# Response ID ANON-2NKN-9S9V-M

Submitted to Health Technology Assessment Policy and Methods Review – Consultation 1
Submitted on 2023-06-05 13:54:31

## 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:
Louise

Surname:
Hardy

### What is your email address?

Email:
lhardy@arthritisaustralia.com.au

### What is your organisation?

Represented Organisation 1:
Arthritis Australia

### What is the type of organisation?

Patient or consumer organisation

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:
Impacts on people with arthritis

Topic area 2:

Topic area 3:

### Declaration of interests

No conflicts

Details of declarable interests:

## 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.:

• It is positive that there is a pathway for consumers to make submissions to the PBAC in support of applications, to highlight the impacts of access or lack of access to particular medicines and the outcomes that are important to them. However, there is not much information provided about how these submissions are used and how they have impacted on the outcome of the application.
• The Consumer Evidence and Engagement Unit is also a very positive development and appears to play an important role in supporting consumer groups navigating HTA processes, at some degree of arms length from the Department.

• Arthritis Australia had a positive experience being approached by a pharmaceutical company that was involved in piloting a new approach for briefing of consumer groups on PBAC applications. A briefing meeting was provided along with a plain language summary of the application. This was a helpful process, albeit the only time in recent memory that this has been proactively offered.
• The Conversations for Change initiative has also provided a useful forum to inform and provide feedback.
• However, PBAC papers and communications tend to be highly technical and not readily understandable to people who lack familiarity with bureaucratic and scientific processes and language. It should also be noted that engaging with PBAC and MSAC processes is time consuming, and consumers and consumer organisations, which are often small with many competing priorities, sometimes run by volunteers, are expected to provide their input without any remuneration.
• Once a medicine is approved, consumers do not have a seat at the table or visibility of the negotiations between the sponsor and the Department which will determine whether/when the medicine will be reimbursed.
• Clearly, it is important that new therapies are assessed for cost-effectiveness when considering public funding. However, there are challenges with current processes that can result in inequitable outcomes particularly for patients with rare diseases (see responses below).

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

• A recent example of a positive outcome is the PBAC recommendation to expand access to Tofacitinib for children under that age of 18 years who are living with juvenile idiopathic arthritis. The previous inequitable access of Tofacitinib only being available to people over 18 years had meant children did not had the opportunity to take an oral formulation of medication instead of an injection, and some who had not had adequate responses to other medications could not access a new treatment.

## 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 :

• We welcome speed of access as a shared goal. Australians with arthritis and their loved ones expect that they should have access to the right therapeutics at the right time. Arthritis conditions are degenerative and early diagnosis, treatment and management are key to preventing avoidable irreversible damage, pain and disability.
• Most of our comments on access relate to inequitable access, see relevant survey questions below. Cost and incentives (or lack thereof, for example poor financial return of the drug/procedure) leads to selective reliance on applications by commercial entities, and inequitable access for patients. For example in the rheumatology space, companies have no incentive to apply to PBAC for their drug for low return indications eg adalimumab for uveitis or anakinra for auto-inflammatory disease, rare conditions, or life threatening conditions if the patient is unable to access the drug. Some health services will meet the cost but this varies and introduces another form of inequitable access.

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? :

• Rheumatology, as with many areas of medicine, is likely to see major advances via precision medicine, genomics and other areas. It is critical that reforms be future proofed as much as possible so that patients have access to the right therapies at the right time.

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.:

## 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:

• Currently, HTA processes in Australia do not deliver equitable access to crucial therapeutics for people with forms of arthritis and rheumatic conditions, and this particularly impacts on those with less common conditions and children. Due to the sponsor driven system for reimbursement, and inherent challenges in running trials in these populations, patients often miss out on life changing medicines that they would be able to access if, for example, they had a different diagnosis or were over the age of 18. Patients are then reliant on compassionate access schemes or funding from health services, or risk suffering irreversible damage that could have been prevented with access to the right medicines.
• Funding tends to be for new drugs/procedures, and potential savings or expanded access from review of existing funded items is overlooked.
• A specific example of the inequity and complexity of the current system has been provided by a consumer living with psoriatic arthritis, which affects approximately 200,000 Australians:
"I have psoriatic arthritis that was diagnosed 5 years ago. I am one of the 1 in 3 people with PSA that have no testable blood markers of disease. I also don't have significant psoriasis, just a few dodgy nails. In the first few years, my primary symptoms were frequent episodes of painful enthesitis and bursitis, with random flares of synovitis in my neck, hands and feet. In the past year, the enthesitis and synovitis has become virtually permanent and I've also developed mild sacroiliitis.
I've trialled a bunch of medications - Methotrexate, hydroxychloroquine, naproxen, meloxicam, sulfasalazine and leflunomide. I've been taking the last three for a couple of years now, despite some pretty unpleasant side effects. They've helped a bit, but I haven't had much of a choice because I haven't been able to access biologic medication.
For psoriatic arthritis, I understand that biologics are currently only approved for the following -
• Consistently high ESR or CRP in blood
• 20+ joints impacted (or severe sacroiliitis)
• Severe psoriasis
After 5 years of active and progressing disease, I now have enough joints impacted to be a candidate for biologics. Trouble is, the damage is done! Scans show erosion in nearly every joint of my hands and feet, in fact I'm at the point now where the only shoes I can wear are birkenstocks and I struggle to do

