FIG. 1

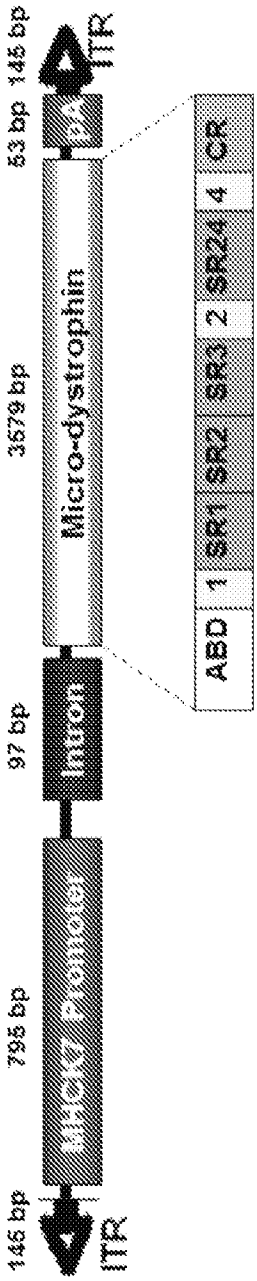

146 bp
ITR
795 bp
MHCK7 Promoter
97 bp
Intron
3679 bp
Micro-dystrophin
ABD 1
SR1
SR2
SR3
2
SR24
4
CR
53 bp
SA
145 bp
ITR

