POLYNUCLEOTIDE COMPOSITIONS, RELATED FORMULATIONS, AND METHODS OF USE THEREOF

ABSTRACT

Compositions of polynucleotide(s), pharmaceutical compositions thereof, and methods of use thereof are disclosed. A polynucleotide may encode for a cystic fibrosis transmembrane conductance regulator (CFTR) protein or a functional fragment thereof. The polynucleotide may be assembled with a lipid composition for targeted delivery to a cell or an organ, such as a lung cell or a lung of a subject. Methods for enhancing an expression or activity of CFTR protein in a cell are provided. Methods for treating a subject having or suspected of having a CFTR-associated condition are also provided.

[Figure 11A]