



8 January 2018

Committee Secretary
Senate Community Affairs Legislation Committee
Parliament House
Canberra ACT 2600

**Submission to the Senate Inquiry regarding the
Therapeutic Goods Amendment (2017 Measures No 1) Bill 2017**

Rare Voices Australia (RVA) is pleased to provide a submission to this Inquiry. This Bill is an important part of the Government's continuing response to the recommendations of the review of Medicines and Medical Devices Regulation (MMDR). RVA welcomes the opportunity to comment on Schedule 1 specifically, as it will impact on the Australian rare disease community.

Schedule 1 will improve access to potentially lifesaving medicines for patients with limited options. In this way, this scheme will more closely align to processes already in use in the USA and EU. It recognises that the traditional risk-benefit balance fails to respond to this group of patients.

RVA knows how important this 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 wait 2-4 years longer than in comparable countries like the UK and Canadaⁱ. The ramifications for such delay are huge for those living with rare disease, as many life-threatening diseases progress over time. It should provide consumers with earlier access to potentially life-saving new medicines and devices. It will also support the important role of industry by accelerating their pathway to market.

RVA supports Schedule 1 of the Bill.

For rare disease medicines, 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.

With Compliments

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RVA also call for the increased participation of and consultation with the rare disease patient community in all parts of the HTA process when dealing with rare disease treatments. There needs to be greater transparency around the patients' need for treatment and the benefits and limitations of specific medicines. The patient voice needs to be an integral part of dialogue between pharmaceutical companies and HTA bodies – at all stages of the process.

About Rare Voices Australia: Rare Voices Australia (RVA) is Australia's non-profit, national peak organisation advocating for all who live with rare disease. RVA provides a strong common voice to advocate for health policy and a healthcare system that works for those with rare diseases. RVA works with patients, key peak bodies, governments, researchers, clinicians and industry to promote rare disease research, diagnosis, treatment and services. The health care system is geared towards diseases with larger patient numbers. Australia needs a health system that is also 'Fair for Rare'.

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ⁱ McKell Institute 2014 'Funding Rare Disease Therapies in Australia'