FIG. 1

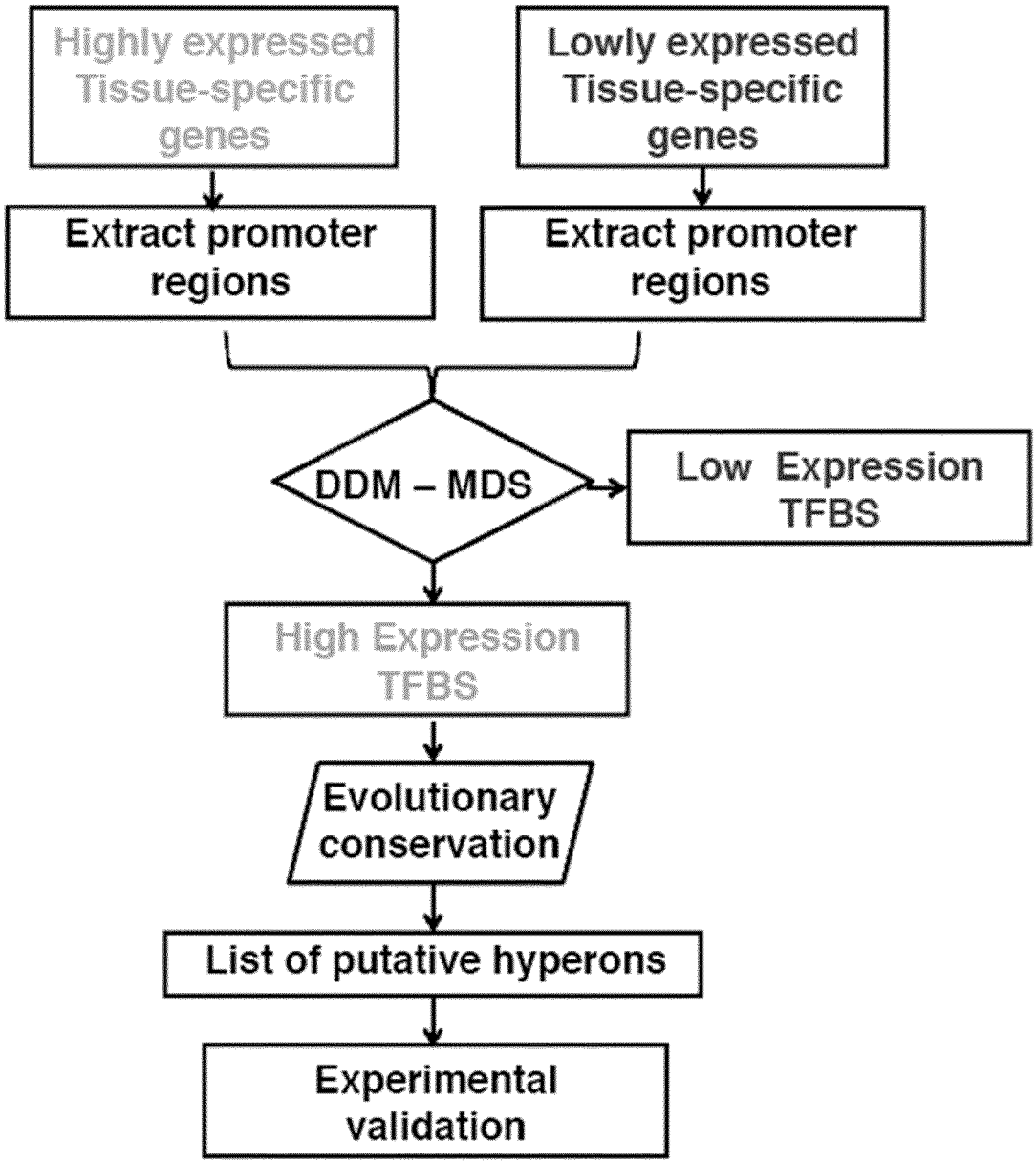

Highly expressed Tissue-specific genes
Lowly expressed Tissue-specific genes
Extract promoter regions
Extract promoter regions
DDM – MDS
Low Expression TFBS
High Expression TFBS
Evolutionary conservation
List of putative hyperons
Experimental validation

**FIG. 2**

A

SEQ ID NO: 1

GTTCTCCTCTATAAATACCCGCTCTGGTATTTGGGGTTGGCAGCTGTTGCTGCCAGGGAGA
TGGTTGGGTTGACATGCGGCTCCTGACAAAACACAAACCCCTGGTGTGTGTGGGCGTGGGT
GGTGTGAGTAGGGGGATGAATCAGGGAGGGGGCGGGGGACCCAGGGGGCAGGAGCCACACA
AAGTCTGTGCGGGGGTGGGAGCGCACATAGCAATTGGAAACTGAAAGCTTATCAGACCCTT
TCTGGAAATCAGCCCACTGTTTATAAACTTGAGGCCCCACCCTCGACAGTACCGGGGAGGA
AGAGGGCCTGCACTAGTCCAGAGGGAAACTGAGGCTCAGGGCTAGCTCGCCCATAGACATA
CATGGCAGGCAGGCT

B

SEQ ID NO: 2

CGTCTATAAATTCCAGGGAAGGTCTCTGATTGGCCCTGCTCATTCCCAGGCCCATTCCTTG
ACCCAGTCACTGAAGTCAGGGAGATGCAGTAATAAGACTGGCTGGAATCAGGGTCTTTAGG
GGTGGAGGGATGGGGAGGAGGCACAGCATGTCATCAAAATAAGGAAATTGCAAAAGAAAGC
TTGCAGGCTACTTTGAATGACAATGAGAAAGACGGTGCTGCCTGAGTGTGTTAAGGATCCA
CATGGTCTCCAAAATCCTCCAGGAGCATACAGTCTAGTCTGGGAGATGAGACACAAAAATA
ACCAGAACACAACAGCTTGCACTGACTCGAGGGCTGGATAAGAATATCTGGAACTCCCCCA
TCTATTTCAGAAGCTTGTCTCTTGGATGAAAATTAGACACTTAATGGGAAAGGGCTTTGAA
AAGAGTGC

C

SEQ ID NO: 3

AAAAAATAAAAATAAAAAATAAAAATAAATAAAGTGGATGGCTTAGAGTATCTTTTCTGTT
TAGACCTGACTAAAGCTTAGACATAATTGTTAGTTTAGGCTCTCAGGGTAAAATTTATTAC
TGTAAATCCAAAAAATCCCTTCTTCTTCTTTTTTTTTTTTTTTTGGTCCTTGAATTAAAT
GCTGTCACCTCCTTCTTGAAAGGAGAAACTATTAGTCAGATTTGAAAATCCTCTTTATCAC
CCAGGAAAATCATTTTTATGGACACTTTGTCTTTCTGTAGTCTGACTTAGAAGCAGCCTGT
TTTTGATAGGTTGAAGTTTTCATCTTGAACACAAACCCTGTTTGTGTGTCCCCTACTCCCC
AGTTTGATGTGCCAGGCACTTTGTTCTCAAGCCCAGCAGCTGTTGTGGGATGAGGGGACAT
TTTGCATGCTTAGCCAGCAGCTGCCAGAAACATTTCTAATCTGGTTTTGGCAGGAAATAGG
GCACAAGTGGAAGCCAAGTTAAAAGAAGCTGGAAAAATAAACAGAATAACTTTAGATGTCA
CT

**FIG. 2**

D

SEQ ID NO: 4

TGCACTGTAGACTATTTATGTTTGCATATTTTTCAGGATTGTGTACAGTAAACCTTAAGTG
AATACAAAGGATCCAATTGTCCTGTAAGACTCACTTCAATTACAGATTGTGCTCAGTATTA
AACTTTGCTAGTACTTTCAGGATGCAGTCAATCAAGAGGCAGGGAAAGGTCTGTCAGCATC
CACAGCCTCCTTTTCAAATTGGACACTTAGTCTCTGGTCCGAATAATAGGTGCCCCCAGTG
TCACCTCACACAATGGGGTATACACAGTTTTTAAAAATTTATTTTCAGCTGGGCACAGTAG
CTCACACCTAAAATCCCAGCACTTTGGGAGGCTGAGGCAGGTGGATCACCTGAGGTCAGGA
GTTCAAGACCAGCCTGGCCAACGTGGCAAAATCCTGTCTCTACTAAAAATACAAAAATTAA
CCG

E

SEQ ID NO: 5

CAGTTTACTCACCAGGGATTCAGAGGCAGCACTGCTGAACCCTGAGCCCTTGGCACATCAG
GTTGGCTGTCAGAAGTCGGCCTTTGTACATACACAGTTCCCTTGTGAGGCCCAGCTGCGTG
TCCTAGGAGCGGGGCCTCTCTCCACAGCAGAGCTCAGCCTCTCAAGTGTATGGACAGCACG
GGTGCCTGATGGGTGGATTTAGCCATGAGTTGAAGGTGGCTTGGGGAGAATGAGAGTTCTA
GAGATAGGGAGAAGGGGTTGCCAATAGGAGAGTGGAATTCCTGAGCACCTCGTCACAGGCA
GCCGACAGAACATGAGCCGCAGGGCCCAGGCTATTTATACCTCGCCTGTCACTATCAGGGT
CCCCACAGCTCCCCCCACCTCCAGCCACACACAGCAGGTCCTTTTGCTCTTTCTGGTCCCT
TCTCTACTCCTCCCCCTCCCTACCTAA

F

SEQ ID NO: 6

CCCTTTCCCTGGCAGGATCTGCCCTGTGGCCCAAATGGGCATGTTGCCCAGGGGGCTCCCT
GGCACTATGGGGGAAGAGTCTCTCCTTCCCCTCTTATCATCTCAGTTGAGTCAGACTTGGG
GGAGGGGGATACACAGTGTGAGTCACTGGGTACCCTTTTCCTGAGCTCAGCTTCATACCGA
GGCGATGAGGCCAAACGGGCTGGTGACAGGGACACTGAGTCAGGGGCAGGGGCCCCGGTCT
TACTCCTGGGCCTCTGGATTTGGGCCCTACATGAGGCTTTTCTATCTGTAAAGTCAAGCAA
TGGCTGGGAGGCACACACAACCCCCCGCCCCCCCGCAGGCTTCTCCTTCATTGGCCCGGGC
AAGGTCCCTGCTTCCTCTCAGGCCGTCTCTGCACAAGCACACACACTTCCCTTCCCTGTCC
ACAGGTGGACAATGCCCTGGGCTAGG

**FIG. 3**

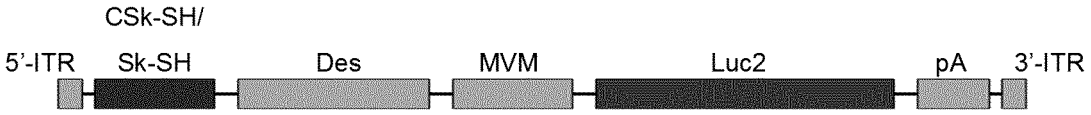


FIG 4

A

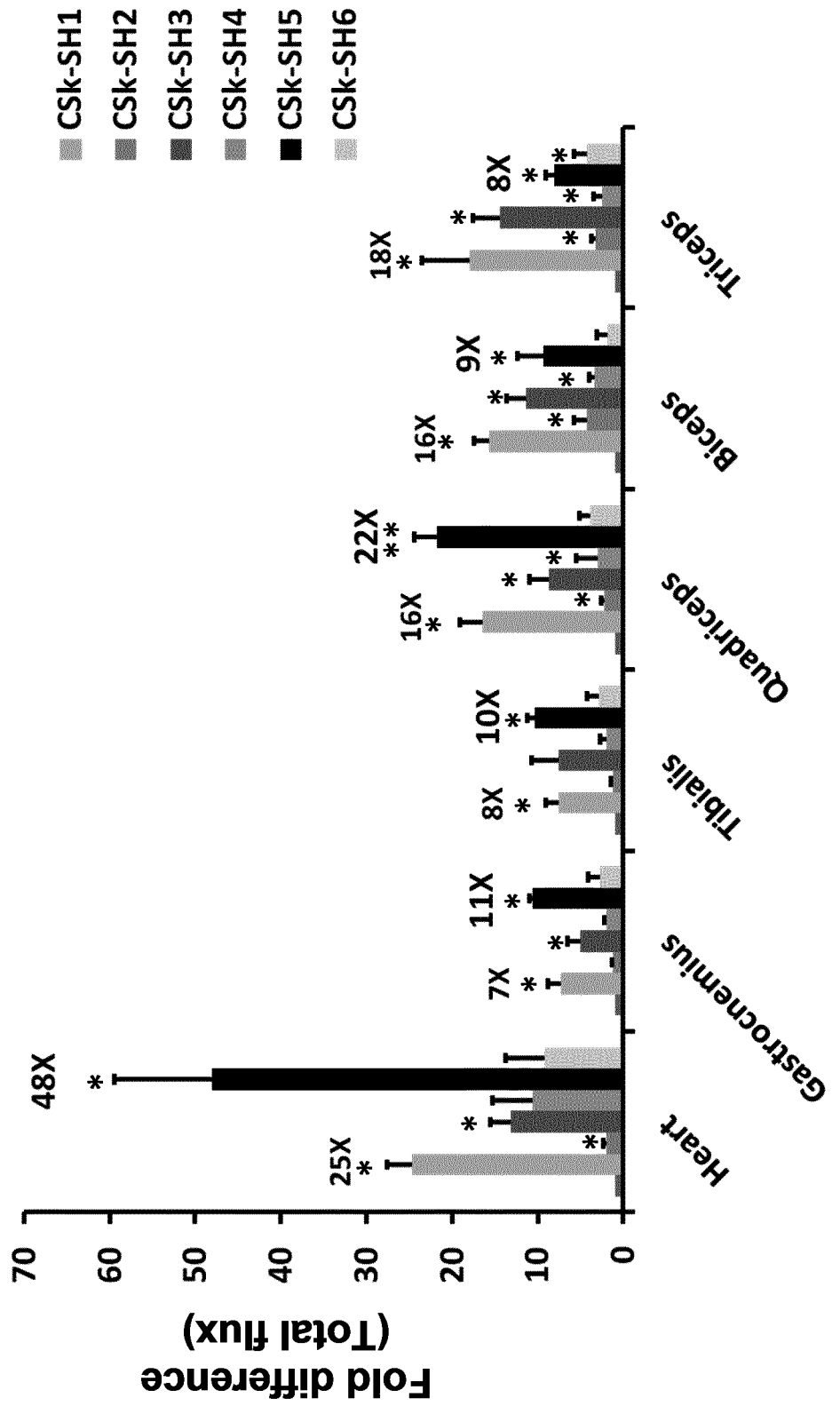

CSk-SH1
CSk-SH2
CSk-SH3
CSk-SH4
CSk-SH5
CSk-SH6
Fold difference
(Total flux)
0
10
20
30
40
50
60
70
Heart
Gastrocnemius
Tibialis
Quadriceps
Biceps
Triceps
48X
25X
11X
7X
10X
8X
22X
16X
16X
9X
18X
8X

FIG 4

B

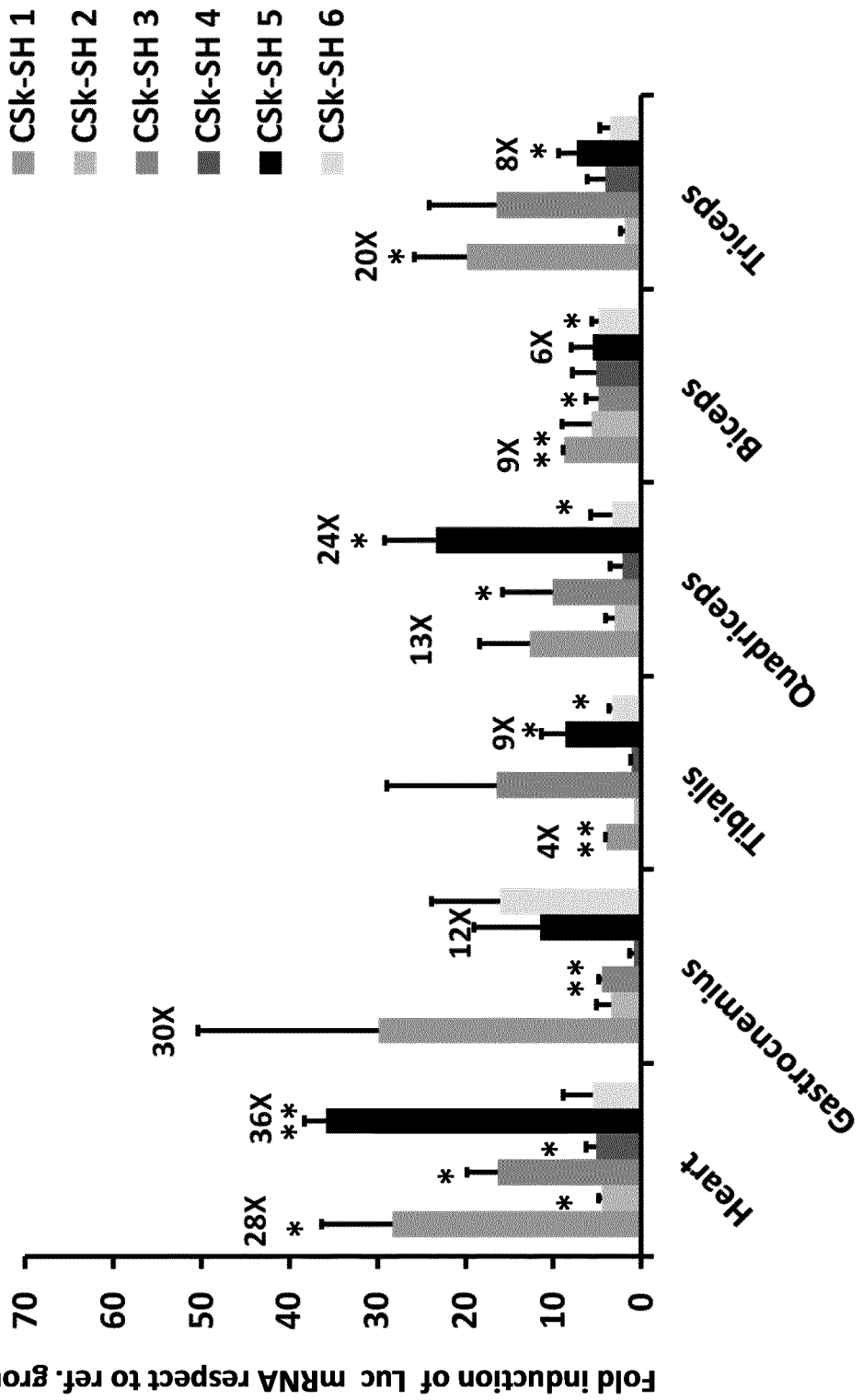

CSk-SH 1
CSk-SH 2
CSk-SH 3
CSk-SH 4
CSk-SH 5
CSk-SH 6
Fold induction of Luc mRNA respect to ref. group
0
10
20
30
40
50
60
70
Heart
Gastrocnemius
Tibialis
Quadriceps
Biceps
Triceps
28X
36X
30X
12X
4X
9X
13X
24X
9X
6X
20X
8X

