Figure 1

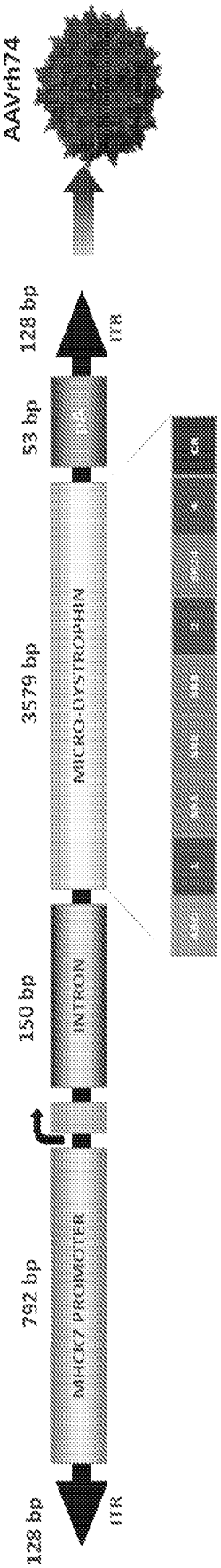

AAVrh74
128 bp
ITR
53 bp
3579 bp
MICRO-DYSTROPHIN
150 bp
INTRON
792 bp
MHCK7 PROMOTER
128 bp
ITR

Figure 2

SEQ ID NO: 3

rAAVrh74.MHCK7.microdystrophin

Main features:

ITR
MHCK7 promoter
Chimeric intron sequence
Human micro-dystrophin sequence
Poly A tail
Ampicillin resistance

