MedicalXpress

Contradiction keeps US policy at standstill on mitochondrial therapy

June 9 2016

Scientists believe they can now remove disease-causing mitochondrial DNA from human embryos, providing new cures for previously untreatable conditions, but the policy signals coming from Washington DC are in stark contradiction, according to a new Viewpoint essay published in *JAMA* June 9, 2016.

On Feb. 3, 2016, the Health and Medicine Division of the National Academies issued a report on mitochondrial replacement therapy (MRT) commissioned by the Food and Drug Administration. The division (formerly known as the Institute of Medicine) recommended that under certain conditions, MRT clinical trials should be allowed to proceed. But just six weeks before, President Obama signed an appropriations bill that included a bit of language essentially forbidding those trials, the *JAMA* authors wrote.

"One big step forward was taken by the IOM report when it concluded that it is "ethically permissible" to embark on first-in-human clinical trials of MRT subject to rigorous safety and efficacy imperatives," wrote Dr. Eli Adashi, former dean of medicine and biologic sciences at Brown University and I. Glenn Cohen, professor of law at Harvard University. "However, two steps back were taken with the enactment of a policy rider which precludes the FDA from further consideration of MRT."

In MRT, when a mother has mitochondria with problematic DNA, scientists propose to replace it in an embryo with that of a donor. The reconstituted embryo would then be implanted in the mother. If

successful, that replacement procedure would ensure that the nuclear DNA of the resulting child would come from mom and dad, but would be paired with mitochondrial DNA (from a third party donor) that would not carry the risk of disease.

Because males do not pass down mitochondria from generation to generation, the National Academies report recommended that trials proceed only in male embryos. That would prevent altering whole lineages.

Meanwhile in the United Kingdom, the government has approved MRT clinical trials and some might begin this year. But despite the National Academies recommending a similar advance to the FDA, legislation has left U.S. policy at a standstill, wrote Adashi and Cohen.

"Whether or not the eventual births of disease-free children in the UK will change congressional hearts and minds remains to be seen," they wrote. "Failing such, progress in the prevention of mitochondrial DNA diseases will remain the domain of a biomedical enterprise an ocean away."

More information: *JAMA* , jama.jamanetwork.com/article.a … px?articleid=2528505

Provided by Brown University