SEQUENCE LISTING

<110> Ospedale San Raffaele S.r.l
Fondazione Telethon

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<141> 2019-10-11

<150> EP 18199952.5
<151> 2018-10-11

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ggctgttgcc ggtgctgtgc caggaccatg gcctgactcc ggaccaagtg gtggctatcg 660

ccagccacga tggcggcaag caagcgctcg aaacggtgca gcggctgttg ccggtgctgt 720

gccaggacca tggcctgacc ccggaccaag tggtggctat cgccagcaac ggtggcggca 780

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ctccggacca agtggtggct atcgccagcc acgatggcgg caagcaagcg ctcgaaacgg 900

tgcagcggct gttgccggtg ctgtgccagg accatggcct gaccccggac caagtggtgg 960

ctatcgccag caacggtggc ggcaagcaag cgctcgaaac ggtgcagcgg ctgttgccgg 1020

tgctgtgcca ggaccatggc ctgactccgg accaagtggt ggctatcgcc agccacgatg 1080

gcggcaagca agcgctcgaa acggtgcagc ggctgttgcc ggtgctgtgc caggaccatg 1140

gcctgacccc ggaccaagtg gtggctatcg ccagcaacgg tggcggcaag caagcgctcg 1200

aaacggtgca gcggctgttg ccggtgctgt gccaggacca tggcctgacc ccggaccaag 1260

tggtggctat cgccagcaat cacggcggca agcaagcgct cgaaacggtg cagcggctgt 1320

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cgctcgaaac ggtgcagcgg ctgttgccgg tgctgtgcca ggaccatggc ctgaccccgg 1560

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ccccggacca agtggtggct atcgccagca acattggcgg caagcaagcg ctcgaaacgg 1920

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gtctgggagg cagacctgcc ctggatgccg tgaaaaaagg actgcctcac gcccctgccc 2460

tgatcaagag aacaaataga agaatccccg agcggacctc tcacagagtg gccggatcac 2520

a 2521

<210> 49
<211> 2391
<212> DNA
<213> Artificial Sequence

<220>
<223> ETT TALE3 DBD (SMArTER)

<400> 49
gtggacctga gaacactggg atattctcag cagcagcagg agaagatcaa gcccaaggtg 60

agatctacag tggcccagca ccacgaagcc ctggtgggac acggatttac acacgcccac 120

attgtggccc tgtctcagca ccctgccgcc ctgggaacag tggccgtgaa atatcaggat 180

atgattgccg ccctgcctga ggccacacac gaagccattg tgggagtggg aaaacagtgg 240

tctggagcca gagccctgga agccctgctg acagtggccg gagaactgag aggacctcct 300

ctgcagctgg atacaggaca gctgctgaag attgccaaaa ggggcggagt gaccgcggtg 360

gaagccgtgc acgcctggag aaatgccctg acgggtgccc ccctgaacct gaccccggac 420

caagtggtgg ctatcgccag caacaatggc ggcaagcaag cgctcgaaac ggtgcagcgg 480

ctgttgccgg tgctgtgcca ggaccatggc ctgaccccgg accaagtggt ggctatcgcc 540

agcaacggtg gcggcaagca agcgctcgaa acggtgcagc ggctgttgcc ggtgctgtgc 600

caggaccatg gcctgactcc ggaccaagtg gtggctatcg ccagccacga tggcggcaag 660

caagcgctcg aaacggtgca gcggctgttg ccggtgctgt gccaggacca tggcctgacc 720

ccggaccaag tggtggctat cgccagcaac attggcggca agcaagcgct cgaaacggtg 780

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ctgtgccagg accatggcct gaccccggac caagtggtgg ctatcgccag caacattggc 960

ggcaagcaag cgctcgaaac ggtgcagcgg ctgttgccgg tgctgtgcca ggaccatggc 1020

ctgactccgg accaagtggt ggctatcgcc agccacgatg gcggcaagca agcgctcgaa 1080

acggtgcagc ggctgttgcc ggtgctgtgc caggaccatg gcctgacccc ggaccaagtg 1140

gtggctatcg ccagcaacat tggcggcaag caagcgctcg aaacggtgca gcggctgttg 1200

ccggtgctgt gccaggacca tggcctgacc ccggaccaag tggtggctat cgccagcaat 1260

cacggcggca agcaagcgct cgaaacggtg cagcggctgt tgccggtgct gtgccaggac 1320

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ctcgaaacgg tgcagcggct gttgccggtg ctgtgccagg accatggcct gaccccggac 1440