FIG 5

A

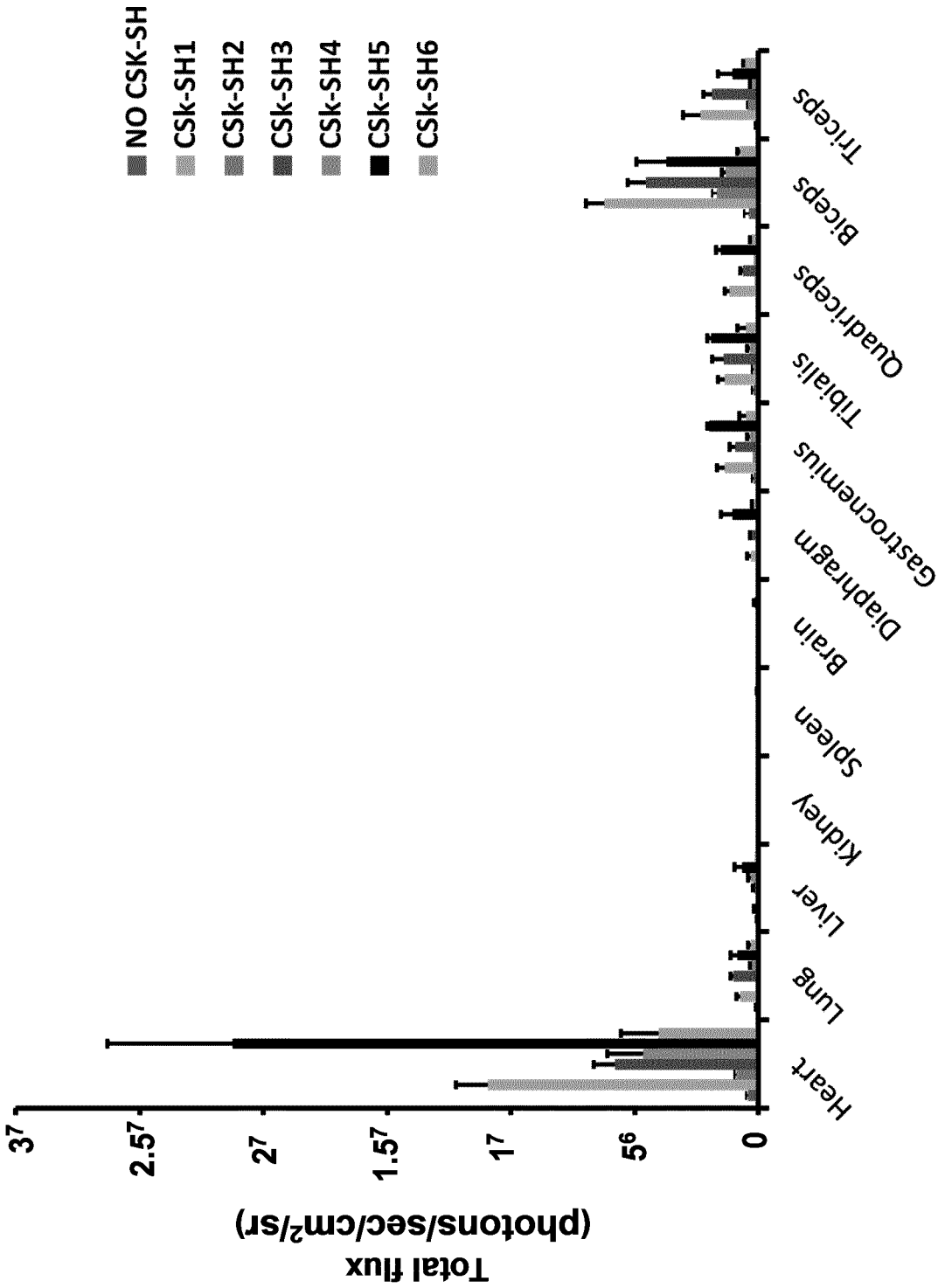

NO CSK-SH
CSk-SH1
CSk-SH2
CSk-SH3
CSk-SH4
CSk-SH5
CSk-SH6
Total flux
(photons/sec/cm²/sr)
3⁷
2.5⁷
2⁷
1.5⁷
1⁷
5⁶
0
Heart
Lung
Liver
Kidney
Spleen
Brain
Diaphragm
Gastrocnemius
Tibialis
Quadriceps
Biceps
Triceps

FIG. 5

B

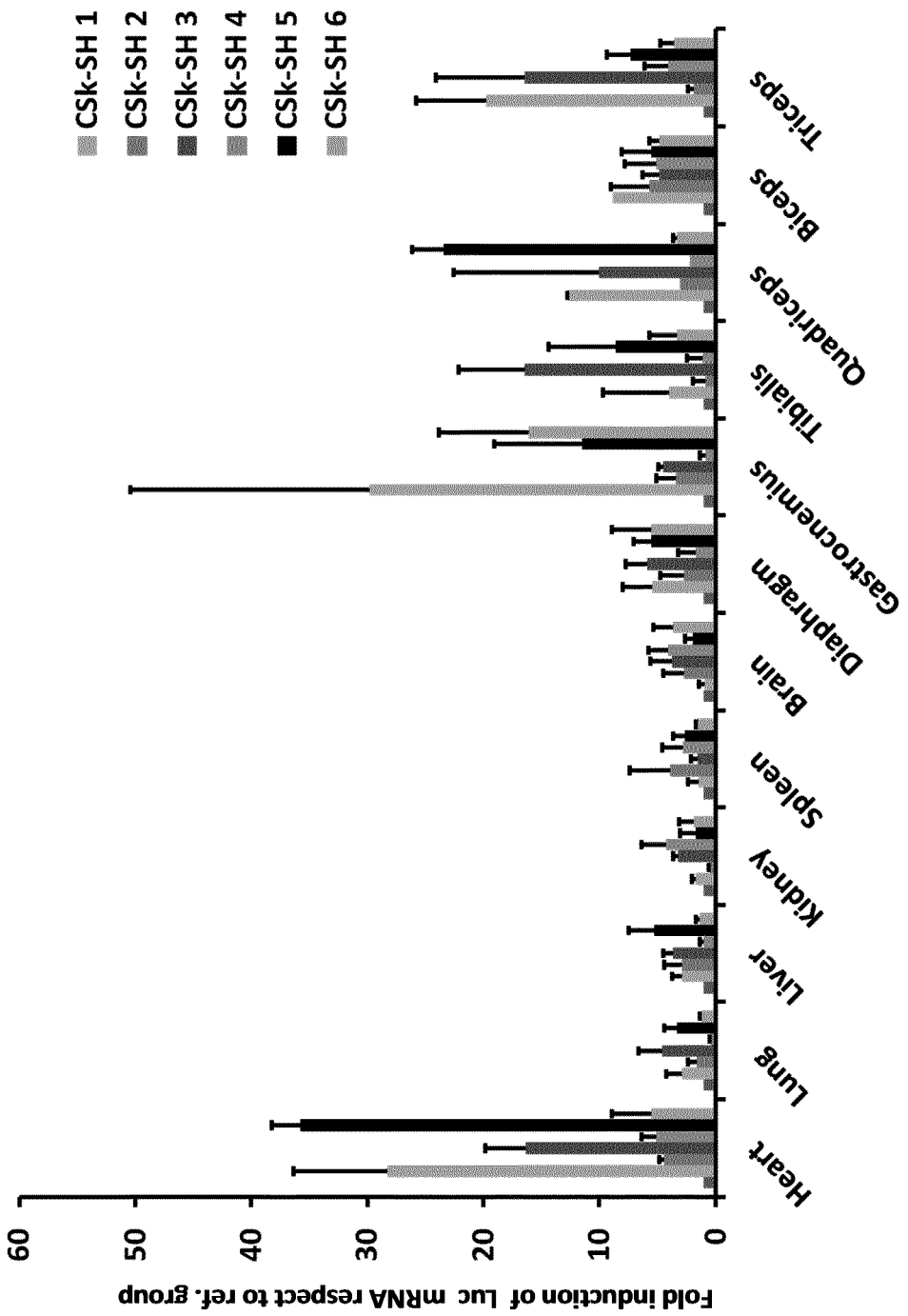
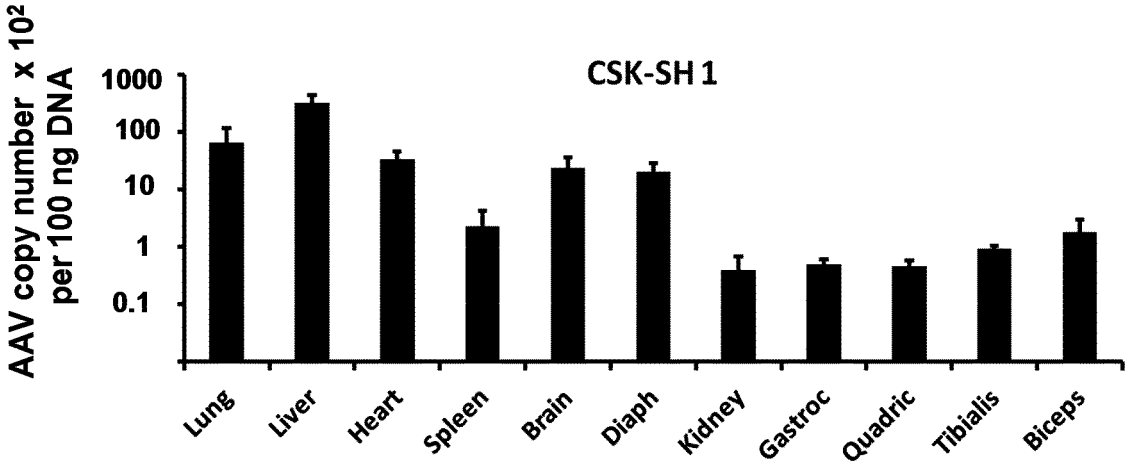

CSk-SH 1
CSk-SH 2
CSk-SH 3
CSk-SH 4
CSk-SH 5
CSk-SH 6
Heart
Lung
Liver
Kidney
Spleen
Brain
Diaphragm
Gastrocnemius
Tibialis
Quadriceps
Biceps
Triceps
0
10
20
30
40
50
60
Fold induction of Luc mRNA respect to ref. group

FIG. 6

A


AAV copy number x 10²
per 100 ng DNA
1000
100
10
1
0.1
CSK-SH 1
Lung
Liver
Heart
Spleen
Brain
Diaph
Kidney
Gastroc
Quadric
Tibialis
Biceps

B

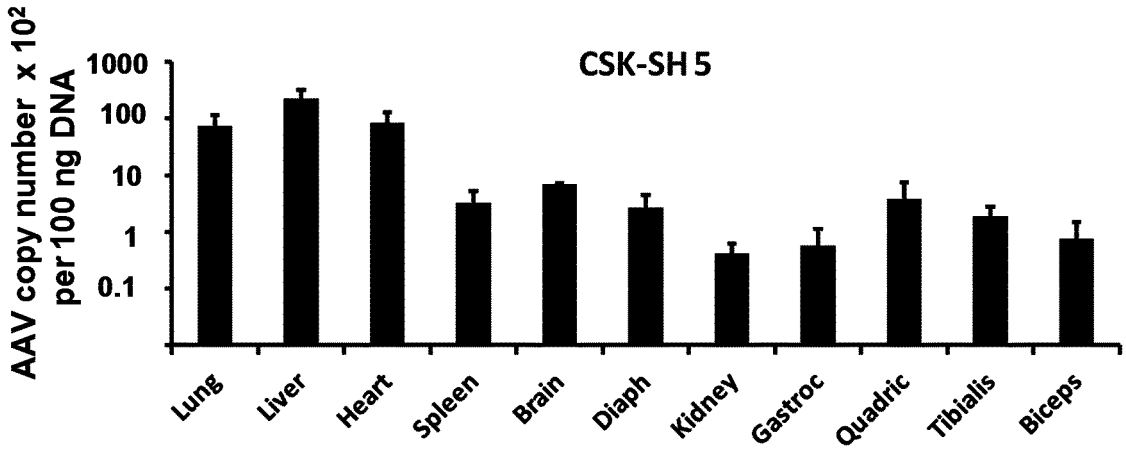

AAV copy number x 10²
per 100 ng DNA
1000
100
10
1
0.1
CSK-SH 5
Lung
Liver
Heart
Spleen
Brain
Diaph
Kidney
Gastroc
Quadric
Tibialis
Biceps

**FIG. 7**

A

SEQ ID NO: 7

CTCACTCCCCGCCCAGGCAGCAAGGAGCCCACACCCTCATGCCCCTCAGCTTCAGCCCCCA
CCTCCAGGAGGCCCTACCCACGCTCATGACCTTGCTATTCTGGGCCTTGTGTCCTGTAGGG
AGATGGACAGGAGACAGCTGGGCTTCCAGGCCACCCAGGCGGGGGGCTAGCCGAGGGAAGC
CTGCTGGCTCTCCTGCTTGCTCTAATTTCTGGGGCTCCCCAAACCTTGGCCTCAGGAGACT
GGGGATAGGACCGGCCTTGAAAGTGGGGGAAGCTTTGGAGAGCCGGGTGCTGGGTTCTTAG
TGAGATGGCCAGTGAAGGCTGTGGTGCCCCGAGGTAAGCAGGGCCTGATCCCCTCCTAATC
TTCCAGCAGCAACTGGTGCTCTGAGGCTCCCCCTCCCCCAGCCCTGCCAGCCTTCAGGGAC
CTGCCTTCCAAAGATGGGCAGGGGAGGGGGACGAGGACACCCACCCACTCCTCAGACCAGC
ATGTCTT

B

SEQ ID NO: 8

CCCTCCAGATGGGTTTCCTGGAATCTAGATTTCCCAGGTTCCAAAGGACACCCGAGTCTCA
TGCCTGGAACTCAGTGAGACTAATTCACCTCTCCTCTGCCCTAATCTTCATCTCCAGCCAG
AAGCCAACAGATCCCAGGGGACTGGAGCCACAGGGGCTGCACCTGTTTACCGGGTATTTTT
AGGATGGTTGATGAACACATAATACCCACCCTATAGTCAGAGAAAGACAATGCCTGCTATG
TTAATCCTGTGGCTATTATAGTCTGTCATCTCATGGGTTGGGGCAGGACACTGACCCTCTC
AGAGGCCAGAGAGAGGCCTCGCAAGCAGGAGGTTAGGGA

C

SEQ ID NO: 9

ATGGAGACAATCCATGAATTCCTGAGATGCTTGGCTGGTATTAGATTTTATGGGCAGCTGC
TTATTCTTAGGGCTCTGCTTCTCCAAAGACACTGAGGAAGTCCAAAGGAAACACCAGCTGG
CGAAGAGCCACCTCCAGGCCCATCTGTCCATCATCAGCCTCCAGGAATGCCAGTGTCCAGA
GGGCACCAGGTCTGCGTCTGTCTCCCTGGGATGTGCCTTGTCCTTGGTGGGCATTTGGCAG
TGATCATGCCTCCCTGTCTCCCTCAGAGATCCAACTGTCCCCATTGTGGGGCCCTACCTTC
CAAGGCCGGTTTACACCTCCTGCCAAGCTCCGGGGCCTGCCCCCAGCCTGCCTCACTGACA
AATGCCAGACCAAGGGGTCCCACGTCAGGCAAGAGGCCTCAGCCTGTGCTCTGACACCCCT
CAG

D

SEQ ID NO: 10

TTCTGAGTCCTCTAAGGTCCCTCACTCCCAACTCAGCCCCATGTCCTGTCAATTCCCACTC
AGTGTCTGATCTCCTTCTCCTCACCTTTCCCATCTCCCGTTTGACCCAAGCTTCCTGAGCT
CTCCTCCCATTCCCCTTTTTGGAGTCCTCCTCCTCTCCCAGAACCCAGTAATAAGTGGGCT
CCTCCCTGGCCTGGACCCCCGTGGTAACCCTATAAGGCGAGGCAGCTGCTGTCTGAGGCAG
GGAGGGGCTGGTGTGGGAGGCTAAGGGCAGCTGCTAAGTTTAGGGTGGCTCCTTCTCTCTT
CTTAGAGACAACAGGTGGCTGGGGCCTCAGTGCCCAGAAAAGAAAATGTCTTAGAGGTATC
GGCATGGGCCTGGAGGAGGGGGGACAGGGCAGGGGGAGGCATCTTCCTCAGGACATCGGGT
CCTAGAGG

**FIG. 7**

E

SEQ ID NO: 11

GACTAGGAATAAATCACATATCCTCAATCCCTGGACAACTTGTTTACTTCTAGTGTTAGTT
TTTTCTTAAAAAAAAAATTGAAATCATTCTGAGGCTGGAATACTTTGGACATGCCCAGCAG
TTCCTGGCAGTTCCCACAGAAGCATTACCTCATGACTGGAGTGGGTAAAGCATACTGTGGG
CTATGGATAAGACTGACATTAACCACAAGCATGTTTGGCAGCAGACTGGTGCTTTACAAGC
TCCATGTTCAGCAGGAGCTGCAAAGTGTTCCTCCAAACCAATATTTGTCATTCTTGGATTC
TATTTAGGAGGTCCTGTTACTCACATGTTTCAATATCAGCAGAAGCCAGTTTCCCTGTGGT
ACCGAAGTGGATCCTGATGAATTTACCCTTGTAAGTAAAAAAAATGATGTTATACCCAAAG
CTTGAAGTACGTAGTGGGGATGCCACTGAAATAATTCAGACATGCTT

F

SEQ ID NO: 12

GTGCTCATAGCTCCACCTTTTGTTCCTAATATGGTCTTTCCAGCTCCCTCCACCCCATCAT
TGTTCTCCTGGGGGAACACAGGGTGAGACGCTTTGATGAACTGACATCACCAGCAAAAAAA
ATATCTAGCAACAGCTGAGGCTGATTTTAGACAATGGAAAGTGGGGGAGGGAAGAGGTTCT
CCCTGACCCTGAAACTTTCCACTCATTCTGGGCAGCTCTATGGATGTTTTAAAAGAAGAGG
AAGAGGGGAGGGAAGAACATTGAAATAGAGAAGTGTACTTTGGCAATTCTAGGTTGGCAGT
TTGCATCCAGGGGGTCCTGGTTGCCTTTCAGCTTCCCGTTTCACTCTCCCCCAGACTGTGT
TGAATGCTGGTCAAACTCCGTTAGTTGAGTTTTAGCTTTTGATTCCTGGTATTCAAGGAGC
TTGGGCACAGGGAAGAGGGGAGGTCACTCATGATCCTTAACAATTCTCCCAGATCCCCAGA
TCAAATTGCTGTGCTATTCTGGGAGTCTCCG

G

SEQ ID NO: 13

ATCGTGTGTCAGAGGTTTGTGTCAGCTTCCCAGCAAGGGAACCAGAAAGGAAAAGGAACCG
GTTCCTCATGCTTCCTAGGGGAATGCATGCATATCTGAAGAGAAGGGAATCTTATATAAGG
CTGTTTAGCTAAGGGCAGCCACCAGCCAGGTGAGCCTTACAGAAGCACAGGGCTGGGTGTC
TGCAGTTCCCTAGCAGATTAACCTGGGTCACAGTGACTCAGAGCTCCAGCATGCGAGTTCC
AGGTGTGGAACTGAGCAAGTACAGATCTGCTTTTGCTCCACTTGGGAGTATTTTTCCTTCT
TAGTGAGCATGGGCAGCCTCCTGGCCAGGGAAGTCTGGCACTGTCTGGGCCTGACAGGGAA
ACCCTG

