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Submitted to Health Technology Assessment Policy and Methods Review – Consultation 1
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Introduction

Privacy information

I understand that my personal information will be used in accordance with the 'How will your input be used' and 'Privacy information' sections above:
Yes

What is your name?

First name:
Matilda

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What is your organisation?

Represented Organisation 1:
Australian Genomics

What is the type of organisation?

Research

Please specify:

Are you making a submission on behalf of an organisation?

Making a submission on behalf of an organisation

What topic area/s does your submission relate to?

Topic area 1:
All topics areas in the survey

Topic area 2:

Topic area 3:

Declaration of interests

No conflicts

Details of declarable interests:

In response to professional conflict 2: Australian Genomics does discuss outcomes of MSAC (and other HTA) applications with our professional network.

Elements and features that are working effectively

Are there any elements and features of HTA policy and methods in Australia that are working effectively?

Yes - there are elements or features that are working effectively and should not change.:
Yes

Are you able to provide detail of any elements and features of HTA policy and methods that are working effectively? Please use specific details where possible.:

• The process of HTA review, key dates, and advice to applicants is easily accessible on the Government website. This is particularly useful if applicants already know that MSAC/PBAC are the appropriate channels for public funding of a particular intervention.

• Secretariat functions are clear and communicative, and there is opportunity to engage with health technology resourcing team members at post-MSAC sub-/committee meetings.

• The review of economic evidence is effective and there is good transparency in outcomes, with reports developed by different review committees becoming publicly available in a timely way.

• Members of the health technology resourcing team available to provide guidance to applicants are experienced, and knowledgeable, and greatly facilitate the application process. This was experienced both through MSAC application 1637, and particularly for MSAC application 1476 – which was the first 'true' genomic testing application that went through MSAC review pathway. Consideration of how to fit the 'square peg' of phenotype driven WES into the 'round hole' of the 'star performer gene' model was collaborative and iterative.

Are you able to provide details of positive outcomes resulting from Australia's HTA policies and methods? Please use specific examples where possible.:

• The formal inclusion of the "value of knowing" as a clinical claim in applications for new medical services is a step forward. HTA processes should look to incorporate more patient and person experience measures in their assessments. (See also question about detraction from person-centredness).

Current or future barriers to earliest possible access

Elements and features of HTA policy and methods in Australia acting as a current or future barrier to earliest possible access.

details of any elements and/or features acting as a current barrier to earliest possible access :

• The COVID-19 pandemic has made us all aware of how quickly new medical services and medicines can be approved. This has served to highlight the lack of agility and lengthy timelines associated with usual review pathways including HTA processes.

• Lengthy timelines are more pronounced in cases where the proposed intervention does not fit neatly into a clearly defined funding application pathway. Potentially highly impactful health interventions can become stranded, and / or go through repeated reapplication without a clear outcome or recommendation. A recent example of this is the application for rapid whole genome sequence for critically ill children and their families (also known as Acute Care Genomics). Although MBS/Medicare was known not to be the appropriate funding pathway for this inpatient service, an MSAC application was advised, so that the HTA could be enabled. The eventual recommendation was to pursue IHACPA funding. However, without a readily identifiable process, and little engagement with the applicants, the case for funding lacked momentum and progress. Most recently, enquiries have suggested that the service may not be considered by IHACPA as suitable for standalone code development. This drawn-out process has occurred in an environment where more than 300 Australian children could benefit from access to this medical service per year, which has profound implications for clinical management, and demonstrated cost-effectiveness. In the interim, bridge funding has been put forward in some jurisdictions but not others - leading to a situation of considerable inequity (see response to question about equitable access).

• One factor contributing to lengthy timelines in the MSAC process is the pathway of review by different committees. There is a concern among stakeholders that the different committees do not harmonise with the advice of each other (e.g., reflected in recent PSD and committee reports of MSAC application 1675). Further, decisions made by committees earlier in the review process (such as PASC) don't seem to be able to be revisited when new evidence arises or expert stakeholders are engaged at later stages. This is also a problem when applications take a long time to work their way through the pipeline (9 months or more), especially for those related to rapidly changing interventions such as genomics and other novel technologies.

