

FIG. 1

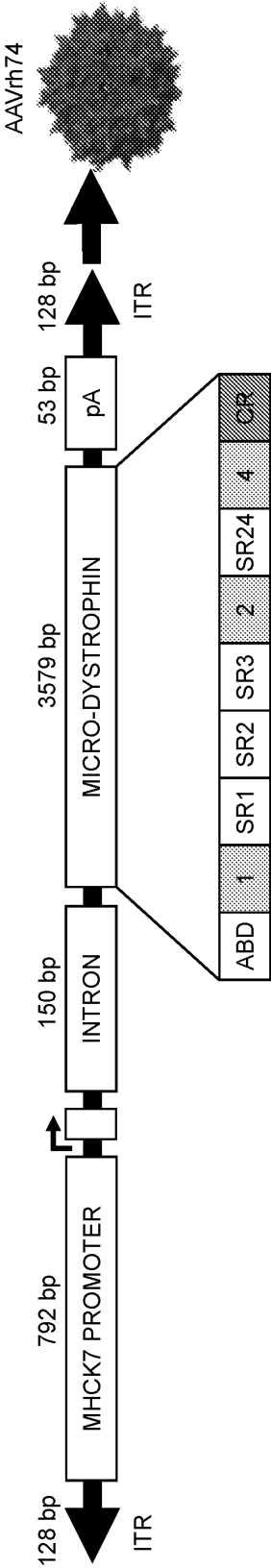


FIG. 2

SEQ ID NO: 3

rAAVrh74.MHCK7.microdystrophin

Main features:HRMHCK7 monomerChimeric micro sequenceHuman micro-dystrophin sequencePoly A tailAmpicillin resistance

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 CGCTCGCTCGCTCAGAGCGCCCGCCGGCAAGCCCGGGCTCGGGCGACC
 TTGGTCGCCCGGCTCAGTGAGCGAGCGCGCGCAGAGGGAGTGGCCA
 ACTCCATCACIAGGGGTTCCTTGTAAGTAAATGATTAACCCGCCATGCTACTTATCT
 ACGTAGCCATGCTCTAGAgtttaacaaacttgcattgtatagatgaaccccttcaattaaaaataactaaggtggt
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 GTGCCAGCAGTCTGCCACACAGCGCTGGACATGCCCTTCAATATCGCTCGGTACCA
 GCTGGGGATCGAAAACTGCTGTGACCCAGAGGATGTGGACACTACATACCCAGATA
 AAAAGTCTATTCTGATGTACATTACTAGCCTGTCTCCAGGTGCTGCCACAGCAGGTGT

FIG. 2 con't

SEQ ID NO: 3

rAAVrh74.MHCK7.microdytrophin

Main features:IIRMHC7 monomerChimeric micro sequenceHuman micro-dytrophin sequencePoly A tailAmpicillin resistance

CGCTACAGGAGTGTGAACAGATTTAACTAGGATATTGGCAGAGCTGCTCTTTTCGG
 GAAGAGTGGCCAAAGGACACAAGAGTCACTACCTATGGTGGATATZTGGACCCCA
 ACTAGATGGGGAAGATGTGGCGATTTTGGCCAGGTCCTGAAGAATAAGTTTGGG
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 CCGCGAGCGCCGGCTTTGGCCGGCGGCTCAGTGTGAGCGAGCGAGCGCGC
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 GACGGGTGTGTGCTCCGGCATCCGCTACAGCAAGCTGTGATCCGCTCCGGGA
 GCTGCAZGTGTGAGAGGTTTTCACCGTCAZCACCAGAACCGCGGAGACGAAAGGGC
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 CAGCTGGCACTTTTCGGGAAATGTCCGGGAACCCCTATTTTATTTTTCTAAAT
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 TCGCGCATACACTATCTCAGAAZTACTTGGTGTGAGTACTCAGCAGTCACAGAAA
 GCAZCTTACGGATGGCAZGACAGTAAGAGAAATTAAGCAGTGTGCTGCTATAACCATGA

FIG. 2 con't

SEQ ID NO: 3

rAAVrh74.MHCK7.microdystrophin

Main features:IIRMHCK7 monomerChimeric microdystrophin sequenceHuman micro-dystrophin sequencePoly A tailAmpicillin resistance

GTGATAACACIGCGGCCAACTTACCTCTGACAAAGATCGGAGGACCGAAGGAGCTA
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 ACAATTAAATAGACTGGATGGAGGCGGATAAAGTTCAGGAOCCACTTTCGGCTCGG
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 GCGGTATCAATGACGACCTGGGCGCAGATGCTTAAGCCCTCCGTAATCGTAGTAACT
 ACAGCAGCGGGAGTCAAGCAACTAIGGATGAACGAAATAGACAGATCGCTGAGATA
 GGTCCTCACTGATTAAGCAATGGTAACCTGTACAGACCAAGTTTACTCATATATACTTT
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 CGTAGAAAAGATCAAGGAATCTCTTGAGATCCTTTTTCGCGGTAATCTGCTGC
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 TCTTCTAGTGTAGCCGTAGTTAGGCCACCACCTTCAAGAACTCTGTAGCACCGCTAC
 ATACCTGCTCTGCTAATCCTGTTACCAAGTGGCTGTGCCAGTGGGATAAAGTCGTG
 TCTTACCGGGTTGGACTCAAGACCATAGTTACCGGATAAGCGCAGCGTCCGGCT
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 AGATACCTACAGCGTGAGCTATGAGAAAGCGCACGCTTCCGAAAGGAGAAAGGC
 GGACAGGTATCCGGTAACCGGACGGGTGGGAACAGGAGAGCGCACGAGGGAGCTT
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 GAGCGTCGATTTTGTGATGCTGTACAGGGGGGGGAGCCTATGGAAAAACGCCAG
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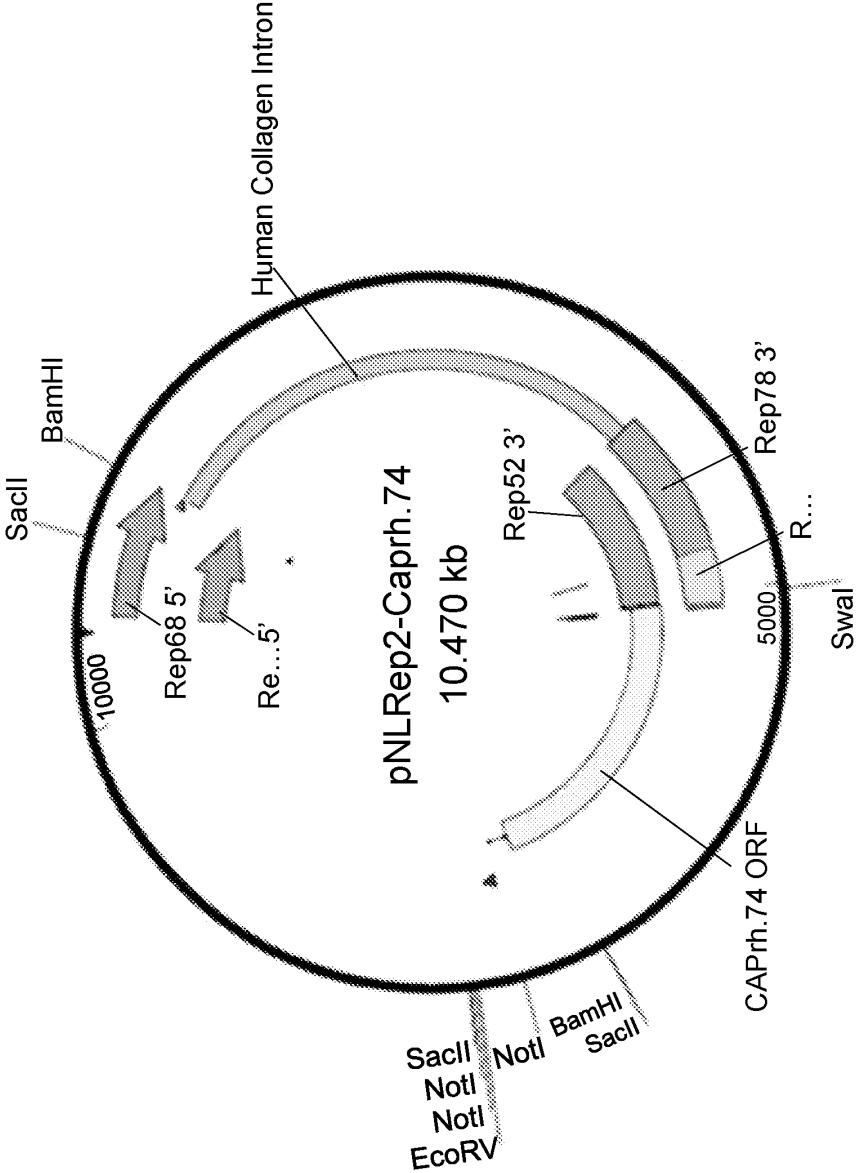


FIG. 3

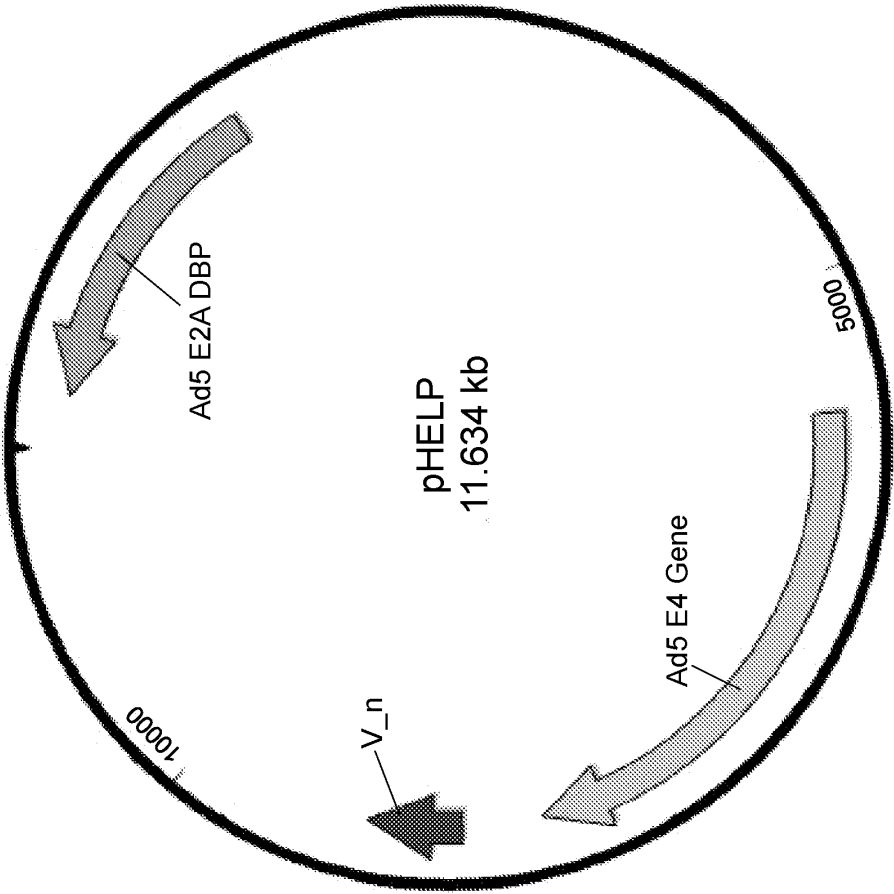


FIG. 4

FIG. 5

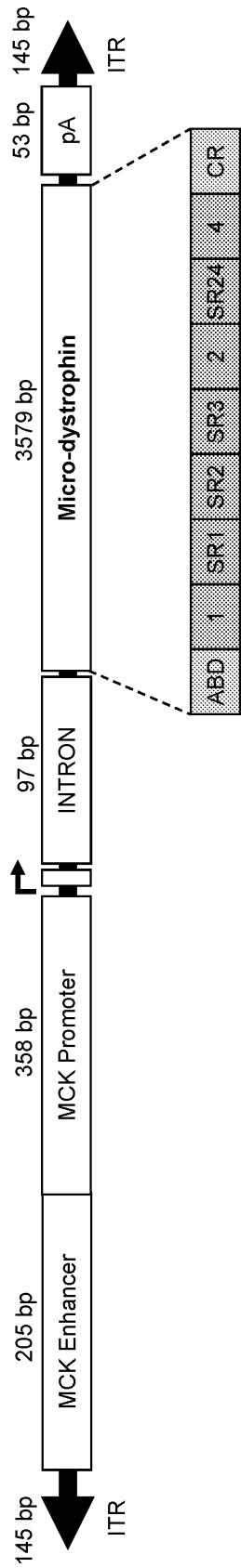


FIG. 6

SEQ ID NO: 5

rAAVrh74-MCK-micro-dystrophin

Main features:ITRMCK promoterChimeric intron sequenceHuman codon optimized micro-dystrophin sequence -Poly A tailAmpicillin resistancepGEX plasmid backbone with pBR322 origin of replication

