SUBMISSION TO CONSULTATION 2 FOR THE HEALTH TECHNOLOGY ASSESSMENT POLICY AND METHODS REVIEW

FEBRUARY 2024

Contents

Our Credo

We believe our first responsibility is to the patients, doctors and nurses, to mothers and fathers and all others who use our products and services. In meeting their needs everything we do must be of high quality. We must constantly strive to provide value, reduce our costs and maintain reasonable prices. Customers' orders must be serviced promptly and accurately. Our business partners must have an opportunity to make a fair profit.

We are responsible to our employees who work with us throughout the world. We must provide an inclusive work environment where each person must be considered as an individual. We must respect their diversity and dignity and recognize their merit. They must have a sense of security, fulfillment and purpose in their jobs. Compensation must be fair and adequate and working conditions clean, orderly and safe. We must support the health and well-being of our employees and help them fulfill their family and other personal responsibilities. Employees must feel free to make suggestions and complaints. There must be equal opportunity for employment, development and advancement for those qualified. We must provide highly capable leaders and their actions must be just and ethical.

We are responsible to the communities in which we live and work and to the world community as well. We must help people be healthier by supporting better access and care in more places around the world. We must be good citizens – support good works and charities, better health and education, and bear our fair share of taxes. We must maintain in good order the property we are privileged to use, protecting the environment and natural resources.

Our final responsibility is to our stockholders. Business must make a sound profit. We must experiment with new ideas. Research must be carried on, innovative programs developed, investments made for the future and mistakes paid for. New equipment must be purchased, new facilities provided, and new products launched. Reserves must be created to provide for adverse times. When we operate according to these principles, the stockholders should realize a fair return.

Submission Information & Company Overview

Organisation: Janssen (Australia) Pty Ltd
Type of Organisation: Proprietary Limited Company
[redacted] [redacted]

Janssen-Cilag Pty Ltd (**Janssen**) is a subsidiary of Johnson & Johnson, the world's most comprehensive and broadly-based healthcare company. In Australia we provide products and services including medical devices, diagnostics, pharmaceuticals.

The Johnson & Johnson Family of Companies in Australia includes:

- Johnson & Johnson MedTech (Johnson & Johnson Medical Pty Ltd) – medical devices and related technology; and
- Janssen Australia (Janssen-Cilag Pty Ltd) – pharmaceuticals.

This submission has been made by Janssen Australia.

Section 1 Overall response and feedback to the HTA review options paper

Janssen-Cilag Pty Ltd (herein referred to as Janssen) welcomes the publication of the Health Technology Assessment (HTA) Policy and Methods Review Consultation Options paper (referred to as the HTA Options paper) and the opportunity for feedback on the Options proposed.

In the Commonwealth Government and Medicines Australia Strategic Agreement there was a commitment made by both parties for shared goals of (i) reducing time to access for Australian patients so that they can access new health technologies as early as possible, and (ii) 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. This was to be achieved by ensuring that our assessment processes keep pace with rapid advances in health technology and that barriers to access are minimised.

The vision of Medicines Australia for reform to our HTA system, is that Australia is a country where new medicines are launched early, and that all Australians should have access to the latest medical technologies within 60 days of TGA registration (Medicines Australia pre-budget submission). In line with the stated goals of the strategic agreement, and the Medicines Australia vision, Janssen strongly advocates that all stakeholders, including Government, support and work towards this ambitious and bold system reform goals.

In its submission to Consultation 1, Janssen outlined that both Value Assessment barriers and Process barriers are key reasons as to why access to new and innovative therapies in Australia is delayed when compared to other countries with comparable health systems. In particular, the key Value Assessment barriers that needs to be addressed to close the patient access gap between registration and reimbursement are:

1. Managing clinical, economic and financial uncertainty commonly takes a conservative approach,
2. Willingness to pay and value attributed to an Australian life is low and outdated,
3. Selection of the comparator using the least costly alternative, and
4. Decision making remit increasingly focussed on budget impact.

Janssen maintain that true reform of the HTA system to achieve the goals of Strategic Agreement requires that these barriers in the assessment and recognition of value of innovative medicines are addressed. Our submission to Consultation 1 provided a range of recommendations to overcome these barriers which we maintain would result in significant improvements in timeliness and equity of access to innovative therapies in Australia.

HTA Options paper addresses process but not value assessment barriers

The HTA Options paper poses a raft of possible options regarding HTA process, methods and policies. Janssen acknowledge that there are some positive steps for reform outlined in these options, such as options to:

- streamline HTA submission pathways,

- remove duplication of processes,
- bringing forward aspects of the current HTA process including timing of a submission,
- introducing a bridging fund for therapies of likely highly added therapeutic value (HATV) in areas of high unmet clinical need (HUCN),
- improving the ability for generation of strategic real-world evidence (RWE) to inform HTA,
- earlier, more transparent and more effective engagement of patients and clinician stakeholders in HTA processes and decision making, and
- improved First Nations People involvement in HTA.

Many of the positive Options relate to process changes and thereby intend to address process barriers in the current system. In considering these process changes, many process Options link to other process change options. As such, all process change components must be operationalised cohesively, rather than implementing some but not others selectively. Otherwise, the system risks becoming more fragmented and clunky, and results in changes being applied to some submissions but not all (for example, only the minority with HATV in HUCN see any benefits and the large majority do not).

Of concern to Janssen is that the HTA Options paper provides very limited commitment to addressing the Value Assessment barriers in HTA and as such many of the Options outlined will not result in improved value recognition of innovative therapies. This can be seen by the following:

- No significant change in how the comparator will be selected with ongoing reference to the current interpretation of Section 101(3B) of the *National Health Act 1953* (Cth) (**NHA**),
- No change in the long-term valuation of therapies either through the discount rate, or the way in which clinical or economic uncertainty is managed in the cost-effectiveness assessment,
- No change in the HTA systems willingness to pay, with ICER thresholds relatively low and remaining unchanged in decades, and
- No direct recognition of broader benefits of therapies (e.g., carer, indirect) beyond direct clinical benefits in the value assessment.

There are options which have the potential for improving value recognition. However, the details are not available, and the commitment only goes as far as further exploration and investigation of options. These include;

- Valuing overall; Conduct workshops to understand where it may be reasonable for HTA Committees to accept higher prices for health technologies, and
- Investigate further options to address budget impact implications of high cost/high impact health technologies.

Janssen note that these options have the potential for positive change, but they are not (yet) tangible actions that will have a direct positive impact on value assessment. Janssen consider that these HTA reform options can and should go beyond recommending further investigation to ensure that the value of innovative therapies is being better recognised as a result of the HTA review.

