SEQUENCE LISTING

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CONSORCIO CENTRO DE INVESTIGACION BIOMEDICA EN RED, M.P.
FUNDACION INSTITUTO DE INVESTIGACION SANITARIA FUNDACION JIMENEZ DIAZ
ROCKET PHARMACEUTICALS, LTD.

<120> METHODS FOR GENE MODIFICATION OF HEMATOPOIETIC CELLS

<130> ROPA-010/01WO 329592-2074

<150> US 62/712,146
<151> 2018-07-30

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Glu Gly Pro Leu Cys Lys Lys Leu Ser Leu Ser Lys Val Ile Asp Cys
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Phe Ser Arg Leu Ser Phe Cys Gln Glu Leu Trp Lys Ile Gln Ser Ser
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Leu Gln Glu Leu Leu Glu Ser His Pro Asp Met His Ala Val Gly Ser
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Trp Leu Phe Arg Asn Leu Cys Cys Leu Cys Glu Gln Met Glu Ala Ser
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Cys Gln His Ala Asp Val Ala Arg Ala Met Leu Ser Asp Phe Val Gln
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Val Glu Pro Glu Lys Met Pro Gln Val Thr Val Asp Val Leu Gln Arg
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His Trp Gln Arg Val Leu Ser Phe Val Ser Ala Leu Val Val Cys Phe
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Thr Gly Asn Ile Pro Val Thr Val Met Glu Ala Ser Ile Phe Arg Arg
                565                 570                 575

Pro Tyr Tyr Val Ser His Phe Leu Pro Ala Leu Leu Thr Pro Arg Val
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Leu Pro Lys Val Pro Asp Ser Arg Val Ala Phe Ile Glu Ser Leu Lys
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Arg Ala Asp Lys Ile Pro Pro Ser Leu Tyr Ser Thr Tyr Cys Gln Ala
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Cys Ser Ala Ala Glu Glu Lys Pro Glu Asp Ala Ala Leu Gly Val Arg
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Ala Glu Pro Asn Ser Ala Glu Glu Pro Leu Gly Gln Leu Thr Ala Ala
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Leu Gly Glu Leu Arg Ala Ser Met Thr Asp Pro Ser Gln Arg Asp Val
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Ile Ser Ala Gln Val Ala Val Ile Ser Glu Arg Leu Arg Ala Val Leu
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    850                 855                 860

Ile Lys Lys Phe Gln Phe Leu Met Phe Arg Leu Phe Ser Glu Ala Arg
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Glu His Phe Leu Pro Glu Ser Ser Ala Ser Gly Gly Cys Asp Gly Asp
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<211> 769
<212> PRT
<213> Homo sapiens

<400> 4

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Leu Gly Cys Val Leu Ser Gln Glu Cys Thr Lys Phe Lys Val Ser Ser
20 25 30

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Cys Arg Glu Cys Ile Glu Ser Gly Pro Gly Cys Thr Trp Cys Gln Lys
        35                  40                  45

Leu Asn Phe Thr Gly Pro Gly Asp Pro Asp Ser Ile Arg Cys Asp Thr
    50                  55                  60

Arg Pro Gln Leu Leu Met Arg Gly Cys Ala Ala Asp Asp Ile Met Asp
65                  70                  75                  80

Pro Thr Ser Leu Ala Glu Thr Gln Glu Asp His Asn Gly Gly Gln Lys
                85                  90                  95

Gln Leu Ser Pro Gln Lys Val Thr Leu Tyr Leu Arg Pro Gly Gln Ala
            100                 105                 110

Ala Ala Phe Asn Val Thr Phe Arg Arg Ala Lys Gly Tyr Pro Ile Asp
        115                 120                 125

Leu Tyr Tyr Leu Met Asp Leu Ser Tyr Ser Met Leu Asp Asp Leu Arg
    130                 135                 140

Asn Val Lys Lys Leu Gly Gly Asp Leu Leu Arg Ala Leu Asn Glu Ile
145                 150                 155                 160

