FIG. 1

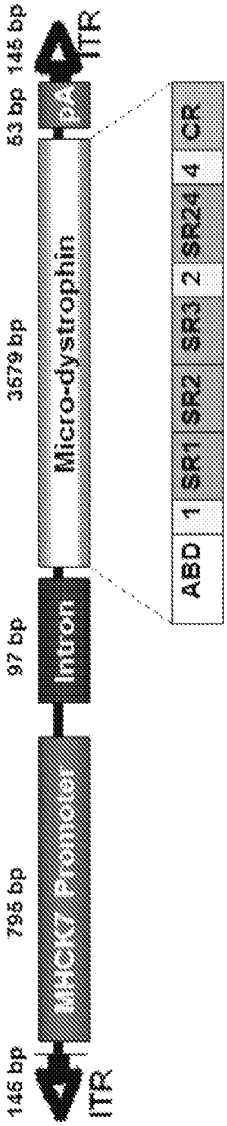

146 bp
ITR
795 bp
MHCK7 Promoter
97 bp
Intron
3679 bp
Micro-dystrophin
53 bp
pA
145 bp
ITR
ABD
1
SR1
SR2
SR3
2
SR24
4
CR

FIG. 2

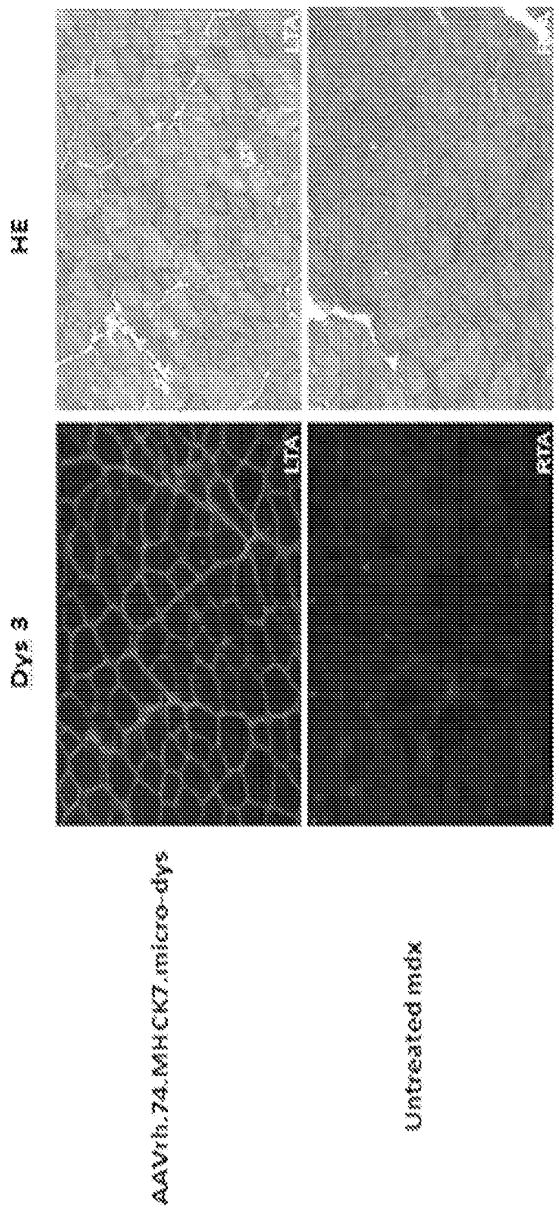


FIG. 3C

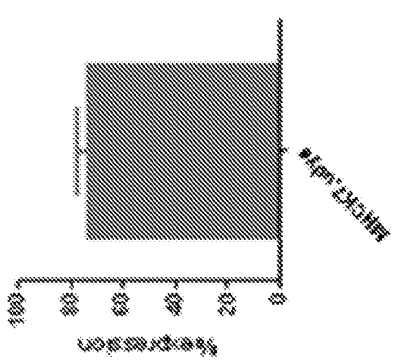


FIG. 3B

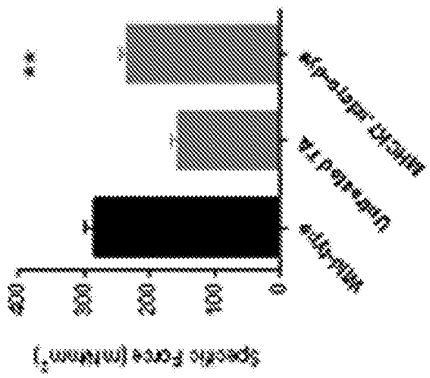
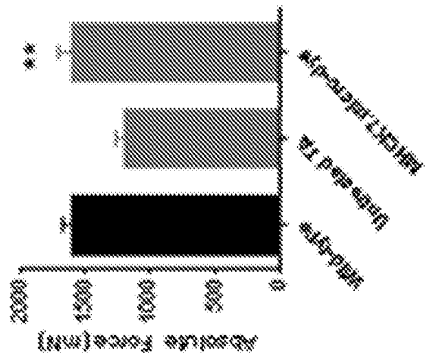