Janssen does not support options which seek further price reductions and cost savings

In addition, and of high concern to Janssen, some options proposed in the Options paper create barriers to value recognition and will have the effect of delaying and preventing patient access to new

and innovative therapies. **Janssen does not support the proposed options requiring or incentivising sponsors to offer lower prices for cost-minimisation submissions. It is not appropriate, and we do not accept any recommendations that seek price reductions or cost savings for new and innovative medicines.** Through multiple strategic agreements, industry has provided the Australian government with billions of dollars in savings through pricing policies targeting both F1 single-branded medicines (anniversary and catch-up price reductions) and F2 medicines (first new brand price reductions and price disclosure). Therefore, it is not reasonable that Options be proposed which seek further savings.

The rationale provided in the Options paper for this option is that in a non-subsidised market where products with similar profiles compete for market share, they would differentiate on price. The paper considers that in the Australian system, as they are funded at the same price the government is not gaining the benefits of such competition. Thus, this option is aimed at allowing the Australian government to realise some of this competition on price that would supposedly occur. However, as outlined in detail in Table 3 below, Janssen considers that this option is contrary to the intended goals of the HTA review for a person-centred HTA approach, and equitable and timely patient access to new and innovative therapies. This option undermines the goals of the Medicines Australia and Commonwealth Government strategic agreement for Australia to be a first launch country and will in fact reduce competition as fewer medicines will be reimbursed given fewer sponsors will agree to these terms of reimbursement. Furthermore, in some cases, sponsors are not able to accept price parity for these listings, so further mechanisms that lower prices in these circumstances, will not improve patient access, and is likely to limit patient access instead.

Janssen also point out that the underlying assumptions of the Options Paper in justifying the proposed options are not necessarily true. While some new health technologies will be considered by the PBAC as similar in effectiveness compared to current treatment options based on a technical framework of assessment, these medicines are commonly not considered as similar by clinicians and patients, who would benefit from their availability and having additional options. This option fails to acknowledge that new medicines can offer additional advantages over those considered as similar within a technical comparative effectiveness assessment framework. These advantages include different safety profiles, different mechanisms of action, different effects on quality of life, different effects on other efficacy outcomes which are not directly taken into account in their assessment of comparative effectiveness, and different forms of medicine delivery which can have convenience/treatment simplification benefits. All of these factors have important implications, and there is no one size fits all approach when selecting the right medicine, at the right time, for the right patient. Thus, Janssen consider that the underlying premise for this option outlined in the Options paper is not supported or justified.

Value assessment barriers must also be addressed for true reform

No single option is going to achieve the reforms necessary to reach the goals of the strategic agreement. Furthermore, improving the HTA processes alone will not achieve this. Both wholistic process improvement and value recognition are needed to achieve fast and equitable access to innovative therapeutics. The recognition of the value of therapies and speed of reimbursed patient access are inherently linked. However, the HTA options paper appears to treat these elements as though they are independent of one another. While the options paper highlights that the guidance provided for conducting and evaluating HTA reports in the PBAC Guidelines are predominantly on par

with most other international jurisdictions (with some exceptions), it is the acceptability and how the guidance are used by decision makers in the assessment of value and uncertainty that impact access.

It is noted that the HTA Review Options paper includes an option to develop an explicit value framework that captures domains beyond clinical and cost effectiveness, which has the potential to support improved value recognition. However, this is undermined by comments elsewhere in the Options paper which suggest that increasing the valuation of therapies would require a greater allocation of public resources to fund them and thereby reduce the net welfare gain to society and increase producer profit (Page 119 of the Options paper). In contrast to the position put forward by the Options paper, Jansen consider that recognising the total societal value of therapies is in fact critical to increasing the welfare gain to society. The recognition of the full societal value of health interventions will incentivise the biomedical innovation ecosystem to find solutions for the most pressing public health care challenges. As outlined in a recent paper which offers a detailed discussion on the relationship between incentives and innovation in healthcare and concludes: "It would be unwise to discourage the development of new solutions without first appreciating the cost of leaving the problems unsolved" (Ramagopalan 2024).

Ultimately, Janssen consider that options which support the appropriate recognition of value as well as process changes to speed up access are necessary and will work synergistically to achieve the desired reform.

Conclusion

The HTA review represents an opportunity for transformative reform of our HTA system, putting Australian patients at the centre of the process and delivering faster access to medicines. To achieve this, greater investment in innovative medicines (through the PBS, NHRA etc) is required by recognising the value of innovative therapies in a better way than is currently proposed in the Options paper. Janssen is not confident that the proposal in the current form will achieve the intended goals of the review.

Janssen seeks commitment from government in delivering a roadmap for true HTA reform and we look forward to working collaboratively with stakeholders in implementing HTA reform recommendations that will ensure reimbursed access to innovative therapies for Australian patients as early as possible and maintains Australia as a first launch country.

Section 2 Detailed responses to proposed options

The tables in this section provides more detailed specific feedback on the proposed options HTA review Options paper. The comments made on each option are based on the information provided in the Options paper, noting that many Options provided limited information. As such, the comments made by Janssen at this stage, may not reflect our position in the future when further work has been undertaken.

Janssen have also completed the online survey together with submitting this document.

The document provides detailed comments as follows:

- Section 2.1 - Health technology funding and assessment pathways
- Section 2.2 - Methods for HTA for Australian government subsidy

- Section 2.3 - Health technology funding and purchasing approaches and managing uncertainty
- Section 2.4 - Futureproofing Australia's systems and processes
- Section 2.5 - Key issues not addressed in the Options paper

Section 2.1 Health technology funding and assessment pathways

Table 1 Comments on proposed options for Health technology funding and assessment pathways

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Streamlining and aligning HTA pathways and advisory committees				
Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program [LSDP])	Supportive	Removing duplication in the LSDP pathway with a single HTA committee consideration (with expertise input) has the potential to improve timeliness of access to treatments which meet the criteria		Eligibility criteria for the LSDP should be reviewed and amended to include a broader range of treatments which treat rare diseases that cause severe disability, impact on quality of life, or have a lesser impact on mortality than the current definition of life-threatening.
Vaccine Pathway	Supportive	Streamlining the process for submissions and seeking expert advice has the potential to improve timeliness of access		Janssen understand that NIP procurement processes following a PBAC recommendation also adds to the time taken for vaccines access, and this does not appear to be addressed in this option. Janssen would support further considerations on reducing the timelines to list on the NIP following PBAC recommendation.
Expanding the role of PBAC	Supportive in principle	Streamlining and simplifying the process for HTA submission and consideration by a single committee (PBAC in short-term, single HTA committee in long-term) regardless of funding program is likely to be beneficial to reduce duplication, and delays in recommendations due to multiple committees making a decision (e.g., co-dependent submissions). This has the potential to improve timelines for access. However, this proposed option must also be accompanied by positive	An important factor in current decision making by the various HTA committees that exist is that there is consistency in membership and decision making. Under a unified HTA Committee, consistency will continue to be important and is more likely to be achieve with a defined group of members, rather than a fluid committee where membership changes depending on the intervention/funding pathway being considered. Under this model, it is important that the Committee has access to the appropriate experts who can provide advice to the unified HTA	Under the option for a single HTA committee, it is unclear from the information provided whether expanded PBAC or unified HTA Committee would be a legislated body (as is currently the case for the PBAC when recommending medicines for the PBS). Janssen note that other HTA bodies which recommend public funding are not legislated, nor have the same requirements as imposed on the PBAC by the National Health Act when considering medicines for the PBS. Any changes to legislation must be carefully considered as a result of this option and must be discussed with stakeholders in a transparent and consultative manner.
Unified HTA pathway for all health technologies Commonwealth funding	Supportive in principle			

