NEUROLOGICAL ALLIANCE AUSTRALIA

Submission to the Health Technology Assessment Policy and Methods Review

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The Neurological Alliance Australia (NAA) is *an alliance of 15 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: *Dementia Australia, Brain Injury Australia, Emerge Australia, Huntington's Australia, Motor Neurone Disease (MND) Australia, MJD Foundation, MS Australia, Muscular Dystrophy Australia, Muscular Dystrophy Foundation Australia, Parkinson's Australia, Spinal Muscular Atrophy Australia, Leukodystrophy Australia, the Childhood Dementia Initiative, the Mito Foundation, Polio Australia and the Fragile X Association of Australia.*

The Neurological Alliance Australia represents *nearly 2 million Australians living with the conditions represented by the members of the Alliance with an annual impact on the Australian economy of over $50 billion.*

Introduction

The Neurological Alliance Australia (NAA) is pleased to provide a submission to the Health Technology Assessment Policy and Methods Review.

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 for which our member organisations provide services, support and advocacy. Included are comments provided by our member organisations and, in some instances, directly from people living with those conditions represented by the NAA.

The Neurological Alliance Australia is an alliance of 16 national not-for-profit peak or national patient organisations representing adults and children living with neurological disorders or progressive neurological and neuromuscular diseases in Australia. The Alliance was established in 2010 to promote improved quality of life, coordinated services and greater research investment in these diseases.

The Alliance represents nearly 2 million Australians living with 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. While this broad group contains conditions with various characteristics, different disease trajectories and life expectancy, nearly all are degenerative, all are incurable and few 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.

The Alliance works collaboratively to identify and advocate for opportunities that will drive improved quality of life for people living with these conditions and funding to support research.

What does the NAA want from this review?

Overall, Australia's high quality health system is bolstered by its regulatory system - 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.

The NAA is very pleased to see that as part of the HTA reform process, there is a commitment in the terms of reference "to improve communication and engagement and to better support consumers, patients and carers during the HTA process".

The NAA is also pleased to see the intention, under clause 6.3 of the Strategic Agreement, to co-design an "Enhanced Consumer Engagement Process" to capture consumer voices in respect of applications to list new medicines on the PBS.

The New Frontier report

The Report of an inquiry into approval processes for new drugs and novel medical technologies in Australia titled, "The New Frontier - Delivering better health for all Australians" was released in November 2021[1]. The Report contained 31 recommendations and many are relevant to this HTA review. The implementation of these recommendations would make significant improvements to HTA processes, particularly to the enhanced involvement of consumers.

The Federal Government is yet to provide a response to this Report.

1. Elements and features that are working effectively

Medicine Status website

The Medicine Status website enables consumers to search for and monitor the status of medicines as they progress through the Pharmaceutical Benefits Scheme (PBS) listing process[2]. Since its introduction, the Medicine Status website has been improved to add information about the government processes following a PBAC decision to recommend and definitions of PBAC outcomes such as "recommended" and "deferred" have also been added.

The explanation of the "government processes" that occur between the PBAC recommendation and the PBS listing, including an indication of the time-line is a very helpful improvement, as this addresses a common inquiry from consumers.

[1] https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report

[2] https://www.pbs.gov.au/medicinestatus/home.html

Further summary information, or helpful links, about the processes that lead to a submission being made to PBAC would enhance the consumer information on this site.

National Mutual Acceptance Scheme

Regarding ethics approval for clinical trials, the current National Mutual Acceptance Scheme (designed to harmonise ethics approvals within all jurisdictions) has been an improvement, but more work needs to be done to achieve a truly national and all-inclusive scheme.

As stated in the New Frontier Report, "governance requirements must be streamlined into a simplified national system."

2. Current or future barriers to earliest possible access

Small patient populations and high cost of clinical research

Due to the progressive and severe nature of many neurological or neuromuscular diseases and disorders and the current state of clinical development, often the only opportunity for those living with these conditions is to access therapy via experimental clinical trials.

Small patient populations coupled with the high cost of clinical research, means the imperative for pharmaceutical companies to bring these trials to Australia is not adequate. Also, there is currently little incentive for pharmaceutical companies to apply for regulatory approval and reimbursement in Australia. As a result, patients may be forced to travel overseas to seek experimental treatment at their own cost.