• Stakeholders with experience with MSAC system find it rigid and inflexible, and question whether evidence and advice provided by subject matter experts engaged in the process is taken on board. In addition, there seems to be a lack of motivation to refine the details related to the item number inclusion criteria, reimbursement value, item descriptor and practice notes. There is little opportunity for knowledge exchange and shared decision making between the applicant, MSAC and advisory groups. For example, if stakeholders are invited to call into a committee meeting but only one spokesperson is allowed to participate, it does not foster a collaborative approach. Stakeholders involved in the process reflect that if they had all been brought together a more meaningful discussion, and potentially a better outcome, would have been achieved, resulting in more usable MBS item numbers.

• Post-approval, there is a political element to when funding commencement is announced. Recommendations by MSAC/PBAC are independent of the implementation challenges then faced by Government. This, for example, led to a long delay in the intended rollout of funding for the reproductive carrier screening test known as the triple screen (for Fragile X, SMA and CF).

• There is no mechanism for formal acknowledgement of experts who have supported HTA applications or assessments. This may have represented a considerable commitment over a period, and without recognition there may be little incentive to participate again.

• Stakeholders report that a particular barrier to early access is where co-dependent applications are reviewed in parallel but separately by MSAC/PBAC.

• Aspects of HTA legislation will have limiting implications for:
o Workforce capacity (for example, Genetic Counsellors being unable to request MBS-subsidised carrier screening as they're not eligible 'treating practitioners' under the Health Insurance Act 1973)
o Definition of 'patient' (for example, Genomic Autopsy could not be an MBS-subsidised intervention because stillborn babies aren't a 'patient' according to the HIA 1973)

• Incremental cost effectiveness ratios / cost minimisation approaches which only incorporate health costs underestimates the impact of an intervention. For example, the financial impact of the evaluations associated with Expanded Reproductive Carrier Testing are underestimated, because this intervention identifies carriers at risk of having a child with a severe, often life-limiting condition. The appalling severity of these conditions – often causing death in childhood – means the health costs associated with these children is not great. However, this underestimates the true value of this knowledge to

families.

• One lesson from the past is that the earliest genomic applications were instructed to use the clinical utility card model, which had been developed for cancer genetic tests. This was meant to streamline applications, but instead made the application process harder.

• Assigning the wrong value to reimbursement for MBS items numbers has had post-approval implications, whereby laboratory services consider it unfeasible to offer a particular test. This leads to broad ranging issues, from lack of competition in the market through to inequity of access for patients.

Details of any elements and features of HTA policy and methods in Australia that you think will act as a future barrier to earliest possible access? :

• The lack of expertise of committees to assess genomic or other new technologies will become an increasing barrier. Recommendation 29 of the New Frontier report discusses the inflexibility of HTA and how they are not fit for purpose for new technologies, drugs, and other precision medicine approaches. Further, those content experts who are part of committees must step out of deliberations if they have a conflict of interest, meaning the opportunity for expert guidance is missed.

• The boundaries dictating the types of medical services that MSAC can and cannot review will become an increasing barrier to new medical services. As an example, population genetic screening programs being trialled in research are showing great promise, feasibility, and acceptability amongst consumers, however, it is not clear how HTA is done for screening programs which makes the process difficult to navigate.

• The lack of a national register of clinical trials associated data (Recommendation 23 of New Frontier) and rare and genetic disorder registries will hinder the development and collection of evidence required for novel therapies. This will in turn slow the gathering of evidence for HTA. It is noted that high quality registries are also considered a requirement for making Australia a premier clinical trials destination (Recommendation 24 of New Frontier).

• Real world evidence in HTA is accepted in Australia but the extent to which it is considered/applied is variable. This is due to lack of guidance as to use of real-world evidence, and lack of longitudinal outcome data (either through the absence of infrastructure to capture it, or control of the data by sponsors).

