AusBiotech submission in response to the *Priority Review pathway for biologicals: feasibility, potential eligibility criteria and determination process*

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Introduction

AusBiotech welcomes the opportunity to submit a response to the Therapeutic Goods Administration's consultation paper on a *Priority Review pathway for biologicals: feasibility, potential eligibility criteria and determination process.*

AusBiotech is the Australian representative body for one of Australia's most innovative industries with a well-connected network of over 3,000 members in the life sciences industry, which includes biotherapeutics, medical technology (devices and diagnostics), food technology and agricultural biotechnology sectors.

Australia has a substantial life sciences and biotechnology sector, which is consistently ranked as one of the top countries for biotechnology innovation globally when adjusted for population. Industry employs almost 100,000 Australians and consists of more than 1,425 biotechnology companies. Around 80 per cent of these industry companies are classified as small to medium enterprises (SMEs) and are working to commercialise their research, with an important number developing new and novel technologies.

This *Priority Review pathway* response has been developed together with the AusBiotech's AusMedtech Regulatory Affairs Advisory Group, which provides guidance and advice on operational and policy-related regulatory matters, and its Regenerative Medicines Advisory Group, which provides advice on current and emerging issues and trends facing the regenerative medicine sector in Australia and overseas. The submission represents AusBiotech members actively engaged in delivering social and economic benefits to Australia through the commercialisation of biotechnologies and medical technologies – in particular those developing human cell and tissue therapies.

Responses have been framed around the TGA's feedback questions, as requested in the consultation paper.

Question 1: Do you support introduction of a priority pathway for biologicals?

A priority review pathway for biologicals is available in other, comparable and similarly robust global regulatory jurisdictions, and the opportunity to introduce one into Australia is warmly welcomed by industry. It offers a regulatory pathway that will enable patients with unmet clinical needs more timely access to innovative, novel technologies.

Question 2: Is there any expected impact if the proposed Priority Review pathway was to be implemented?

The implementation of the proposed regulatory pathway is a clear indicator that the TGA has committed to an expedited pathway and offers industry greater confidence and a consistent approval process. It also enables quicker access to novel technologies for patients with unmet need requiring urgent products.

Question 3: Do you agree that the 4 proposed criteria for Priority Review of biologicals address the objectives of an expedited pathway?

The proposed criteria offer a balanced approach to unmet clinical needs against resourcing requirements.

Question 4: Do you believe any eligibility criteria should be added, amended, or removed from the proposed Priority Review pathway?

With the clarification amendments noted below, the TGA's suggested eligibility criteria are fit for purpose.

Common considerations

For clarity, it is recommended that the common consideration of '*existence of effective interventions*' is amended to: 'lack of existence of effective interventions'.

Criterion two:

"The determination application must justify the nature of the disease or condition based on figures of morbidity or mortality and life expectancy in Australia"

The Australian life sciences ecosystem actively participates and services a global environment. Oftentimes the ultimate market is international, therefore it is recommended that impact assessment is considered at a global, rather than national, level.

Criterion four

"Major therapeutic advantage - there is substantial evidence demonstrating that the biological provides a major therapeutic advantage in patient outcomes when compared to existing treatments as defined by a magnitude well beyond the minimum threshold of clinical significance."

The consultation suggests that, in order to demonstrate substantial improvement over existing therapies, companies need to have exhausted the search for all of the different modalities that have been used for a particular indication, and provide evidence of its major advantage. Undertaking clinical trials against all other biologicals is unfeasible due to the time and costs involved; therefore, to demonstrate efficacy and a clinically-meaningful improvement, it is recommended that this eligibility criteria is updated to:

Major therapeutic advantage – the Priority Review Pathway is intended for biologicals that demonstrate safety and effectiveness equivalent to or better than standard of care, or for conditions or diseases unresponsive to any other treatment. Specific vulnerable patient populations should also be considered where the existing standard of care medicines are associated with a significant incidence of serious adverse events.

Question 5: Do you agree that the proposed determination process and timing of the steps is appropriate?

The proposed determination process is aligned to international programmes, including the Breakthrough and Fast Track regulation processes, and AusBiotech members are supportive of the 'stop clock' included for sponsors.

As it currently stands, it appears that companies have three months to understand the requirements and to ensure that their application is appropriate for this pathway.

To ensure that only applications proceeding to assessment stages are at a 'readiness to file' standard, it is strongly recommended to extend the optional early engagement period – currently outlined for 3-6 months prior to submission for registration.

Earlier engagement will ensure that the TGA isn't overwhelmed with unnecessary applications, and also ensures that companies can adequately understand the requirements and ensure their applications are appropriate for consideration in the new pathway. Extending this early engagement will also align with the FDA's Fast Track process, where early and frequent communication between the FDA and a life sciences company is encouraged throughout the entire drug development and review process.

Earlier engagement ensures questions and issues are resolved quickly and, as noted by the FDA[1], its benefits include earlier approvals, and therefore access by patients.

As well as earlier conversations, a TGA pre-assessment on whether the company fits its Priority Review Pathway criteria would be welcomed by industry, in order for companies to attain an indication of relevance. For example, to understand whether the TGA considers the company to be targeting a serious or life-threatening condition. This will enable a more efficient and effective application period, with more targeted and comprehensive evidence delivered, enabling the TGA to make its designation within the three-month window indicated within the process outlined.

Question 6: Do you agree that there should be a six-month limit on the duration for the determination for Priority Review of biologicals?

With the above opportunity to engage with the TGA early, applicants should be well advanced at the point of the pre-meeting, and therefore a six-month limit is appropriate. This cap also ensures that the patients' unmet needs are expedited.

Question 7: Do you agree that we should publish the outcomes of approved applications for Priority Review determination of biologicals?

Industry is agreeable with the decision of approved applications being published, however, the details of the decision should remain confidential. This is consistent with orphan drug and other medicines applications, and a standard approval.

Question 8: Do you agree that Decision Summaries and/or Australian Public Assessment Reports (AusPARs) should be published for applications approved through the Priority Review pathway?

Yes, please see above.

Question 9: Does the proposed application fee for a Priority Review determination and the expectation that a higher evaluation fee for an application through the Priority Review pathway seem reasonable?

Yes. Given the flexible resources required the increased fee is appropriate.

Question 10: Do you anticipate utilising the Priority Review process for your products in the future?

AusBiotech Members regularly utilise these expedited pathways internationally, and warmly welcome the opportunity to also utilise a Priority Review process in Australia.

Question 11: Please tell us any other suggestions or comments that you believe will improve the proposed Priority Review pathway for biologicals.

N/a.

[1] https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track#:~:text=Fast%20track%20is%20a%20process,broad%20range%20of%20serious%20conditions.