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
		changes in the value assessment and management of risk in the assessment of value for this option to have the intended benefit of faster access to innovative therapies	Committee. The expert subgroups must also be consistent in membership and appropriately resourced as their input and advice is increasingly critical. Without appropriate advice, there is a risk in the Committee misunderstanding the technical specifics of a request, potentially delaying access.	Other practical considerations such as duration and frequency of meetings would need to be considered, and how this impact with the evaluation cycle given the number of applications considered by a broader PBAC or single HTA committee is likely to be substantial.
Proportionate appraisal pathways				
Triaging submissions	Supportive in principle	Under the proposed options to have a single HTA entry point and the potential for evaluation under one of a number of pathways, a triaging stage would be required to ensure the submissions are evaluated under the appropriate pathway. However, some concerns on implementation as noted in the next column	Further details on the triage process are needed. However, Janssen are concerned that the triaging phase is being performed prior to the full evaluation and therefore is potentially not fully informed of the submission context at the time of triage. This is particularly concerning given it appears that submissions are being assessed at triage as to whether they are addressing HUCN and are of likely HATV, as well as level of uncertainty and risk posed by the submission (factors which are complex considerations and require rigorous review and appraisal to determine). As a result there is high potential for subsequent mis-classification which could impede the appropriate submission evaluation and thus reduce efficiency in the system that this process is intended to create. Further, it is not clear what options sponsors have if they don't agree with the triage outcome.	Noting the triaging criteria be simple, any criteria for submission pathways and triaging of submissions must be transparent and available to stakeholders. They should be driven by the sponsor, and effectively enable the sponsor to know with high certainty, and predictability of the pathway it will follow. This will provide greater certainty for sponsors on process and milestones and help to avoid any unintended outcomes of increasing process unpredictability or inconsistency and reducing sponsor certainty.

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
			Janssen consider it is not appropriate for a triage step to be making judgement calls on HATV, HUCN or level of uncertainty in a submission, and thus propose that a more simple triage be taken which differentiates between the streamlined pathway for cost-minimisations and the rest which follow a complete evaluation path. Determination of HATV and HUCN for subsequent processes in the system could be later in the process following more rigorous evaluation, and is reasonable given the impact of this determination on the submission pathway is not seen until later (pending the early resolution option).	
Streamlined pathways for cost-minimisation submissions	Supportive of a streamlined pathway, but not supportive of any requirement or incentivisation of a lower price for cost-minimisations	The streamlined pathway will reduce resources required for submissions, for the sponsor, as well as the evaluation and appraisal committee. The pathway also has benefits of potentially speeding up time to listing provided it is not coupled with the requirements or incentivisation for a lower price.	This option will not achieve the goal of faster access to more therapeutic options when sponsors are required or incentivised for a lower price (see Table 3).	The criteria for the streamlined pathway should be simple and straightforward, based upon what is being requested by the sponsor. Submissions to the pathway should be voluntary by the sponsor (given there may be times where a cost-minimisation may need to go through a more complete evaluation and thus an alternative pathway). The criteria for the streamlined cost-minimisation pathway should be developed in consultation with stakeholders, including industry to ensure it is capturing all relevant "low-risk" submissions but allowing for more complete evaluation for those submissions requiring it.

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
				It is unclear from the Options paper the level of assessment that is done following submission to confirm that the medicine is non-inferior to the comparator. However, Janssen consider a check in the process (ie minimum level of assessment) is needed to ensure the pathway is being utilised appropriately. It is also unclear whom would do that assessment. From the Options paper, Janssen understands that the streamlined cost-minimisations would not be reviewed by the HTA committee but go straight to pricing negotiation. Janssen supports the proposal for information regarding the price of the comparator for the cost-minimisation in the streamlined pathway to be provided following confirmation of the medicine as non-inferior. Janssen notes that as this information would be provided to sponsors anyway in the post PBAC process, this proposal is effectively replacing this step, and moving the product to the listing process stage.
Early resolution mechanisms for submissions of major therapeutic advances in areas of HUCN	Supportive in principle of an optional accelerated issues resolution process for therapies that offer likely HATV in areas of HUCN. Of the options, option 4 is the most preferred –	Process changes to accelerate resolution of issues is positive, but as noted elsewhere positive changes in value assessment and management of risk in the assessment of value must occur for early resolution to achieve its intended benefit. Should this not happen, there is likely to be submissions that go down early	Janssen is concerned that placing a limit on resubmissions (one) could have the unintended consequence of no access (as opposed to delayed access), should no resolution be identified at the time but could eventuate later due to changing circumstances, additional data etc. Janssen is not supportive of a restriction on the number of	Janssen consider that revisions are required to the criteria for early resolution pathway. From the Options paper, Janssen interpret criteria b and c encourage sponsors to submit their HTA submissions earlier by bringing forward their TGA, as well as HTA submissions. Whilst Janssen acknowledge that in some cases, this may be sufficient to bring some sponsor HTA submissions earlier, this is certainly not always going to be the

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
	although concerns and unknowns remain	resolution paths for which resolution is not achieved.	resubmissions. It is noted that the sponsor and Department can meet to determine criteria for future submissions, however Janssen is concerned that this may still inappropriately limit the ability for sponsors to seek reimbursement following new situational developments. too restrictive. Janssen consider that to avoid unintended consequences of delay, the post HTA committee resolution process must be constructive. Further, irrespective of the changes made to the submission, under the early resolution approach, it would appear that should stakeholders align on the approach to address concerns this would go to the next HTA committee meeting rather than going through an extensive process of evaluation (i.e., review by evaluation group and ESC). As noted in the Triaging submissions option, Janssen is concerned that submissions are being classified for HATV and HUCN prior to full assessment and thus likely not taking a fully informed view. Under Option 4, Janssen consider this assessment could be made later in the process, potentially still at the point of the PBAC/HTA Committee (or in the few	case. These criteria do not account for a significant limiting factor in being able to submit an HTA dossier at the earliest possible time, which is data availability, and the time that it takes to include into an HTA submission which requires the development of economic and financial models. The criteria also do not account for the variation in regulatory pathways which impact on their evaluation and approval times, and thus will impact on the feasibility of meeting these criteria. Lastly, these criteria would suggest that the promise of early resolution would be sufficient to change the regulatory filing strategies of most global organisations, which may not be the case. As such, Janssen consider further consultation with industry is required on the criteria for any early resolution pathway. Janssen note that these criteria for early resolution do not mention submissions that are those undergoing evaluation through the TGA provisional or priority review pathways, therapies for which the TGA have already made an assessment of having significant (or potential significant) clinical benefit in an area of clinical need. These pathways could be considered in any alternative early resolution criteria.

