ADENO-ASSOCIATED VIRUS VECTOR DELIVERY OF MUSCLE SPECIFIC MICRO-DYSTROPHIN TO TREAT MUSCULAR DYSTROPHY

ABSTRACT

The invention provides gene therapy vectors, such as adeno-associated virus (AAV) vectors, expressing a miniaturized human micro-dystrophin gene and method of using these vectors to express micro-dystrophin in skeletal muscle s including diaphragm and cardiac muscle and to protect muscle fibers from injury, increase muscle strength and reduce and/or prevent fibrosis in subjects suffering from muscular dystrophy.

[FIG. 1]