SEQUENCE LISTING

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Fundacion Instituto de Investigacion Sanitaria Fundacion Jimenez Diaz
Centro de Investigacion Biomedica en Red

<120> LENTIVIRAL VECTORS FOR DELIVERY OF PKLR TO TREAT PYRUVATE KINASE DEFICIENCY

<130> ROPA-004/01US 329592-2050

<150> US 62/573,037
<151> 2017-10-16

<160> 26

<170> PatentIn version 3.5

<210> 1
<211> 234
<212> DNA
<213> Artificial Sequence

<220>
<223> Rev responsive element (RRE)

<400> 1
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gacgctgacg gtacaggcca gacaattatt gtctggtata gtgcagcagc agaacaattt 120

gctgagggct attgaggcgc aacagcatct gttgcaactc acagtctggg gcatcaagca 180

gctccaggca agaatcctgg ctgtggaaag atacctaaag gatcaacagc tcct 234

<210> 2
<211> 126
<212> DNA
<213> Artificial Sequence

<220>
<223> HIV-1 packaging signal (psi)

<400> 2
ctctctcgac gcaggactcg gcttgctgaa gcgcgcacgg caagaggcga ggggcggcga 60

ctggtgagta cgccaaaaat tttgactagc ggaggctaga aggagagaga tgggtgcgag 120

agcgtc 126

<210> 3
<211> 181
<212> DNA
<213> Artificial Sequence

<220>
<223> HIV-1 5' LTR

<400> 3
gggtctctct ggttagacca gatctgagcc tgggagctct ctggctaact agggaaccca 60

ctgcttaagc ctcaataaag cttgccttga gtgcttcaag tagtgtgtgc ccgtctgttg 120

tgtgactctg gtaactagag atccctcaga ccctttttagt cagtgtggaa aatctctagc 180

a 181

<210> 4
<211> 234
<212> DNA
<213> Artificial Sequence

<220>
<223> HIV-1 self-inactivating 3' LTR

<400> 4
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tctggttaga ccagatctga gcctgggagc tctctggcta actagggaac ccactgctta 120

agcctcaata aagcttgcct tgagtgcttc aagtagtgtg tgcccgtctg ttgtgtgact 180

ctggtaacta gagatccctc agacccttttt agtcagtgtg gaaaatctct agca 234

<210> 5
<211> 204
<212> DNA
<213> Artificial Sequence

<220>
<223> human cytomegalovirus (CMV) immediate early promoter

<400> 5
gtgatgcggt tttggcagta catcaatggg cgtggatagc ggtttgactc acggggattt 60

ccaagtctcc accccattga cgtcaatggg agtttgtttt ggcaccaaaa tcaacgggac 120

tttccaaaat gtcgtaacaa ctccgcccca ttgacgcaaa tgggcggtag gcgtgtacgg 180

tgggaggtct atataagcag agct 204

<210> 6
<211> 118
<212> DNA
<213> Artificial Sequence

<220>
<223> central polypurine tract and central termination sequence of HIV-1 (cPPT/CTS)

<400> 6
ttttaaaaga aaaggggga ttgggggta cagtgcaggg gaaagaatag tagacataat 60

agcaacagac atacaaacta aagaattaca aaaacaaatt acaaaaattc aaaatttt 118

<210> 7
<211> 511
<212> DNA
<213> Artificial Sequence

<220>
<223> human phosphoglycerate kinase 1 (hPGK) promoter

<400> 7
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tgggcgtggt tccgggaaac gcagcggcgc cgaccctggg tctcgcacat tcttcacgtc 120

cgttcgcagc gtcacccgga tcttcgccgc taccttgtg ggcccccgg cgacgcttcc 180

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ggaagccgca cgtctcacta gtaccctcgc agacggacag cgccagggag caatggcagc 300

gcgccgaccg cgatgggctg tggccaatag cggctgctca gcagggcgcg ccgagagcag 360

cggccgggaa ggggcggtgc gggaggcggg gtgtggggcg gtagtgtggg ccctgttcct 420

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cgttgaccga atcaccgacc tctctcccca g 511