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
			weeks preceding the Committee review)	
Expanding resolution step to all relevant cost-effectiveness submissions	As above			Consideration of expanding early resolution to all cost-effectiveness submissions should include a transparent and consultative review of the early resolution mechanism for therapies with HATV for HUCN including whether the process has achieved its intended outcomes
Development of a disease specific common model (reference case) for disease areas with high active product development	Not supportive (on the basis that this option is referring to economic models)	The rationale for this option and the issue this option is proposed to address is unclear. This option appears to be referring to a common model for cost-effectiveness, and Janssen does not consider that this option would improve time to access and is not necessary to improve decision making.	Significant practical challenges are likely with this option, given economic models require therapy-specific data, which may not be available. Additionally, model complexity can be high, likely requiring simplification of assumptions for the indication and the impact of a therapy being modelled. Such models can potentially be black boxes and impair sponsors ability to use and understand common models. All these aspects will have negative implications for value assessment and could delay reimbursed patient access.	
Decouple the requirements for the TGA Delegate's Overview to support PBAC advice	Supportive in principle	Janssen acknowledge the intent of this option is to encourage earlier HTA submissions, on the basis that this could result in earlier reimbursed access. However, as outlined in the early resolution pathway comments, there may be practical limitations and challenges – refer next column	Practical challenges to submitting HTA submissions at the time of TGA submission are likely, particularly if the TGA submission is made at a similar time to the first regulatory submissions worldwide. Issues may include availability of the pivotal trial data to develop HTA submissions, such as economic models to a sufficient robustness and quality for the HTA committee. Janssen have typically	It is unclear from the information provided how those submissions for which the TGA did not approve or approved with substantial modifications to the indication, dosing or other relevant parameter, would be managed by the HTA evaluation system. Further consideration of this possibility is required under this option. The practical challenges associated with this option must be considered if this decoupling of TGA Delegates Overview to PBAC option is

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
			submitted HTA dossiers at its earliest practical opportunity. In addition, if there is high uncertainty in the indication or dosing for TGA approval, any changes in these aspects will have significant flow-on consequences to the HTA submission, which could have been avoided or reduced if there was some time difference between TGA and HTA submission.	used as a basis for other decisions or assessments on eligibility of the submission for other parts of the HTA system. Sponsor should not be worse off because it wasn't feasible to submit any HTA dossier at the same time as the TGA submission.
Case manager	Supportive if option increases and improves communications during the process	This option is unlikely to improve timeliness of access as the HTA submission cycles have set deadlines, unlike the pricing pathways where case managers in Pathway A currently operate.		The appointment of a case manager for submissions could have the most benefit in facilitating communication and information transfer if it meant that sponsors were working with a known person whom could be reached by phone and email, rather than the current process through the Health Products Portal (whereby it is unknown who a sponsor may be communicating with). The case manager would be most useful if they know the submission details and can provide advice rather than a facilitator of information only. Janssen considers the level of interaction in the process with the Department needs to increase for this option to be useful.

Section 2.2 Methods for HTA for Australian Government subsidy

Table 2 Comments on proposed options for Methods for HTA for Australian Government subsidy

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Determination of the PICO				
Increased early stakeholder input on the PICO	Supportive	This option could improve the robustness and acceptability of HTA submissions as it aligns with patient and clinicians needs and expectations. Also has the potential help with value assessment if the medicine is aligned to these needs. Thus, there is potential for improvements in patient access		
Increased transparency of the PICO for stakeholders via plain language summaries on the PBAC agenda	Supportive	Potential to improve stakeholder input and understanding of HTA processes		
Guidance on the explicit consideration of equity and priority populations	Supportive	Potential to improve equity of access to therapies by making the requirements and decision-making parameters clearer to all stakeholders		
Clinical evaluation methods				
Overarching principles for adopting methods in Australian HTA	Supportive. The principles are consistent with the foundations for evidence-based medicine, and the hierarchy of evidence and help to provide greater clarity and transparency on decision making by HTA committees on the evidence.	Janssen considers that these principles are already adopted in Australian HTA, as noted in the options paper "the PBAC guidelines and MSAC guidelines instruct applicants to present the best available clinical evidence to support the effectiveness and safety of the proposed medicine and patient indication". Thus, this option is unlikely to impact directly on improving timeliness of access.		Janssen acknowledges accepting higher clinical uncertainty is warranted in HUCN areas. However, the definition of HUCN remains unclear. Janssen consider that higher clinical uncertainty should also be acceptable in other disease areas which may not be formally considered HUCN, but where higher levels of clinical evidence (i.e., RCTs) may not be feasible yet therapies may still provide substantial improvements in

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
				health. In these cases, it would be equitable to also accept greater levels of clinical uncertainty and more complex methods if such methods are appropriately justified.
Methods for the assessment of nonrandomised and observational evidence Methods for the assessment of surrogate endpoints Generate a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors Methods for assessing therapies that target biomarkers Guidance for assessing genomic and gene therapies	Supportive Improved guidance on clinical evaluation methods may improve HTA submissions by ensuring appropriate justification, and presentation of the evidence, is provided in the first submission for HTA decision making.	These recommendations may improve the analysis and presentation of clinical data in HTA submissions but are unlikely to have a significant impact on the timeliness of medicines access. This is because to achieve the intended goals of the review, the tolerability of HTA decision-makers to clinical uncertainty within the value assessment will need to increase. As such, the HTA system must become more accepting of more complex methods. Janssen note that the PBAC has previously demonstrated pragmatism in accepting less certain clinical methods (e.g., surrogate endpoints, ITCs) in certain cases where there is a high, unmet needs. Thus, given the proposed guidance and principles follow these lines, the availability of further guidance is of itself, unlikely to significantly improve timeliness.	Guidance should remain as guidance and not become prescriptive criteria or rules for the presentation of evidence. Maintaining a degree of flexibility is important to ensure that a wide range of situations are accounted for and sponsors are not held to unattainable thresholds of evidence acceptability that results in unintended consequences of delayed or no patient access to innovative therapies.	Janssen consider that higher clinical uncertainty, as well as more complex methods for determining comparative effectiveness and use of RWE should also be acceptable in other disease areas which may not be formally considered HUCN, but where higher levels of clinical evidence (i.e., RCTs) may not be feasible yet therapies may still provide substantial improvements in health. Collaborative consultation with stakeholders on the development of all guidance documents is essential to ensure the guidance is practical, suitable for use, and provides sufficient flexibility. When adopting new methods, training on the guidance material is important and should be rolled out upon implementation.
Develop an explicit qualitative value framework	Supportive in principle. Janssen considers that any value assessment framework must include a broad range of	There is limited detail to ascertain whether the option as proposed will improve timeliness of access. The value assessment framework must directly translate into improved value	Any framework that is developed should result in measurable differences in HTA decision making and value assessment. It would be	