FIG. 8

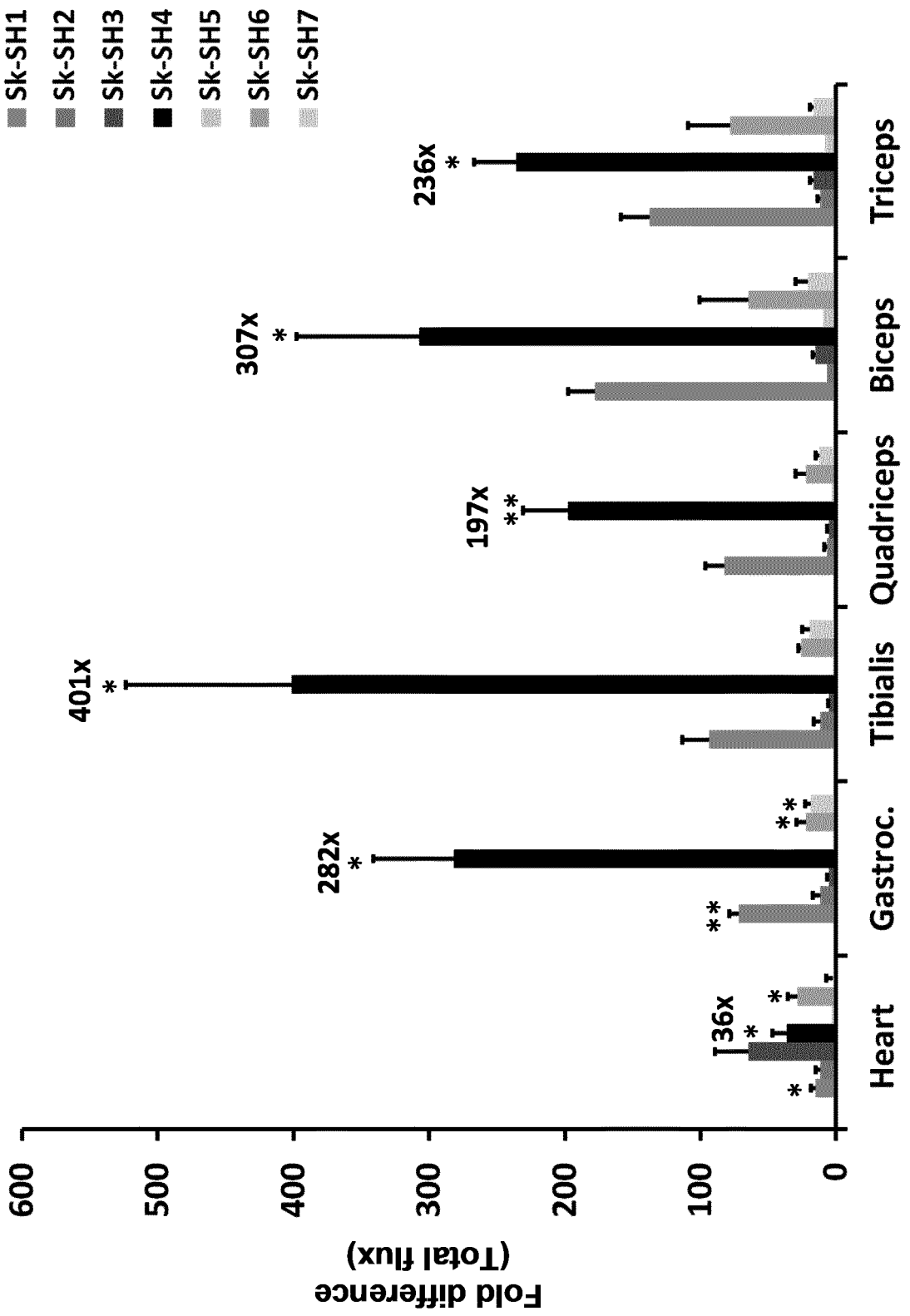

Sk-SH1
Sk-SH2
Sk-SH3
Sk-SH4
Sk-SH5
Sk-SH6
Sk-SH7
Fold difference
(Total flux)
600
500
400
300
200
100
0
Heart
Gastroc.
Tibialis
Quadriceps
Biceps
Triceps
36x
282x
401x
197x
307x
236x

FIG. 9

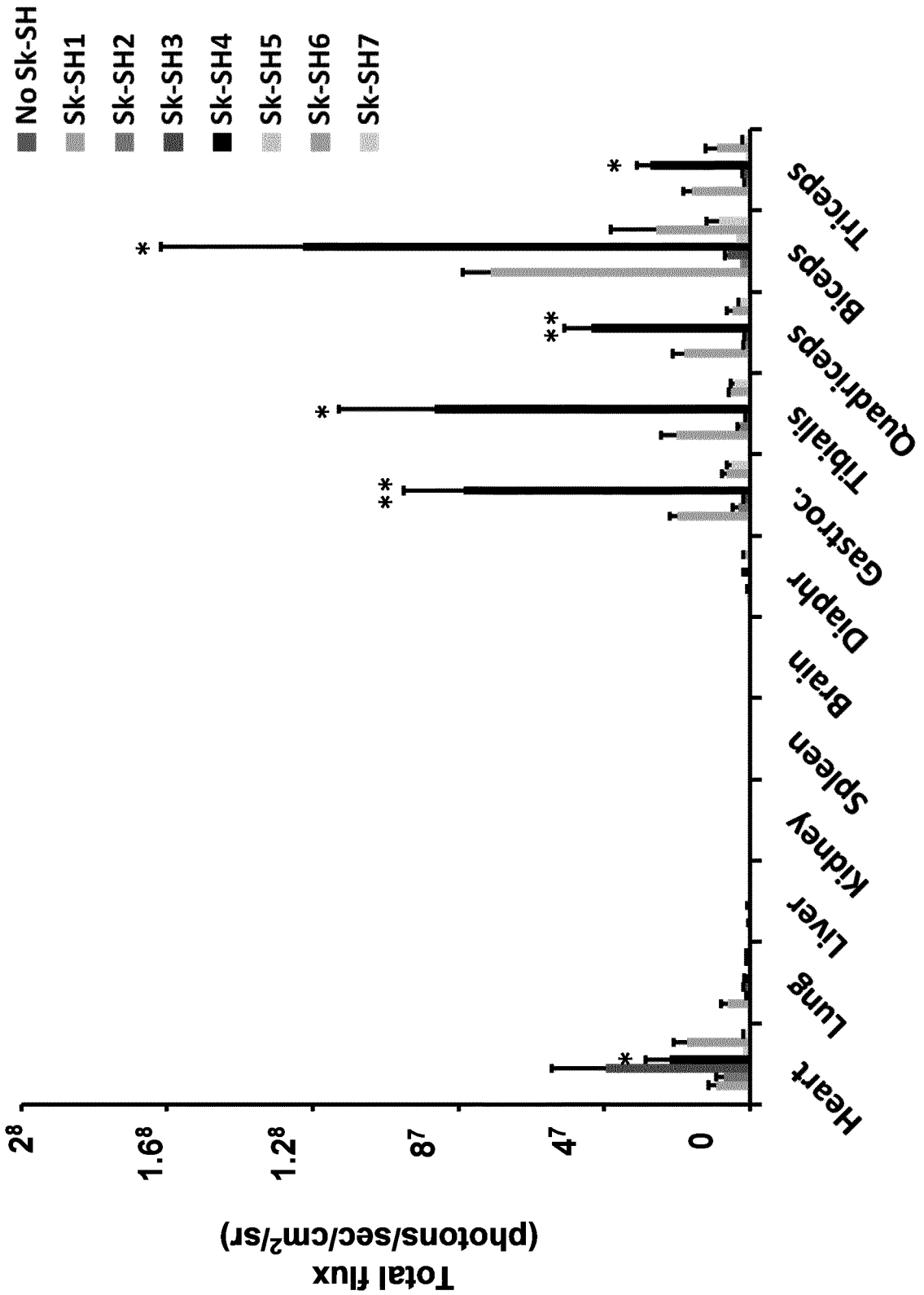

No Sk-SH
Sk-SH1
Sk-SH2
Sk-SH3
Sk-SH4
Sk-SH5
Sk-SH6
Sk-SH7
Total flux
(photons/sec/cm²/sr)
2⁸
1.6⁸
1.2⁸
8⁷
4⁷
0
Heart
Lung
Liver
Kidney
Spleen
Brain
Diaphr
Gastroc.
Tibialis
Quadriceps
Biceps
Triceps

**FIG. 10**

A.

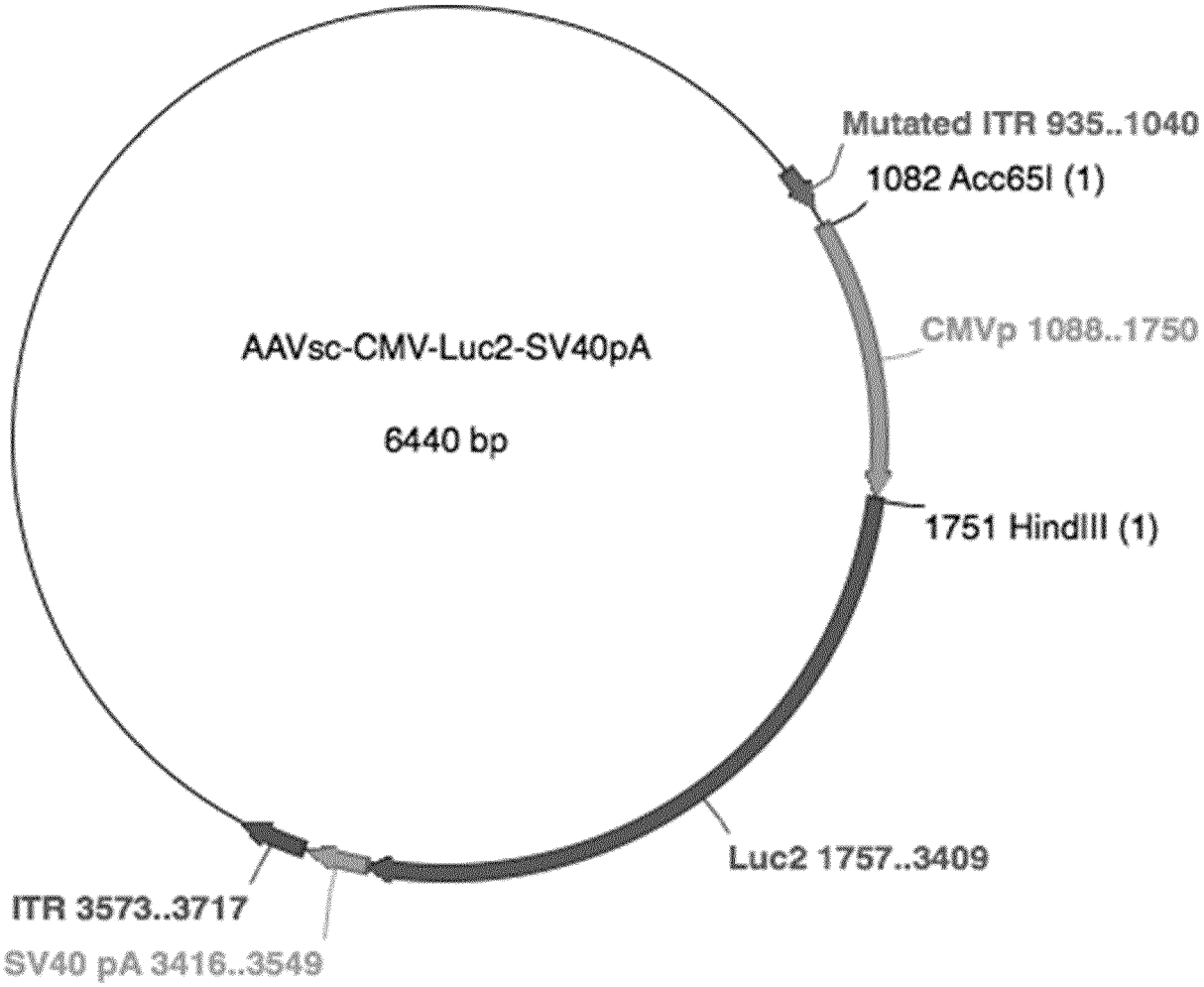

Mutated ITR 935..1040
1082 Acc65I (1)
CMVp 1088..1750
AAVsc-CMV-Luc2-SV40pA
6440 bp
1751 HindIII (1)
Luc2 1757..3409
ITR 3573..3717
SV40 pA 3416..3549

FIG.10

B

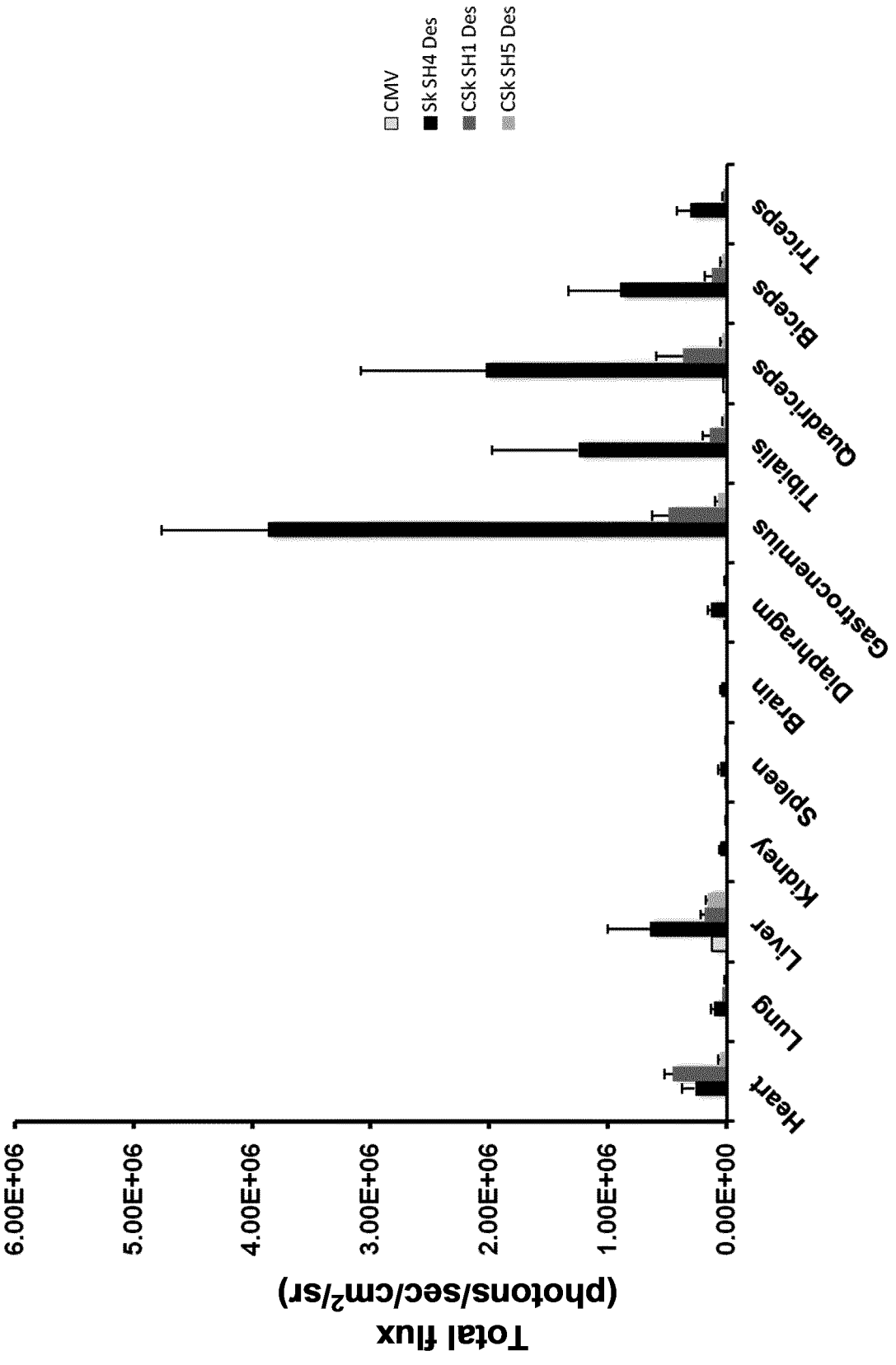

CMV
Sk SH4 Des
CSk SH1 Des
CSk SH5 Des
Heart
Lung
Liver
Kidney
Spleen
Brain
Diaphragm
Gastrocnemius
Tibialis
Quadriceps
Biceps
Triceps
0.00E+00
1.00E+06
2.00E+06
3.00E+06
4.00E+06
5.00E+06
6.00E+06
Total flux
(photons/sec/cm²/sr)

FIG.10

C

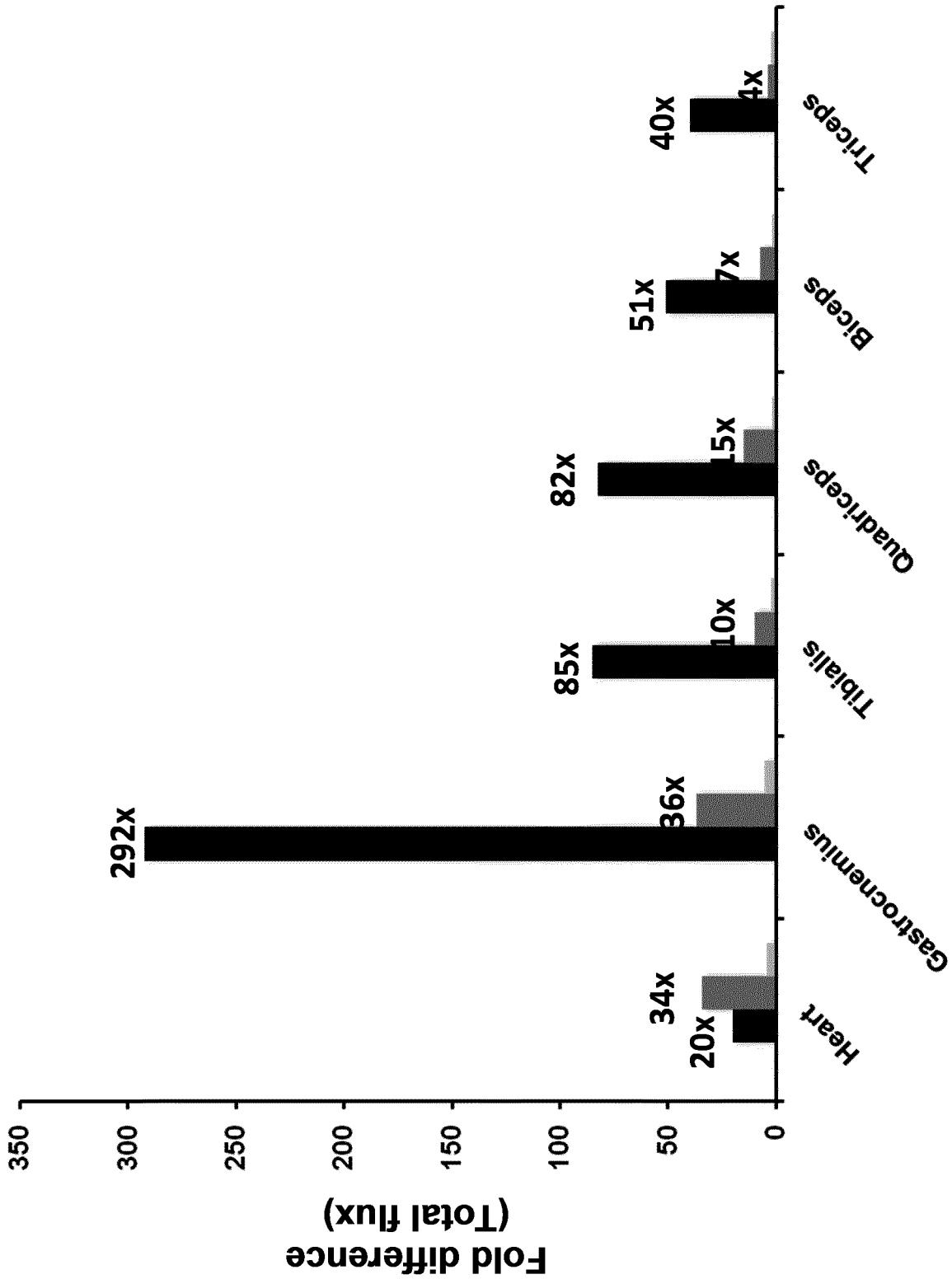

Fold difference
(Total flux)
350
300
250
200
150
100
50
0
Heart
Gastrocnemius
Tibialis
Quadriceps
Biceps
Triceps
20x
34x
292x
36x
85x
10x
82x
15x
51x
7x
40x
4x