<210> 8
<211> 1725
<212> DNA
<213> Artificial Sequence

<220>
<223> codon-optimized version of the human PKLR cDNA (coRPK)

<400> 8
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agagacctgg ccaagagcat cctgatcgga gcccctggcg gaccagccgg atacctgaga 120

agggctagcg tggcccagct gacccaggaa ctgggcaccg ccttttttcca gcagcagcag 180

ctgccagccg ccatggccga cacctttctg gaacacctgt gcctgctgga catcgactct 240

gagcccgtgg ccgccagaag caccagcatc attgccacca tcggccctgc cagcagaagc 300

gtggagcggc tgaaagagat gatcaaggcc ggcatgaata tcgcccggct gaacttctcc 360

cacggcagcc acgagtacca cgcagagagc attgccaacg tccgggaggc cgtggagagc 420

tttgccggca gccccctgag ctacagaccc gtggccattg ccctggacac caagggcccc 480

gagatcagaa caggaattct gcagggaggg cctgagagcg aggtggagct ggtgaagggc 540

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tgggtggact accccaacat cgtgcgggtg gtgcctgtgg gcggcagaat ctacatcgac 660

gacggcctga tcagcctggt ggtgcagaag atcggacctg agggcctggt gacccaggtc 720

gagaatggcg gcgtgctggg cagcagaaag ggcgtgaatc tgccaggcgc ccaggtggac 780

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ggcatcgaga tccccgccga gaaggtgttc ctggcccaga aaatgatgat cggacggtgc 1080

aacctggccg gcaaacctgt ggtgtgcgcc acccagatgc tggaaagcat gatcaccaag 1140

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caggccgcta gacaggtcca cctgtgcaga ggcgtgttcc ccctgctgta ccgggagcct 1560

cccgaggcca tctgggccga cgacgtggac agacgggtgc agttcggcat cgagagcggc 1620

aagctgcggg gcttcctgag agtgggcgac ctggtgatcg tggtgacagg ctggcggcct 1680

ggcagcggct acaccaacat catgagggtg ctgtccatca gctga 1725

<210> 9
<211> 380
<212> DNA
<213> Artificial Sequence

<220>
<223> human CMV enhancer

<400> 9
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catatatgga gttccgcgtt acataactta cggtaaatgg cccgcctggc tgaccgccca 120

acgacccccg cccattgacg tcaataatga cgtatgttcc catagtaacg ccaatagggа 180

ctttccattg acgtcaatgg gtggagtatt tacggtaaac tgcccacttg gcagtacatc 240

aagtgtatca tatgccaagt acgcccccta ttgacgtcaa tgacggtaaa tggcccgcct 300

ggcattatgc ccagtacatg accttatggg actttcctac ttggcagtac atctacgtat 360

tagtcatcgc tattaccatg 380

<210> 10
<211> 122
<212> DNA
<213> Artificial Sequence

<220>
<223> simian virus 40 (SV40) poly(A) signal

<400> 10
aacttgttta ttgcagctta taatggttac aaataaagca atagcatcac aaatttcaca 60

aataaagcat ttttttcact gcattctagt tgtggtttgt ccaaactcat caatgtatct 120

ta 122

<210> 11

<211> 136
<212> DNA
<213> Artificial Sequence

<220>
<223> SV40 origin of replication

<400> 11
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tttatttatg cagaggccga ggccgcctcg gcctctgagc tattccagaa gtagtgagga 120

ggctttttg gaggcc 136

<210> 12
<211> 28
<212> DNA
<213> Artificial Sequence

<220>
<223> central polypurine tract (cPPT)

