Mitochondrial therapeutics: novel approaches to treating mitochondrial diseases.

Nouros Johns*

Department of Pharmacology and Toxicology, University of Toronto, Toronto, ON M5S 1A8, Canada

Introduction

Mitochondria are organelles found in the cells of most eukaryotic organisms, including humans. They are responsible for producing energy in the form of ATP through the process of oxidative phosphorylation. Mitochondrial dysfunction has been implicated in a range of diseases, including neurodegenerative disorders, metabolic disorders, and cancer. Mitochondrial diseases are a group of genetic disorders caused by mutations in the mitochondrial DNA or nuclear DNA that affect the function of the mitochondria. Currently, there are no effective treatments for mitochondrial diseases. However, researchers are investigating novel approaches to treating mitochondrial diseases, including mitochondrial therapeutics. Mitochondrial therapeutics encompass a broad range of approaches aimed at improving mitochondrial function or replacing damaged or dysfunctional mitochondria. These approaches include gene therapy, small molecule therapeutics, and stem cell therapy [1,2].

Gene therapy is a promising approach for treating mitochondrial diseases caused by mutations in the mitochondrial DNA. Gene therapy involves the delivery of a functional copy of the mutated gene to the affected cells using a viral vector. The functional gene can then be incorporated into the mitochondrial DNA, restoring mitochondrial function. This approach has shown promise in preclinical studies, and clinical trials are underway to evaluate the safety and efficacy of gene therapy for mitochondrial diseases.

Small molecule therapeutics are another approach to treating mitochondrial diseases. Small molecules are compounds that can modulate cellular processes and signaling pathways. Several small molecules have been identified that can improve mitochondrial function and reduce oxidative stress. For example, MitoQ is a small molecule antioxidant that selectively targets mitochondria, reducing oxidative stress and improving mitochondrial function. Another small molecule, idebenone, has been shown to improve energy production in cells affected by mitochondrial diseases. Clinical trials are ongoing to evaluate the efficacy of these small molecule therapeutics for the treatment of mitochondrial diseases.

Stem cell therapy is another promising approach to treating mitochondrial diseases. Stem cells are cells that have the ability to differentiate into different cell types and can regenerate damaged tissues. Researchers have developed methods for generating stem cells from patients with mitochondrial diseases, which can then be differentiated into functional cells, such as neurons or muscle cells. These functional cells can be used for transplantation, replacing damaged or dysfunctional cells and restoring mitochondrial function. However, more research is needed to optimize this approach and ensure its safety and efficacy [3,4].

Mitochondrial replacement therapy is a novel approach to treating mitochondrial diseases that involves replacing damaged or dysfunctional mitochondria with healthy mitochondria from a donor. This approach has been used successfully to prevent the transmission of mitochondrial diseases from mother to child. The technique involves transferring the nucleus of an egg or embryo from a woman with mitochondrial disease into a donor egg or embryo that has had its nucleus removed. The resulting embryo contains nuclear DNA from the mother and father and mitochondrial DNA from the donor. This technique has been used successfully to prevent the transmission of mitochondrial diseases in several cases, but it remains controversial and is not widely available. In addition to these approaches, researchers are investigating other novel therapies for mitochondrial diseases. For example, photobiomodulation therapy involves using light to stimulate mitochondrial function and improve energy production. This approach has shown promise in preclinical studies and clinical trials are underway to evaluate its safety and efficacy. Another approach involves using mitochondrial-targeted peptides to improve mitochondrial function and reduce oxidative stress.

Overall, mitochondrial therapeutics are a promising approach to treating mitochondrial diseases. While there are currently no effective treatments for mitochondrial diseases, researchers are investigating a range of novel approaches, including gene therapy, small molecule therapeutics, stem cell therapy, and mitochondrial replacement therapy. These approaches have shown promise in preclinical studies, and clinical trials are underway to evaluate their safety and efficacy. Mitochondrial therapeutics have the potential to provide new hope for patients with mitochondrial diseases and improve their quality of life. However, more research is needed to optimize these approaches and ensure their safety and One promising approach to treating mitochondrial diseases is gene therapy. This involves introducing a healthy copy of a gene into a patient's cells to replace a defective or mutated gene that is causing the disease. For example, in 2019, the first successful

***Correspondence to:** Nouros Johns, Department of Pharmacology and Toxicology, University of Toronto, Toronto, ON M5S 1A8, Canada, E-mail: john@torn.ca

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gene therapy clinical trial for a mitochondrial disease called Leber hereditary optic neuropathy (LHON) was conducted. In this trial, a healthy copy of a gene called ND4 was introduced into the mitochondria of patients with LHON, resulting in improved vision for some of the patients.

Another approach is to use small molecules or drugs to target specific aspects of mitochondrial function. For example, some drugs have been developed that can improve mitochondrial biogenesis, the process by which new mitochondria are produced. Other drugs can increase the production of ATP, the primary source of energy for cells, by targeting enzymes involved in the electron transport chain [5].

There is also growing interest in using dietary interventions to improve mitochondrial function. For example, some studies have shown that a ketogenic diet, which is high in fat and low in carbohydrates, can improve mitochondrial function and increase energy production. Other dietary interventions, such as fasting and calorie restriction, have also been shown to improve mitochondrial function in some cases.

In addition to these approaches, there is ongoing research into stem cell therapies for mitochondrial diseases. Stem cells have the potential to differentiate into various cell types, including cells that make up different tissues in the body, including the brain, heart, and muscle. By transplanting healthy stem cells into a patient with a mitochondrial disease, it may be possible to replace damaged cells and improve overall function. Overall, there are many different approaches being explored for treating mitochondrial diseases, and it is likely that a combination of these approaches will be needed to effectively treat these complex and often debilitating conditions.

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