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ctgttgccgg tgctgtgcca ggaccatggc ctgactccgg accaagtggt ggctatcgcc 1560

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atcgccagca acattggcgg caagcaagcg ctcgaaacgg tgcagcggct gttgccggtg 1920

ctgtgccagg accatggcct gaccccggac caagtggtgg ctatcgccag caacggtggc 1980

ggcaagcaag cgctcgaaac ggtgcagcgg ctgttgccgg tgctgtgcca ggaccatggc 2040

ctgaccccgg accaagtggt ggctatcgcc agcaacggtg gcggcaagca agcgctcgaa 2100

acggtgcagc ggctgttgcc ggtgctgtgc caggaccatg gcctgacccc ggaccaagtg 2160

gtggctatcg ccagcaacgg tggcggcaag caagcgctcg aaagcattgt ggcccagctg 2220

agccggcctg atccggcgtt ggccgcgttg accaacgatc acctggtggc cctggcctgt 2280

ctgggaggca gacctgccct ggatgccgtg aaaaaaggac tgcctcacgc ccctgccctg 2340

atcaagagaa caaatagaag aatccccgag cggacctctc acagagtggc c 2391

<210> 50
<211> 801
<212> DNA
<213> Artificial Sequence

<220>
<223> Left homology arm of AAVS1-targeting constructs

<400> 50
ccactgtggg gtggagggga cagataaaag tacccagaac cagagccaca ttaaccggcc 60

ctgggaatat aaggtggtcc cagctcgggg acacaggatc cctggaggca gcaaacatgc 120

tgtcctgaag tggacatagg ggcccgggtt ggaggaagaa gactagctga gctctcggac 180

ccctggaaga tgccatgaca gggggctgga agagctagca cagactagag aggtaagggg 240

ggtaggggag ctgcccaaat gaaaggagtg agaggtgacc cgaatccaca ggagaacggg 300

gtgtccaggc aaagaaagca agaggatgga gaggtggcta aagccaggga gacggggtac 360

tttggggttg tccagaaaaa cggtgatgat gcaggcctac aagaagggga ggcgggacgc 420

aagggagaca tccgtcggag aaggccatcc taagaaacga gagatggcac aggccccaga 480

aggagaagga aaagggaacc cagcgagtga agacggcatg gggttgggtg agggaggaga 540

gatgcccgga gaggacccag acacggggag gatccgctca gaggacatca cgtggtgcag 600

cgccgagaag gaagtgctcc ggaaagagca tccttgggca gcaacacagc agagagcaag 660

gggaagaggg agtggaggaa gacggaacct gaaggaggcg gcagggaagg atctgggcca 720

gccgtagagg tgacccaggc cacaagctgc agacagaaag cggcacaggc ccaggggaga 780

gaatgctggt cagagaaagc a 801

<210> 51
<211> 256
<212> DNA
<213> Artificial Sequence

<220>
<223> SV40polyA of AAVS1-targeting constructs

<400> 51
gagatccaga catgataaga tacattgatg agtttggaca aaccaaaact agaatgcagt 60

gaaaaaaatg ccttatttgt gaaatttgtg atgctattgc cttatttgta accattataa 120

gctgcaataa acaagttaac aacaacaatt gcattcattt tatgtttcag gttcaggggg 180

aggtgtggga ggtttttaa agcaagtaaa acctctacaa atgtggtatg gctgattatg 240

atcagatctc tcgagg 256

<210> 52
<211> 147
<212> DNA
<213> Artificial Sequence

<220>
<223> Shortened left homology arm of AAVS1-targeting constructs

<400> 52

gcaaggagag agatggctcc aggaaatggg ggtgtgtcac cagataagga atctgcctaa 60

caggaggtgg gggttagacc caatatcagg agactaggaa ggaggaggcc taaggatggg 120

gcttttctgt caccaatcct gtcccta 147

<210> 53
<211> 290
<212> DNA
<213> Artificial Sequence

<220>
<223> Left homology arm of IL2RG-targeting constructs

<400> 53
agaggaaacg tgtgggtggg gaggggtagt gggtgaggga cccaggttcc tgacacagac 60

agactacacc cagggaatga agagcaagcg ccatgttgaa gccatcatta ccattcacat 120

ccctcttatt cctgcagctg cccctgctgg gagtggggct gaacacgaca attctgacgc 180

ccaatgggaa tgaagacacc acagctggtg ggaaatctgg gactggaggg ggctggtgag 240

aagggtggct gtgggaaggg gccgtacaga gatctggtgc ctgccactgg 290

<210> 54
<211> 1076
<212> DNA
<213> Artificial Sequence

<220>
<223> IL2RG recoded corrective cDNA (for intron 1 gene correction strategy) of IL2RG-targeting constructs

