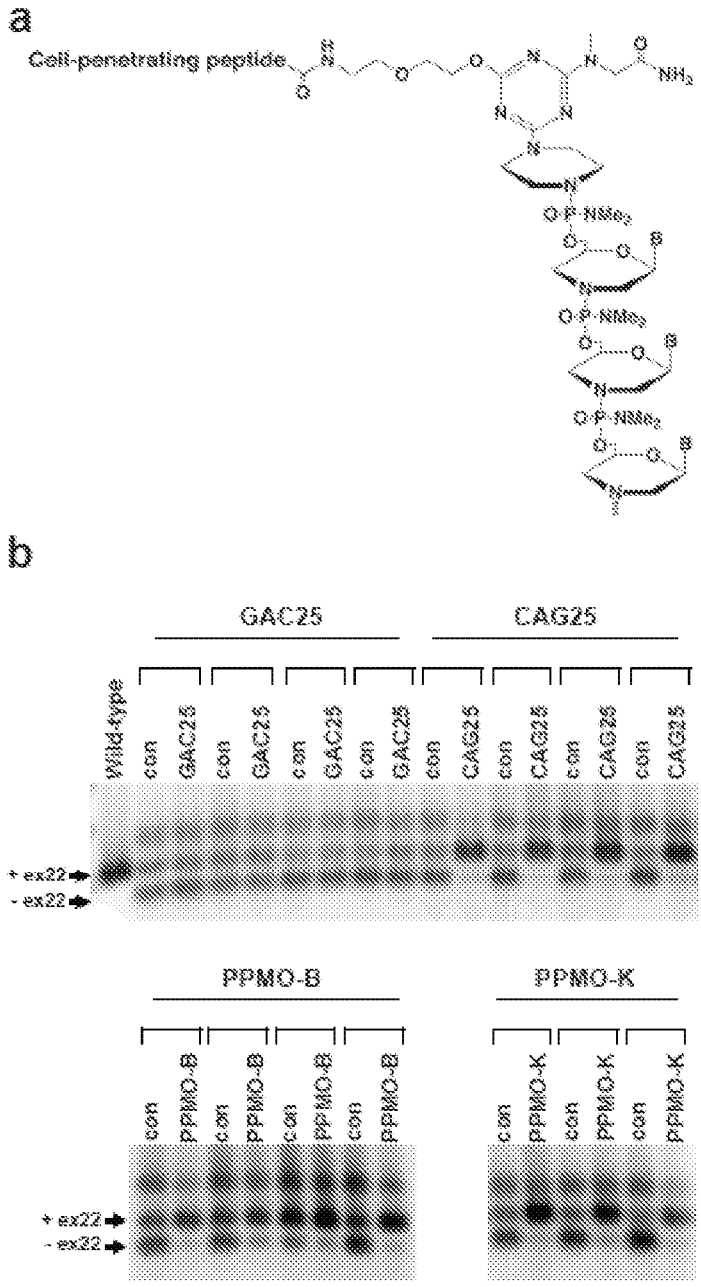

a
Cell-penetrating peptide
b
GAC25
CAG25
Wild-type
con
GAC25
CAG25
+ ex22
- ex22
PPMO-B
PPMO-K