FIG. 2

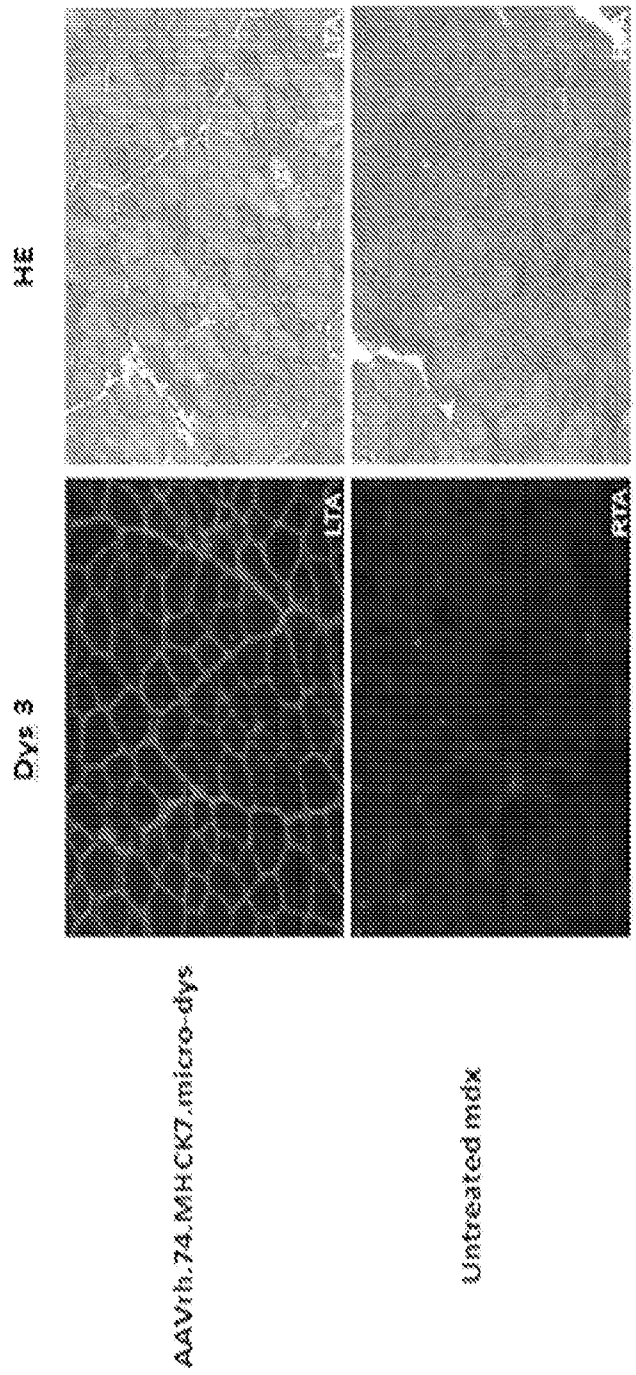


FIG. 3A

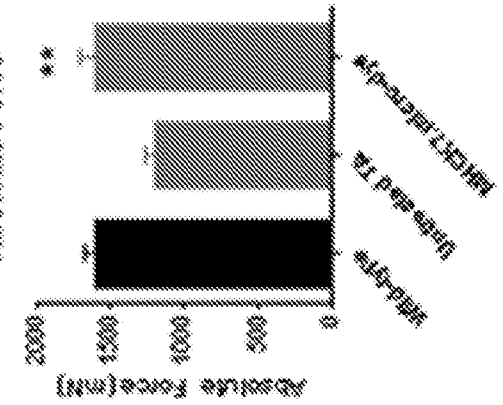


FIG. 3B

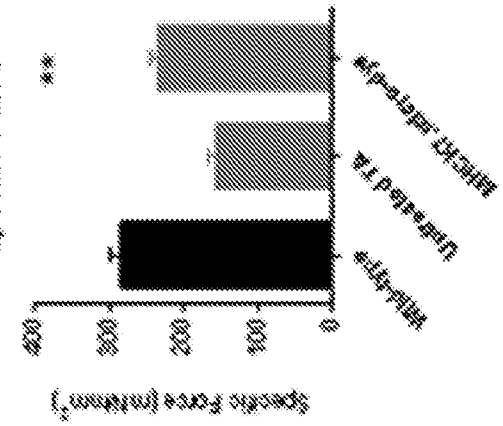


FIG. 3C

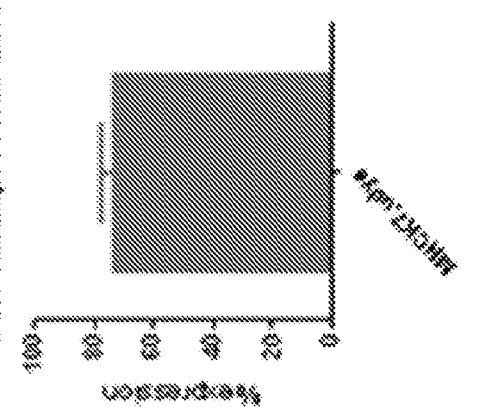


FIG. 4A

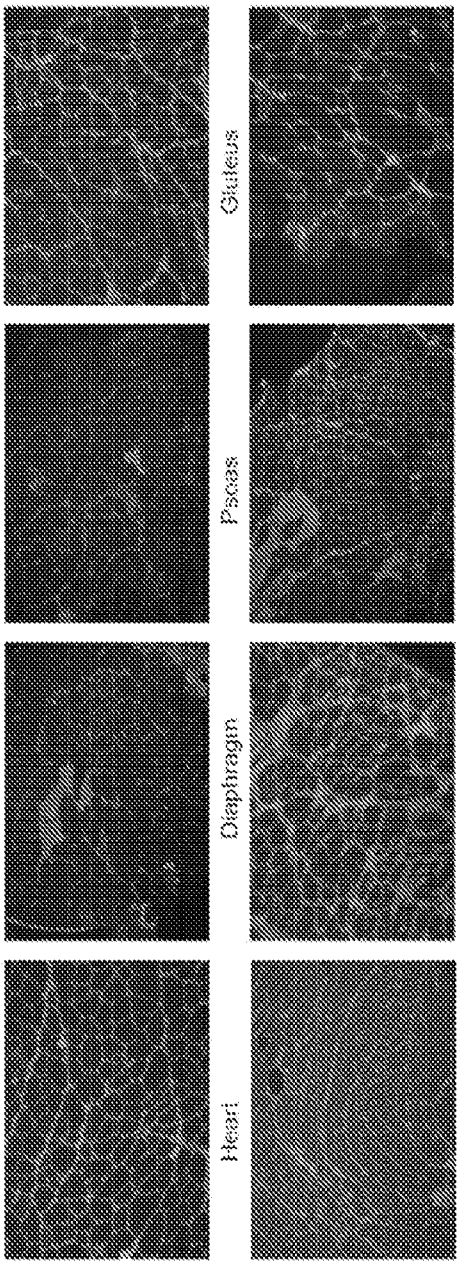


FIG. 4B

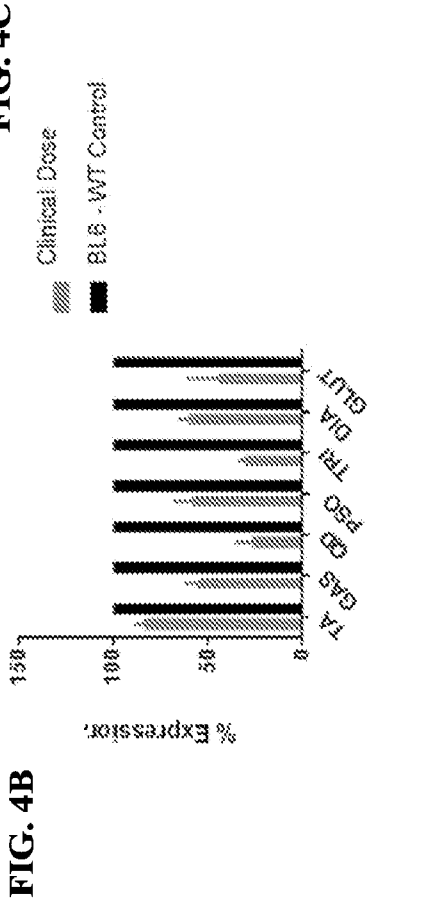


FIG. 4C

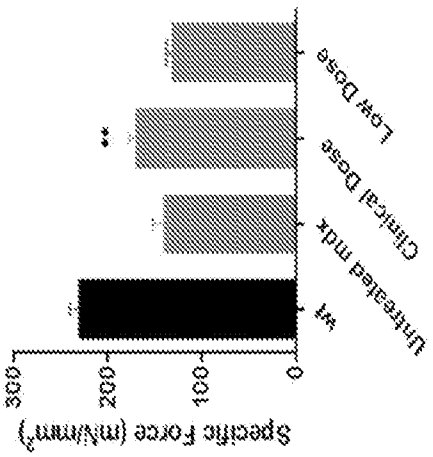


FIG. 5

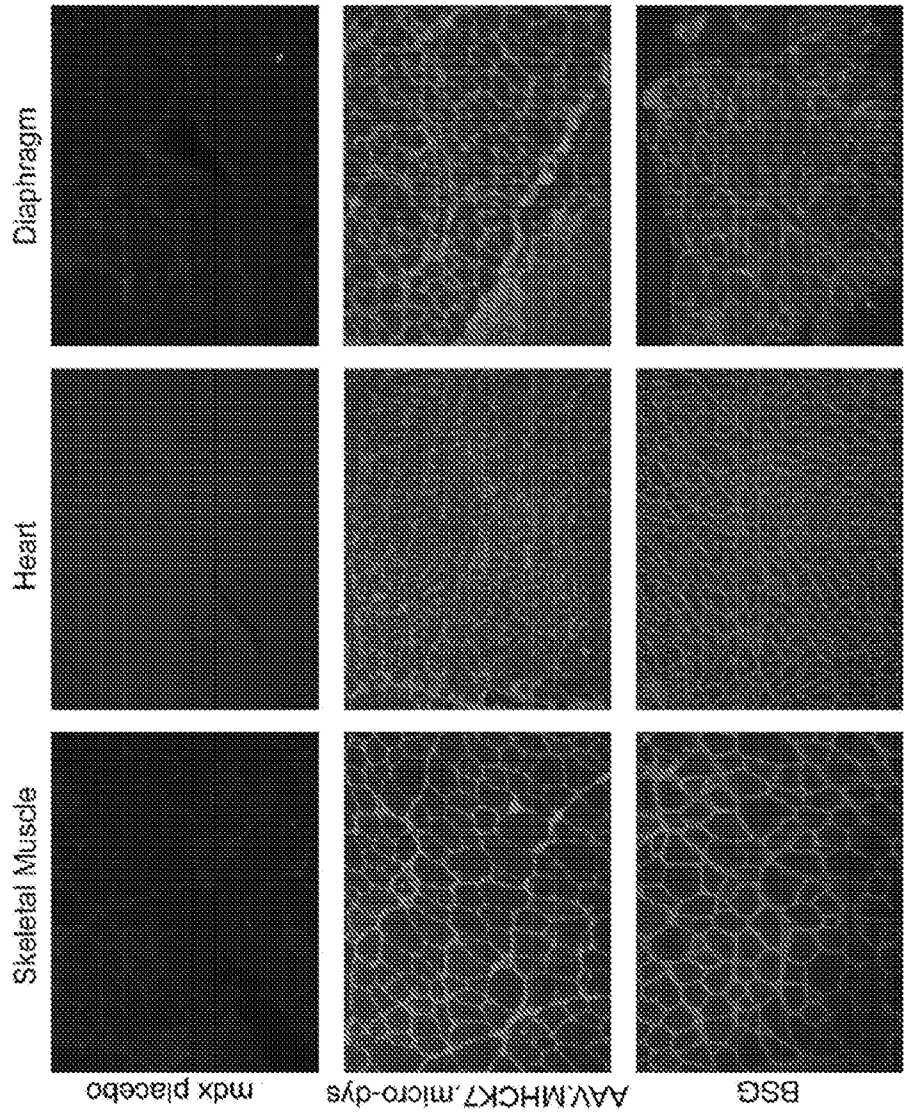

Diaphragm
Heart
Skeletal Muscle
mdx placebo
AAV.MHCK7.micro-dys
BSG

FIG. 6A

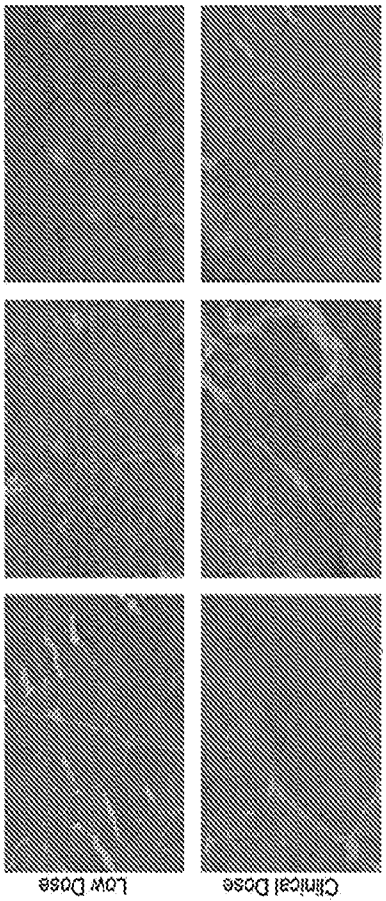


FIG. 6B

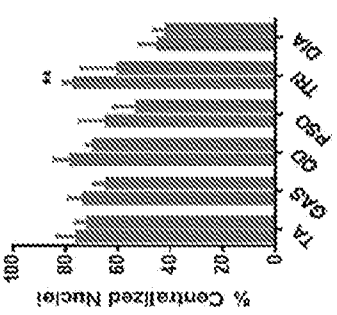


FIG. 6C

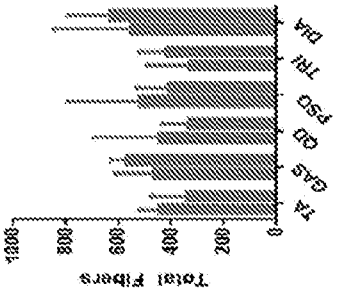


FIG. 6D

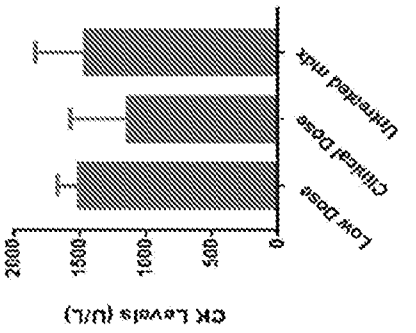


FIG. 7

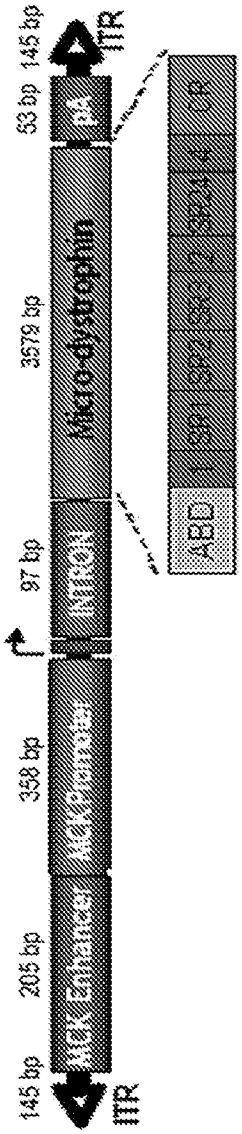

145 bp
ITR
53 bp
pA
3579 bp
Micro-dystrophin
97 bp
Intron
358 bp
MCK Promoter
205 bp
MCK Enhancer
145 bp
ITR
ABD
CR

FIG. 8

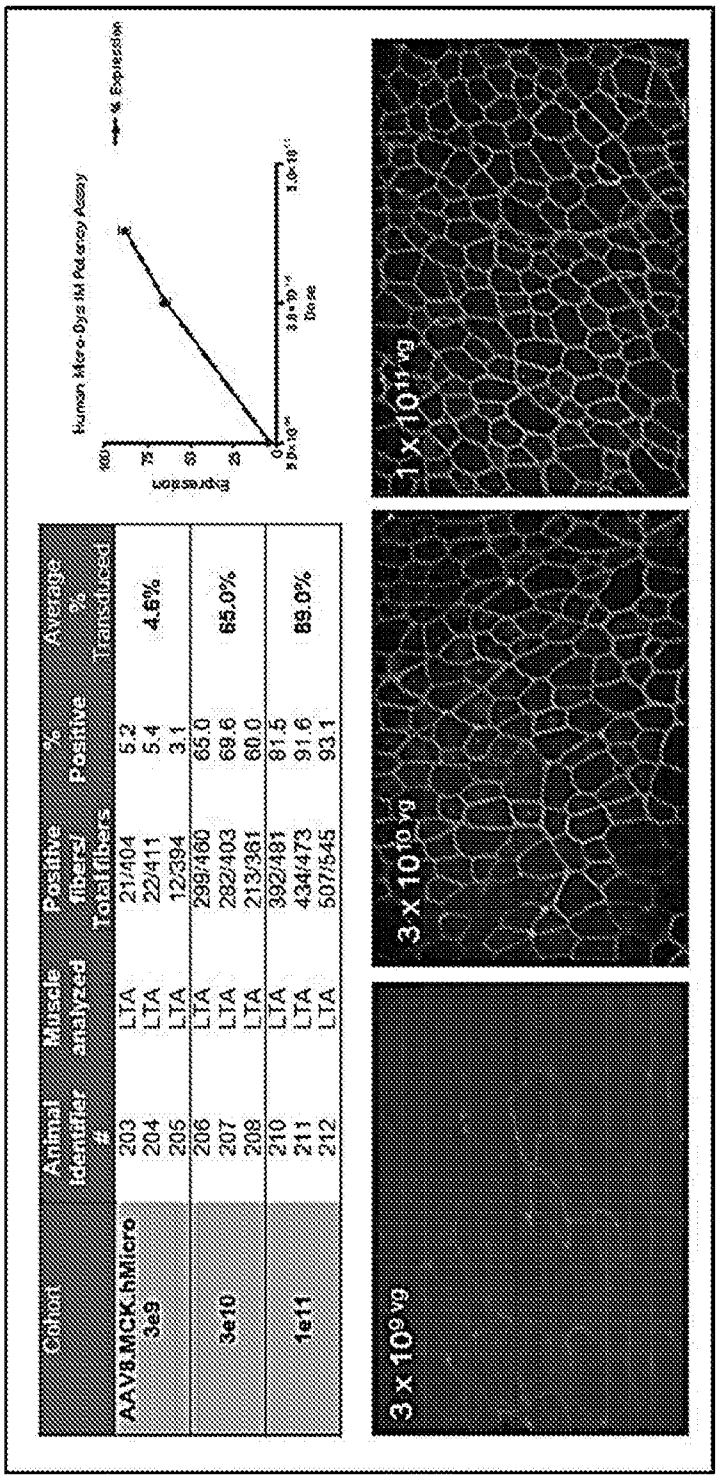

Cohort
Animal Identifier #
Muscle Analyzed
Positive fibers/ Total fibers
% Positive
Average % Transduced
AAV8.MCK.hMicro 3e9
203 LTA 21/404 5.2
204 LTA 22/411 5.4 4.6%
205 LTA 12/394 3.1
3e10
206 LTA 299/460 65.0
207 LTA 282/403 69.6 65.0%
208 LTA 213/361 60.0
1e11
210 LTA 392/481 81.5
211 LTA 434/473 91.6 89.0%
212 LTA 507/545 93.1
Human Micro-Dystrophy Assay
% Expression
Dose
3 x 10^9 vg
3 x 10^10 vg
1 x 10^11 vg