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Figure 3

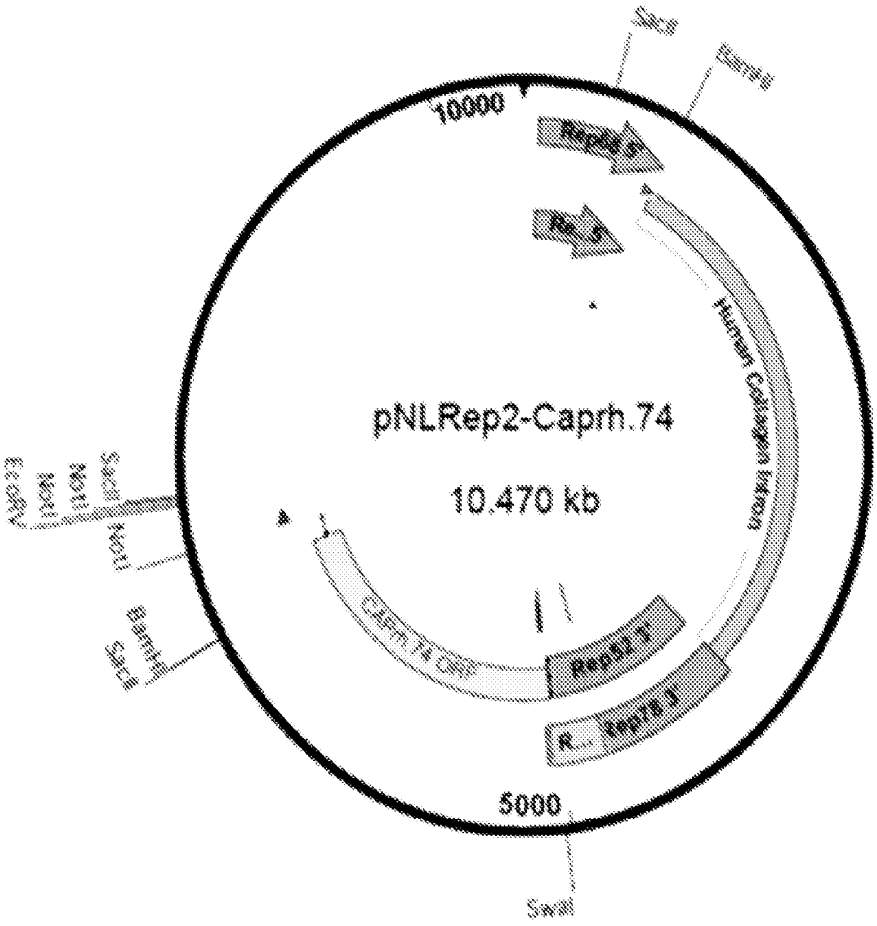

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Figure 4

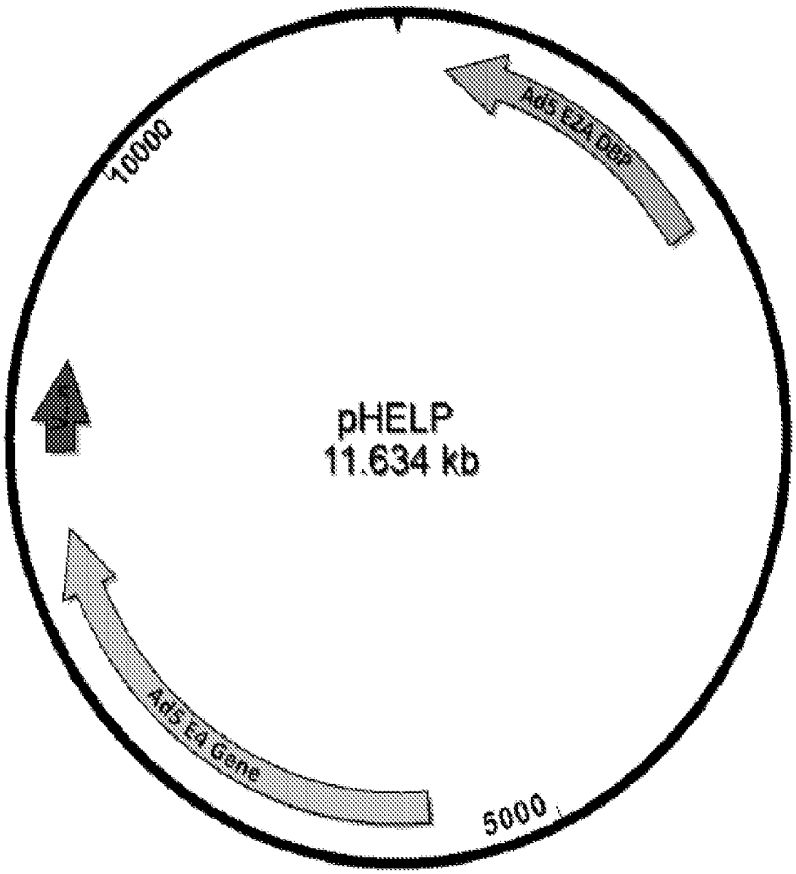


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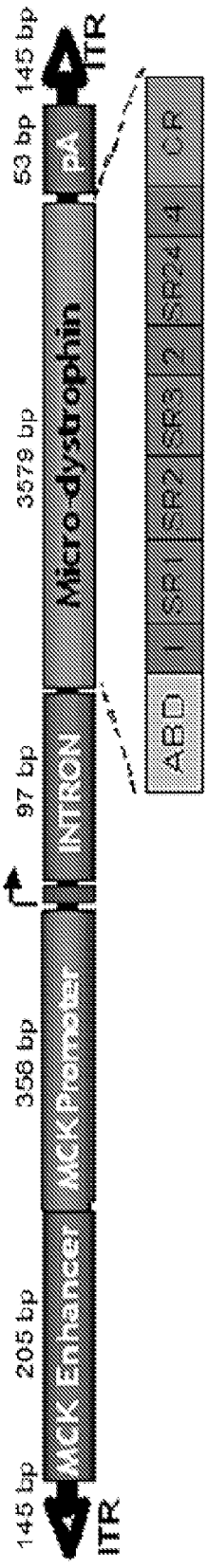

145 bp
ITR
53 bp
pA
3579 bp
Micro-dystrophin
97 bp
INTRON
356 bp
MCK Promoter
205 bp
MCK Enhancer
145 bp
ITR
ABD
1
SR1
SR2
SR3
2
SR4
4
CR

Figure 6

SEQ ID NO: 5

rAAVrh74.MCK.micro-dystrophin

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

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Figure 7

SEQ ID NO: 9
rAAVrh74.MHCK7.microdystrophin

Main Features:
ITR
MHCK7 promoter
Chimeric Intron sequence
Human micro-dystrophin sequence
Poly A tail