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
	factors beyond clinical-effectiveness, cost-effectiveness and financial impact, and that these other factors have a material impact on the value recognition of the therapy being considered.	recognition of innovative therapies for this option to lead to faster patient access. However, Janssen appreciate that this option would provide transparency and additional context around decision making which are important aspects of HTA.	disappointing if the value framework became a mechanism to justify existing value assessments, and not result in more wholistic and broader recognitions of value.	
Economic evaluation				
Selection of the comparator	Not supportive as there is no significant change from current practice proposed in the option	Whilst Janssen notes the options paper's consideration to investigate situations where it may be appropriate to move away from the current approaches of applying Section 101(3B) of the National Health Act for comparators, overall, the proposed option will not achieve the intended outcomes of faster patient access and is contrary to the person-centred approach to HTA being sought. This is because, and as outlined in Janssen's submission to consultation 1, the existing practice of comparator selection values innovative medicines with incremental patient-relevant benefits (but where comparative effectiveness frameworks consider the medicines similar) at the same level of F2 (generic/biosimilar) medicines whose prices have eroded due to competition. This approach combined with reference pricing impacts all F1 medicines cost-minimised with each other leading to value erosion. This prices innovative medicines well below the price that PBAC had originally considered cost-effective for that indication. This in turn results in delayed medicines access, or no access at all. The	Without significant revision to the approach in applying Section 101(3B) of the National Health Act, there will be ongoing negative impacts to the value of new and innovative medicines that deliver patient-relevant incremental benefit, and thus impairing patient access.	Janssen supports the comparator recommendations in the Medicines Australia submission to consultation 2, and as outlined in our submission to consultation 1 (that the selection of comparator should revert back to the internationally accepted and best practice definition of a comparator for HTA and pricing assessments, that the comparator should be the treatment most likely to be replaced by the proposed medicine). This approach would more appropriately value medicines and improve equitable, and timely access.

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
		current approach disincentivises sponsors from seeking reimbursement of medicines in the future, and as such is contrary to the goals of the HTA review.		
Valuing of long-term benefits; discount rate	Not supportive as there is no significant change from current practice proposed in the option	This option as currently proposed will not achieve the intended outcome of the review given there is no change from the current discount rates proposed. It must also be noted that changing the discount rate alone will not fully address the system issues with valuing the long-term benefits of therapies. The discount rate is but one aspect on how longer-term benefits are valued and considered in HTA, with other elements including model time horizon, choice of extrapolations, requirement for convergence of benefits etc. Thus, the valuation of benefits overall needs to be addressed to achieve the intended outcome of the review (refer to Section 2.5 for additional comments on what is missing from the Options paper).		Janssen is supportive of reducing the discount rate and aligning the valuation of longer term costs and benefits to other international HTA countries. This position was previously noted in our response to the consultation on the discount rate in 2022 and has been acknowledged in the Options paper (*"The base case discount rates used in comparable countries ranged from 1.5% to 5% with the majority using less than 5%"*).
Valuing overall; Conduct workshops to understand where it may be reasonable for HTA Committees to accept higher prices for health technologies	Janssen support efforts to improve the value recognition of innovative therapies. However, the recommendation as proposed lacks any commitment for greater value recognition as a result of the HTA review.	As there is no commitment to increase the value recognition of therapies, it is difficult to determine whether the proposed option will achieve the intended outcomes of facilitating faster access.		Janssen consider that this HTA reform option can and should go beyond recommending further investigation to ensure that the value of innovative therapies is being better recognised as a result of the HTA review. If this options proceeds as presented, there must be a commitment to implementing the value recognition outcomes that come from it.

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
				Janssen consider that a number of issues outlined in its submission to consultation 1 have not been addressed in this options paper as it relates to economic evaluation and recognising the value of innovative therapies. This is discussed in Section 2.5.

Section 2.3 Health technology funding and purchasing approaches and managing uncertainty

Table 3 Comments on proposed options for Health technology funding and purchasing approaches and managing uncertainty

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Approaches to funding or purchasing new health technologies				
Recognising competition between new health technologies that deliver similar outcomes	Not supportive Janssen does not support any requirements for companies to offer a lower price, rather than pricing parity for new health technologies with similar efficacy and safety to treatment options available on the PBS. As noted in Table 1, Janssen support the release of competitors price earlier in the HTA process in the cost-minimisation streamlined pathway, noting that it occurs in the post-PBAC process anyway.	This option does not achieve the intended goals of the review that relate to a person-centred HTA approach, and equitable & timely patient access. This option undermines the goals of the Medicines Australia and Commonwealth Government strategic agreement for Australia to be a first launch country. The introduction of new medicines which are therapeutically non-inferior to existing medicines, should not be considered an opportunity to reduce the price, of the proposed product or of currently listed products on the PBS through flow-on pricing effects.	This option will preclude access to medicines that can improve a patient's health-related quality of life, lived experience and day-to-day activities. As such, it will in fact reduce competition as fewer medicines will agree to these terms for reimbursement. While some new health technologies will be considered by the PBAC as similar in effect over current treatment options based on a technical framework of assessment, these medicines are commonly not considered as similar by clinicians and patients, who would benefit from their availability and having additional options. This option fails to acknowledge that new medicines can offer additional advantages over those considered as similar within a technical comparative effectiveness assessment framework. These advantages include different safety profiles, different mechanisms of action, differential efficacy on other outcomes not formally considered by PBAC, different impacts on quality of life, and different forms of medicine delivery which can have convenience/treatment simplification benefits. All of these factors have important implications, and there is no one size fits all approach when selecting the right medicine,	Janssen remain supportive of price parity for medicines offering similar comparative effectiveness and safety. Under the current system and approach, there is no increase to the budget impact by allowing the same prices. It is important to recognise that the industry has already agreed to mechanisms that ensure the prices of medicines are appropriately managed and reduce over time, such as those related to statutory price reductions, first new brand reductions, and reference pricing.