FIG.11

A

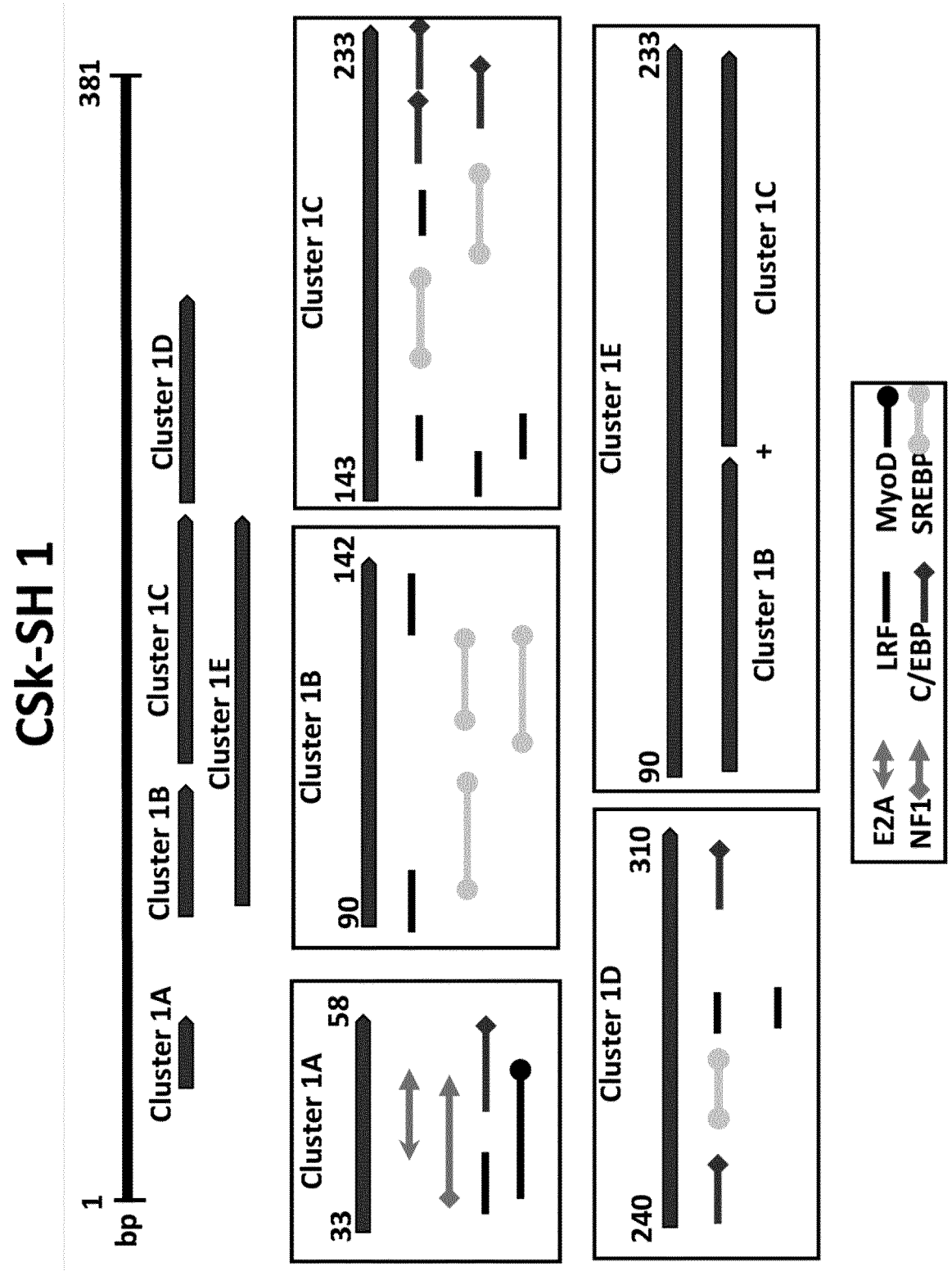

CSk-SH 1
1
bp
381
Cluster 1A
Cluster 1B
Cluster 1C
Cluster 1D
Cluster 1E
Cluster 1A
33
58
Cluster 1B
90
142
Cluster 1C
143
233
Cluster 1D
240
310
Cluster 1E
90
233
Cluster 1B
+
Cluster 1C
E2A
NF1
LRF
C/EBP
MyoD
SREBP

FIG. 11

B

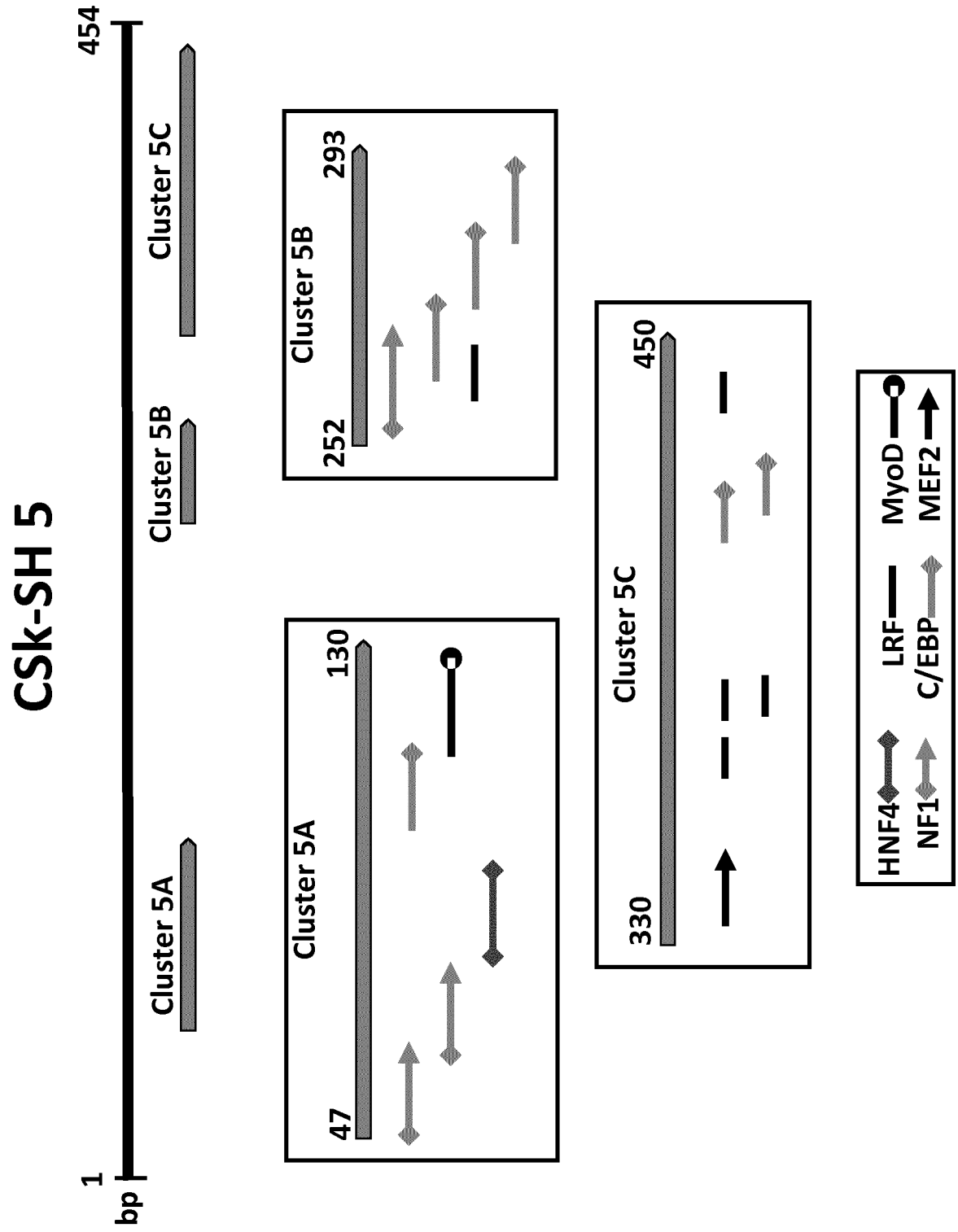

CSk-SH 5
454
1
bp
Cluster 5A
Cluster 5B
Cluster 5C
Cluster 5A
47
130
Cluster 5B
252
293
Cluster 5C
330
450
HNF4
NF1
LRF
C/EBP
MyoD
MEF2

FIG. 11

C

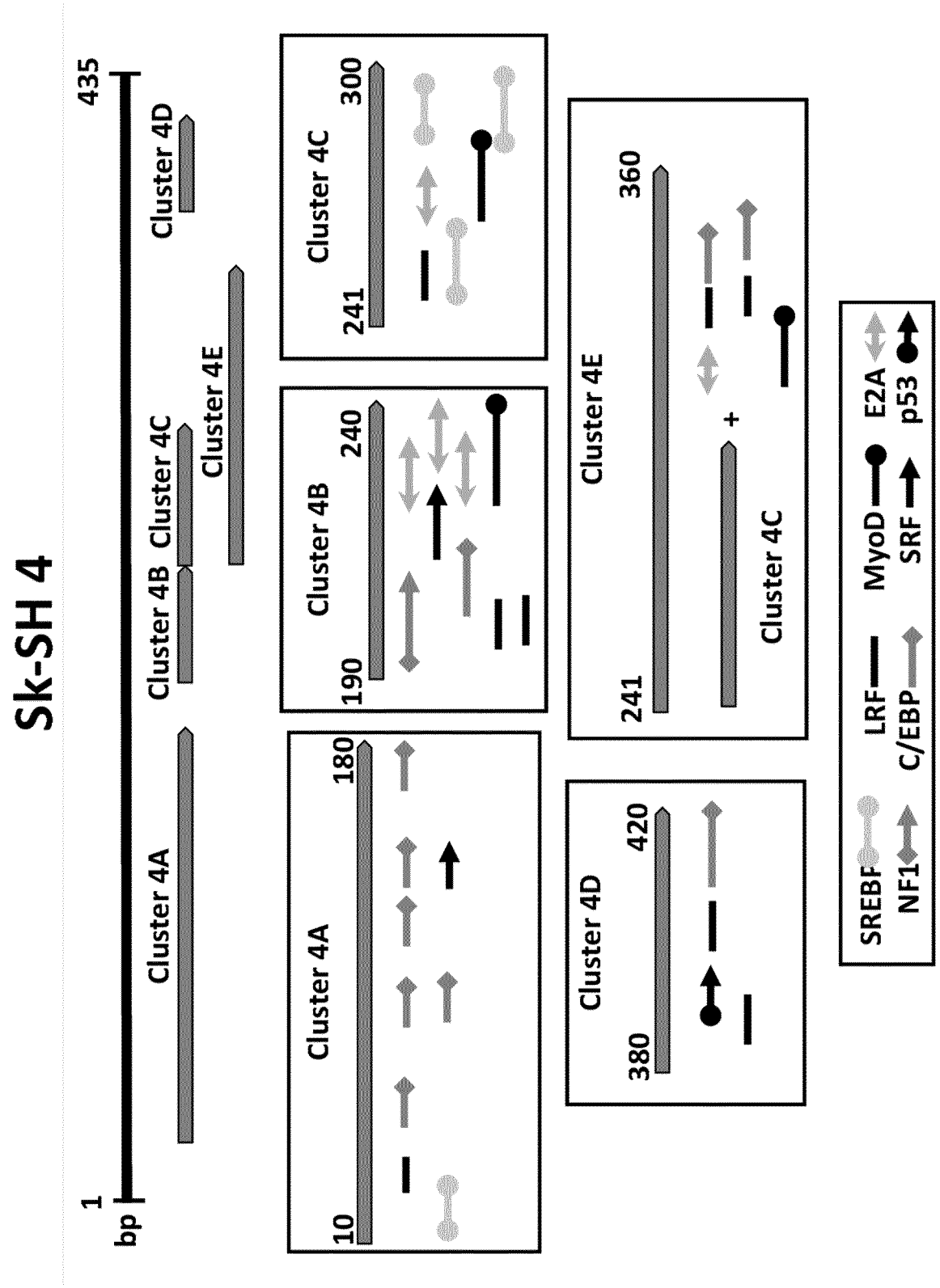

Sk-SH 4
1
bp
435
Cluster 4A
Cluster 4B
Cluster 4C
Cluster 4D
Cluster 4E
10
180
190
240
241
300
360
380
420
+
SREBF
LRF
MyoD
E2A
NF1
C/EBP
SRF
p53

FIG. 12

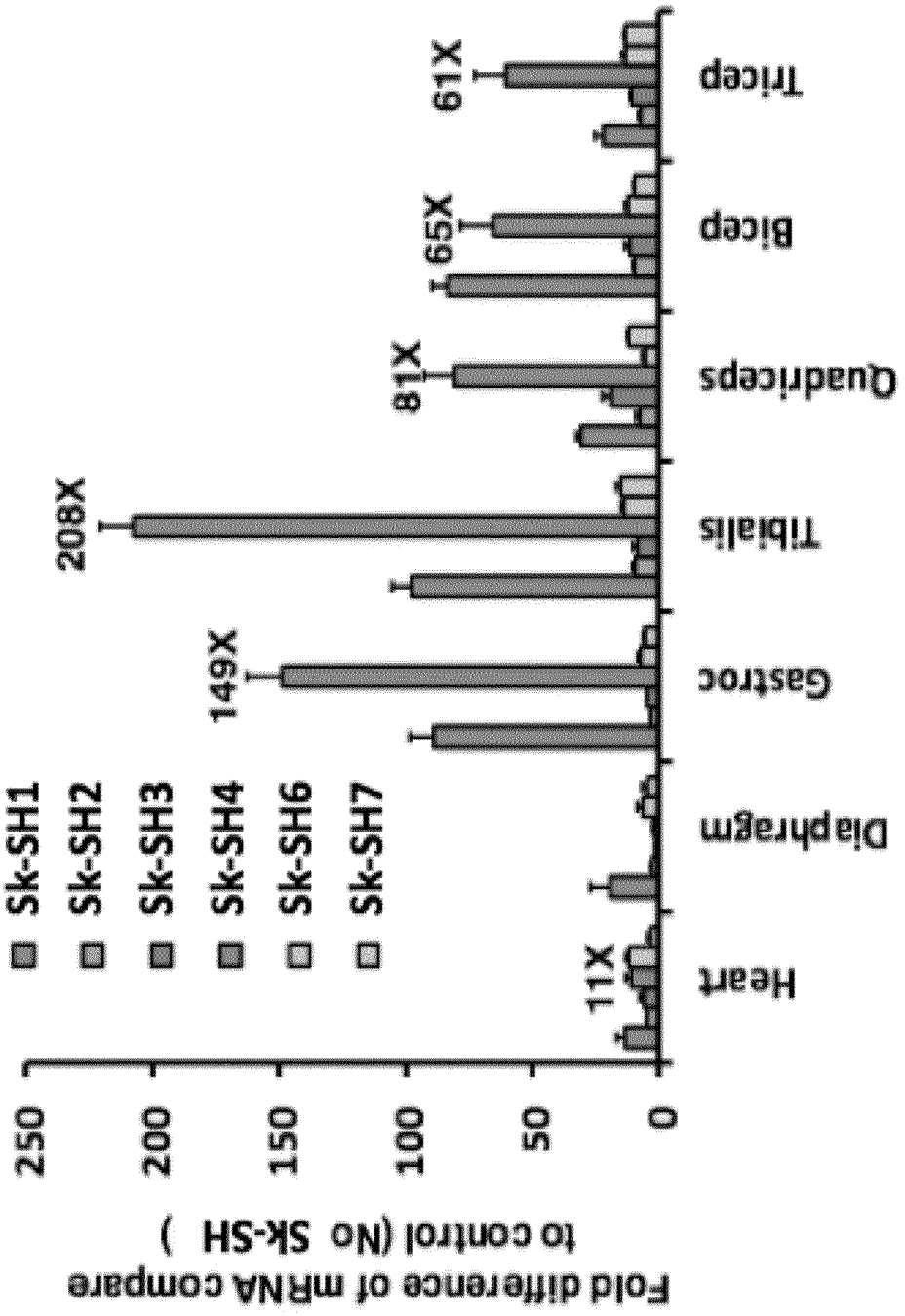
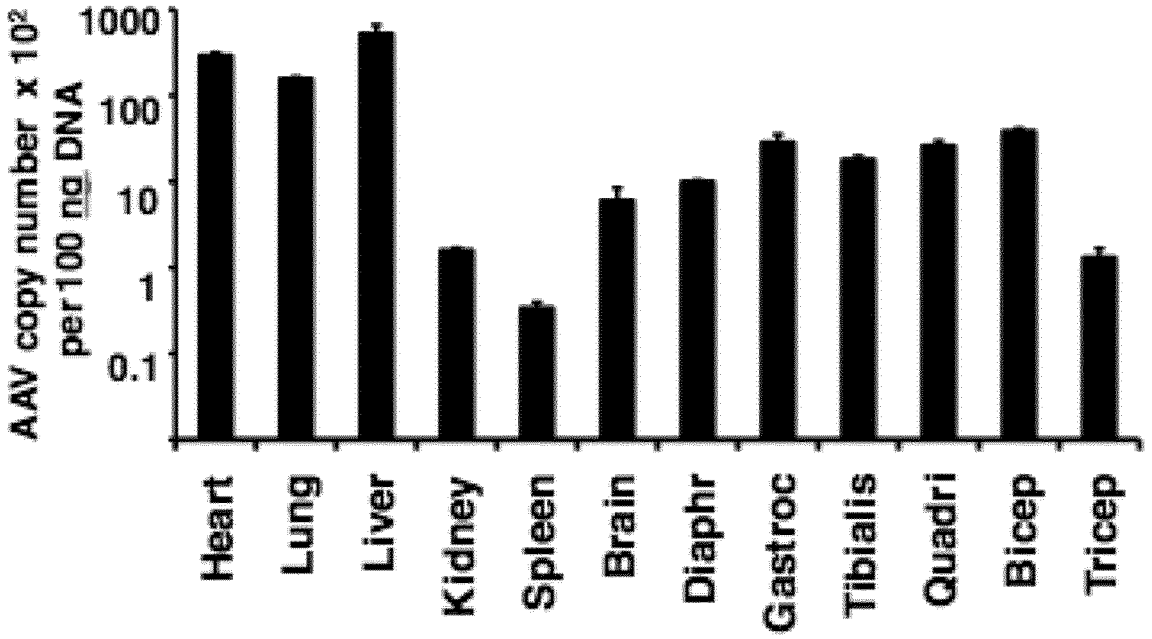

Fold difference of mRNA compare
to control (No Sk-SH )
250
200
150
100
50
0
Sk-SH1
Sk-SH2
Sk-SH3
Sk-SH4
Sk-SH6
Sk-SH7
11X
149X
208X
81X
65X
61X
Heart
Diaphragm
Gastroc
Tibialis
Quadriceps
Bicep
Tricep

FIG. 13

A


AAV copy number x 10²
per 100 ng DNA
1000
100
10
1
0.1
Heart
Lung
Liver
Kidney
Spleen
Brain
Diaphr
Gastroc
Tibialis
Quadri
Bicep
Tricep