Thr Glu Ser Gly Arg Ile Gly Phe Gly Ser Phe Val Asp Lys Thr Val
                165                 170                 175

Leu Pro Phe Val Asn Thr His Pro Asp Lys Leu Arg Asn Pro Cys Pro
            180                 185                 190

Asn Lys Glu Lys Glu Cys Gln Pro Pro Phe Ala Phe Arg His Val Leu
        195                 200                 205

Lys Leu Thr Asn Asn Ser Asn Gln Phe Gln Thr Glu Val Gly Lys Gln
    210                 215                 220
```

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Leu Ile Ser Gly Asn Leu Asp Ala Pro Glu Gly Gly Leu Asp Ala Met
225                 230                 235                 240


Met Gln Val Ala Ala Cys Pro Glu Glu Ile Gly Trp Arg Asn Val Thr
                245                 250                 255


Arg Leu Leu Val Phe Ala Thr Asp Asp Gly Phe His Phe Ala Gly Asp
            260                 265                 270


Gly Lys Leu Gly Ala Ile Leu Thr Pro Asn Asp Gly Arg Cys His Leu
        275                 280                 285


Glu Asp Asn Leu Tyr Lys Arg Ser Asn Glu Phe Asp Tyr Pro Ser Val
    290                 295                 300


Gly Gln Leu Ala His Lys Leu Ala Glu Asn Asn Ile Gln Pro Ile Phe
305                 310                 315                 320


Ala Val Thr Ser Arg Met Val Lys Thr Tyr Glu Lys Leu Thr Glu Ile
                325                 330                 335


Ile Pro Lys Ser Ala Val Gly Glu Leu Ser Glu Asp Ser Ser Asn Val
            340                 345                 350


Val His Leu Ile Lys Asn Ala Tyr Asn Lys Leu Ser Ser Arg Val Phe
        355                 360                 365


Leu Asp His Asn Ala Leu Pro Asp Thr Leu Lys Val Thr Tyr Asp Ser
    370                 375                 380


Phe Cys Ser Asn Gly Val Thr His Arg Asn Gln Pro Arg Gly Asp Cys
385                 390                 395                 400


Asp Gly Val Gln Ile Asn Val Pro Ile Thr Phe Gln Val Lys Val Thr
                405                 410                 415


Ala Thr Glu Cys Ile Gln Glu Gln Ser Phe Val Ile Arg Ala Leu Gly
            420                 425                 430
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```
Phe Thr Asp Ile Val Thr Val Gln Val Leu Pro Gln Cys Glu Cys Arg
        435                 440                 445

Cys Arg Asp Gln Ser Arg Asp Arg Ser Leu Cys His Gly Lys Gly Phe
    450                 455                 460

Leu Glu Cys Gly Ile Cys Arg Cys Asp Thr Gly Tyr Ile Gly Lys Asn
465                 470                 475                 480

Cys Glu Cys Gln Thr Gln Gly Arg Ser Ser Gln Glu Leu Glu Gly Ser
                485                 490                 495

Cys Arg Lys Asp Asn Asn Ser Ile Ile Cys Ser Gly Leu Gly Asp Cys
            500                 505                 510

Val Cys Gly Gln Cys Leu Cys His Thr Ser Asp Val Pro Gly Lys Leu
        515                 520                 525

Ile Tyr Gly Gln Tyr Cys Glu Cys Asp Thr Ile Asn Cys Glu Arg Tyr
    530                 535                 540

Asn Gly Gln Val Cys Gly Gly Pro Gly Arg Gly Leu Cys Phe Cys Gly
545                 550                 555                 560

Lys Cys Arg Cys His Pro Gly Phe Glu Gly Ser Ala Cys Gln Cys Glu
                565                 570                 575

Arg Thr Thr Glu Gly Cys Leu Asn Pro Arg Arg Val Glu Cys Ser Gly
            580                 585                 590

Arg Gly Arg Cys Arg Cys Asn Val Cys Glu Cys His Ser Gly Tyr Gln
        595                 600                 605

Leu Pro Leu Cys Gln Glu Cys Pro Gly Cys Pro Ser Pro Cys Gly Lys
    610                 615                 620
```

