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Gene Technology

Opinion Article

Restoring Mitochondrial Function: The Role of Gene Therapy in *dGK* Deficiency

Isabella Cooper*

Department of Biotechnology, University of California, San Francisco, United States of America

DESCRIPTION

Gene therapy has become a potential approach for treating genetic disorders, particularly those caused by enzyme deficiencies. One such disorder is deoxyguanosine Kinase (*dGK*) deficiency, a rare metabolic disorder that leads to impaired purine metabolism and mitochondrial dysfunction in the liver. Recent studies have demonstrated that gene therapy can effectively prevent hepatic mitochondrial dysfunction in murine models of *dGK* deficiency, providing new hope for treating this otherwise devastating condition. This breakthrough provides valuable insights into the potential of gene therapy for correcting metabolic imbalances and improving mitochondrial function in inherited disorders.

Deoxyguanosine kinase plays a critical role in purine metabolism, specifically in the phosphorylation of deoxyguanosine, which is necessary for DNA replication and repair. In individuals with *dGK* deficiency, this enzyme is either absent or nonfunctional, leading to an accumulation of deoxyguanosine in tissues and subsequent mitochondrial dysfunction, particularly in the liver. Mitochondria are the driving force of the cell, responsible for energy production and their dysfunction can lead to a wide range of metabolic disturbances, including liver damage, altered energy production and increased oxidative stress.

Hepatic mitochondrial dysfunction is a defining characteristic of *dGK* deficiency and it significantly impacts the overall health of affected individuals. This dysfunction is primarily driven by the accumulation of deoxyguanosine, which disrupts the mitochondrial respiratory chain, leading to impaired oxidative phosphorylation and reduced ATP production. As a result, affected cells and tissues suffer from energy deficits and cellular damage accumulates over time. This can ultimately lead to liver failure, highlighting the need for effective therapeutic strategies to address the root cause of the disease.

Gene therapy provides a powerful solution to address this issue. By delivering a functional copy of the *dGK* gene to the liver, gene therapy can restore the enzyme's activity, thereby correcting the the metabolic imbalance and preventing mitochondrial dysfunction. Recent studies in murine models have shown that viral vectors can be used to efficiently deliver the *dGK* gene to the liver, leading to sustained expression of the enzyme and significant improvements in mitochondrial function. This gene therapy approach has been shown to prevent the accumulation of deoxyguanosine, restore normal mitochondrial function and reduce oxidative stress in the liver.

The ability of gene therapy to prevent hepatic mitochondrial dysfunction in *dGK* deficiency highlights its potential as a therapeutic strategy for other metabolic disorders that involve mitochondrial dysfunction. While *dGK* deficiency is rare, it shares similarities with other conditions that affect mitochondrial function, such as certain inherited mitochondrial diseases and metabolic syndromes. The success of gene therapy in this context could create the path for broader applications in the treatment of mitochondrial diseases, many of which currently have no effective therapies.

There are several reasons why gene therapy is particularly well-suited for treating diseases like *dGK* deficiency. First, gene therapy provides the potential for a long-lasting, if not permanent, correction of the root cause of the genetic defect. In the case of *dGK* deficiency, a single administration of the gene therapy has been shown to result in sustained expression of the *dGK* enzyme in the liver, leading to lasting improvements in mitochondrial function and overall metabolic health. This is a significant advantage over traditional treatments, which often require continuous intervention and may not address the root cause of the disease.

In conclusion, gene therapy holds immense potential for preventing and potentially curing diseases associated with mitochondrial dysfunction, such as deoxyguanosine kinase deficiency. By restoring normal enzyme function in the liver, gene therapy can prevent the accumulation of toxic metabolites and correct the mitochondrial dysfunction that drives disease progression. While challenges remain, particularly in terms of efficient gene delivery and personalized treatment approaches,

Correspondence to: Isabella Cooper, Department of Biotechnology, University of California, San Francisco, United States of America, E-mail: isabella.cooper@ucsf.edu

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the success of gene therapy in murine models is an exciting development. With continued research and refinement, gene therapy could provide a transformative solution for patients with *dGK* deficiency and similar metabolic disorders, marking a new era in the treatment of mitochondrial diseases and genetic conditions.