Senate Community Affairs References Committee Inquiry into the Mitochondrial Donation Law Reform (Maeve's Law) Bill 2021

Rare Voices Australia Submission

July 2021

Introduction

Rare Voices Australia (RVA) is the peak national body for Australians living with a rare disease. RVA led the collaborative development of the National Strategic Action Plan for Rare Diseases[1] (the Action Plan) and is now leading its collaborative implementation. Launched in February 2020 by the Minister for Health with bipartisan support, the Action Plan specifically addresses the need for legislative change to ensure that legislation and policy align with advances in health technology. RVA also strongly advocates for increased reproductive confidence for those at higher risk of passing on genetic conditions. As such, RVA supports Maeve's Law being legislated with some minor amendments to ensure that access during Stage 1 is equitable and timely to enable families to make reproductive decisions with confidence. The suggested minor amendments will also ensure continuity of care in the transitions from Stage 1 to Stage 2 (in the event that the evidence from Stage 1 supports mitochondrial donation in clinical practice). Additionally, RVA recommends that specific provisions be made for priority populations identified in the Action Plan[1 (pp 12-14)].

Alignment Between the Action Plan and Proposed Bill

RVA is supportive of the key features of the Bill including the two-stage process that allows real world evidence regarding safety and efficacy to be collected during Stage 1. We also support the approach that gives rare disease families increased choice and reproductive confidence. The Action Plan addresses the need for changes in legislation and policy to support reforms such as Maeve's Law including:

Priority 2.3: Facilitate increased reproductive confidence.

Priority 2.4: Enable all Australians to have equitable access to the best available health technology.

Action 2.4.1: Develop policy that supports people living with a rare disease to have timely and equitable access to new and emerging health technologies.

Implementation 2.4.1.5. Ongoing review of health technology policy in line with advancements in health technology. For example, mitochondrial donation involves removing the nuclear DNA from a woman's egg containing faulty mitochondria and inserting it into a healthy donor egg, which has had its nuclear DNA removed. This prevents mitochondrial DNA defects from being inherited by a genetically related offspring. Mitochondrial donation is not yet legal in Australia.[1 (pp 28-30)]

The proposed two stage implementation of the Bill aligns with the Action Plan. RVA commends the focus on person-centred research, particularly in regard to the Stage 1 focus

on clinical trials, research, data collection and translational research leading into Stage 2. These aspects of the Bill align with several implementation steps identified in the Action Plan including:

Implementation
3.2.4.1. Develop recommendations to encourage and enable more clinical trials for rare diseases to take place in Australia.

3.2.4.2. Increase the economies of scale of research into rare diseases by, for example, operating multi-trial sites that share common resources.

3.2.4.3. Encourage the adoption of unique and appropriate trial designs that overcome rare disease research challenges.

Priority 3.3: Ensure research into rare diseases is collaborative and person-centred.

Priority 3.4: Translate research and innovation into clinical care; clinical care informs research and innovation.

Action 3.4.1: Support partnerships between researchers and clinicians in research into rare diseases.[1(pp39-43)]

Additionally, RVA supports measures included in the Bill for privacy and protection of the rights of the child.

Recommendations to Strengthen Alignment with the Action Plan

RVA recommends minor changes to the proposed Bill so that it aligns more closely with the Action Plan and to ensure equity and increased certainty for families living with mitochondrial disease, including those from priority populations identified in the Action Plan.

Recommendation #1

The limited number of licenses for clinical trials (1-3) may create inequity with access being available in only some states and territories. Timeliness of access is important to families making reproductive decisions and the opportunity to participate in a mitochondrial donation trial should be available to all Australian families that are eligible and wish to access the trial. RVA recommends that either a) trial licenses be multi-site and license recipients be required to offer the trial in all states and territories or b) support is provided to families to access the trial at the licensed sites even if they live in another state or territory.

Recommendation #2

It is important that, should the Stage 1 clinical trial phase demonstrate the safety and efficacy of mitochondrial donation, that there is continuity of access from Stage 1 to Stage 2.
Stage 2 implementation into clinical practice requires legislative change in all states and territories.

To ensure ongoing certainty and continuity of access to mitochondrial donation, this Bill should include provisions to ensure that the transition from Stage 1 to Stage 2 does not give rise to any further uncertainty or inequity should one state or territory implement legislation faster than another. This could be done by engaging states and territories in the early phases of Stage 1 to begin necessary discussions and plans for change. If a state or territory chooses not to make mitochondrial donation available in their jurisdiction, families should be supported to access it in another state or territory. Families living with a rare disease already experience high levels of uncertainty and inequity. Policy and legislation should aim to reduce uncertainty and inequity.

Recommendation #3

Ensure that priority populations identified in the Action Plan, have equitable access to support, educational materials, the Stage 1 clinical trial and, if applicable, access to mitochondrial donation at Stage 2.

The relevant priority populations as identified in the Action Plan include:

- Australians with an increased chance of developing a rare disease or of having a child with a rare disease;
- Aboriginal and Torres Strait Islander people;
- People living in regional, rural and remote areas;
- People from culturally and linguistically diverse (CALD) backgrounds; and

- People experiencing socio-economic disadvantage.[1(pp12-14)]

The Action Plan requires that implementation of this Bill will*:*

Implementation
2.1.1.4. Ensure care and support is responsive to the specific needs of rural and remote communities and health services, Aboriginal and Torres Strait Islander people, those with CALD backgrounds, and other priority populations.[1(p21)]

In relation to the proposed Bill, the license of any single provider for Stage 1 clinical trials should require adequate support and adjustment to ensure priority populations that may face barriers to access can participate in the trials. The provider should also ensure that care and counselling is responsive to the needs of these priority populations. This includes the provision of appropriate, non-directive educational materials developed as described in the Action Plan:

Implementation
1.1.1.4. Collaborate with targeted stakeholders to maximise the reach and appropriateness of materials to Aboriginal and Torres Strait Islander people, those with CALD backgrounds, and other priority populations.[1(p16)]

Summary

RVA is generally supportive of the proposed Bill as it addresses the needs of the mitochondrial community, provides appropriate and measured safeguards and aligns with Government policy on rare diseases (the Action Plan). RVA believes the Bill includes appropriate protections regarding confidentiality for families, while also protecting the rights and privacy of children born via mitochondrial donation. RVA supports the two-stage approach as it enables the collection of evidence to ensure the safety and efficacy of the procedure prior to translation into clinical practice. The Bill would be strengthened by minor yet important amendments to increase equity and certainty for families impacted by mitochondrial disease.

References

1. National Strategic Action Plan for Rare Diseases (2020) ©Commonwealth of Australia (Department of Health) 2020. Cited under Creative Commons Attribution 4.0 International Public License.