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(54) Title: GENE THERAPY VECTORS FOR TREATMENT OF DANON DISEASE

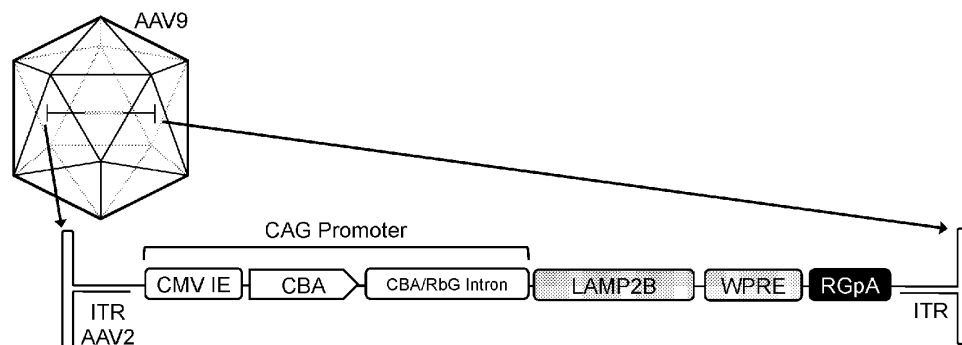


FIG. 1A

(57) Abstract: The invention relates generally to gene therapy for diseases associated with mutations in lysosome-associated membrane protein 2 (LAMP-2, also known as CD107b). In one aspect, the disclosure provides a gene therapy vector comprising an expression cassette comprising a transgene encoding an isoform of LAMP-2 or a functional variant thereof, wherein the transgene is codon-optimized for expression in a human host cell. In another aspect, the disclosure provides methods of preventing, mitigating, ameliorating, reducing, inhibiting, eliminating and/or reversing one or more symptoms of Danon disease or another autophagy disorder in a subject in need thereof, comprising administering to the subject any gene therapy vector of the disclosure.

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**Declarations under Rule 4.17:**

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