FIG. 3A



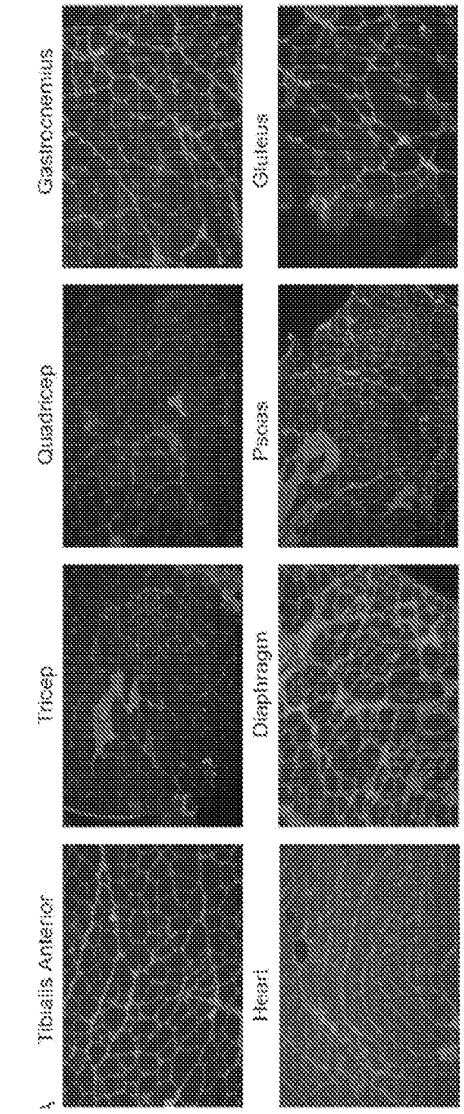


FIG. 4A

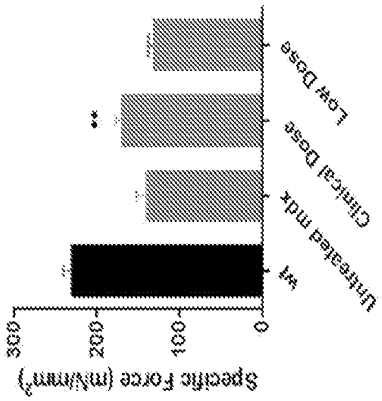


FIG. 4C

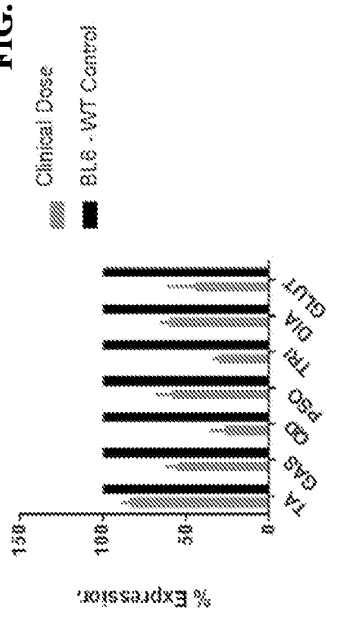


FIG. 4B

FIG. 5

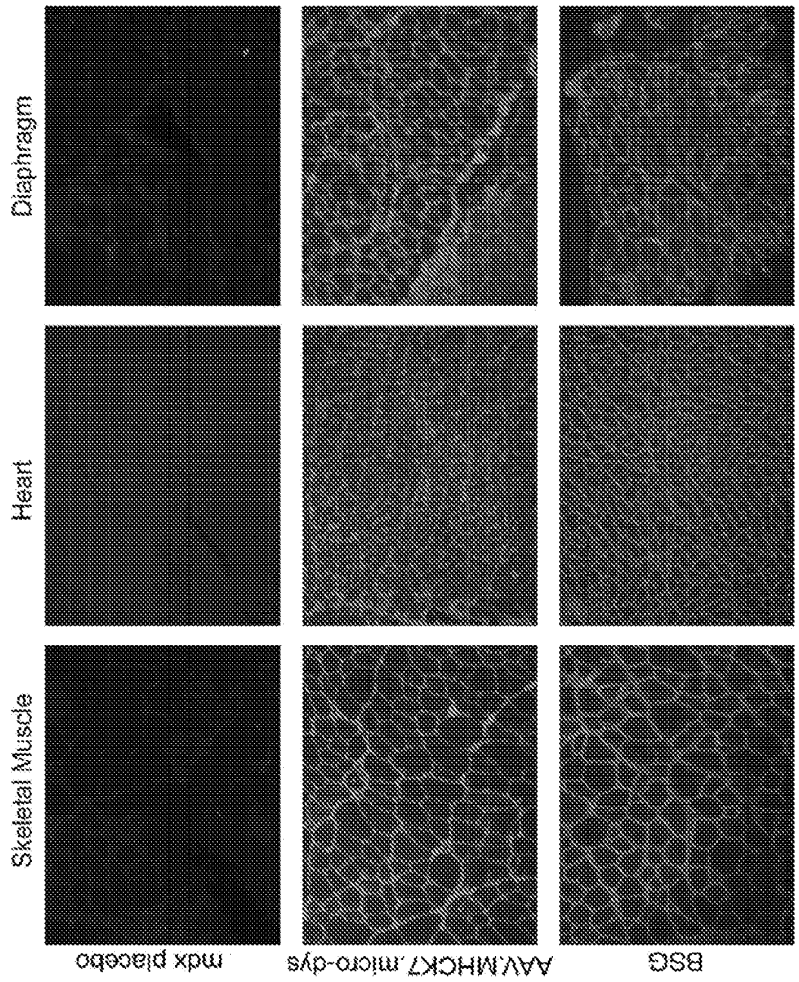

Diaphragm
Heart
Skeletal Muscle
mdx placebo
AAV.MHCK7.micro-dys
BSG

FIG. 6A

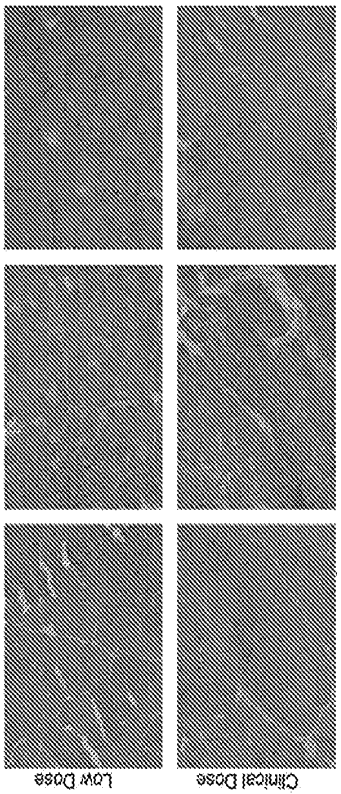


FIG. 6B

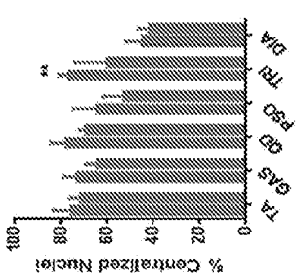


FIG. 6C

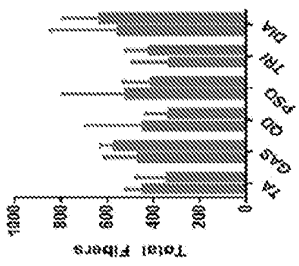


FIG. 6D

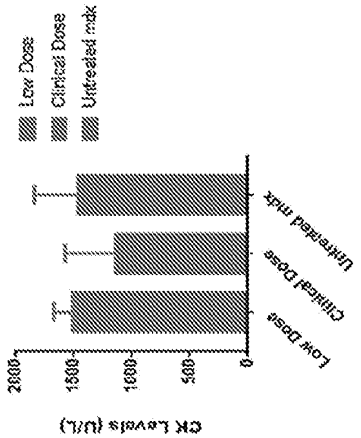


FIG. 7

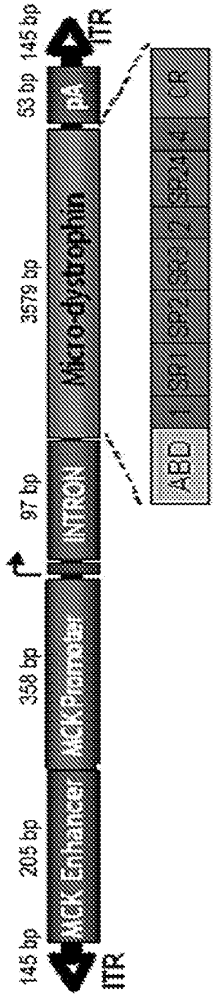
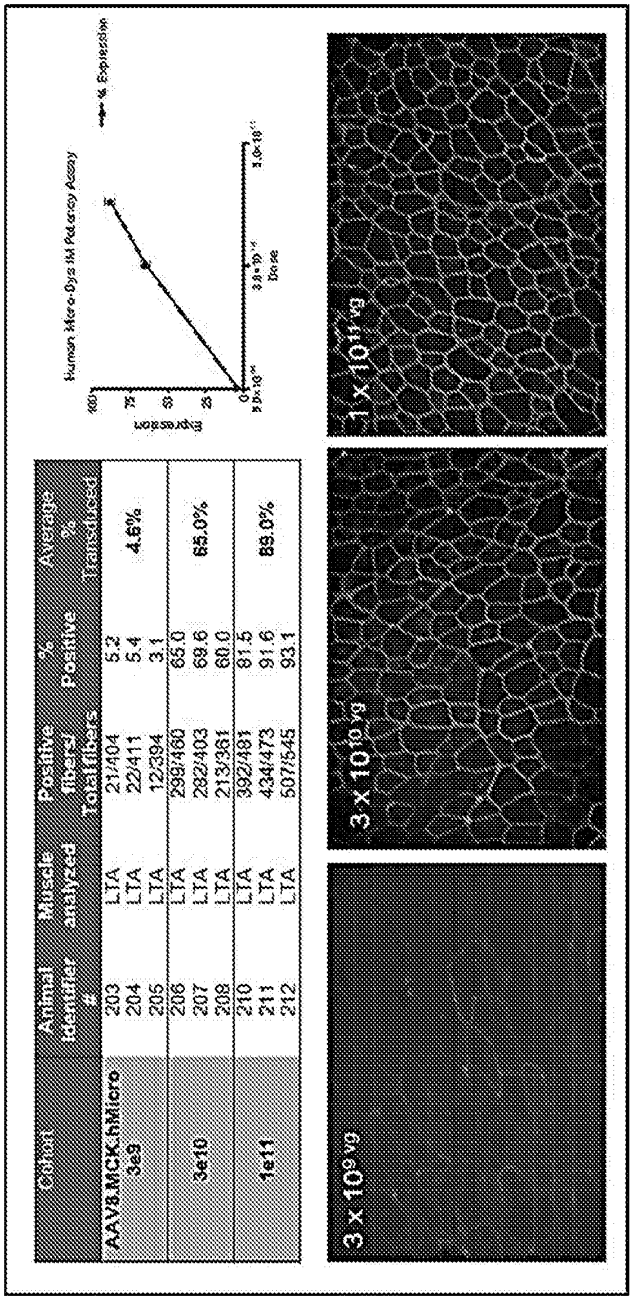

145 bp
ITR
53 bp
pA
3579 bp
Micro-dystrophin
97 bp
INTRON
358 bp
MCK Promoter
205 bp
MCK Enhancer
145 bp
ITR
ABD
R

FIG. 8


Cohort
Animal Identifier #
Muscle Analyzed
Positive fibers/ Total fibers
% Positive
Average % Transduced
AAV8.MCK.hMicro 3e9
203 LTA 21/404 5.2
204 LTA 22/411 5.4 4.6%
205 LTA 12/394 3.1
3e10
206 LTA 298/460 65.0
207 LTA 282/403 69.6 65.0%
208 LTA 213/381 60.0
1e11
210 LTA 392/481 81.5
211 LTA 434/473 91.6 86.0%
212 LTA 507/545 93.1
Human Micro-Dys IM Potency Assay
% Expression
Expression
Dose
3 x 10^9 vg
3 x 10^10 vg
1 x 10^11 vg

