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(54) **Title:** NUCLEIC ACID THERAPEUTICS FOR GENETIC DISORDERS

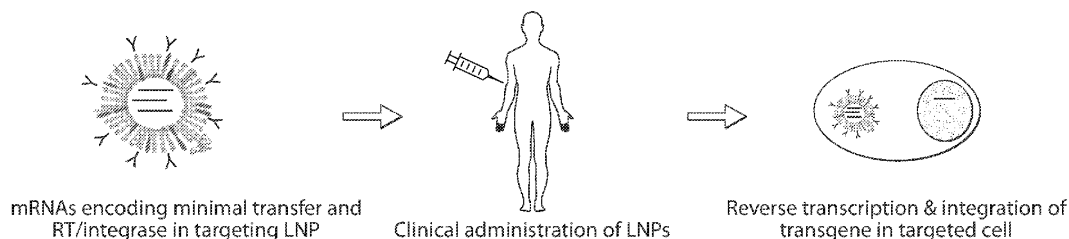


FIG. 1

(57) **Abstract:** Provided herein, are compositions based on retroviruses (e.g., lentiviruses) comprising one or more nucleic acid molecules encoding retroviral Pol polypeptide components and a nucleic acid molecule comprising one or more transgene sequences flanked by long terminal repeat sequences, for delivery of the one or more transgenes to a target cell *ex vivo* or *in vivo*. The compositions are useful for delivering to a target cell (e.g., hematopoietic stem cells (HSCs), liver cells, ocular cells, muscle cells, epithelial cells, T cells, etc.) and/or stably expressing any transgene (e.g., beta-globin, Factor VIII, RP GTPase regulator (RPGR), dystrophin, cystic fibrosis transmembrane conductance regulator (CFTR), a chimeric antigen receptor, etc.) with a biological effect to treat and/or ameliorate the symptoms associated with any disorder related to gene expression (e.g., sickle cell disease, beta-thalassemia, haemophilia B, retinitis pigmentosa, Duchenne muscular dystrophy, cystic fibrosis, cancer, etc.).



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