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Fabry Australia's Submission to the Inquiry into the approval processes for new drugs and novel medical technologies in Australia, with a particular focus on those for the treatment of rare diseases and conditions where there is high and unmet clinical need.

Dear Committee Secretariat,

Fabry Australia, welcomes the opportunity to provide comment on the *Inquiry into the approval processes for new drugs and novel medical technologies in Australia, with a particular focus on those for the treatment of rare diseases and conditions where there is high and unmet clinical need.*

New drugs and novel medical technologies which focus on unmet clinical needs are urgently needed to adequately treat Fabry disease and provide much hope to the people diagnosed with this rare disease and their families. Fabry Clinic experts look to optimally manage or arrest the ongoing symptoms caused by the disease. As established by Fabry Australia's research findings, the lived experiences of Fabry patients is that symptom control is imperative.

As was highlighted in the Fabry Australia Submissions to the LSDP in 2020, 2014, 2010 and 2008, Fabry Disease treatments which are listed on the Life Saving Drugs Program, have provided essential treatments to Australians living with rare and life-threatening diseases (in this case Fabry Disease) since its inception.

It is essential Australians diagnosed with Fabry Disease receive optimal care, support, and medical management for their chronic, progressive, life-threatening condition.

Fabry Australia is concerned for the whole Australian Fabry community and their health. This encompasses access to early diagnosis, coordinated medical care, support, access to treatment without delay and ongoing research.

TOR 1 The range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies;

Fabry Australia is advocating for the current criteria to obtain LSDP funded Fabry disease medicines be urgently reviewed, including adoption of a new set of Guidelines that reflect the knowledge of today and that of global research. There are three funded therapies approved on the Life Saving Drugs Program, two of which were listed in 2004. However, the criteria to obtain such medical treatment is very restrictive and not all of the 300+ Fabry patients are on therapy, actually less than a third receive formal Fabry disease medical treatment. The current guidelines do not allow children and young adults to access any treatment until the disease has progressed significantly. In the meantime, there is considerable individual suffering. Current guidelines do not consider the most recent clinical knowledge, such as our improved understanding of the use of cardiac MRI scanning in Fabry disease.

Incentivising big global pharmaceutical companies to bring international research to Australia is imperative. There is uncertainty about how the Australian regulatory system works and the reimbursement model is unclear, and complex compared to other global models. The pathways need to

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be clearer and for all stakeholders, particularly those with financial investment in new novel medical technologies to ensure businesses are confident to come to the Australian market.

If the Government developed clear policy and tools to solidify this further, it is hoped more research would be conducted in Australia. More incentives need to be provided. Fabry Australia has built strong relationships with international companies who do not have an Australian office, as such, but are willing to work with us and the Fabry clinic Doctors to bring clinical studies to the Australian Fabry patients.

The length of time from clinical trial outcomes to actually listing the medicine/ therapy on the PBS is too long and needs shortening. This is too long when living with a rare, chronic, life-threatening progressive disease. Fabry Australia have members who are enrolled in international clinical studies using novel treatment approaches such as substrate reduction therapy and gene therapy with study sites in both Victoria and Western Australia at the respective Fabry Clinics.

Adopting processes to approve and list new novel therapies such as gene therapy is critical as there are many global studies now enrolling Fabry patients including Australia. These clinical studies are small, and criteria is restrictive, but it is pleasing to see some activity happening now in Australia. It is crucial Australia develops policy and processes in preparation for when these new therapies are ready for listing in the Australian market.

It is important that the timelines for regulatory processes and reimbursement is shortened and delays are minimised to ensure patients with such chronic progressive conditions who have already suffered leading into their diagnosis, access and benefit from such novel therapies without delay.

TOR 2 Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed and used to treat new conditions;

Legislation and regulation that inhibits development and use of novel therapies for Fabry disease.

The Australian Government needs to ensure clinical research is supported formally and actively promoted. The science being used in such studies not only brings hope and treatment benefits to Fabry patients, but outcomes can be extrapolated to other similar conditions.

There appears to be a lack of funding and support including legislation for development and use of novel therapies to treat Fabry disease in Australia.