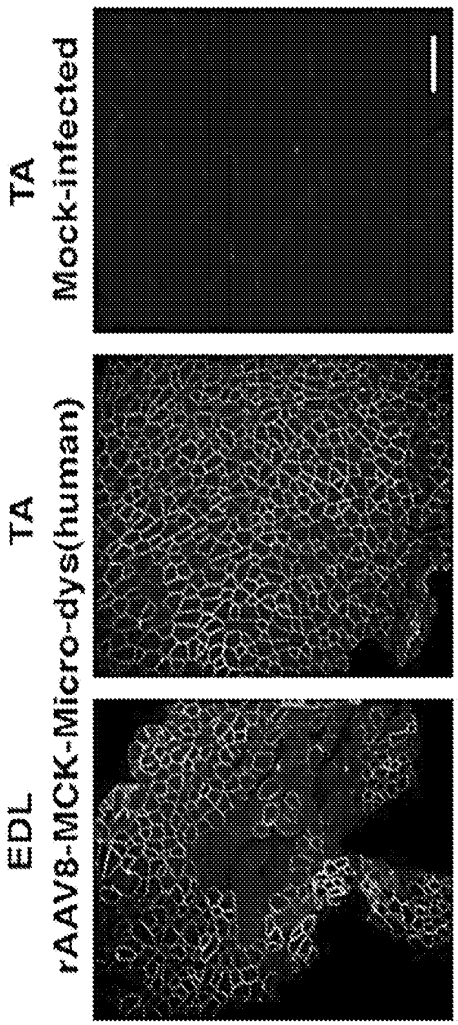


FIG. 9A

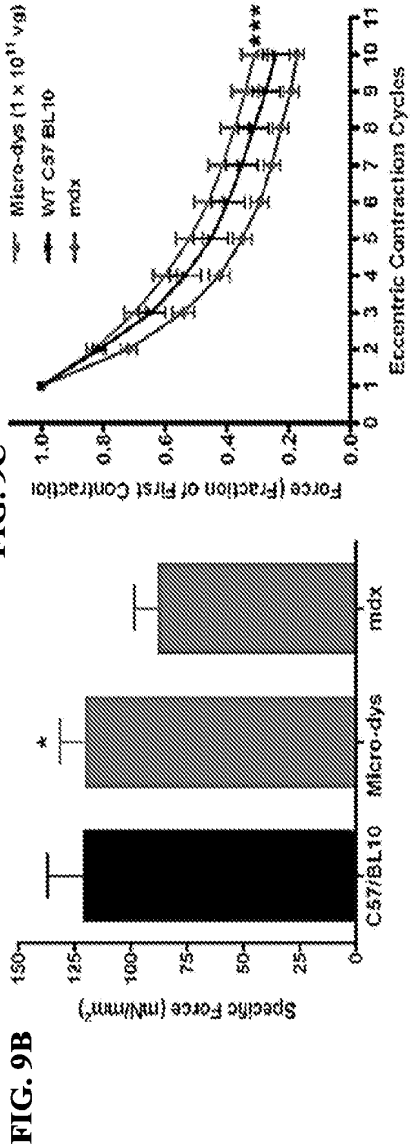


FIG. 9B

FIG. 9C

Figure 10

SEQ ID NO: 3

rAAVrh74.MHCK7.microdystrophin

Main features:
MHCK7 promoter
Chimeric intron sequence
Human micro-dystrophin sequence
Poly A tail
Ampicillin resistance
pGEX plasmid backbone with pBR322 origin or replication

GCCCAATACGCAAACCGCCTCTCCCCGCGCGTTGGCCGATTCATTAATGCAGCTGGCGCGCTCGCTC
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CGAGCGAGCGCGCAGAGAGGGAGTGGCCAACTCCATCACTAGGGGTTCCTTGTAGTTAATGATTAA
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GGGGGTTCGTGCACACAGCCCAGCTTGGAGCGAACGACCTACACCGAACTGAGATACCTACAGCGT
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CAGCCGAACGACCGAGCGCAGCGAGTCAGTGAGCGACCAAGCGGAAGAGC

Figure 11

SEQ ID NO: 5

rAAVrh74.MCK.micro-dystrophin

Main features:
MCK promoter
Chimeric intron sequence
Human codon optimized micro-dystrophin sequence -
Poly A tail
Ampicillin resistance
pGEX plasmid backbone with pBR322 origin or replication

GCCCAATACGCAAACCGCCTCTCCCCGCGCGTTGGCCGATTCATTAATGCAGCTGGCGCGCTCGCTC
GCTCACTGAGGCCGCCCGGGCAAAGCCCGGGCGTCGGGCGACCTTTGGTCGCCCGGCCTCAGTGAG
CGAGCGAGCGCGCAGAGAGGGAGTGGCCAACTCCATCACTAGGGGTTCCTTGTAGTTAATGATTAA
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CGCAGCGAGTCAGTGAGCGACCAAGCGGAAGAGC

