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16 July 2021

Senator Wendy Askew
Senator Rachel Siewert
C/O
Committee Secretary
Senate Standing Committees on Community Affairs
PO Box 6100
Parliament House
Canberra ACT 2600

Dear Senator Wendy Askew and Senator Rachel Siewert:

On behalf of the International Society for Stem Cell Research (ISSCR), the leading professional organization of stem cell scientists, I write to share our perspective on mitochondrial replacement techniques (MRT) and express our support for the Mitochondrial Donation Law Reform (Maeve's Law) Bill 2021. The ISSCR is the leading professional organization of stem cell researchers and represents more than 4,000 members in Australia and around the world. Our members are scientists, clinicians, ethicists, and educators dedicated to the responsible advancement of stem cell research and its translation to the clinic. The ISSCR appreciates the ongoing [public conversation](#) in Australia regarding the ethical and policy issues associated with enabling mitochondrial replacement techniques and supports legislation to advance the clinical investigation of MRT with careful regulatory oversight to refine the procedure and assess its safety and efficacy.

Mitochondrial Replacement Techniques Can Reduce the Transmission of Mitochondrial Disease

New scientific advances make it possible to significantly reduce the transmission of maternally inherited mitochondrial diseases by replacing abnormal mitochondria in an egg or embryo with healthy mitochondria from a donor. It is important to note that cells have two different genomes: the nuclear genome that contains the vast majority of genes that encode our traits and a small mitochondrial genome that encodes a small number of the proteins required for energy metabolism. Heritable mitochondrial genome replacement does not involve nuclear genome editing

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and can be performed safely to prevent the transmission of mitochondrial diseases to children. It should be regulated separately from heritable nuclear genome editing, which remains unsafe because of its potential to unpredictably and permanently alter traits involved in many different aspects of human physiology.

Mitochondrial diseases are devastating because they damage the parts of the body that need the most energy—such as the heart, brain, and muscles. Women carrying a high proportion of mutated mitochondria in their oocytes (eggs) are often unable to have biologically related children that do not suffer from severe mitochondrial diseases. MRT can significantly reduce their risk of transmitting mitochondrial diseases by replacing the mitochondrial DNA of a mother affected by mitochondrial disease with healthy mitochondrial DNA from a donor. MRT enables parents at high risk of transmitting mitochondrial disease to have healthy children that are biologically related to both parents.

The ISSCR supports enabling the clinical use of MRT to prevent the transmission of serious mitochondrial diseases and offered new recommendations to support the [clinical use of MRT](#) in the society's updated [Guidelines for Stem Cell Research and Clinical Translation](#). The Guidelines recommend limiting the initial uses of MRT to "clinical investigation that is subject to strict regulatory oversight, limited to patients at high risk of transmitting serious mitochondrial DNA-based diseases to their offspring, when no other treatments are acceptable, and where long-term follow-up is feasible" (Guidelines Recommendation 3.4.8.1).

Mitochondrial Donation Law Reform (Maeve's Law) Bill 2021

The ISSCR supports the Mitochondrial Donation Law Reform (Maeve's Law) Bill 2021 because it would establish a rigorous, incremental approach to enable MRT to prevent the transmission of mitochondrial diseases that is consistent with the process described in the ISSCR Guidelines. Importantly, the bill provides a process for evaluating the safety and efficacy of MRT before making it available more broadly in clinical practice. In particular, the ISSCR appreciates that the bill ensures MRT will be subject to a well-regulated oversight process that limits the use of MRT to

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the prevention of mitochondrial diseases. This bill would align Australia's oversight of MRT with the United Kingdom, which was the first country to [enable MRT](#) to prevent the transmission of serious and devastating mitochondrial diseases.

Thank you for considering our views as you consider the Mitochondrial Donation Law Reform (Maeve's Law) Bill 2021. If the ISSCR can be of further assistance to you as you move forward on this issue, please contact Eric Anthony, ISSCR's Director of Policy at eanthony@isscr.org.

Sincerely,

Melissa H. Little
President, ISSCR
Murdoch Children's Research Institute and
University of Melbourne, Australia

Promoting excellence in stem cell science and applications to human health.

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