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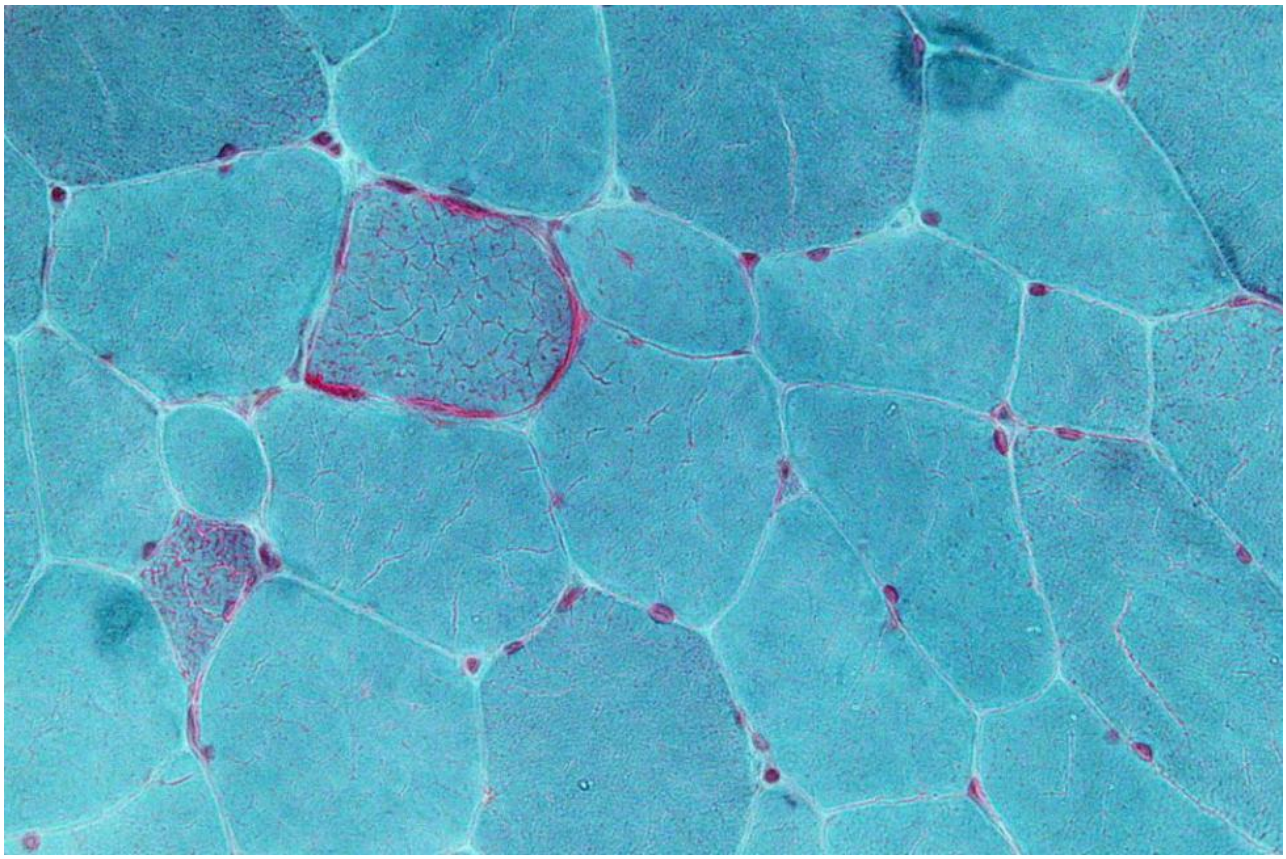

Gene Therapy Can Now Treat Mitochondrial Diseases

BY CHARLES O ON AUGUST 2, 2019

Researchers and scientists in Europe and the U.S. have successfully corrected mitochondrial mutations in live mice by using two gene-editing techniques. The findings were led by researchers at the University of Cambridge in London. The results propose that the tools, including transcription activator-like effector nucleases (TALENs) and zinc-finger nucleases (ZFNs), may one day be used for treating mitochondrial diseases in people.

Illness-causing mutations in mitochondrial DNA (mtDNA) happen in around 1 of every 5,000 adults and result in serious and often fatal conditions ranging from muscle weakness to coronary diseases. Even though three-parent IVF—a dubious technique that uses a third individual's mitochondria notwithstanding a mother's egg nucleus and a father's sperm—has been proposed as an approach to avoid the legacy of mtDNA changes, there are at present no medications for a person born with the deformities.

CRISPR gene-editing approach has achieved extraordinary headway in efforts to alter changes found in nuclear DNA, yet scientists have experienced issues applying a similar approach to mtDNA in light of the fact that mitochondria appear not to take up the guide RNAs that enable the tool to home in on the correct sequence. In the two most recent studies, scientists rather used older gene-editing approaches that don't require guide RNAs.

In one study, the University of Miami's Carlos Moraes and colleagues infused adeno-related infections containing mitochondrial-targeted on TALENs into the muscles of mice carrying a mtDNA change. Following a half year, levels of mutant mtDNA in the creatures' muscle tissue had dropped by more than 50 percent—below the levels generally associated with indications of the mitochondrial disease.

In the other study, led by a group at the University of Cambridge in the UK, scientists infused viruses containing mitochondrial-targeted on ZFNs into the tail veins of the mice to be delivered fundamentally to the heart. More than two months later, the levels of mutant mtDNA had dropped by around 40 percent in heart tissue, the group reports.

“These are momentous discoveries that make it conceivable to even consider doing this in people,” Martin Picard, a mitochondrial researcher at the Columbia University Irving Medical Center who was not engaged with the work, tells Science. The two groups are reportedly intending to dispatch clinical trials, Science reports.

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