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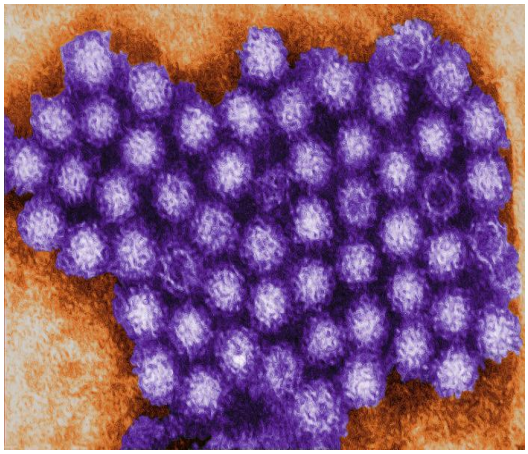
City of Hope

NATIONAL MEDICAL CENTER AND
BECKMAN RESEARCH INSTITUTE

Lentiviral Vector for Site-Specific Gene Integration



DESCRIPTION

Most viral vectors that have previously been used to deliver genes to hematopoietic stem cells (HSCs) integrate randomly into the host genome, leading to disruption or inactivation of vital host genes. The present invention discloses a novel lentiviral vector gene delivery system engineered to overcome this problem, through integration into the host genome in a site-specific manner. The vector includes a lentiviral genomic sequence, the gene to be inserted, and flanking sequences recognized by a sequence-specific endonuclease. The endonuclease is capable of coupling cleavage of the vector with cleavage of the host genome and facilitating the site-specific recombination between the vector and the host genome. This invention creates a safer approach to gene delivery that provides high levels of durable gene expression.

KEY ASPECTS

- This technology has broad applications in the delivery of therapeutic genes to treat an unlimited array of therapeutic indications
- Specific target sequences direct the site-specific endonuclease to insert desired gene in a defined site within host genome
- This vector system is be engineered to stimulate the homologous recombination of a desired gene with host genome

INTELLECTUAL PROPERTY

Title	US Patent Number	Issued
Novel Lentiviral Vectors for Site-Specific Gene Insertion	7,402,436	7/22/2008
Novel Lentiviral Vectors for Site-Specific Gene Insertion	7,531,647	5/12/2009

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