MSC-mediated mitochondrial transfer holds therapeutic promise for Leber's hereditary optic neuropathy patients

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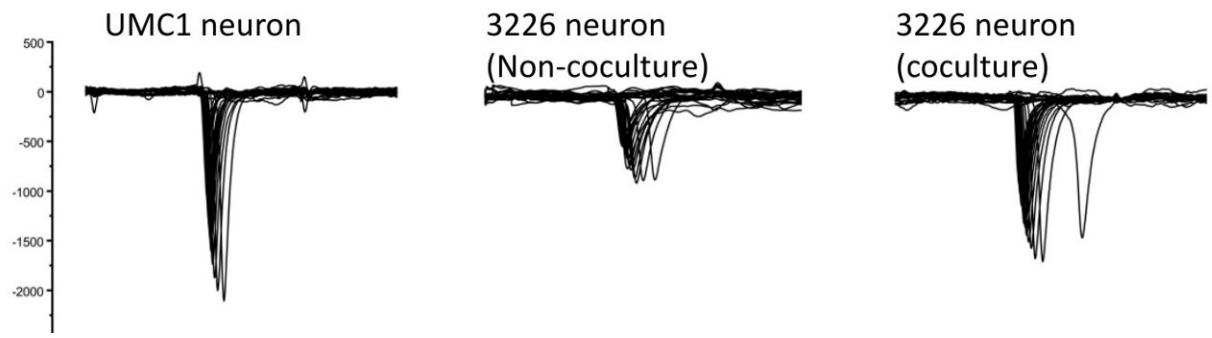

Patch-clamp recordings demonstrated that LHON neurons, which typically exhibit impaired sodium and potassium currents, showed restored electrophysiological function after co-culture with MSCs. Specifically, both inward sodium and outward potassium currents were significantly enhanced in LHON neurons following the mitochondrial transfer from MSCs. Credit: Science China Press

In a recent study published in *Science China Life Sciences*, it has been demonstrated that MSC-mediated mitochondrial transfer can effectively restore mitochondrial DNA (mtDNA) and improve mitochondrial function in neural progenitor cells derived from patients with Leber's hereditary optic neuropathy (LHON).

LHON is a genetic disorder that leads to vision loss and blindness, primarily due to mutations in mtDNA that impair the function of the respiratory chain.

The research team reprogrammed urine cells from LHON patients into induced pluripotent stem cells (iPSCs) and subsequently differentiated them into neural progenitor cells. By co-culturing these neural progenitor cells with MSCs, the study observed significant improvements in mitochondrial function and an increase in the proportion of normal mtDNA.

The results suggest that MSCs can transfer functional mitochondria to neural progenitor cells, thus restoring their function and potentially offering a new therapeutic strategy for mitochondrial diseases. This study provides a promising avenue for future research and clinical applications in treating mitochondrial disorders.

More information: Rui Wang et al, MSC-mediated mitochondrial transfer restores mitochondrial DNA and function in neural progenitor cells of Leber's hereditary optic neuropathy, *Science China Life Sciences* (2024). DOI: 10.1007/s11427-024-2647-8

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