



Narcolepsy is a neurological disorder that affects approximately 3 in 10,000 Australians. There are limited effective treatment options available in Australia. Currently the first line treatment from the USA and Europe which is a drug called Xyrem is not registered as a prescription medication in Australia and is only available through the TGA Special Access Scheme at a personal cost of between \$1200 and \$1800 per month. As there is such a small potential ongoing market for this drug in Australia understandably the pharmaceutical company is unwilling to apply to register Xyrem as a prescription medication at this time.

Narcolepsy Australia is a health promotion charity which has over 750 registered members nationally, each one of these members is affected by Narcolepsy. Narcolepsy (with and without cataplexy) dramatically affects the person suffering from it. They suffer from Excessive Daytime Sleepiness which affects their ability to work and drive, their cognitive function and often leaves them unable to carry out simple tasks, suffer from anxiety and become reclusive.

Question 1: Do you support criterion one?

Criterion one: Rare disease threshold or lack of financial viability and seriousness of the condition

EITHER

threshold of 5/10,000 (*less restrictive than status quo, more diseases may qualify as rare*) AND life threatening /chronically debilitating (*more restrictive than status quo*)

OR

life threatening /seriously debilitating or serious and chronic condition AND that without incentives it is unlikely that marketing would generate sufficient return to justify the necessary investment¹ (*more restrictive than status quo*)

Yes we agree with Criterion one, an increase of the rate of incidence threshold to bring Australia in line with the EMA. It would also mean that narcolepsy will meet the new criteria, even though it is not life-threatening it is a seriously debilitating and chronic condition. There is a very limited potential market so the return to the pharmaceutical company does not justify the current cost to register a medication. This would apply to any future treatment options that may become available in the future.

Xyrem is legal in Australia but only available via the TGA Special Access Scheme and at a very high financial cost to the patient. 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?

Criterion two: Alternative methods of diagnosis, prevention or treatment

There's no existing therapy (*more restrictive than status quo*)

OR

if there is existing therapy, the product represents a significant benefit over existing therapies¹
(*more restrictive than status quo*)

Yes.

The existing treatment options for Narcolepsy in Australia are limited and are used to treat the symptoms. The most common treatment is dexamphetamine which is used to try and counteract the Excessive Daytime Sleepiness (EDS). The cause of the EDS in narcoleptic patients is extremely reduced slow Wave Sleep (SWS) and increased Rapid Eye Movement (REM) sleep. Xyrem inhibits REM sleep thereby increasing SWS which in turn improves the sufferers EDS.

Xyrem improves the patient's quality of life and potential productivity.

Question 3: Do you support criteria three and four?

Criteria three and four: Medical plausibility

A justification for medical plausibility is required to support the orphan indication and to support subgrouping of indications.

Yes

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

Yes

Children are as equally affected by narcolepsy as adults.



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

Box 2: Proposed modifications to the EMA process reflecting adaptation to the Australian context

1. Changes to the designation process:

- the orphan designation will lapse within a set period if no registration application is made
- the designation can be withdrawn by the sponsor or cancelled by the TGA if any of the criteria are demonstrably no longer satisfied at any time

2. No changes to the TGA incentives:

- The current 100% waiver is proposed to be retained.

We agree that there should be a period of 6-12 months is a suitable time for a designation to lapse if no registration application is received. 3-6 months seems to be to a very short length of time to submit a registration application.

We also agree that the designation can be withdrawn by the sponsor at any time. If the criteria is no longer being satisfied we agree that the TGA can cancel the designation.

We also agree that the current 100% waiver of fees be retained.

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

Australia currently has a very low incidence threshold which means that there are many rare diseases that don't meet the current criteria but would meet the new proposed levels. Bringing Australia in line with the EMA threshold guidelines is very appropriate for our population and also would mean that better treatment options for patients affected by rare diseases.