FIG. 9A

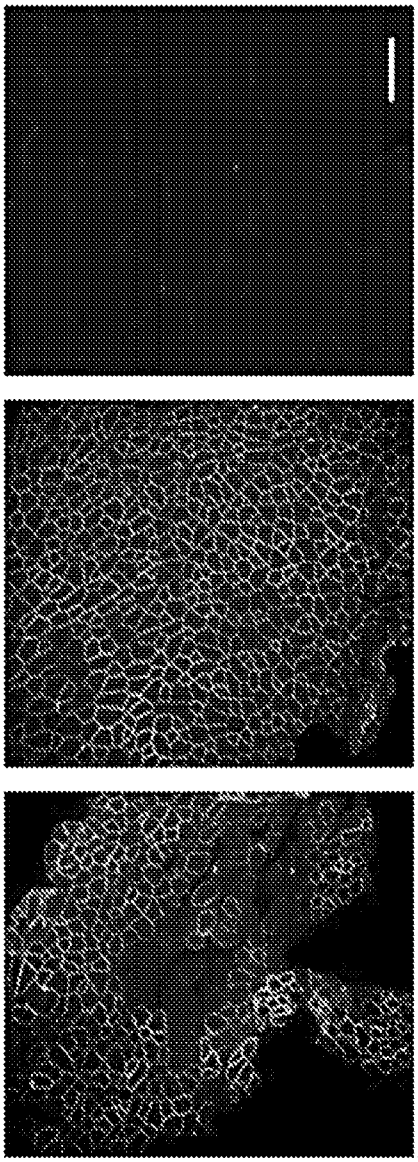


FIG. 9B

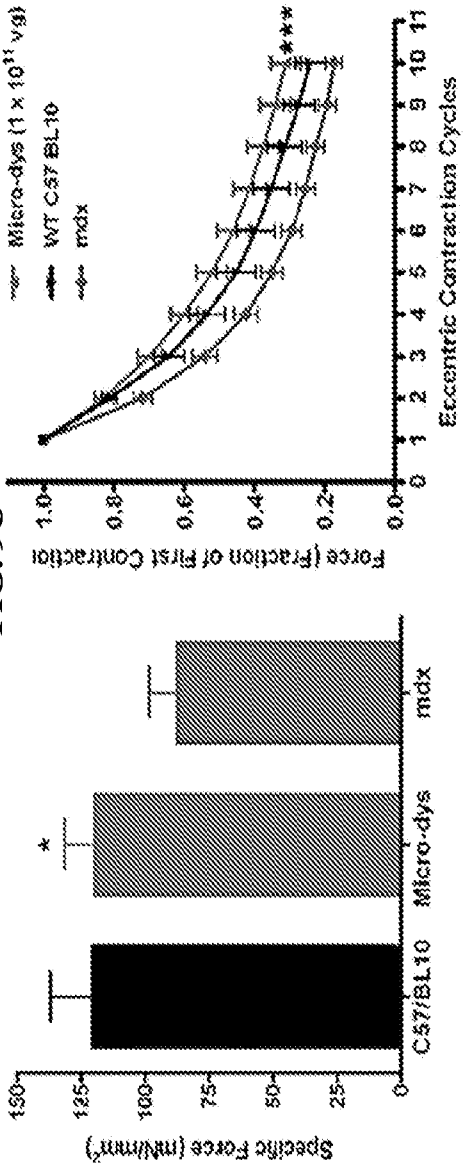


FIG. 9C

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
GCTCACTGAGGCCGCCCGGGCAAAGCCCGGGCGTCGGGCGACCTTTGGTCGCCCGGCCTCAGTGAG
CGAGCGAGCGCGCAGAGAGGGAGTGGCCAACTCCATCACTAGGGGTTCCTTGTAGTTAATGATTAA
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GCCCTGTAGCGGCGCATTAAGCGCGGCGGGTGTGGTGGTTACGCGCAGCGTGACCGCTACACTTGC
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GGTCGGAACAGGAGAGCGCACGAGGGAGCTTCCAGGGGGAAACGCCTGGTATCTTTATAGTCCTGT

CGGGTTTCGCCACCTCTGACTTGAGCGTCGATTTTTGTGATGCTCGTCAGGGGGGCGGAGCCTATGG
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CCTGCGTTATCCCCTGATTCTGTGGATAACCGTATTACCGGGTTTGAGTGAGCTGATACCGCTCGCCG
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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CCTTGGGGCAGCCCATACAAGGCCATGGGGCTGGGCAAGCTGCACGCCTGGGTCCGGGGTGGGCA
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TGAAGCTGAGGCAGGCCGAAGTGATTAAGGGGTCTTGGCAGCCTGTGGGCGATCTGCTGATTGATT
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GATAAGTAGCATGGCGGGTTAATCATTAACTACAAGGAACCCCTAGTGATGGAGTTGGCCACTCCCT
CTCTGCGCGCTCGCTCGCTCACTGAGGCCGGGCGACCAAAGGTCGCCCGACGCCCGGGCTTTGCCCG
GGCGGCCTCAGTGAGCGAGCGAGCGCGCCAGCTGGCGTAATAGCGAAGAGGCCCGCACCGATCGC
CCTTCCCAACAGTTGCGCAGCCTGAATGGCGAATGGAAGTTCCAGACGATTGAGCGTCAAAATGTAG
GTATTTCCATGAGCGTTTTTCCTGTTGCAATGGCTGGCGGTAATATTGTTCTGGATATTACCAGCAAG
GCCGATAGTTTGAGTTCTTCTACTCAGGCAAGTGATGTTATTACTAATCAAAGAAGTATTGCGACAAC
GGTTAATTTGCGTGATGGACAGACTCTTTTACTCGGTGGCCTCACTGATTATAAAAACACTTCTCAGG
ATTCTGGCGTACCGTTCCTGTCTAAAATCCCTTTAATCGGCCTCCTGTTTAGCTCCCGCTCTGATTCTA
ACGAGGAAAGCACGTTATACGTGCTCGTCAAAGCAACCATAGTACGCGCCCTGTAGCGGCGCATTAA
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CTTTCGCTTTCTTCCCTTCCTTTCTCGCCACGTTCGCCGGCTTTCCCCGTCAAGCTCTAAATCGGGGGCT
CCCTTTAGGGTTCCGATTTAGTGATTTACGGCACCTCGACCCCAAAAAACTTGATTAGGGTGATGGTT
CACGTAGTGGGCCATCGCCCTGATAGACGGTTTTTCGCCCTTTGACGTTGGAGTCCACGTTCTTTAAT
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CGGCCTTTTTACGGTTCCTGGCCTTTTGCTGGCCTTTTGCTCACATGTTCTTTCCTGCGTTATCCCCTGA
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CGCAGCGAGTCAGTGAGCGACCAAGCGGAAGAGC

