Novartis Pharmaceuticals Australia Pty Ltd
[redacted]

23 Feb 2024

HTA Policy and Methods Review: Response to Consultation 2

Dear HTA Reference Committee,

Novartis Australia is committed to ensuring that Australians are amongst the first in the world to access medicines that provide benefits not only to them, but to wider society. As such we welcome the opportunity to engage and contribute to the Options Paper consultation.

The HTA review offers a significant opportunity to decrease the time Australians need to wait to access medicines and improve the overall health ecosystem within Australia. Given the importance of this review Novartis Australia has, in the interests of collaboration, provided open and honest feedback on those areas we support, those areas we believe more detail is needed before any firm view can be reached and those areas that we cannot support.

Novartis Australia is committed to working with all stakeholders to ensure that the goals of this review can be realized. As such if further clarity or context is needed, please do not hesitate to contact me.

Sincerely,

Dylan Jones
Country Head of Value & Access

Executive summary

As the Committee will be aware the HTA review stems from a commitment in the 2022-2027 Strategic Agreement, with shared goals between the Commonwealth and Industry of:

- reducing time to access for Australians so that they can access new health technologies as early as possible.
- maintaining the attractiveness of Australia as a first-launch country to build on Australia's status as a world leader in providing access to affordable healthcare.

Australia has long been a leader in its ability to achieve the aims of our National Medicines Policy, namely that all Australians have fair, timely, reliable and affordable access to high-quality medicines and medicines services. However, it is important to note that the current policies which govern how Australia assesses and values medicines mean that the future of timely and reliable patient access to new medicines is no longer guaranteed. That is, issues such as lowest cost comparator pricing, the level of uncertainty that is accepted in an economic model, reference pricing and how medicines are valued mean that Australia is no longer the attractive priority launch country that it used to be. As new innovative platforms, such as Radio Ligand Therapies, come through into health eco system at scale, improvements to the overall HTA system are critical to ensure Australia is set up to benefit from the health and economic benefits these new platforms can bring.

The relative size of the commercial opportunity in the Australian market compared with the issues identified above mean that pharmaceutical companies are increasingly choosing to either deprioritise and delay launching new medicines, as Novartis has done, or simply not launch specific medicines at all in Australia. By more appropriately valuing new medicines and providing a predictable access and pricing environment for those medicines post-launch, Australia can quickly regain its place as a leader in the global healthcare space.

Whilst the Options Paper provides potential for some reform and reducing the time to access for patients, Novartis Australia is of the overall view that the options, as presented, risk increasing the time to patient access by jeopardising Australia's position as a first-launch country. This response sets out in detail the areas we support, the areas where we have concerns and those areas where further detail is required.

In summary Novartis Australia is of the view that:

- Increasing consumer, clinician, First Nations people and other stakeholder involvement in HTA is positive and will ensure that a wider perspective supports HTA decision making.
- Establishing a fit for purpose valuation framework in Australia is key to accelerating access for patients and maintaining the attractiveness of Australia as a first-launch country.
- A change to the way in which the PBAC assesses the underlying evidence or deals with the inherent uncertainty of early data, is necessary to ensure the potential of many of the proposed options is realised. Without this many options will have the unintended consequence of increasing the time that many Australians will have to wait for access to new health technologies.
- Several process options could result in prioritising some areas of healthcare, at the detriment of others. This would create in an inequity of access for patients and result in Australia falling behind other similar health systems.

- The continued ambiguity on the issue of comparator selection is disappointing. The issue of lowest cost comparator is longstanding and a significant barrier to access that worsens over time. The current option as presented further delays resolution to this important issue and does not provide sufficient clarity.
- Options that either explicitly or implicitly compel sponsors to accept prices that do not properly value the innovation or add additional pricing negotiations following HTA evaluation are unacceptable. To do so will only result in increasing the time to patient access and put at risk the sustainability of medicines and medicines-related services in Australia.

Below is a curated summary of Novartis Australia's views on the options, which have been categorised as :

- support as stated;
- support with amendments;
- require more detail on the option to form a final view; or
- cannot support.

Areas that cannot be supported

- 2.2 Proportionate appraisal pathways: Development of pathways to calibrate the level of appraisal required for HTA submissions to the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents.
    - Development of a disease specific common model
- 3.3 Economic evaluation
    - Selection of the comparator
    - Valuing of long-term benefits
- 4.1 Approaches to funding or purchasing new health technologies
    - Recognising competition between new health technologies that deliver similar outcomes
    - Post-listing re-assessment of health technologies
- 5.5. Capacity and capability in the HTA systems

Areas that are supported as stated

- 1.2 Consumer, clinician and other stakeholder engagement and consideration in HTA:
    - Develop an engagement framework.
- 2.2 Proportionate appraisal pathways: Development of pathways to calibrate the level of appraisal required for HTA submissions to the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents.
    - Decouple requirements for the TGA delegates overview to support PBAC advice.