FIG. 12B

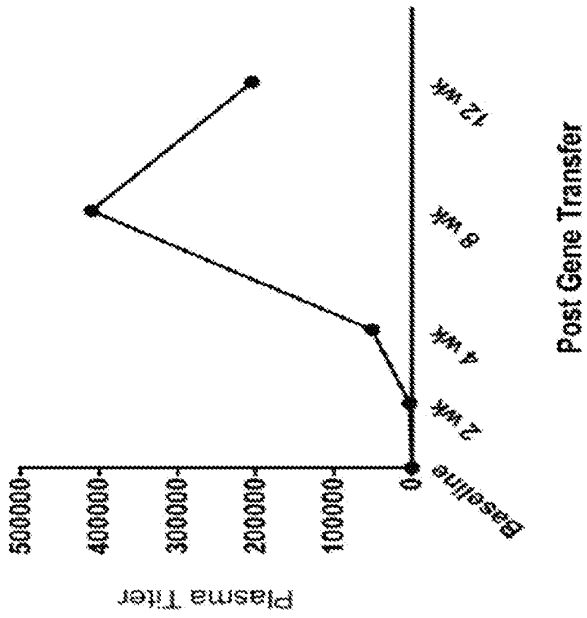


FIG. 12A

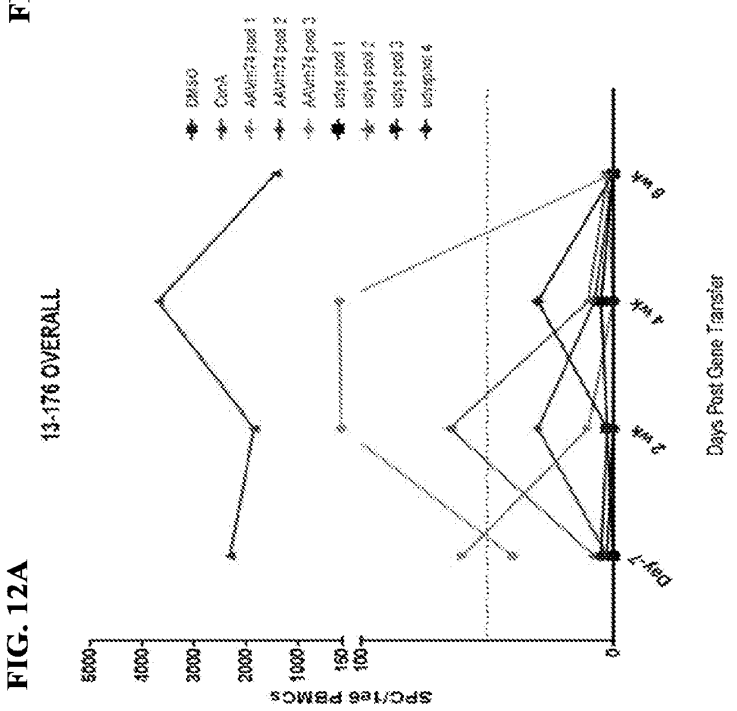


FIG. 13A

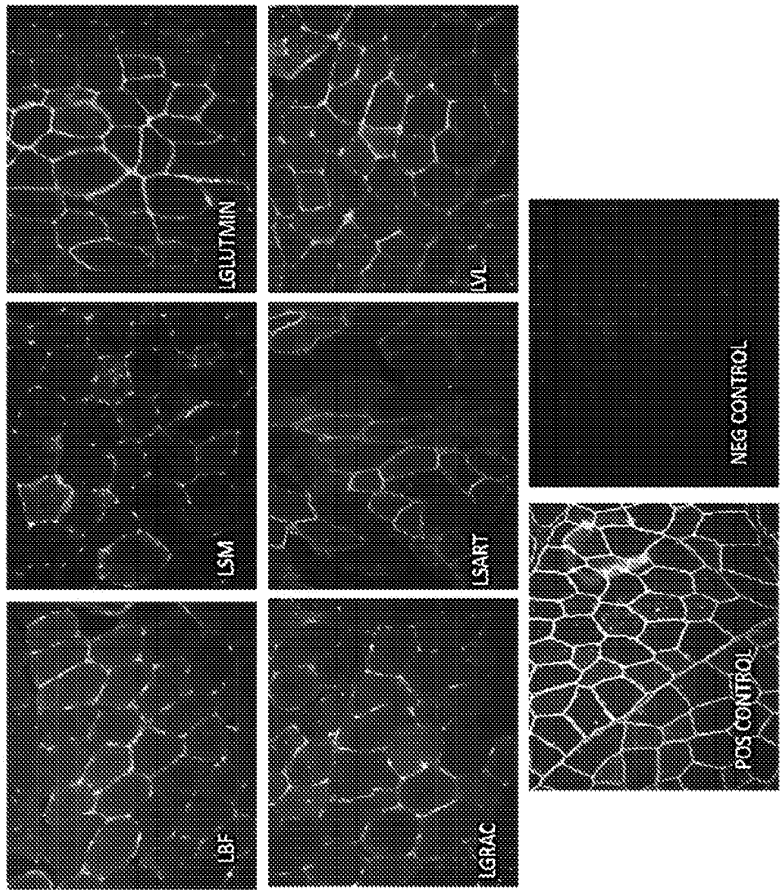


FIG. 13B

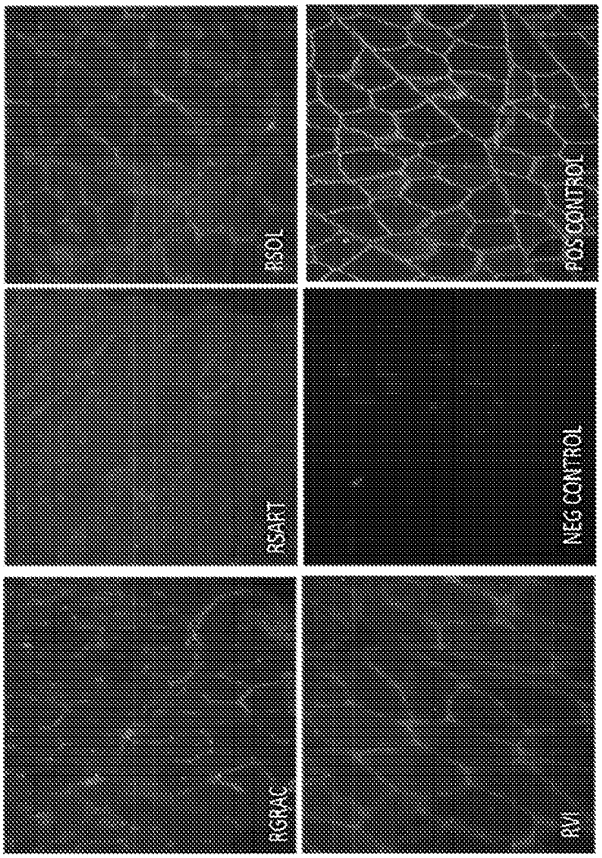


FIG. 14

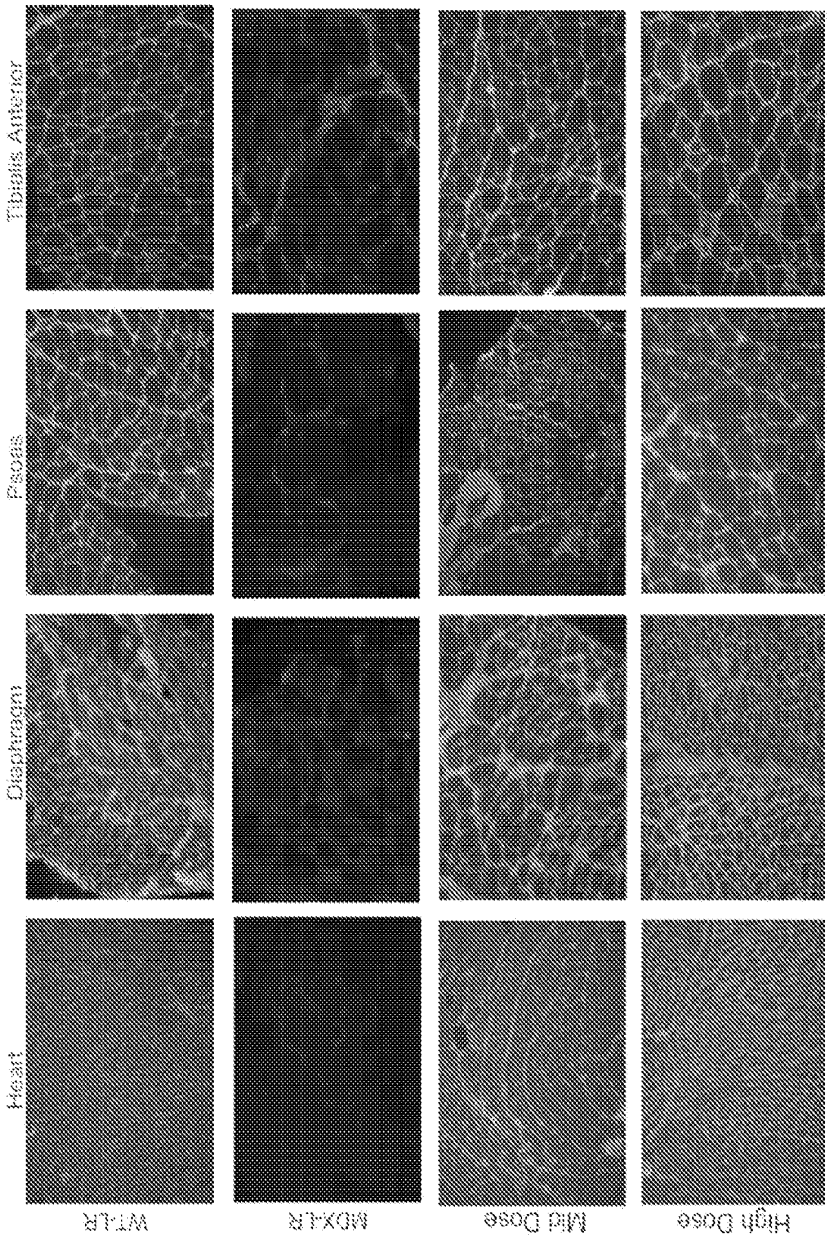

Tibialis Anterior
Psoas
Diaphragm
Heart
WT-LR
MDX-LR
Mid Dose
High Dose

FIG. 15

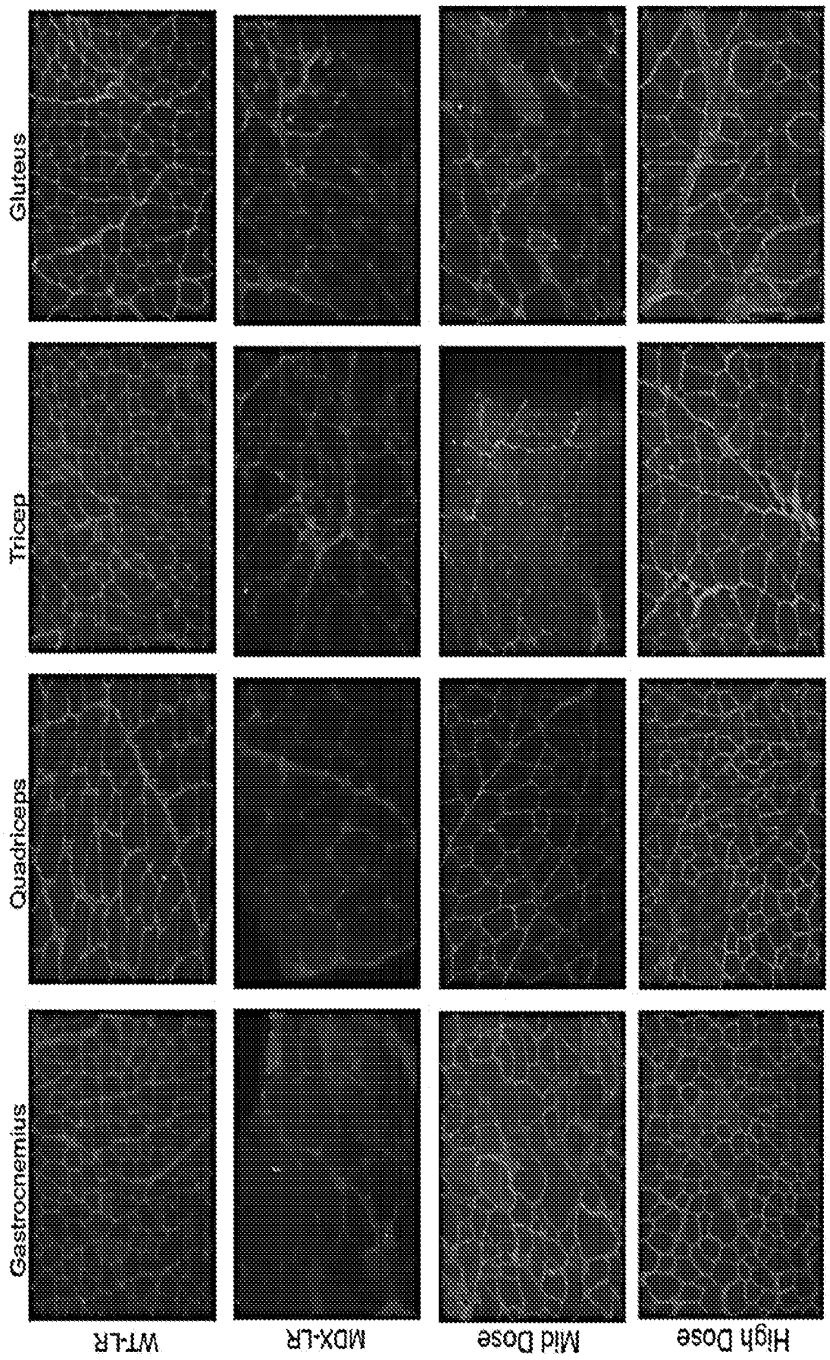

Gastrocnemius
Quadriceps
