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(54) Title: TARGETED GENE THERAPY FOR DM-1 MYOTONIC DYSTROPHY

(57) Abstract: Provided herein are RNAi molecules for treating myotonic dystrophy type 1 (DM1). Further provided herein are expression cassettes, vectors (*e.g.*, rAAV), viral particles, and pharmaceutical compositions containing the RNAi. Yet further provided herein are methods and kits related to the use of the RNAi, for example, to treat DM1.