Areas supported with amendments

- 1.4 State and territory government collaboration in HTA
    - Increase opportunities for consultation and work sharing
    - Development of central standardised data sharing system for utilisation and outcome data
    - Health technologies that are jointly funded by the Commonwealth and state and territory governments (such as high cost, Highly Specialised Therapies (HSTs) delivered to public hospital inpatients)
- 3.1 Determination of the population, intervention, comparator, outcome
- 3.3 Economic evaluation
    - Valuing overall
- 5.1. Proactively addressing areas of unmet clinical need and gaps in funded access
- 5.2. Horizon Scanning
- 5.4. Mechanisms for continuous review and improvement

Areas that require more detail to form a final view

- 2.1. Streamlining and aligning HTA pathways and advisory committees
- 2.2 Proportionate appraisal pathways: Development of pathways to calibrate the level of appraisal required for HTA submissions to the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents.
    - Triaging submissions
    - Streamlined pathway for cost-minimisation submissions (therapies not claiming improvement in health outcomes or reduction in toxicity)
    - Information regarding the price of the comparator the proposed therapy is cost-minimised against
    - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN
- 3.2 Clinical Evaluation Methods
- 4.1 Approaches to funding or purchasing new health technologies
    - Pricing offer (PO) and negotiation guidance framework
    - Approaches for managing uncertainty - revised guidance on the uses of different managed entry tools
- 5.3. Environmental considerations in HTA

The HTA review offers a significant opportunity to decrease the time Australians need to wait to access medicines and improve the overall health ecosystem within Australia. This opportunity can only be achieved through all parties working collaboratively with the best interests of the patient at the centre of all discussions, whilst balancing the sustainability of the medicines industry that supports them. Significant work lies ahead, and Novartis Australia is committed to working with all stakeholders to ensure that the goals of this review can be realised.

Detailed Feedback on the Options paper.

This response addresses selected options detailed in the consultation paper, section by section. For each response we have set out Novartis Australia's viewpoint/concerns on the option as presented and whether we:

- support the option as stated;
- support with amendments;
- require more detail on the option to form a final view; or
- cannot support the option.

Transparency, communication, and stakeholder involvement in HTA

1.2 Consumer, clinician and other stakeholder engagement and consideration in HTA.

Develop an engagement framework.

Viewpoint: Support

Novartis supports the development of an engagement framework that promotes early involvement of consumers, clinicians, and other relevant stakeholders in the HTA process. This early engagement can help ensure that the correct and necessary information is sought from these stakeholders well before the PBAC/MSAC meetings. It is essential that this engagement framework is available to all new health technologies, without any limitations or restrictions based on the type of therapy. All innovative therapies should have the opportunity to benefit from early engagement, as it can provide valuable insights that support the decision making of those with limited experience of the clinical area.

Novartis recognises the importance of considering the impact of health technologies on Aboriginal and/or Torres Strait Islander peoples. If a health technology is identified as potentially benefitting these communities, seeking advice from representatives of Aboriginal and Torres Strait Islander community-controlled health services early in the process will ensure that that the voices and needs of our First Nations Peoples are included. It is important that this perspective is considered by the PBAC in its deliberations as frequently specific evidence within this population will not be available for any submission. In addition, the valuation of any impact from a health technology needs to consider the ability to narrow the health inequality gap that is so harmful to improving longer term outcomes in these communities.

1.4 State and territory government collaboration in HTA.

The state and territory collaboration and possible amendments are discussed in the sections below. However, an outstanding issue that needs addressing is the funding of high-cost technologies which are currently funded through NHRA. Novartis has listed 2 gene therapies, one on the PBS and one through NHRA and two indications for a cell therapy through NHRA and believe that the funding mechanism for those through NHRA has caused delays in access to treatment for a group of patients who cannot afford to wait. A large part of the delay is the lack of post-recommendation timelines and the need to negotiate with each state and territory separately. A federally funded scheme for these therapies which do

not fit within the purview of the PBS would reduce the need for negotiation and increase the time to access for patients.

Increase opportunities for consultation and work sharing.