• Genomics generates broader societal implications and impacts beyond the individual's health. Carer impacts and impacts on other sectors are now well documented and a consistent framework to account for these in HTA processes needs to be introduced. Currently, HTA processes prioritise impacts on healthcare systems and the health of the individual which is a narrow view of the real economic impacts of genomics.

• There is no unified framework for HTA. There is increasing link between what is evaluated by MSAC and PBAC. The cost-effectiveness of testing may be dependent on availability or price of gene therapy. Changes in one affect the other and HTA processes are static and so this wouldn't be accounted for.

Details of feasible options or suggestions you have to improve elements of HTA policy and methods that are acting as a current or future barrier to earliest possible access.:

• HTA pathways should more readily accept evidence generated overseas. This would be advantageous for genomic testing given the fast pace of change in the field. Stakeholders describe the need for a mechanism that can assess new clinical indications more rapidly, without the need to present repetitive evidence which applies across a range of rare diseases. This aligns with Recommendation 16 of New Frontier - to form and international HTA consortium, and the National Medicines Policy, which outlines an approach to "pursuing opportunities to collaborate with recognised international regulatory agencies to facilitate a standardised approach, reduce unnecessary duplication and facilitate earlier access to medicines for all Australians".

• HTA decisions can be made under conditions of high uncertainty and at a single point in time. Significant inefficiencies arise from this. Evidence comes from small cohorts and short-term evidence. HTA processes instead need to rely on real world evidence and have mechanisms for flexibility so that they can be updated after implementation to optimise the equity and efficiency of the system. There is increasing literature on life-cycle assessments and living HTAs.

• There is risk aversion in the decision-making process. There should be more risk sharing agreements for early treatments that do not have sufficient or are developing evidence. More "reviews" will be required, and price negotiations as real-world evidence is developed. Waiting for large multinational clinical trials to complete should no longer be the approach used, and real-world evidence should be used. Access to medicines based on phase 2 trials with an implementation and reporting plan (along with patient information on risks) will enable medicines to get to market sooner.

• The purpose and regularity of auditing of tests (the Continuous Review Committee for MBS item numbers) should be reviewed. This should be an opportunity to seek stakeholder feedback on what is and is not working, and whether item numbers need to be modified. Rather than viewing the underusage of item number (compared to that predicted) as a cost saving, it should trigger an investigation into why the item number has not been used. This activity would be another avenue to reveal where HTA policy and methods could be improved.

• MBS item numbers should be amended, where possible, rather than adding new ones. The mechanism also needs to allow for changes to existing item numbers, e.g., as the technology evolves and potentially as costs fall, but also as eligibility criteria become more refined. This will also reduce the complexity in determining the right test to order, especially as ordering tests is expanded to a wider range of medical professionals (for example, non-genetics specialists ordering genomic tests).

• Medical Research Future Fund (and other translational research funders) should co-design projects with researchers at the post award stage, so the right evidence collection and evaluations are being done from the outset. This will serve to avoid wastage of valuable research dollars and to accelerate the translation of high value medical services, new technologies and therapies.

• HTA committees could develop a more forward-looking approach, for example by developing a roadmap of new medical services and medicines that will be critical to improving health outcomes for Australians. The roadmap could be used to inform research priorities (related to the point above) and provide a basis to seek MSAC/PBAC applications led by relevant, expert organisations.

• The submitting organisation and government department (and/or evaluation group) could work together more collaboratively to develop submissions. This creates efficiencies and allows early identification of issues that will halt the submission (e.g., lack of evidence, wrong comparator/not listed or relevant for the country of submission).

• Some applications submitted to for MSAC/PBAC do not necessarily fit the current guidelines and are less easily assessed. The Acute Care Genomics application (see also question about barriers to earliest access) has passed through various review bodies and forums despite overwhelming evidence of clinical utility and cost effectiveness. In these cases, there should be greater efforts to pull together different parts of the health funding sector to tackle the big issues.