anything that involves hand grip. I have a constant dull ache in my elbows, shoulders and heels which I hope will be helped by my new treatment.
It's so frustrating to me that if I had rheumatoid arthritis, I probably would've trialled methotrexate for 3 months then be put straight on humira/enbrel for early disease intervention. But because the system doesn't acknowledge variations in disease course, or the enormous amount of research showing the benefits of early biologic treatment for PSA, I'm stuck with lifelong damage."
• The Australian Rheumatology Association's submission to the House of Representatives Standing Committee on Health, Aged Care and Sport Inquiry into approval processes for new drugs and novel medical technologies in Australia clearly articulates the "catch 22" faced by rheumatology patients and proposes a mechanism for highly specialised drug approval for rarer diseases with high unmet need:
"Both TGA and PBS approval are given in terms of a specific medicine for a specific condition. Where a phase 3 trial program has shown efficacy and acceptable safety, this is an entirely appropriate mechanism to provide reimbursement approval. A consequence of this system is that PBS approval of medicines, and their subsequent use, is limited to the disease for which pharma company submissions are based. This mechanism prevents 'off-label' prescription by physicians. To seek use of a PBS-subsidised drug for a new disease, a fresh application backed by fresh trial data is required.
An unintended consequence of this is that patients with uncommon or rare diseases, in which traditional major trials are basically impossible, have no mechanism through which to obtain PBS funded access to specialised medicine, and never will. As a result, patients who have a clear opportunity to benefit from a given medicine, supported by evidence such as appropriate molecular mechanisms and peer-reviewed case series, cannot gain this benefit.
For our members, and their patients, this creates a scenario wherein a rheumatologist might see consecutive patients on a given day whose disease has the potential to be successfully treated with a medicine, one of whom can access this medicine and one of whom cannot. In some cases, this difference is simply the result of human-determined disease nomenclature which classifies patients in, or out, of PBS funded diagnostic groups for otherwise similar diseases. In other cases, a disease is simply too rare for large scale trials to ever be done, and so under the current regimen PBS-funded access to life saving and/or life changing therapy will never occur.
The ARA urges this Parliamentary inquiry to review mechanisms for access to medicines, PBS listed for a given disease, in other diseases of high unmet need where the traditional pathway for PBS listing is unfeasible. An outcome of this review could include a highly specialised drug approval mechanism, potentially limited to particular specialists and a list of medicines paired to rare diseases with an appropriate evidence base.
Under such a regime, subject to approval, patients with these diseases could access specialised medicines subsidised by the PBS. The potential examples of this scenario are many in terms of individual diseases, but few in terms of total patient numbers. For example, a typical practice might manage some dozens to low hundreds of patients with rheumatoid arthritis, but only a handful (single digits) of cases suited to what we propose."

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? :

As above per the ARA's submission to the House of Reps inquiry "An outcome of this review could include a highly specialised drug approval mechanism, potentially limited to particular specialists and a list of medicines paired to rare diseases with an appropriate evidence base.
Under such a regime, subject to approval, patients with these diseases could access specialised medicines subsidised by the PBS. The potential examples of this scenario are many in terms of individual diseases, but few in terms of total patient numbers. For example, a typical practice might manage some dozens to low hundreds of patients with rheumatoid arthritis, but only a handful (single digits) of cases suited to what we propose."

## 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 current focus on cost-effectiveness diminishes the importance placed on mortality and disability.
• Increased transparency and accountability is needed, including how cost factors into decision making. There is a lack of user friendliness and transparency around PBAC processes as mentioned above. Documentation is not easy for people with a non technical background to engage with, and limited feedback is provided on the impact of consumer submissions on decisions, or the process of negotiation following a positive PBAC recommendation.

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

• In order to achieve person centredness, a diverse range of consumers must have input at every level and be supported to actively participate. Key information must be communicated in a way that can be readily accessed and understood, including for people without internet access.

## 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:

• The complexity and sophistication required for successful applications favours applications by pharmaceutical companies and makes patient or clinician led applications difficult to succeed.
• The current process favours high return drugs/procedures.

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:

## 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::

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:

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## 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?

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