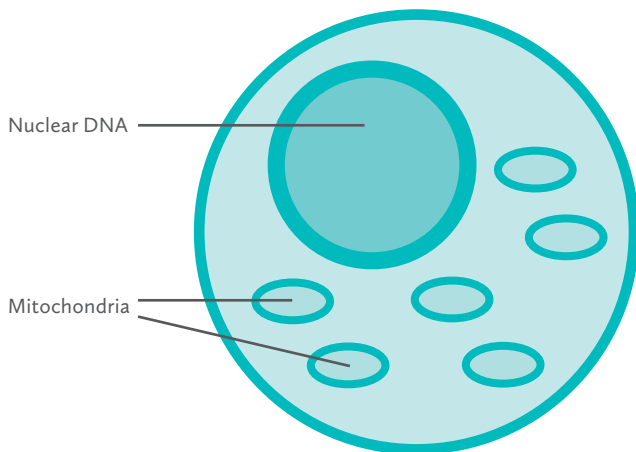


Around 1 in 6500 children is thought to develop a serious mitochondrial disorder. There is no cure and our current treatments only focus on managing the symptoms.



What are mitochondria?

Mitochondria are small structures found in our cells, which generate the cellular energy used to power every part of our body. Mitochondria have their own DNA, which only controls mitochondrial function and energy production. This is separate from our 'nuclear DNA', which makes us who we are and determines appearance and personality.

What is mitochondrial DNA disease?

Mitochondrial DNA disease commonly affects multiple different organs, with symptoms including

- loss of movement control,
- muscle weakness,
- diabetes,
- heart problems,
- epilepsy and stroke-like episodes,
- and in serious cases death.

What are mitochondrial donation techniques?

Mitochondrial DNA disease is passed down from mother to child. Two new IVF techniques have been developed which transfer the nuclear DNA (the unique genetic information that makes us who we are) out of a patient's egg containing faulty mitochondria and put it into a donor egg, containing healthy mitochondria, which has had its nuclear DNA removed. This enables the birth of a healthy child, free from devastating mitochondrial DNA disease.

Why are new regulations needed?

Currently the law only allows for these techniques to be used in research. For the IVF techniques to be used in patients, Parliament must pass new regulations. The Nuffield Council on Bioethics and the Human Fertilisation and Embryology Authority (HFEA) held extensive public consultations in 2012 into the ethical and social issues raised by mitochondrial donation techniques. They identified broad public support for use of these techniques within a robust regulatory framework. An Expert Scientific Review panel in April 2011, March 2013 and June 2014 have found no evidence to suggest that the techniques are unsafe for clinical use and concluded that both techniques have the potential to be used in patients with mitochondrial disease.

What happens after the regulations are approved?

If the regulations are passed, specialist clinicians will have to obtain a licence from the HFEA to use the techniques. This will only be granted once the HFEA is satisfied that any risk of their use is low. These IVF techniques will only be suitable for a specific group of women with mitochondrial DNA mutations and doctors will discuss in detail the different reproductive options for each patient.

Why should we allow this?

Mitochondrial donation will enable mothers to choose to have children who are genetically related to them, with a natural combination of nuclear genes from both parents, while being free from a potentially devastating disease.

Nuclear DNA is not altered and so mitochondrial donation will not affect the child's appearance, personality or any other features that make a person unique – it will simply allow the mitochondria to function normally and the child to be free of mitochondrial DNA disease. The healthy mitochondria will also be passed on to any children of women born using the technique.

The Lily Foundation has produced an excellent short animation which explains mitochondrial disease: thelilyfoundation.org.uk/animation

The Wellcome Trust has produced a short film about mitochondrial disease and the potential of the new IVF techniques: wellc.me/brokenbatteries

Q&A: Mitochondrial donation

Is it safe?

Safety is and will always be of paramount importance and has received unprecedented scrutiny by the Human Fertilisation and Embryology Authority's (HFEA) specially convened Expert Scientific Review panel. Through three separate reviews, the panel found no evidence to suggest that the techniques are unsafe for clinical use and concluded that both techniques have the potential to be used in patients with mitochondrial disease. Never before has a new reproductive technology been subjected to such thorough investigation before it has been approved.

It is never possible to answer every safety question before new medical procedures are used in people, but the scientific evidence suggests that any risks of mitochondrial donation are proportionate to the severity of mitochondrial disease and the well-recognised significant risk that children will continue to be born who will die in infancy if these techniques are not used.

Will it create 'three-person' babies?

In mitochondrial donation, almost all of the child's genes will come from its biological parents; the mitochondrial donor will only contribute 37 genes (0.1% of total DNA), which enable the mitochondria to produce energy. So whilst there is a genetic contribution from the mitochondrial donor, that contribution is very small. During the HFEA's public dialogue exercise, most people rejected the idea that this is "three-parent IVF". Medical procedures that introduce a donor's biological material into the body are also long accepted. Being a parent involves much more than making a genetic contribution.

Is it Genetic Modification?

The proposed mitochondrial donation treatment techniques only allow for unaltered nuclear DNA to be transferred to an egg or embryo that has unaltered healthy mitochondria. These techniques only replace, rather than alter, a small number of unhealthy genes in the "battery pack" of the cells with healthy ones. Mitochondrial donation does not alter nuclear DNA, which carry the personal characteristics and traits of the person; it will simply allow future generations to be born without mitochondrial DNA disease. It is not, and cannot, be used for 'eugenics' (deliberate alteration of physical traits). Genetic modification of nuclear DNA will remain illegal.

Is it ethical?

The public has been extensively consulted on the ethical acceptability of mitochondrial donation, and there is broad support. The Nuffield Council on Bioethics found that given the benefit to individuals, if shown to be sufficiently safe, the techniques are ethical for families to use. The HFEA's public dialogue exercise found the public to be broadly supportive of mitochondrial donation, and the Department of Health consultation on the regulations reached a supportive conclusion.

What happens next?

If Parliament passes regulations, it will not immediately become possible for clinics to treat patients using mitochondrial donation. As the regulator, the HFEA will be responsible for deciding how and in which cases the treatment can be offered in the UK. Clinicians will need to get approval for each case by applying to the HFEA for permission and it will only be provided at specialist clinics.

This is a tried and tested method of regulating novel reproductive treatments, such as embryo screening: Parliament rules on the ethics, then the expert regulator rules on scientific issues of safety and efficacy. In rapidly advancing reproductive medicine, the HFEA is charged with ensuring robust and responsive regulation of novel reproductive techniques which change people's lives.