RECORDATI
RARE DISEASES AUSTRALIA
GROUP

14 September 2020

The Chair
Mr. Trent Zimmerman MP
Standing Committee on Health, Aged Care and Sport
PO Box 6021
Parliament House
CANBERRA ACT 2600

Dear Mr Zimmerman

Re: Inquiry into Approval Processes for New Drugs and Novel Medical Technologies in Australia

By way of introduction, I am the General Manager of Recordati Rare Diseases Australia (RRDA). RRDA is a small pharmaceutical company specialising in rare and ultra-rare pharmaceutical products for Australian and New Zealand patients. Our products treat acute and chronic conditions in neonates, paediatric as well as adult conditions. If left untreated our patients can experience lifelong morbidity and/or death. I welcome the opportunity to make a submission to the above committee. In particular, I would like to address my comments to point two of the inquiry which is as follows:

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;

In regards to registration of medicines by the Therapeutic Goods Administration (TGA), RRDA believes that the TGA is very efficient in registering medical products. The listing of medicines on the Australian Register of Therapeutic Goods (ARTG) is a very involved process ensuring that medicines for use in Australia are safe and efficacious. We are also pleased that the TGA will waive the submission fee for products that are deemed to be orphan, for products that treat a very small number of patients in Australia.

The main issue for my company in commercialising drugs is the cost of applying for reimbursement from the Pharmaceutical Benefits Advisory Committee (PBAC). This is a very real impediment to RRDA supplying medicines to Australians suffering from rare diseases.

Recordati Rare Diseases Australia Pty. Ltd.

Level 6, 69 Reservoir Street
Surry Hills NSW
Australia 2010
RRD.australia@recordati.com

ABN: 26 627 263 094
ACN 627 263 094

RECORDATI
RARE DISEASES AUSTRALIA
GROUP

RRDA welcomes the PBAC initiative (2019) that allows for an exemption from the evaluation fee for the first submission for medicines that have been designated as orphan by the TGA. The issue for a small rare disease company is that we struggle to afford any subsequent submissions with the PBAC if our first submission is rejected. Rejection of submissions is very common, very few get though in the first round. Our products treat only a very small number of patients, usually children. RRDA sales revenue is only a fraction of other pharmaceutical companies. Second round evaluation of submissions can cost over $300,000.00. This fee does not include the cost of market access consultants preparing submissions which adds another $150 000.00 to the overall cost.

As a result of the above costs we can only afford to put one product forward for reimbursement. This puts us in a very difficult position as too which product we will choose to submit. We are only able to put one of the following products forward:

Medicine	Disease/condition	If left untreated
Carbaglu	Hyper ammonia /NAGS	Physical & mental retardation, death
Cystadrops	Cystinosis	Reduced visual acuity, blindness
Ledaga (topical chemotherapy)	Mycosis Fungoides T-Cell lymphoma	Increased growth of malignant skin tumours
Isturisa	Cushing's disease	Hypertension, diabetes, osteoporosis, depression

I believe that the we would be able to submit more medicines to the PBAC if the fees for the first 2 - 3 submissions were also waived.

I fully understand that both the TGA and the PBAC need to recover their costs. The original intention many years ago of waiving fees for orphan drugs was to ensure that Australian suffering from rare diseases had access to the best medicines available. This was because pharmaceutical companies were not interested in rare disease. There were not the same financial returns especially compared to big block buster medicines (Viagra, Prozac, Vioxx etc). The age of the block buster drug is now over. Major pharmaceutical companies now look very much to rare disease as the next area of growth. These companies apply to have fees waived, even though they may be a $1-billion-dollar annual turnover business. I believe that it would be much fairer for the Australian tax payer and to the TGA and the PBAC if the fees were means tested. Both the TGA and PBS would recoup a greater amount of their costs. As an example, companies with an annual turnover of $50 million should not be eligible for fee relief. This would not turn larger companies off seeking to supply orphan drugs, their business now depends on sales of orphan drugs. Many of them making 10s millions from orphan medicines.

RECORDATI
RARE DISEASES AUSTRALIA
GROUP

I thank you very much for the opportunity of making a submission to The Standing Committee on Health, Aged Care and Sport. Please let me know if you have any questions. I would be happy to discuss any issues that you may have in regards to my submission.

Kind regards

[redacted]

Tony Shelton
General Manager
Recordati Rare Diseases
e-mail: [redacted]