Tricep
Gluteus
WT-LR
MDX-LR
Mid Dose
High Dose

FIG. 16

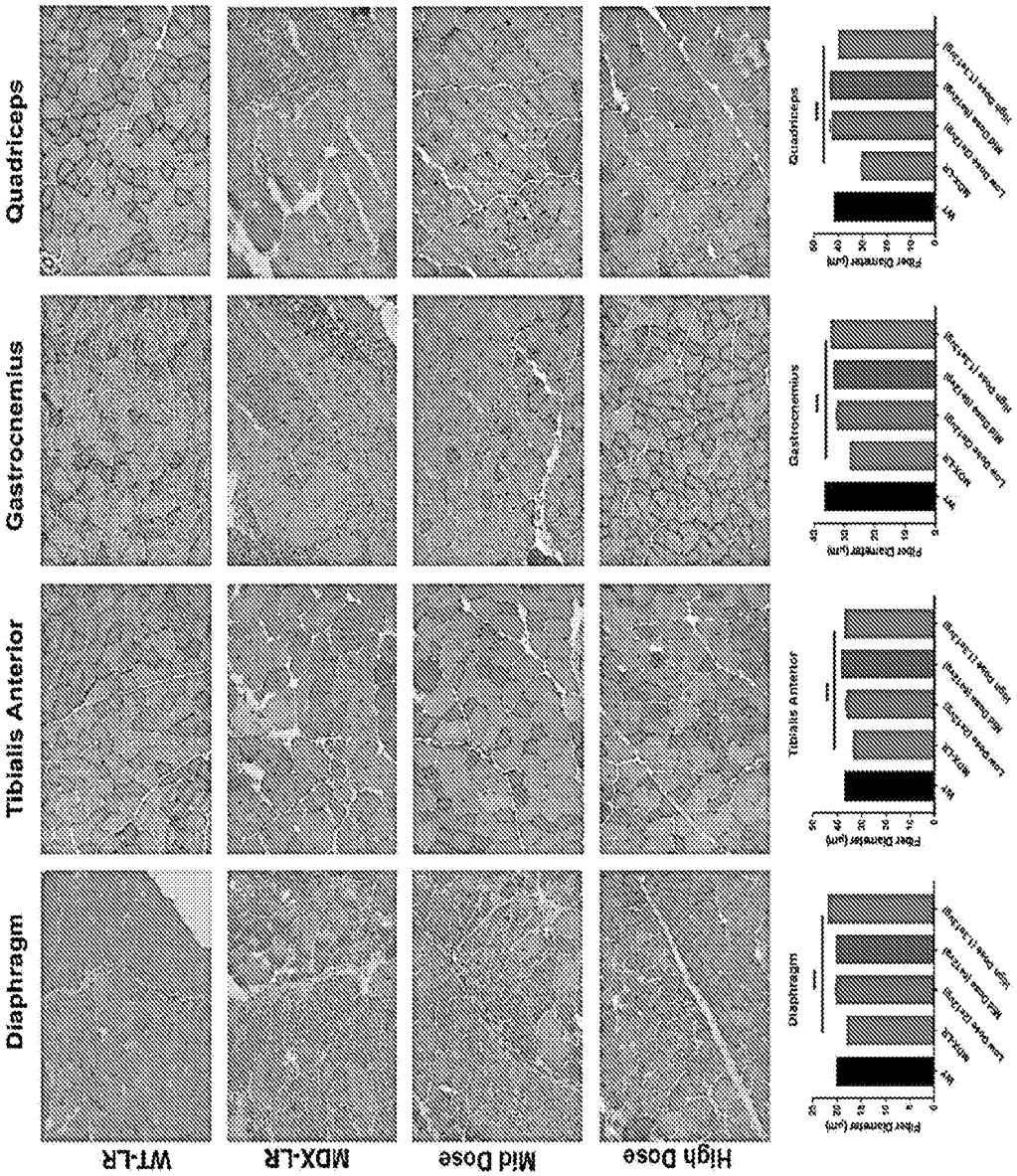

WT-LR
MDX-LR
Mid Dose
High Dose
Diaphragm
Tibialis Anterior
Gastrocnemius
Quadriceps

FIG. 17

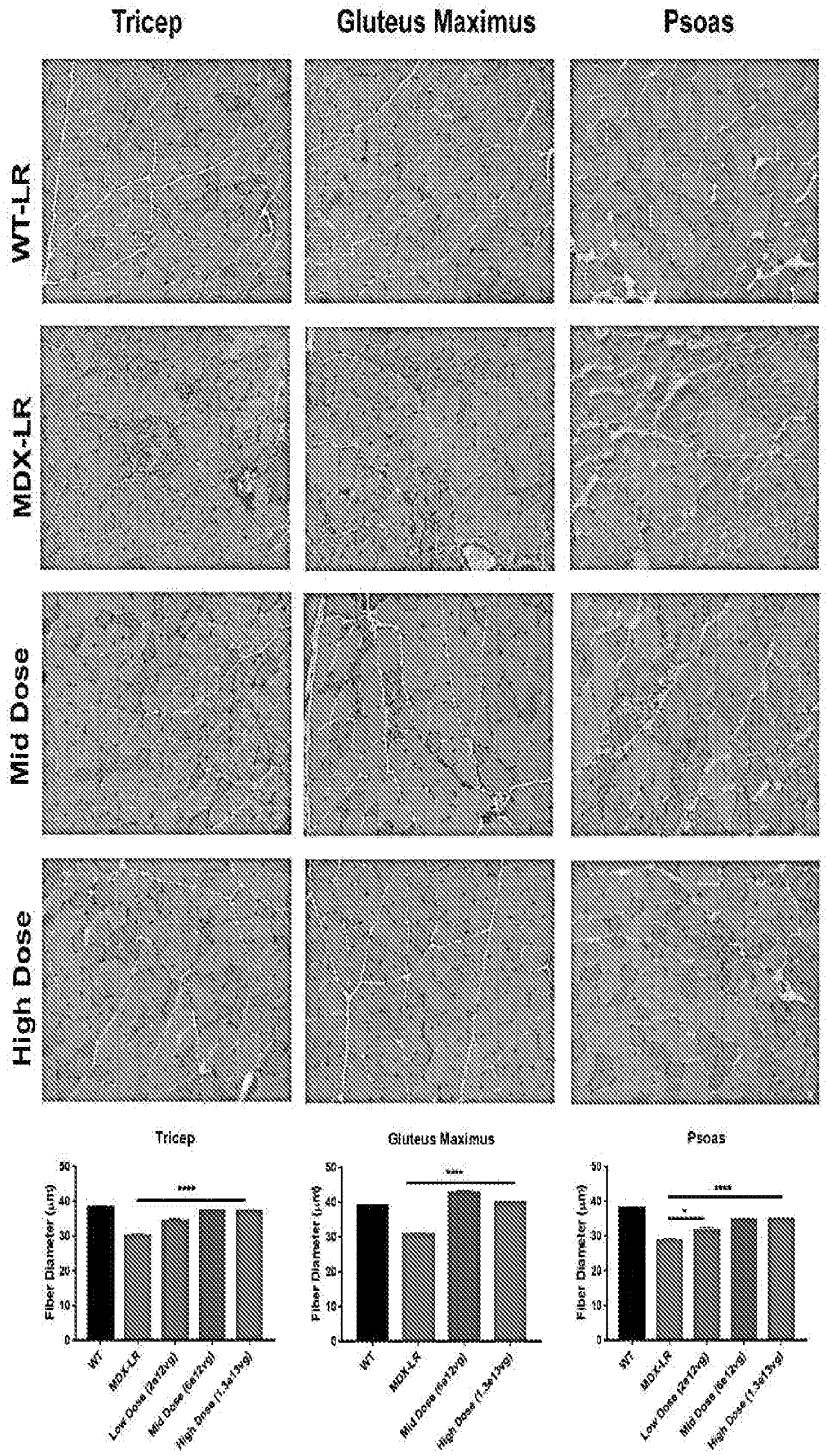

Tricep
Gluteus Maximus
Psoas
WT-LR
MDX-LR
Mid Dose
High Dose
Tricep
Gluteus Maximus
Psoas
Fiber Diameter (um)
WT
MDX-LR
Low Dose (2e12vg)
Mid Dose (6e12vg)
High Dose (1.3e13vg)