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
			at the right time, for the right patient. Thus, this option contravenes the patient centric goal of this review. Furthermore, while other healthcare systems may implement similar frameworks as proposed in the option paper. It is important to recognise that these countries have broader access to medicines than we have in Australia. Janssen is aware of medicines recommended in Australia on a cost-minimisation basis that under the current system are unable to progress their PBS listing when pricing parity is requested. Thus, any mechanism that further undercuts value, will have adverse consequences in the context of the Australian system. There is a strong likelihood that companies will not be able to bring some medicines to the Australian market if this option is pursued.	
Investigate further options to address budget impact implications of high cost/high impact health technologies	Cautiously supportive of further investigation of options such as patient level product warranties, annuity/mortgage payments and subscription-style" bulk-funding programs to manage cost.	Janssen acknowledge the need to manage budget and consider that additional tools for this purpose would be useful to enable equitable, timely and affordable patient access. However, there is little detail in the options paper to assess whether this will result in improved patient access or greater speed to access.	Janssen consider that with any potential tool to manage budget impact, no stakeholder should be worse off, and in particular patients should not be required to incur additional out of pocket expenses.	It is noted that the current system has the ability to manage budget impact through mechanisms such as RSAs, and pay for performance (eg CAR-Ts). It is therefore important that any investigations also explore how these mechanisms can also be improved to achieve the goals of the review.
Pricing offer (PO) and negotiation	Support in principle the concept of speeding up the pricing negotiation process. However,	Has potential to minimise negotiation time and therefore minimise delays to	It is unclear how this option would be operationalised when as noted in the paper, price negotiations are implicitly included in	Additional information required about how this could be proposed/implemented in the

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
guidance framework	further detail is required to understand the full implications of the option. Janssen cannot support as described above any expectations or requirements for lower prices for health technologies recommended on a cost-minimisation approach.	listing following recommendation by PBAC/HTA Committee.	the HTA system (i.e. via the PBAC submission process due to legislated requirement to be considered cost-effective). As such, further information is required about how a prescriptive framework would be operationalised in the Australian system to be able to assess any potential challenges and unintended consequences.	Australian system, and how leveraged insights, or tools from other healthcare systems in this manner would deliver on the intended outcome of the review.
Post-listing reassessment of health technologies	Not required as the post-market review framework was recently updated and should be used as the basis for post-listing re-assessment of health technologies. Janssen acknowledge that as a result of earlier listings there may be some medicines that are found not to be cost-effective after they have been reimbursed for a period of time and further data has been collected. If an agreement between payer and sponsor cannot be made which allows the medicine to be cost-effective (i.e. through price reduction, RSA, change in listing), then it is noted that delisting/disinvestment may be the resulting course of action. Further work on this aspect of post-listing reassessment is needed		Under this option, there is potential that patients for whom the medicine is working lose a treatment option. No details are provided on what would happen to these patients who are responding to the medicine that is removed from the PBS.	Further work is required on the disinvestment/delisting of medicines aspect of this option, and in particular the patient impact. The goal should be that delisting following reassessment is a rare exception and that the benefit of earlier funded access outweighs this risk. Mechanisms should be in place whereby patients responding to such a therapy can remain on treatment at no additional cost.

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Approaches for managing uncertainty – bridging funding coverage for earlier access to therapies of likely HATV and HUCN	Supportive of further consideration and consultation of this option, noting that there is limited detail provided in the options paper	This option has the potential to improve timeliness of access to innovative therapies. To achieve its intended outcome, this option will require many other proposed options to be implemented, such as improvements in the approaches for managed access (below), inclusion and acceptability of RWE in HTA, and will also require positive changes in value assessment and management of risk in the assessment of value. Without all these other changes, the bridging therapy option will not operate as intended.	In acknowledgement that the proposed special funding program is capped, it is important that funding not be limited to a particular therapeutic area (eg cancer drugs) but should be open to all medicines of high clinical significance. See below on concerns around managed access programs which also apply to this option.	Janssen consider that revisions are required to the potential criteria proposed for the bridging funding eligibility. Janssen interpret the criteria around submission lodgement to encourage sponsors to submit their HTA submissions earlier by bringing forward their TGA, as well as HTA submissions. However, these criteria do not account for a significant limiting factor in being able to submit an HTA dossier at the earliest possible time, which is data availability, and the time that it takes to include into an HTA submission which requires the development of economic and financial models. The criteria also do not account for the variation in regulatory pathways which impact on their evaluation and approval times, and thus will impact on the feasibility of meeting these criteria. Lastly, these criteria would suggest that the promise of a bridging fund would be sufficient to change the regulatory filing strategies of most global organisations, which may not be the case. As such, Janssen consider further consultation with industry is required on these eligibility criteria. Janssen note that these criteria for bridging funding do not mention

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
				submissions that are those undergoing evaluation through the TGA provisional or priority review pathways, therapies for which the TGA have already made an assessment of having significant (or potential significant) clinical benefit in an area of clinical need. These pathways could be considered in any alternative criteria.
Approaches for managing uncertainty –revised guidance on the uses of different managed entry tools	Supportive	A review of the guidance and policy arrangement for managed access programs is useful and may improve timeliness of access. As outlined in our submission in Consultation 1, managed entry mechanisms already exist in the Australian system but they aren't commonly used by companies due to inherent difficulties in data collection post listing and financial risks associated with the potential to pay back with interest any difference in price over the period. In addition, the lower prices and value accepted for therapies for initial listing prior into entering into these arrangements further compounds the challenges. There would need to be substantial changes made to the policy and implementation of managed entry mechanisms to ensure earlier listing of medicines.	Without addressing the existing problems with the managed entry mechanisms in place that have resulted in minimal uptake by Sponsors there is a high risk that there will continue to be minimal uptake of these mechanisms. This in turn means that earlier patient access will not be achieved.	
Understanding the performance of health technologies in practice				

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Oversight: Establish a multi-stakeholder advisory group to optimise access and use of RWD in HTA.	Supportive			In line with recommendation 1.2, the proposed advisory group should include consumers and could also consider how PROMs and PREMs could be incorporated into the use of RWD in HTA in order to increase the voice of the patient.
Develop a strategic approach to increase confidence, awareness, and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTA	Supportive	Currently it is difficult to carry out studies using local real world data sources to support HTA submissions or carry out any productive health outcomes research for other purposes (e.g. academic, commercial, etc). Efforts to increase data standardisation across jurisdictions, sharing and access in a manner that is cohesive nationally will support improved access to therapies and quality use of medicines.		Privacy and data security are of the utmost importance when accessing real world data. Therefore, the strategic approach should seek to maintain the highest standard of quality and ethics through robust dialogue, transparency and collaboration between government, academics, providers, manufacturers and payers.
Data infrastructure	Supportive	As noted in the Options paper, an all-of-government approach to data infrastructure is needed to ensure optimal use of RWE to support patient access to innovative therapies		Janssen is supportive of plans proposed in the HTA RWE expert paper in order to develop a coherent data strategy that encompasses not only RWD for HTA purposes but to think how Australia can build a data infrastructure for the future that helps improve health system delivery and provide patient with the highest quality of care. Any acceleration of the proposed timelines in the aforementioned paper is also encouraged.
Methods development	Supportive of the establishment of a multi stakeholder approach to recommend suitable methods.			Janssen recommends that once the multi-stakeholder group has issued its recommendations that there is a