GCCAATACGCAACCGGCTCTCCGCGGCTTSSCCGATTCATTAAATCCAGCTGGCGGCTGCTGGCTCACTGA
 GGCCTCCCGGGCAAGCTCCGGGCTGGGGGACCTTGGTCTGCTCCGCTCAGTGAGCGAGCGGAGCGGCGAGA
 GAGGAGTGGCCAACTCCATCCTAGGAGGTTCTTGTAGTTAATGATTAAACCGCATGCTAATTATCTACGTAGC
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 GTATAATTAAACAGACATGTGCTCTCCCTCCGCAACACCTGCTGCTGAGCTCAGCCCAACCCCGGCTGCC
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 ACTAGGCGCAGGCTGTAAAGGCTTGGGGCCAGGAGCTTATAGTGGCTGGGACTGCCAAGTATTACTGTCCA
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 CCTTGGGGCAGCCCATACAGGCTATGGGCTGGGCAAGCTGCAAGCTGGGCTGGGCTGGGCTGGGCTGGGCTGGC
 GGGCAAGCAGCTGAAGCTCACTGCTCTCAGGGGCTGCTGCTGGGCTGCTGCTGGGCTGCTGCTGGGCTGCTGCT
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 GGGAGCACTAGGCTGAGGCTGAGGCTGAGGCTGAGGCTGAGGCTGAGGCTGAGGCTGAGGCTGAGGCTGAGGCTGAGGCT
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 GACTGGCAGGCTGAAGTGAAGTGAAGTGAAGTGAAGTGAAGTGAAGTGAAGTGAAGTGAAGTGAAGTGAAGTGAAG
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[illegible]

FIG. 6 con't

SEQ ID NO: 5

rAAVrh74 MCK micro-dystrophin

Main features:ITRMCK promoterChimeric intron sequenceHuman codon optimized micro-dystrophin sequence -Poly A tail

Ampicillin resistance

pGEX plasmid backbone with pBR322 origin of replication

CCSCCTACAGACAAGCTGTGACCGCTCTCGGGAGCTGCATGTGTGTCAGAGGTTTTCACCGTCATCACCAGAAACGGCC
 GAGACAAAGGGCCTCGTATACGCTATTTTATAGGTTAATGTGTCATGATAATAATGTTTCTTAGACGTGAGGT
 GGCACCTTTTCGGGGAATGTGCGGGAACCGCTATTTGTTTATTTTCTAAATACATTCAAATATGTATCGCTCAT
 GAGACAATAACCTGATATAATGTCTCAATAATATTGAAAAAGGAAGATGATGAGATATCAGCAATTCGGTGTGCGC
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 GATCAGTTGGGTGACGATGGGTTACATCGAATGGATCTCAGACAGGGTAAAGATCTTGAGAGTTTTCGGCC
 GAAGACGTTTTCGAATGATGAGCAATTTAAGGTTCTGCTATGTGGCGGGTATTAATCCGTATGACCGCGGC
 AAGAGCACTCGGTGCGCATACCTATTTTCAGAAATGACTTGGTTGAGTACTCACAGTCCAGAGAAAGCATCT
 TACGATGTCATGACAGATAGAGAAATATGCAATGCTGCCATAGCATGAGTGAATAACATTCGGGCCAATTACTT
 CTBACAAGATCGGAGGACGGAAGGAGCTAACCGTCTTTTTCACAAAGATGGGGATCAATGTAACCTGGTTGAT
 CGTTGGGAACCGGAGCTGATGAAGCCATACCAACGACGAGGTGACACACGATGCTGTAGCAATGGCAAC
 AACGTTGGCAAACTAATTAATGGGCACTACTACTCTAGCTTCCCGGCACAAATTAATAGATGGATGGAGGCG
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 GGGGAGTCAGGCAACTATGGAATGACGAATAGACAGATGCTGAGATAGTGGTCACTGATTAAGATTTGGT
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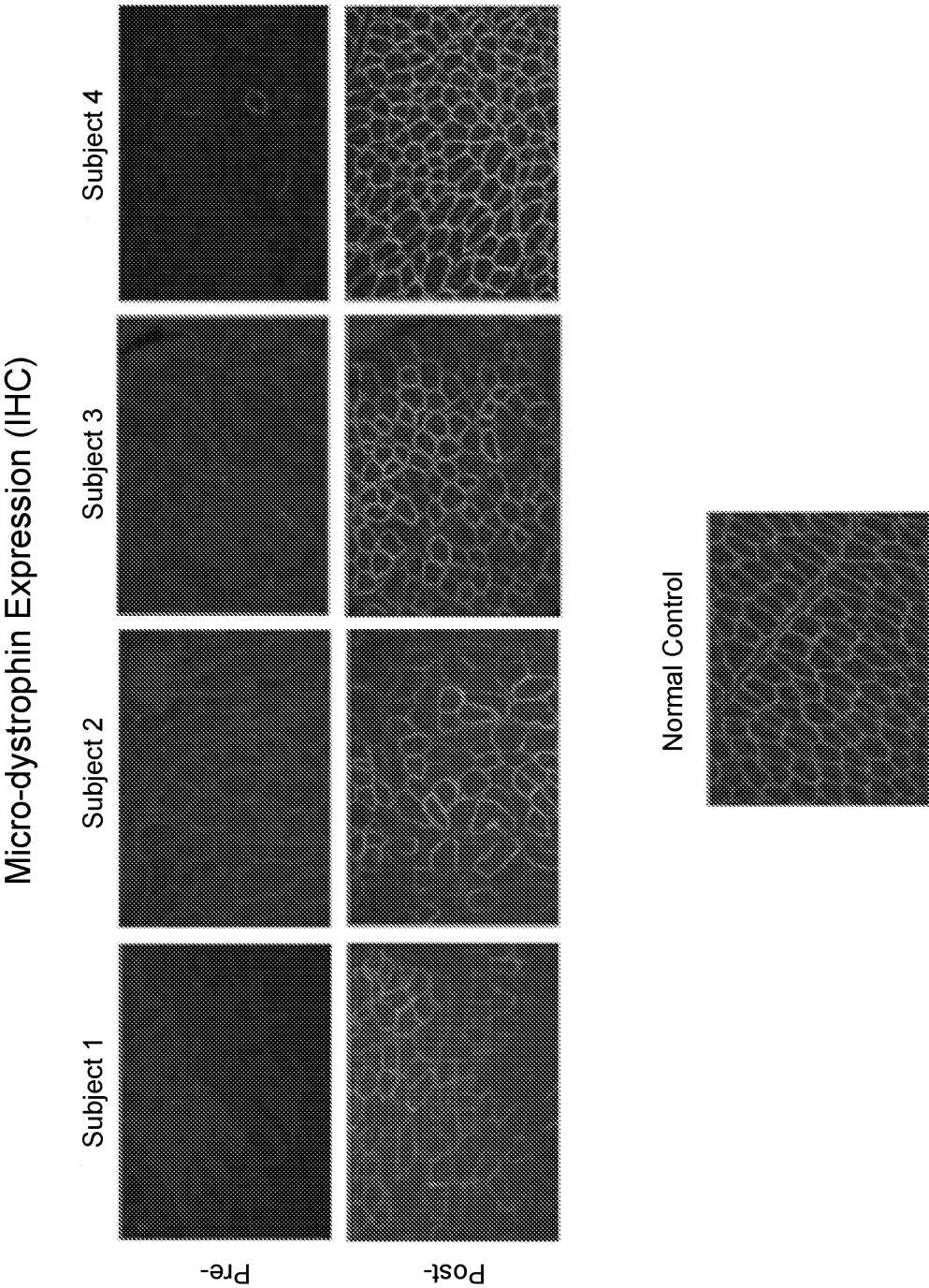


