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(54) Title: GENE THERAPY FOR TREATMENT OF MUCOPOLYSACCHARIDOSIS IIIA

(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 engineered nucleic acid sequence encoding a functional hSGSH, optionally comprising stabilizing amino acid changes, a regulatory sequence which direct expression of hSGSH 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 treating a human subject diagnosed with MPS IIIA.