25 November 2016

Att: Mr Adrian Bootes
Orphan Drugs Consultation
Regulatory Operation Unit
Market Authorisation Division
Therapeutic Goods Administration
PO Box 100
WODEN ACT 2606

Via email: adrian.bootes@tga.gov.au

Dear Mr Bootes

Re: Consultation Paper: Orphan Drug Medicines (2016)

Medicines Australia thanks the Therapeutic Goods Administration (TGA) for the opportunity to respond on behalf of the innovative, research-based pharmaceutical industry in Australia, to the recent consultation paper Consultation: Orphan Drug Program (October 2016). We welcome the ongoing commitment of the TGA to recognise the special place of orphan medicines in our health care system and we support reforms that will enhance access to medicines for patients with rare disease. However, such reforms should also reflect the costs of research and development in this area and help incentivise even more of it where possible.

Overall, we consider that the proposals presented in the paper are moving in the right direction, in particular, the proposed increase to the prevalence threshold (5 per 10,000) and the continuation of paediatric indications as a recognised rare disease area. However, some of the proposals potentially work to undermine innovation and the earliest possible access to rare disease medicines for patients. We appreciate the opportunity to have raised these issues informally with the TGA already, and look forward to its advice regarding likely implementation timeframes, the possibility of further consultations, and generally being kept informed of the timing of relevant amendments to Regulations.

In brief, the key issues for our sector are as follows:

- The key objective for reforms to the Orphan Medicines Programme should not centre first on the TGA's capacity to handle applications for Orphan Medicinal Products (OMPs) as suggested by a separate communication from the TGA, but rather, on how the right frameworks for considering OMPs will help enable earliest possible access to medicines that foster the health outcomes of Australian healthcare consumers whilst also recognising the investment being made in these 'difficult to reach' areas.
- The notion that designations should lapse after a certain timeframe is supported in-principle but the current proposal that designations automatically lapse after 3 months to 6 months is problematic. The timeframe is somewhat short and appears arbitrary. We suggest instead that the period before which designations lapse be 12 months at a minimum. That said, we are very open to working with the TGA on alternative approaches that balance the TGA's objectives in this area as well as the industry's. For example, by undertaking a forward-looking analysis based on real applications for orphan drugs designations in order to calibrate a more realistic time period that does not become unnecessarily burdensome for sponsors having to reapply for orphan designation. Having to reapply for orphan designation would detract from the aims of the Medicines and Medical Devices Review

(MMDR), as well as the objectives of the orphan drug program. Procedural fairness should also not be forgotten in the drive to increase efficiencies at the TGA's end.

- Fee waivers are an important feature of the Orphans Drugs Programme but they generally represent only a small fraction of the overall costs of undertaking research and development in rare disease areas, especially given that trials in this area are harder to run and patients more difficult to recruit. A better incentive for all companies, large and small, would be to offer a longer period for data exclusivity than is currently offered to orphans in Australia. This would foster better overall innovation including for SMEs, and we hope to continue discussions across the whole-of-government to help foster this outcome.
- The "significant benefit over existing treatment" criterion requires further close consideration in our view, as it potentially ignores issues like tolerability. If the TGA were wishing to explore this path further, we would encourage it to establish arrangements that enable sponsors of new molecular/biological entities applying for orphan status to have the opportunity to present their case as to why the molecule will help improve patient outcome(s).

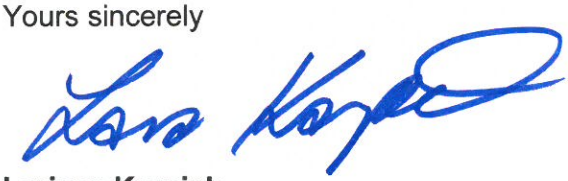
All reforms to the Orphan Drugs Programme should be consistent with the impending implementation of the MMDR recommendations that were accepted by the Australian Government, especially the Expedited Pathways recommendations. How and where the new pathways may interact with orphans is very important and industry would be interested in knowing more about their integration.

The release of the Orphan Drug Medicines 2016 paper comes at a busy time for the TGA and industry as we move to implement the Australian Government's response to the MMDR. We support earliest possible progress being made on these reforms provided they balance industry and regulatory needs. Where possible, we would ask that consultations be co-ordinated across the TGA to reduce the chances of 'consultation fatigue'.

We note our members may make their own submissions to the Orphan Drug Medicines paper, and we expect that these will reflect uniquely the position and nature of each company involved in this area.

Again, thank you for the opportunity to present our comments on this very important paper, and we look forward to working with you further.

Yours sincerely



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