POLYNUCLEOTIDE COMPOSITIONS, RELATED FORMULATIONS, AND METHODS OF USE THEREOF

ABSTRACT

Compositions of polynucleotide(s) are disclosed. A polynucleotide may encode for a polypeptide, protein, or functional fragment thereof associated with primary ciliary dyskinesia (PCD), such as dynein axonemal intermediate chain 1 (DNAI1). Pharmaceutical compositions, kits, and methods for treating a disease or condition associated with cilia maintenance and function, and impaired function of the axoneme are also disclosed. The polynucleotide may be assembled with a lipid composition for delivery to an organ, such as the lung, of a subject. The lipid composition may comprise an ionizable cationic lipid. The polynucleotide can be expressed within cells of the organ of the subject.

[Figure 30C]