Viewpoint: Support with amendments

Amendments:

1. **Inclusion of Sponsors within any data sharing activities with Federal and State and Territory governments for health technologies which are being evaluated through NHRA and for those outside the NHRA process.**
2. **A clear process involving hospital costs and process must be agreed before the evaluation process. This must also be independently reviewed in the post-market period. A clear process that independently evaluates hospital processes and associated costs should be agreed to in advance of the evaluation process. This process would also apply in any post- reimbursement review process.**

The NHRA is a collaboration in funding between the Federal and State and Territory governments for high-cost drugs. High-cost drugs are assessed through the MSAC evaluation pathway and require a cost-effectiveness model as part of the evaluation and then require a subsequent post-market review. However, of note is that the funding mechanism is split between the Federal and State and Territory budgets.

The experience of Novartis with both the Kymriah and Luxturna submissions is that state hospital data was not made available for the development of the model, this is despite repeated contact with IHPA (now IHACPA). Models therefore were developed for the submission using publicly available data and using inputs such as DRGs and MBS items. The impact of utilising the publicly available data, and not being granted shared access to the state hospital data, became apparent in the first review of Kymriah for ALL. In this review the comments received from the states suggested that the hospital costs were underestimated and therefore impacted the cost-effectiveness and value of the intervention. Of concern was that the costs provided by the states were unable to be verified by either the sponsor or MSAC, yet they became an integral part of the cost-effectiveness analysis of Kymriah, likely undervaluing it. There is a concern therefore that without greater opportunities for consultation, work-sharing and verification of data from all parties, that Highly Specialised Technologies will struggle to gain initial and continued reimbursement within the current system.

An early opportunity for consultation and work-sharing between all parties would allow:

- More complete hospital costs to be included in the initial submission;
- The establishment and costing of an appropriate hospital pathway during the initial evaluation rather than hospitals developing various pathways independent of the health technology;
- A standardisation of the hospital pathway across jurisdictions to allow for accurate costing of the procedures and care plan;
- A clearer path forward in the further evaluation of the health technology removing the uncertainty that current processes bring to the review.

The idea of consultation with work and data sharing between sponsors, state and federal funding is also important outside the space of NHRA. Health technologies, such as radioligand therapies, that may sit outside the scope of the requirements of PBS or MBS listings and may not always meet the high-cost requirement applicable to NHRA reimbursement. For these technologies, it will be imperative to be able to accurately determine the costs to the whole health system to enable access at the time of evaluation, rather than costs being provided after the reimbursement. This is critical to avoid putting at risk sustained access for patients.

Development of central standardised data sharing system for utilisation and outcome data.

Viewpoint: Support with amendments

Amendment:

1. **Outcomes collected need to be agreed to by sponsors and the subsequent data need to be made available to all parties including the sponsor.**

Novartis supports in principle the development of a central and standardised system for utilisation and outcome data. The requirements set out in the negotiated deeds for products evaluated through the NHRA pathway involve substantial data collection requirements for the review process, the development, cost and maintenance of which are met by the sponsor.

The comments provided during the evaluation of the Kymriah ALL review process revealed that the data provided from the registry did not meet the expectations of the evaluator to complete the review process. These comments were made even though the registry was set up based on agreed parameters as part of the initial reimbursement submission. A central standardised system may allow for changes to be made as needs are identified thereby supporting any future review process. This level of agility is not available when commercial agreements are in place for the collection of specific outcomes which are agreed in advance. Of critical importance in the establishment of any system is the open and transparent sharing of these data across parties, including the affected sponsor. This will ensure a smoother and effective review process not impacted by an information imbalance.

Health technologies that are jointly funded by the Commonwealth and state and territory governments (such as high cost, Highly Specialised Therapies (HSTs) delivered to public hospital inpatients).

Viewpoint: Support with Amendments

Amendments

1. **Clear timelines for evaluation, funding and review need to be established across Federal, State and Territory governments.**

Novartis supports the idea of a federally funded pool of funding for high-cost technologies. However, in the current environment, Novartis supports the idea of Horizon scanning for looking to determine disruptive technologies to be prepared for the evaluation of these technologies through NHRA. However, Horizon scanning will not help with some of the specific issues that prevent access. Novartis is more experienced than most with the NHRA

evaluation, reimbursement and review process and we would welcome the ability to input into more deeply into any review as to how to improve this process.

