PEPTIDE-LINKED MORPHOLINO ANTISENSE OLIGONUCLEOTIDES FOR TREATMENT OF MYOTONIC DYSTROPHY

Abstract

Provided herein are peptide-linked morpholino (PPMO) antisense oligonucleotides that target the poly CUG repeat tract in the 3' untranslated region of the gene encoding dystrophia myotonica-protein kinase (DMPK) and methods for systemic administration of the same for the treatment of mytonic dystrophy type I (DM1).

[No figure]