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
				process of familiarisation and training in the methods that include the submission evaluators. Janssen also recommends that the methods approach is kept relevant and updated with the most relevant technologies as needed.
Develop Guidance framework	Supportive	To ensure this option meets its intended outcome, it is recommended that the guidelines closely align with those published by other HTA agencies as NICE and ISPE/ISPOR[a]. Australia should utilise the work already done by well-established and highly credible agencies on this matter to expedite the guideline development. Further, using common guidelines and requirements across countries will increase the appropriate and consistent use of RWE as global companies can more easily replicate work done in other countries using local data.	Any bespoke requirement in Australian Guidance on RWE that differ to overseas agencies may be challenging to meet and thus may impair local sponsors in generating real world data for HTA submissions and thus impair patient access to innovative medicines	Janssen notes that the Options paper outlines a number of conditions (see section 3.2) for accepting RWE in determining treatment effectiveness. These conditions are unnecessarily prescriptive at this point. RWE can complement analysis and add important context to the evaluation of technologies in areas beyond those outlined in the paper. While Janssen agrees with the Options paper in that higher quality evidence should be preferred when available, the guidelines proposed in this recommendation should be the place to discuss in more depth the benefits, weaknesses and risks of different sources of RWD as well as the methods to evaluate RWE.
Collection of utilisation and outcome data for provisionally listed health technologies	Support in principle	The use of existing registries which meet the needs of stakeholders is an efficient and appropriate approach to RWD collection. However, to achieve the intended outcomes, and to fully implement this option, the data infrastructure, current guidelines and		Janssen considers this option to be the final output of implementing other RWD and managed access recommendation

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
		methodology expertise options must be addressed as per above. In addition, the revised guidance for managed access programs must also be enacted for this option to meet intended outcomes.		

[A] Wang, Shirley V et al. "HARmonized Protocol Template to Enhance Reproducibility of hypothesis evaluating real-world evidence studies on treatment effects: A good practices report of a joint ISPE/ISPOR task force." Pharmacoepidemiology and drug safety vol. 32,1 (2023): 44-55. doi:10.1002/pds.5507.

Section 2.4 Futureproofing Australia's systems and processes

Table 4 Comments on proposed options for Futureproofing Australia's systems and processes

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Proactively addressing areas of unmet clinical need and gaps in the PBS				
Development of a priority list Identifying therapies to meet the priority list Early assessment and prioritisation of potentially promising therapies Proactive submission invitation and incentivisation PICO scoping for prioritised submissions	Supportive in principle to all options proposed in the draft options paper to proactively address areas of HUCN and gaps in the PBS	Has the potential to improve equity of access noting the focus of the list on First Nations Peoples clinical need. Unclear whether it will speed up access to new therapies which may have been submitted by sponsors regardless of this option. In addition, ultimately, the success of this option is dependent on sponsors progressing identified therapies through to listing at appropriate value.	The presence of a priority list must not mean that other submissions are deprioritised. Where identified and prioritised medicines are re-purposed from existing indications there may be challenges in generating submissions for registration and reimbursement. The issues outlined in the Medicines Australia and Janssen response to the TGA consultation on re-purposing are relevant for consideration here. The proposed timeline of 4-6 weeks for sponsors to accept a submission offer is unlikely to be sufficient to fully assess implications and further consideration of appropriate and realistic timelines is needed. Janssen consider that such processes can take months to work through.	
Establishment of horizon scanning programs to address specific informational needs within HTA and the health system				
Horizon scanning for advanced therapies and other potentially disruptive technologies	Supportive of horizon scanning for purposes of achieving the goal of speeding up access to clinically	The key success factor for horizon scanning relates to whether it will trigger meaningful		Further consideration needed on who (which organisation) would conduct horizon scanning and

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Horizon scanning to meet priority areas Horizon scanning to help operational capacity planning for HTA and health systems	important therapies which includes horizon scanning options proposed in the Options paper.	positive action for identified products to submit for reimbursed and achieve a listing.		measuring the success of horizon on patient access and health system readiness. It is suggested that the costs of horizon scanning for advanced therapies be shared between partners including industry. The rationale for this is unclear but Janssen considers that the governance arrangements of horizon scanning be further developed before recommending approaches for funding the costs of the activity
Consideration of environmental impacts in HTA				
Environmental impact reporting	Supportive of promoting environmental sustainability in healthcare. However, Janssen questions whether incorporating this into HTA is the best approach and thus further detail and consultation is needed on this option	This option does not address the stated goals of the HTA review of improving timely and equitable access to innovative therapies.	There is insufficient detail provided on this option, however it must not come at the expense of impeding patient access to innovative therapies	Further consultation needed on this option including whether inclusion of environmental impact reporting in HTA is the best approach to supporting environmental sustainability. Whilst there is limited detail provided, Janssen notes that implementing this option appears to be inconsistent with the systems current approach to only considering the direct healthcare costs and benefits of a therapy in HTA (noting indirect costs and benefits can be included as supplementary).
Mechanisms for continuous review and improvement				

Recommendation	Janssen supportive?	Comments on whether it achieves the intended outcomes of the review	Potential challenges and/or unintended consequences	Further suggestions for changes to improve the proposed options
Program of continuous review and improvement	Support in principle the need for the system to remain up to date with current HTA methods and policies. However, further consideration on system monitoring of the impact of changes is needed, which this option doesn't appear to consider.	A mechanism for monitoring and measuring the success of the system changes stemming from the HTA review is critical to ensuring that the intended goals of the review are met. As such, agreed metrics/KPIs are needed to support this option		It remains unclear how progress on the implementation of options will be monitored, including the agreed metrics. Janssen consider that for this program of review to be most effective, it should consider where the changes may not be meeting pre-agreed metrics, investigate the root causes of the issue, and make changes in a transparent and consultative manner, thereby introducing more agility into the system.
Strengthen international partnerships and work-sharing				
Harmonisation of HTA evaluations Work sharing for individual submissions Collaboration with international jurisdictions to deliver sustainable access to health technologies	Not supportive	Given there is limited detail and significant risks introduced by work-sharing (including to other countries that are work sharing with Australia), it is unclear how these options would facilitate the goals of the HTA review of faster patient access. Janssen consider that value assessment is local and cannot be borrowed or translated across countries. As HTA assessment feeds into value assessment, the HTA assessment should be done locally and separately to other jurisdictions.	There are significant practical issues in this option given there are likely to be significant differences in timing of HTA submissions, and the clinical situations across countries in the work sharing. There is the potential for increased complexity in HTA and risks introduced from other HTA system that may not be relevant in Australia. As a matter of principle, changes stemming from the HTA review should be looking to reduce complexity rather than increase it, where possible.	