Schedule C of the NHRA outlines 6 reforms of which 'nationally cohesive health technology assessment – improving health technology decisions will deliver safe, effective and affordable care' is most relevant to this review. There is sometimes uncertainty around which pathway a technology should be evaluated through (for example Zolgensma initially went to MSAC for NHRA and then was sent through the PBAC pathway) and when a positive recommendation through NHRA is received, the process of negotiating to listing is unclear. In the review process, that occurs after reimbursement, it appears that there is not a clear understanding of how the outcomes of any review will be implemented, challenged or how it links to the individual reimbursement agreements.

Developing timelines from agreement to access is crucial in ensuring access is achieved quickly. Post a positive recommendation from MSAC, the process of agreement between the federal and state and territory governments is a significant factor in the delay to access. Having to negotiate individual agreements with each jurisdiction and in the process, starting at the beginning with each one, increases time to the access for patients. Also, there are no clear timelines associated with the review process, making the continuity of access uncertain. Timelines across the entire NHRA evaluation and review process need to be standardized, published and adhered to.

Health technology funding and assessment pathways

2.1. Streamlining and aligning HTA pathways and advisory committees.

Viewpoint: More detail required

Novartis acknowledges the importance of streamlining and aligning Health Technology Assessment (HTA) pathways. This approach can potentially improve the efficiency and consistency of the HTA process, facilitating timely access to innovative therapies for patients. However, it is crucial to carefully consider the resource requirements necessary to achieve this ambitious goal.

Insufficient resources could result in an overburdened committee, which may not have the capacity to adequately evaluate the submissions. This could potentially lead to submission churn, the delaying of those submissions not deemed a priority and hinder timely access to therapies rather than solving for it. Novartis emphasises the need to carefully consider the allocation of resources to ensure that the goal of efficiency and streamlining recommendations is achieved.

All of the suggestions presented involve the development of new processes or require investigations with little detail around what these new processes will be, the timelines involved in their development and implementation and the involvement of industry in this. As such it is not possible to support these options until additional detail is shared.

2.2 Proportionate appraisal pathways: Development of pathways to calibrate the level of appraisal required for HTA submissions to the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents.

Triaging submissions.

Viewpoint: More detail required

The proposal of triaging submissions as part of proportionate appraisal and streamlining of HTA pathways is one approach to improving efficiency and determining the appropriate level of evaluation. What is not made clear in the options paper is who will be responsible for the triaging of submissions, the associated framework and the information that will be applied to reach a decision. We note that an outcome within the triaging process is to determine the meeting at which assessment will take place. Novartis Australia would not support a position that resulted in some submissions having their assessment unduly delayed.

Streamlined pathway for cost-minimisation submissions (therapies not claiming improvement in health outcomes or reduction in toxicity).

Viewpoint: More detail required

Criteria which allow a streamlined cost-minimisation pathway would be welcomed if developed in consultation with industry. However, support for this pathway is contingent on removing the requirement to provide a lower price than the comparator. Novartis Australia cannot agree to any proposal that requires these submissions to offer or accept a lower price when claiming non-inferiority to the standard of care.

Novartis supports the idea of streamlining for cost-minimisation submissions to allow for faster access for patients. However, further detail is required including:

- The information needed to allow a fast-track submission and at what point this is determined;
- What an abbreviated process would entail and how this would interact with the current meeting schedules. We note that if the submission passed through the normal process prior to consideration by ESC, the proposed process would not reduce the time to access. Hence additional detail is required before it is possible to support the option.

Information regarding the price of the comparator the proposed therapy is cost-minimised against would be shared with the sponsor early in the process prior to HTA committee consideration. This would allow sponsors to make an informed decision regarding whether to process or withdraw the submission from consideration.

Viewpoint: More detail required

In respect of the proposal of sharing the confidential price of the comparator there are significant risks in terms of the maintenance of price confidentiality. Price confidentiality is a significant issue for sponsors and significant controls would need to be in place about when such information could be released. Novartis Australia notes the potential benefits of such an option but more detail is required before this can be supported to ensure appropriate controls are in place.

Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN.

Viewpoint: More detail required

The proposed early resolution pathways for areas of HUCN puts forward many different options about how to resolve outstanding issues prior or before the HTA committee meeting and that any option that is taken forward would be trialled for these options prior to being applied to other areas.

Novartis Australia has concerns about the proposal, these include:

- This pilot's impact on the current early re-entry/resolution pathways that have enabled faster access for patients;
- The negotiation of price before the PBAC have provided a view on value, would undermine their role in a HTA system with no fixed ICER;
- The number of interventions that will meet the HATV/HUCN criteria will be small therefore raising the question as to how much would be learnt from any pilot. The pilot should be applied to all submissions;
- By assessing these interventions so early, data will typically be immature therefore creating uncertainty and an increase the likelihood that an agreement of value could not be reached;
- Limiting the number of resubmissions will result in sponsors waiting to bring forward interventions for consideration until the uncertainty is minimized – thereby delaying access.