<400> 12
tttaaaagaa aaggggggat tgggggt 28

<210> 13
<211> 83
<212> DNA
<213> Artificial Sequence

<220>
<223> dNEF signal sequence

<400> 13
gaattcgagc tcggtacctt taagaccaat gacttacaag gcagctgtag atcttagcca 60

cttttaaaa gaaaaggggg gac 83

<210> 14
<211> 795
<212> DNA
<213> Artificial Sequence

<220>
<223> NeoR/KanR sequence

<400> 14
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ggctatgact gggcacaaca gacaatcggc tgctctgatg ccgccgtgtt ccggctgtca 120

gcgcaggggc gtccggttct ttttgtcaag accgacctgt ccggtgccct gaatgaactg 180

caagacgagg cagcgcggct atcgtggctg gcgacgacgg gcgttccttg cgcggctgtg 240

ctcgacgttg tcactgaagc gggaagggac tggctgctat tgggcgaagt gccggggcag 300

gatctcctgt catctcacct tgctcctgcc gagaaagtat ccatcatggc tgatgcaatg 360

cggcggctgc atacgcttga tccggctacc tgcccattcg accaccaagc gaaacatcgc 420

atcgagcgag cacgtactcg gatggaagcc ggtcttgtcg atcaggatga tctggacgaa 480

gagcatcagg ggctcgcgcc agccgaactg ttcgccaggc tcaaggcgtc tatgcccgac 540

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atagcgttgg ctacccgtga tattgctgaa gagcttggcg gcgaatgggc tgaccgcttc 720

cttgtgcttt acggtatcgc cgcgcccgat tcgcagcgca tcgccttcta tcgccttctt 780

gacgagttct tctga 795

<210> 15
<211> 137
<212> DNA
<213> Artificial Sequence

<220>
<223> rrnG terminator (transcription terminator from the E.coli ribosomal RNA rrnG operon)

<400> 15
gcattggcgc agaaaaaaat gcctgatgcg acgctgcgcg tcttatactc ccacatatgc 60

cagattcagc aacggatacg gcttccccaa cttgcccact tccatacgtg tcctccttac 120

cagaaattta tccttaa 137

<210> 16
<211> 589
<212> DNA
<213> Artificial Sequence

<220>
<223> ori (high-copy-number ColE1/pMB1/pBR322/pUC origin of replication)

<400> 16
ttgagatcct ttttttctgc gcgtaatctg ctgcttgcaa acaaaaaaac caccgctacc 60

agcggtggtt tgtttgccgg atcaagagct accaactctt tttccgaagg taactggctt 120

cagcagagcg cagataccaa atactgttct tctagtgtag ccgtagttag gccaccactt 180

caagaactct gtagcaccgc ctacatacct cgctctgcta atcctgttac cagtggctgc 240

tgccagtggc gataagtcgt gtcttaccgg gttggactca agacgatagt taccggataa 300

ggcgcagcgg tcgggctgaa cgggggggttc gtgcacacag cccagcttgg agcgaacgac 360

ctacaccgaa ctgagatacc tacagcgtga gctatgagaa agcgccacgc ttcccgaagg 420

gagaaaggcg gacaggtatc cggtaagcgg cagggtcgga acaggagagc gcacgaggga 480

gcttccaggg ggaaacgcct ggtatcttta tagtcctgtc gggtttcgcc acctctgact 540

tgagcgtcga tttttgtgat gctcgtcagg ggggcggagc ctatggaaa 589

<210> 17
<211> 22
<212> DNA
<213> Artificial Sequence

<220>
<223> CAP binding site sequence

<400> 17
taatgtgagt tagctcactc at 22

<210> 18
<211> 31
<212> DNA
<213> Artificial Sequence

<220>
<223> E.coli lac promoter

<400> 18
tttacacttt atgcttccgg ctcgtatgtt g 31

<210> 19
<211> 17
<212> DNA
<213> Artificial Sequence

<220>
<223> lac operator sequence

<400> 19
ttgtgagcgg ataacaa 17

<210> 20
<211> 19
<212> DNA
<213> Artificial Sequence

<220>
<223> T3 promoter (promoter for bacteriophage T3 RNA polymerase)