FIG. 8A

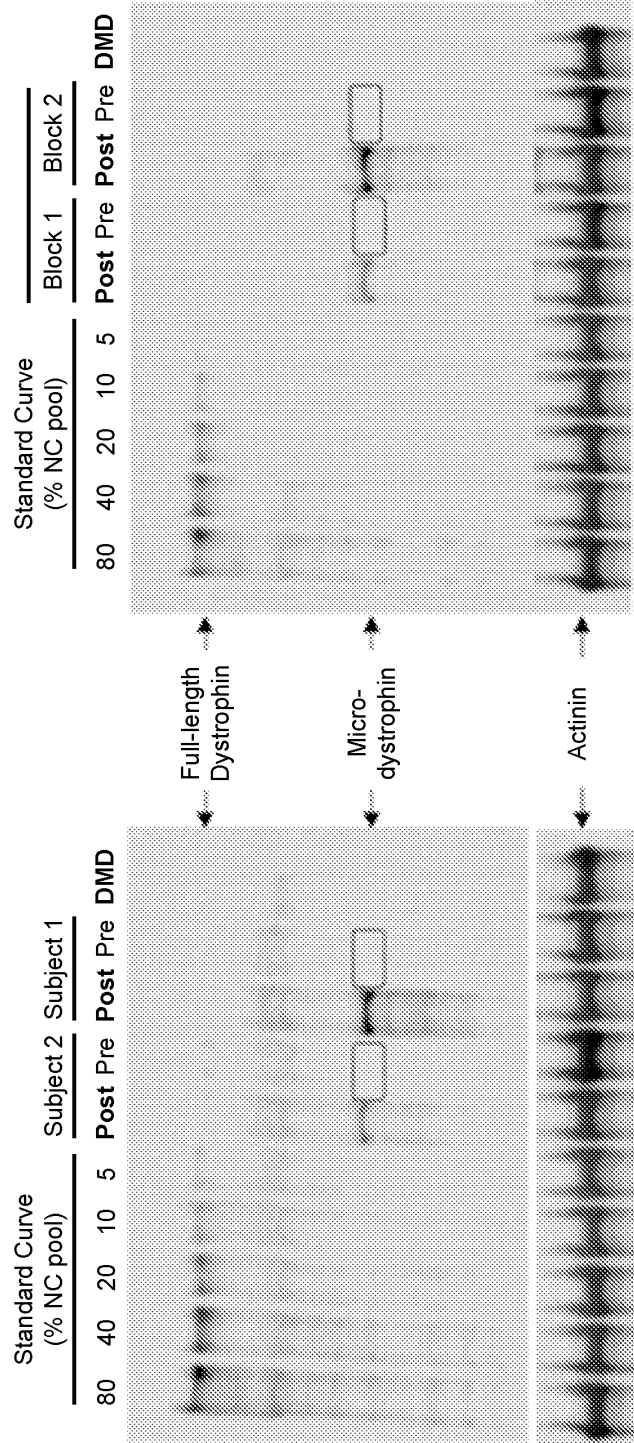


FIG. 8B

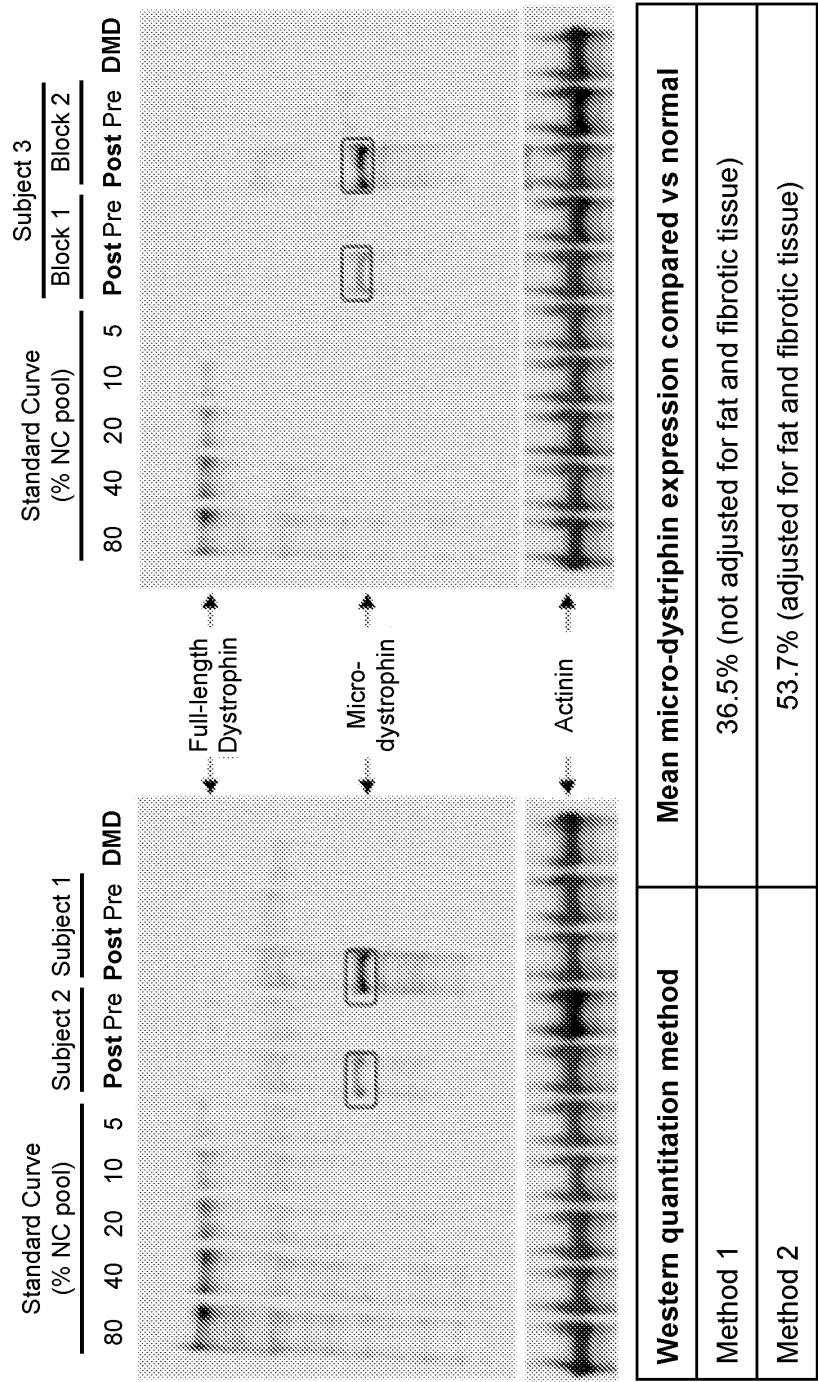
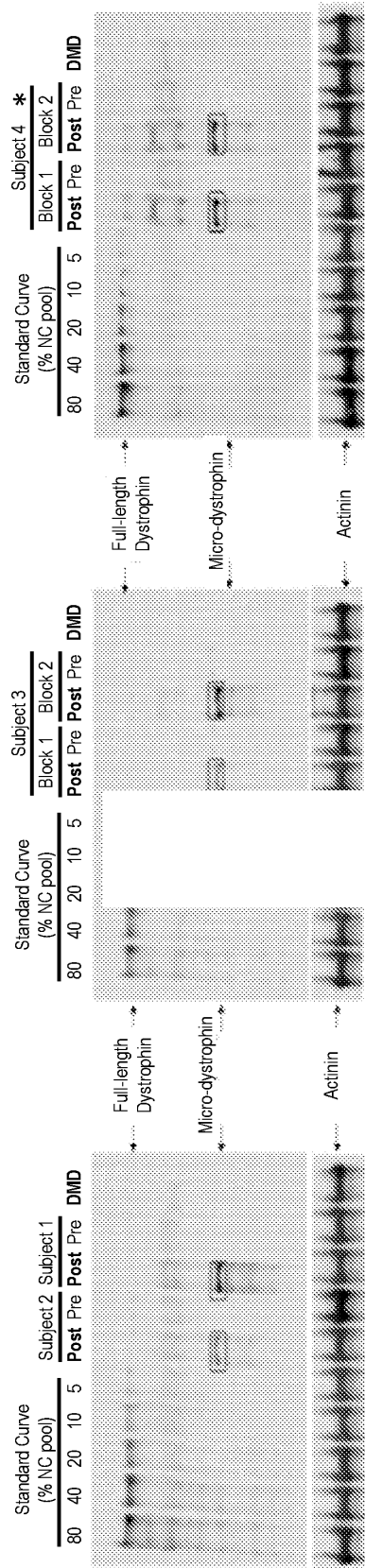


FIG. 8C



Western quantitation method	Mean micro-dystrophin Expression (N=4) vs Normal
Method 1	74.3% (not adjusted for fat and fibrotic tissue)
Method 2	95.8% (adjusted for fat and fibrotic tissue)

