

8 October 2021

### **Re Mito Foundation's submission to the review of the National Medicines Policy (NMP)**

The Mito Foundation welcomes the opportunity to contribute to the refresh of this important policy.

#### **About mitochondrial disease and Mito Foundation**

The Mito Foundation supports patients with mitochondrial disease (mito) and their families, funds essential research into the prevention, diagnosis, treatment and cures of mito, and increases awareness and education about these devastating diseases.

Mito is a debilitating genetic disorder that starves the body's cells of energy, causing multiple organ dysfunction or failure and death. Depending on which parts of a person's body are affected and to what degree, people with mito may: have strokes or seizures; be unable to walk, eat, swallow or talk normally; develop liver disease or diabetes; suffer heart, respiratory or digestive problems; lose their sight or hearing; suffer muscle weakness and pain; develop childhood dementia and experience developmental delays or intellectual disability.

Approximately 120,000 Australians carry a mitochondrial DNA mutation that can potentially cause mito, and around one child will be born every six days who will inherit this deadly disease.

#### **The importance of future medicines policy to Australians with mito**

A Cochrane Review in 2012 confirmed that 'there is currently no clear evidence supporting the use of any intervention in mitochondrial disorders'<sup>1</sup>. Little has changed for patients since 2012. There is an urgent, unmet need for treatments and cures for patients with mito.

Currently patients and their families are left to rely on a 'mito cocktail' of vitamins and supplements as a primary source of relief from symptoms. This is often coupled with changes to diet and exercise that can relieve symptoms and may slow progression for some people with mito.

This submission aims to highlight mito specific issues for consideration in the review of the NMP. Broadly, Mito Foundation supports the submissions from both Rare Voices Australia

and Genetic Undiagnosed And Rare Disease (GUARD) Collaborative Australia. Mito Foundation encourages the review of the NMP to consider how it can align to the *National Strategic Action Plan for Rare Diseases*<sup>2</sup>.

**Terms of Reference 1: Evaluate the current NMP objectives and determine whether these should be modified or additional objectives included. This includes consideration of the proposed Principles to be included within the NMP.**

*Are these proposed principles appropriate? With regard to the proposed principles, is anything missing or needing to change?*

Mito Foundation supports the proposed principles and suggests that a new principle, Innovation, is added.

The proposed principles are important to the mito community, particularly:

- Equity – Due to small patient numbers, research and development for rare diseases is difficult, costly and risky. This often results in a very high cost per patient for effective medicines. The principle of equity will be important to ensure that people with mito have affordable access to future medicines.
- Consumer centred approach – The mito community, like many rare disease communities in Australia, is willing and able to contribute to all stages of policy development, research, resource development and decision making. The NMP needs to prioritise empowering a diverse range of consumers in these processes.
- Partnership based – Mito Foundation is the key catalyst for development of medicines for mito in Australia. This is through funding of research into mito in Australia and overseas, connecting with international organisations and medicines companies. Australia's medicines policy needs to recognise the important role and expertise of rare disease organisations.

Mito Foundation also supports proposals from Rare Voices Australia and GUARD to introduce a new principle, Innovation. This principle will help to guide policy development to ensure that policies and processes encourage investment in medicines research in Australia and support re-use and re-purposing and other flexible approaches to translation of research findings to effective treatments for Australians living with mito.

*Are these four Objectives still relevant? Should any be modified, or any additional objectives considered? If so, how and why?*

Mito Foundation supports the four objectives, and the connection 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. Mito Foundation has made a submission to this inquiry.

Timely access to medicines is particularly important for Australians with mito. Mito is a degenerative condition and most experts predict future therapies will need to be administered in early stages of the disease to preserve remaining organ function.

Quality use of medicines is an important goal for the NMP and an area that the mito community struggles with. Due to the nature of a disease that affects metabolism, there are specific medicines that are safe and unsafe for people with mito, including specific antibiotics, anti-epileptic and anaesthetics<sup>3</sup>. Being a rare disease, quality use of medicine for people with mito is often left to chance – chance that the patient knows about these lists, chance that their health professionals have sufficient knowledge of their rare disease.