FIG. 12A

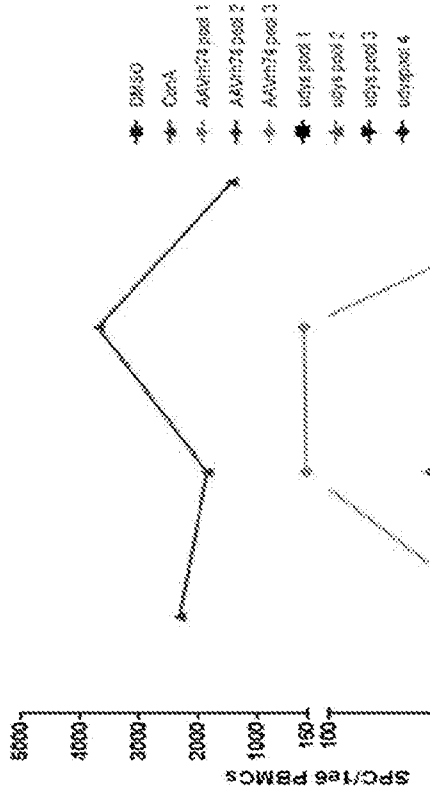


FIG. 12B

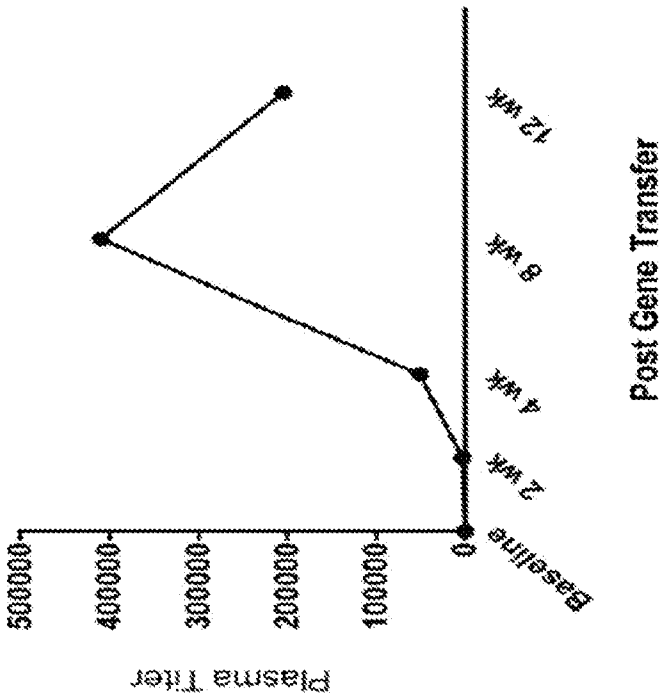


FIG. 13A

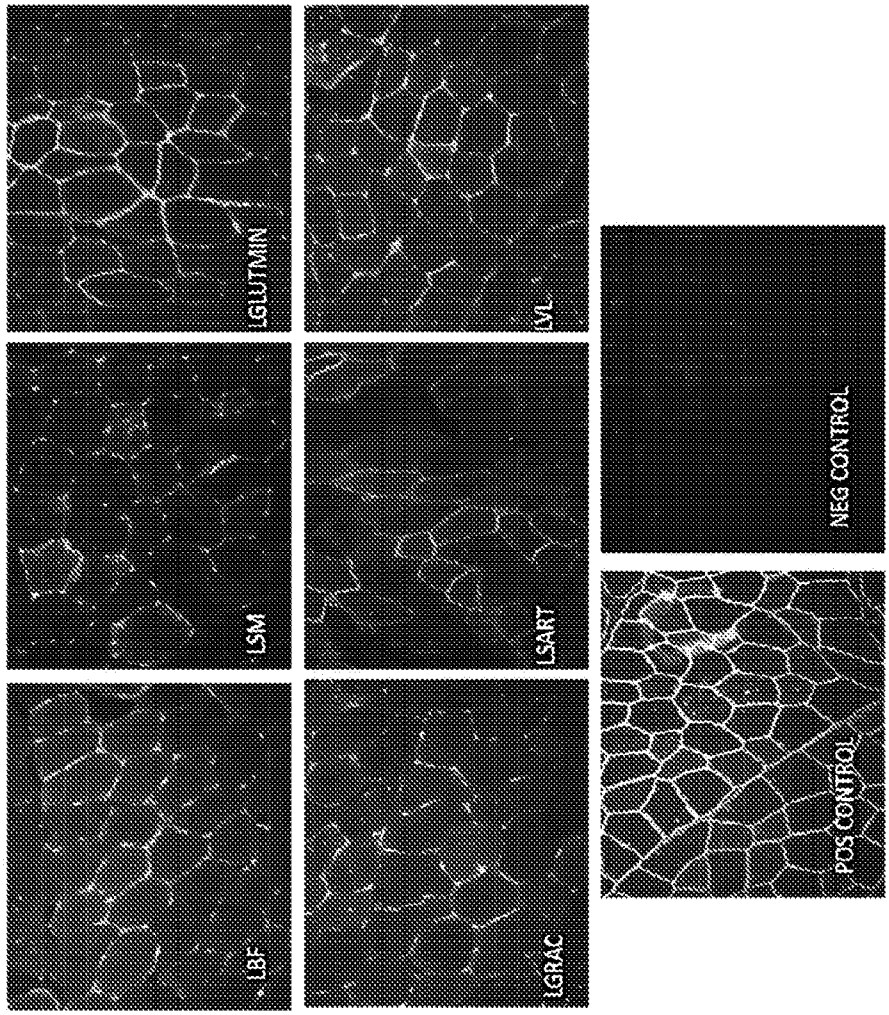


FIG. 13B

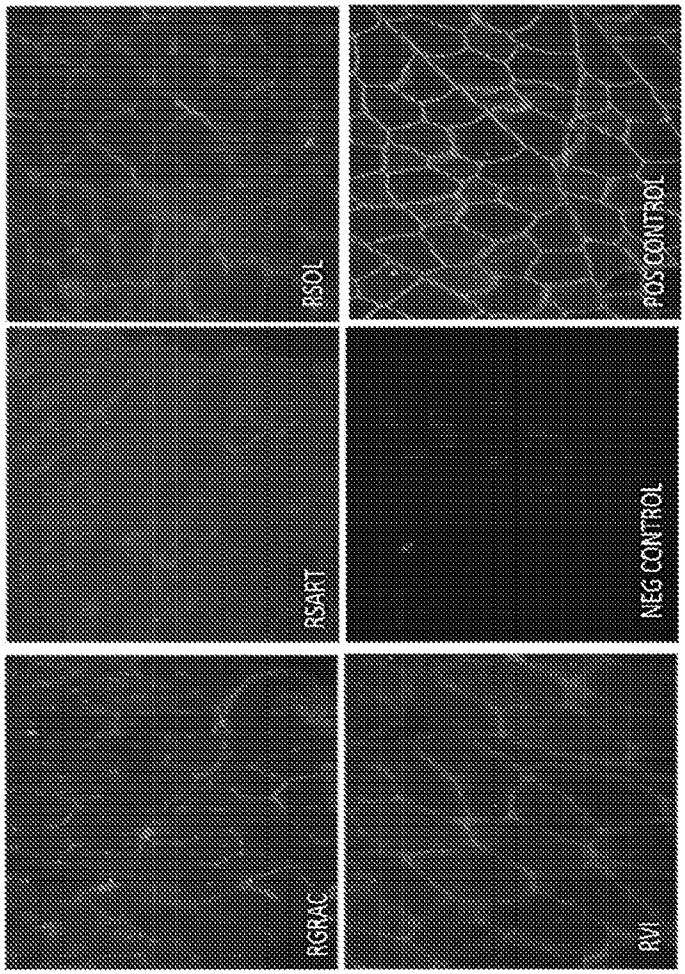


FIG. 14

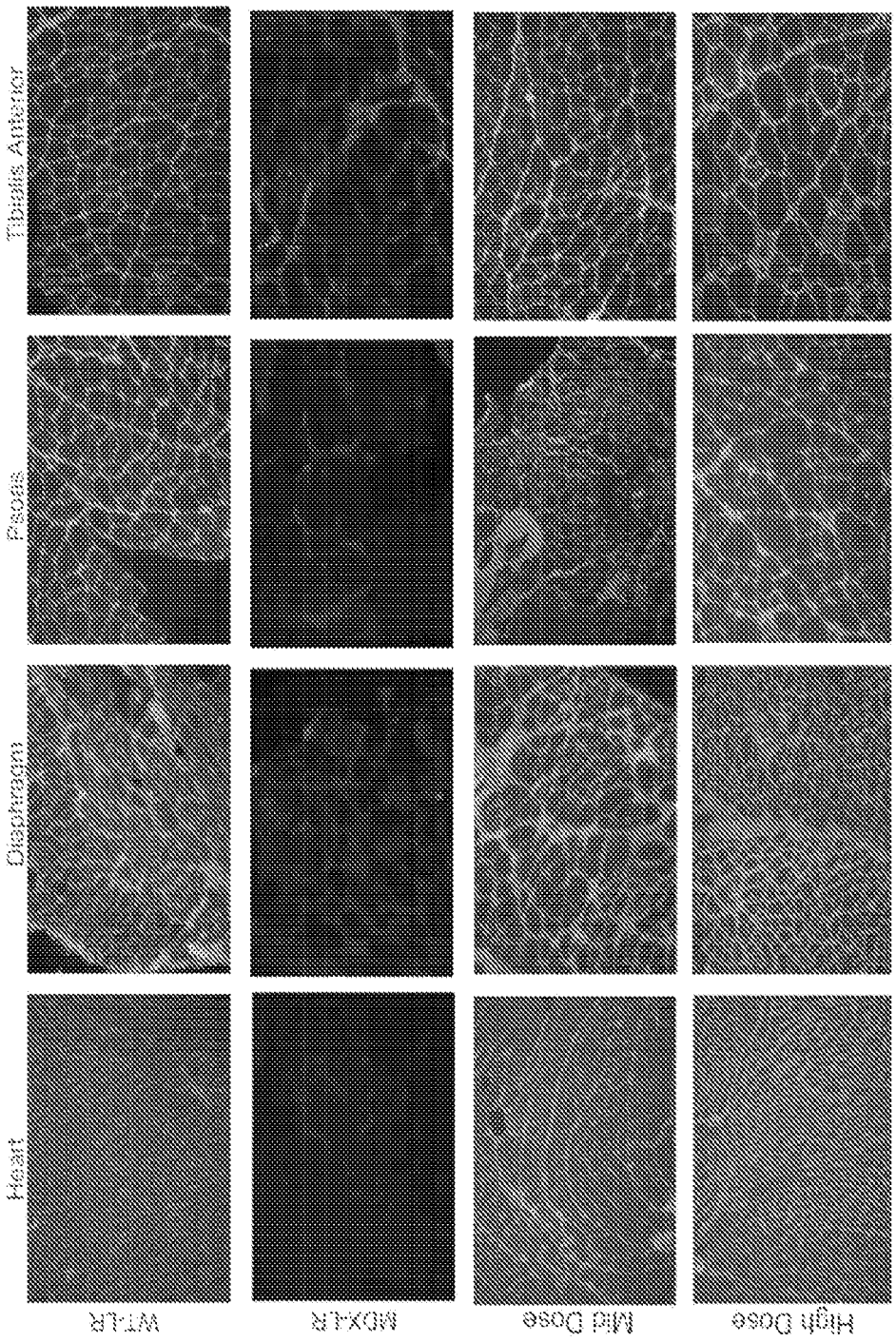

Tibialis Anterior
Psoas
Diaphragm
Heart
WT-LR
MDX4LR
Mid Dose
High Dose

FIG. 15

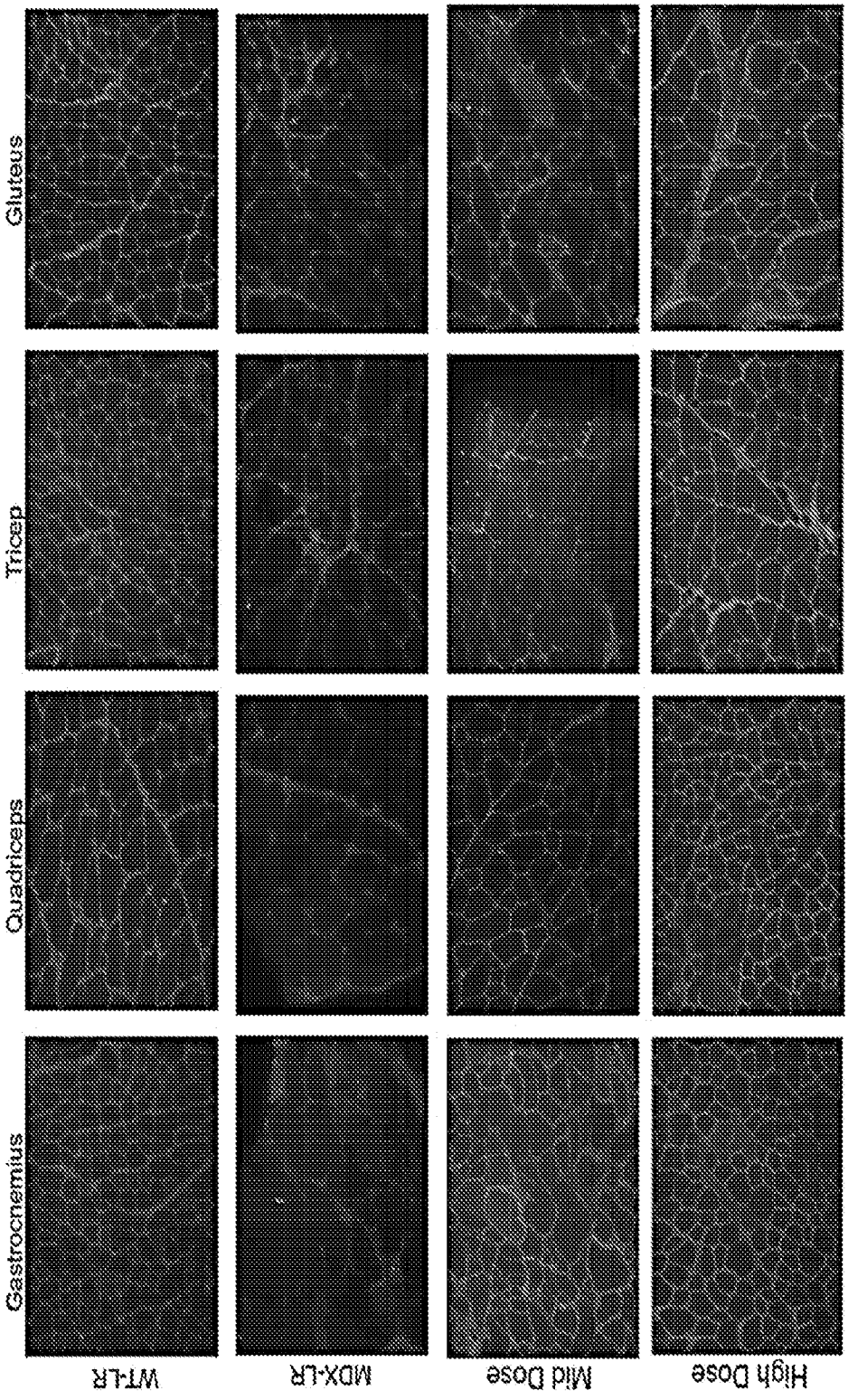

Gastrocnemius
Quadriceps
Tricep
Gluteus
WT-LR
MDX-LR
Mid Dose
High Dose