Figure 1

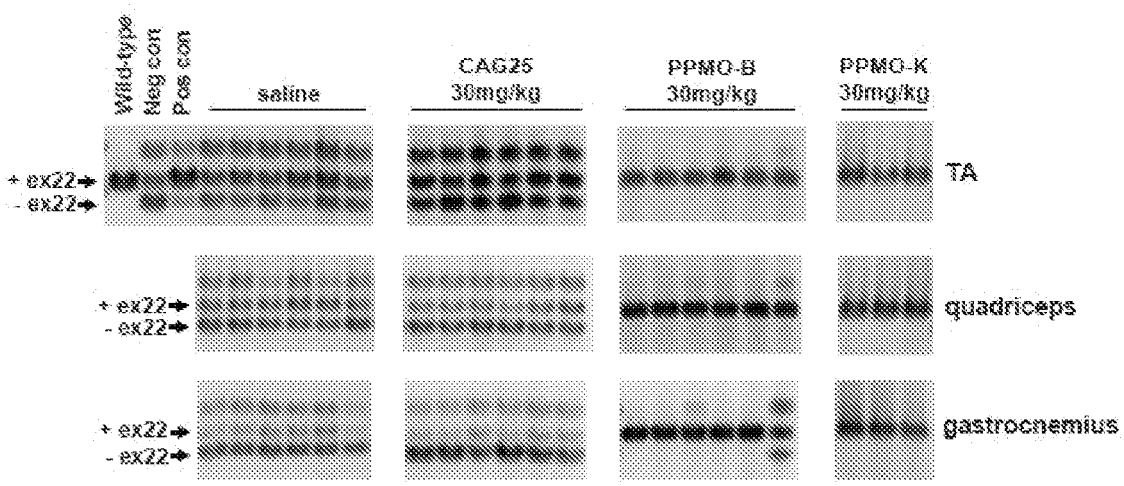

Wild-type
Neg con
Pos con
saline
CAG25
30mg/kg
PPMO-B
30mg/kg
PPMO-K
30mg/kg
+ ex22
- ex22
TA
quadriceps
gastrocnemius

Figure 2

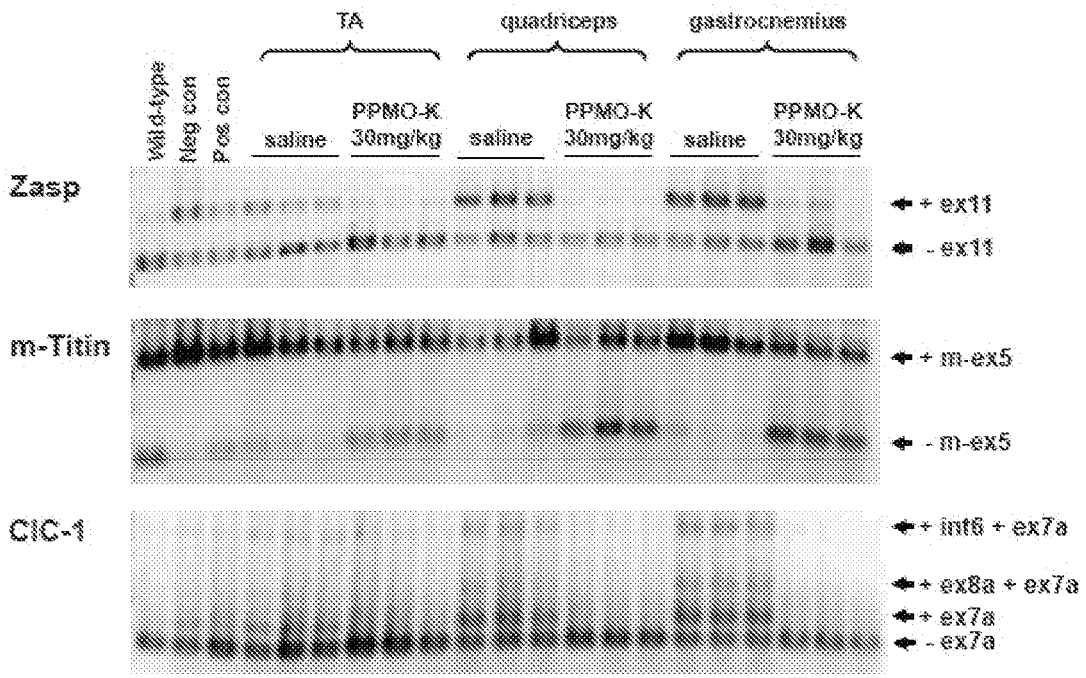

TA
quadriceps
gastrocnemius
Wild-type
Neg con
Pos con
saline
PPMO-K 30mg/kg
saline
PPMO-K 30mg/kg
saline
PPMO-K 30mg/kg
Zasp
+ ex11
- ex11
m-Titin
+ m-ex5
- m-ex5
CIC-1
+ int6 + ex7a
+ ex8a + ex7a
+ ex7a
- ex7a