FIG. 9A

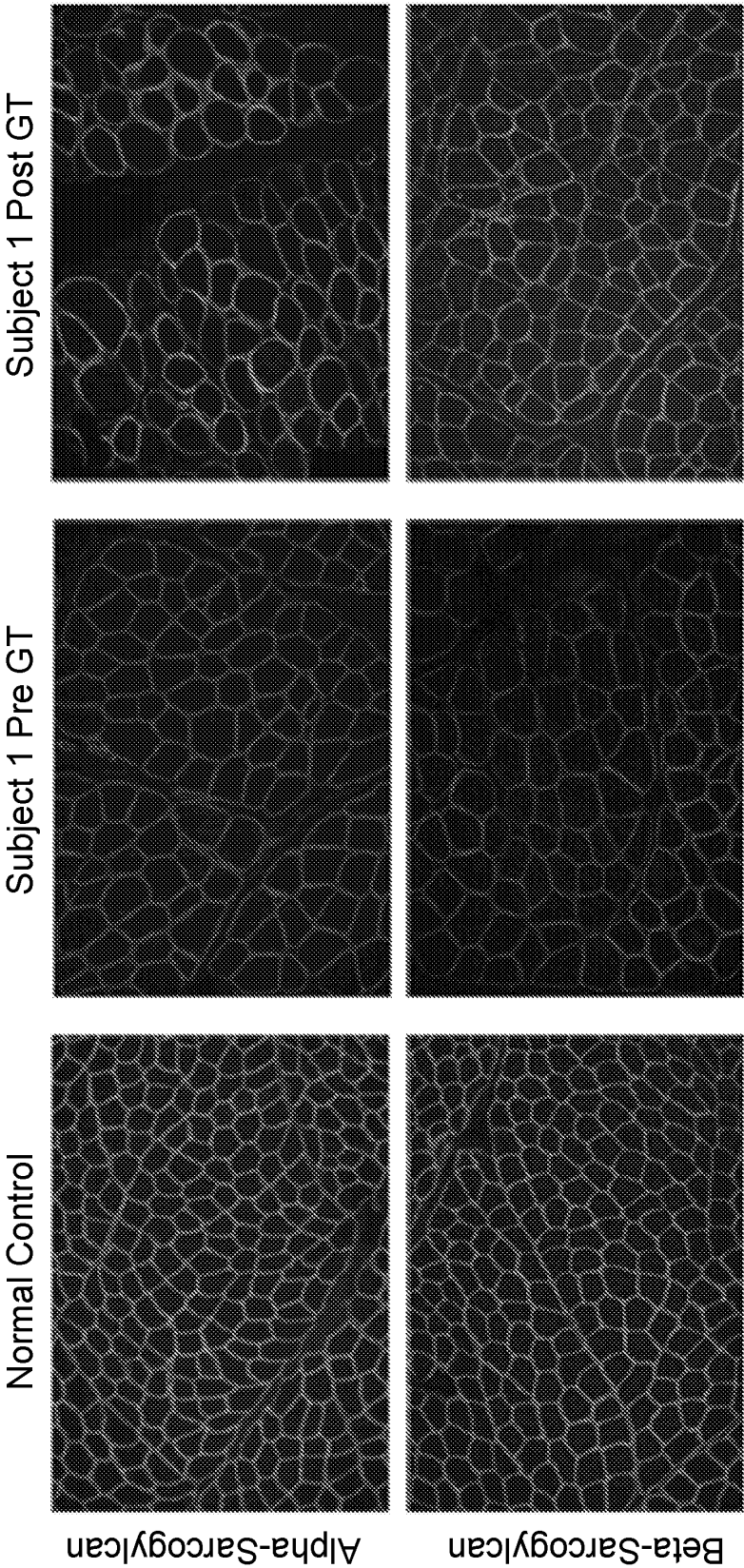


FIG. 9B

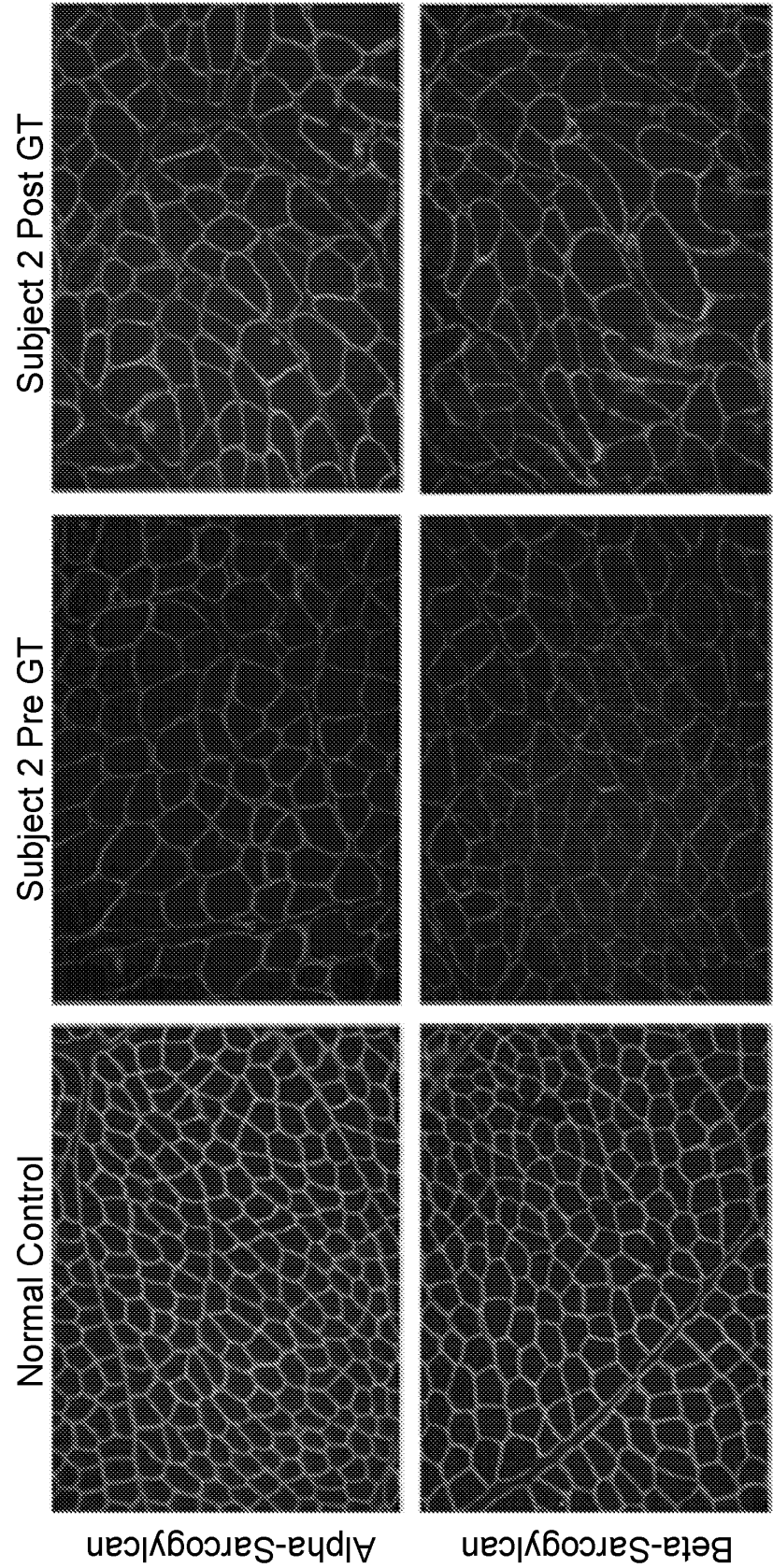
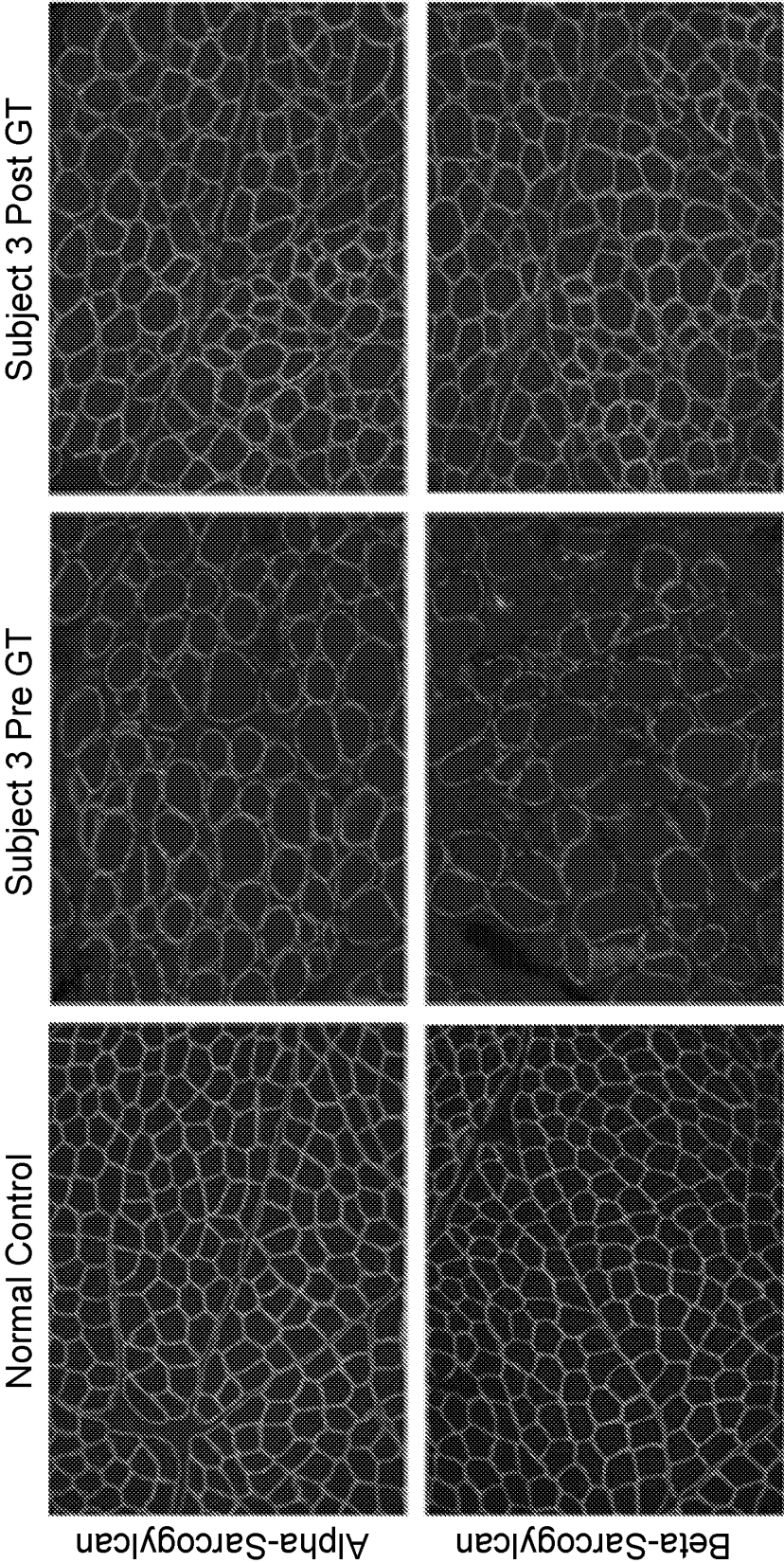


