ABSTRACT

RAAV-GUANYLATE CYCLASE COMPOSITIONS AND METHODS FOR TREATING LEBER'S CONGENITAL AMAUROSIS-1 (LCA1)

Disclosed are viral vector compositions comprising polynucleotide sequences that express one or more biologically-active mammalian guanylate cyclase proteins. Also disclosed are methods for their use in preventing, treating, and/or ameliorating at least one or more symptoms of a disease, disorder, abnormal condition, or dysfunction resulting at least in part from a guanylate cyclase deficiency in vivo. In particular embodiments, the use of recombinant adeno-associated viral (rAAV) vectors to treat or ameliorate symptoms of Leber's congenital amaurosis, as well as other conditions caused by an absence or reduction in the expression of a functional retinal-specific guanylate cyclase 1 (retGC1).

[Fig. 22A]