Section 2.5 Key issues not addressed by the Options paper

This section outlines a number of key issues that Janssen raised in its submission to Consultation 1 that we consider have not been addressed in the Options paper and are not discussed in the Tables above.

These issues predominantly relate to the economic evaluation and how innovative therapies are valued. The issues include:

- Valuing long-term benefits where this uncertainty – conservative approach to managing clinical, economic and financial uncertainty, and
- Willingness to pay and valuing an Australian life.

An additional issue not addressed in the Options paper is the challenges faced by combination medicines, and this is discussed in this section also.

Economic model – valuing long-term benefits

The Options paper states that "stakeholders considered that uncertainty about the costs and outcomes was treated too conservatively by advisory committees and government. This particularly impacted treatments that have benefits that accrue over a long period of time where uncertainty about costs and outcomes increases over time" (page 117). Janssen's submission to Consultation 1 included detailed feedback on how the current system undervalues innovative therapies through highly conservative approaches to valuation and managing uncertainty (refer to Section 2.2.1.1). The submission provided evidence and lived experience that relative to other HTA cost-effectiveness markets, the PBAC accepts shorter time horizons, more frequently requests convergence of longer-term benefits, and more frequently has a preference for applying multiple conversative assumptions in the presence of uncertainty.

The Options paper notes the collective feedback of stakeholders but makes no recommendations on improving the valuation of innovative therapies, and in particular how clinical and economic uncertainty is addressed in the economic evaluation, despite this being a key factor in the delays currently seen in reimbursed access. Without any improvement in the management of uncertainty and tolerance to risk in the economic evaluation, then there will be ongoing issues with value recognition. This will continue to result in delays in access and the process and other changes being outlined in the Options paper will not achieve the intended reform goals.

The best and only way to improve speed of access and ensure optimal patient access to the most innovative therapies is to appropriately value these therapies, and the approach to managing uncertainty in the economic model must change to become more balanced as outlined in the box below is a key part of this.

Valuing long-term benefits where there is uncertainty: Janssen recommend that there should be a more balanced sharing of risk between the sponsor and Government, and that a process and framework be developed to support this objective. Any framework should adopt the most likely or

plausible outcome to manage uncertainty rather than the most conservative. This can be achieved by:

- Enhancing the pre-submission and evaluation phase for HTA submissions whereby there is early discussion of identified uncertainties and how these could be managed. This dialogue is then continued through the submission evaluation phase through set opportunities for the sponsor, evaluation group and ESC to interact or meet with the intention of reducing misunderstandings and determining a most plausible base case assessment for the economic modelling and value assessment. There are Options within the paper that are consistent with this recommendation.
- Consistent with the burden of proof being on a sponsor when making claims on their medicine, the HTA evaluation and decision-making bodies (i.e., evaluators, ESC and PBAC/MSAC) should provide clear rationale and evidence to support their position on the suitability and plausibility of model parameters when discussing the plausible base case.
- The most plausible base case assessment for the economic modelling by implication must also include a plausible assessment and management of the clinical uncertainties identified in the submission, whether this be the result of the evidence using indirect comparisons, the data being immature, or other reasons.

Economic model – willingness to pay and valuing an Australian life

The Options paper does not comment on the PBAC's willingness to pay and accepted ICERs for therapies. However, Janssen's submission to Consultation 1 included detailed feedback on how the current system undervalues innovative therapies through a low and outdated willingness to pay for the value attributed to an Australian life. Janssen acknowledges that the PBAC does not have an explicit threshold in valuing a medicine or a person's life (i.e., PBAC do not have an explicit cost per QALY threshold to recommend a medicine). This lack of an explicit threshold in the willingness to pay is a positive feature of the Australian HTA system which should be retained and it is noted that the PBAC can and does at times recommend therapies with high ICERs. However, for the large majority of therapies, Janssen's Consultation 1 submission provided evidence and justification for the conclusions that the ICERs accepted by PBAC are lower than other areas of government and have not evolved over time, despite inflation and the increase in all other costs inputs that go into models.

Willingness to pay and valuing an Australia life: Janssen recommend that;

- The HTA system should increase its implicit willingness to pay and valuation of a life to be more in line with other Federal Government Departments, international recommendations and keep pace with inflation and Australia's increasing GDP per capita.
- The PBAC minutes provide a rationale for why a particular ICER is considered appropriate and how the less quantifiable factors have been accounted for in the cost-effectiveness assessment and HTA decision. This recommendation appears to have been included in the Options paper.
- The lack of an explicit threshold is positive system feature which should be retained.

Challenges faced by combination medicines

The Options paper does not address the issues currently faced by combination medicines and does not provide any Options that specifically help to address the delays in access to these types of therapies. As outlined in Janssen's Consultation 1 submission challenges in the funding of combination therapies in a cost-effectiveness framework occur when component medicines are supplied by different sponsors, and retain their patent protection (i.e., are in the F1 formulary). There are further challenges when one component is already reimbursed (e.g., as monotherapy or in another combination), and when each component is used until a particular outcome occurs (e.g., disease progression) and the addition of the new therapy to the existing medicine prolongs the use of the existing (or backbone) medicine (and thus increases its cost).

The issue stems from combination treatments being evaluated as single technologies but are composed of component treatments that are each priced independently. The existing therapy or "backbone treatment" is often funded as a monotherapy and was reimbursed at the acceptable cost per QALY. For the combination treatment, the add-on component, has a much-reduced opportunity to demonstrate value. For multi-sponsor combinations, there is no clear and legally compliant way for different manufacturers to determine appropriate combination prices. Therefore, manufacturers of the add-on therapy only have input to the price of their own products and cannot provide input or insights into the overall cost of the treatment combination.

The detailed challenges and complexities are provided in Janssen's Consultation 1 submission. However, the box below provides Janssen's proposed recommendations to help address the challenges faced by combination medicines, and which are critical to address.

Recommendations for combination medicines HTA:
Janssen recommend the following is needed to optimise the assessment and funding of combination therapies in Australia to ensure patients access the benefits of these therapies as quickly as possible;

- Development of clear guidance and a legally compliant, transparent, and independently facilitated process that enables sponsors to engage on the pricing of medicines which are components of a combination.
    - This may identify the need for an independent third party to facilitate any engagement.
- Any process to allow engagement would need to start prior to a submission and continue throughout given the component pricing is not only impacted by the attribution of value, but also the total value considered cost-effective by the HTA decision maker.
- To aid any compliant engagement on the attribution of value of the components in the combination, the development of guidance or a framework that supports sponsors to compliantly engage with respect to pricing would be beneficial.

References

Ramagopalan S.V., Diaz J., Mitchell G., Garrison Jr L.P, Kolchinsky P. Is the price right? Paying for value today to get more value tomorrow. BMC Medicine (2024), 22;45