SEQUENCE LISTING

<110> Rocket Pharmaceuticals, Ltd.
Fundacion para la Investigacion Biomedica del Hospital Infantil Universitario Nino Jesus

<120> COMPOSITIONS AND METHODS FOR STEM CELL TRANSPLANT

<130> ROPA-008/01WO 326219-2068

<150> US 62/656,292
<151> 2018-04-11

<160> 37

<170> PatentIn version 3.5

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<223> Recombinant packaging signal sequence

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<223> Recombinant truncated HIV-1 5 prime LTR sequence

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<212> DNA
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<220>
<223> Recombinant HIV-1 self-inactivating 3 prime LTR sequence

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<210> 5
<211> 204
<212> DNA
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<220>
<223> Recombinant human cytomegalovirus immediate early promoter

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<211> 118
<212> DNA
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<220>
<223> Recombinant HIV-1 central polypurine tract and central termination sequence

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agcaacagac atacaaacta aagaattaca aaaacaaatt acaaaaattc aaaatttt 118

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<211> 511
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<213> Artificial Sequence

<220>
<223> Recombinant human phosphoglycerate kinase 1 promoter sequence

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<212> DNA
<213> Artificial Sequence

<220>
<223> Codon optimized FANCA sequence

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<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant human CMV enhancer sequence

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

<220>
<223> Recombinant Simian virus 40 polyA signal sequence

<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> Recombinant Simian virus 40 origin of replication sequence

<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> Recombinant HIV-1 central polypurine tract and central termination sequence

<400> 12
tttaaaagaa aaggggggat tgggggt 28

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

<220>
<223> Recombinant dNEF signal sequence

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cttttaaaa gaaaaggggg gac 83

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

<220>
<223> Recombinant KanR sequence

<400> 14
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gcgcaggggc gtccggttct ttttgtcaag accgacctgt ccggtgccct gaatgaactg 180

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

<220>
<223> Recombinant rrnG terminator sequence

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cagaaattta tccttaa 137

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

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<223> Recombinant high-copy-number ColE1, pMB1, pBR322,
pUC origin of replication

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<400> 17
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<223> Recombinant lac promoter sequence

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<223> Recombinant T3 promoter sequence

<400> 20
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<223> Recombinant T7 promoter sequence

<400> 21
cctatagtga gtcgtatta 19

<210> 22
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acgcgaatt 429

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<211> 677
<212> DNA
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<220>
<223> Chimeric wPRE sequence

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<211> 11433
<212> DNA
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<210> 26
<211> 858
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant Rev response element

<400> 26
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gcagagagaa aaaagagcag tgggaatagg agctttgttc cttgggttct tgggagcagc 180

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tgctgtgcct tggaatgcta gttggagtaa taaatctctg gaacagattt ggaatcacac 480

gacctggatg gagtgggaca gagaaattaa caattacaca agcttaatac actccttaat 540

tgaagaatcg caaaaccagc aagaaaagaa tgaacaagaa ttattggaat tagataaatg 600

ggcaagtttg tggaattggt ttaacataac aaattggctg tggtatataa aattattcat 660

aatgatagta ggaggcttgg taggtttaag aatagttttt gctgtacttt ctatagtgaa 720

tagagttagg cagggatatt caccattatc gtttcagacc cacctcccaa ccccgagggg 780

acccgacagg cccgaaggaa tagaagaaga aggtggagag agagacagag acagatccat 840

tcgattagtg aacggatc 858

<210> 27
<211> 44
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant packaging signal sequence

<400> 27
tgagtacgcc aaaaattttg actagcggag gctagaagga gaga 44

<210> 28
<211> 188
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant truncated HIV-1 5 prime LTR sequence

<400> 28
gtctctctgg ttagaccaga tctgagcctg ggagctctct ggctaactag ggaacccact 60

gcttaagcct caataaagct tgccttgagt gcttcaagta gtgtgtgccc gtctgttgtg 120

tgactctggt aactagagat ccctcagacc cttttagtca gtgtggaaaa tctctagcag 180

tggcgccc 188

<210> 29
<211> 234
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant HIV-1 self-inactivating 3 prime LTR sequence