As currently presented only Alternative Option 4 presents a viable way forward as it retains the importance of the PBAC in determining the value of the intervention in question. However, until further detail is provided it is not possible to support any option that appears to amend the current streamlined pathways.

Development of a disease specific common model.

Viewpoint: Cannot support

Novartis does not see any value in a disease specific common model as there are product specific requirements that would need to be changed for each submission. This would also mean standardisation of parameters used in the models which may not always be acceptable to the sponsor.

Decouple requirements for the TGA delegates overview to support PBAC advice.

Viewpoint: Support

Novartis would support this decoupling if there were not an implied requirement for early PBAC submission and that it was at the discretion of the sponsor when the product was submitted to the PBAC.

Methods for HTA for Australian Government Subsidy (technical methods)

3.1 Determination of the population, intervention, comparator, outcome.

Viewpoint: Support with amendments

Amendments:

1. **Simplified, time limited, PICO development to be carried out for all submissions for reimbursement.**
2. **Stakeholder involvement for all reimbursement submissions.**

The determination of the appropriate PICO is an important step in the development of all evidence base guidance. A PICO ensures alignment between all stakeholders early in the process and sets the direction for the assessment of the clinical and economic assessments. This subsequently reduces the risk of submissions being rejected due to disagreements around the question at hand and therefore can decrease the time to patient access. However, it is critical that any PICO process does not replicate the current PASC process which is unnecessarily long and will result in delays to patient access.

Novartis believe that the development of a PICO (and involvement of stakeholders) should apply to all submissions, not just new molecules and or major expanded indications claiming added therapeutic value. This is necessary given the difficulties that can exist in determining the appropriate comparator(s), especially given the challenges surrounding the operation of section 101(3B) of the National Health Act 1953.

Following the development of the draft PICO it is ultimately for the Sponsor of any medicine to determine the population or sub-population for which reimbursement is sought. Compelling sponsors to apply for reimbursement in populations where it is not possible to reach agreement on a cost-effective price will only delay patient access and result in therapies not being brought forward for reimbursement in Australia.

3.2 Clinical Evaluation Methods.

Viewpoint: Detail required

All the items that are outlined regarding the clinical evaluation methods in the Options Paper are, in principle, extremely important to addressing uncertainty in health technology assessment. Clarity on the methods of assessment of non-randomised and observational evidence, assessment of surrogate endpoints, consideration of qualitative value are all critical to appropriate HTA decision making. The options paper, however, while calling these out as important, provides no detail for the implementation of the work and/or changes to how these will be assessed. Importantly, it does not address whether following the completion of all this work, and application of this by sponsors, these changes will ultimately lead to the PBAC being more comfortable with the outputs of these methods and the residual uncertainty. Increased levels of comfort with the residual uncertainty following the application of contemporaneous methodologies would lead to faster access for patients at a reasonable and appropriate value for the medicines industry. Without that increased acceptance there is little benefit in further adapting the methods at hand.

While the review has presented recommendations, there are differences in what is presented in the Consultation Options Paper compared to the HTA review paper on Clinical Evaluation. There is uncertainty as to whether the Options papers means to implement everything addressed in the HTA Review Paper or just the few points that have been identified for publication in the Consultation paper. For example, the HTA Review paper provides a list of surrogate endpoints that were found acceptable by other jurisdictions for different conditions. The options paper refers to guidance being required for the use of surrogate endpoints, methods to validate these and for the evaluation of evidence using surrogate endpoints. However, it is not specific as to whether the list in the HTA Review Paper is the list surrogate endpoints to be taken forward, or if not, where the considerable amount of guidance required is coming from if not from the work supplied in the HTA Review Paper.

The suggestion of a curated list of methods that are preferred by decision makers is currently lacking detail as to which decision makers are being referred, and what makes a method a preferred technique. There is no information as to who will be curating this list and whether they have access to the most up to date methodologies. It also leads to questions around how often this list would be updated and how that would occur. Again, while it states that that this list would be provided to committees as a list of what has been used elsewhere, it does not provide any indication as to whether the use of methodologies preferred by other agencies would mean the PBAC would accept them as a means of reducing uncertainty in PBAC decision making. Without a change in that approach at the committee level, an updating of associated methods will not have the impact of accelerating access for patients and maintaining the attractiveness of Australia as a first-launch country.

3.3 Economic evaluation.

Selection of the comparator.

