AstraZeneca

Supporting Document

Key Australian perspectives on an Interim Cancer Fund

March 2024

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Executive Summary

Interim funding for cancer medicines has been a recurring consideration for policymakers and key parliamentary stakeholders in Australia for more than a decade.

This **Supporting Document to the Green Paper on an Interim Cancer Fund in Australia** provides a more comprehensive recount of expert Australian perspectives on Interim Funding Agreements.

Key stakeholders include:

- The Senate Community Affairs References Committee;
- The Pharmaceutical Benefits Advisory Committee;
- The House of Representatives Standing Committee on Health, Aged Care and Sport
- The Centre for Health Economics Research & Evaluation (CHERE);
- The Australian Government; and
- The Reference Committee for the Health Technology Assessment Policy and Methods Review ('HTA Review').

Senate Community Affairs References Commitee (2015)

In the 2015 *Inquiry into the availability of new, innovative and specialist cancer drugs in Australia*, the Senate Committee noted "strong support for the introduction of an interim specialist cancer drug fund".[1] In its Conclusions and Recommendations, the Committee summarised **the case for an interim specialist cancer drug fund**:

"6.27 Evidence to the committee stressed that, while a comprehensive review of the current PBAC processes was necessary, such a review would take time to complete and cancer patients do not have time on their side. Submitters advocated for the introduction of an interim cancer drug fund pending completion of a review, particularly for patients diagnosed with rare cancers.

6.28 The committee is cautious around suggestions that advocate for the establishment of separate regulatory mechanisms specifically to deal with cancer drugs. The committee is mindful of concerns raised about the operation of such funds overseas. In particular, the committee is concerned at the potential for such funds to exacerbate some of the issues identified with the current PBAC system around cost and access to cancer medicines, and the impact of separate assessment processes on the rigour and integrity of the PBAC system. […]

6.30 The committee considers that if [a managed access pathway] were to be established, it is preferable that it is established within the current regulatory framework and operates consistently with existing processes. The committee considers that the current Life Saving Drugs Programme (LSDP) may offer a basis for the delivery of an expanded government funded compassionate access program for patients with rare or less common cancers.

6.31 […] The committee considers that there is merit in drawing on [the post-market review of the operation of the LSDP] to examine the scope for modifying the administration of the LSDP to provide an interim means of subsidised access to medicines for the treatment of rare cancers."[2]

[1] Senate Community Affairs Committee Secretariat (Cth), *Availability of new, innovative and specialist cancer drugs in Australia* (Report, 2015) 103 at 5.73.
[2] Ibid, 108 - 109 at 6.27, 6.28, 6.30 & 6.31.

Pharmaceutical Benefits Advisory Committee (2021)

In the Pharmaceutical Benefits Advisory Committee's Submission to the *Inquiry into Approval Processes for New Drugs and Novel Medical Technologies in Australia* in 2021, the PBAC said the following about early access programs for promising new medicines:

"The PBAC would be more comfortable in its decision-making if there was a specific legislative basis for conditional recommendations and managed access programs. This would also enhance transparency about how and when such conditions could be applied. It would make it clearer to clinicians and patients that continued listing may depend on their participation in additional data collection." [2.1.7][3]

"Noting this is a policy decision for government, based on our observations in countries with similar health systems to ours, such a program should have a legislated framework which is binding on sponsors in relation to negotiated entry price, the period and requirements for establishing a cost-effective price as determined by the PBAC, and agreement to continuation of supply for existing patients for free in the event that the cost-effective price is not agreed between parties. Such legislated frameworks have been implemented in similar government-funded reimbursement systems. Legislated frameworks will enable requirements for data collection and patient participation to be reasonable, relevant and mandated for the PBAC purposes. Such a program would require resourcing for clinical and patient participation, as well as the oversight of access protocols." [2.1.8][4]

"The PBAC notes that such special access programs as practiced in other countries may have a limit on the level of total financial exposure for Government in any year as a mechanism for controlling some of the uncertainty." [2.1.9][5]

"The PBAC strongly believes an early access program should not be limited to a specific disease or condition although the eligibility criteria of a medicine for such a program should refer to high unmet need and disease severity/prognosis." [2.1.10][6]

[3] Department of Health, Submission No 15.3 to House of Representatives Standing Committee, Parliament of Australia, Inquiry into Approval Processes for New Drugs and Novel Medical Technologies in Australia (24 June 2021) 4 at 2.1.7.
[4] Ibid at 2.1.8.
[5] Ibid at 2.1.9.
[6] Ibid at 2.1.10.