FIG. 9C



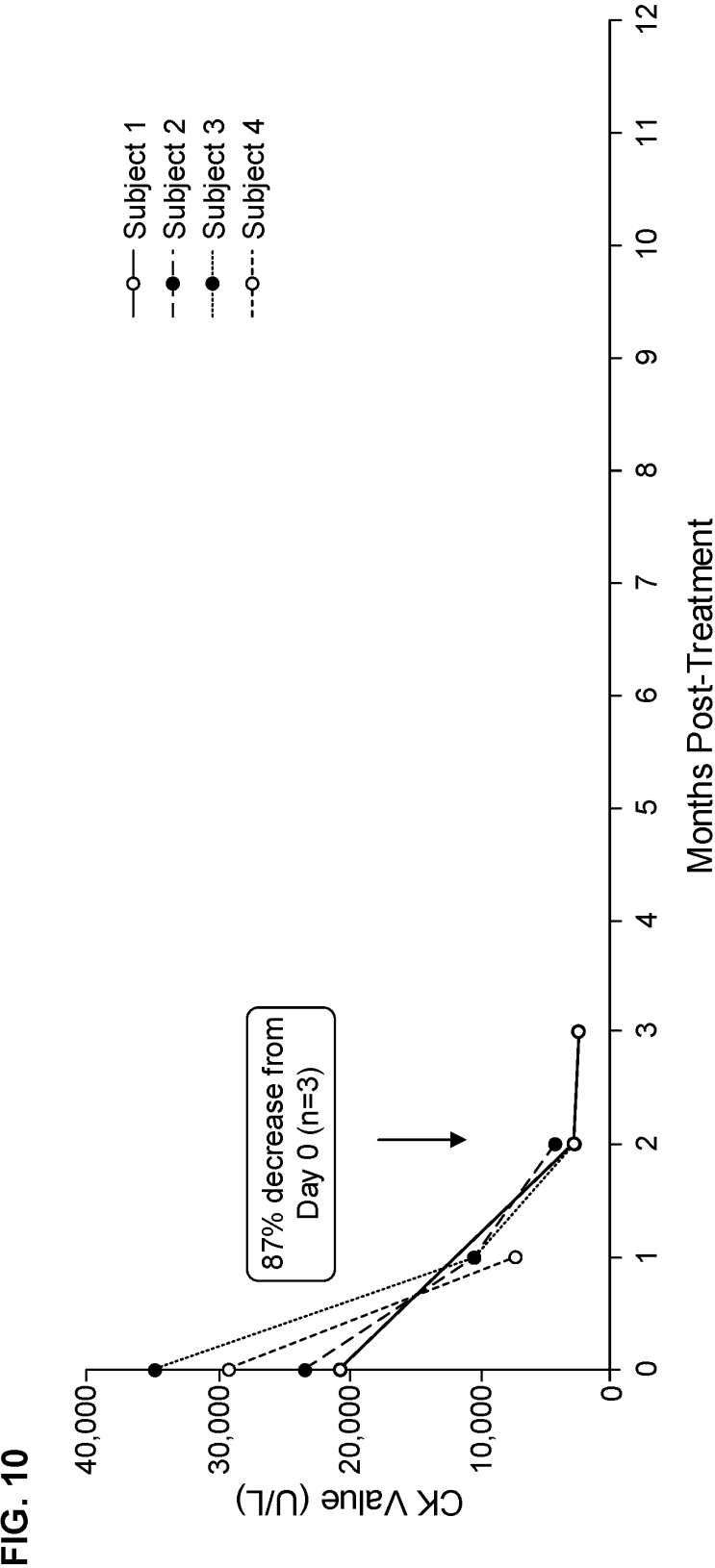


FIG. 11

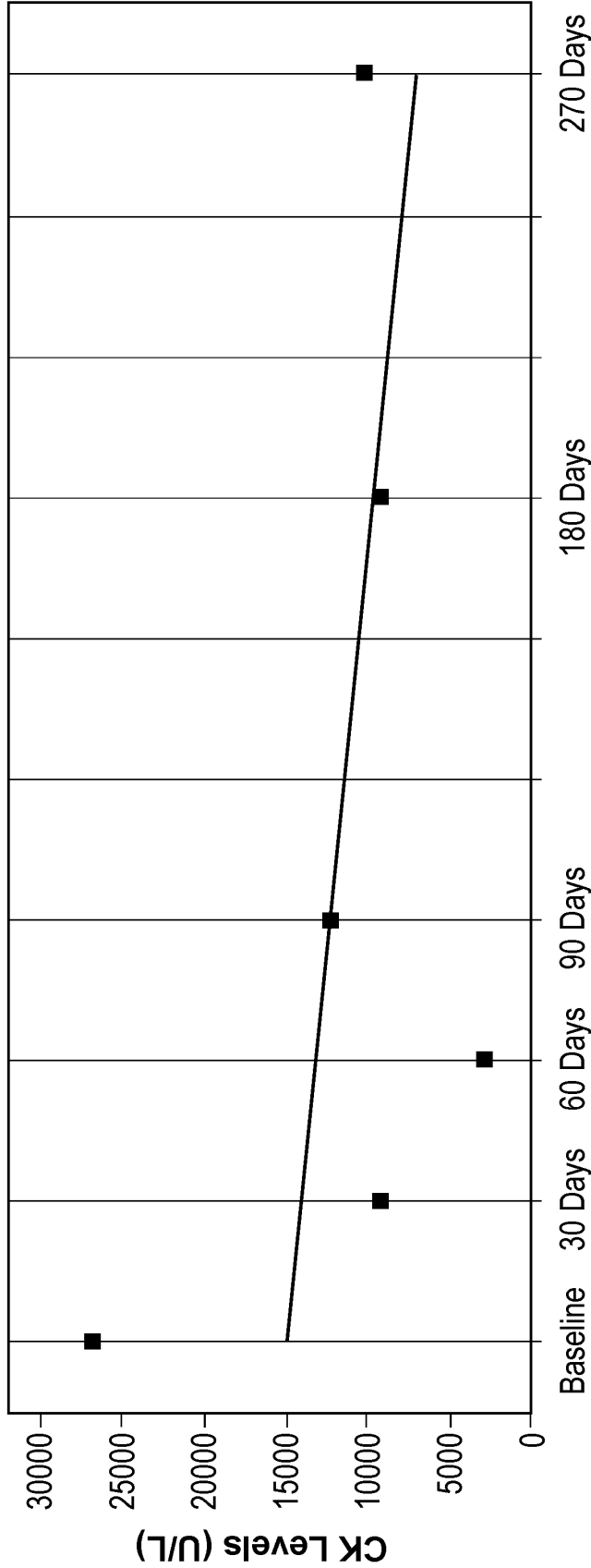


FIG. 12

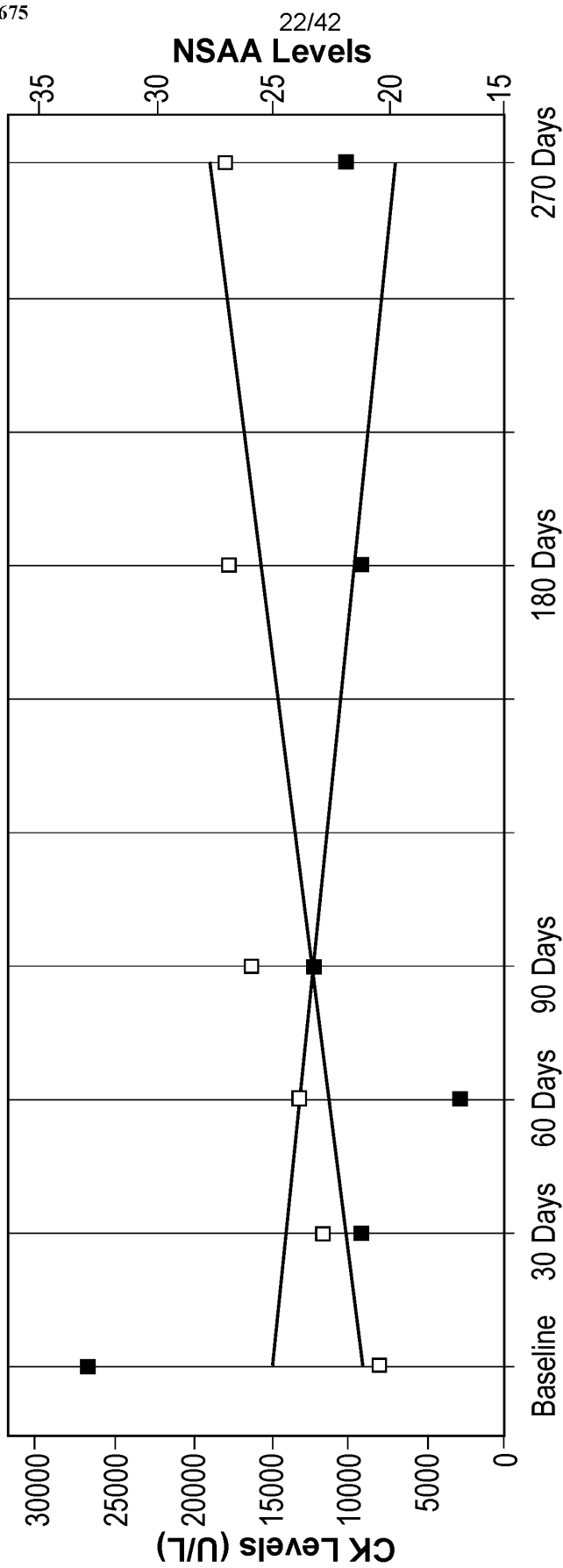


FIG. 13

GCGCGCTGCTGCTACTAGGCGCGCGGCAAAAGCCCGGGCTCGGGCGACCTTTTGTCGCCCGCTCAGTGAGCGAGCGCGCGCAGAGAGGGAGTGGCCAACTCCATCACTAGGGGTTCTCT
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 AGGAATGCCATTATCGCTACAGGAGTCTGAAACATTTTAACATACGATTTTCCGGAAGAGTGGCCAAAGAGTGGCCAAAGACACAGATGACACTACCTATGTTGGGAATATTG
 ACCCAACTACATCTGCGGAAGATGTCGCGATTTTCCAAAGTGTGAAGATAAGTTTCGAGCTAAGAGTACTTCGCCAAAGACCCCGCATGGGTATCTGCCAGTGCAGACAGTCTGGGAAGGA
 GACAATATGGAGACGATACATGTAGCGGCCGCAATAAAGATCTTTATTTTCTATAGATCTGTGTGTTTTTTGTGTGtctagtagtcagcagtaggctagtagtaagtagcaggggttaatacttaactac
 aAGGAACCCCTAGTGGAGTTGGCCACTCCCTCTGCGGCTCGCTCACTAGAGCGCGGGGACCAAGAGTGCAGGAGCGGCGCTTGGCCGCGGCGCTCAGTGAGCGAGCGGCGCGG

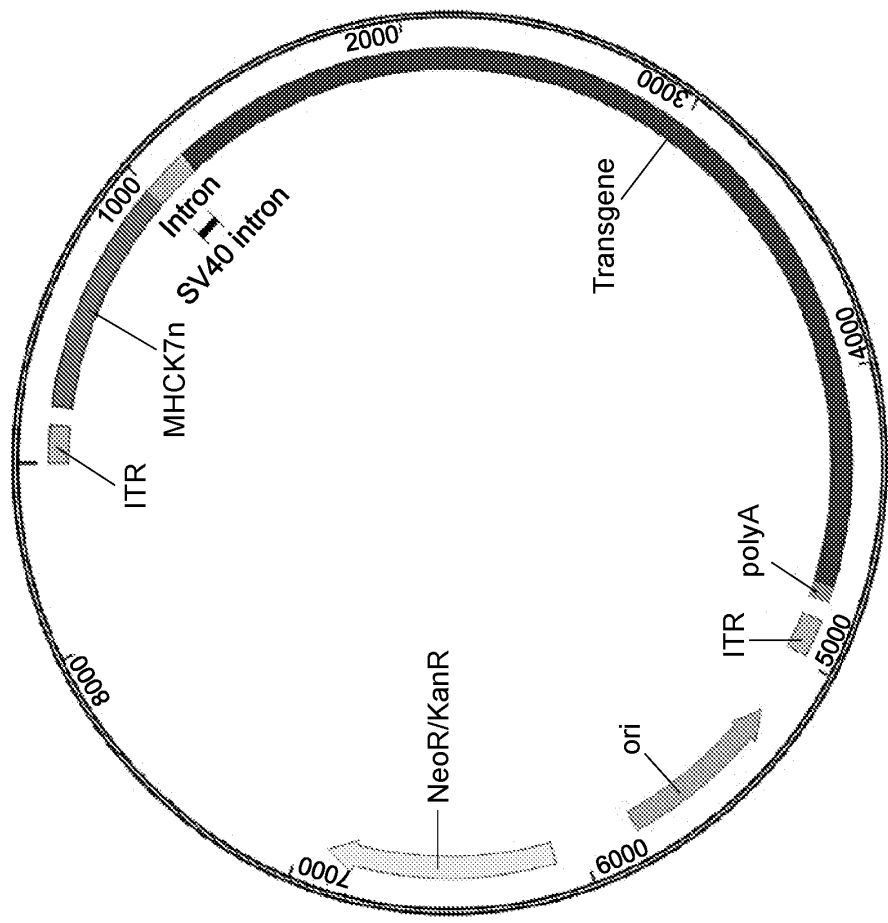


FIG. 14

80-106

[illegible]

FIG. 15 con't

SEQ ID NO: 8

Main Features:

ILRMHC2 promoterChimeric Intron sequenceHuman micro-dystrophin sequencePoly A tailKanamycin resistance

TGAGCTGGCTCAGGGATATGAGCGCACCGAGTAGTGCCAAAACCCAGGTTCAAGTCC
 TACGCTATACCCAGGGTGGCTACGTGACAACCTAGCGACCTACTAGATGCCCTTT
 CCATCCAGCACTGGAGGGTCCAGAGGACAAGAGCTTTGGGTCCAGCCTGATGGA
 AAGCGAGCTGAATCTGGATGGTACCAGACAGCCCTGGAGGAGGTGGCTGAGCTGGC
 TGTGTAGTGTGAAGACACACTGCAAGGCCAAGGGGGAATTTCGAATGACHTGGAA
 GTGTGAAGGATCAGTTCCACACACAGAGGGCTATATGAIGGACCTGACAGTCA
 CCAGGGCGCTGGCCAATATCTGACGTGGGTCTAACTGATCGGCACCGGA
 AACTGAGTGAAGGAGGAAACAGAAGTGCAGGAGCAGATGAACCTGCTGAACAG
 CCGCTGGGAGTGTGTGAGATGGCTAGTATGGAGAACAGTCCAACTGCAACGGG
 TGCTGATGGACCTGCAGAACAGAAACTGAAAGAGCTGACGACTGGCTGACAAAG
 ACTGAGGAACGCACAAAGGAAGATGAGGAGGAGCCACTGGGACCCGACCTGGAGG
 ATGTGAAGAGACAGCTGCAGAGCAGTAAAGGTGCTGCAGCAGGAATCTGGAAACAGGA
 GCAGGTGCGGGTGAATCTCCGTGACACATATGTTGGTGGTGGTGAAGAAATGATGIG
 GAGATCAAGCCACCGCCGCTGGAGGAACAGCTGAAGGTCTGGGGGACCGGTGG
 GCCAAGATTGGCGGTGGACCGAGGAACAGTGGGTCTGCTGAGGAGACATCTGCT
 GAAATGGCAGAGGCTGACCGAGGAGCAGTGTCTGTGTAGTGTGGCTTGGCTGACGGAGA
 AAGAGGACGCGGTGAACAAGATCCACACACACCGGCTTTAAGGAACAGAAAGAAATG
 CTGTAGCTTGGAGAAACTGGCTGTGCTGAAGGCCGATGTGGAGAAAAAGAGGA
 GAGCATGGGCAACTGTATAGCTGAAACAGGAACCTGCTGAGCACTTGAAGAACAA
 AGAGGTGACCCAGAAAGACAGAGCTGGTGGATAACTTTGGCCCTGTCTGGGAC
 AACCTGGTGCAGAACTGGAGAAAGTAAGCTCAGATCTCTCAGGCTGTGACCCAC
 AACCAGCTAGCTGACCCAGACACACCTGTATGGAACCGTGACACACCGTGACAA
 CCGCGGAACAGATCTCTGGTGAACATGCCAGGAAGAGCTGCCACCTGCACCTGCC
 CAGAAGAAGAAACCTTCGAGCGGCTGCAGGAGCTCCAGGAAGCCACTGACGAAC
 TGGACGTGAAGCTGAGGCAAGCCGGAAGTGTATTAAGGGTCTTGGGAGCCTGTGGGC
 GATGTGCTGATTGATCTCTGACGACCACTGTGAAAGGTGAAAGGTGAAAGGTCTGAGAGG
 CGAAATCTCTCCACTGAAGGAAACGTGAGTCAATGTGAACGATCTGGCTAGACAGC
 TGACAACACTGGATACCACTGTAGGCCATAGCAATGTGAGCAACTGGAGGACCTG
 AATACAGGTGGAAAGCTGTCTCAGGTGGGTGTGGAAGACCGGTGCGGACCTGCA
 TGAGGCCCATTCGGACCTTCGACCCAGCCAGCCAGCACTTCGTAGCCACATCCGIGCA
 GGCGCTCTGGGAGAGGCCAATTTCTGGAACAAGGTGGCCCTACTATATTAATCAGGA
 GATCCAGACCACTGTGTGGACCATCCCAAGATGACAGAACTGTACCCAGTCCCTGG
 CCGATCTGAACAACGTGAGGTTTAGGGCTTACAGAACCGCTATGAAGCTGAGACGG
 CTGCAAGGCGCTGTGGGATCTGCTGTCTGTGGCGCGCTGGGATGGCTG
 GATGAGCATATCTGAAGCAGAACGATGACCCCAATGGAATATCTGTCAGATGATCAA
 CTGCTGACCACTATCTACGACAGGCTGGAGGAGGACCAACAACCTGGTGAAG
 TGGCTCTGTGGTGGATATGTGCTGAACCTGGTGTGAACTGTGATGACACTGGGC
 GCACCGCGCGGATCAGAGTGGTGAATTTAAACCTGGGATATCTCTCTGTGTGAAGG
 CCGACCTGGAGGACAAGTACAGTACCTGTTCAGGAGGGGGGTAGTAGGACGTGA
 TTTGTGACCAAGCCCGCTGGGACTGCTGCTGCATGATGATACCAAGATCTTAGA

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[illegible]