There may also be a role for research to inform quality use of supplements, acknowledging that these fall under the definition of medicines in the NMP. There has been insufficient formal research into these supplements in mito, however they are still included in the Australian Patient Care Standards for mito<sup>4</sup> and widely prescribed. This leads to uncertainty of efficacy for individual patients and difficult decisions for patients and clinicians, particularly given high out of pocket costs for these products that do not attract reimbursement.

There is room for improvement in how the important goal of quality use of medicines is realised in Australia.

**Terms of Reference 2: Consider the definition of medicines and whether the NMP needs to be expanded to include health technologies.**

Mito Foundation supports the current definition of medicines, particularly the inclusion of dietary supplements within this definition. It is important that medicines policy includes these products to support safe ongoing supply of these products to Australians with mito.

Mito Foundation also supports the position of Rare Voices Australia on this area, particularly:

- cell therapies and genetic therapies must be included in this definition;
- regulatory and reimbursement pathways must be suitable for precision and personalised medicines and informed by expertise in these treatments.

**Terms of Reference 3. Assess the NMP's utility in the context of rapidly evolving treatment options, population changes, interconnected relationships, and system-wide capacities.**

The NMP must consider how it can ensure timely access to medicines through clinical trials. There have been over 182 interventional clinical trials for mito<sup>5</sup>. Less than 10 of these trials have Australian sites. This means Australian patients are effectively unable to access these medicines and there is no Australian data for any future regulatory approvals or reimbursement. The NMP needs to create a framework that encourages clinical trial sites in

Australia and/or participation by Australian patients. Mito Foundation is directly investing in this goal through the establishment of the Australian Mito Registry and the establishment of an Australian clinical trials network for mito.

The majority of clinical research takes place within health services funded and organised by state health departments and future therapies for mito are likely to be administered in an inpatient setting. Therefore, the NMP needs to emphasise the need increased connections between state and federal policies and funding in this area.

The connection between medicines access and genomics is particularly relevant to the mito community. It is almost certain that therapies for mito will be trialled only on patients with a confirmed genetic diagnosis of mito and many will only be suitable for patients with a specific genetic mutation causing their mito. The NMP must acknowledge the dependency on improved access to genomic testing to support equitable access to medicines in the future.

Mito Foundation welcomes the NMP reviews focus on health literacy and digital health. We encourage the review to consider how the benefits of advances in these areas will benefit people living with rare diseases including mito.

**Terms of Reference 4: Consider the centrality of the consumer within the NMP and whether it captures the diversity of consumers' needs and expectations.**

*How can the NMP's focus on consumer centrality and engagement be strengthened? Is anything missing, and what needs to change?*

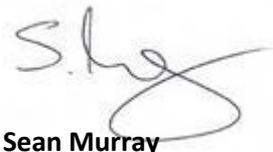
Mito Foundation supports the focus on diversity in a refreshed NMP. Intersections of diversity are important, such consumers with rare diseases that are also from CALD communities or are Aboriginal or Torres Strait Islanders. Deeper and more diverse engagement may require different ways of working and additional resources. For example, the consumer representative sector may need specific support to ensure that they engage with more diverse groups of consumers when engaging with medicines policy and regulatory processes.

Mito Foundation also supports the position of Rare Voices Australia, specifically:

- Earlier engagement with consumers;
- Increased training and support for consumers and consumer representatives;
- Innovative pathways through regulatory and reimbursement processes to increase consumer-led applications.

Thank you for the opportunity to provide input to this review. Mito Foundation is more than willing to work with the Department of Health on this and future reforms. Further information can be provided by contacting our Policy and Advocacy Manager Clare Stuart on 0410 685 181 or [clare.stuart@mito.org.au](mailto:clare.stuart@mito.org.au).

Yours faithfully,



**Sean Murray**  
Chief Executive Officer

#### References

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