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Tyr Ile Ser Cys Ala Glu Cys Leu Lys Phe Glu Lys Gly Pro Phe Gly
625                 630                 635                 640


Lys Asn Cys Ser Ala Ala Cys Pro Gly Leu Gln Leu Ser Asn Asn Pro
                645                 650                 655


Val Lys Gly Arg Thr Cys Lys Glu Arg Asp Ser Glu Gly Cys Trp Val
            660                 665                 670


Ala Tyr Thr Leu Glu Gln Gln Asp Gly Met Asp Arg Tyr Leu Ile Tyr
        675                 680                 685


Val Asp Glu Ser Arg Glu Cys Val Ala Gly Pro Asn Ile Ala Ala Ile
    690                 695                 700


Val Gly Gly Thr Val Ala Gly Ile Val Leu Ile Gly Ile Leu Leu Leu
705                 710                 715                 720


Val Ile Trp Lys Ala Leu Ile His Leu Ser Asp Leu Arg Glu Tyr Arg
                725                 730                 735


Arg Phe Glu Lys Glu Lys Leu Lys Ser Gln Trp Asn Asn Asp Asn Pro
            740                 745                 750


Leu Phe Lys Ser Ala Thr Thr Thr Val Met Asn Pro Lys Phe Ala Glu
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Ser
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<211> 2890
<212> DNA
<213> Artificial Sequence

<220>
<223> pCCL-PGK-coRPKW-82-RO vector sequence

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cgttcgcagc gtcacccgga tcttcgccgc taccttgtg ggccccccgg cgacgcttcc 180

tgctccgccc ctaagtcggg aaggttcctt gcggttcgcg gcgtgccgga cgtgacaaac 240

ggaagccgca cgtctcacta gtaccctcgc agacggacag cgccagggag caatggcagc 300

gcgccgaccg cgatgggctg tggccaatag cggctgctca gcagggcgcg ccgagagcag 360

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gcccgcgcgg tgttccgcat tctgcaagcc tccggagcgc acgtcggcag tcggctccct 480

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gacctggcca agagcatcct gatcggagcc cctggcggac cagccggata cctgagaagg 660

gctagcgtgg cccagctgac ccaggaactg ggcaccgcct ttttccagca gcagcagctg 720

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cccgtggccg ccagaagcac cagcatcatt gccaccatcg gccctgccag cagaagcgtg 840

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<210> 6
<211> 574

<212> PRT
<213> Homo sapiens

<400> 6

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            20                  25                  30


Gly Gly Pro Ala Gly Tyr Leu Arg Arg Ala Ser Val Ala Gln Leu Thr
        35                  40                  45


Gln Glu Leu Gly Thr Ala Phe Phe Gln Gln Gln Gln Leu Pro Ala Ala
    50                  55                  60


Met Ala Asp Thr Phe Leu Glu His Leu Cys Leu Leu Asp Ile Asp Ser
65                  70                  75                  80


Glu Pro Val Ala Ala Arg Ser Thr Ser Ile Ile Ala Thr Ile Gly Pro
                85                  90                  95


Ala Ser Arg Ser Val Glu Arg Leu Lys Glu Met Ile Lys Ala Gly Met
            100                 105                 110


Asn Ile Ala Arg Leu Asn Phe Ser His Gly Ser His Glu Tyr His Ala
        115                 120                 125


Glu Ser Ile Ala Asn Val Arg Glu Ala Val Glu Ser Phe Ala Gly Ser
    130                 135                 140


Pro Leu Ser Tyr Arg Pro Val Ala Ile Ala Leu Asp Thr Lys Gly Pro
145                 150                 155                 160


Glu Ile Arg Thr Gly Ile Leu Gln Gly Gly Pro Glu Ser Glu Val Glu
                165                 170                 175


