

Australian parents-to-be expect pioneering UK approval of 'three-person baby IVF' to prompt changes to Australian law

In a pioneering move, the United Kingdom has become the first country to officially authorise clinical use of mitochondrial donation IVF to enable healthy babies to be born to women carrying deadly mitochondrial disease, which starves major organs of energy.

Regulators meeting in London on 15 December gave the green-light to recommendations by an expert panel to license so-called 'three-person baby IVF', following the world's most comprehensive global scientific and ethical review of the treatment over a ten-year period.

The Human Fertilisation and Embryology Authority (HFEA) approved mitochondrial donation for "cautious" use in "specific circumstances... where inheritance of the disease is likely to cause death or serious disease and where there are no acceptable alternatives".

Mitochondrial donation has been permitted in the UK since legislative changes in October 2015. The decision means specialist IVF clinics in the UK wanting to offer mitochondrial donation to patients may now apply to the HFEA for permission to do so.

Affected Australian women should also be given the chance to have healthy babies through mitochondrial donation, according to Sean Murray, CEO of the Australian Mitochondrial Disease Foundation (AMDF), which supports patients and funds research into the debilitating genetic disorder.

"It's very exciting that mitochondrial donation could soon be available to women in the UK, and the first babies born in 2017," Mr Murray said.

"As the HFEA Chair, Sally Cheshire, said of the decision, it's historic and will be life-changing for parents at very high risk of having a child with a life-threatening mitochondrial disease,"

"Following these ground-breaking UK developments, the Australian Mitochondrial Disease Foundation – and the local mitochondrial disease community – now looks forward to the Australian Government following suit and changing the law to give Australian parents-to-be the choice to access mitochondrial donation and have a healthy biological child.

"At least 60 Australian babies born each year suffer with severe and life-threatening forms of mitochondrial disease that could be prevented by using the mother's and father's nuclear DNA and replacing the mother's defective mitochondrial DNA with healthy mitochondrial DNA from a donor egg.

"Based on the extensive evidence available, the Australian Mitochondrial Disease Foundation believes the potential benefits of mitochondrial replacement outweigh the risks for unborn children who would otherwise almost certainly develop potentially fatal mitochondrial disease.

"The Australian Mitochondrial Disease Foundation supports making this ground-breaking treatment available for in-clinic use under certain specific conditions and strict regulation," he said.

At least one Australian child born each week – or 62 children every year – will develop a severe or life-threatening form of mitochondrial disease, and half will die in childhood. A further 30 Australian children born each week – or 1540 every year – are at risk for developing a mild to moderately disabling form of mitochondrial disease during their lifetime.

Professor David Thorburn is Head of Mitochondrial Research at Murdoch Childrens Research Institute and a member of the AMDF Scientific and Medical Advisory Panel and the AMDF Mitochondrial Donation Working Group, and made a submission to the independent panel.

“Research evidence indicates mitochondrial donation techniques are sufficiently developed and safe for in-clinic use, subject to specific conditions defined by the UK legislation and the expert report recommendations,” Professor Thorburn said.

“The expert report to the HFEA concludes that recent scientific advances have sufficiently addressed the potential carry-over of faulty mitochondrial DNA. It also recommends numerous safeguards such as carefully selecting women to undergo the procedure as a clinical risk reduction treatment, providing full information about potential limitations and risk, and undertaking genetic testing when the embryo is at 15-weeks’ gestation.

“It is important to note that the donor mitochondrial DNA only replaces 37 mtDNA genes – contributing about 0.1 per cent of the baby’s genetic make-up – compared with approximately 20,000 genes in the nucleus, which are not replaced.

“The mitochondrial DNA contribution is important for converting food into energy but appears to make no significant contribution to appearance, behaviour or other features, which are overwhelmingly determined by the nuclear genes and environment,” he said.

Mitochondrial disease (mito) is a debilitating and potentially fatal genetic disorder that starves the body’s cells of energy, depriving our major organs of the power they need to function properly. The ability to walk, run or even just stand up unaided can be a daily struggle for people with mito, which has few treatments and no cure and can cause any symptom in any organ at any age.

Mitochondria are the powerhouses of our cells that generate 90 per cent of the energy fuelling our bodies, particularly muscles and major organs like our brain, heart, liver, ears and eyes. Depending on which parts of their bodies are most affected and to what extent, people with mito can lose their sight or hearing, be unable to walk, eat or talk normally, have strokes or seizures, develop liver disease or diabetes, suffer cardiac, respiratory or digestive problems, or experience developmental delays or intellectual disability.

More than 1 in 200 Australians – at least 120,000 people – have genetic mutations that predispose their mitochondria to fail early, and may develop mitochondrial disease sometime in their lives. Many people are symptomatic but undiagnosed or misdiagnosed, some are not yet symptomatic, and others are unknowingly at risk of passing the disease to their unborn children.

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- Sean Murray, Australian Mitochondrial Disease Foundation
- Prof David Thorburn, Mitochondrial Research Group, Murdoch Childrens Research Institute (MCRI)
- Prof Carolyn Sue, Kolling Institute of Medical Research
- Prof John Christodoulou, Neurodevelopmental Genomics Research Group, MCRI
- Assoc Prof Ainsley Newson, Centre for Values, Ethics and the Law in Medicine, University of Sydney
- Australians affected by mitochondrial disease

Resources:

- AMDF Position Statement on Mitochondrial Donation (including an illustration of the process) and further information: www.amdf.org.au/mitochondrial-donation/
- HFEA media release: <http://www.hfea.gov.uk/10563.html>
- Expert panel report to the HFEA - *Scientific review of the safety and efficacy of methods to avoid mitochondrial disease through assisted conception: 2016 update*: www.hfea.gov.uk/10557.html
- A video primer on mitochondrial donation: www.nature.com/nrdp/animations/mito-dis-16