• The New Frontier report has several recommendations not already mentioned that we support:
o Recommendation 1: to establish a Centre for Precision Medicine and Rare Diseases to enhance timely access to novel drugs and technologies, and to provide advice on HTA pathways for new technologies.
o Recommendation 3: to establish an Office of Clinical Evaluation so that HTA is based on the most up to data global health practices.
o Recommendation 5: to increase the number of health economist in Australia.

Current or future barriers to equitable access

Elements and features of HTA policy and methods in Australia that are acting as a current or future barrier to equitable access.

elements and features of HTA policy and methods that are acting as a current or future barrier to equitable access:

• The pace of regulatory and HTA processes, research and clinical trials means that families who are in the privileged position to do so are seeking treatments for rare conditions and cancers that are only available overseas. There are many examples of families who have decided the benefits to outweigh the risks and have been in a financial position to take steps to received overseas treatment. This sets up a situation of considerable inequity – where only the wealthy can access life changing treatments.

• Further, there have been examples where places on clinical trials for high-cost therapies have been decided via a lottery system – which would be a devastating situation for the families waiting for treatment availability for their children or other family members.

• There are implications of interventions for Aboriginal and Torres Strait Islander peoples, and other culturally and ethnically diverse groups. There is not a clear pathway for researching, evaluating, and resolving social and cultural implications of an intervention in underrepresented groups, including Indigenous Communities. Community engagement processes to appropriately understand these implications and ensure these groups can benefit from advanced health interventions, are absent or inaccessible to most applicants. For example, these issues were raised in the PSD for MSAC application 1637 but determined that these can be addressed by Departmental Policy areas through public and/or consumer consultation. The process, resourcing, timelines, and publication of such consultations are yet to be determined.

• Medical services and therapies related to conditions that predominantly affect populations typically underrepresented in research are also likely to be underrepresented in HTA applications.

• There is no support with post-MSAC approval education of medical professionals, which likely means that some professionals are not accessing tests that would be beneficial for their patients because they are unaware of their availability or significance to their patient. Further, MSAC's approaches to which medical professional can order tests; the rebate amount, and for genomic tests whether there is gene list, or no gene list are all areas where inequity could be introduced, in terms of patient access, timeliness of result and results reported.

• Medical services based on whole exome or genome sequencing have been approved without proper quality assurance in place for the end-to-end diagnostic process, meaning that potentially there are differences in the way labs across Australia report on tests ordered using the same MBS item number. Australian Genomics and RCPAQAP are currently collaborating on a project to address this issue.

• The MBS and Activity Based Funding of services do not operate in isolation of each other, and services will increasingly weigh up the best source of funding/reimbursement. It will be important to ensure the true cost of services/interventions to state and territory health departments and the federal government are captured to ensure equitable implementation across services/jurisdictions that access different funding sources and to reduce gaming and cost-shifting (or facilitate appropriate shifting of costs).

• The downstream implications of an approved health intervention may introduce inequity. That is, an MSAC subsidised test might itself be accessible equitably, but if the knowledge gained by that intervention is costly, the equity is not only compromised, but you're providing people with information they cannot do anything about. For example, the new triple screen test for SMA, Fragile X and CF can inform couples if they are at increased chance of having a child affected by one of these conditions. The conditions could be avoided by accessing Preimplantation Genetic Testing (PGT) or IVF. However, while PGT/IVF is subsidised through the MBS, the out-of-pocket costs of IVF (~$5K per cycle) is beyond the means of many Australians.

• There are often restrictions on medicines being listing for use for a specific condition, but not for other conditions where the drug is known to be beneficial/effective/cost-effective. The issue is the pharmaceutical company might not make a submission due to low numbers of patients/market and/or a lack of evidence against the comparator (often this occurs because the comparator used in a clinical trial might not be listed in Australia). Thus, some people miss out on getting access to a drug for an off-label condition.

details of feasible options / suggestions to improve elements of HTA policy and methods that are acting as a current or future barrier to equitable access? :

• New Frontier Recommendation 2 is to simplify the process and establish a national genomic testing program for equitable access, which we support.