FIG. 16

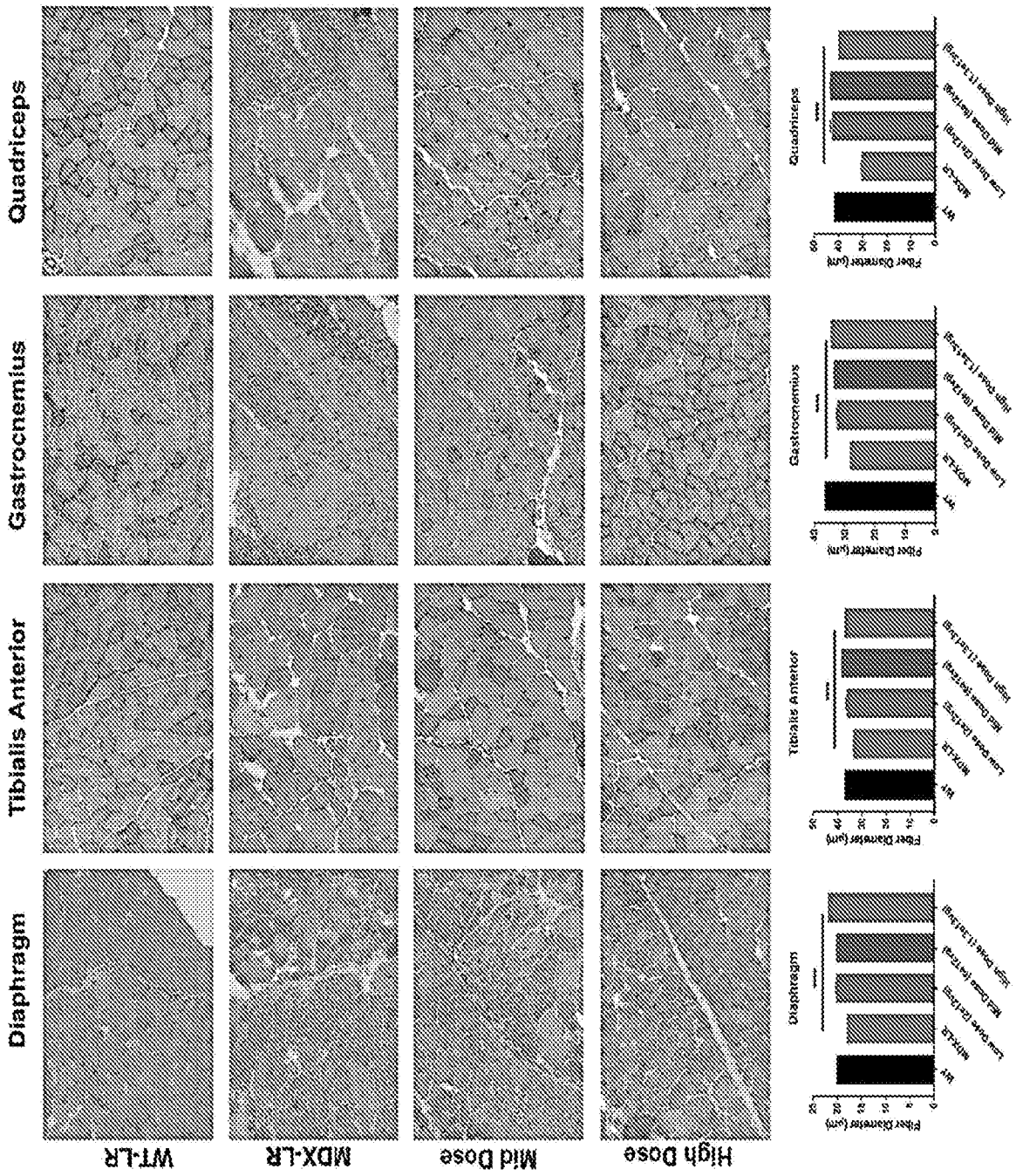

Diaphragm
Tibialis Anterior
Gastrocnemius
Quadriceps
WT-LR
MDX-LR
Mid Dose
High Dose
Fiber Diameter (um)
WT
MDX-LR
Low Dose (1e13vg)
Mid Dose (5e13vg)
High Dose (1.1e14vg)
****

FIG. 17

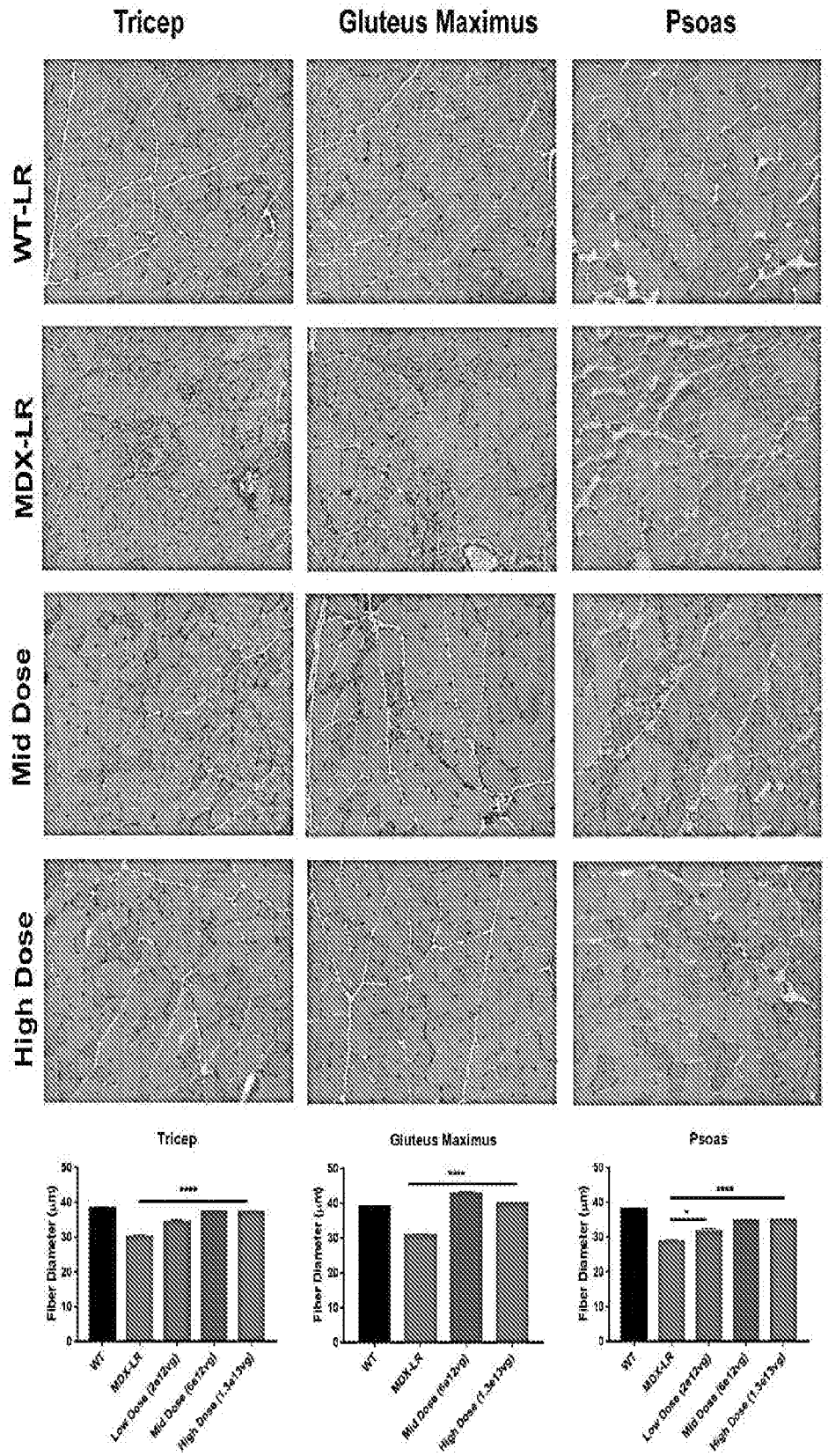

Tricep
Gluteus Maximus
Psoas
WT-LR
MDX-LR
Mid Dose
High Dose
Tricep
Gluteus Maximus
Psoas
Fiber Diameter (µm)
WT
MDX-LR
Low Dose (2e12vg)
Mid Dose (6e12vg)
High Dose (1.3e13vg)