B

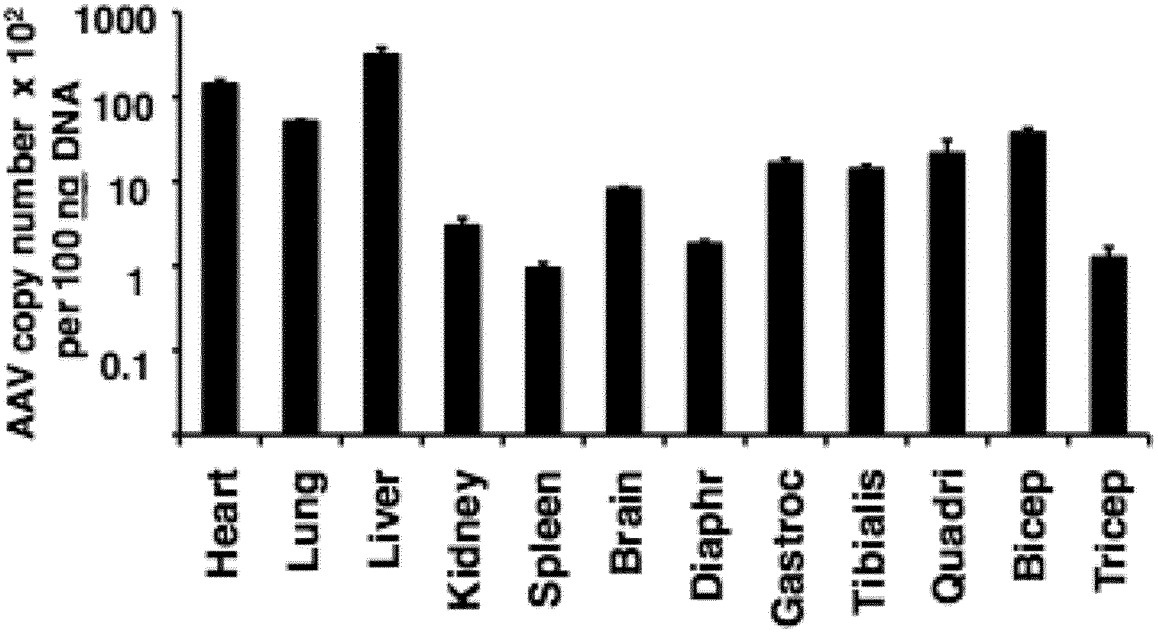

AAV copy number x 10²
per 100 ng DNA
1000
100
10
1
0.1
Heart
Lung
Liver
Kidney
Spleen
Brain
Diaphr
Gastroc
Tibialis
Quadri
Bicep
Tricep

FIG. 14

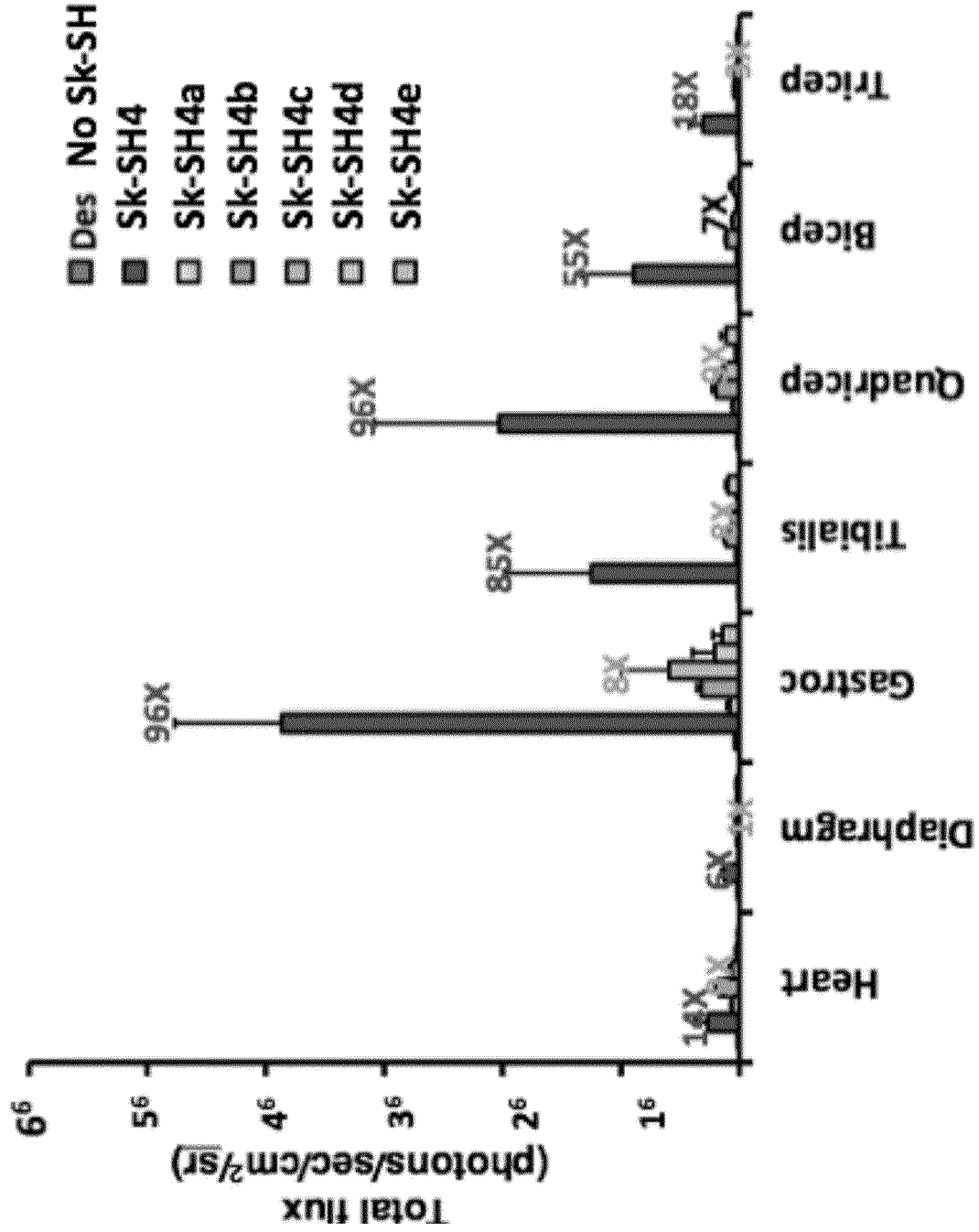

Des
No Sk-SH
Sk-SH4
Sk-SH4a
Sk-SH4b
Sk-SH4c
Sk-SH4d
Sk-SH4e
Total flux
(photons/sec/cm²/sr)
6⁶
5⁶
4⁶
3⁶
2⁶
1⁶
Heart
Diaphragm
Gastroc
Tibialis
Quadricep
Bicep
Tricep
14X
6X
96X
8X
85X
96X
55X
7X
18X

FIG. 15

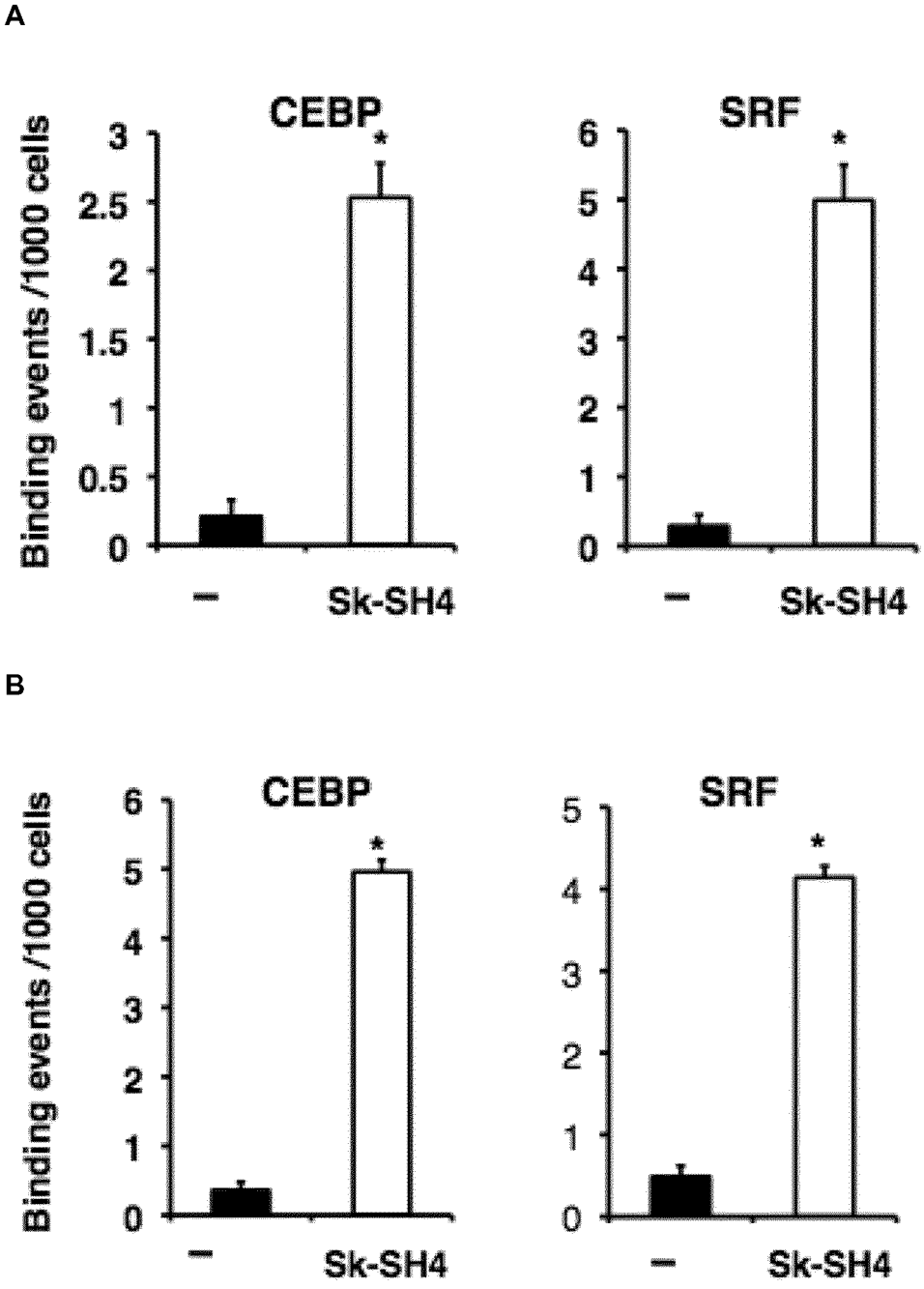
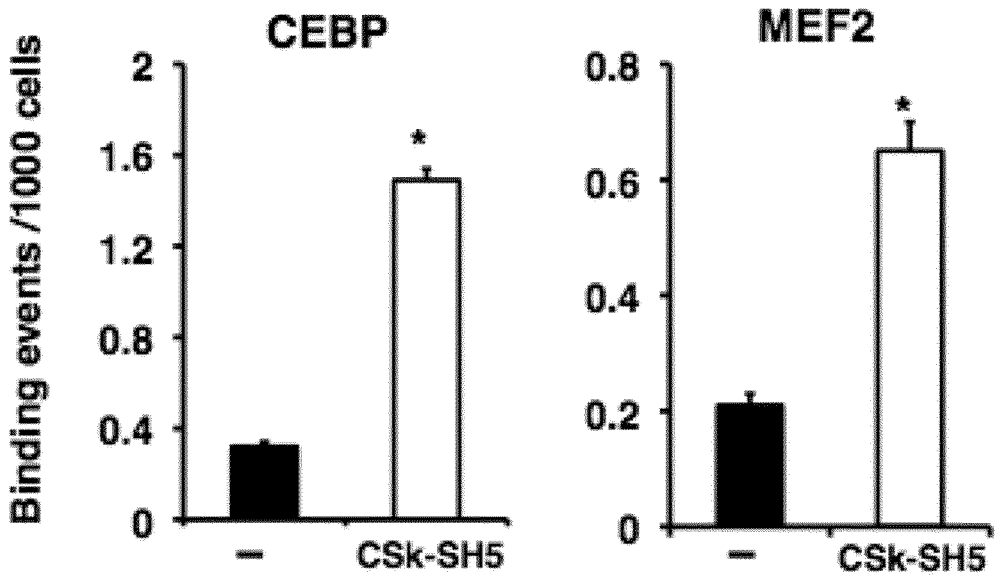

A
CEBP
SRF
Binding events /1000 cells
3
2.5
2
1.5
1
0.5
0
6
5
4
3
2
1
0
*
*
–
Sk-SH4
–
Sk-SH4
B
CEBP
SRF
Binding events /1000 cells
6
5
4
3
2
1
0
5
4
3
2
1
0
*
*
–
Sk-SH4
–
Sk-SH4

FIG. 15

C


CEBP
MEF2
Binding events /1000 cells
2
1.6
1.2
0.8
0.4
0
0.8
0.6
0.4
0.2
0
*
*
–
CSk-SH5
–
CSk-SH5

D

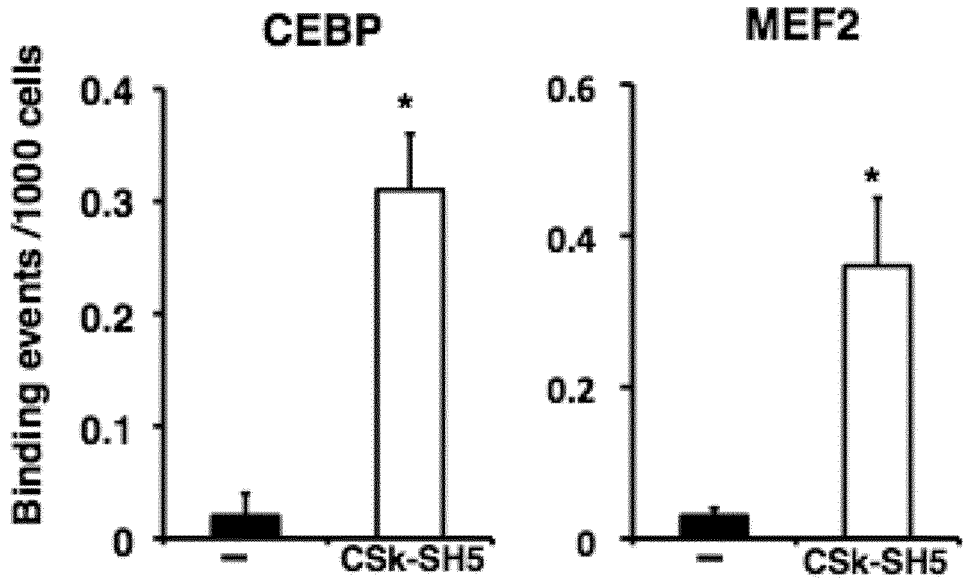

CEBP
MEF2
Binding events /1000 cells
0.4
0.3
0.2
0.1
0
0.6
0.4
0.2
0
*
*
–
CSk-SH5
–
CSk-SH5

FIG. 16

A

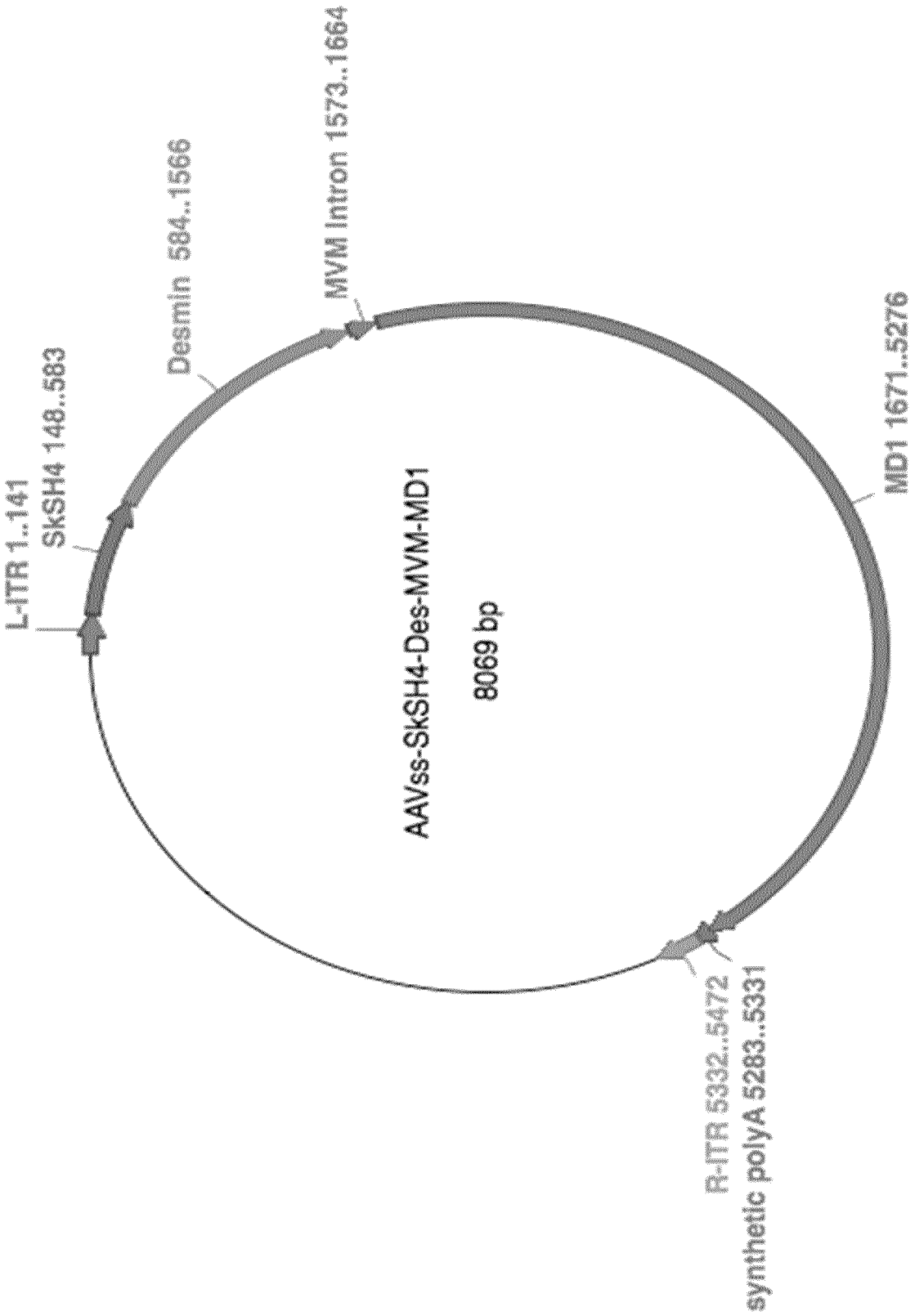

L-ITR 1..141
SkSH4 148..583
Desmin 584..1566
MVM Intron 1573..1664
MD1 1671..5276
AAVss-SkSH4-Des-MVM-MD1
8069 bp
R-ITR 5332..5472
synthetic polyA 5283..5331

