

25 November 2016

Business Review and Reporting Section
Prescription Medicines Authorisation Branch
Therapeutic Goods Administration
PO Box 100
WODEN ACT 2606

RE: Consultation: Orphan drug program

The Royal Australian and New Zealand College of Ophthalmologists (RANZCO) thanks the Therapeutic Goods Administration (TGA) for the opportunity to comment on the orphan drug program, as part of the 2016 round of consultations.

RANZCO strongly supports maintaining and strengthening the orphan drug program, as a key mechanism to assure maintaining access to essential drugs for patients who suffer from uncommon conditions.

RANZCO notes that the discussion paper released by the TGA includes a number of positive proposals that should make for a less restrictive program, including amending the threshold from 2,000 Australians to 5/100,000 (approximately 12,000 Australians).

One aspect that is not currently covered in the consultation paper which RANZCO wishes to emphasise in particular for the TGA's consideration is regarding patients who are resistant to all current treatments. An example of this is posterior uveitis, where treatment-resistant patients may be argued to be a separate subgroup of uveitis, for whom current treatment options are of minimal benefit. It is imperative that the policies of the TGA do not place obstacles in the path of approval of novel drug entities that may benefit small subgroups of patients who do not respond adequately to current treatments.

Furthermore, "debilitating" is used as a criterion for the orphan drug program. It is important that severe chronic visual impairment is recognised as a debilitating condition.

If you require any further information about this matter, please contact RANZCO Policy Officer Guy Gillor on ggillor@ranzco.edu

Kind regards

A/Prof Andrew Symons
Chair, RANZCO Therapeutics Committee