<400> 54
atttctttct gaccaccatg cccaccgaca gcctgagcgt gagcaccctg cccctgcccg 60

aggtgcagtg cttcgtgttc aacgtggagt acatgaactg cacctggaac agcagcagcg 120

agccccagcc caccaatctg accctgcact actggtacaa gaacagcgac aacgacaagg 180

tgcagaagtg cagccactac ctgttcagcg aggaaatcac cagcggctgc cagctgcaga 240

agaaagagat ccacctgtac cagaccttcg tggtgcagct gcaggacccc cgggagcccc 300

gcaggcaggc cacccagatg ctgaagctgc agaacctggt gatcccctgg gcccctgaga 360

acctgacact gcacaagctg tccgagagcc agctggaact gaactggaac aaccgcttcc 420

tgaaccactg cctggaacac ctggtgcagt accggaccga ctgggaccac agctggaccg 480

agcagagcgt ggactaccgg cacaagttca gcctgcccag cgtggacggc cagaagcggt 540

acaccttcag agtgcggagc cggttcaacc ccctgtgcgg cagcgcccag cactggtccg 600

agtggagcca ccccatccac tggggcagca acaccagcaa agagaacccc ttcctgttcg 660

ccctggaagc cgtggtgatc agcgtgggca gcatgggcct gatcatctcc ctgctgtgcg 720

tgtacttctg gctggaacgg accatgccca gaatccccac cctgaagaac ctggaagatc 780

tggtgaccga gtaccacggc aacttcagcg cctggtccgg cgtgagcaag ggcctggccg 840

agagcctgca gcccgactac agcgagcggc tgtgcctggt gtccgagatc ccccccaaag 900

gcggagccct gggcgaaggc cctggcgcca gcccctgcaa ccagcacagc ccctactggg 960

cccctccttg ctacaccctg aagcccgaga cccgggccaa gcgatccgga tccggagcca 1020

ccaacttcag cctgctgaag caggccggcg acgtggagga gaaccccggc ccctga 1076

<210> 55
<211> 81
<212> DNA
<213> Artificial Sequence

<220>
<223> Furin site + self-cleaving 2A peptide of IL2RG-targeting constructs

<400> 55
cgggccaagc gatccggatc cggagccacc aacttcagcc tgctgaagca ggccggcgac 60

gtggaggaga accccggccc c 81

<210> 56
<211> 267
<212> DNA
<213> Artificial Sequence

<220>
<223> Right homology arm of IL2RG-targeting constructs

<400> 56
tacaatcatg tgggcagaat tgaaaagtgg agtgggaagg gcaaggggga gggttccctg 60

cctcacgcta cttcttcttt ctttcttgtt tgtttgtttc tttctttctt ttgaggcagg 120

gtctcactat gttgcctagg ctggtctcaa actcctggcc tctagtgatc ctcctgcctc 180

agcctttcaa agcaccagga ttacagacat gagccaccgt gcttggcctc ctccttctga 240

ccatcatttc tctttccctc cctgcct 267

<210> 57
<211> 516
<212> DNA
<213> Artificial Sequence

<220>
<223> PGK promoter of IL2RG-targeting constructs

<400> 57
ccacggggtt ggggttgcgc cttttccaag gcagccctgg gtttgcgcag ggacgcggct 60

gctctgggcg tggttccggg aaacgcagcg gcgccgaccc tgggtctcgc acattcttca 120

cgtccgttcg cagcgtcacc cggatcttcg ccgctaccct tgtgggcccc ccggcgacgc 180

ttcctgctcc gcccctaagt cgggaaggtt ccttgcggtt cgcggcgtgc cggacgtgac 240

aaacggaagc cgcacgtctc actagtaccc tcgcagacgg acagcgccag ggagcaatgg 300

cagcgcgccg accgcgatgg gctgtggcca atagcggctg ctcagcgggg cgcgccgaga 360

gcagcggccg ggaaggggcg gtgcgggagg cggggtgtgg ggcggtagtg tgggccctgt 420

tcctgcccgc gcggtgttcc gcattctgca agcctccgga gcgcacgtcg gcagtcggct 480

ccctcgttga ccgaatcacc gacctctctc cccagg 516

<210> 58
<211> 1041
<212> DNA
<213> Artificial Sequence

<220>
<223> tTA and 3' UTR

<400> 58
atgtctagac tggacaagag caaagtcata aactctgctc tggaattact caatgaagtc 60

ggtatcgaag gcctgacgac aaggaaactc gctcaaaagc tgggagttga gcagcctacc 120