FIG. 16

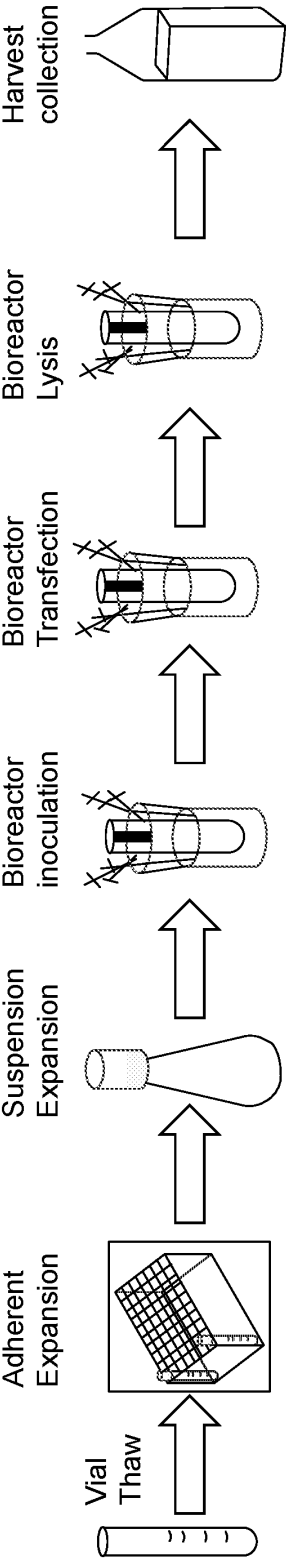


FIG. 17

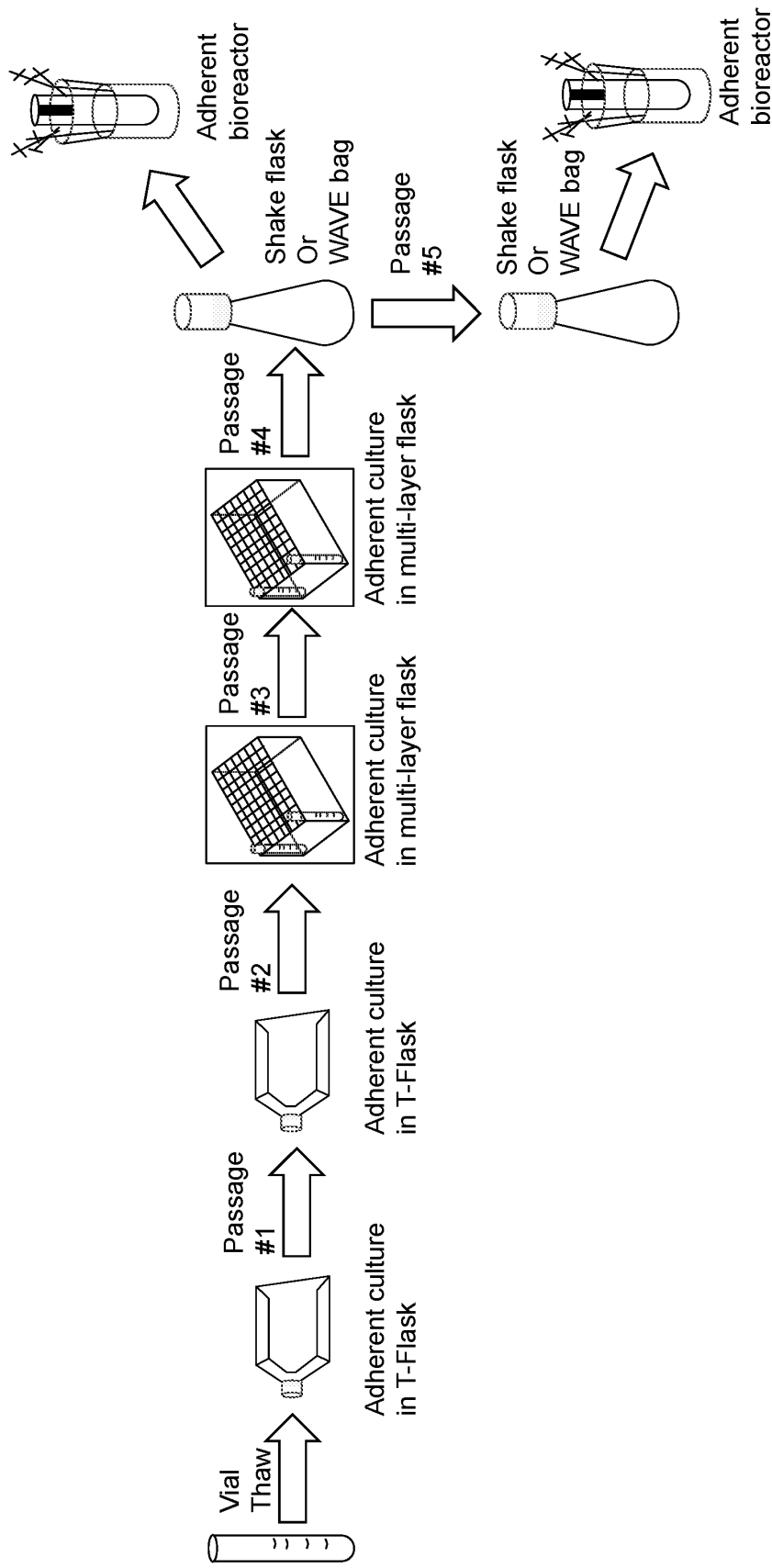


FIG. 18B

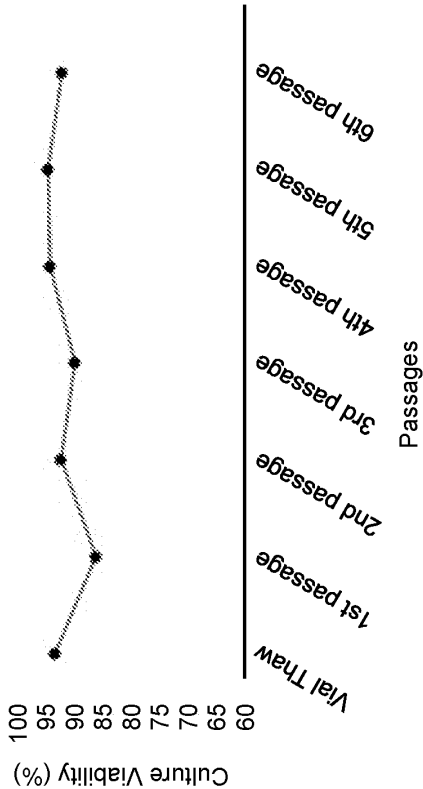


FIG. 18A

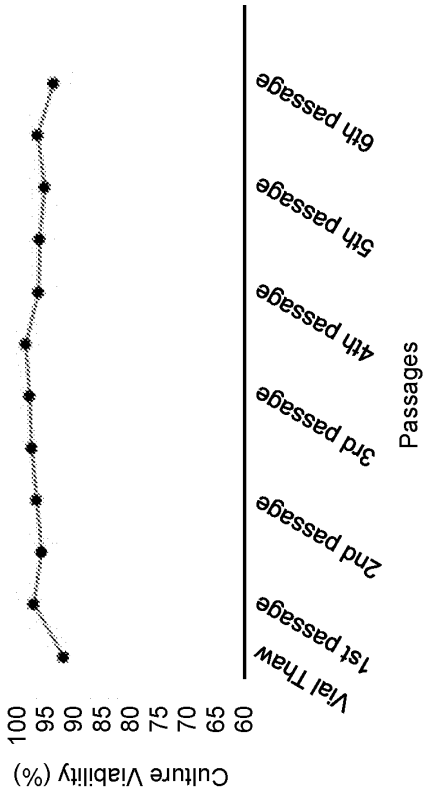


FIG. 19B

