Submission to the Health Technology Assessment Policy and Methods Review – Consultation 2 options paper

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The Neurological Alliance Australia *is an alliance of 21 not-for-profit peak or national patient organisations representing adults and children living with progressive neurological or neuromuscular diseases or neurological disorders in Australia. The Alliance was established to promote improved quality of life for people living with these conditions and increased funding to support research. Members of the Alliance are: the Brain Foundation, Brain Injury Australia, the Childhood Dementia Initiative, Dementia Australia, Emerge Australia, Epilepsy Australia, Epilepsy Foundation, Fragile X Association of Australia, Huntington's Australia, Leukodystrophy Australia, Migraine Australia, the Mito Foundation, MJD Foundation, Motor Neurone Disease (MND) Australia, MS Australia, Muscular Dystrophy Australia, Muscular Dystrophy Foundation Australia, Myasthenia (Gravis) Alliance Australia, Parkinson's Australia, Polio Australia, Spinal Muscular Atrophy Australia and Polio Australia.*

The Neurological Alliance Australia *represents the approximately 1 in 6 Australians[1] living with neurological or neuromuscular conditions or neurological disorders with an annual impact on the Australian economy of over $100 billion[2].*

[1] Based on WHO global study: *Neurological Disorders: Public Health Challenges,* A World Health Organisation Report retrieved from: https://www.who.int/publications/i/item/9789241563369
[2] Based on an aggregation of data from organisations who have commissioned economic impact studies

Introduction

The NAA is pleased to provide this submission to the Health Technology Assessment Policy and Methods Review Consultation 2 options paper. The focus of the comments provided in this submission are on key areas that will impact on people affected by neurological disorders or progressive neurological and neuromuscular conditions.

The NAA was established in 2010 to promote improved quality of life, coordinated services and greater research investment in these conditions that have no cure. This group includes adults and children, carers, families, friends and workmates whose lives have been affected by a progressive neurological or neuromuscular condition or a neurological disorder. The impact of neurological disorders and progressive neurological and neuromuscular conditions on individuals and families can undermine their resilience, which is a vital element of their ability to remain purposeful and in control of their lives in addition to preventing or minimising financial and emotional burden. Progressive neurological and neuromuscular diseases and neurological disorders are a set of complex and disabling conditions often resulting in severe functional impairment. While this broad group contains conditions with various characteristics, different disease trajectories and life expectancy, nearly all are degenerative, all are incurable and only some have proven treatments. This results in significant disability and the need for expert information, specialised care and personal assistance which is responsive to individual needs.

For those conditions where treatments are available, finding the right treatment option for every individual is paramount as suboptimal treatment can lead to an increased symptom burden and irreversible accumulation of disability. This in turn leads to an increased burden on the healthcare system and a further reduction in the quality of life of patients and their families.

What does the NAA want from this review?

Overall, Australia's high quality health system is bolstered by its regulatory system encompassing product efficacy, consumer/patient safety and value for money. These elements are all considered when evaluating new and reviewing existing therapies. As a patient-centred alliance, our driving motivation is for HTA reforms that enable consumers to access the treatment they need, as and when they need it.

This includes:

- Decision-making that engages consumers and incorporates their views into decisions that directly impact their health and healthcare options.

- Integration of the consumer voice in enunciating the value of new and novel therapies so as to improve policy reform and implementation.
- Improved transparency and communication of decision-making to consumers and patient organisations – what's happening when, and why.

Our most important objective for this consultation is improved consumer engagement to achieve improved patient outcomes.

We are also keen to ensure that the options chosen are:
- aligned with the Vision and Aim of Australia's National Medicines Policy[3]
- the Goals of the HTA Policy and Methods Review, in particular the first goal, to "reduce time to access for Australians so that they can access new health technologies as early as possible"[4], and
- the intent of the Strategic Agreement between the Australian Government and Medicines Australia "to elevate patient and consumer voice in access to medicines"[5].

Overall response

Overall, the NAA is generally positive about the Options paper. It includes a wide range of perspectives, is in-depth and comprehensive. Many issues and concerns have been addressed following the previous round of consultation and many very good ideas and solutions are proposed. In particular, the NAA is pleased to see the recognition of the need for transparency and a recognition of the need to improve the consumer voice in HTA processes.

It is hoped that further details and explanations of how and when these ideas and solutions will be implemented will be forthcoming.

This submission seeks to provide feedback on those sections of the Options Paper of most importance to the members of the NAA.

1. Transparency, communication and stakeholder involvement in HTA

1.1. Transparency and communication of HTA pathways, processes and decisions

The NAA is pleased to see the intention to:
- Publish plain language summaries of the various stages of HTA processes
- Make improvements to the HTA webpage including development of a dashboard

[3] https://www.health.gov.au/resources/publications/national-medicines-policy?language=en
[4] https://www.health.gov.au/our-work/health-technology-assessment-policy-and-methods-review#goals
[5] https://www.pbs.gov.au/info/general/medicines-industry-strategic-agreement

These improvements should include some indication of the timeline for listing new or amended medicines on the PBS including any interim arrangements in place between the recommendation to list and the listing itself. Ultimately, consumers are most interested in when the new medicine can be prescribed and is available in the pharmacy.

It will be important to develop key performance indicators to ensure these improvements are aligned to the goals of the HTA review, most importantly, to reduce time to access.

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

The NAA supports all of the proposals in this section as they will strengthen consumer evidence and input into HTA processes.

It is essential that the proposed framework is co-designed with consumer representatives.