House of Representatives Standing Committee on Health, Aged Care and Sport (2021)

Following a referral in 2020 from the former Minister for Health, the Hon Greg Hunt, the House of Representatives Standing Committee on Health, Aged Care and Sport conducted the *Inquiry into approval processes for new drugs and novel medical technologies in Australia*. The former Chair of the Committee, Mr Trent Zimmerman, tabled *The New Frontier – Delivering Better Health for all Australians* report in Parliament in 2021, with the following recommendation:

Recommendation 10 – The PBAC and Managed Access Programs

"The Committee recommends that the Australian Government amend the National Health Act 1953 (Cth) to give the Pharmaceutical Benefits Advisory Committee the power to authorise Managed Access Programs. The eligibility criteria for these Managed Accessed Programs should be aligned as far as possible with the eligibility criteria for the Therapeutic Goods Administration's provisional registration." [7]

[7] House of Representatives Standing Committee on Health, Aged Care and Sport (Cth), The New Frontier – Delivering better health for all Australians (Report, November 2021) 324 at 11.11.

Centre for Health Economics Research & Evaluation (CHERE) – HTA Review Consultation 1: Report (2023)

In CHERE's *HTA Review Consultation 1: Report*, which synthesises all submissions to Consultation 1 of the Health Technology Assessment (HTA) Review in 2023, it was noted that:

"*Options to enhance timeliness and agility within the system focused on introducing new approaches to the process for determining whether technologies are reimbursed and at what price, altering existing processes to deal with evaluation issues as they arise, and recognising the importance of compassionate access programs in facilitating earlier access, notably: […]*

Introduce 'streamlined listing processes':

i. When a therapy is already internationally approved and will only result in Commonwealth expenditure of <$10M in AU, then these should be subject only to a direct price negotiation.

ii. There could be a triaging system which allows a price benchmark – perhaps a percentage of the German price - for new chemical entities or therapies in disease areas with high unmet clinical need (similar to the Innovative and Licensing Access Pathway in the United Kingdom) to allow for immediate access while permanent pricing and post-market requirements are negotiated over a 6–12-month period.

*iii. When TGA approval is granted, patients with high priority conditions, such as cancer, are given immediate and affordable access to the drug or test in question. (Note that TGA approval is often fast-tracked as a result of recent changes to their processes - a paradigm that has not been significantly taken up by the PBAC, but hopefully will be considered as part of the current HTA Review). When such patients are given immediate access to the innovation in question, the PBAC, MSAC, DoH and the sponsor are then given 2-3 years to resolve whether there is sufficient evidence of cost-effectiveness to allow permanent listing on the PBS. If that can't be realised, the drug is effectively delisted and is no longer available to future patients. (*High priority conditions will need to be defined but one such definition might be 'life-threatening illnesses which are inherently unstable over time (e.g., Cancer), that generally worsen progressively and for which treatments that work in one phase of the disease may be much less useful in subsequent phases of an illness.')*

i. **For new technologies or those with substantial benefit it may be possible to have an interim listing process** *– where PBAC has recommended the therapy, and the applicant wants to proceed based on the parameters outlined by the PBAC (such as risk sharing arrangements (RSAs), and special pricing arrangements (SPAs) etc.) – where listing is highly probable but requires further negotiation. During this process, an interim listing may be possible, where the applicant agrees the interim listing would be for a specified period, and after that period (or is the applicant does not want to continue the PBS listing process), the applicant is responsible, both for supply and cost, for all "supply only" scripts for the prescribed population during the interim listing period. This would guarantee the applicant does not prolong negotiations to sustain the status-quo of the interim period prescribing conditions, while also allowing access to therapies for patients in need. However, the Department would also need to put in place additional safeguards for the applicant – to ensure that any delays in negotiation due to the Commonwealth do not cause the timing of the interim period to expire, creating a significant risk for the Manufacturer, and a disincentive to use such a system. These safeguards could include being transparent with timelines and decisions on the Commonwealth's side as well as mandatory extensions of deadlines if the Commonwealth does not address the required issues in time.*

i. Recalibrate the milestone requirements for parallel processing, so that PBAC recommendations are not delayed due to misalignment with the TGA Delegate's Overview. This could be achieved by re-anchoring PBAC consideration to the end of the TGA evaluation phase at Milestone 5 or working with the TGA to bring the timing of the Delegate's Overview forward by several weeks.

ii. For incremental, marginal products, implement a more limited evaluation process (no need for a full HTA) - products can be listed as cost-minimisation."[8]

[8] De Abreu Lourenço, R., Paige, K., Haas, M., Thomas, T., Carrello, J., Viney, R., Manipis, K., & Goodall, S. (2023). *Consultation 1 Report. Australian Health Technology Assessment Methods and Policy Review*. Canberra: Australian Government, Department of Health and Aged Care 38 at 5.2.1.

Australian Government (2023)

The whole-of-government response to the 2021 Recommendation, coordinated by the Department of Health and Aged Care, is:

"The Australian Government accepts this recommendation in principle, noting that amendments to the National Health Act can only be made by the Australian Parliament.