ctgtactggc acgtgaagaa caagcgggcc ctgctcgatg ccctggcaat cgagatgctg 180

gacaggcatc atacccactt ctgccccctg gaaggcgagt catggcaaga ctttctgcgg 240

aacaacgcca agtcattccg ctgtgctctc ctctcacatc gcgacggggc taaagtgcat 300

ctcggcaccc gcccaacaga gaaacagtac gaaaccctgg aaaatcagct cgcgttcctg 360

tgtcagcaag gcttctccct ggagaacgca ctgtacgctc tgtccgccgt gggccacttt 420

acactgggct gcgtattgga ggatcaggag catcaagtag caaaagagga aagagagaca 480

cctaccaccg attctatgcc cccacttctg agacaagcaa ttgagctgtt cgaccatcag 540

ggagccgaac ctgccttcct tttcggcctg gaactaatca tatgtggcct ggagaaacag 600

ctaaagtgcg aaagcggcgg gccggccgac gcccttgacg attttgactt agacatgctc 660

ccagccgatg cccttgacga ctttgacctt gatatgctgc ctgctgacgc tcttgacgat 720

tttgaccttg acatgctccc cgggtaaaag ctcgctttct tgctgtccaa tttctattaa 780

aggttccttt gttccctaag tccaactact aaactggggg atattatgaa gggccttgag 840

catctggatt ctgcctaata aaaaacattt attttcattg ctgcgctaga agctcgcttt 900

cttgctgtcc aatttctatt aaaggttcct ttgttcccta agtccaacta ctaaactggg 960

ggatattatg aagggccttg agcatctgga ttctgcctaa taaaaaacat ttattttcat 1020

tgctgcggga cattcttaat t 1041

<210> 59
<211> 19
<212> DNA
<213> Artificial Sequence

<220>
<223> Tet operon (TetO) sequence

<400> 59
tccctatcag tgatagaga 19

<210> 60
<211> 16
<212> DNA
<213> Artificial Sequence

<220>
<223> spacer sequence

<400> 60
acgatgtcga gtttac 16

<210> 61
<211> 796
<212> DNA
<213> Artificial Sequence

<220>
<223> Left homology arm of AAV6 AAVS1

<400> 61
tgctttctct gaccagcatt ctctcccctg ggcctgtgcc gctttctgtc tgcagcttgt 60

ggcctgggtc acctctacgg ctggcccaga tccttccctg ccgcctcctt caggttccgt 120

cttcctccac tccctcttcc ccttgctctc tgctgtgttg ctgcccaagg atgctctttc 180

cggagcactt ccttctcggc gctgcaccac gtgatgtcct ctgagcggat cctccccgtg 240

tctgggtcct ctccgggcat ctctcctccc tcacccaacc ccatgccgtc ttcactcgct 300

gggttccctt ttccttctcc ttctggggcc tgtgccatct ctcgtttctt aggatggcct 360

tctccgacgg atgtctccct tgcgtcccgc ctccccttct tgtaggcctg catcatcacc 420

gtttttctgg acaaccccaa agtaccccgt ctccctggct ttagccacct ctccatcctc 480

ttgctttctt tgcctggaca ccccgttctc ctgtggattc gggtcacctc tcactccttt 540

catttgggca gctcccctac ccccttacc tctctagtct gtgctagctc ttccagcccc 600

ctgtcatggc atcttccagg ggtccgagag ctcagctagt cttcttcctc caacccgggc 660

ccctatgtcc acttcaggac agcatgtttg ctgcctccag ggatcctgtg tccccgagct 720

gggaccacct tatattccca gggccggtta atgtggctct ggttctgggt acttttatct 780

gtcccctcca ccccac 796

<210> 62
<211> 258
<212> DNA
<213> Artificial Sequence

<220>
<223> Right homology arm of AAV6 AAVS1

<400> 62
tagggacagg attggtgaca gaaaagcccc atccttaggc ctcctccttc ctagtctcct 60

gatattgggt ctaaccccca cctcctgtta ggcagattcc ttatctggtg acacaccccc 120

atttcctgga gccatctctc tccttgccag aacctctaag gtttgcttac gatggagcca 180

gagaggatcc tgggagggag agcttggcag ggggtgggag ggaaggggg gatgcgtgac 240

ctgcccggtt ctcagtgg 258

<210> 63
<211> 496
<212> DNA
<213> Artificial Sequence

<220>
<223> Left homology arm of AAV6 CD40LG

<400> 63