FIG. 18

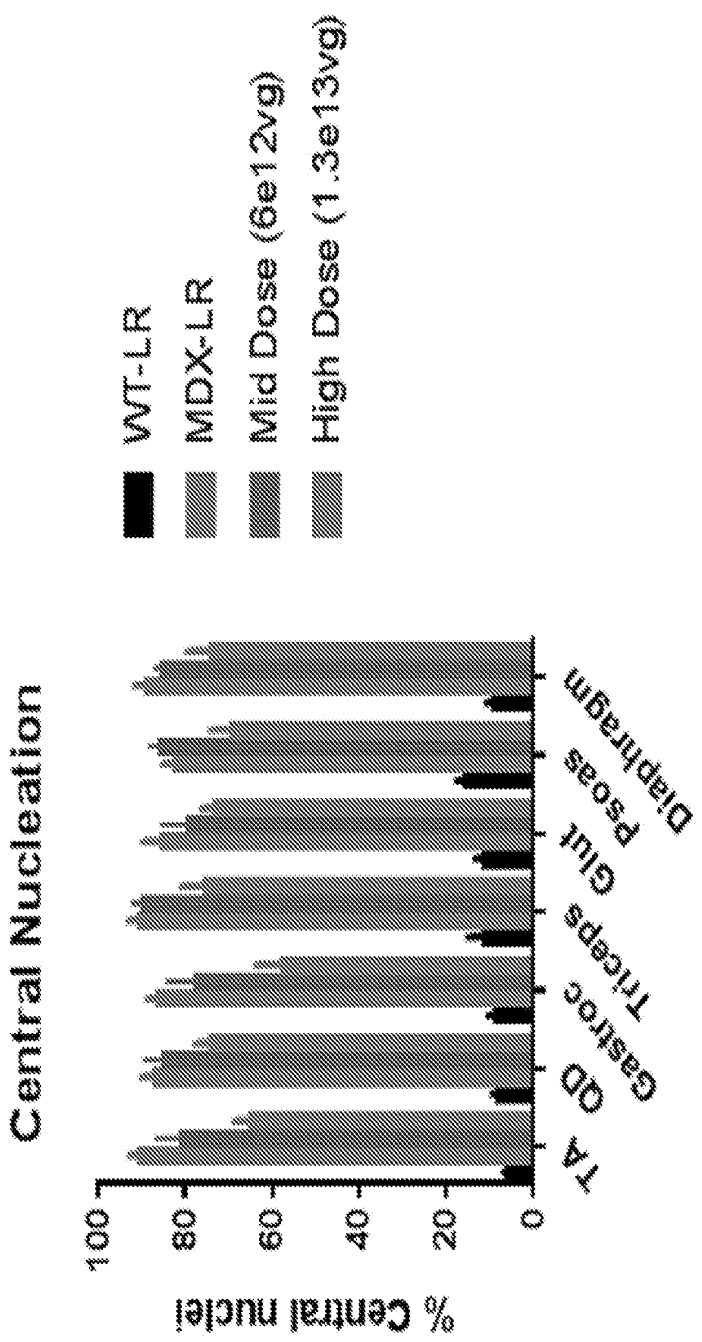

WT-LR
MDX-LR
Mid Dose (6e12vg)
High Dose (1.3e13vg)
Central Nucleation
% Central nuclei
100
80
60
40
20
0
TA
QD
Gastroc
Triceps
Glut
Psoas
Diaphragm

FIG. 19

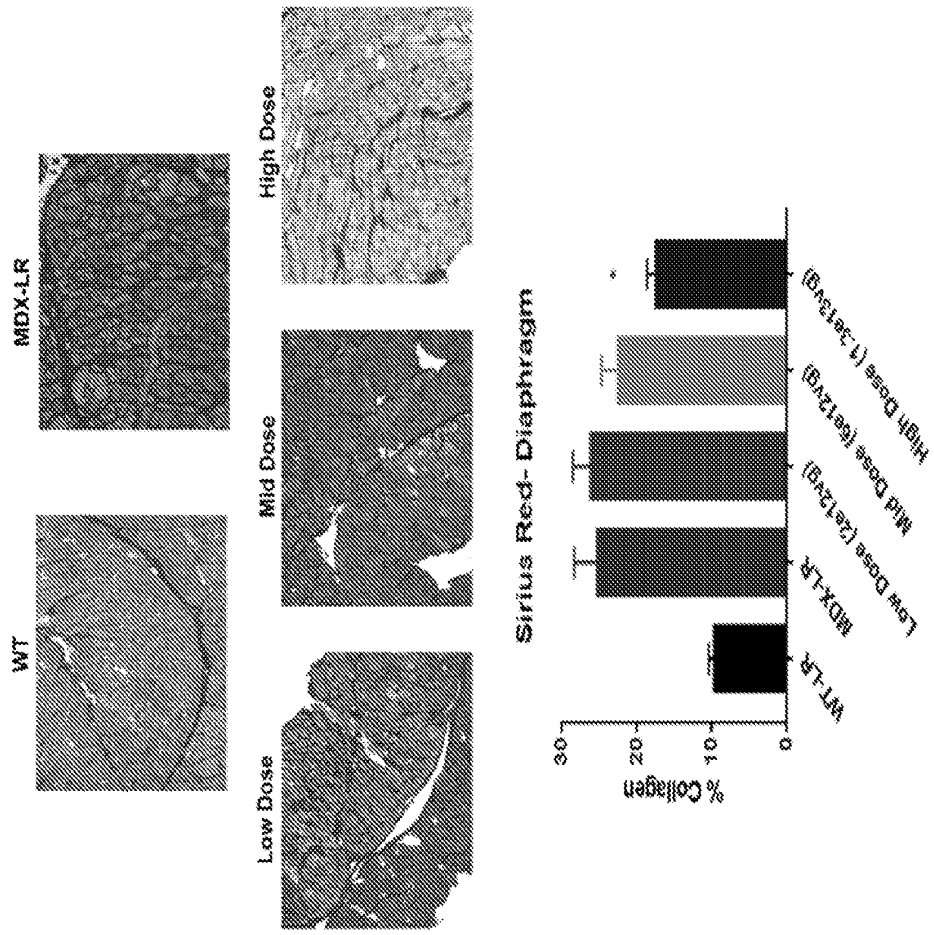

WT
MDX-LR
High Dose
Mid Dose
Low Dose
Sirius Red- Diaphragm
% Collagen
0
10
20
30
WT-LR
MDX-LR
Low Dose (Baz1a+/-)
Mid Dose (Baz1a+/+)
High Dose (Baz1a+/+)