Viewpoint: Cannot Support

Whilst careful thought should be given on the operation of section 101(3B) of the National Health Act (1953) the continued ambiguity on the issue of comparator selection in the Consultation Paper is disappointing. The issue of lowest cost comparator is longstanding and a significant barrier to access that worsens with the listing of each new product in the therapeutic area. Novartis, clinicians, other Sponsors in Australia, and Medicines Australia all agree that the appropriate comparator to new a medicine should be the medicine most likely to be replaced in practice, or an appropriately weighted basket of therapies.

There are examples where the application of this policy can even impact already listed molecules for an indication. For example, when bringing a new, more convenient formulation forward for listing. Instead of the new, patient friendly formulation, being compared to its already listed version, it is instead compared to the lowest cost comparator at the time. Given that the new formulation will only likely replace the already listed version, a misapplication of the lowest cost comparator policy can prevent patients from accessing a more convenient dosing schedule, at no additional cost to the government.

Valuing of long-term benefits.

Viewpoint: Do not support

The issue of the need for a change in the discount rate is longstanding with significant work already carried out by industry and academia. The option as presented advocates further work, assessment, and deliberation, with a potential for a change in the ICER to offset any impact in the valuation of the intervention.

As has been highlighted previously, compared to its peers, including those countries part of the HTA collaboration, Australia is an outlier. The PBAC's base-case discount rate of 5% for health benefits and costs is higher than many other countries with comparable levels of economic development and similarly advanced HTA systems, including: France (4%), Ireland (4%), New Zealand (3.5%), Scotland (3.5%), UK (3.5%), Germany (3%), Singapore (3%), Sweden (3%), US (3%), Japan (2%), Belgium (1.5% benefits, 3% costs), Canada (1.5%), and The Netherlands (1.5% benefits, 4% costs). [1] The assertion in the position paper that the decision of discount rate is a government policy decision further delays resolution to this important issue.

Given the work done, and presented to the PBAC, further work in this area seems unnecessary. Similarly, a suggestion of a corresponding reduction in the ICER is not an appropriate solution as it dissipates the changes to the discount rate. Such approaches maintain the status quo that undervalues the long-term benefits from either changing the trajectory of a child's life through gene therapy or the prevention of a cardiac event in the future. Given the current approach to assessing uncertainty already requires lower ICERs for preventative treatments, the retention of the current discount rate only entrenches the view that prevention of a condition is not valued as much as treatment of that same condition. This is not congruent with a policy view of improving the health of the Australian population. Novartis believes that a reduction in the discount rate to at least 3.5% would bring Australia in line with similar HTA systems, in addition that there is merit in the consideration of differential discount rates where long term health is valued more than costs.

Valuing overall.

Viewpoint: Support with amendments

Amendments:

1. Workshops should focus on the value Australians place on healthcare.

The option as presented is akin to the panels and workshops run by the National Institute for Health and Care Excellence (NICE) to garner views from the public on the principles that should support decision making at NICE.

As presented the option is focused explicitly on price, which does not answer the important question at hand – the value Australians place on health and in what circumstances. A focus on price influences the answer without exploring the view of the Australian public around how health should be valued, the trade-offs that should be made and what value framework should be applied to determine that appropriate value.

[1] https://www.pbs.gov.au/industry/listing/elements/pbac-meetings/psd/2022-07/files/review-of-discount-rate-psd-july-2022.pdf

Focussing on how health should be valued (including in different circumstances and across government areas of expenditure) and the frameworks that should be applied to determine this, would provide a clearer picture and guidance for the PBAC to apply in their decision making processes.

Health technology funding and purchasing approaches and managing uncertainty

4.1 Approaches to funding or purchasing new health technologies.

Recognising competition between new health technologies that deliver similar outcomes.

Viewpoint: Cannot support

As discussed in the streamlined submission section, Novartis Australia does not support any implied speed versus price trade off with the streamlined submission pathway that requires cost-minimisation submissions to offer or accept a lower price because of this pathway being chosen.

For technologies which provide no additional benefit of efficacy or safety, a cost-minimisation analysis provides a well-accepted approach to HTA evaluation and is currently used by the PBAC and MSAC and is widely applied overseas. Although the proposal suggests an improvement in the listing timeline by a few months, there is no reason why this acceleration could not be applied in the current context where the same price is offered. To request lower prices would have flow on effects to other sponsors which have listings in the same therapeutic area under the current 'price referencing' policy.