The NAA is concerned about:

- Lack of any indication of funding or resources for consumer bodies, to ensure they can engage equitably, especially for those smaller peak bodies representing rare conditions
- Lack of any indication of providing training for consumers – HTA processes are necessarily complex, consumers need to be trained to engage effectively with the pharmaceutical industry and Department of Health representatives who have expertise and resources at hand
- Commercial-in-confidence issues – these are often a barrier to transparency and should be addressed through the framework process

1.3. First Nations people involvement and consideration in HTA

The NAA supports all of the proposals in this section that are intended to lead to improved outcomes for First Nations people. Once again, KPIs will need to be developed to measure progress.

1.4. State and territory government collaboration in HTA

There is a need to significantly improve the relationship between Federal and State governments/regulators (possibly through the National Health Reform Agreement (NHRA)) to ensure there is more clarity around improved data sharing arrangements and speeding up/alignment of approval processes and funding arrangements, especially the funding of specialised therapies.

2. Health technology funding and assessment pathways

2.1. Streamlining and aligning HTA pathways and advisory committees

The NAA supports proposed measures to reduce delays and increase timeliness and equity in access for patients.

The NAA is pleased to see:

- Recognition of the need to create more efficient timelines
- Recognition of the need for clarity of pathways, especially for new, innovative technologies
- Proposal to streamline processes through a single entry point, though a concern is that this may become unmanageable and lead to further delays in achieving timelines. This proposal for expansion, will need significant investment to ensure it is appropriately resourced given the current workload of PBAC members.

NAA members representing rare disease groups, including Mito Foundation, are cautious about the proposal to integrate the life-saving drug program (LSDP) decision-making process into the PBAC process.

It is essential that the existing guidelines and clinical expertise - a strength of the existing LSDP process - be retained, while still realising the timeliness benefits of a single assessment process.

2.2. Proportionate appraisal pathways

The NAA supports the proposal to develop a disease specific common model, which has the potential to benefit a number of conditions represented by the NAA.

3. Methods for HTA for Australian government subsidy (technical methods)

3.1. Determination of the Population, Intervention, Comparator, Outcome

The NAA supports the proposals in this section though in relation to increased early stakeholder input there is a need for equity, especially where relationships have not been established between peak consumer bodies and sponsors.

In supporting these proposals, we reinforce the need to undertake this work while not creating further delays. Early engagement with relevant consumer groups and expert clinicians, particularly as a result of horizon scanning, is a key means for this to be accomplished.

3.2. Clinical Evaluation Methods

NAA members are particularly interested in clarity and equity in how high unmet clinical need (HUCN) are defined in the implementation of these reforms. There are many rare diseases among NAA members, with no or

limited treatment options/therapeutics, and there is a risk that the new HUCN criteria will prioritise larger cohorts.

3.3. Economic evaluation

In this section, the Options paper makes use of the term "welfare gain", that is the welfare gain to society, to describe where the gain to society from funding a health technology is greater than the cost. The "welfare loss" should also be considered, where the time taken to negotiate and make decisions, including the need for resubmissions leads to delays in patient access to treatments.

Some members of the NAA expressed concern about the "lowest cost comparator" policy and its implications for treatment access to the consumer. As quoted in the HTA Options Paper (from Medicines Australia):
"a) The lowest cost comparator policy does not reflect the true value of the new therapy because it does not allow pricing at parity to the most commonly used alternative.
b) It acts as a barrier to accessing innovative treatments, which can compound over time as new therapies are also directly or indirectly price-referenced to an older, increasingly rarely used lowest-cost comparator".

In addition, concerns have been raised by stakeholders (noted in the HTA Options paper) that economic evaluation should not be used as price negotiation - as it currently serves in practice. During the evaluation process, parameters are often adjusted to reflect conservative estimates, resulting in a reduced economically justifiable price for new medicines. As a result, sponsors are incentivised to submit a higher initial price, anticipating negotiation and multiple resubmissions. This can lead to prolonged timelines for PBS listing, limiting patient access to essential medicines and increasing costs for both sponsors and the government.

A focus on the value assessment of new medicines when evaluating new innovative health technologies rather than the undercurrent of cost-minimisation and pricing would address this.

4. Health Technology funding and purchasing mechanisms and decisions

4.1. Approaches to funding or purchasing new health technologies

The NAA supports the proposals in this section that have the potential to improve timely and equitable access for consumers.

In addition, there should be a clear process to enable submissions by organisations other than pharmaceutical companies such as peak consumer or clinical bodies or a consortium of these bodies, such as the submission in 2010 to list nicotine patches on the PBS made by a consortium consisting of

Cancer Council Australia, Heart Foundation, Australian Council on Smoking and Health and Quit Victoria.[6]

5. Futureproofing Australia's systems and processes

5.1 Proactively addressing areas of unmet clinical need and gaps in the PBS

As stated earlier, NAA members are particularly interested in clarity and equity in how areas of high unmet clinical need (HUCN) are defined in the implementation of these reforms. There are many rare diseases among NAA members, with no or limited treatment options/therapeutics, and there is a risk that the new HUCN criteria will prioritise larger cohorts.

5.2 Establishment of horizon scanning programs to address specific informational needs within HTA and the health system

NAA members are excited about the prospect of horizon scanning to both address inequity and support timely access and look forward to further details on how this process will be implemented, including the evaluations process.

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[6] https://www.pbs.gov.au/pbs/news/2011/02/Extension_of_the_listing_of_nicotine_patches