tgctttaaaa gtaagctatt tttttatgga gacagctttt ttcttttaaa tttccagcta 60

ggcaagaaga gcgtcaattt gatctaaaat ttcataatgc ttcagattaa catagacatg 120

gataagtccc agaatttgca gtcttttagt aaaagtagca ttttctgtgt aattcttcac 180

aagcactgat tgtagttgca ggatgctcag tctccctctg agatgtttta catttttaaa 240

tggttagact tgcaggaaca aaagagcaga gtaacttagt aggctgtttt gcattcttag 300

gaaaagaaaa ccatcaggac ttattttgtt ttcatgtatt ttttcacttc cactgaggag 360

tataattggc tggtgttgac aaaataccaa tcatagatgt aaaggagaaa gttgattagt 420

tttctggctg ttcctaaaat tctggatgca ggaactgtgg ctagaaagca tctggatgat 480

tgcactttac cttagg 496

<210> 64
<211> 499
<212> DNA
<213> Artificial Sequence

<220>
<223> Right homology arm of AAV6 CD40LG

<400> 64
cagggatact tgagtgtcct ctcttaggat ctggacctag aattaatgtc atgagatttt 60

tctaacagga taaggtgagg tagtgagggc tgaagtcatc cactgggtta tccaaatatt 120

aggtttcact gctgacaaaa gagggggctt ctggtctggt tggttatttg tgtttggcct 180

gatgtgctct gtcaatcaaa tgtatggaca taggcctagc ttctaaaggg gcaatagtga 240

cctcagtgga ctgatattta ccgtactatt tacatgtgct cttaattaca gcagaagctg 300

ccagctaact gaatcttgtt ttgaatctaa aaaatctact cttaaagcaa gaaaatggta 360

taaaattagt tgataatgca agtgaattct gtacatttaa ttattctaag acattggaaa 420

ataaaatatc ttgttacttt gaggataaaa gatgatttct ttaaaaatgc aaatgttttc 480

tacaaatact aaagttaaa 499

<210> 65
<211> 371
<212> DNA
<213> Artificial Sequence

<220>
<223> TETO7-SK sequence

<400> 65
ccctatcagt gatagagaac gtatgaagag tttactccct atcagtgata gagaacgtat 60

gcagacttta ctccctatca gtgatagaga acgtataagg agtttactcc ctatcagtga 120

tagagaacgt atgaccagtt tactccctat cagtgataga gaacgtatct acagtttact 180

ccctatcagt gatagagaac gtatatccag tttactccct atcagtgata gagaacgtat 240

aagctttagg cgtgtacggt gggcgcctat aaaagcagag ctcgtttagt gaaccgtcag 300

atcgcctgga gcaattccac aacacttttg tcttatacca actttccgta ccacttccta 360

ccctcgtaaa g 371

<210> 66
<211> 18
<212> DNA
<213> Artificial Sequence

<220>
<223> Tet operon (TetO) sequence

<400> 66
ccctatcagt gatagaga 18

<210> 67
<211> 843
<212> DNA
<213> Artificial Sequence

<220>
<223> delta LNGFR codon optimized for human expression

<400> 67
atgggagctg gtgctaccgg cagagctatg gatggaccta gactgctgct cctgctgctg 60

ctcggagttt ctcttggcgg agccaaagag gcctgtccta ccggcctgta tacacactct 120

ggcgagtgct gcaaggcctg caatcttgga gaaggcgtgg cacagccttg cggcgctaat 180

cagacagtgt gcgagccttg cctggacagc gtgaccttta gcgacgtggt gtctgccacc 240

gagccatgca agccttgtac cgagtgtgtg ggcctgcaga gcatgtctgc cccttgtgtg 300

gaagccgacg atgccgtgtg tagatgcgcc tacggctact accaggacga gacaacaggc 360

agatgcgagg cctgtagagt gtgtgaagcc ggctctggac tggtgttcag ctgccaagac 420

aagcagaaca ccgtgtgcga ggaatgcccc gatggcacct atagcgacga ggccaaccat 480

gtggatccct gcctgccttg tactgtgtgc gaagataccg agcggcagct gcgcgagtgt 540

acaagatggg ctgatgccga gtgcgaagag atccccggca gatggatcac cagaagcaca 600

cctccagagg gcagcgatag cacagcccct tctacacaag agcccgaggc tcctcctgag 660

caggatctga ttgcctctac agtggccggc gtggtcacaa cagtgatggg atcttctcag 720

cccgtggtca ccagaggcac caccgacaat ctgatccccg tgtactgtag catcctggcc 780

