Dr Falk Pharma Australia Pty Ltd

Committee Secretariat
The House of Representatives Standing Committee
on Health, Aged Care and Sport
PO Box 6021
Parliament House
CANBERRA, ACT 2600

E Mail: Health.Reps@aph.gov.au

Dear Committee Secretariat,

Re: House of Representatives Standing Committee on Health, Aged Care and Sport inquiry into approval processes for new drugs and novel medical technologies in Australia

Thank you for the opportunity to make a submission to the Inquiry as above. The published Terms of Reference for this enquiry cover 4 distinct areas, however, this submission will particularly focus on Item 4, specifically how this relates to Orphan Drugs and treatments for Rare Diseases:

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.

The current TGA approved definition for an Orphan Drug is one where the prevalence rate is that "the condition affects fewer than 5 in 10,000 individuals in Australia when the application is made". Based on a 2020 estimate of total population of approx. 25m people, this equates to a maximum of 12,500 patients. Where a medicine is indicated specifically for use in children or adults then the population size is amended accordingly. It is further accepted in the TGA Orphan Drug Eligibility Criteria that "it is not likely that it would be financially viable for the sponsor to market the medicine in Australia unless each fee referred to in paragraph 45(12)(c) of the Therapeutic Goods Regulations 1990 were waived in relation to the medicine". This acknowledges the financial reality of low expected patient population impact on the financial viability of new medicines.

The TGA Orphan Drug Eligibility Criteria also state, as an ineligibility for designation, that "none of the following has refused to approve the medicine for the treatment, prevention or diagnosis of the condition for a reason relating to the medicine's safety:

i. the Secretary;
ii. the United States Food and Drug Administration;
iii. the European Medicines Agency;
iv. Health Canada;
v. the Medicines and Healthcare Products Regulatory Agency of the United Kingdom;"

It would seem reasonable that if the above list of international regulatory bodies can be accepted as 'exclusion criteria' for not registering a medicine, they equally should be accepted as 'inclusion criteria' for registering a medicine, so long as the medicine to be registered in Australia is exactly the same as that already registered in any of these 4 jurisdictions. In New Zealand, a 'streamlined pathway' is available for treatments of rare diseases where approval has already been granted in approved 'reference states' including Australia. This more pragmatic approach, allows them to gain the benefit of 'international approval processes'.

Due to their nature, Orphan Drugs are developed to treat 'Rare Diseases' which in most cases means the majority of their development, clinical trials, and initial registration will happen in larger population areas such as the USA or EU, where there are more likely to be higher patient numbers, available to participate in trials. There are certainly patients in Australia who would benefit from participating in these trials, but as the cost of additional 'trial investigation centres' adds to the cost of drug development, companies are unlikely to extend these trial centres beyond their main investigators, unless there is compelling reason to do so.

With treatments for Rare Diseases, due simply to the low treatment populations and treatment comparators, some of the more usual activities associated with medicine development (ie fully-powered multi-centre, randomised double-blind, placebo controlled trials) are unable to be performed. This should be accepted as 'normal' for medicines of this type, and as noted previously if the medicine has been approved for registration in other 'reference' countries, there is no public good served by Australian authorities requesting more data that simply hasn't been able to be created.

Following the registration and availability of these medicines in markets, there is always significant follow up by the pharmaceutical company that developed the products, and the trial investigators, as more knowledge is gained through 'real world' use of the medicine. This can lead to further medical development that enhances its benefit to patients, and may necessitate updates to the registration dossier. Under current regulations, an Orphan Drug designation can only be used once to obtain a 'fee exemption'. After this initial submission, normal fees and approval timelines are levied by both the TGA & PBS, which acts as a disincentive to further develop the medicine or delivery methods/ techniques. It would be more reasonable that, so long as the Orphan Drug continues to fulfill the eligibility criteria, it should continue to benefit from fee exemption. This would act as an incentive for 'post marketing' studies and clinical experience trials to be conducted in Australia.

New medicines based on new research and technologies can be expensive – even more so on a 'per patient' basis for Rare Diseases where the cost of development is spread over fewer patients. When are Rare Disease/ Orphan medicine has been approved for reimbursement in either of the 4 reference markets already mentioned (or in an agreed reference 'basket of markets'), it would simplify PBS submissions if economic modelling used in other countries, could be utilised here, rather than creating Australian specific ones. Naturally, it is accepted these models would need updating with local population and prevalence data (if separately available). These models are expensive to create and are generally a re-configuration of data previously reviewed in these other markets. For the Department to review these reimbursement submissions and then build their own validation models is a further time constraint on bringing these products to market as quickly as possible for the benefit of Australian patients.

We have all seen the incredible frustration of parents whose children could be helped by medicines approved and reimbursed in other countries, that aren't available in Australia. The Australian healthcare budget is not limitless, and for many conditions where existing therapies exist and are reimbursed, it is right that a complete economic evaluation is completed. However, for these rare diseases where no existing therapies exist a more pragmatic approach would seem to take the existing reimbursed prices in other 'reference' markets as the base, and then the Department and Product sponsor negotiate in an transparent and collaborative manner. The end goal is to get a product available for doctors and their patients as soon as possible, and without re-doing what has already been done in other countries. Post market assessments are always a way the Department can monitor the medicine utilisation to the criteria accepted in the initial approval – as is currently undertaken by the Drug Utilisation Sub-Committee (DUSC).

SUMMARY

- Once a medicine receives Orphan designation then this should cover all submissions relating to that medicine with the same active ingredient for the treatment of the same condition as that originally approved – so long as the condition being treated continues to fulfil Orphan Drug eligibility criteria. This will encourage innovation based on local 'real world experience' and with the objective to improve patient outcomes.

- Australia should adopt a 'reference market (or basket of markets)' model for Rare Disease/ Orphan Drugs where the exact product is submitted for registration/ reimbursement locally. If this has already been approved in those markets, then an 'accelerated pathway' for local approval should be available. This will reduce time and cost. Product sponsors would be expected to provide documentation comparing criteria used in the foreign approval, to local conditions, and set out why this is either the same or different in Australia.

- These similarities/ differences can be used as the basis for a transparent & collaborative review with the Department for both registration and reimbursement.

- This would allow Australia to benefit from the work already done in recognised international markets and make these products available sooner whilst still preserving Australian independence.

The above suggestions are for the Committee's consideration, along with other submissions, that all have the end goal of improving treatment options in these rare diseases - for doctors, carers, and patients. I appreciate the opportunity to share the above.

Yours faithfully

[redacted]

Stephen Voordouw
Managing Director
Dr Falk Pharma Australia P/L
M: [redacted]
E: [redacted]
W: drfalkpharma.com.au

8 October 2020