

## **Germline Topic: The Efficacy of Mitochondrial Donation to Treat Mitochondrial Diseases** Rep. Nathaniel Smith

Using a method called mitochondrial donation to cure mitochondrial diseases is a tactic that is highly contentious, yet has undergone debate on whether or not it is ethical or not. Mitochondrial diseases typically affect tissues that require lots of energy, such as the brain, heart, muscle, and central nervous system, making these diseases fatal. However, there is no known treatment for patients with mitochondrial disease, which leads scientists to turn towards preventing the transmission of mitochondrial diseases via germline gene replacement therapy.

From the articles that I researched, the conclusion is that there is promise in this methodology, however we are simply not there yet technologically and scientifically, as the ethical and legal limitations of germline modification in humans is strictly prohibited in most of the world. Similar mitochondrial donation in animals that lead to treatment may not directly translate to treatment in humans. The overall long-term safety and effectiveness of these techniques in humans is unknown, and additional scientific research is essential to establish a concrete conclusion on whether or not germline gene replacement therapy for mitochondrial diseases is worth pursuing.

### Bibliography

**Castro, Rosa J.**

**“Mitochondrial replacement therapy: the UK and US regulatory landscapes”**

*Journal of law and the biosciences* vol. 3,3 726-735. 22 Nov. 2016, doi:10.1093/jlb/lsw051  
<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5570689/>

This article brings to the table information about what mitochondrial replacement therapy is, and the current legality of it in the UK and USA. I found this article by searching up for scholarly articles on google, “the efficacy of mitochondrial donation,” and it was the fifth link.

**Dimond, Rebecca.**

**“Social and ethical issues in mitochondrial donation”**

*British medical bulletin* vol. 115,1 (2015): 173-82.  
<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4562371/>

This article brings up the exact process of maternal spindle transfer, which is the key underlying element of mitochondrial diseases. It goes into detail about the controversy behind this methodology. I found this article from another research article, which will be cited below.

**Ishii, Tetsuya.**

**“Germline Genome-Editing Research and Its Socioethical Implications.”**

*Trends in Molecular Medicine, Elsevier Current Trends*, 12 June 2015,  
[www.sciencedirect.com/science/article/pii/S1471491415001070](http://www.sciencedirect.com/science/article/pii/S1471491415001070)

I found this article by searching up scholarly articles on google for “international regulations on germline modification,” where it was the second link. It provided germline modification experiments of targeted genes on monkey, bovine, ovine, porcine, mouse, and rat zygotes. This research provides context on the potential application of similar methodology in humans.