rare voices
AUSTRALIA

MMDR Consultation
Provisional approval pathway for prescription medicines
Reform Coordination and Support Section
Regulatory Services & Improvement Branch
Therapeutic Goods Administration

Submission to the Consultation: Provisional Approval pathway for prescription medicines – proposed registration process and post-market requirements

Rare Voices Australia (RVA) is pleased to provide a submission to this Consultation. The aim of the Provisional Approval pathway is to ensure the expedited pathway for the registration of new medicines/ medical devices for life-threatening and serious unmet clinical need. It theoretically allows quicker access to new and innovative medicines and medical devices whilst still maintaining high levels of safety and efficacy while decreasing regulatory burden.

RVA knows how important an expedited pathway is for the almost 2 million Australians living with rare disease. Arguably the rare disease community has the greatest clinical unmet needs. There are only limited treatment options for rare disease (many rare diseases have no treatment options) and even where there are developed treatments, access to these treatments is uncertain and circuitous. Australians are not only being denied access to new therapies funded overseas, they are having to waiting 2-4 years longer than in comparable countries like the UK and Canada.[1] The ramifications for such delay are huge for those living with rare disease, as many life-threatening diseases progress over time.

RVA regularly liaises with industry and there has been an increasing number of positive reports about the TGA's flexibility and timely responsiveness in relation to crucial rare disease treatments. RVA strongly supports this further formalisation of this best practice. For rare disease treatments, the TGA is only one part of the reimbursement process however, and similar policy reform to reduce current delay experienced in PBS/LSDP also needs to be prioritised. RVA continues to call for the recommendations from the Life Saving Drugs Program to be released and for much-needed policy reform. An expedited TGA process may have only limited effect on rare disease patients without similar expedition of the reimbursement process. In acknowledgement of this, RVA calls for rare disease medicines that have TGA provisional approval to be eligible for an expedited reimbursement pathway.

RVA also calls for increased and earlier consumer/ patient involvement for those medicines considered for Provisional Approval. Application for Provisional Approval could easily include initial stakeholder mapping activity which would highlight the key patient group/s and key clinicians, to further inform the process. Those rare diseases without any formal or only limited patient group could potentially be appropriately supported by RVA, who would be happy to work in partnership with the TGA to promote increased patient voice, transparency and better patient outcomes in the rare disease space.

About Rare Voices Australia
Rare Voices Australia is a national, not-for profit peak organisation that works with all stakeholders to be the unified voice and advocate for all Australians living with rare disease.

Yours sincerely

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[1] (McKell Institute 2014) Funding Rare Disease Therapies in Australia