Committee Secretariat
Standing Committee on Health, Aged Care and Sport
PO Box 6021
Parliament House
CANBERRA ACT 2600

15 October 2020

Dear Committee Secretariat,

The Mito Foundation welcomes the opportunity to provide a submission to the House of Representatives Standing Committee on Health, Aged Care and Sport Inquiry into approval processes for new drugs and novel technologies in Australia. The foundation and the community that we support also thank the Australian Government for its interest in this issue and their willingness to investigate it further.

Background

The Mito Foundation supports patients with mitochondrial disease and their families, funds essential research into the prevention, diagnosis, treatment and cures of mitochondrial disorders, and increases awareness and education about these devastating diseases. This submission provides information regarding the Mito Foundation's support for the review of approval processes for new drugs and novel medical technologies.

Mitochondrial disease is a debilitating genetic disorder that starves the body's cells of energy, causing multiple organ dysfunction or failure and potentially death. Mitochondrial disease occurs when a person's mitochondria are not functioning properly. It primarily affects the muscles and major organs, such as the brain, heart, liver, inner ears and eyes, but can cause any symptom in any organ at any age.

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

Depending on which parts of a person's body are affected and to what degree, people with mitochondrial disease 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.

In about half of the cases diagnosed, the disease is caused by changes in mitochondrial genes (mtDNA) which contribute about 0.1 per cent of a child's genetic make-up and are inherited only from the mother. The foundation is currently working with the Australian Government to bring forward legislation to allow mitochondrial donation to be made available to women who are at risk of passing on mitochondrial disease to their children. Given that this is not a treatment as such but rather an IVF technique that will enable children to be born without the need for future treatment for mitochondrial disease, mitochondrial donation and its related considerations are not reflected within this submission.

The Mito Foundation's mission is to support the mito community whilst the search for meaningful treatments and cures is ongoing. A Cochrane Review in 2012 confirmed that 'there is currently no clear evidence supporting the use of any intervention in mitochondrial disorders'[1]. As such, there is an urgent, unmet need for treatments and cures for patients with mitochondrial disease.

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. As well as being a costly investment for patients and their families, there is no solid evidence that these cocktails provide any relief or aid in any form of recovery. This is often coupled with 'lifestyle' changes around diet and exercise. While there is some evidence that lifestyle changes may aid in the treatment of symptoms, this does not compare to a pharmaceutical treatment for patients.

Whilst there are no treatments at present, as these are developed it is of critical importance to ensure that this under-served community has the speediest access to these as possible. At this time, there are currently more drugs in mitochondrial disease clinical trials than ever before, creating a promising pipeline for future drugs. The foundation supports patients having immediate access to drugs following safety and efficacy approval.

Terms of Reference 1– The range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies

The Mito Foundation supports the review of approval processes for new drugs and novel medical technologies to ensure that they are as rapid as possible. This will include future drugs that are made available for patients with mitochondrial disease. Currently it is taking far too long for drugs and technologies to be approved, even after safety and efficacy has been demonstrated, meaning that patients suffering with debilitating, life-limiting diseases such as mitochondrial disease have no viable options available to them.

[1] Pfeffer G, Majamaa K, Turnbull DM, Thorburn D, Chinnery PF. Treatment for mitochondrial disorders. Cochrane Database of Systematic Reviews 2012, Issue 4. Art. No.: CD004426. DOI: 10.1002/14651858.CD004426.pub3.

In February 2020, the Australian Government published the National Strategic Action Plan for Rare Diseases (Action Plan). A resulting action from the priorities listed in the Action Plan states that the Government will be required to 'develop policy that supports people living with a rare disease have timely and equitable access to new and emerging health technologies'.

This policy is urgently needed by patients who often are forced to look internationally for access to the latest advances in mitochondrial clinical care. The Mito Foundation supports development of this policy being made a priority by the Australian Government.

Gene therapies, targeting nuclear DNA or mitochondrial DNA, are an emerging area of potential for treatments and cures for mitochondrial diseases. There are numerous studies[2][3][4] taking place globally involving gene therapies and many provide tangible hope for the mitochondrial disease community. However, at this time these studies are out of reach for Australian patients. The foundation urges the Australian Government to provide immediate access to gene therapies for patients with mitochondrial disease as they become available. To achieve this, the foundation supports the creation of a clear pipeline for gene therapy approval processes for mitochondrial diseases.