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Figure 8

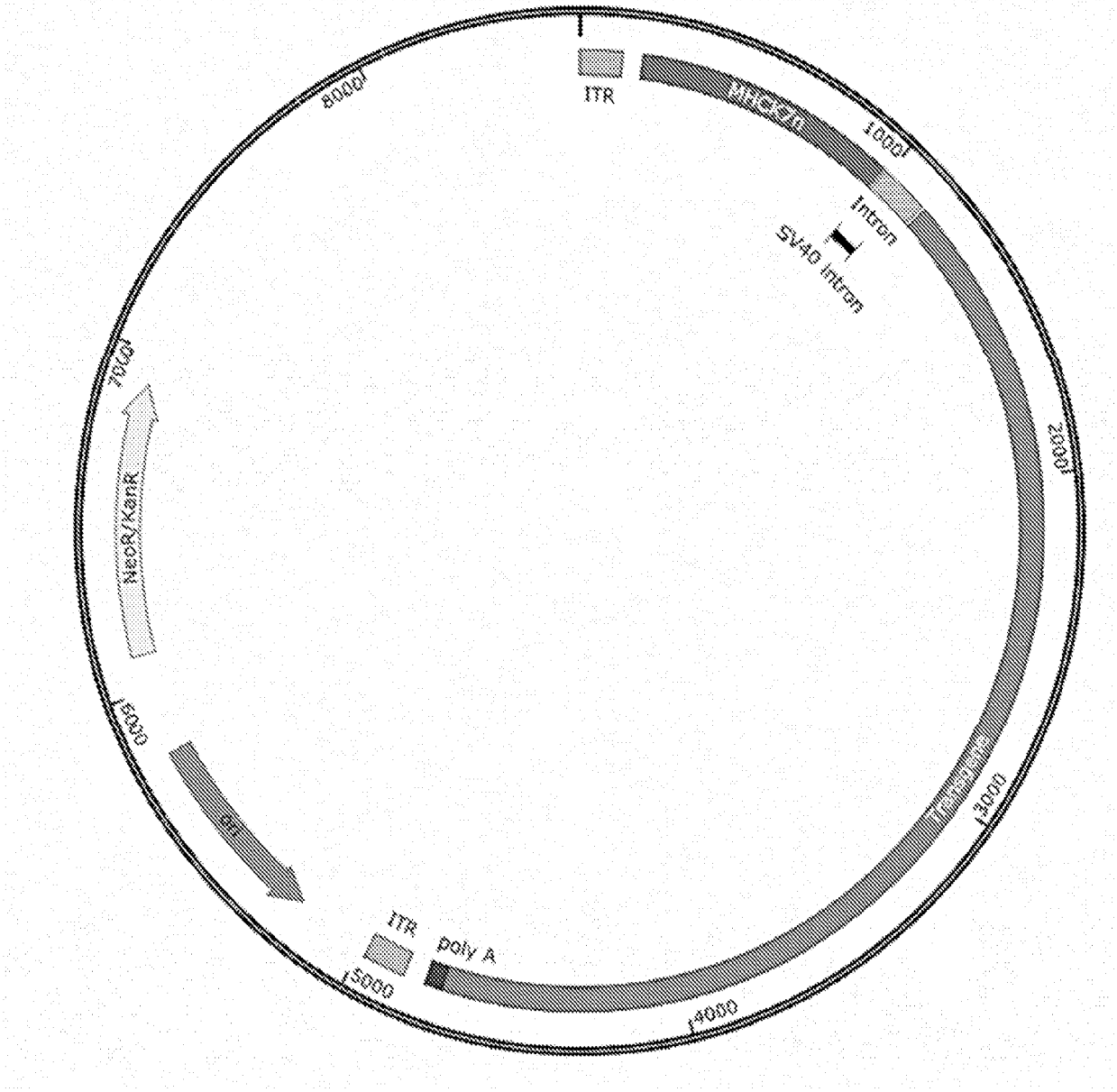

ITR
1000
Intron
SV40 intron
2000
3000
4000
poly A
ITR
5000
6000
AR
7000
NeoR/KanR
8000

Figure 9

SEQ ID NO: 8

Main Features:
ITR
MHCK7 promoter
Chimeric Intron sequence
Human micro-dystrophin sequence
Poly A tail
Kanamycin resistance

GCGCGCTCGCTCGCTCACTGAGGCCGCCCGGGCAAAGCCCGGGCGTCGGGCG
ACCTTTGGTCGCCCGGCCTCAGTGAGCGAGCGAGCGCGCAGAGAGGGAGTGG
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tcttcgctattacgccagct