FIG. 18

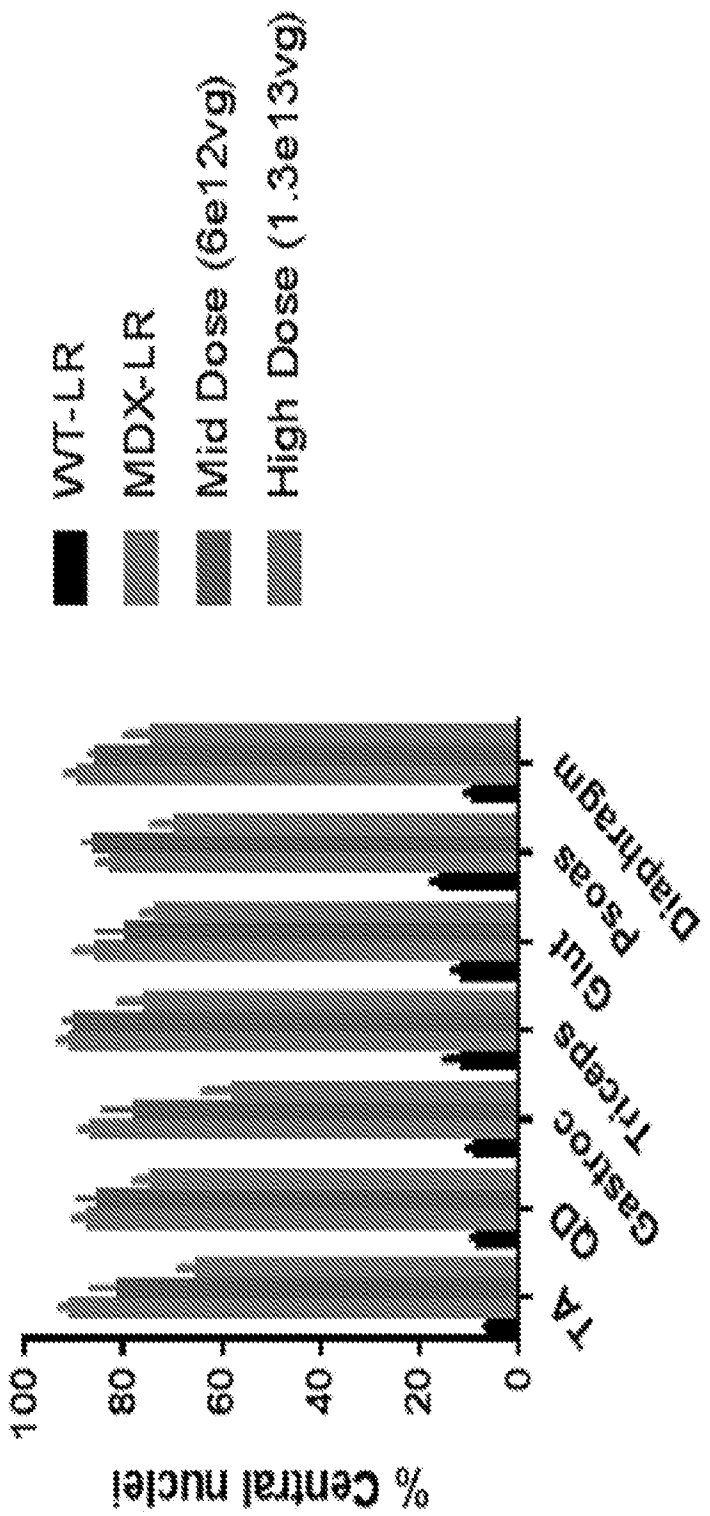

Central Nucleation
WT-LR
MDX-LR
Mid Dose (6e12vg)
High Dose (1.3e13vg)
% Central nuclei
100
80
60
40
20
0
TA
QD
Gastroc
Triceps
Glut
Psoas
Diaphragm

FIG. 19

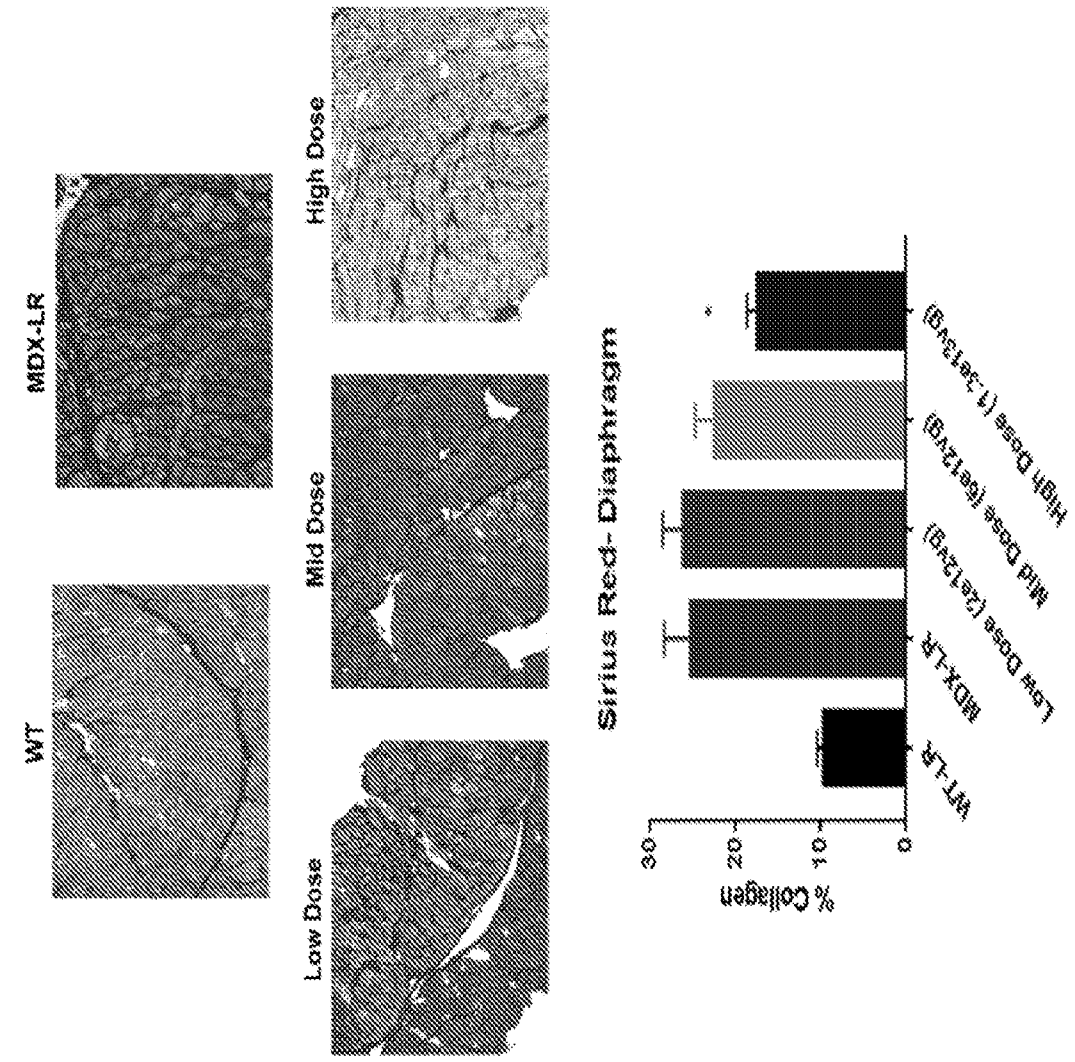

WT
MDX-LR
Low Dose
Mid Dose
High Dose
Sirius Red- Diaphragm
% Collagen
0
10
20
30
WT-LR
MDX-LR
Low Dose (2e12vg)
Mid Dose (6e12vg)
High Dose (1.2e13vg)