The pipeline from basic discovery research through to clinical trials and commercialisation for Fabry disease therapies in Australia is too slow

Lack of clear pathways to access orphan drugs, new treatments, and personalised medicines.

Lack of knowledge and information available to patients / Fabry Australia (Patient Organisation) about upcoming novel treatments. All of this information is obtained from the international patient organisation networks via Fabry Australia. The Government does not promote or support this formally.

Lack of approval pathways for personalised medicines for Fabry disease.

The current guidelines state that patients are not permitted to take part in clinical trials whilst on LSDP funded therapy. This guideline is a major disincentive to undertake clinical trials and needs to be urgently reviewed. A process for the evaluation of clinical trials for therapies undertaken at the same time as receiving LSDP funded therapy needs to be established.

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TOR 3 Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies;

Attracting and incentivising research companies to conduct Fabry disease clinical studies in Australia

Inadequate local resources to set up and conduct clinical trials in Australia. In the US – the National Institute of Health funds clinical trial infrastructure in numerous tertiary Hospital/University centres – e.g. staff and physical office space; even fund clinical wards purely to admit subjects for trials.

Enabling Australian Fabry disease patients to participate in international clinical studies

Need for education and support to rare disease patient groups like Fabry Australia, to participate in clinical study design and delivery of trials.

Lack of coordinated infrastructure to support clinical trials;

- Fabry Disease Registry Network
- clinical trial network that supports a national approach to Fabry disease clinical trials with very small patient numbers.
- Streamlined, single point ethics approval processes

TOR 4 Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.

Fabry disease therapies are unable to meet the criteria for subsidy under the current Pharmaceutical Benefits Advisory Committee (PBAC) / Medical Services Advisory Committee (MSAC) pathways which were designed for the evaluation of common disease therapies as opposed to rare diseases.

Thankfully, in the mid 1990's under the 'Act of Grace', The Life Saving Drugs Program (LSDP) was established. The LSDP has allowed Fabry Disease treatments to be listed. This in turn has provided specific treatment for some of the Australian Fabry patient population who meet its specific criteria.

There is inequitable access to the current listed Fabry disease treatments due to the stringent criteria stipulated by the LSDP. Access to treatment for a child diagnosed with Fabry disease is extremely difficult as well as for some adults (male and female). The criteria for receiving LSDP funded Fabry disease treatment needs urgent review and to be in line with international guidelines and standards.

The reimbursement pathways are unclear and inequitable for rare disease treatments, which can add to the time delays or prove too difficult for sponsor companies to bring a particular treatment to Australia.

The multiple and complex approval pathways mean reimbursement is uncertain, lengthy, and delayed.

Fabry Australia would like to see a single Fabry Disease registry, ideally international, as opposed to multiple registries to collect data on all Fabry patients (not just those receiving LSDP funded therapies for their Fabry Disease).

Summary

The Australian Fabry community is very grateful to the Australian Government for funding Fabry treatments. In Australia, the introduction of enzyme replacement therapy and more recently oral chaperone therapy for amenable mutations, funded by the LSDP has significantly changed the lives of

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the Fabry patients fortunate to be eligible to obtain such therapies. The patients who are fortunate enough to receive therapy could not afford to pay for it themselves, so they are very thankful.

However, Fabry Australia believes there is opportunity to do more. Initiation of treatment in Australia is delayed, and in some cases not at all. This is particular the case for the paediatric Fabry disease population who suffer for many years as the disease progresses before individuals become eligible for treatment under the current LSDP guidelines. This is not in keeping with international treatment guidelines.

Fabry disease is a rare, fatal, and progressive condition with a poor prognosis. The current policies, legislations and funding mechanisms are not equipped to address the urgency and the severity of this condition. The pathways to fund and reimburse companies bringing novel therapeutic approaches for rare diseases need to clear, transparent with appropriate timelines. There should be clear indications of the process for all concerned. Adequate resources are required for the Australian Fabry Clinics to best manage Fabry patients and incentivise novel, translational research.

Fabry Australia look forward to reading the outcomes to this inquiry in due course.

Kindest Regards,

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