DELIVERY, USE AND THERAPEUTIC APPLICATIONS OF THE CRISPR-CAS SYSTEMS AND COMPOSITIONS FOR GENOME EDITING

ABSTRACT

We describe a composition comprising a CRISPR-Cas system for treating an ocular or auditory genetic disease, in which the CRISPR-Cas system comprises: a CRISPR-Cas system RNA or a polynucleotide encoding the CRISPR-Cas system RNA, in which the CRISPR-Cas system RNA comprises: (a) a guide sequence capable of hybridizing to a target sequence associated with the ocular or auditory genetic disease, (b) a tracr mate sequence, and (c) tracr sequence; and a Cas9 or a polynucleotide encoding the Cas9; in which the composition is formulated for localized administration to a subject's eye or cochlea, and in which when locally administered the CRISPR-Cas system is capable of forming a CRISPR-Cas complex comprising the Cas9 and the CRISPR-Cas system RNA.

No Figure to Accompany Abstract