FIG. 20

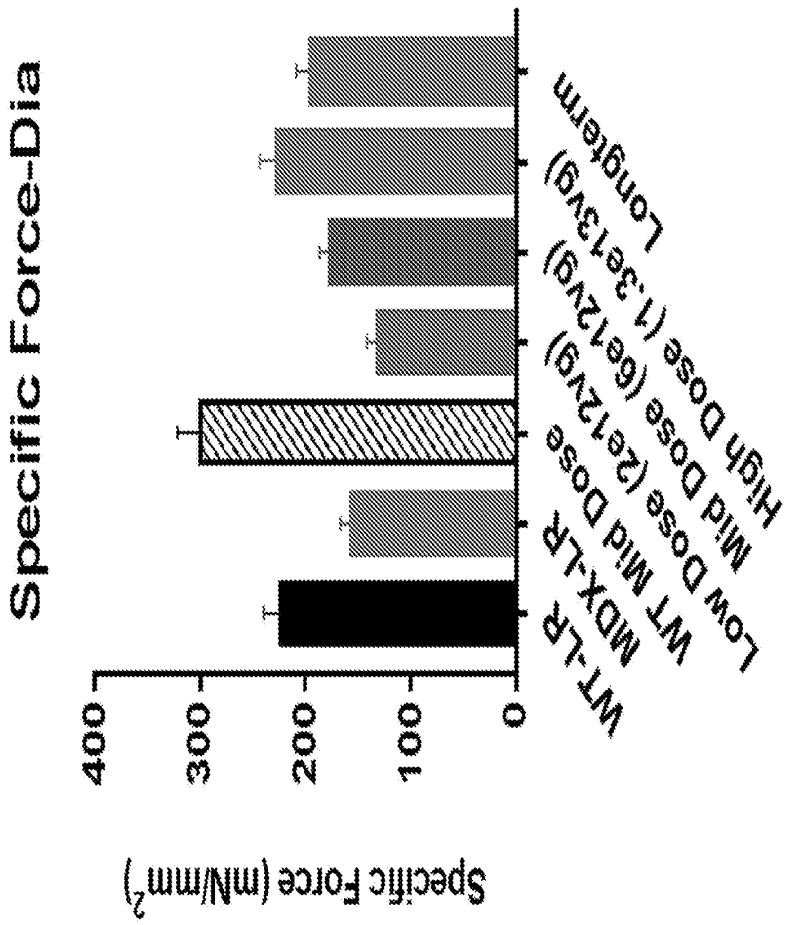

Specific Force-Dia
Specific Force (mN/mm²)
400
300
200
100
0
WT-LR
MDX-LR
WT Mid Dose
Low Dose (2e12vg)
Mid Dose (6e12vg)
High Dose (1.3e13vg)
Longterm

FIG. 21

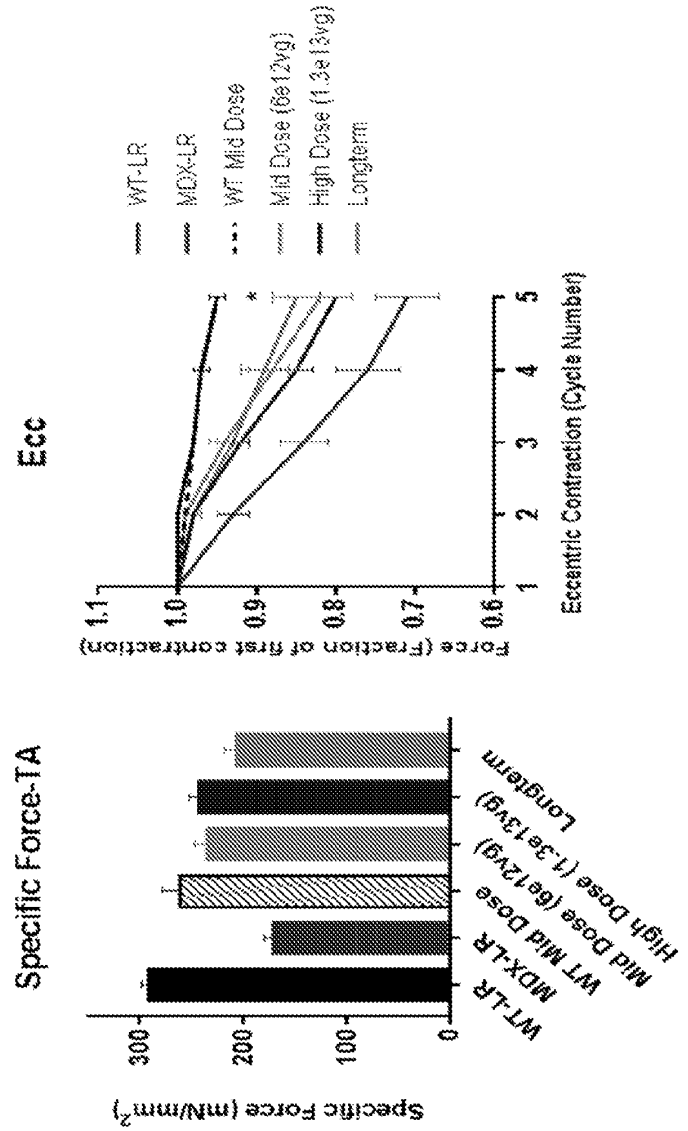

Ecc
WT-LR
MDX-LR
WT Mid Dose
Mid Dose (6e12vg)
High Dose (1.3e13vg)
Longterm
Force (Fraction of first contraction)
1.1
1.0
0.9
0.8
0.7
0.6
1
2
3
4
5
Eccentric Contraction (Cycle Number)
Specific Force-TA
Specific Force (mN/mm²)
300
200
100
0
WT-LR
MDX-LR
WT Mid Dose
Mid Dose (6e12vg)
High Dose (1.3e13vg)
Longterm

FIG. 22

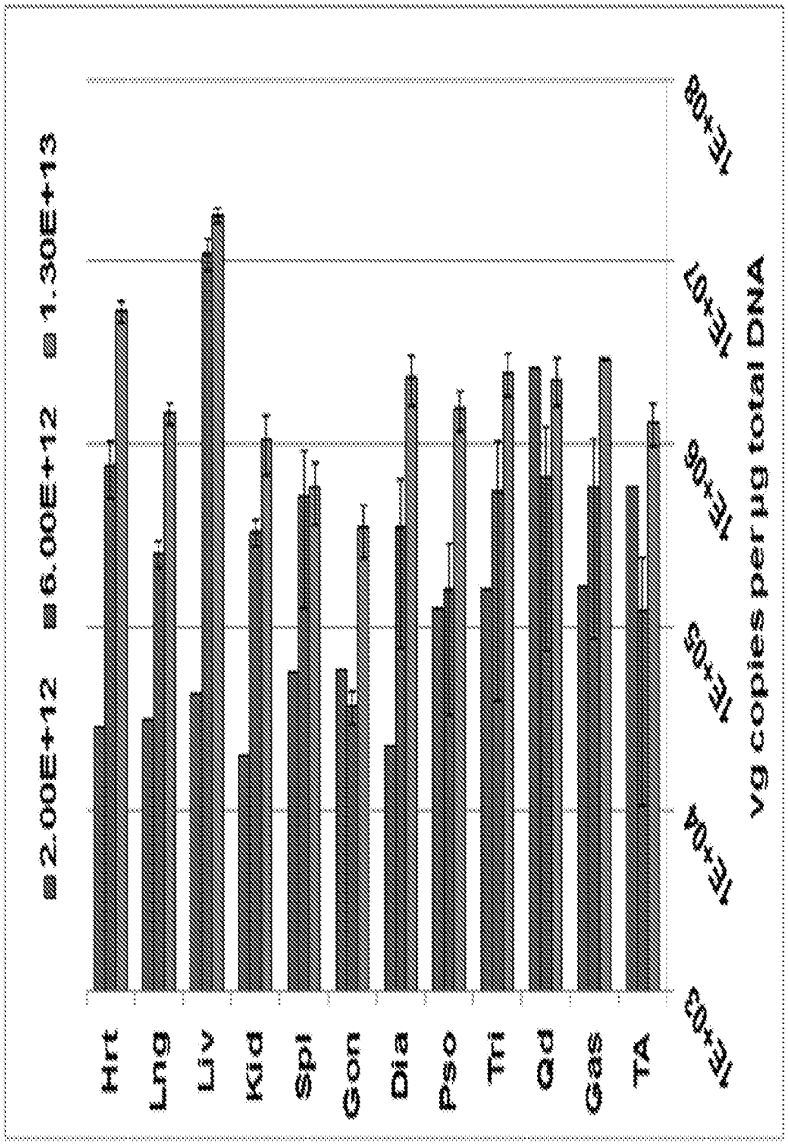

2.00E+12
6.00E+12
1.30E+13
vg copies per µg total DNA
1E+03
1E+04
1E+05
1E+06
1E+07
1E+08
Hrt
Lng
Liv
Kid
Spl
Gon
Dia
Pso
Tri
Qd
Gas
TA