• The HTA process should consider the introduction of a standard step for review by an Aboriginal and Torres Strait Islander Advisory Group for HTA; identification by Government of communities with unmet need for a specific intervention and facilitate processes for expedited application to provide access if inequity is identified.

• Continued support of national, coordinated approaches to the implementation of genomic interventions will be required to support consumer safety, workforce education and capacity, and ensure policy and practice are adequate for new interventions.

Elements and features that detract from person centredness

Elements and features of HTA policy and methods in Australia that may be detracting from person-centeredness.

detracting from person-centeredness? :

• The consumer and clinician consumer voices are not engaged early enough in process. They should be engaged at all stages of HTA processes. There should be:
o Feedback mechanisms to capture the patient/consumer voice pre submission, during evaluation and post-funding decision
o Process should be equitable, so the input people from underrepresented communities is captured
o Development of validated tools to ensure consumer voice is considered credible assessment evidence

• The details of MBS items numbers are not always consistent with best practice and may affect the person-centred design of the medical service. Examples include the inclusion of cardiac genes which have disputed/refuted relationships with disease and providing funding for carrier testing of partners where chance of affected child is exceedingly low. MBS items that are not delivering best practice put the person at risk of non-diagnosis, over-diagnosis, or receiving information that is not useful to their health care or related to the condition for which they are seeking health care.

• Decisions are usually made based on “the average”. This is reasonable for policy, but there are always some who benefit more and some who have few (or negative) benefits. “Responder” analyses should be more widely used rather than a “mean efficacy” approach.

• The exclusion of non-health costs in economic modelling (such as education, NDIS, other support) means that HTA does not represent the true impact of a person’s condition, which in turn means HTA analyses are incomplete. This would be frustrating for families who want their lived experience to be communicated holistically, especially where it supports the case for health system funded services and treatments.

• As noted above, the limitation of current HTA evaluations to health costs does not permit the incorporation of real-world evidence, that is, evidence beyond just comparative health gain; which has implications for comparative cost effectiveness; financial implications for PBS etc. An example is Genomic Autopsy. The heath system utilisation of a couple who have had multiple unexplained miscarriages is not significant, however the impact on their wellbeing, mental health and productivity is significant.

options / suggestions to improve elements that are detracting from person-centeredness:

• New Frontier Recommendation 28 is to integrate the patient voice upfront in HTA and to consider making patient evidence compulsory in some circumstances. This is a model we support.

• New Frontier Recommendation 6 is to increase education about the regulation and reimbursement system. This would empower consumers to become more involved in HTA processes and have their voice heard, as well as to help individuals maximise the services and medicines they are entitled to through the health system.

• Stakeholders have expressed the need to bring all stakeholders together for the HTA conversation. This is considered a much more productive strategy than the sequentially seeking input from different groups.

• Exploring the incorporation of more assessment measures like the value of knowing. The cost-effectiveness of high-cost therapies is likely to be weak, at least in the beginning, and so incorporating other measures to demonstrate value will be critical to their implementation. An exploration of societal expectations for high-cost therapies may be insightful.

Perverse incentives

Elements or features of HTA policy and methods in Australia that are causing or could cause unintended consequence or perverse incentives.

provide details of elements of features of HTA policy and methods that are causing or could cause unintended consequence or perverse incentives:

• Lack of specificity in MBS item number descriptors could lead to the ordering of tests for groups with similar phenotypes that the test was not initially intended for. There has also been an example where an approved drug is so effective in some cases that health care professionals are trying to fit their patients into the criteria in case it has some positive benefits. This leads to a potential for misuse and over prescribing.

• Depending on the stakeholders consulted, it can be hard to maintain impartiality e.g., for advice on appropriate costing of a medical service.