Of note is that the options paper suggests that cost-minimised interventions provide no incremental therapeutic advantage to the current comparator. Whilst this is technically accurate in respect of terminology, in several instances new therapies are cost minimized despite being superior to the current standard of care, as the evidentiary threshold to meet superiority is too high and to address that barrier would cause significant delays to access.

As an example, in the case of biologics for psoriasis the evidence shows that the IL-17s are safer and more effective that the older anti-TNFs when using the now accepted PASI 90 outcome rather than the older PASI 75. As a result, when priced the same as the older comparator the newer medicine is dominant in respect of cost effectiveness analysis. The added complication to the listing of newer technologies and expecting superiority is that an initially listed product is accepted in comparison to standard of care using placebo trials and unless the subsequent treatment has head-to-head trials with the first product, the PBAC determines there is uncertainty. As outlined in Section 3.2, there are outstanding questions regarding how the PBAC can become more comfortable with the uncertainly an indirect comparison brings. Given this, many sponsors currently utilise the cost-minimisation pathway to gain access and avoid unnecessary delays for patients, or the PBAC recommends a cost-effectiveness submission on a cost-minimisation basis. As result the Commonwealth is provided with an incremental improvement in efficacy at the same price as the current standard of care.

Implementation of an option requiring a price decrease would be damaging for patients, clinicians and the sustainability of medicines related industry in Australia. It would limit the number of therapeutic options available to patients and clinicians, either through less medicines being made available on the PBS, increasing the length of time to access through

more cost effectiveness submissions being made and/or medicines de-listing due to continual price erosion.

Pricing offer (PO) and negotiation guidance framework.

Viewpoint: More detail required

Novartis Australia is unsure as to how this step in the process would meet the objectives of the HTA review. Fundamentally the PBAC should set the value and the subsequent price of the medicine in question. It is not appropriate for there to be a further negotiation following this, which accounts for the comparative/incremental health benefit of the health technologies compared to existing available subsidised products, as well as overall budget impact implications. This would only further delay access to medicines and detrimentally impact any agreement with the PBAC as sponsors would be cognisant of further price reductions in the post recommendation process.

It is important that further detail and rationale is provided which supports changes to the current process. In its current form it is not possible to form a view without further detail.

Post-listing re-assessment of health technologies.

Viewpoint: Cannot support

There is currently no need for the post-listing reassessment of health technologies. The regular application of DUSC reviews in the post-listing environment is sufficient to determine the use of the drugs as per the agreed usage. To add in reassessment criteria would be a disincentive for investment.

Once listing has occurred, there is unlikely to be any further investment in randomised controlled trials which results in the information that would be available for a post-listing reassessment being of lower-level evidence. Given this level of evidence is not accepted and deemed uncertain by the PBAC for the listing of a medicine this raises the question as to how and why this level of evidence would be suitable for a reassessment of a health technology.

There are many pricing mechanisms, including statutory price cuts and price disclosure which help keep the costs of older medications at a level where investment in them is still warranted. To consider disinvestment based on post-listing reassessments, using potentially lower quality data, negates an important issue regarding the quality use of medicines regarding patients being moved from medications unnecessarily.

Approaches for managing uncertainty - revised guidance on the uses of different managed entry tools.

Viewpoint: More detail required

A clear and definitive plan for any bridging funding program must be articulated which also includes information about how therapies are identified and importantly what happens once the capped bridging funding ends. Information regarding who is responsible for the evidence gathering and requirements and the administration that enables evidence to be collected

and reviewed is not included in the current draft. The financial burden associated with these requirements may be such that bridging funding is financially unviable for therapies in small populations despite there being HUCN.

In addition, the status of any medicine within this group is important to establish and whether whilst within this fund new unlisted medicines can cost minimise to a medicine in the fund in to gain access to the PBS – thereby creating a new standard of care which the medicine within the fund would then need to be compared to upon exiting leaving the fund and seeking PBS listing. This would prevent an ability to adjust their value as their data matures. This is a critical issue as without resolution the use of managed entry tools will continue to have a lower level of uptake than expected.

Futureproofing Australia's systems and processes

5.1. Proactively addressing areas of unmet clinical need and gaps in funded access.

Viewpoint: Support with amendments

Amendments:

1. **Industry should be involved in the development of a priority list for HUCN.**
2. **Horizon scanning should be supplemented by input from industry.**
3. **Incentives for early reimbursement applications or for repurposing products need to be long term for sustainable listing.**

Novartis supports the development of a priority list for areas of HUCN. However, we believe that industry should be involved in the development and review of this list with all other stakeholders mentioned in the options paper (clinicians, patients and patient organisations, and community).

