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Singapore discovers novel gene therapy offering hope for epilepsy patients

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Novel gene therapy approach for a rare genetic form of epilepsy linked to a mutation in the KCNA2 gene in the human brain



Researchers from the Yong Loo Lin School of Medicine, National University of Singapore (NUS Medicine) are working on a therapy that holds potential in treating patients with epilepsy, a neurological disorder defined by recurring seizures due to abnormal brain activity.

The team has trialled a novel gene therapy approach for a rare genetic form of epilepsy linked to a mutation in the KCNA2 gene in the human brain, which is associated with recurring seizures.

A specialised treatment called a Gapmer antisense oligonucleotide (ASO) is designed to specifically target and break down faulty ribonucleic acids (RNA) while keeping normal gene function intact. Using this RNA therapy led to a notable decrease in a problematic potassium channel protein encoded in the KCNA2 gene, which helped restore normal potassium flow and reduce excessive neuron activity linked to epilepsy.

The new Gapmer technology being worked on by the research team could also be adapted to target other mutations in the same gene or other ion channel genes, opening the possibility of creating personalised treatments for different KCNA2-related issues, potentially offering hopeful outcomes for patients with rare forms of epilepsy that are unresponsive to standard medications.

Professor Soong Tuck Wah from the Department of Physiology and Electrophysiology Core Facility at NUS Medicine, a co-author of the study, said, “Our research seeks not only to address the unique challenges posed by this specific mutation, but also stems from our team’s desire to improve the quality of life for patients. Since the therapy has shown promise in targeting a specific gene mutation causing epilepsy, we hope to eventually pioneer new treatment options for patients suffering from this condition, and other similar gene mutations.”