3. Current or future barriers to equitable access

Advanced therapeutics including gene therapies and gene modified cell therapies

The pathways for approval and reimbursement for such therapies is currently not clear in Australia. This is potentially discouraging pharmaceutical companies from pursuing Australia as a market territory resulting in patients not having the same access to therapies as their counterparts in other countries.

Recommendation 2 of the New Frontier report seeks to address this issue, though is not yet implemented.

Given the relatively small patient populations of some disease groups in Australia and therefore low financial return, incentives need to be in place for companies to apply for regulatory approval and give patients access including:

- clear pathways for regulatory approval and reimbursement of new and emerging advanced technologies, aligned with international processes.

- stronger orphan drug incentives to bring Australian orphan drug designation in line with other countries, in particular offering advice and assistance to small companies that have not previously interacted with the Australian regulatory system.
- a program like the FDA's 'Rare Paediatric Disease Priority Review Voucher Program'[3] should also be considered.

Patient registries

A unified patient registry or network of patient registries is urgently needed for the rarer neurological or neuromuscular diseases and disorders. Data capture from existing medical records should be integrated into a resource that allows accurate identification and characterisation of the patient population for clinical trial planning.

This could be expanded to include all types of dementia and mild cognitive impairment (i.e. a neurological diseases patient registry). Currently the system is not geared to capturing people early enough in the disease stage, which means that there is limited clinical trial ready cohorts and infrastructure in place to identify early cognitive change, or common outcome measures.

A patient registry with provision for clinical trial recruitment would enable increased participation of people with early cognitive impairment or dementia to participate in clinical trials for new medicines and technologies and access greater post-diagnosis support. This may be especially important as new disease modifying treatments for dementia are developed which are targeted to early disease stage.

In addition, a comprehensive patient registry with provision for clinical trial recruitment will improve equity of access to clinical trials. Currently patients will often only be given the opportunity to take part in a trial if they are treated by a clinician who is involved in or linked to the clinical trial. Patients living in smaller centres or remotely are particularly disadvantaged.

4. Elements and features that detract from person centredness

Whilst there are currently opportunities for patient involvement in the PBAC decision-making process, once a submission to PBAC is made, the process can proceed without any consumer involvement at all.

To improve the person centredness of the PBAC processes, sponsors could:

- alert patient organisations of their intention to submit at the point of seeking registration of a new medicine with the TGA, including summary information about the eligible patient population, what the clinical studies measured, key

[3] https://www.fda.gov/regulatory-information/search-fda-guidance-documents/rare-pediatric-disease-priority-review-vouchers

clinical outcomes and what they mean for patients and any safety considerations

- alert patient organisations of their intention to submit to PBAC and provide any additional information not provided at the time of registration with the TGA
- alert patient organisations of their intention to withdraw or somehow change existing listings on the PBS, so that patients and their healthcare teams can be alerted in a timely way
- include a section in their submission to PBAC that describes interactions with the patient population such as focus groups, discussions, surveys, etc about the impact of the new medicine on quality of life, (including the impact on mental health, carers, employment, etc) and share this information with the patient organisation (as it may have a bearing on any submission they will make to PBAC)

In addition, to improve person centeredness, the Department of Health could:

- Provide brief feedback to patient organisations on the quality and value of their submissions
- Ensure consumer hearings and stakeholder meetings include the presence of at least two people from the patient community impacted, *in addition* to any representatives from the patient organisation
- Compensate or reimburse any involvement of volunteer consumers in HTA processes including sitting fees, travel and accommodation costs and ensure protocols for consumer involvement are implemented; this involvement should be appropriately supported so that people with cognitive impairment are able to meaningfully participate
- Create pools of funding for those patient organisations with limited resources to enable their development of submissions and their own consumer engagement processes

Declaration of interest

The NAA is making this submission as we have an interest in the health and well-being of all people affected by neurological disorders or progressive neurological and neuromuscular conditions. We work with governments at all levels, engaging on the issues that concern the lives of people living with these conditions, their families and carers, the community and the economy. We declare that, in the past, our member organisations may have received funding support from pharmaceutical companies, with an interest in the conditions we represent in the form of grants for projects and in support of our research activities.

For more information about this submission and the work of the Neurological Alliance Australia please contact:

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