Figure 3

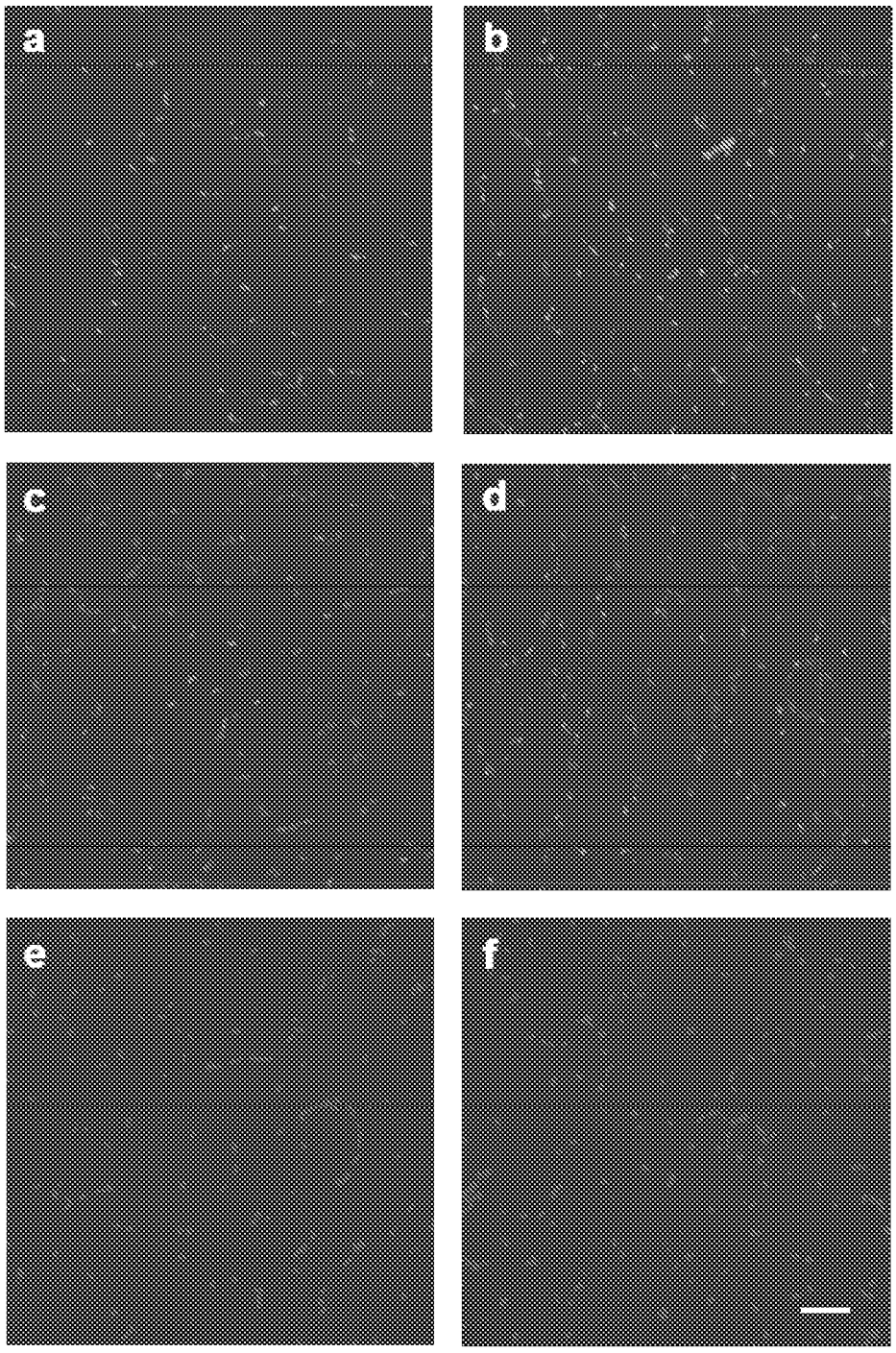


Figure 4

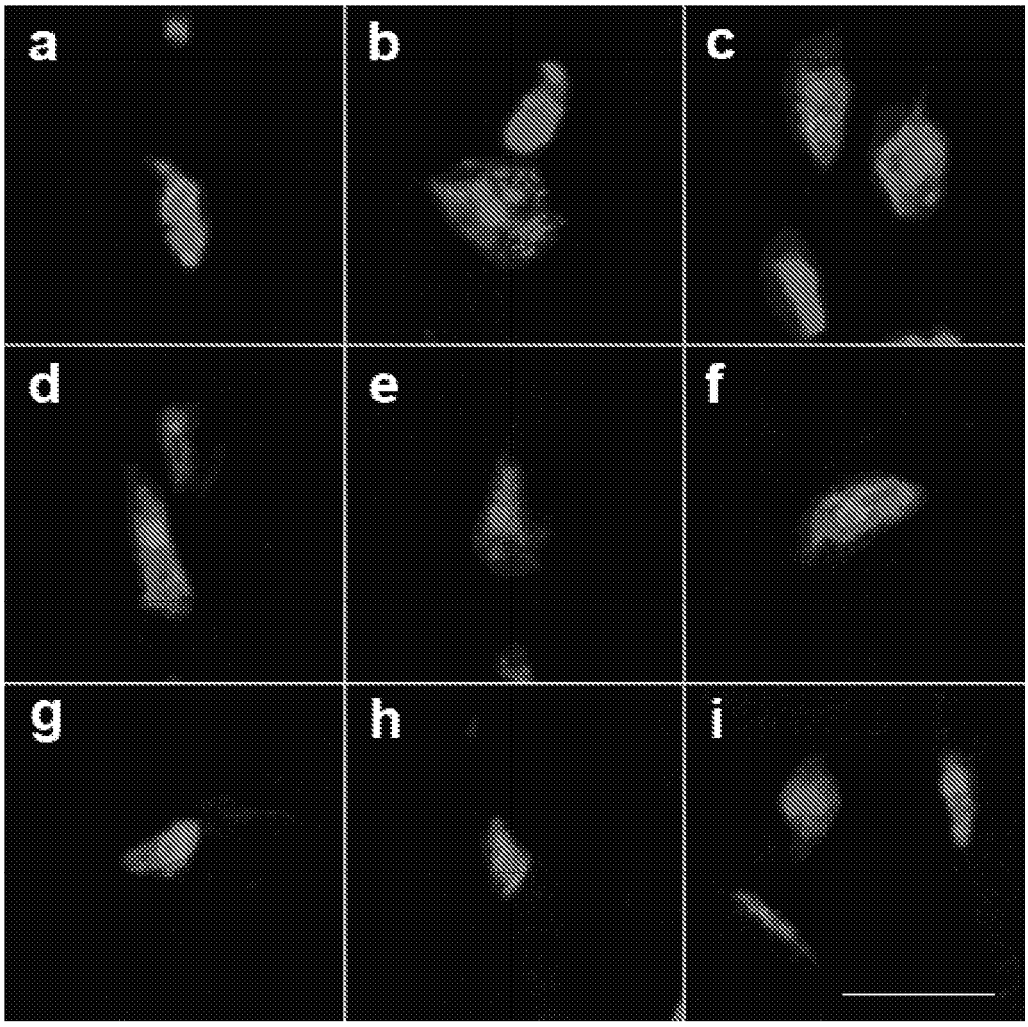


Figure 5

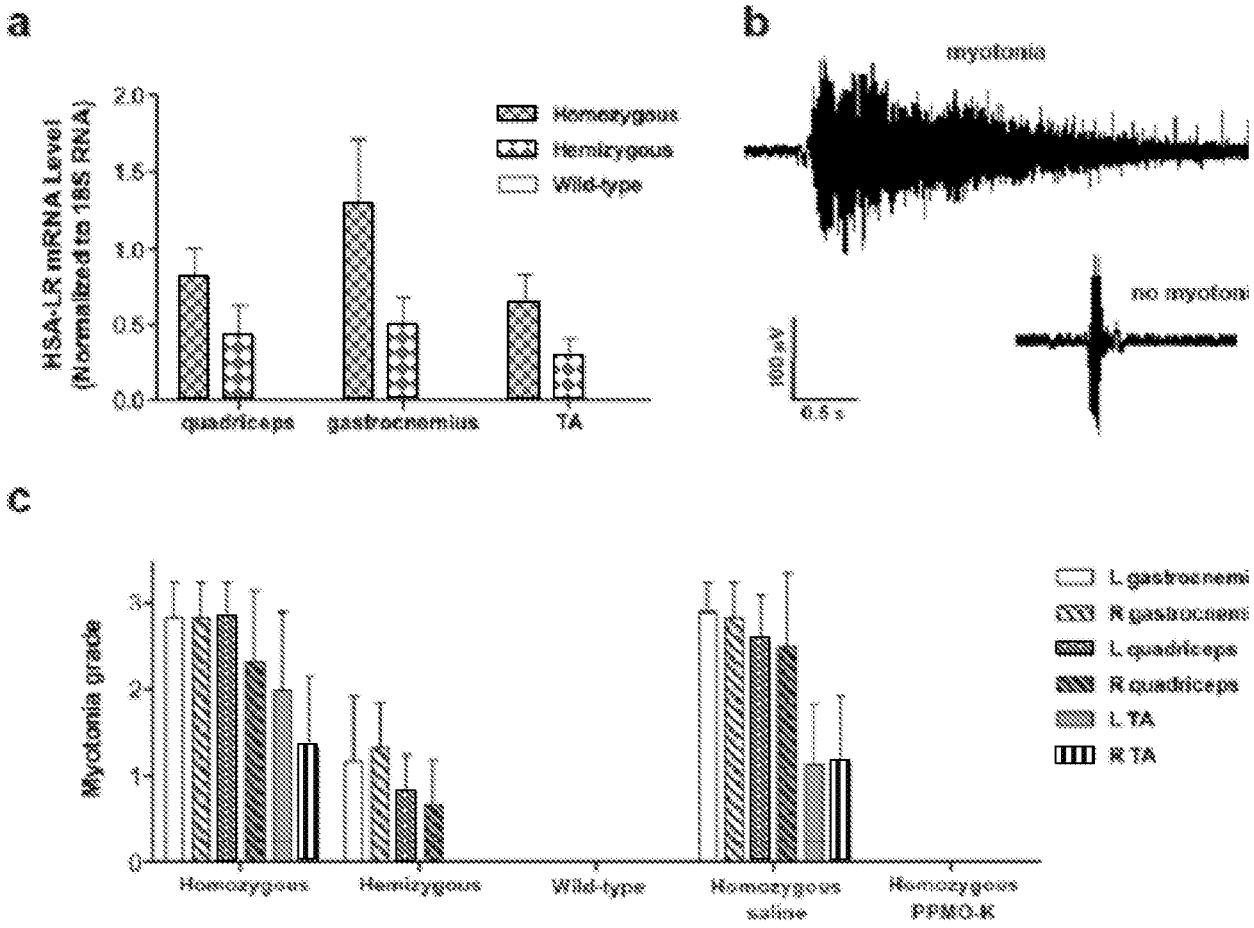

a
HSA-LR mRNA Level
(Normalized to 18S RNA)
2.0
1.5
1.0
0.5
0.0
Homozygous
Hemizygous
Wild-type
quadriceps
gastrocnemius
TA
b
myotonia
no myotonia
100 μV
0.5 s
c
Myotonia grade
3
2
1
0
L gastrocnemi
R gastrocnemi
L quadriceps
R quadriceps
L TA
R TA
Homozygous
Hemizygous
Wild-type
Homozygous
saline
Homozygous
PPMO-K

Figure 6