Leu Val Lys Gly Ser Gln Val Leu Val Thr Val Asp Pro Ala Phe Arg
```

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            180                 185                 190

Thr Arg Gly Asn Ala Asn Thr Val Trp Val Asp Tyr Pro Asn Ile Val
        195                 200                 205

Arg Val Val Pro Val Gly Gly Arg Ile Tyr Ile Asp Asp Gly Leu Ile
    210                 215                 220

Ser Leu Val Val Gln Lys Ile Gly Pro Glu Gly Leu Val Thr Gln Val
225                 230                 235                 240

Glu Asn Gly Gly Val Leu Gly Ser Arg Lys Gly Val Asn Leu Pro Gly
                245                 250                 255

Ala Gln Val Asp Leu Pro Gly Leu Ser Glu Gln Asp Val Arg Asp Leu
            260                 265                 270

Arg Phe Gly Val Glu His Gly Val Asp Ile Val Phe Ala Ser Phe Val
        275                 280                 285

Arg Lys Ala Ser Asp Val Ala Ala Val Arg Ala Ala Leu Gly Pro Glu
    290                 295                 300

Gly His Gly Ile Lys Ile Ile Ser Lys Ile Glu Asn His Glu Gly Val
305                 310                 315                 320

Lys Arg Phe Asp Glu Ile Leu Glu Val Ser Asp Gly Ile Met Val Ala
                325                 330                 335

Arg Gly Asp Leu Gly Ile Glu Ile Pro Ala Glu Lys Val Phe Leu Ala
            340                 345                 350

Gln Lys Met Met Ile Gly Arg Cys Asn Leu Ala Gly Lys Pro Val Val
        355                 360                 365

Cys Ala Thr Gln Met Leu Glu Ser Met Ile Thr Lys Pro Arg Pro Thr
    370                 375                 380
```

```
Arg Ala Glu Thr Ser Asp Val Ala Asn Ala Val Leu Asp Gly Ala Asp
385                 390                 395                 400

Cys Ile Met Leu Ser Gly Glu Thr Ala Lys Gly Asn Phe Pro Val Glu
                405                 410                 415

Ala Val Lys Met Gln His Ala Ile Ala Arg Glu Ala Glu Ala Ala Val
            420                 425                 430

Tyr His Arg Gln Leu Phe Glu Glu Leu Arg Arg Ala Ala Pro Leu Ser
        435                 440                 445

Arg Asp Pro Thr Glu Val Thr Ala Ile Gly Ala Val Glu Ala Ala Phe
    450                 455                 460

Lys Cys Cys Ala Ala Ala Ile Ile Val Leu Thr Thr Thr Gly Arg Ser
465                 470                 475                 480

Ala Gln Leu Leu Ser Arg Tyr Arg Pro Arg Ala Ala Val Ile Ala Val
                485                 490                 495

Thr Arg Ser Ala Gln Ala Ala Arg Gln Val His Leu Cys Arg Gly Val
            500                 505                 510

Phe Pro Leu Leu Tyr Arg Glu Pro Pro Glu Ala Ile Trp Ala Asp Asp
        515                 520                 525

Val Asp Arg Arg Val Gln Phe Gly Ile Glu Ser Gly Lys Leu Arg Gly
    530                 535                 540

Phe Leu Arg Val Gly Asp Leu Val Ile Val Val Thr Gly Trp Arg Pro
545                 550                 555                 560

Gly Ser Gly Tyr Thr Asn Ile Met Arg Val Leu Ser Ile Ser
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<212> DNA
<213> Artificial Sequence

<220>
<223> PKLR polynucloetide seqeunce

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tgcctgtatg ccaagtggca gatgaacttc actgtgagat atgagactac caacaagacc 180

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<213> Artificial Sequence

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<212> DNA
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<220>
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<212> DNA
<213> Artificial Sequence

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20 25 30

Gly Leu Val Glu Phe Arg Asp Leu Asn Ala Ser Val Ser Ala Phe Gln