gccgtggttg tgggactcgt ggcctatatc gccttcaagc ggtggaaccg gggcatcctg 840

taa 843

<210> 68
<211> 1077
<212> DNA

<213> Artificial Sequence

<220>
<223> CXCR4 WT sequence

<400> 68
gccaccatgt ctattcctct gcccctgctg cagatctaca ccagcgacaa ctacaccgag 60

gaaatgggca gcggcgacta cgacagcatg aaggaaccct gcttccggga agagaacgcc 120

aacttcaaca agatcttcct gcccacaatc tacagcatca tctttctgac cggcatcgtg 180

ggcaacggac tcgtgatcct cgtgatgggc taccagaaaa agctgcggag catgaccgac 240

aagtaccggc tgcacctgag cgtggccgac ctgctgttcg tgatcaccct gcctttctgg 300

gccgtggacg ccgtggccaa ttggtacttc ggcaacttcc tgtgcaaggc cgtgcacgtg 360

atctacacag tgaacctgta cagcagcgtg ctgatcctgg ccttcatcag cctggacaga 420

tacctggcca tcgtgcacgc caccaacagc cagcggccta gaaagctgct ggccgagaag 480

gtggtgtacg tgggcgtgtg gattcccgcc ctgctgctga ccatccccga cttcatcttc 540

gccaacgtgt ccgaggccga cgaccggtac atctgcgacc ggttctaccc caacgacctg 600

tgggtggtgg tgttccagtt ccagcacatc atggtgggac tgatcctgcc tggcatcgtg 660

attctgagct gctactgcat catcatcagc aagctgagcc acagcaaggg ccaccagaag 720

cggaaggccc tgaaaaccac cgtgatcctg attctggctt tcttcgcctg ctggctgccc 780

tactacatcg gcatcagcat cgacagcttc atcctgctgg aaatcatcaa gcagggctgc 840

gagttcgaga acaccgtgca caagtggatc agcattaccg aggccctggc ctttttccac 900

tgctgcctga accctatcct gtacgccttc ctgggcgcca agttcaagac ctctgcccag 960

cacgccctga ccagcgtgtc cagaggaagc agcctgaaga tcctgagcaa gggcaagaga 1020

ggcggccaca gctccgtgtc tacagagagc gagagcagca gcttccacag cagctga 1077

<210> 69
<211> 1077
<212> DNA
<213> Artificial Sequence

<220>
<223> CXCR4 WHIM sequence

<400> 69
gccaccatgt ctattcctct gcccctgctg cagatctaca ccagcgacaa ctacaccgag 60

gaaatgggca gcggcgacta cgacagcatg aaggaaccct gcttccggga agagaacgcc 120

aacttcaaca agatcttcct gcccacaatc tacagcatca tctttctgac cggcatcgtg 180

ggcaacggac tcgtgatcct cgtgatgggc taccagaaaa agctgcggag catgaccgac 240

aagtaccggc tgcacctgag cgtggccgac ctgctgttcg tgatcaccct gcctttctgg 300

gccgtggacg ccgtggccaa ttggtacttc ggcaacttcc tgtgcaaggc cgtgcacgtg 360

atctacacag tgaacctgta cagcagcgtg ctgatcctgg ccttcatcag cctggacaga 420

tacctggcca tcgtgcacgc caccaacagc cagcggccta gaaagctgct ggccgagaag 480

gtggtgtacg tgggcgtgtg gattcccgcc ctgctgctga ccatccccga cttcatcttc 540

gccaacgtgt ccgaggccga cgaccggtac atctgcgacc ggttctaccc caacgacctg 600

tgggtggtgg tgttccagtt ccagcacatc atggtgggac tgatcctgcc tggcatcgtg 660

attctgagct gctactgcat catcatcagc aagctgagcc acagcaaggg ccaccagaag 720

cggaaggccc tgaaaaccac cgtgatcctg attctggctt tcttcgcctg ctggctgccc 780

tactacatcg gcatcagcat cgacagcttc atcctgctgg aaatcatcaa gcagggctgc 840

gagttcgaga acaccgtgca caagtggatc agcattaccg aggccctggc ctttttccac 900

tgctgcctga accctatcct gtacgccttc ctgggcgcca agttcaagac ctctgcccag 960

cacgccctga ccagcgtgtc cagaggaagc agcctgaaga tcctgagcaa gggcaagtga 1020

ggcggccaca gctccgtgtc tacagagagc gagagcagca gcttccacag cagctga 1077

<210> 70
<211> 744
<212> DNA
<213> Artificial Sequence

<220>
<223> reverse-tTA (rtTA)