Novartis also supports the idea of proactive horizon scanning. Industry should play an active role in this process by presenting new and emerging clinical data to the horizon scanning body at specific timepoints. Novartis supports, in theory, the idea of early assessments and prioritisation for potentially promising therapies for areas of HUCN. These promising therapies in areas of HUCN are likely to be global priorities for the relevant Industry sponsor and, if identified early, will have immature and incomplete data packages. As such, the sponsor may be risk adverse when it comes to early access and reimbursement attempts, especially if there is an international risk to pricing in other larger jurisdictions. The third option on the list includes products which do not have market authorisation in Australia and where there is evidence that this could be repurposed. In these cases, while they may not have the same sensitivities as a high-profile new product, the costs of registration and reimbursement are significant for a patient population a company may have already decided is not viable. The data packages for these unregistered products identified for repurposing will also be small and immature. Much work has already been completed about the repurposing of products and comments made through the TGA consultation on this issue. In summary more detail is required on how repurposed medicines would be assessed for listing and how they would interact with the various interconnecting policies and affect other medicines both existing and within company pipelines.

Any "incentivisation" that comes with the invitation of a proactive reimbursement submission for these therapies must deal explicitly with how the new therapy will be valued by the HTA

system, both from a published and effective pricing perspective and by the payer from a budget impact perspective. The Review document describes some incentives such as provision of a case worker and cost-recovery fee exemptions but the costs of listing either new therapies early or repurposing other products would require different types of incentives. For example, potential incentives to encourage an early reimbursement submission could include a greater willingness to accept clinical and economic uncertainty when evaluating the therapy, complete confidential pricing for the period that the therapies clinical data is considered immature, or no budget expenditure caps. For products that are repurposed exemptions from price referencing, lowest cost comparator and impact on the other indications of the product would be required.

5.2. Horizon Scanning.

Viewpoint: Support with amendments

Amendments:

1. **Costs should not be cost-recovered.**

As stated, Novartis supports the idea of more formalised horizon scanning, including pathways to identify and find solutions for new therapies and technologies that do not suit the traditional HTA pathways. Novartis also supports the idea that industry play an active role in the horizon scanning process.

Novartis acknowledges that this process will require resources and therefore have associated costs. However, given the process will benefit all stakeholders across the health eco-system and participants in the horizon scanning process, cost recovery from sponsors would not be appropriate.

5.3. Environmental considerations in HTA.

Viewpoint: More detail required

Novartis supports environmental sustainability. As a company we are working towards our 2030 environmental sustainability strategy[2]. The Review document does not give details on how this is to be used in HTA and questions whether it is relevant for HTA. The options cover both a company's commitment to overall environmental impact as well as a product's individual impact. Novartis is cautious of environmental impact reporting being used in the regulatory, reimbursement or clinical decision making and would need to see further detail on these options. Novartis would also like to ensure the inclusion of this does not bias products may require nuclear technologies for development.

[2] https://www.novartis.com/esg/environmental-sustainability

5.4. Mechanisms for continuous review and improvement.

Viewpoint: Support with amendments

Amendments

1. **Improvements made with the input of industry and stakeholders.**

Novartis is generally supportive of a program of continuous review and improvement for current HTA policies and methods. However, it is important that any “improvements” made to the HTA policy and methods are agreed upon by relevant stakeholders, including Industry, as one stakeholder’s improvement is not necessarily aligned with all stakeholders' priorities. Additionally, the time expected to make improvements needs to be scheduled and limited.

5.5. Capacity and capability in the HTA systems.

Viewpoint: Cannot support

Novartis strongly believes that there needs to be an improvement in the HTA capacity and workforce in Australia. This includes an appropriately valued and resourced Department of Health and HTA evaluation teams. The paid internships with the Department and Evaluation groups will not address to the capacity and capability problem. There needs to be material investment in long-term capacity solutions for both the Department and the Evaluation groups. This investment should come from Government using the currently collected cost-recovery fees and further Government funded investment as warranted.

Novartis is supportive of the intent behind the option for HTA evaluation harmonization and international collaboration but has concern regarding how the differences in decision making policies and HTA guidelines will be overcome across jurisdictions without creating more work for each respective jurisdiction. Novartis is not supportive of international collaboration if the motivation is to “improve its (Australia’s) ability to negotiate in relation to purchasing of innovative health technologies” (Options paper p. 168) as this is counter to the principles of HTA in that the price (or purchasing) of the relevant health technology is determined by the health outcome it delivers. It also puts at risk price confidentiality across jurisdictions which is critical for companies to maintain.