Figure 10

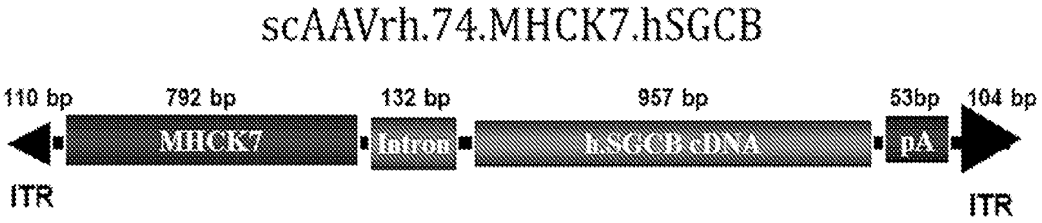

scAAVrh.74.MHCK7.hSGCB
110 bp
792 bp
132 bp
957 bp
53bp
104 bp
MHCK7
Intron
hSGCB cDNA
pA
ITR
ITR

Figure 11

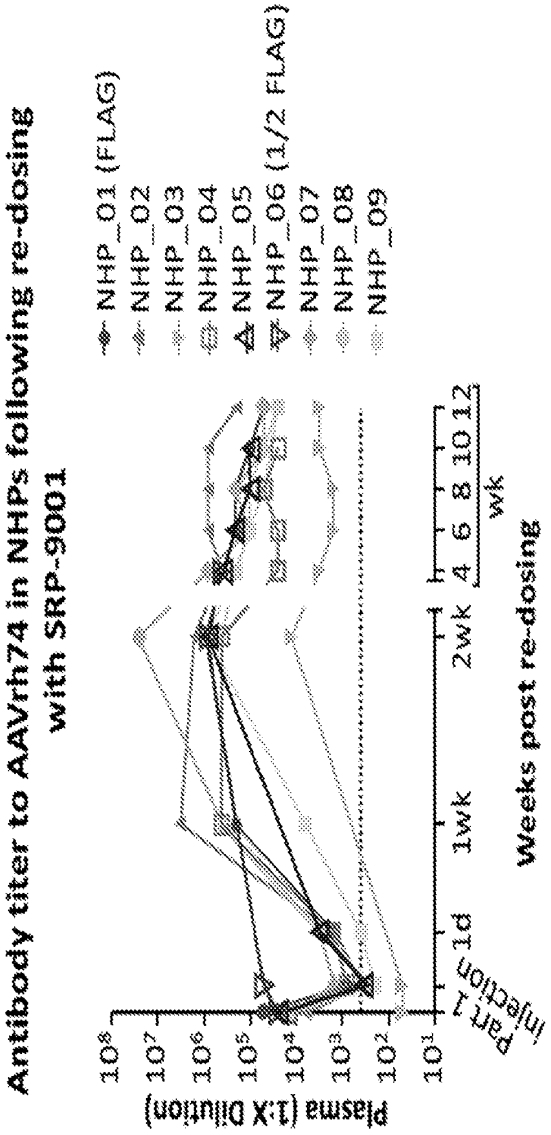

Antibody titer to AAVrh74 in NHPs following re-dosing with SRP-9001
NHP_01 (FLAG)
NHP_02
NHP_03
NHP_04
NHP_05
NHP_06 (1/2 FLAG)
NHP_07
NHP_08
NHP_09
Plasma (1:X Dilution)
Part 1 injection
1d
1wk
2wk
4 6 8 10 12
wk
Weeks post re-dosing

