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Arthritis Australia submission on Medicines Repurposing Program framework consultation

ABOUT US

Arthritis Australia is the peak arthritis organisation in Australia and is supported by affiliate organisations in ACT, New South Wales, Northern Territory, Queensland, South Australia, Tasmania and Western Australia.

Arthritis Australia provides support and information to people with arthritis as well as their family and friends. It promotes awareness of the challenges facing people with arthritis across the community, and advocates on behalf of consumers to leaders in business, industry and government.

In addition, Arthritis Australia funds research into potential causes and possible cures as well as better ways to live with the disease.

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Introduction

Arthritis Australia welcomes the opportunity to provide a response to this targeted consultation.

For many people suffering from arthritis, access to safe and effective medicines is an important part of being able to manage their conditions. We therefore welcome initiatives that have the potential to widen access to suitable and effective medicines such as the Medicines Repurposing Program (MRP).

Subject to the comments below, Arthritis Australia is supportive of the proposal to introduce this program, and in particular welcomes the potential for consumers to gain access to existing medicines with new therapeutic uses that may not otherwise happen under the existing arrangements due to the requirement for a sponsor to proactively go through the process of seeking an extended indication.

The proposed framework appears to be generally appropriate – noting that as a new program some aspects of the framework will likely need to evolve in light of experience with its implementation.

Notwithstanding our general support for the proposed approach, we would however like to make the following comments in relation to the consultation document.

Cap

We question whether the cap of "up to five" medicines that can be taken forward for evaluation in any one year is set too low.

From reviewing the proposed framework, it appears that significant technical work will be required by non-sponsor stakeholders in order to bring forward a proposal for repurposing under this new program.

We note that the completion of the initial nomination form will require information to be provided on:

- *current registration details of the medicine*
- *proposed dose for relative dose comparison with registered medicine*
- *evidence of efficacy of proposed treatment, e.g. evidence types used for literature based submissions*
- *evidence of the clinical impact the proposed candidate medicine will have if repurposed*
- *evidence that the proposed treatment will be more accessible than currently available treatments; and*
- *whether sponsor's interest to repurpose the product has been ascertained by the nominating party*

The resources and technical knowledge required to provide the above information is not insignificant. As these are proposals from non-sponsors, most, if not all, of the work required to assemble this information will need to be undertaken pro bono – often by already very busy clinicians and/or charities. If the perceived likelihood of success is very low, non-sponsor stakeholders are likely to be discouraged from coming forward and initiating this process.

The introduction of the MRP stems from the recognition that sponsors often perceive that the work required in extending a medicine's indications is not worth their (commercial) worthwhile. It would be a missed opportunity for consumers if a low likelihood of success similarly deterred

non-sponsor stakeholders from undertaking the work required to bring forward nominations under the MRP.

Arthritis Australia recognises the need to ensure the introduction of the Medicines Repurposing Program does not disrupt the existing process for assessing sponsor applications for extending indications for existing medicines, however we note that between 2015 and early 2022 over 230 medicines have been repurposed as a result of sponsor applications. In this context, we would submit that the proposed cap could be set at a higher level than five applications per year without disrupting existing repurposing work.

Equity in selection of proposals for evaluation

Patient volumes

The issue of ensuring equity in selecting proposals to be taken forward is an important one. The selection criteria as proposed includes the following criterion, which is strongly supported by Arthritis Australia

> *The available evidence suggests that the proposed treatment has potential to achieve significantly better quality of life, survival, or other benefit for patients who can access it.*

The framework however does not include any indication of the weighting that this criterion will have vis a vis the other criterion. From an arthritis disease perspective, there are over a hundred different types of arthritis. Many of these types are rare but are still extremely debilitating. It is not clear from the proposed criteria how much weight will be given to nominations covering conditions that have very low numbers of patients yet cause high morbidity within that group.

We are concerned that the application of the cap will mean that proposals for medicine indication extensions for disease conditions with a very low number of patients – no matter how debilitating - are unlikely to be selected.

An option to address this potential inequity would be to reserve one or two slots for nominations for medicine indication extensions that are directed towards the treatment of rare diseases. The adoption of an option along these lines would be easier to achieve under a higher cap than five.

Clinician availability and willingness

A further equity issue arises from the willingness and resources available to different clinical and patient groups to undertake the work required to bring a nomination forward.

As noted above, completing a nomination is likely to require significant technical knowledge and resources – with much of this work being required to be provided by expert clinicians on a pro-bono basis. The outcome for patients could be a lottery – with those “lucky” enough to have stakeholders/clinicians willing and able to put together nominations likely to be advantaged.

An examination of the proportion of applications received from different parts of the health sector will likely provide a window into any emerging systemic discrimination under the MRP and enable proposals to level the playing field to be identified.

Resource inequities for potential non-sponsor nominations could, in part, be addressed by enabling access to Departmental support to assist with the completion of the initial nomination form. This could be provided by assistance from Departmental staff (or contracted researchers).

Equity across community cohorts

An important consideration in prioritisation and selection relates to ensuring equity across different community groups, including first nations people, those from rural and remote locations, people born overseas, those from diverse gender and sexuality backgrounds.

We know that much of the research leading to the initial registration of new medicines is often based on limited cohorts of subjects (generally derived from mainstream populations), which is then reflected in the populations for which a medicine is indicated. Further research and real world experience in the use of approved medicines can bring to light additional information on the therapeutic benefits that may accrue to non-mainstream cohorts and were not part of the initial indications for a new medicine.

The design and implementation of the proposed framework would benefit from consideration of the ongoing systemic discrimination that can accrue from systemic biases in initial research and the ability for the repurposing mechanism to correct these biases.

Governance and transparency

Arthritis Australia strongly supports the principle of consumer involvement and input at all levels and stages of health program design and development in order to realign the health system to being more consumer centric.

We acknowledge the technical nature of much of the decision-making process that will need to be brought to bear in implementing the MRP. Nevertheless, given the very small number of applications that will be taken forward for evaluation and the even smaller number of applications that will be successful, not many worthy applications will be able to be successful. Value judgements about “relative worthiness” will be necessarily have to be made by the TGA Chief Medical Adviser as the ultimate decision-maker.

We note that the process will be informed by the TGA’s Advisory Committee on Medicines (ACM), whose current 20 members has only one consumer representative. We would ask the TGA and the Department to give greater consideration to how consumers can be more involved in this process – particularly where decisions based on value judgement about ‘relative worthiness’ are going to be made.

We also note that the framework does not consider transparency or evaluation performance measures.

Subject to commercial in confidence constraints, the implementation of the program needs to be as transparent as possible. Noting the equity issues identified above, timely information on the proportion of applications received and taken forward by disease group should be made publicly available.

The proposed evaluation process should also be included in the framework.

Summary of recommendations from Arthritis Australia

- Increasing the cap from 5 to up to 10 medicines that can be considered for evaluation each year
- Include at least 1 medicine each year used in the treatment of rare diseases
- Publish information on the medicines that are selected for further evaluation by:
    - Disease type

    - Numbers of patients suffering from the disease/condition to be addressed by the extended indication
        - including demographic data where available such as gender, sexual orientation, age, first nations or non-english speaking background
- Provide assistance to non-sponsor applicants in completing the initial nomination form
- Provide greater consumer representation and involvement in the governance of the MRP
- Publish the plan for the evaluation and review of the MRP, including analysis of emerging systemic biases in relation to the medicines selected for evaluation