<400> 70
atgtctagac tggacaagag caaagtcata aactctgctc tggaattact caatggagtc 60

ggtatcgaag gcctgacgac aaggaaactc gctcaaaagc tgggagttga gcagcctacc 120

ctgtactggc acgtgaagaa caagcgggcc ctgctcgatg ccctgccaat cgagatgctg 180

gacaggcatc atacccactt ctgccccctg gaaggcgagt catggcaaga ctttctgcgg 240

aacaacgcca agtcattccg ctgtgctctc ctctcacatc gcgacggggc taaagtgcat 300

ctcggcaccc gcccaacaga gaaacagtac gaaaccctgg aaaatcagct cgcgttcctg 360

tgtcagcaag gcttctccct ggagaacgca ctgtacgctc tgtccgccgt gggccacttt 420

acactgggct gcgtattgga ggaacaggag catcaagtag caaaagagga aagagagaca 480

cctaccaccg attctatgcc cccacttctg agacaagcaa ttgagctgtt cgaccggcag 540

ggagccgaac ctgccttcct tttcggcctg gaactaatca tatgtggcct ggagaaacag 600

ctaaagtgcg aaagcggcgg gccggccgac gcccttgacg attttgactt agacatgctc 660

ccagccgatg cccttgacga ctttgacctt gatatgctgc ctgctgacgc tcttgacgat 720

tttgaccttg acatgctccc cggg 744

<210> 71
<211> 23
<212> DNA
<213> Artificial Sequence

<220>
<223> genomic sequence, Guide ID AAVS1gRNA

<400> 71
gtcaccaatc ctgtccctag tgg 23

<210> 72
<211> 23
<212> DNA
<213> Artificial Sequence

<220>
<223> genomic sequence, Guide ID IL2RG gRNA

<400> 72
actggccatt acaatcatgt ggg 23

<210> 73

<211> 23
<212> DNA
<213> Artificial Sequence

<220>
<223> genomic sequence, Guide ID CD40LG gRNA

<400> 73
tggatgattg cactttatca ggg 23

<210> 74
<211> 618
<212> DNA
<213> Artificial Sequence

<220>
<223> TetR sequence

<400> 74
atgtctagac tggacaagag caaagtcata aactctgctc tggaattact caatgaagtc 60

ggtatcgaag gcctgacgac aaggaaactc gctcaaaagc tgggagttga gcagcctacc 120

ctgtactggc acgtgaagaa caagcgggcc ctgctcgatg ccctggcaat cgagatgctg 180

gacaggcatc atacccactt ctgcccctg gaaggcgagt catggcaaga ctttctgcgg 240

aacaacgcca agtcattccg ctgtgctctc ctctcacatc gcgacggggc taaagtgcat 300

ctcggcaccc gcccaacaga gaaacagtac gaaaccctgg aaaatcagct cgcgttcctg 360

tgtcagcaag gcttctccct ggagaacgca ctgtacgctc tgtccgccgt gggccacttt 420

acactgggct gcgtattgga ggatcaggag catcaagtag caaaagagga aagagagaca 480

cctaccaccg attctatgcc cccacttctg agacaagcaa ttgagctgtt cgaccatcag 540

ggagccgaac ctgccttcct tttcggcctg gaactaatca tatgtggcct ggagaaacag 600

ctaaagtgcg aaagcggc 618

<210> 75
<211> 36
<212> DNA
<213> Artificial Sequence

<220>
<223> VP16 sequence

<400> 75
gccgacgccc ttgacgattt tgacttagac atgctc 36

<210> 76
