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(54) Title: GENE THERAPY FOR LAMIN A - ASSOCIATED DEFICIENCIES

(57) Abstract: Provided herein is a recombinant AAV (rAAV) comprising an AAV capsid and a vector genome packaged therein, wherein the vector genome comprises an AAV 5' inverted terminal repeat (ITR), an expression cassette comprising an open reading frame (ORF) for a mature human Lamin A (hLaminA) under control of a regulatory sequence which direct expression of mature hLaminA in a target cell, and an AAV 3' ITR. Also provided is a pharmaceutical composition comprising a rAAV as described herein in a formulation buffer, and a method of treatment of idiopathic dilated cardiomyopathy (DCM) or a disease associated with a mutation in a Lamin A (LMNA) gene.



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