<400> 20
aattaaccct cactaaagg 19

<210> 21
<211> 380
<212> DNA
<213> Artificial Sequence

<220>
<223> CMV enhancer

<400> 21
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catatatgga gttccgcgtt acataactta cggtaaatgg cccgcctggc tgaccgccca 120

acgacccccg cccattgacg tcaataatga cgtatgttcc catagtaacg ccaatagga 180

ctttccattg acgtcaatgg gtggagtatt tacggtaaac tgcccacttg gcagtacatc 240

aagtgtatca tatgccaagt acgccccta ttgacgtcaa tgacggtaaa tggcccgcct 300

ggcattatgc ccagtacatg accttatggg actttcctac ttggcagtac atctacgtat 360

tagtcatcgc tattaccatg 380

<210> 22
<211> 19
<212> DNA
<213> Artificial Sequence

<220>
<223> T7 promoter (promoter for bacteriophage T7 RNA polymerase)

<400> 22

cctatagtga gtcgtatta 19

<210> 23
<211> 429
<212> DNA
<213> Artificial Sequence

<220>
<223> f1 ori (f1 bacteriophage origin of replication)

<400> 23
acgcgccctg tagcggcgca ttaagcgcgg cgggtgtggt ggttacgcgc agcgtgaccg 60

ctacacttgc cagcgcccta gcgcccgctc ctttcgcttt cttcccttcc tttctcgcca 120

cgttcgccgg ctttccccgt caagctctaa atcgggggct ccctttaggg ttccgattta 180

gtgctttacg gcacctcgac cccaaaaaac ttgattaggg tgatggttca cgtagtgggc 240

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gactcttgtt ccaaactgga acaacactca accctatctc ggtctattct tttgatttat 360

aagggatttt gccgatttcg gcctattggt taaaaaatga gctgatttaa caaaaattta 420

acgcgaatt 429

<210> 24
<211> 677
<212> DNA
<213> Artificial Sequence

<220>
<223> chimeric wPRE RNA export signal

<400> 24
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tactagttca ggtgtattgc cacaagacaa acatgttaag aaactttccc gttatttacg 180

ctctgttcct gttaatcaac ctctggatta caaaatttgt gaaagattga ctgatattct 240

taactatgtt gctcctttta cgctgtgtgg atatgctgct ttaatgcctc tgtatcatgc 300

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cgcaaccccc actggctggg gcattgccac cacctgtcaa ctcctttctg ggactttcgc 480

tttccccctc ccgatcgcca cggcagaact catcgccgcc tgccttgccc gctgctggac 540

aggggctagg ttgctgggca ctgataattc cgtggtgttg tcggggaagg gcctgctgcc 600

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ggccgcctcc ccgcctg 677

<210> 25
<211> 9087
<212> DNA
<213> Artificial Sequence

<220>
<223> self-limiting lentivirus gene delivery vector

<400> 25
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gcaaacatct atcaagagac aggatcagca ggaggctttc gcatgattga acaagatgga 120

ttgcacgcag gttctccggc ggcttgggtg gagaggctat tcggctatga ctgggcacaa 180

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acccacggcg atgcctgctt gccgaatatc atggtggaaa atggccgctt ttctggattc 720

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tgctcgtcag gggggcggag cctatggaaa aacgccagca acgcggcctt tttacggttc 1920

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gataaccgta ttaccgcctt tgagtgagct gataccgctc gccgcagccg aacgaccgag 2040

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taatcaatta cggggtcatt agttcatagc ccatatatgg agttccgcgt tacataactt 2520

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aattcggtta aggccagggg gaaagaaaaa atataaatta aaacatatag tatgggcaag 3480

cagggagcta gaacgattcg cagttaatcc tggcctgtta gaaacatcag aaggctgtag 3540

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ataaatataa agtagtaaaa attgaaccat taggagtagc acccaccaag gcaaagagaa 3840

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