<211> 18
<212> DNA
<213> Artificial Sequence

<220>
<223> TetO sequence

<400> 76
ccctatcagt gatagaga 18

<210> 77
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> NHEJ AAVS1 FW primer

<400> 77
cttcaggaca gcatgtttgc 20

<210> 78
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> NHEJ AAVS1 RV primer

<400> 78
acaggaggtg ggggttagac 20

<210> 79
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> NHEJ Intron 1 IL2RG FW primer

<400> 79

caccctctgt aaagccctgg 20

<210> 80
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> NHEJ Intron 1 IL2RG RV primer

<400> 80
aagaaatcta gattggggag 20

<210> 81
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> Intron 1 IL2RG 3' integration junction ddPCR FW primer

<400> 81
ctagattggg gagaaaatga 20

<210> 82
<211> 19
<212> DNA
<213> Artificial Sequence

<220>
<223> Intron 1 IL2RG 3' integration junction ddPCR RV primer

<400> 82
gtgggaaggg gccgtacag 19

<210> 83
<211> 24
<212> DNA
<213> Artificial Sequence

<220>
<223> Intron 1 IL2RG 3' integration junction ddPCR probe (FAM)

<400> 83
gtagctccta tgctaggcgt agcc 24

<210> 84
<211> 21
<212> DNA
<213> Artificial Sequence

<220>
<223> AAVS1 3' integration junction ddPCR FW (SK) primer

<400> 84
caccgtacac gcctaaagct a 21

<210> 85
<211> 21
<212> DNA
<213> Artificial Sequence

<220>
<223> AAVS1 3' integration junction ddPCR FW (mCMV) primer

<400> 85
cttatatagg cctcccaccg t 21

<210> 86
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> AAVS1 3' integration junction ddPCR RV primer

<400> 86
tcttgggaag tgtaaggaag 20

<210> 87
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> AAVS1 3' integration junction ddPCR probe (FAM)

<400> 87
ccagataagg aatctgccta 20

<210> 88

<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> AAVS1 3' integration junction ddPCR FW primer

<400> 88
gattgggaag acaatagcag 20

<210> 89
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> CD40LG 3' integration junction ddPCR FW primer

<400> 89
ttaggagggg gtctgataca 20

<210> 90
<211> 25
<212> DNA
<213> Artificial Sequence

<220>
<223> CD40LG 3' integration junction ddPCR RV primer

<400> 90
tcctcgatct gtgggaggaa gagaa 25

<210> 91
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> CD40LG 3' integration junction ddPCR Probe (FAM)

<400> 91
tcagtctccc tctgagatgt 20