GENE THERAPY FOR MUCOPOLYSACCHARIDOSIS IIIB

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 human N-acetyl-alpha-glucosaminidase (hNAGLU), a regulatory sequence which direct expression of hNAGLU 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 IIIB.

Figure for publication: None