COMPOSITIONS USEFUL FOR TREATING GM1 GANGLIOSIDOSIS

ABSTRACT

A therapeutic regimen useful for treatment of GM1 gangliosidosis comprising administration of a recombinant adeno-associated virus (rAAV) vector having an AAV capsid and a vector genome comprising a sequence encoding human β-galactosidase is provided. Also provided are compositions containing a rAAV vector and methods of treating GM1 gangliosidosis in patient comprising administration of a rAAV vector.

FIG 1A