Figure 12

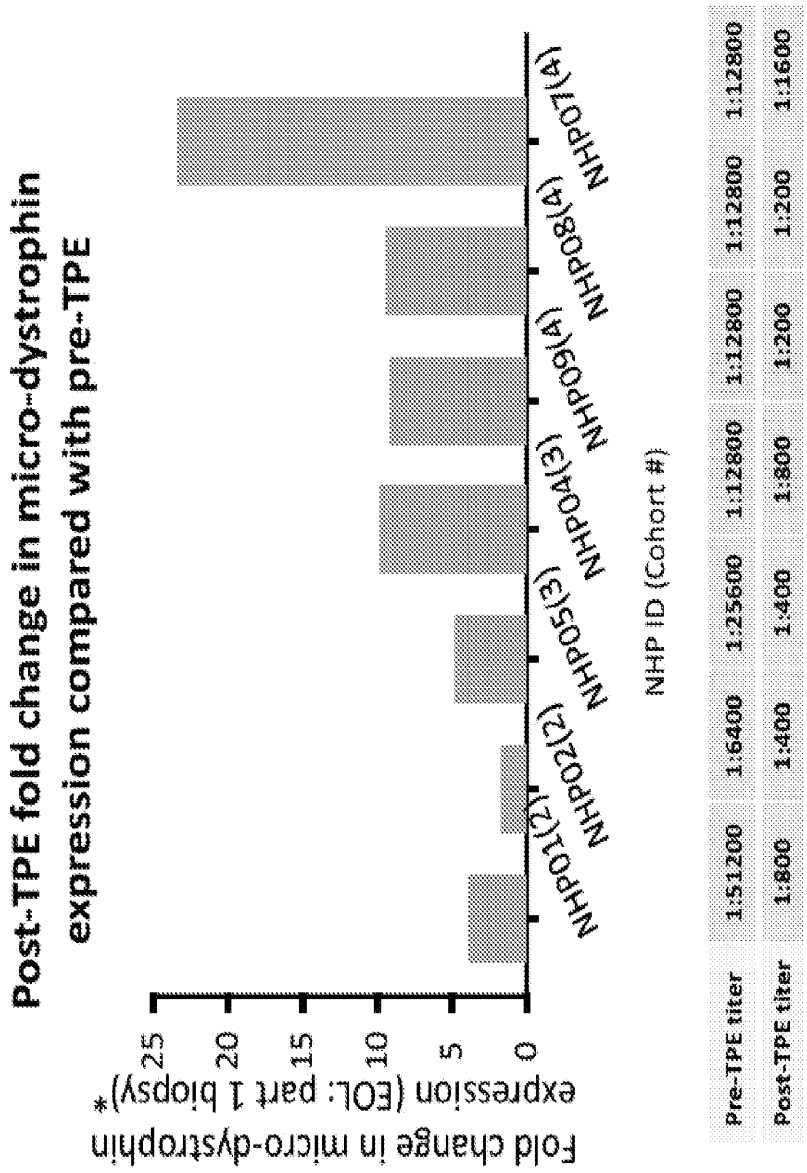
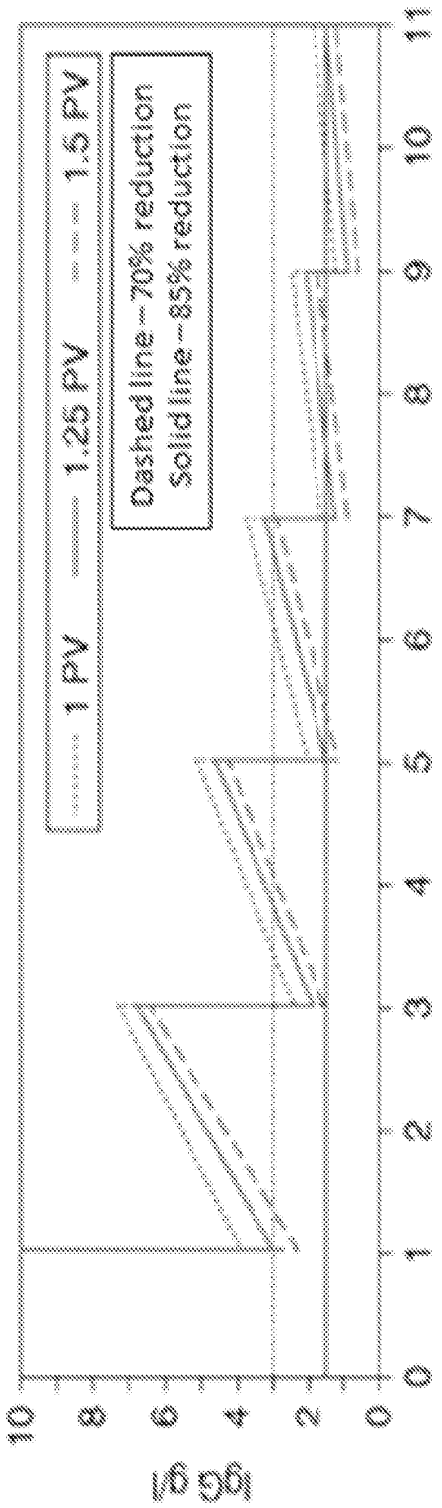

Post-TPE fold change in micro-dystrophin expression compared with pre-TPE
Fold change in micro-dystrophin expression (EOL: part 1 biopsy)*
0
5
10
15
20
25
NHP01(2)
NHP02(2)
NHP05(3)
NHP04(3)
NHP09(4)
NHP08(4)
NHP07(4)
NHP ID (Cohort #)
Pre-TPE titer 1:51200 1:6400 1:25600 1:12800 1:12800 1:12800 1:12800
Post-TPE titer 1:800 1:400 1:400 1:800 1:200 1:200 1:1600

Figure 13



Source: Shaz BH, Transfusion Medicine and Hemostatis, Chapter 68 – Therapeutic Plasma Exchange, San Diego 2009