**FIG. 16**

**B**

SEQ ID NO:44

CCTGCAGGCAGCTGCGCGCTCGCTCGCTCACTGAGGCCGCCCGGGCAAAGCCCGGGCGTCG
GGCGACCTTTGGTCGCCCGGCCTCAGTGAGCGAGCGAGCGCGCAGAGAGGGAGTGGCCAAC
TCCATCACTAGGGGTTCCTCGTACGTTCTGAGTCCTCTAAGGTCCCTCACTCCCAACTCAG
CCCCATGTCCTGTCAATTCCCACTCAGTGTCTGATCTCCTTCTCCTCACCTTTCCCATCTC
CCGTTTGACCCAAGCTTCCTGAGCTCTCCTCCCATTCCCCTTTTTGGAGTCCTCCTCCTCT
CCCAGAACCCAGTAATAAGTGGGCTCCTCCCTGGCCTGGACCCCCGTGGTAACCCTATAAG
GCGAGGCAGCTGCTGTCTGAGGCAGGGAGGGGCTGGTGTGGGAGGCTAAGGGCAGCTGCTA
AGTTTAGGGTGGCTCCTTCTCTCTTCTTAGAGACAACAGGTGGCTGGGGCCTCAGTGCCCA
GAAAAGAAAATGTCTTAGAGGTATCGGCATGGGCCTGGAGGAGGGGGGACAGGGCAGGGGG
AGGCATCTTCCTCAGGACATCGGGTCCTAGAGGGACCTTGCTTCCTAGCTGGGCCTTTCCT
TCTCCTCTATAAATACCAGCTCTGGTATTTCGCCTTGGCAGCTGTTGCTGCTAGGGAGACG
GCTGGCTTGACATGCATCTCCTGACAAAACACAAACCCGTGGTGTGAGTGGGTGTGGGCGG
TGTGAGTAGGGGGATGAATCAGAGAGGGGGCGAGGGAGACAGGGGCGCAGGAGTCAGGCAA
AGGCGATGCGGGGGTGCGACTACACGCAGTTGGAAACAGTCGTCAGAAGATTCTGGAAACT
ATCTTGCTGGCTATAAACTTGAGGGAAGCAGAAGGCCAACATTCCTCCCAAGGGAAACTGA
GGCTCAGAGTTAAAACCCAGGTATCAGTGATATGCATGTGCCCCGGCCAGGGTCACTCTCT
GACTAACCGGTACCTACCCTACAGGCCTACCTAGAGACTCTTTTGAAAGGATGGTAGAGAC
CTGTCCGGGCTTTGCCCACAGTCGTTGGAAACCTCAGCATTTTCTAGGCAACTTGTGCGAA
TAAAACACTTCGGGGGTCCTTCTTGTTCATTCCAATAACCTAAAACCTCTCCTCGGAGAAA
ATAGGGGGCCTCAAACAAACGAAATTCTCTAGCCCGCTTTCCCCAGGATAAGGCAGGCATC
CAAATGGAAAAAAAGGGGCCGGCCGGGGGTCTCCTGTCAGCTCCTTGCCCTGTGAAACCCA
GCAGGCCTGCCTGTCTTCTGTCCTCTTGGGGCTGTCCAGGGGCGCAGGCCTCTTGCGGGGG
AGCTGGCCTCCCCGCCCCCTCGCCTGTGGCCGCCCTTTTCCTGGCAGGACAGAGGGATCCT
GCAGCTGTCAGGGGAGGGGCGCCGGGGGGTGATGTCAGGAGGGCTACAAATAGTGCAGACA
GCTAAGGGGCTCCGTCACCCATCTTCACATCCACTCCAGCCGGCTGCCCGCCCGCTGCCTC
CTCTGTGCGTCCGCCCAGCCAGCCTCGTCCACGCCGCCACCTCTAGAAAGAGGTAAGGGTT
TAAGGGATGGTTGGTTGGTGGGGTATTAATGTTTAATTACCTGGAGCACCTGCCTGAAATC
ACTTTTTTTCAGGTTGGACGCGTGCCACCATGCTGTGGTGGGAGGAAGTGGAGGACTGCTA
CGAGAGAGAGGACGTGCAGAAGAAAACCTTCACCAAGTGGGTGAACGCCCAGTTCAGCAAG
TTCGGCAAGCAGCACATCGAGAACCTGTTCAGCGACCTGCAGGATGGCAGGAGACTGCTGG
ATCTGCTGGAGGGACTGACCGGCCAGAAGCTGCCCAAGGAGAAGGGCAGCACCAGAGTGCA
CGCCCTGAACAACGTGAACAAGGCCCTGAGAGTGCTGCAGAACAACAACGTGGACCTGGTG
AATATCGGCAGCACCGACATCGTGGACGGCAACCACAAGCTGACCCTGGGCCTGATCTGGA
ACATCATCCTGCACTGGCAGGTGAAGAACGTGATGAAGAACATCATGGCCGGCCTGCAGCA
GACCAACAGCGAGAAGATCCTGCTGAGCTGGGTGAGGCAGAGCACCAGAAACTACCCCCAG
GTGAACGTGATCAACTTCACCACCTCCTGGAGCGACGGCCTGGCCCTGAACGCCCTGATCC
ACAGCCACAGACCCGACCTGTTCGACTGGAACAGCGTGGTGTGTCAGCAGAGCGCCACCCA
GAGACTGGAGCACGCCTTCAACATCGCCAGATACCAGCTGGGCATCGAGAAGCTGCTGGAC
CCCGAGGACGTGGACACCACCTACCCCGACAAGAAAAGCATCCTGATGTATATTACCTCTC
TGTTTCAGGTGCTGCCCCAGCAGGTGTCCATCGAGGCCATCCAGGAAGTGGAAATGCTGCC
CAGGCCCCCCACCGTGTCCCTGGCCCAGGGCTATGAGAGAACCAGCAGCCCCAAGCCCAGA
TTCAAGAGCACCGTGTCCCTGGCCCAGGGCTATGAGAGAACCAGCAGCCCCAAGCCCAGAT
TCAAGAGCTACGCCTACACCCAGGCCGCCTACGTGACCACCTCCGACCCCACCAGAAGCCC
CTTCCCCAGCCAGCACCTGGAGGCCCCCGAGGACAAGAGCTTCGGCAGCAGCCTGATGGAG

AGCGAAGTGAACCTGGACAGATACCAGACCGCCCTGGAGGAAGTGCTGTCTTGGCTGCTGT
CCGCCGAGGACACCCTGCAGGCCCAGGGCGAGATCAGCAACGACGTGGAAGTGGTGAAGGA
CCAGTTCCACACCCACGAGGGCTACATGATGGATCTGACCGCCCACCAGGGCAGAGTGGGC
AATATCCTGCAGCTGGGCAGCAAGCTGATCGGCACCGGCAAGCTGAGCGAGGACGAGGAGA
CCGAAGTGCAGGAGCAGATGAACCTGCTGAACAGCAGATGGGAGTGCCTGAGAGTGGCCAG
CATGGAGAAGCAGAGCAACCTGCACCGCGTGCTGATGGACCTGCAGAACCAGAAGCTGAAG
GAGCTGAACGACTGGCTGACCAAGACCGAGGAGCGGACCAGAAAGATGGAGGAGGAGCCCC
TGGGCCCCGACCTGGAGGACCTGAAGAGACAGGTGCAGCAGCACAAAGTGCTGCAGGAGGA
CCTGGAACAGGAGCAGGTGCGCGTGAACAGCCTGACCCACATGGTGGTCGTGGTGGACGAG
AGCAGCGGCGACCACGCCACAGCCGCCCTGGAAGAGCAGCTGAAAGTGCTGGGCGACAGAT
GGGCCAACATCTGCCGGTGGACCGAGGACAGATGGGTGCTGCTGCAGGACATCCTGCTGAA
GTGGCAGAGACTGACAGAGGAGCAGTGCCTGTTTAGCGCCTGGCTGAGCGAGAAGGAGGAC
GCCGTGAACAAGATCCACACCACCGGCTTCAAGGACCAGAACGAGATGCTGAGCAGCCTGC
AGAAGCTGGCCGTGCTGAAGGCCGATCTGGAGAAGAAAAAGCAGAGCATGGGCAAGCTGTA
CTCCCTGAAGCAGGACCTGCTGTCCACCCTGAAGAACAAGAGCGTGACCCAGAAAACCGAG
GCCTGGCTGGACAATTTCGCCCGGTGCTGGGACAATCTGGTGCAGAAACTGGAGAAGAGCA
CCGCCCAGATCAGCCAGGCCGTGACCACCACCCAGCCCAGCCTGACACAGACCACCGTGAT
GGAGACCGTGACCACAGTGACCACCAGGGAGCAGATCCTGGTGAAGCACGCCCAGGAGGAG
CTGCCCCCTCCCCCCCCCTCAGAAGAAGCGGCAGATCACAGTGGACACCCTGGAGAGACTGC
AGGAGCTGCAGGAAGCCACCGACGAGCTGGACCTGAAGCTGAGACAGGCCGAAGTGATCAA
GGGCAGCTGGCAGCCTGTGGGCGATCTGCTGATCGACAGCCTGCAGGACCACCTGGAGAAA
GTGAAGGCCCTGCGGGGCGAGATCGCCCCCCTGAAGGAGAATGTGAGCCACGTGAACGACC
TGGCCAGACAGCTGACCACCCTGGGCATCCAGCTGAGCCCCTACAATCTGAGCACCCTGGA
AGATCTGAACACCCGGTGGAAACTGCTGCAGGTGGCCGTGGAGGATAGAGTGAGGCAGCTG
CACGAGGCCCACAGAGACTTCGGCCCTGCCTCCCAGCACTTCCTGAGCACCAGCGTGCAGG
GCCCCTGGGAGAGAGCCATCTCCCCCAACAAAGTGCCCTACTACATCAACCACGAGACCCA
GACCACCTGCTGGGACCACCCTAAGATGACCGAGCTGTACCAGAGCCTGGCCGACCTGAAC
AATGTGCGGTTCAGCGCCTACAGAACCGCCATGAAGCTGCGGAGACTGCAGAAGGCCCTGT
GCCTGGACCTGCTGAGCCTGAGCGCCGCCTGCGACGCCCTGGACCAGCACAACCTGAAGCA
GAACGACCAGCCCATGGACATTCTGCAGATCATCAACTGCCTGACCACCATCTACGATCGG
CTGGAGCAGGAGCACAACAACCTGGTGAACGTGCCCCTGTGCGTGGACATGTGCCTGAATT
GGCTGCTGAACGTGTACGACACCGGCAGGACCGGCAGAATCAGAGTGCTGTCCTTCAAGAC
CGGCATCATCAGCCTGTGCAAGGCCCACCTGGAGGATAAGTACCGCTACCTGTTCAAGCAG
GTGGCCAGCAGCACCGGCTTCTGCGATCAGAGGAGACTGGGCCTGCTGCTGCACGATAGCA
TCCAGATCCCTAGGCAGCTGGGCGAAGTGGCCAGCTTTGGCGGCAGCAACATCGAGCCCTC
TGTGAGGAGCTGCTTCCAGTTCGCCAACAACAAGCCCGAGATCGAGGCCGCCCTGTTCCTG
GATTGGATGAGGCTGGAGCCCCAGAGCATGGTGTGGCTGCCTGTGCTGCACAGAGTGGCCG
CCGCCGAGACCGCCAAGCACCAGGCCAAGTGCAACATCTGCAAGGAGTGCCCCATCATCGG
CTTCCGGTACAGGAGCCTGAAGCACTTCAACTACGACATCTGCCAGAGCTGCTTTTTCAGC
GGCAGAGTGGCCAAGGGCCACAAGATGCACTACCCCATGGTGGAGTACTGCACCCCCACCA
CCTCCGGCGAGGATGTGAGAGACTTCGCCAAAGTGCTGAAGAATAAGTTCCGGACCAAGCG
GTACTTTGCCAAGCACCCCAGGATGGGCTACCTGCCCGTGCAGACCGTGCTGGAGGGCGAC
AACATGGAGACCGACACCATGTGATGATGACTCGAGAATAAAAGATCTTTATTTTCATTAG
ATCTGTGTGTTGGTTTTTTGTGTGAGGAACCCCTAGTGATGGAGTTGGCCACTCCCTCTCT
GCGCGCTCGCTCGCTCACTGAGGCCGGGCGACCAAAGGTCGCCCGACGCCCGGGCTTTGCC
CGGGCGGCCTCAGTGAGCGAGCGAGCGCGCAGCTGCCTGCAGGGGCGCCTGATGCGGTATT
TTCTCCTTACGCATCTGTGCGGTATTTCACACCGCATACGTCAAAGCAACCATAGTACGCG
CCCTGTAGCGGCGCATTAAGCGCGGCGGGTGTGGTGGTTACGCGCAGCGTGACCGCTACAC
TTGCCAGCGCCCTAGCGCCCGCTCCTTTCGCTTTCTTCCCTTCCTTTCTCGCCACGTTCGC

CGGCTTTCCCCGTCAAGCTCTAAATCGGGGGCTCCCTTTAGGGTTCCGATTTAGTGCTTTA
CGGCACCTCGACCCCAAAAAACTTGATTTGGGTGATGGTTCACGTAGTGGGCCATCGCCCT
GATAGACGGTTTTTCGCCCTTTGACGTTGGAGTCCACGTTCTTTAATAGTGGACTCTTGTT
CCAAACTGGAACAACACTCAACCCTATCTCGGGCTATTCTTTTGATTTATAAGGGATTTTG
CCGATTTCGGCCTATTGGTTAAAAAATGAGCTGATTTAACAAAAATTTAACGCGAATTTTA
ACAAAATATTAACGTTTACAATTTTATGGTGCACTCTCAGTACAATCTGCTCTGATGCCGC
ATAGTTAAGCCAGCCCCGACACCCGCCAACACCCGCTGACGCGCCCTGACGGGCTTGTCTG
CTCCCGGCATCCGCTTACAGACAAGCTGTGACCGTCTCCGGGAGCTGCATGTGTCAGAGGT
TTTCACCGTCATCACCGAAACGCGCGAGACGAAAGGGCCTCGTGATACGCCTATTTTTATA
GGTTAATGTCATGATAATAATGGTTTCTTAGACGTCAGGTGGCACTTTTCGGGGAAATGTG
CGCGGAACCCCTATTTGTTTATTTTTCTAAATACATTCAAATATGTATCCGCTCATGAGAC
AATAACCCTGATAAATGCTTCAATAATATTGAAAAAGGAAGAGTATGAGTATTCAACATTT
CCGTGTCGCCCTTATTCCCTTTTTTGCGGCATTTTGCCTTCCTGTTTTTGCTCACCCAGAA
ACGCTGGTGAAAGTAAAAGATGCTGAAGATCAGTTGGGTGCACGAGTGGGTTACATCGAAC
TGGATCTCAACAGCGGTAAGATCCTTGAGAGTTTTCGCCCCGAAGAACGTTTTCCAATGAT
GAGCACTTTTAAAGTTCTGCTATGTGGCGCGGTATTATCCCGTATTGACGCCGGGCAAGAG
CAACTCGGTCGCCGCATACACTATTCTCAGAATGACTTGGTTGAGTACTCACCAGTCACAG
AAAAGCATCTTACGGATGGCATGACAGTAAGAGAATTATGCAGTGCTGCCATAACCATGAG
TGATAACACTGCGGCCAACTTACTTCTGACAACGATCGGAGGACCGAAGGAGCTAACCGCT
TTTTTGCACAACATGGGGGATCATGTAACTCGCCTTGATCGTTGGGAACCGGAGCTGAATG
AAGCCATACCAAACGACGAGCGTGACACCACGATGCCTGTAGCAATGGCAACAACGTTGCG
CAAACTATTAACTGGCGAACTACTTACTCTAGCTTCCCGGCAACAATTAATAGACTGGATG
GAGGCGGATAAAGTTGCAGGACCACTTCTGCGCTCGGCCCTTCCGGCTGGCTGGTTTATTG
CTGATAAATCTGGAGCCGGTGAGCGTGGGTCTCGCGGTATCATTGCAGCACTGGGGCCAGA
TGGTAAGCCCTCCCGTATCGTAGTTATCTACACGACGGGGAGTCAGGCAACTATGGATGAA
CGAAATAGACAGATCGCTGAGATAGGTGCCTCACTGATTAAGCATTGGTAACTGTCAGACC
AAGTTTACTCATATATACTTTAGATTGATTTAAAACTTCATTTTTAATTTAAAAGGATCTA
GGTGAAGATCCTTTTTGATAATCTCATGACCAAAATCCCTTAACGTGAGTTTTCGTTCCAC
TGAGCGTCAGACCCCGTAGAAAAGATCAAAGGATCTTCTTGAGATCCTTTTTTTCTGCGCG
TAATCTGCTGCTTGCAAACAAAAAAACCACCGCTACCAGCGGTGGTTTGTTTGCCGGATCA
AGAGCTACCAACTCTTTTTCCGAAGGTAACTGGCTTCAGCAGAGCGCAGATACCAAATACT
GTCCTTCTAGTGTAGCCGTAGTTAGGCCACCACTTCAAGAACTCTGTAGCACCGCCTACAT
ACCTCGCTCTGCTAATCCTGTTACCAGTGGCTGCTGCCAGTGGCGATAAGTCGTGTCTTAC
CGGGTTGGACTCAAGACGATAGTTACCGGATAAGGCGCAGCGGTCGGGCTGAACGGGGGGT
TCGTGCACACAGCCCAGCTTGGAGCGAACGACCTACACCGAACTGAGATACCTACAGCGTG
AGCTATGAGAAAGCGCCACGCTTCCCGAAGGGAGAAAGGCGGACAGGTATCCGGTAAGCGG
CAGGGTCGGAACAGGAGAGCGCACGAGGGAGCTTCCAGGGGGAAACGCCTGGTATCTTTAT
AGTCCTGTCGGGTTTCGCCACCTCTGACTTGAGCGTCGATTTTTGTGATGCTCGTCAGGGG
GGCGGAGCCTATGGAAAAACGCCAGCAACGCGGCCTTTTTACGGTTCCTGGCCTTTTGCTG
GCCTTTTGCTCACATGT

FIG. 16

C

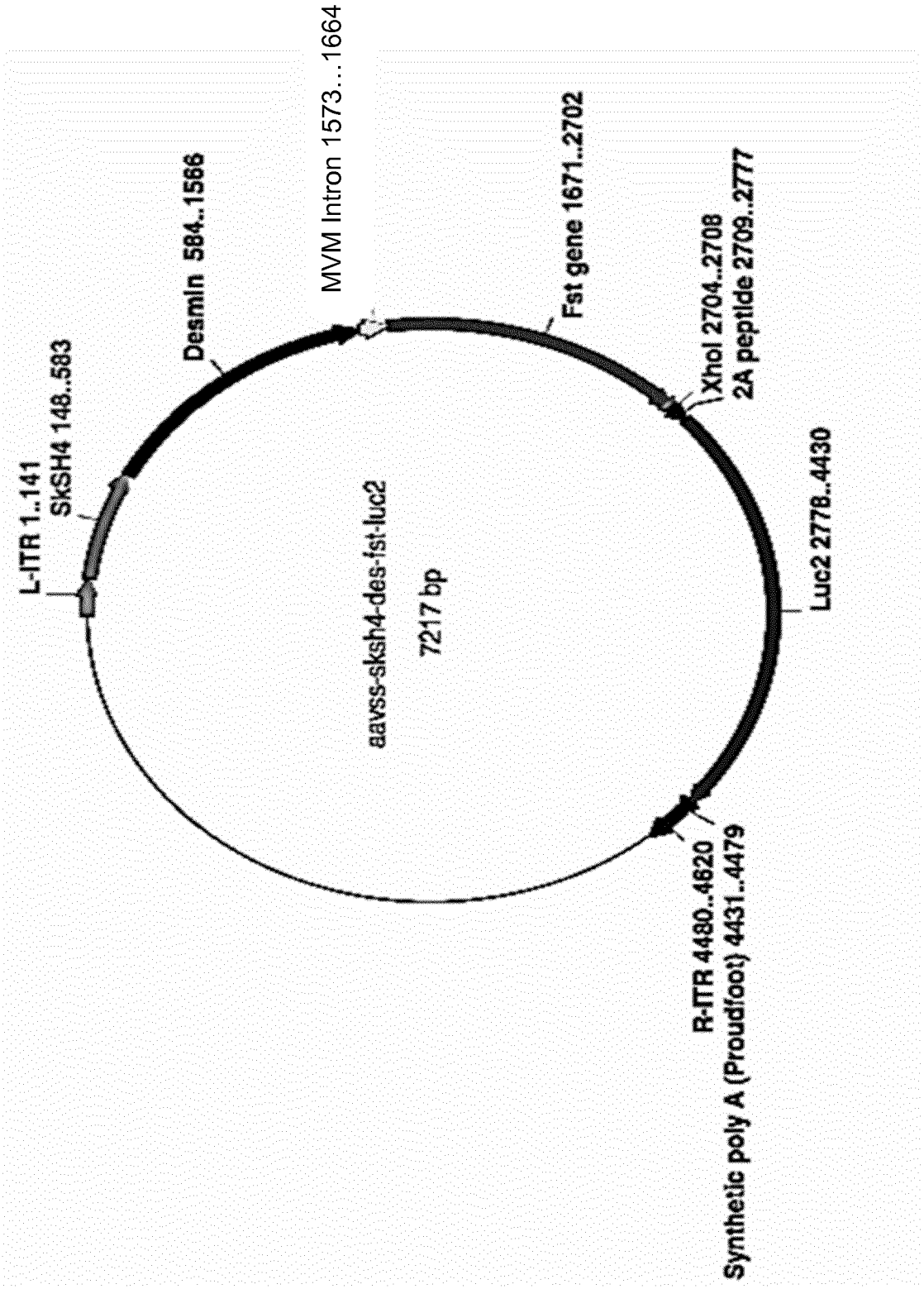

MVM Intron 1573…1664
Fst gene 1671..2702
XhoI 2704..2708
2A peptide 2709..2777
Luc2 2778..4430
R-ITR 4480..4620
Synthetic poly A (Proudfoot) 4431..4479
L-ITR 1..141
SkSH4 148..583
Desmin 584..1566
aavss-sksh4-des-fst-luc2
7217 bp