Terms of Reference 2 – Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed and used to treat new conditions

One of the priorities for the Action Plan is to 'Translate research and innovation into clinical care; clinical care informs research and innovation'. At the right time, the speedy translation of mitochondrial research into clinical care would allow timely access to patients who are desperately in need of assistance. The Mito Foundation supports the implementation strategies from the Action Plan be put into effect here, namely that the Government should 'Encourage and facilitate greater research collaboration nationally, internationally and within industry' and offer financial incentives for research teams that can demonstrate this collaboration.

The Mito Foundation urges the Australian Government to offer further incentives to international research and pharmaceutical companies who are willing to bring their work to Australia, allowing patients with mitochondrial disease here speedy access to new drugs and novel medical technologies when they become available.

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[2] GS010 for LHON - GenSight Biologics <https://www.gensight-biologics.com/product/gs010-for-lhon/>

[3] Mok, B.Y., de Moraes, M.H., Zeng, J. et al. A bacterial cytidine deaminase toxin enables CRISPR-free mitochondrial base editing. Nature 583, 631–637 (2020)

[4] Reynaud-Dulaurier, R, Benegiamo, G, Marrocco, E, Al-Tannir, R, Maria Surace,E, Auwerx, J, Decressac, M, Gene replacement therapy provides benefit in an adult mouse model of Leigh syndrome, Brain, 143, Issue 6, 1686–1696 (2020)

The foundation encourages the Australian Government to prioritise and encourage fundamental discovery research for mitochondrial disease through research funding.

The foundation supports a clear pathway for personalised medicine be established, that benefits the wellbeing of patients with mitochondrial disease.

Terms of Reference 3 – Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies

At the time of submission, there are currently around 200 clinical trials (source: clinicaltrials.gov) for mitochondrial diseases that are either recruiting patients or completed. None of these trials are available for patients living in Australia.

A study completed by the Department of Health found that one of the key barriers to both Australian and international pharmaceutical companies looking to conduct trials in Australia was cost, noting that this country was more expensive than South-East Asia and Latin American sites[5]. Costs to host a clinical trial in Australia come with a fixed set-up price per trial. Due to Australia's relatively small population size, especially when considering numbers of patients of a rare disease such as mitochondrial disease, this can lead to a trial becoming prohibitively expensive. The Mito Foundation urges the Australian Government to consider trial set-up costs for diseases with a small population.

The Mito Foundation has been working to combat the issue of available mitochondrial disease trials in Australia. The foundation has set up the Mitochondrial Disease Patient Registry, which currently has over 500 patients registered. The aim of the registry is to allow patients the first possible chance to be involved in a study, should one become available. The registry also allows the foundation to demonstrate to pharmaceutical companies that Australia has a strong patient population who are keen to be involved in trials.

The foundation is also supporting Australian clinical trials by providing funding for support for infrastructure. Since 2018, the Mito Foundation has provided $450,000 through its Clinical Trial Support Grants program, to help in the set-up of two mitochondrial disease trials within Australia. One of these grants supports the creation of an integrated, national clinical trial network for mitochondrial disease in Australia.

The foundation supports the work the Australian Government is already doing to provide access to clinical trials. However, there is more work that can be done. The foundation urges the Australian Government to consider the barriers restricting pharmaceutical companies from bringing trials to Australian patients with mitochondrial disease.

[5] Department of Health. Analysis of Recently Conducted Clinical Trials Final report August 2015

The foundation also supports the establishment of a clear pathway that outlines the process from discovery, to clinical trial, to commercialisation for novel drugs that would be of assistance to patients with mitochondrial disease.

In line with the Action Plan, the foundation supports 'Building on existing initiatives, (to) continue to foster an environment conductive to clinical trials for rare diseases taking place in Australia.'

Terms of Reference 4 – Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment

The foundation supports the inclusion of experts in the process for approval of new drugs and novel medical technologies for mitochondrial disease. Australia is privileged to be home to a number of the top mitochondrial disease researchers in the world. The foundation highly supports the approval process for mitochondrial disease treatments to involve their expertise, ensuring that patients are receiving the best possible care.

The Mito Foundation appreciates the opportunity to present this submission to the Inquiry and is available for clarification of any of this information.

Yours sincerely,

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