Precision In Vivo Gene Editing Using Dual-Vector Delivery Systems

Tech ID: 33751 / UC Case 2024-122-0

TECHNOLOGY DESCRIPTION

Current gene therapy techniques often face challenges such as off-target effects, limited delivery to specific cell types, and immune rejection. There is a critical need for more precise, efficient, and safe methods to edit genes directly within the human body, ensuring that therapies are both effective and minimally invasive. This innovative therapeutic approach addresses a broad spectrum of genetic disorders, including cancers, autoimmune diseases, and inherited genetic disorders where precise genomic editing is crucial for treatment.

The product that could be developed from this technology is a dual-vector system for in vivo gene editing. It can be tailored to target various immune cells using cell-specific binding molecules.

Our dual-vector system uniquely integrates several competitive advantages:

Cell-Type Specificity: The inclusion of cell-specific binding molecules enables precise targeting of the therapy to desired cell types, reducing off-target effects and enhancing safety.

Enhanced Delivery Efficiency: Utilizes vectors that increase the efficiency of vector entry into cell nuclei.

Reduced Immune Response: The use of capsid variants with reduced antibody-mediated neutralization minimizes immune rejection, a common hurdle in gene therapy.

Versatile Editing Capabilities: The platform can be adapted to different genes and cell types by simply changing the guide RNA and the binding molecule, making it widely applicable across various diseases.

PATENT STATUS

Patent Pending

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OTHER INFORMATION

KEYWORDS

In Vivo Gene Editing, Dual-Vector

CATEGORIZED AS

- Medical
  - Disease: Autoimmune and Inflammation
  - Disease: Cancer
  - Gene Therapy
  - Therapeutics

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2024-122-0, 2024-127-0

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