**FIG. 16**

**D**

SEQ ID NO:45

CCTGCAGGCAGCTGCGCGCTCGCTCGCTCACTGAGGCCGCCCGGGCAAAGCCCGGGCGTCG
GGCGACCTTTGGTCGCCCGGCCTCAGTGAGCGAGCGAGCGCGCAGAGAGGGAGTGGCCAAC
TCCATCACTAGGGGTTCCTCGTACGTTCTGAGTCCTCTAAGGTCCCTCACTCCCAACTCAG
CCCCATGTCCTGTCAATTCCCACTCAGTGTCTGATCTCCTTCTCCTCACCTTTCCCATCTC
CCGTTTGACCCAAGCTTCCTGAGCTCTCCTCCCATTCCCCTTTTTGGAGTCCTCCTCCTCT
CCCAGAACCCAGTAATAAGTGGGCTCCTCCCTGGCCTGGACCCCCGTGGTAACCCTATAAG
GCGAGGCAGCTGCTGTCTGAGGCAGGGAGGGGCTGGTGTGGGAGGCTAAGGGCAGCTGCTA
AGTTTAGGGTGGCTCCTTCTCTCTTCTTAGAGACAACAGGTGGCTGGGGCCTCAGTGCCCA
GAAAAGAAAATGTCTTAGAGGTATCGGCATGGGCCTGGAGGAGGGGGGACAGGGCAGGGGG
AGGCATCTTCCTCAGGACATCGGGTCCTAGAGGGACCTTGCTTCCTAGCTGGGCCTTTCCT
TCTCCTCTATAAATACCAGCTCTGGTATTTCGCCTTGGCAGCTGTTGCTGCTAGGGAGACG
GCTGGCTTGACATGCATCTCCTGACAAAACACAAACCCGTGGTGTGAGTGGGTGTGGGCGG
TGTGAGTAGGGGGATGAATCAGAGAGGGGGCGAGGGAGACAGGGGCGCAGGAGTCAGGCAA
AGGCGATGCGGGGGTGCGACTACACGCAGTTGGAAACAGTCGTCAGAAGATTCTGGAAACT
ATCTTGCTGGCTATAAACTTGAGGGAAGCAGAAGGCCAACATTCCTCCCAAGGGAAACTGA
GGCTCAGAGTTAAAACCCAGGTATCAGTGATATGCATGTGCCCCGGCCAGGGTCACTCTCT
GACTAACCGGTACCTACCCTACAGGCCTACCTAGAGACTCTTTTGAAAGGATGGTAGAGAC
CTGTCCGGGCTTTGCCCACAGTCGTTGGAAACCTCAGCATTTTCTAGGCAACTTGTGCGAA
TAAAACACTTCGGGGGTCCTTCTTGTTCATTCCAATAACCTAAAACCTCTCCTCGGAGAAA
ATAGGGGGCCTCAAACAAACGAAATTCTCTAGCCCGCTTTCCCCAGGATAAGGCAGGCATC
CAAATGGAAAAAAAGGGGCCGGCCGGGGGTCTCCTGTCAGCTCCTTGCCCTGTGAAACCCA
GCAGGCCTGCCTGTCTTCTGTCCTCTTGGGGCTGTCCAGGGGCGCAGGCCTCTTGCGGGGG
AGCTGGCCTCCCCGCCCCCTCGCCTGTGGCCGCCCTTTTCCTGGCAGGACAGAGGGATCCT
GCAGCTGTCAGGGGAGGGGCGCCGGGGGGTGATGTCAGGAGGGCTACAAATAGTGCAGACA
GCTAAGGGGCTCCGTCACCCATCTTCACATCCACTCCAGCCGGCTGCCCGCCCGCTGCCTC
CTCTGTGCGTCCGCCCAGCCAGCCTCGTCCACGCCGCCACCTCTAGAAAGAGGTAAGGGTT
TAAGGGATGGTTGGTTGGTGGGGTATTAATGTTTAATTACCTGGAGCACCTGCCTGAAATC
ACTTTTTTTCAGGTTGGACGCGTATGGTCCGCGCGAGGCACCAGCCGGGTGGGCTTTGCCT
CCTGCTGCTGCTGCTCTGCCAGTTCATGGAGGACCGCAGTGCCCAGGCTGGGAACTGCTGG
CTCCGTCAAGCGAAGAACGGCCGCTGCCAGGTCCTGTACAAGACCGAACTGAGCAAGGAGG
AGTGCTGCAGCACCGGCCGGCTGAGCACCTCGTGGACCGAGGAGGACGTGAATGACAACAC
ACTCTTCAAGTGGATGATTTTCAACGGGGGCGCCCCCAACTGCATCCCCTGTAAAGAAACG
TGTGAGAACGTGGACTGTGGACCTGGGAAAAAATGCCGAATGAACAAGAAGAACAAACCCC
GCTGCGTCTGCGCCCCGGATTGTTCCAACATCACCTGGAAGGGTCCAGTCTGCGGGCTGGA
TGGGAAAACCTACCGCAATGAATGTGCACTCCTAAAGGCAAGATGTAAAGAGCAGCCAGAA
CTGGAAGTCCAGTACCAAGGCAGATGTAAAAAGACTTGTCGGGATGTTTTCTGTCCAGGCA
GCTCCACATGTGTGGTGGACCAGACCAATAATGCCTACTGTGTGACCTGTAATCGGATTTG
CCCAGAGCCTGCTTCCTCTGAGCAATATCTCTGTGGGAATGATGGAGTCACCTACTCCAGT
GCCTGCCACCTGAGAAAGGCTACCTGCCTGCTGGGCAGATCTATTGGATTAGCCTATGAGG
GAAAGTGTATCAAAGCAAAGTCCTGTGAAGATATCCAGTGCACTGGTGGGAAAAAATGTTT
ATGGGATTTCAAGGTTGGGAGAGGCCGGTGTTCCCTCTGTGATGAGCTGTGCCCTGACAGT
AAGTCGGATGAGCCTGTCTGTGCCAGTGACAATGCCACTTATGCCAGCGAGTGTGCCATGA
AGGAAGCTGCCTGCTCCTCAGGTGTGCTACTGGAAGTAAAGCACTCCGGATCTTGCAACTC
CATTTCGGAAGACACCGAGGAAGAGGAGGAAGATGAAGACCAGGACTACAGCTTTCCTATA

TCTTCTATTCTAGAGTGGGTCGAGAGGTCCGGCGGCGGAGAGGGCAGAGGAAGTCTTCTAA
CATGCGGTGACGTGGAGGAGAATCCCGGCCCAATGGAAGATGCCAAAAACATTAAGAAGGG
CCCAGCGCCATTCTACCCACTCGAAGACGGGACCGCCGGCGAGCAGCTGCACAAAGCCATG
AAGCGCTACGCCCTGGTGCCCGGCACCATCGCCTTTACCGACGCACATATCGAGGTGGACA
TTACCTACGCCGAGTACTTCGAGATGAGCGTTCGGCTGGCAGAAGCTATGAAGCGCTATGG
GCTGAATACAAACCATCGGATCGTGGTGTGCAGCGAGAATAGCTTGCAGTTCTTCATGCCC
GTGTTGGGTGCCCTGTTCATCGGTGTGGCTGTGGCCCCAGCTAACGACATCTACAACGAGC
GCGAGCTGCTGAACAGCATGGGCATCAGCCAGCCCACCGTCGTATTCGTGAGCAAGAAAGG
GCTGCAAAAGATCCTCAACGTGCAAAAGAAGCTACCGATCATACAAAAGATCATCATCATG
GATAGCAAGACCGACTACCAGGGCTTCCAAAGCATGTACACCTTCGTGACTTCCCATTTGC
CACCCGGCTTCAACGAGTACGACTTCGTGCCCGAGAGCTTCGACCGGGACAAAACCATCGC
CCTGATCATGAACAGTAGTGGCAGTACCGGATTGCCCAAGGGCGTAGCCCTACCGCACCGC
ACCGCTTGTGTCCGATTCAGTCATGCCCGCGACCCCATCTTCGGCAACCAGATCATCCCCG
ACACCGCTATCCTCAGCGTGGTGCCATTTCACCACGGCTTCGGCATGTTCACCACGCTGGG
CTACTTGATCTGCGGCTTTCGGGTCGTGCTCATGTACCGCTTCGAGGAGGAGCTATTCTTG
CGCAGCTTGCAAGACTATAAGATTCAATCTGCCCTGCTGGTGCCCACACTATTTAGCTTCT
TCGCTAAGAGCACTCTCATCGACAAGTACGACCTAAGCAACTTGCACGAGATCGCCAGCGG
CGGGGCGCCGCTCAGCAAGGAGGTAGGTGAGGCCGTGGCCAAACGCTTCCACCTACCAGGC
ATCCGCCAGGGCTACGGCCTGACAGAAACAACCAGCGCCATTCTGATCACCCCCGAAGGGG
ACGACAAGCCTGGCGCAGTAGGCAAGGTGGTGCCCTTCTTCGAGGCTAAGGTGGTGGACTT
GGACACCGGTAAGACACTGGGTGTGAACCAGCGCGGCGAGCTGTGCGTCCGTGGCCCCATG
ATCATGAGCGGCTACGTTAACAACCCCGAGGCTACAAACGCTCTCATCGACAAGGACGGCT
GGCTGCACAGCGGCGACATCGCCTACTGGGACGAGGACGAGCACTTCTTCATCGTGGACCG
GCTGAAGAGCCTGATCAAATACAAGGGCTACCAGGTAGCCCCAGCCGAACTGGAGAGCATC
CTGCTGCAACACCCCAACATCTTCGACGCCGGGGTCGCCGGCCTGCCCGACGACGATGCCG
GCGAGCTGCCCGCCGCAGTCGTCGTGCTGGAACACGGTAAAACCATGACCGAGAAGGAGAT
CGTGGACTATGTGGCCAGCCAGGTTACAACCGCCAAGAAGCTGCGCGGTGGTGTTGTGTTC
GTGGACGAGGTGCCTAAAGGACTGACCGGCAAGTTGGACGCCCGCAAGATCCGCGAGATTC
TCATTAAGGCCAAGAAGGGCGGCAAGATCGCCGTGTAAAATAAAAGATCTTTATTTTCATT
AGATCTGTGTGTTGGTTTTTTGTGTGAGGAACCCCTAGTGATGGAGTTGGCCACTCCCTCT
CTGCGCGCTCGCTCGCTCACTGAGGCCGGGCGACCAAAGGTCGCCCGACGCCCGGGCTTTG
CCCGGGCGGCCTCAGTGAGCGAGCGAGCGCGCAGCTGCCTGCAGGGGCGCCTGATGCGGTA
TTTTCTCCTTACGCATCTGTGCGGTATTTCACACCGCATACGTCAAAGCAACCATAGTACG
CGCCCTGTAGCGGCGCATTAAGCGCGGCGGGTGTGGTGGTTACGCGCAGCGTGACCGCTAC
ACTTGCCAGCGCCCTAGCGCCCGCTCCTTTCGCTTTCTTCCCTTCCTTTCTCGCCACGTTC
GCCGGCTTTCCCCGTCAAGCTCTAAATCGGGGGCTCCCTTTAGGGTTCCGATTTAGTGCTT
TACGGCACCTCGACCCCAAAAAACTTGATTTGGGTGATGGTTCACGTAGTGGGCCATCGCC
CTGATAGACGGTTTTTCGCCCTTTGACGTTGGAGTCCACGTTCTTTAATAGTGGACTCTTG
TTCCAAACTGGAACAACACTCAACCCTATCTCGGGCTATTCTTTTGATTTATAAGGGATTT
TGCCGATTTCGGCCTATTGGTTAAAAAATGAGCTGATTTAACAAAAATTTAACGCGAATTT
TAACAAAATATTAACGTTTACAATTTTATGGTGCACTCTCAGTACAATCTGCTCTGATGCC
GCATAGTTAAGCCAGCCCCGACACCCGCCAACACCCGCTGACGCGCCCTGACGGGCTTGTC
TGCTCCCGGCATCCGCTTACAGACAAGCTGTGACCGTCTCCGGGAGCTGCATGTGTCAGAG
GTTTTCACCGTCATCACCGAAACGCGCGAGACGAAAGGGCCTCGTGATACGCCTATTTTTA
TAGGTTAATGTCATGATAATAATGGTTTCTTAGACGTCAGGTGGCACTTTTCGGGGAAATG
TGCGCGGAACCCCTATTTGTTTATTTTTCTAAATACATTCAAATATGTATCCGCTCATGAG
ACAATAACCCTGATAAATGCTTCAATAATATTGAAAAAGGAAGAGTATGAGTATTCAACAT
TTCCGTGTCGCCCTTATTCCCTTTTTTGCGGCATTTTGCCTTCCTGTTTTTGCTCACCCAG
AAACGCTGGTGAAAGTAAAAGATGCTGAAGATCAGTTGGGTGCACGAGTGGGTTACATCGA

ACTGGATCTCAACAGCGGTAAGATCCTTGAGAGTTTTCGCCCCGAAGAACGTTTTCCAATG
ATGAGCACTTTTAAAGTTCTGCTATGTGGCGCGGTATTATCCCGTATTGACGCCGGGCAAG
AGCAACTCGGTCGCCGCATACACTATTCTCAGAATGACTTGGTTGAGTACTCACCAGTCAC
AGAAAAGCATCTTACGGATGGCATGACAGTAAGAGAATTATGCAGTGCTGCCATAACCATG
AGTGATAACACTGCGGCCAACTTACTTCTGACAACGATCGGAGGACCGAAGGAGCTAACCG
CTTTTTTGCACAACATGGGGGATCATGTAACTCGCCTTGATCGTTGGGAACCGGAGCTGAA
TGAAGCCATACCAAACGACGAGCGTGACACCACGATGCCTGTAGCAATGGCAACAACGTTG
CGCAAACTATTAACTGGCGAACTACTTACTCTAGCTTCCCGGCAACAATTAATAGACTGGA
TGGAGGCGGATAAAGTTGCAGGACCACTTCTGCGCTCGGCCCTTCCGGCTGGCTGGTTTAT
TGCTGATAAATCTGGAGCCGGTGAGCGTGGGTCTCGCGGTATCATTGCAGCACTGGGGCCA
GATGGTAAGCCCTCCCGTATCGTAGTTATCTACACGACGGGGAGTCAGGCAACTATGGATG
AACGAAATAGACAGATCGCTGAGATAGGTGCCTCACTGATTAAGCATTGGTAACTGTCAGA
CCAAGTTTACTCATATATACTTTAGATTGATTTAAAACTTCATTTTTAATTTAAAAGGATC
TAGGTGAAGATCCTTTTTGATAATCTCATGACCAAAATCCCTTAACGTGAGTTTTCGTTCC
ACTGAGCGTCAGACCCCGTAGAAAAGATCAAAGGATCTTCTTGAGATCCTTTTTTTCTGCG
CGTAATCTGCTGCTTGCAAACAAAAAAACCACCGCTACCAGCGGTGGTTTGTTTGCCGGAT
CAAGAGCTACCAACTCTTTTTCCGAAGGTAACTGGCTTCAGCAGAGCGCAGATACCAAATA
CTGTCCTTCTAGTGTAGCCGTAGTTAGGCCACCACTTCAAGAACTCTGTAGCACCGCCTAC
ATACCTCGCTCTGCTAATCCTGTTACCAGTGGCTGCTGCCAGTGGCGATAAGTCGTGTCTT
ACCGGGTTGGACTCAAGACGATAGTTACCGGATAAGGCGCAGCGGTCGGGCTGAACGGGGG
GTTCGTGCACACAGCCCAGCTTGGAGCGAACGACCTACACCGAACTGAGATACCTACAGCG
TGAGCTATGAGAAAGCGCCACGCTTCCCGAAGGGAGAAAGGCGGACAGGTATCCGGTAAGC
GGCAGGGTCGGAACAGGAGAGCGCACGAGGGAGCTTCCAGGGGGAAACGCCTGGTATCTTT
ATAGTCCTGTCGGGTTTCGCCACCTCTGACTTGAGCGTCGATTTTTGTGATGCTCGTCAGG
GGGGCGGAGCCTATGGAAAAACGCCAGCAACGCGGCCTTTTTACGGTTCCTGGCCTTTTGC
TGGCCTTTTGCTCACATGT

FIG. 17

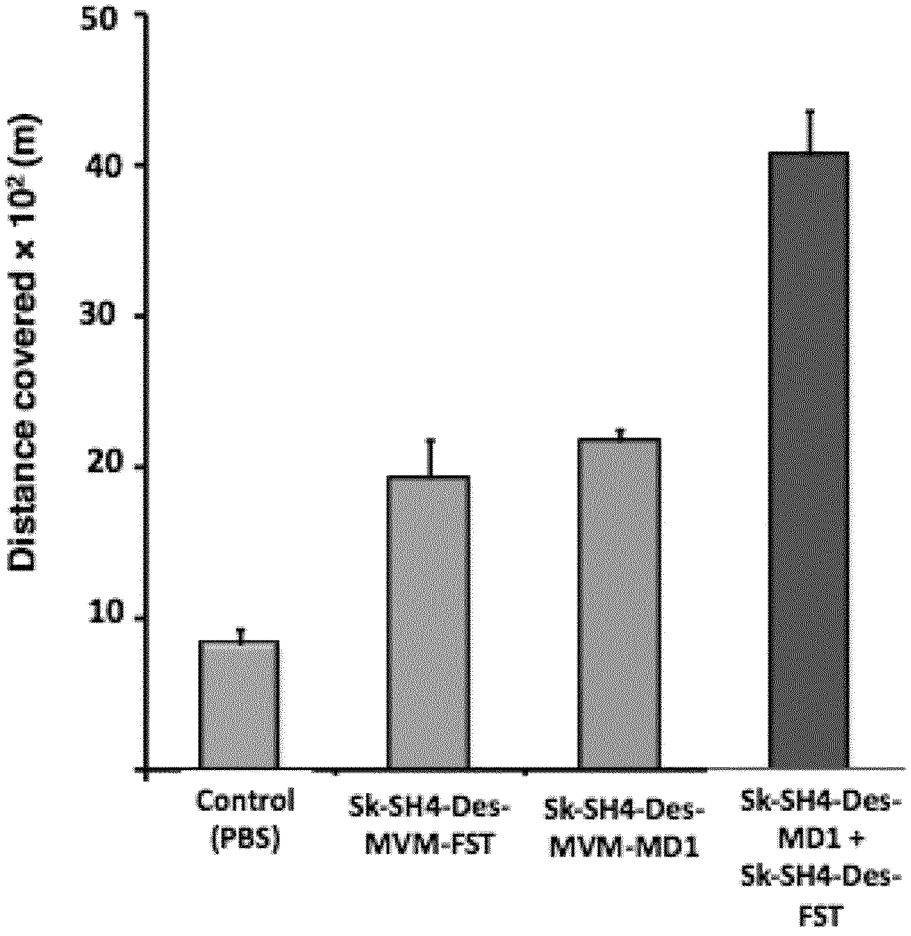
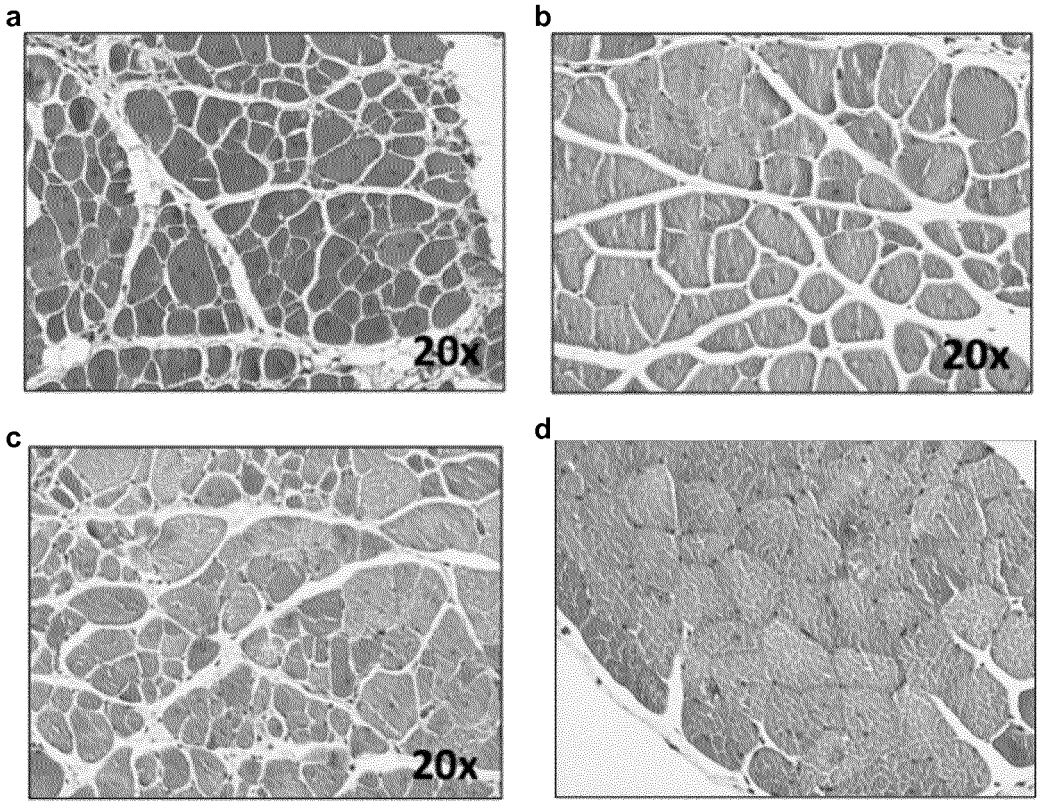

