapacitated. A change in the incidence rate criteria for the Orphan Drug Program would be beneficial. If this drug was able to be registered by the pharmaceutical company we would be able to claim some of this excessive expense back through our private health insurance, and hopefully be able to get this drug listed on the PBS in the future.