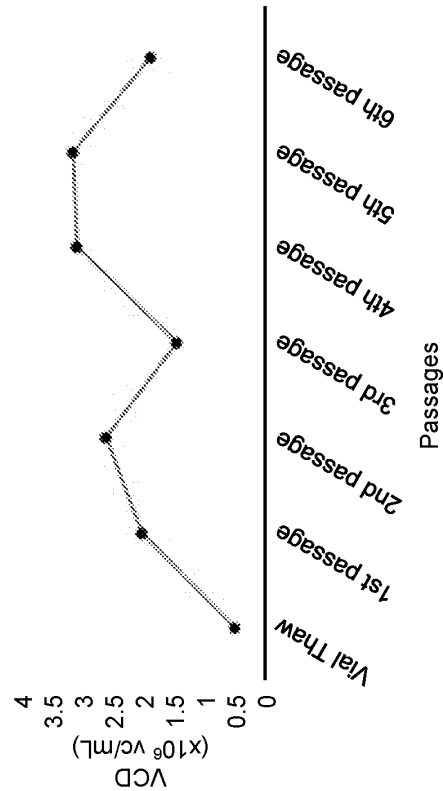


FIG. 19A

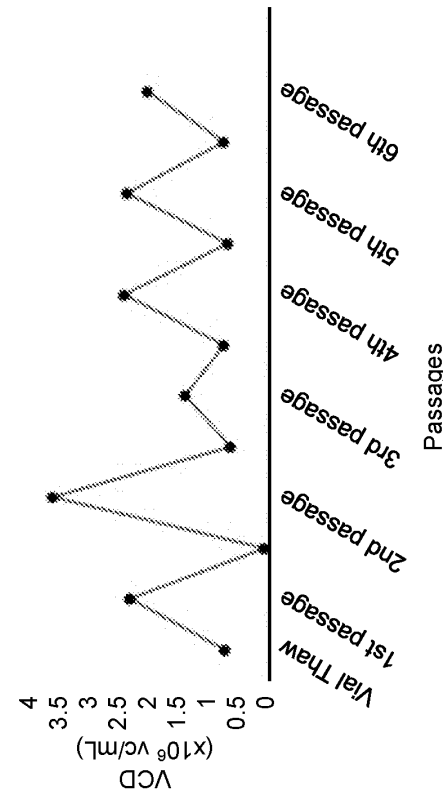
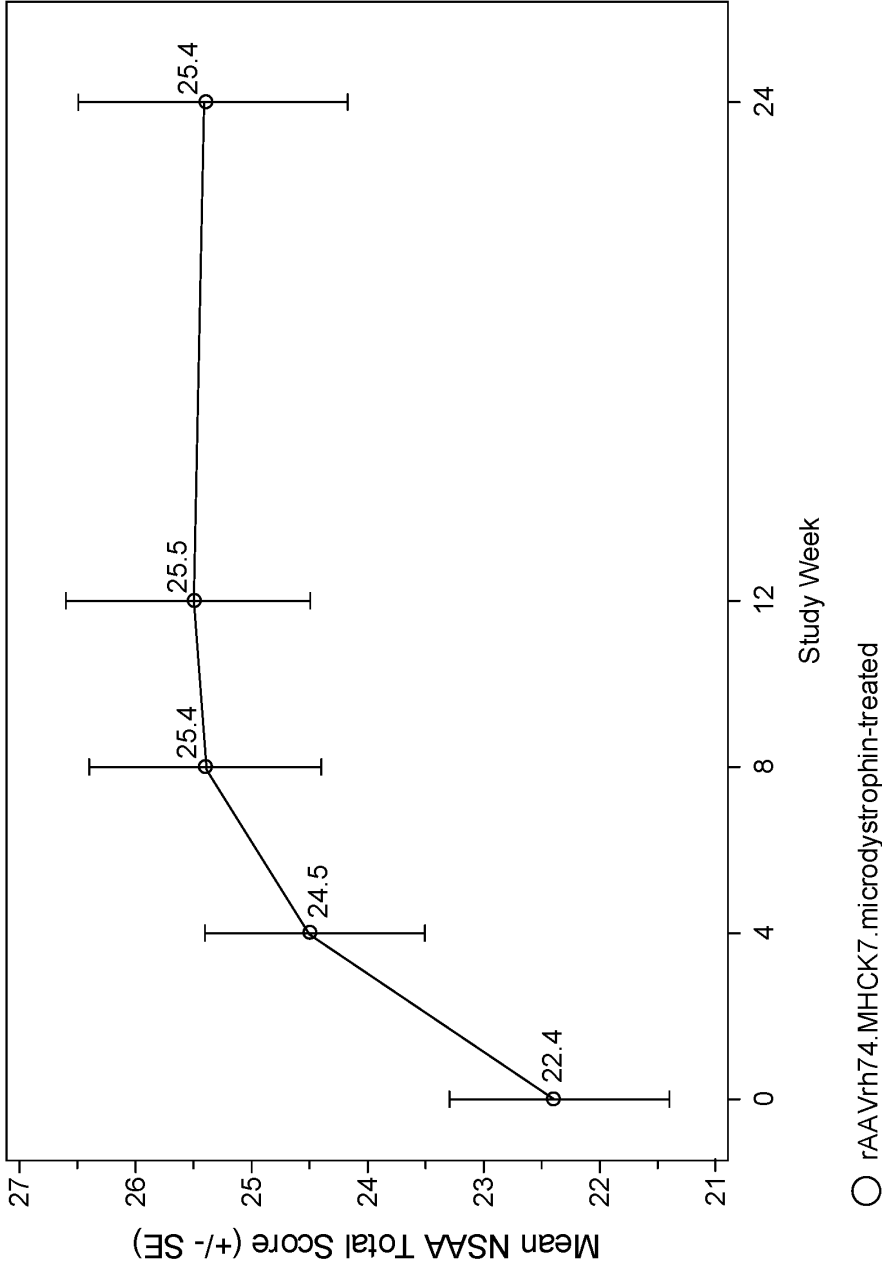
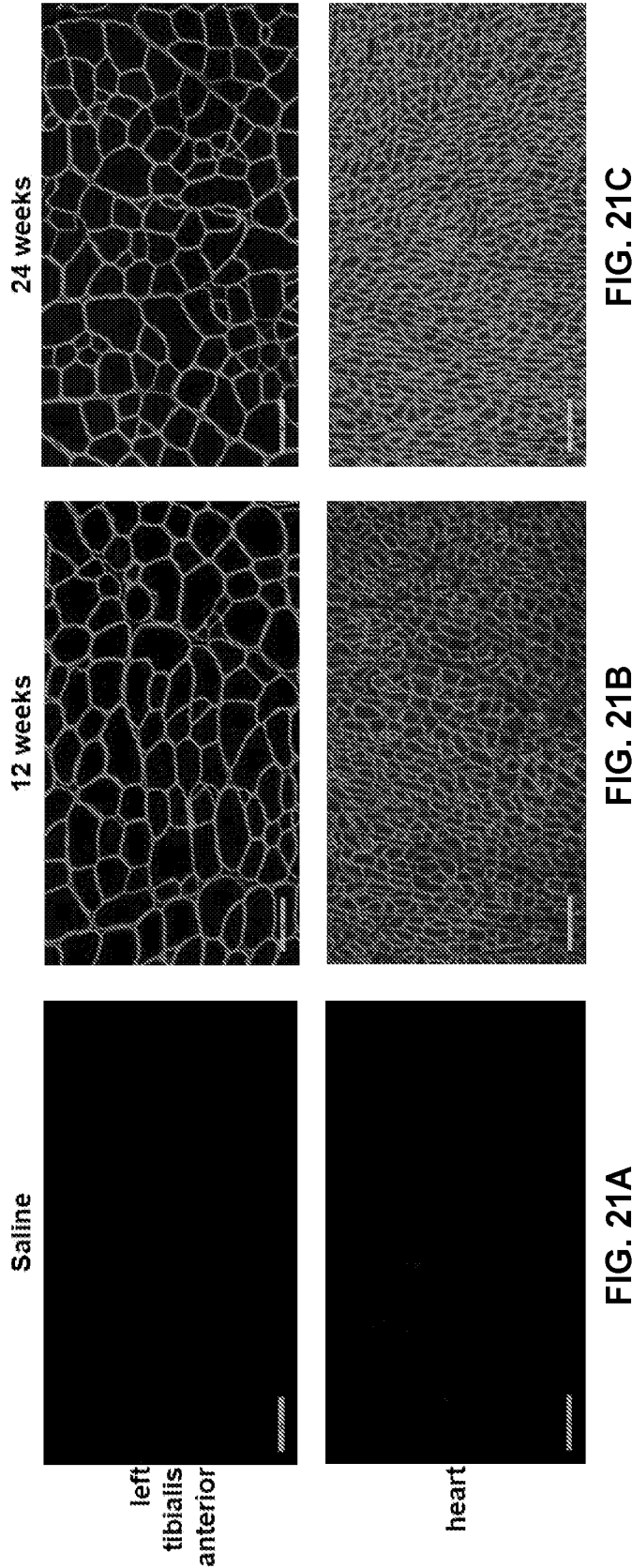
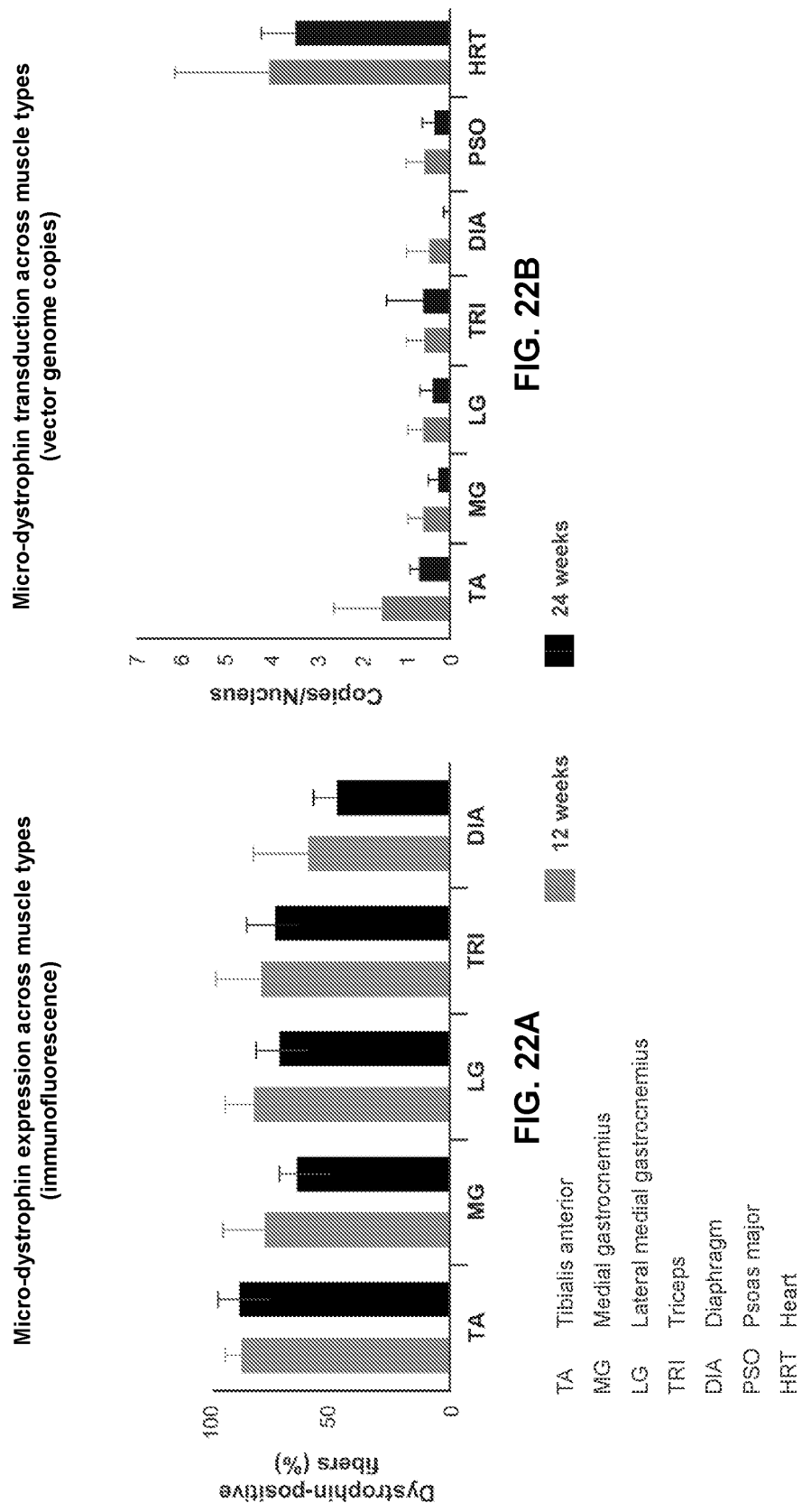
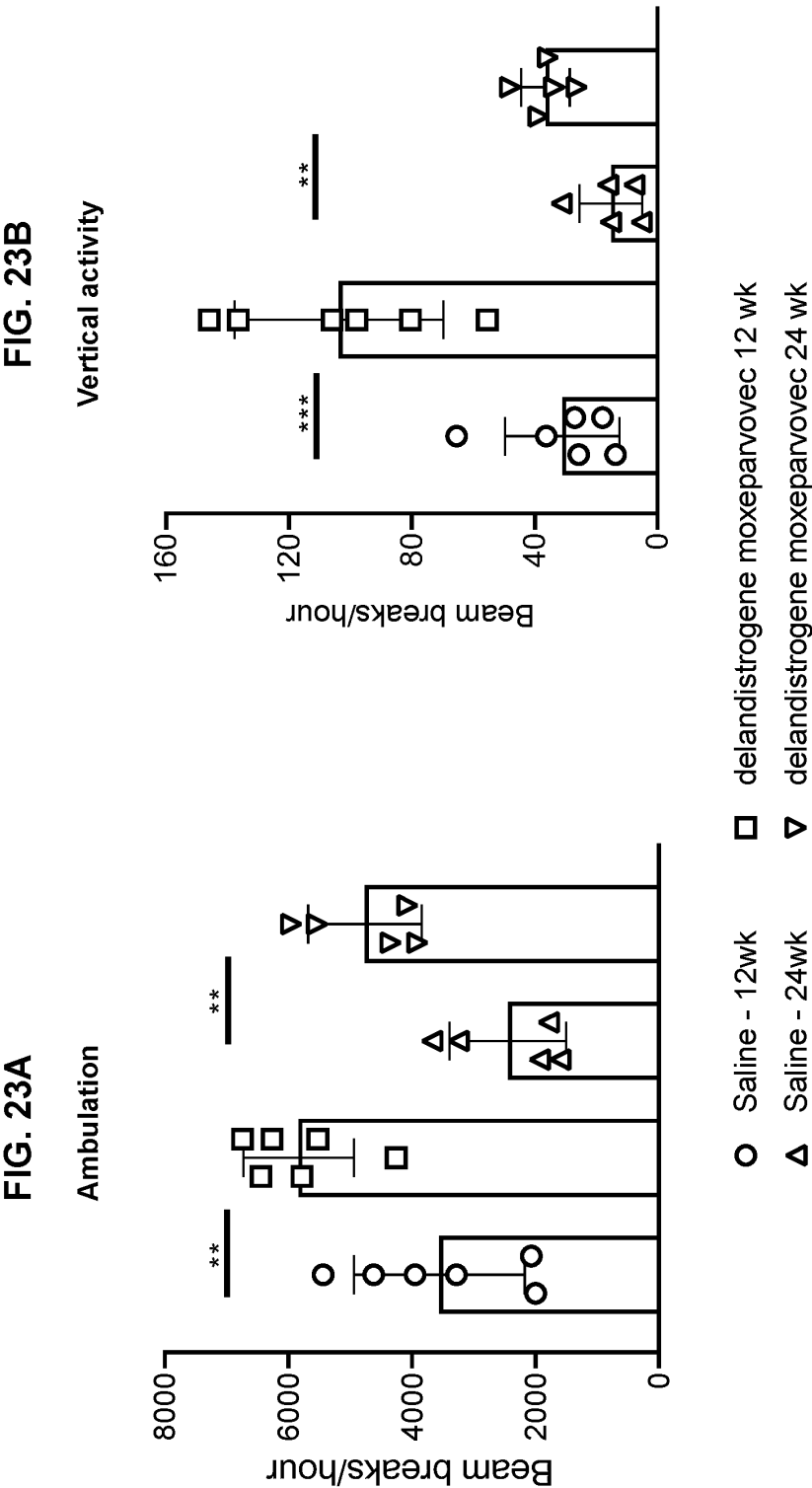