Distance covered x $10^2$ (m)
50
40
30
20
10
Control (PBS)
Sk-SH4-Des-MVM-FST
Sk-SH4-Des-MVM-MD1
Sk-SH4-Des-MD1 + Sk-SH4-Des-FST

FIG. 18

A


a
b
c
d
20x
20x
20x

B

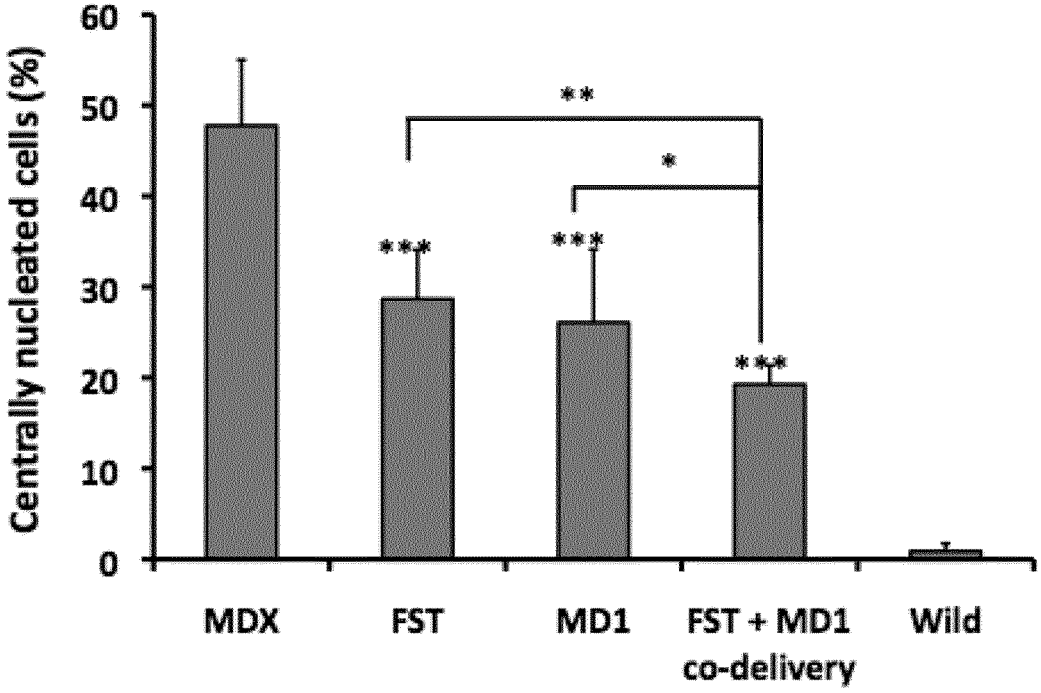
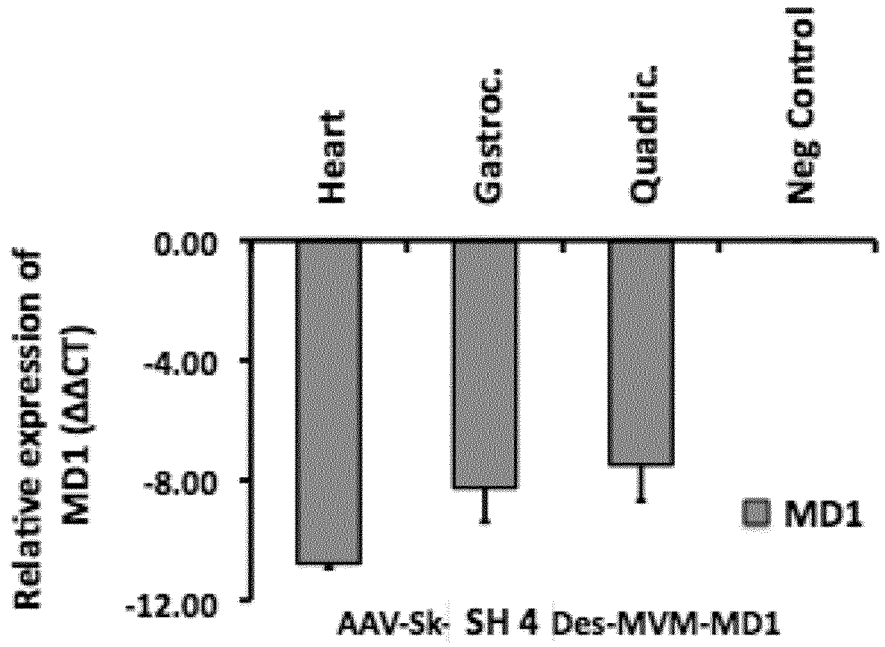

Centrally nucleated cells (%)
60
50
40
30
20
10
0
**
*
***
***
***
MDX
FST
MD1
FST + MD1 co-delivery
Wild

FIG. 19

A


Heart
Gastroc.
Quadric.
Neg Control
0.00
-4.00
-8.00
-12.00
Relative expression of MD1 (ΔΔCT)
MD1
AAV-Sk- SH 4 Des-MVM-MD1

B

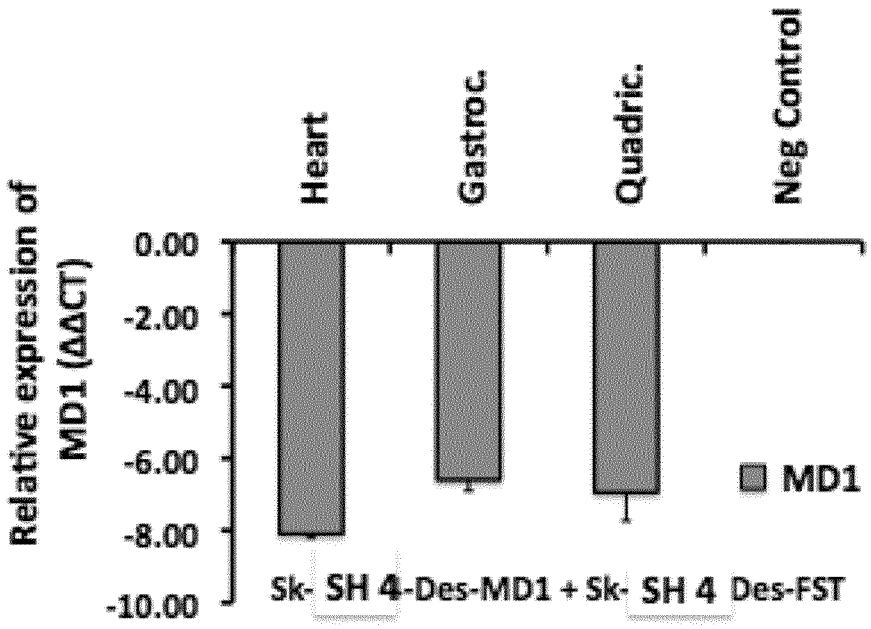

Heart
Gastroc.
Quadric.
Neg Control
0.00
-2.00
-4.00
-6.00
-8.00
-10.00
Relative expression of MD1 (ΔΔCT)
MD1
Sk- SH 4-Des-MD1 + Sk- SH 4 Des-FST

FIG. 19

C

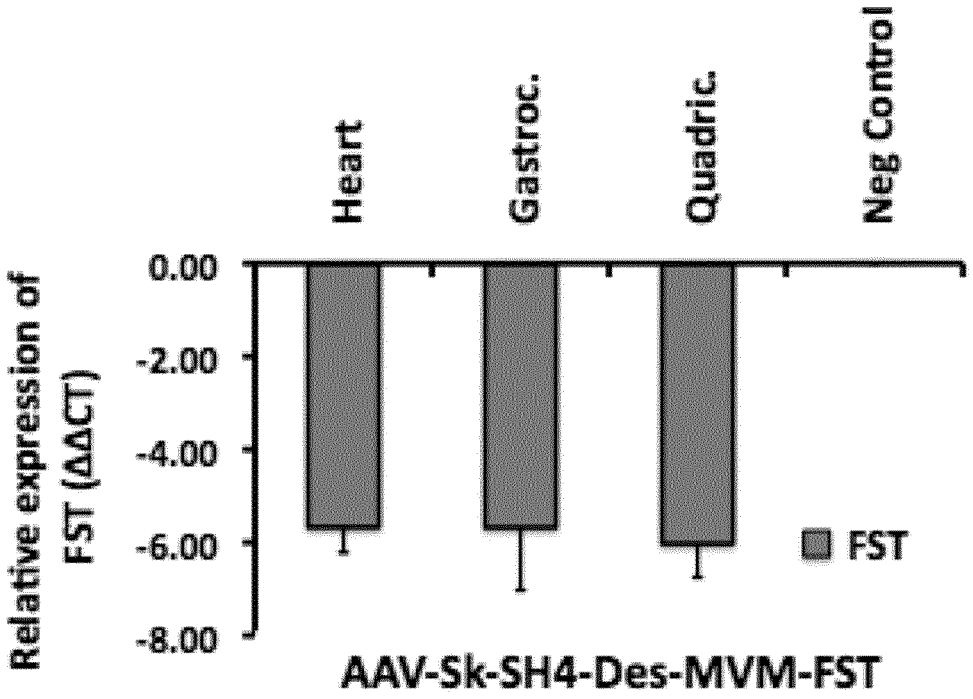

Heart
Gastroc.
Quadric.
Neg Control
0.00
-2.00
-4.00
-6.00
-8.00
Relative expression of FST (ΔΔCT)
FST
AAV-Sk-SH4-Des-MVM-FST

FIG. 20

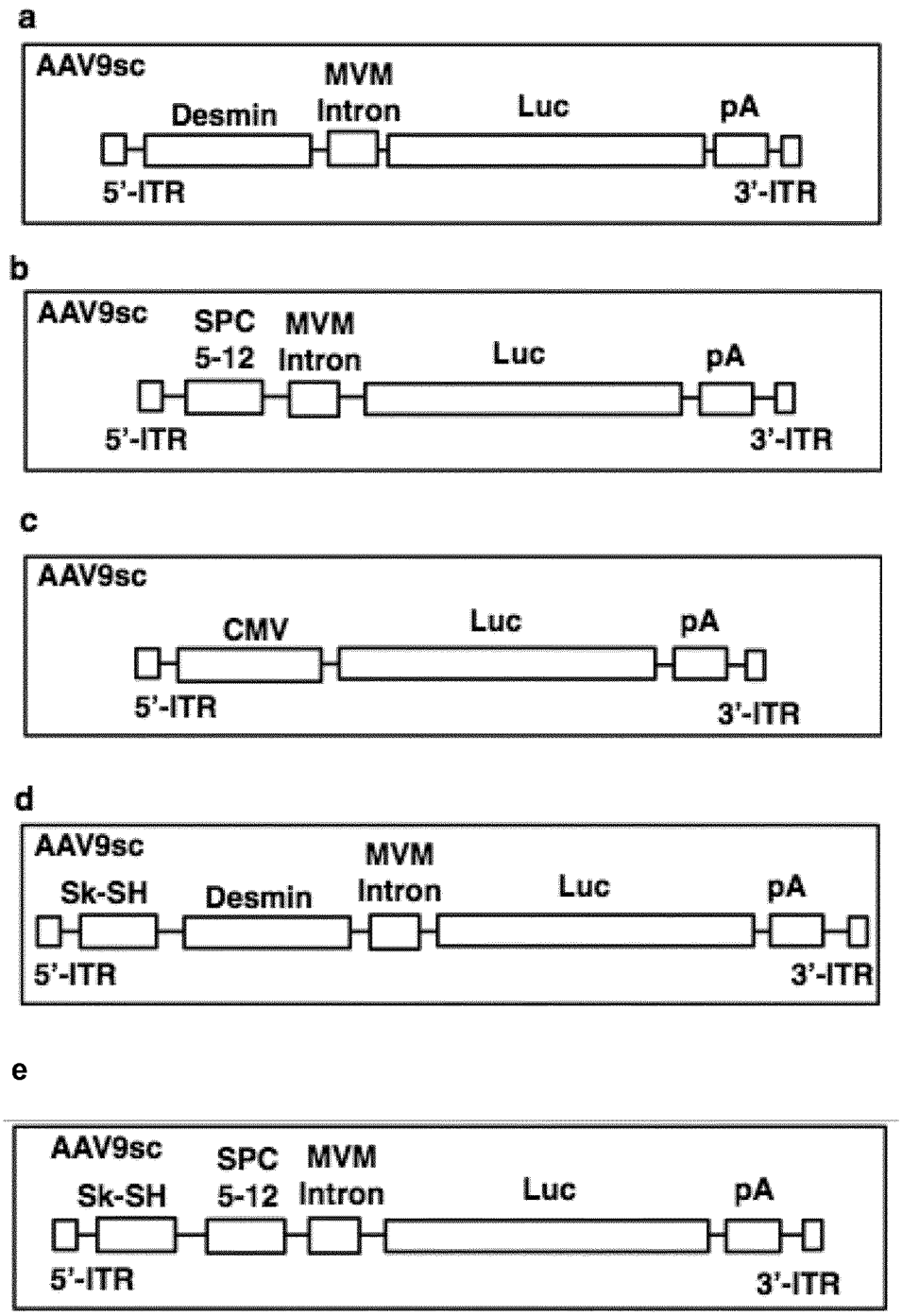
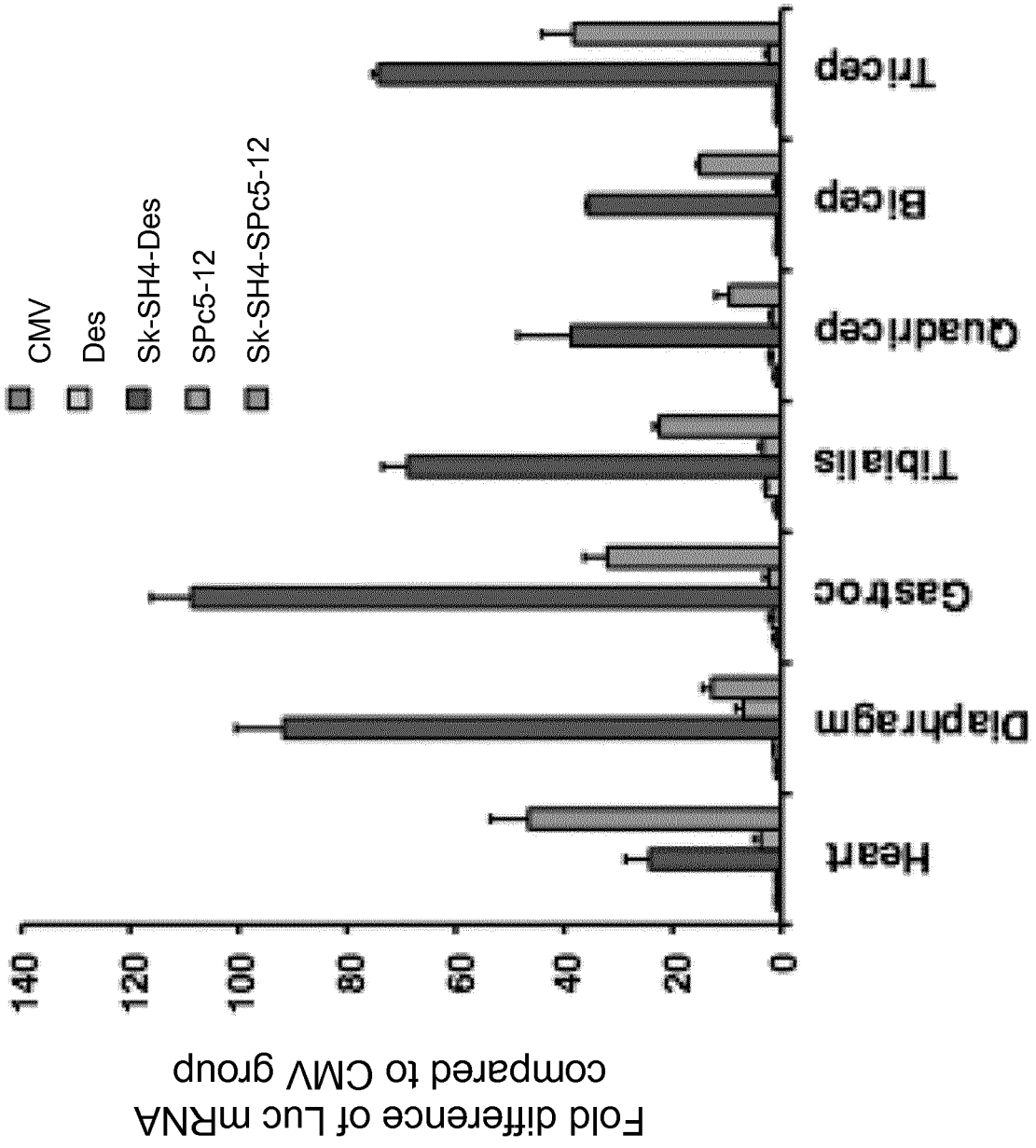

a
AAV9sc
MVM
Intron
Desmin
Luc
pA
5'-ITR
3'-ITR
b
AAV9sc
SPC
5-12
MVM
Intron
Luc
pA
5'-ITR
3'-ITR
c
AAV9sc
CMV
Luc
pA
5'-ITR
3'-ITR
d
AAV9sc
MVM
Intron
Sk-SH
Desmin
Luc
pA
5'-ITR
3'-ITR
e
AAV9sc
SPC
MVM
Sk-SH
5-12
Intron
Luc
pA
5'-ITR
3'-ITR

FIG. 21


Fold difference of Luc mRNA
compared to CMV group
0
20
40
60
80
100
120
140
Heart
Diaphragm
Gastroc
Tibialis
Quadricep
Bicep
Tricep
CMV
Des
Sk-SH4-Des
SPc5-12
Sk-SH4-SPc5-12