FIG. 23

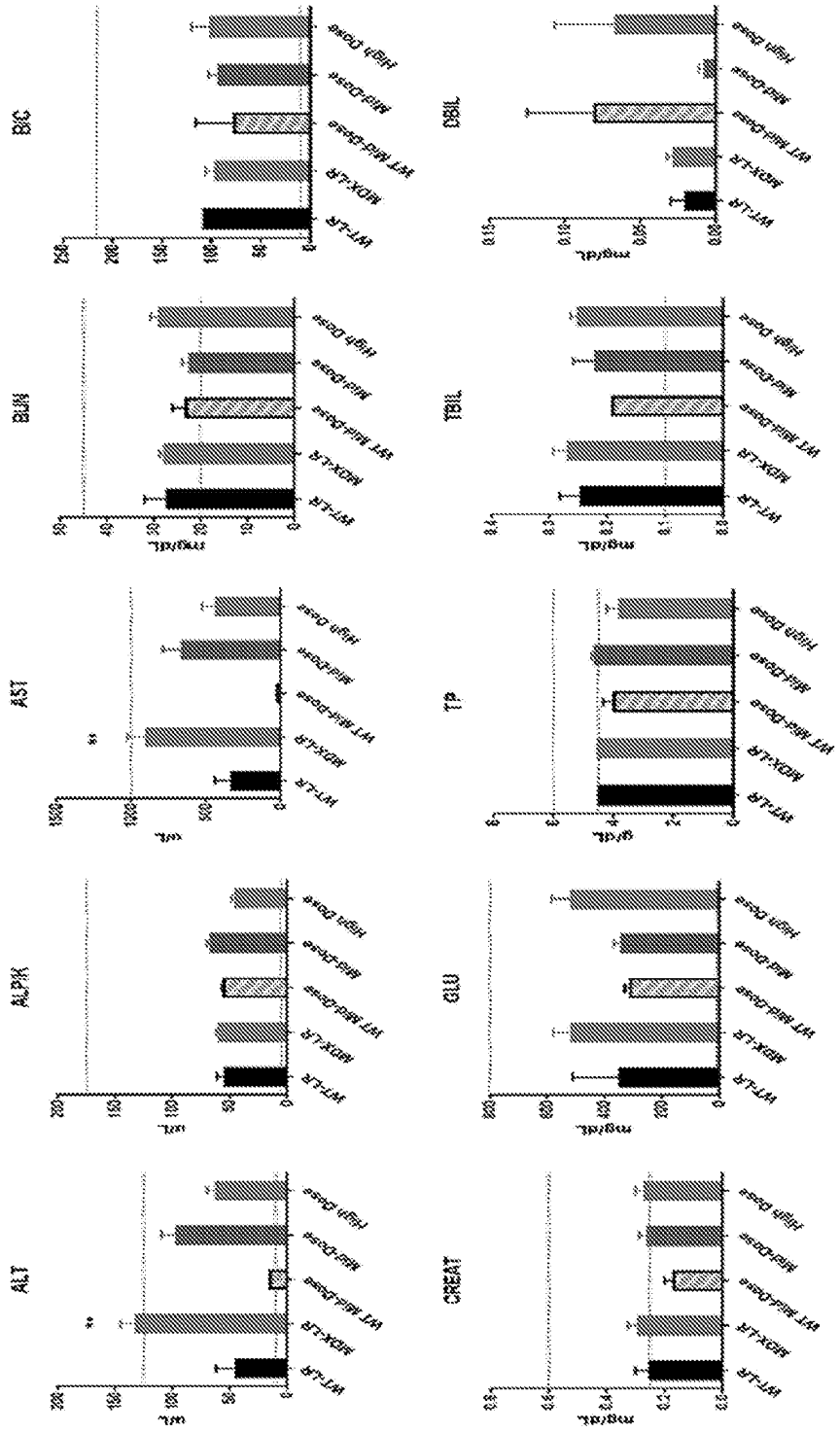

BIC
DBIL
BUN
TBIL
AST
TP
ALPK
GLU
ALT
CREAT
WT-LR
KDX-LR
WT-KDX-LR
Mid-Dose
High Dose

FIG. 24

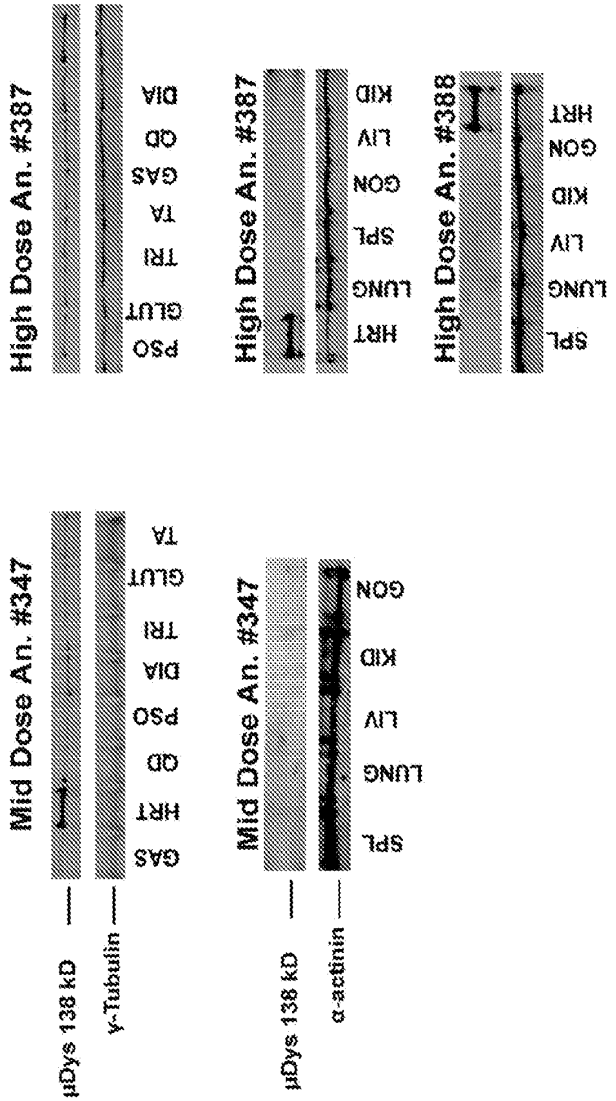

High Dose An. #387
PSO
GLUT
TRI
TA
GAS
QD
DIA
High Dose An. #387
HRT
LUNG
SPL
GON
LIV
KID
High Dose An. #388
SPL
LUNG
LIV
KID
GON
HRT
Mid Dose An. #347
μDys 138 kD
γ-Tubulin
GAS
HRT
QD
PSO
DIA
TRI
GLUT
TA
Mid Dose An. #347
μDys 138 kD
α-actinin
SPL
LUNG
LIV
KID
GON

FIG. 25

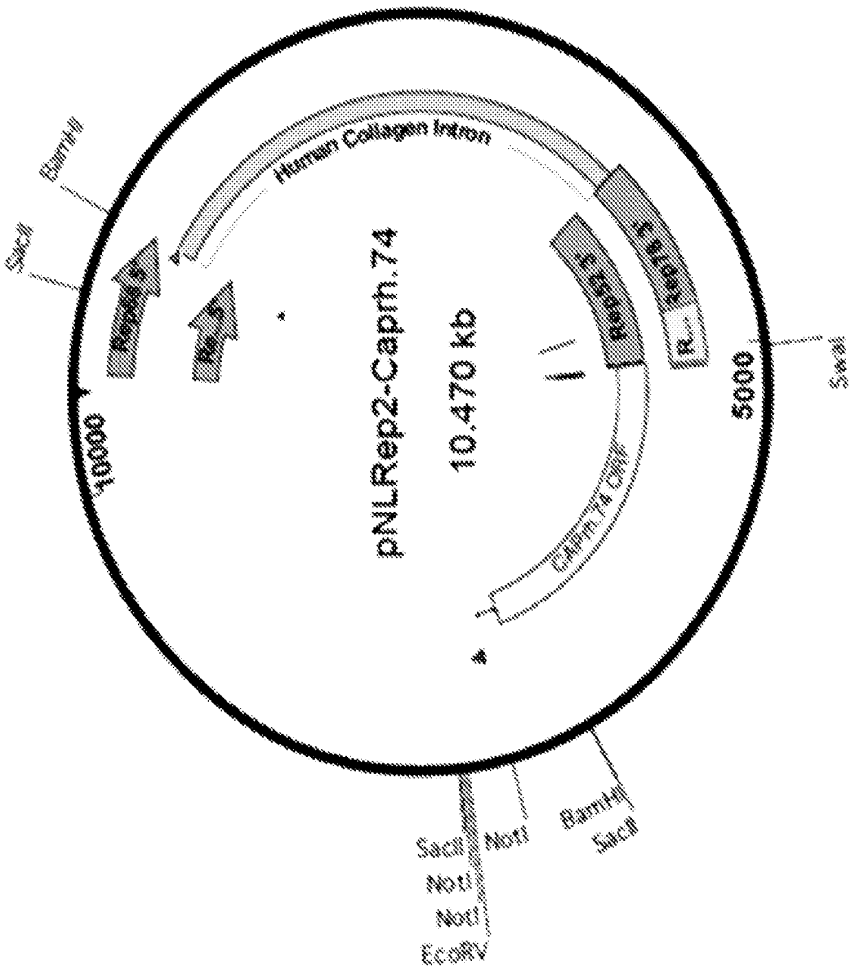

pNLRep2-Caprh.74
10.470 kb
Human Collagen Intron
Rep2-R
Rep-F
Ampr
Ampr
R_
Caprh.74 ORF
1
5000
10000
BamHI
SacII
SacII
NotI
NotI
NotI
EcoRV
NotI
BamHI
SacII
SwaI

FIG. 26