FIG. 20









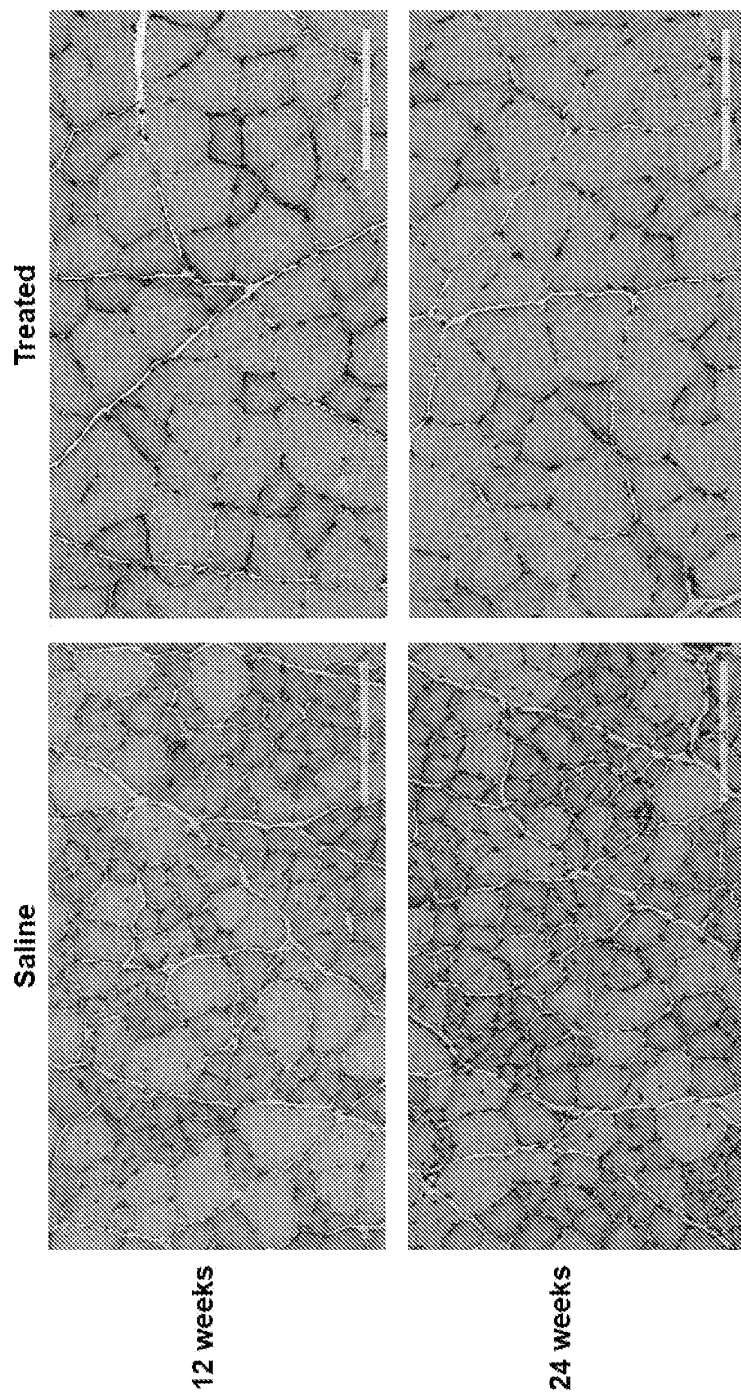


FIG. 24A

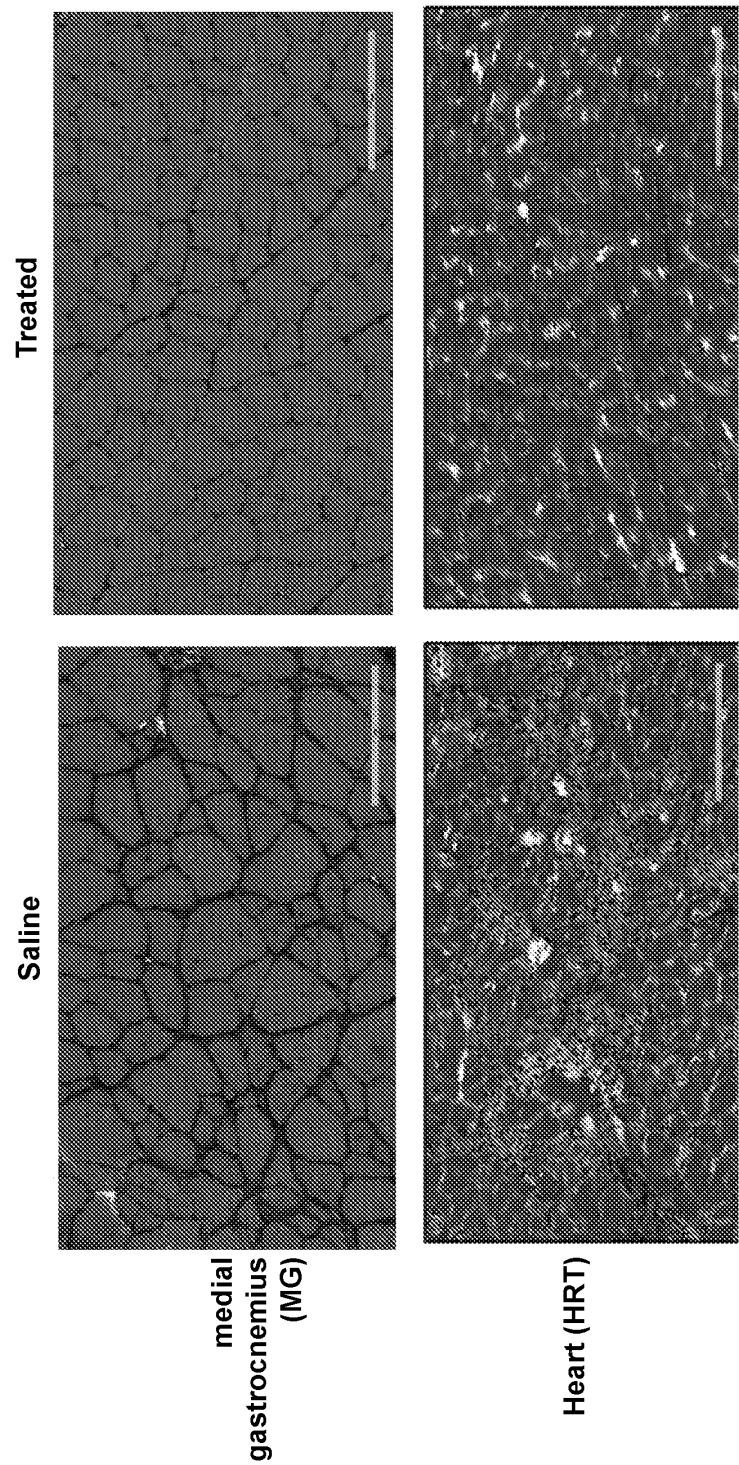
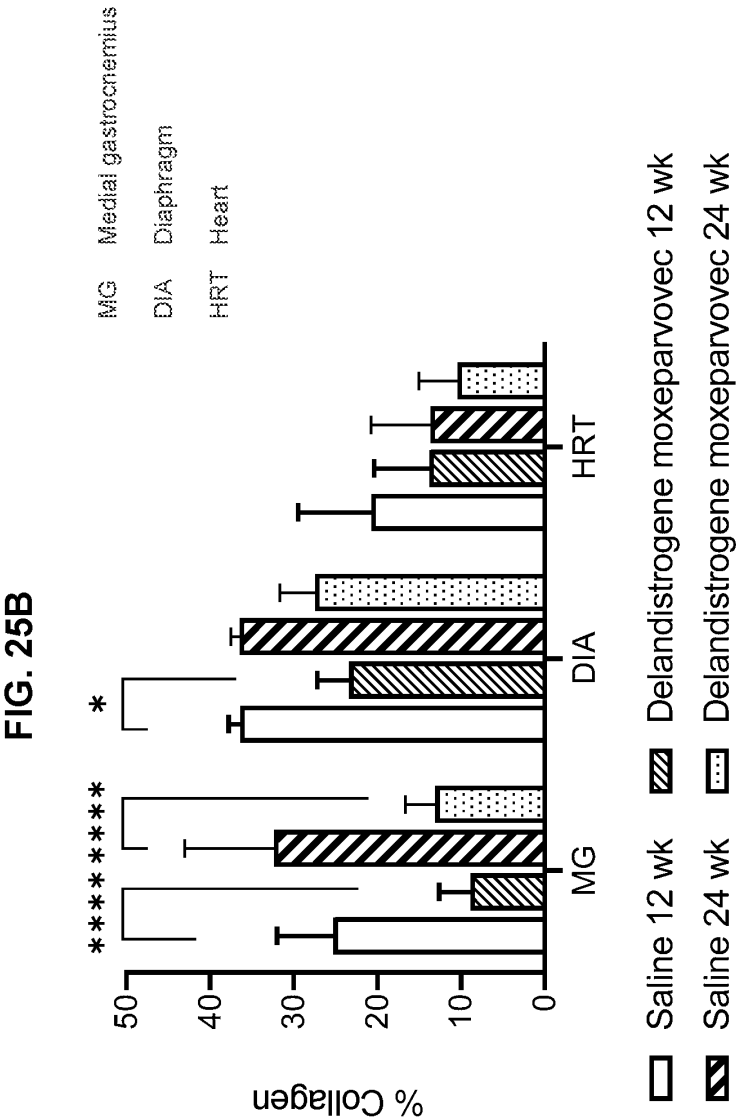


FIG. 25A



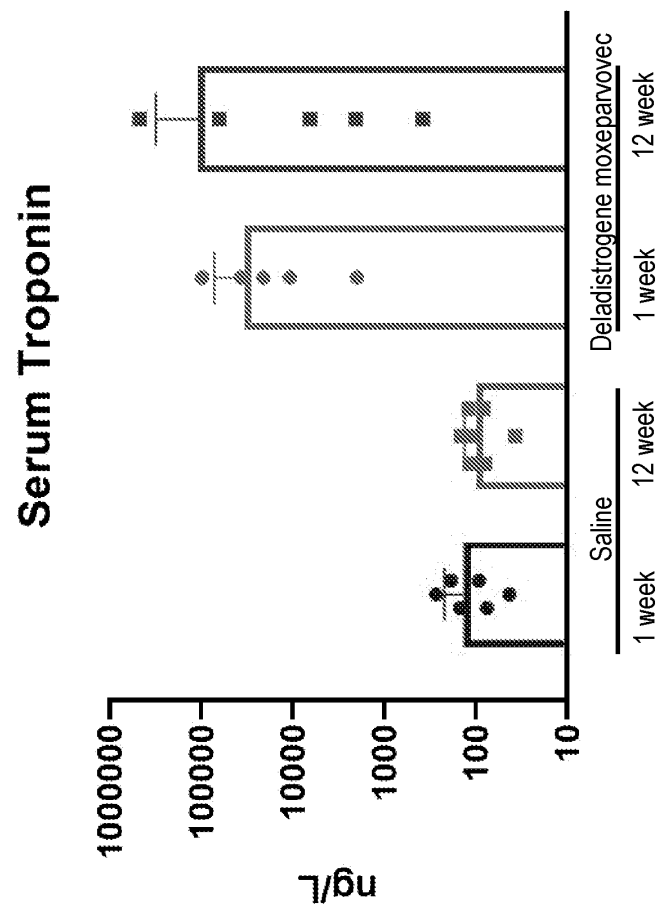


FIG. 26