• Limited consultation on applications can result in low service provision, despite MBS subsidisation – compromising the supply/demand curve. Currently only two Australian laboratories are offering testing for genetic cardiomyopathies and arrhythmias (items 73392, 73416 and related item numbers, available since mid 2022). It is likely that a major factor is that this is complex work that is difficult to set up and deliver, and the uncertainty about volume of testing contributes to this being an unattractive proposition for a laboratory that is not already working in this area – particularly with potentially problematic item wording (requesting copy number variant analysis off an exome backbone is not technically feasible for most labs) and the listed fee is

less than most labs would commercially undertake for this test.

• International pathology companies performing MBS-subsidised testing when components of the test provision are undertaken overseas. This will compromise Australia's self-sovereign capability in genomic interventions, undermine workforce competencies and local infrastructural investment. For example -https://www.invitae.com/en/providers/test-catalog/medicare-australia?tab=cardiology

• The "first to market" for a medicine can obtain a protected price premium, which is acceptable only for a limited time. An analysis of the costs of the premium would be useful to undertake along with the premium of second to market etc. Whether the 85% reimbursement value for the second drug to market is reasonable is not known.

feasible options / suggestions to improve elements of HTA policy and methods that are creating unintended outcomes or perverse incentives either currently or in the future:

• As previously discussed, consultation and careful consideration of inclusion criteria (with quantitative elements where possible) would reduce the incidence of misuse of MBS item numbers, as would refining MBS item numbers over time as they are used in a cycle of continuous evaluation.

• The incorporation of real-world evidence of uptake, costs associated with the service or medicine, cost offsets, workforce implications etc. would serve to better identify and act upon perverse incentives if they arise.

Areas for further investigation or analysis

Noting the overall scope of the analysis from the HTA expert will be in line with the ToR and agreed by the Reference Committee, are there any HTA or reimbursement models, or elements thereof, utilised in other countries that you believe should be considered for potential adoption in Australia, or that it would be good for the Reference Committee to understand?

Country / Jurisdiction::
AUSTRALIA

Details of: Which elements of the HTA policy, method, mechanism for suggested for consideration; Any outcomes that the suggestion is achieving that should be considered; Any unintended consequences that the suggestion is having or may have if adapted in Australia:

• We seek clarification on the suggestion that there is a limitation on the use of data from MBS-funded interventions for research purposes. MBS and other administrative data are an important source for research and evaluation and limitations on its use would impact planned research including MRFF supported research.

Country / Jurisdiction::
UNITED KINGDOM

Details of: Which elements of the HTA policy, method, mechanism for suggested for consideration; Any outcomes that the suggestion is achieving that should be considered; Any unintended consequences that the suggestion is having or may have if adapted in Australia:

• The UK's National Health Service process for adding new tests to the national genomic test directory appears to be an efficient, high-throughput process that engages a panel of experts to consider new genomic tests. Elements of this process should be investigated for consideration for adoption in Australia.

Country / Jurisdiction::

Details of: Which elements of the HTA policy, method, mechanism for suggested for consideration; Any outcomes that the suggestion is achieving that should be considered; Any unintended consequences that the suggestion is having or may have if adapted in Australia:

Other details of importance to the HTA Policy and Methods Review not covered above + document / attachment upload point.

Noting the objectives of the review set out in the Terms of Reference, is there any other information relevant to the Review not provided above that you would like to add?

Noting the objectives of the review set out in the Terms of Reference, is there any other information relevant to the Review not provided above that you would like to add?:

• There has been a perception amongst stakeholders that the HTA review is focussed on medicines and the PBS rather than MBS and other pathways and services, partially due to the wording of sections of the Terms of Reference. We believe that medical services are, and should be, firmly in scope of this review, according to the ToR (e.g., sections 4.1 and 4.2). Our submission is focussed on medical services and new technologies. We hope that our submission will be considered with high relevance and that others have not been discouraged from making submissions addressing similar issues.

Would you like to upload any attachments/supporting evidence to your submission?:
No file uploaded