MUSCLE TARGETING COMPLEXES AND USES THEREOF FOR TREATING MYOTONIC DYSTROPHY

ABSTRACT

The present application relates to oligonucleotides (e.g., antisense oligonucleotides such as gapmers) designed to target DMPK RNAs and targeting complexes for delivering the oligonucleotides to cells (e.g., muscle cells) and uses thereof, particularly uses relating to treatment of disease. In some embodiments, the muscle-targeting agent specifically binds to an internalizing cell surface receptor on muscle cells. In some embodiments, the molecular payload inhibits expression or activity of DMPK.

FIG. 1A