FIG. 20

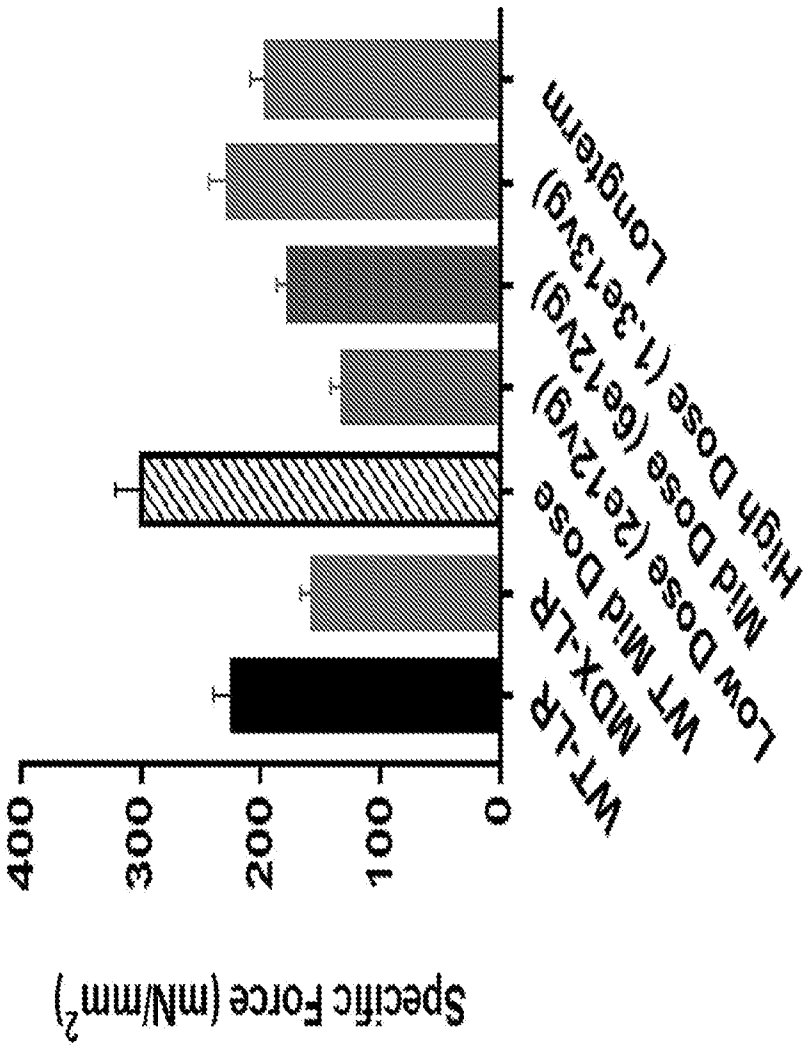

Specific Force-Dia
Specific Force (mN/mm²)
400
300
200
100
0
WT-LR
MDX-LR
WT Mid Dose
Low Dose (2e12vg)
Mid Dose (6e12vg)
High Dose (1.3e13vg)
Longterm

FIG. 21

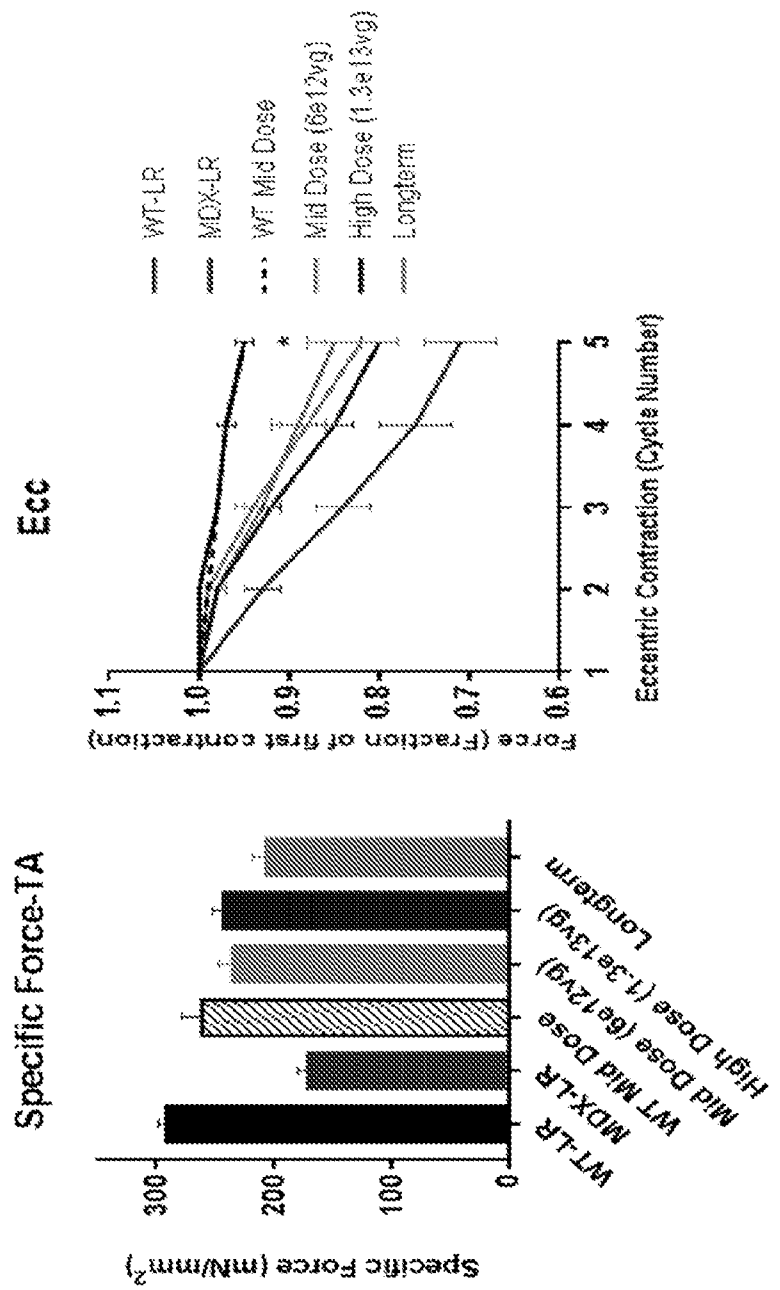

Ecc
WT-LR
MDX-LR
WT Mid Dose
Mid Dose (6e12vg)
High Dose (1.3e13vg)
Longterm
Force (Fraction of first contraction)
1.1
1.0
0.9
0.8
0.7
0.6
1
2
3
4
5
Eccentric Contraction (Cycle Number)
Specific Force-TA
Specific Force (mN/mm²)
300
200
100
0
WT-LR
MDX-LR
WT Mid Dose
Mid Dose (6e12vg)
High Dose (1.3e13vg)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

FIG. 22

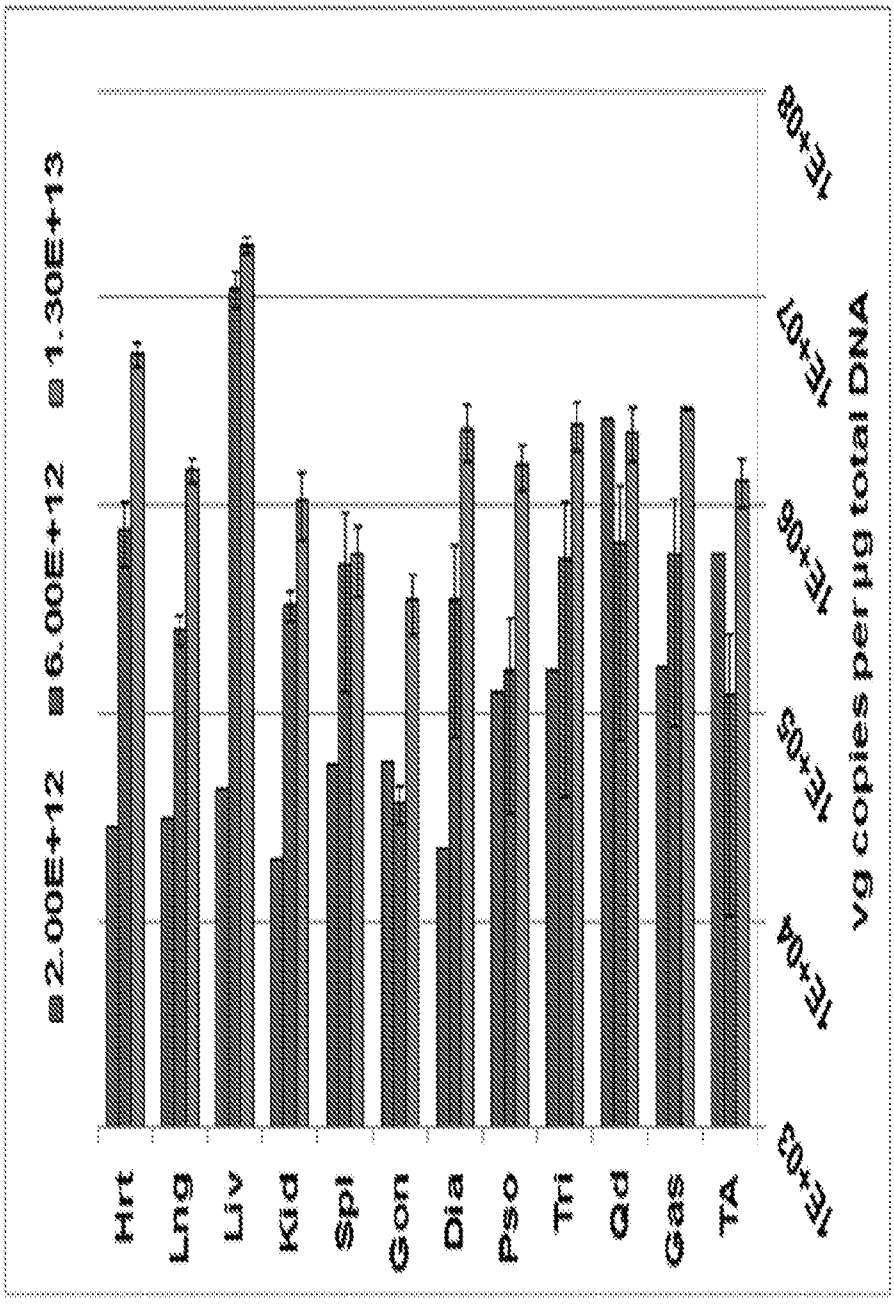

2.00E+12
6.00E+12
1.30E+13
Hrt
Lng
Liv
Kid
Spl
Gon
Dia
Pso
Tri
Qd
Gas
TA
vg copies per µg total DNA