<400> 29
tggaagggct aattcactcc caacgaagac aagatctgct ttttgcttgt actgggtctc 60

tctggttaga ccagatctga gcctgggagc tctctggcta actagggaac ccactgctta 120

agcctcaata aagcttgcct tgagtgcttc aagtagtgtg tgcccgtctg ttgtgtgact 180

ctggtaacta gagatccctc agacccttttt agtcagtgtg gaaaatctct agca 234

<210> 30
<211> 577
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant human cytomegalovirus immediate early promoter sequence

<400> 30
acattgatta ttgactagtt attaatagta atcaattacg gggtcattag ttcatagccc 60

atatatggag ttccgcgtta cataacttac ggtaaatggc ccgcctggct gaccgcccaa 120

cgacccccgc ccattgacgt caataatgac gtatgttccc atagtaacgc caatagggac 180

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gggcggtagg cgtgtacggt gggaggtcta tataagc 577

<210> 31
<211> 233

<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant Rous sarcoma virus promoter sequence

<400> 31
ttaatgtagt cttatgcaat actcttgtag tcttgcaaca tggtaacgat gagttagcaa 60

catgccttac aaggagagaa aaagcaccgt gcatgccgat tggtggaagt aaggtggtac 120

gatcgtgcct tattaggaag gcaacagacg ggtctgacat ggattggacg aaccactgaa 180

ttgccgcatt gcagagatat tgtatttaag tgcctagctc gatacaataa acg 233

<210> 32
<211> 16
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant HIV-1 central polypurine tract and central termination sequence

<400> 32
aaaagaaaag ggggga 16

<210> 33
<211> 121
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant HIV-1 central polypurine tract and central termination sequence

<400> 33
ttgggggggta cagtgcaggg gaaagaatag tagacataat agcaacagac atacaaacta 60

aagaattaca aaaacaaatt acaaaaattc aaaattttat cgatcacgag actagcctcg 120

a 121

<210> 34
<211> 156
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant Simian virus 40 origin of replication sequence

<400> 34
ggcctccaaa aaagcctcct cactacttct ggaatagctc agaggccgag gcggcctcgg 60

cctctgcata aataaaaaaa attagtcagc catggggcgg agaatgggcg gaactgggcg 120

gagttagggg cgggatgggc ggagttaggg gcggga 156

<210> 35
<211> 141
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant RNA-OUT sequence

<400> 35
gtagaattgg taaagagagt cgtgtaaaat atcgagttcg cacatcttgt tgtctgatta 60

ttgatttttg gcgaaaccat ttgatcatat gacaagatgt gtatctacct taacttaatg 120

attttgataa aaatcattag g 141

<210> 36
<211> 4371
<212> DNA
<213> Artificial Sequence

<220>
<223> Codon optimized FANCA sequence

<400> 36
atgtccgact cgtgggtccc gaactccgcc tcgggccagg acccaggggg ccgccggagg 60

gcctgggccg agctgctggc gggaagggtc aagagggaaa aatataatcc tgaaagggca 120

cagaaattaa aggaatcagc tgtgcgcctc ctgcgaagcc atcaggacct gaatgccctt 180

ttgcttgagg tagaaggtcc actgtgtaaa aaattgtctc tcagcaaagt gattgactgt 240

gacagttctg aggcctatgc taatcattct agttcattta taggctctgc tttgcaggat 300

caagcctcaa ggctgggggt tcccgtgggt attctctcag ccgggatggt tgcctctagc 360

gtgggacaga tctgcacggc tccagcggag accagtcacc ctgtgctgct gactgtggag 420

cagagaaaga agctgtcttc cctgttagag tttgctcagt atttattggc acacagtatg 480

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<210> 37
<211> 1015
<212> DNA
<213> Artificial Sequence

<220>
<223> Recombinant high copy number ColE1, pMB1, pBR322,
pUC origin of replication sequence

<400> 37
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ttaagcattg gtaactgtca gaccaagttt actcatatat actttagatt gatttaaaac 120

ttcatttta atttaaaagg atctaggtga agatcctttt tgataatctc atgaccaaaa 180

tcccttaacg tgagttttcg ttccactgag cgtcagaccc cgtagaaaag atcaaaggat 240

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ctgctgccag tggcgataag tcgtgtctta ccgggttgga ctcaagacga tagttaccgg 540

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