OLIGONUCLEOTIDES AND COMPOSITIONS THEREOF FOR NEUROMUSCULAR DISORDERS

ABSTRACT

Disclosed herein are engineered DUX4-targeting oligonucleotides for selective inhibition of RNA transcripts associated with a neuromuscular disease such as facioscapulohumeral muscular dystrophy. Also disclosed are vectors containing any of these, pharmaceutical formulations containing any of the these, and kits containing any of the these. Also disclosed herein are methods of selectively inhibiting polypeptide expression and activity by contacting a DUX4-targeting oligonucleotide with an RNA transcript associated with a neuromuscular disease such as facioscapulohumeral muscular dystrophy.

[Figure 1]