FIG. 23

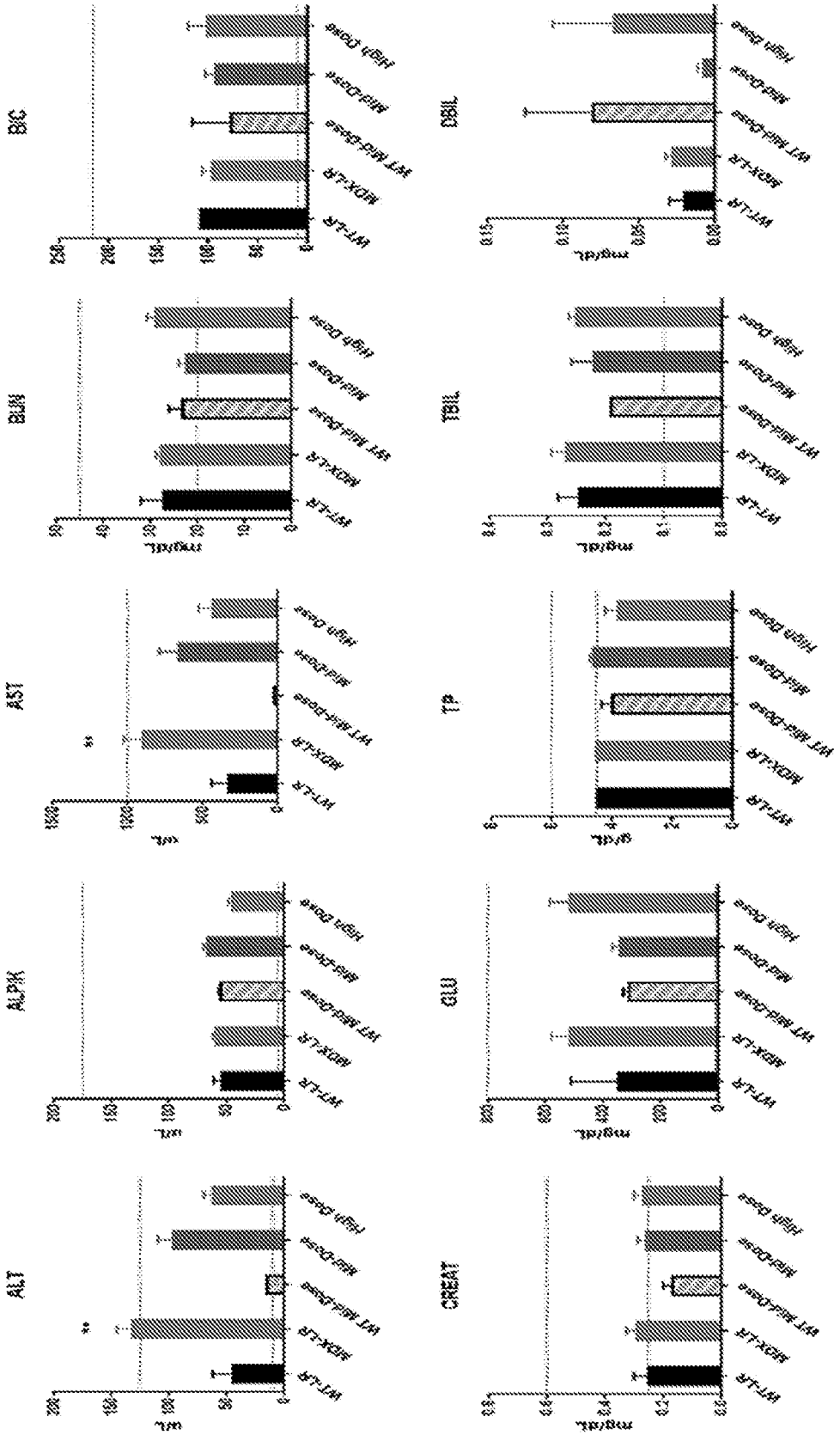

ALT
AST
ALPK
BUN
BIC
CREAT
GLU
TP
TBIL
DBIL
WT-LR
KDX-LR
WT Mid-Dose
Mid-Dose
High Dose

FIG. 24

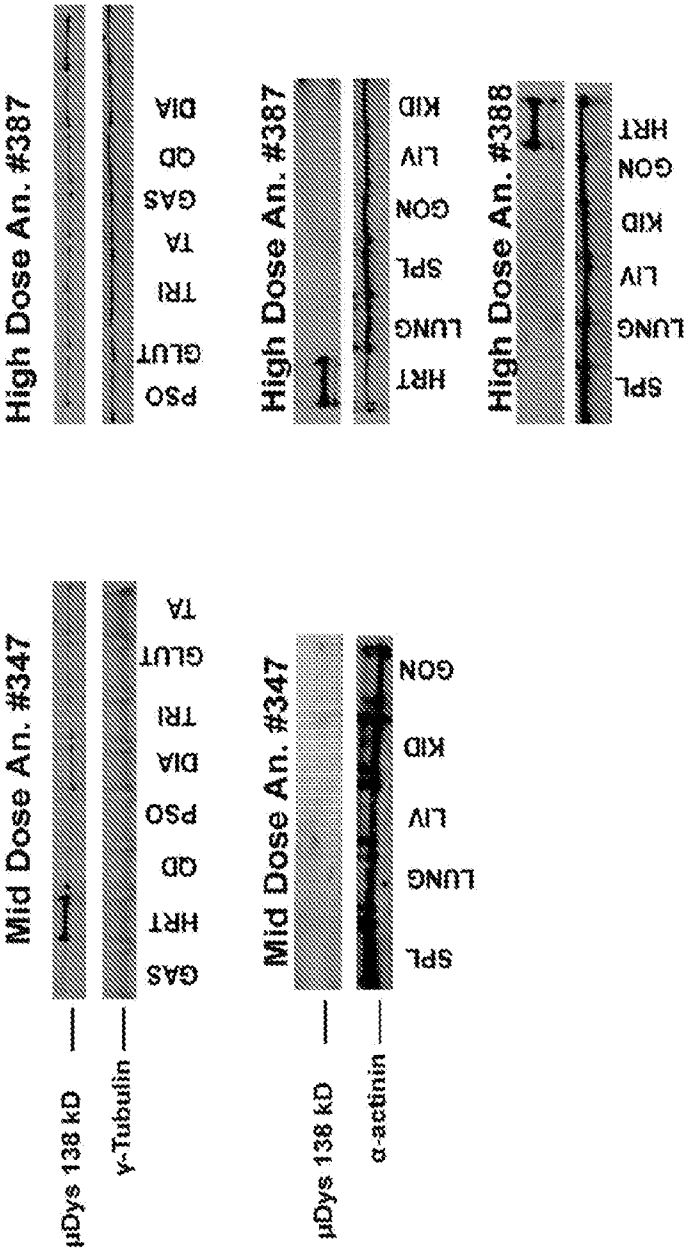

High Dose An. #387
PSO
GLUT
TRI
TA
GAS
QD
DIA
High Dose An. #387
HRT
LUNG
SPL
GON
KID
LIV
High Dose An. #388
SPL
LUNG
LIV
KID
GON
HRT
Mid Dose An. #347
GAS
HRT
QD
PSO
DIA
TRI
GLUT
TA
μDys 138 kD
γ-Tubulin
Mid Dose An. #347
SPL
LUNG
LIV
KID
GON
μDys 138 kD
α-actinin

FIG. 25

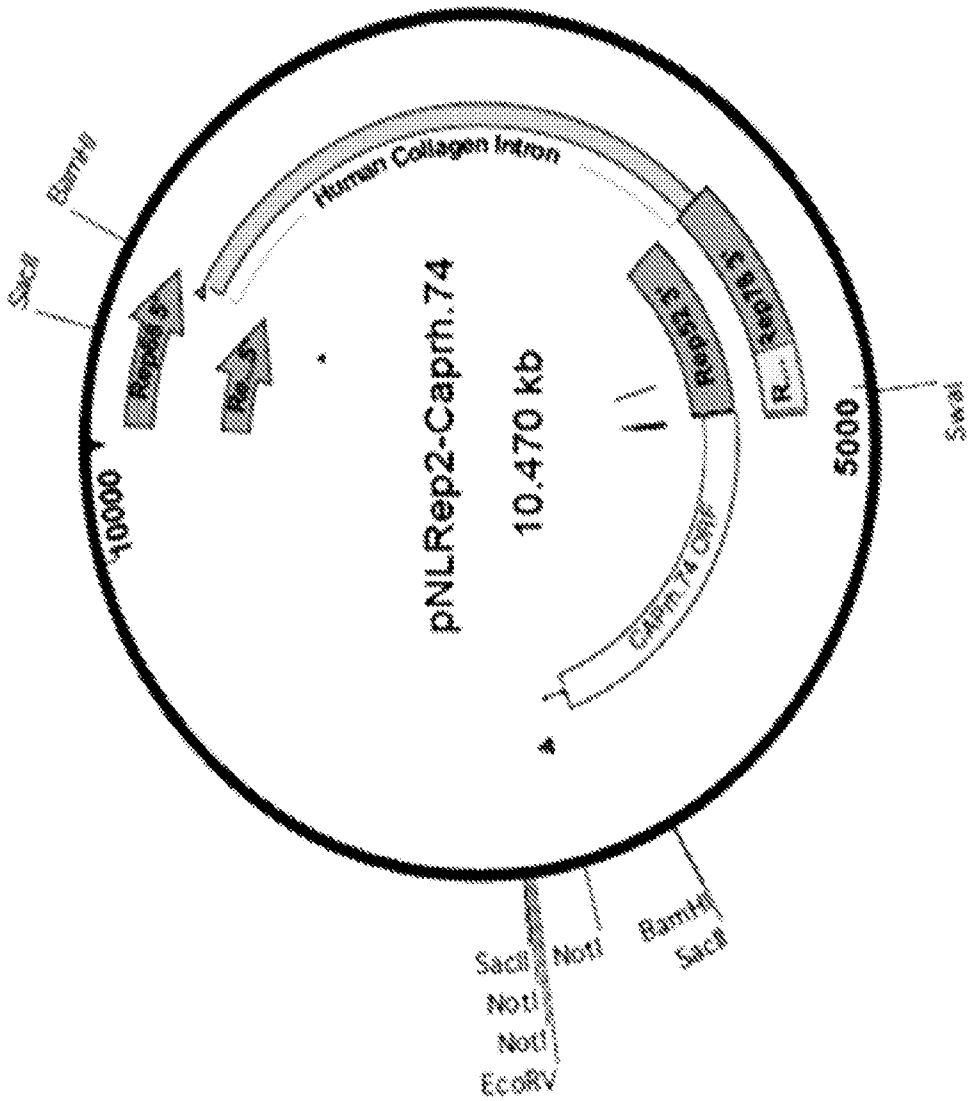

pNLRep2-Caprn.74
10.470 kb
Human Collagen Intron
Rep2-5'
Rep-3'
R-
10000
5000
SacII
BamHI
SwaI
SacII
NotI
NotI
NotI
EcoRV
BamHI
SacII

FIG. 26