The Managed Access Program is an existing mechanism that enables the listing of medicines registered by the TGA on the PBS under special circumstances of high unmet clinical need, on terms that allow for the resolution of otherwise unacceptable clinical or economic uncertainty for the PBAC. The PBAC is able to recommend products for listing under a Managed Access Program as outlined in the 2015 framework.

While these arrangements can lawfully be given effect under current legislation, options to specifically legislate the arrangements will need to be considered by the Australian Government.

Under the Strategic Agreement with Medicines Australia, the Commonwealth has acknowledged the need to complement the priority and provisional medicines pathways used by the TGA, and agreed to work with Medicines Australia to consider options for conditional funding arrangements. Matters in respect of efficient and equitable funding approaches and pathways for medicines will also be considered as part of the HTA Review."[9]

Additionally, the government has recognised the appetite for faster access for cancer patients. Minister for Health, the Hon Mark Butler MP, intervened to secure early access for Australians to a first-in-class therapy in development for Diffuse Intrinsic Pontine Glioma (DIPG), an aggressive brain cancer, in February 2024.[10] Median survival for Australians living with DIPG is nine months. Australian patients who are ineligible for a trial will now be able to apply for early access to the treatment.

[9] Department of Health and Aged Care (Cth), *Australian Government Response to the Standing Committee on Health, Aged Care and Sport report: The New Frontier – Delivering better health for all Australians (*November 2023) 26.
[10] Hon Mark Butler MP, *Special access to childhood brain cancer medicine* (Media release, 5 February 2024).

Reference Committee for the Health Technology Assessment Policy and Methods Review (2024)

The Consultation Options Paper, published on 29 January 2024, is the most recent report of the HTA Review Reference Committee (the Reference Committee), which oversees the HTA Review. It presents options for reform being considered by the Reference Committee to improve Australia's HTA policies and methods and funding and approval pathways.

In **Chapter 4.1: approaches to funding or purchasing new health technologies and managing uncertainty**, options for revised MAPs to accelerate funding for therapies that qualify as High Added Therapeutic Value (HATV) with High Unmet Clinical Need (HUCN) are provided. The Paper provides options in relation to Managing Uncertainty:

Bridging funding coverage for earlier access to therapies of likely HATV and HUCN

Establish bridging funding through a capped special funding program (separate and distinct from the PBS special appropriations) or legislate to enable conditional listings on the PBS.

The purpose of either of these options would be to provide for a time-limited period, bridging funding coverage for earlier access to exceptionally promising, time-critical, therapies of HATV and HUCN, but that have significant clinical, economic and/or financial-based uncertainty. The program would need to be designed in a way that does not introduce further complexity into the system nor create perverse incentives that would prolong assessment and commercial negotiations.

The design of this program should incorporate specific details on the eligibility requirements that health technologies need to meet to qualify for funding from this program that aligned with the core HTA and pricing negotiation steps that are features of the Australian HTA process and include, but not be limited to:

- *Early identification and nomination via horizon scanning and/or designation on a Priority List of HUCN conditions.*
- *Eligibility requirements to lodge TGA and PBAC submissions (simultaneously) for the health technology within 6 months of receiving first international regulatory approval (i.e. FDA/EMA)*
- *Requirement for parallel TGA/HTA Committee submission lodgement as part of a broader overall approach to support timely recommendations.*

- *Approach undertaken by the applications and evaluation that:*
  - *provides the HTA committee with options to make recommendations for interim conditions of funding for the purposes of bridging access, and recommendations that inform further price and access negotiations; or*
  - *facilitates finalisation of price and access negotiations between the sponsor and the healthcare payer prior to presentation to the HTA committee for consideration.*
- *Administration that enables clinical data to be collected and reviewed.*
- *A clear process for re-assessment and final decision-making on whether (and when) the health technology should transition onto ongoing funding arrangements (such as the PBS, MBS or NHRA-style arrangements, with or without additional evidence development), or whether bridging funding should be withdrawn. Such decisions would be based on what pre-defined evidence has accrued during the time limited period, and whether the health technology is performing as anticipated.*

Revised Guidance on the uses of different managed entry tools

Revised guidance and policy arrangements that encourage the creative proposition and utilisation of managed entry arrangement instruments by the respective parties, supported by more explicit HTA committee recommendations enabled by appropriate changes to current policy and legislation, would facilitate greater uptake and provide more options to sponsors and the Commonwealth to engage with uncertainty more constructively and collaboratively, as part of improving timely access to health technologies.

Note: this may need to be accompanied by changes to negotiation guidance, policy, regulations and/or legislation to facilitate implementation.[11]

[11] Health Technology Assessment Policy and Methods Review Reference Committee, Health Technology Assessment Policy and Methods Review: Consultation Options Paper (Report, 2024) 137 – 139.

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