35 40 45

Arg Arg Phe Val Val Asp Val Arg Arg Cys Glu Glu Leu Glu Lys Thr
50 55 60

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Phe Thr Phe Leu Gln Glu Glu Val Arg Arg Ala Gly Leu Val Leu Pro
65                  70                  75                  80

Pro Pro Lys Gly Arg Leu Pro Ala Pro Pro Pro Arg Asp Leu Leu Arg
                85                  90                  95

Ile Gln Glu Glu Thr Glu Arg Leu Ala Gln Glu Leu Arg Asp Val Arg
            100                 105                 110

Gly Asn Gln Gln Ala Leu Arg Ala Gln Leu His Gln Leu Gln Leu His
        115                 120                 125

Ala Ala Val Leu Arg Gln Gly His Glu Pro Gln Leu Ala Ala Ala His
    130                 135                 140

Thr Asp Gly Ala Ser Glu Arg Thr Pro Leu Leu Gln Ala Pro Gly Gly
145                 150                 155                 160

Pro His Gln Asp Leu Arg Val Asn Phe Val Ala Gly Ala Val Glu Pro
                165                 170                 175

His Lys Ala Pro Ala Leu Glu Arg Leu Leu Trp Arg Ala Cys Arg Gly
            180                 185                 190

Phe Leu Ile Ala Ser Phe Arg Glu Leu Glu Gln Pro Leu Glu His Pro
        195                 200                 205

Val Thr Gly Glu Pro Ala Thr Trp Met Thr Phe Leu Ile Ser Tyr Trp
    210                 215                 220

Gly Glu Gln Ile Gly Gln Lys Ile Arg Lys Ile Thr Asp Cys Phe His
225                 230                 235                 240

Cys His Val Phe Pro Phe Leu Gln Gln Glu Glu Ala Arg Leu Gly Ala
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Leu Gln Gln Leu Gln Gln Gln Ser Gln Glu Leu Gln Glu Val Leu Gly
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Glu Thr Glu Arg Phe Leu Ser Gln Val Leu Gly Arg Val Leu Gln Leu
        275                 280                 285

Leu Pro Pro Gly Gln Val Gln Val His Lys Met Lys Ala Val Tyr Leu
    290                 295                 300

Ala Leu Asn Gln Cys Ser Val Ser Thr Thr His Lys Cys Leu Ile Ala
305                 310                 315                 320

Glu Ala Trp Cys Ser Val Arg Asp Leu Pro Ala Leu Gln Glu Ala Leu
                325                 330                 335

Arg Asp Ser Ser Met Glu Glu Gly Val Ser Ala Val Ala His Arg Ile
            340                 345                 350

Pro Cys Arg Asp Met Pro Pro Thr Leu Ile Arg Thr Asn Arg Phe Thr
        355                 360                 365

Ala Ser Phe Gln Gly Ile Val Asp Ala Tyr Gly Val Gly Arg Tyr Gln
    370                 375                 380

Glu Val Asn Pro Ala Pro Tyr Thr Ile Ile Thr Phe Pro Phe Leu Phe
385                 390                 395                 400

Ala Val Met Phe Gly Asp Val Gly His Gly Leu Leu Met Phe Leu Phe
                405                 410                 415

Ala Leu Ala Met Val Leu Ala Glu Asn Arg Pro Ala Val Lys Ala Ala
            420                 425                 430

Gln Asn Glu Ile Trp Gln Thr Phe Phe Arg Gly Arg Tyr Leu Leu Leu
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Leu Met Gly Leu Phe Ser Ile Tyr Thr Gly Phe Ile Tyr Asn Glu Cys
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Phe Ser Arg Ala Thr Ser Ile Phe Pro Ser Gly Trp Ser Val Ala Ala
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Met Ala Asn Gln Ser Gly Trp Ser Asp Ala Phe Leu Ala Gln His Thr
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Met Leu Thr Leu Asp Pro Asn Val Thr Gly Val Phe Leu Gly Pro Tyr
            500                 505                 510

Pro Phe Gly Ile Asp Pro Ile Trp Ser Leu Ala Ala Asn His Leu Ser
        515                 520                 525

Phe Leu Asn Ser Phe Lys Met Lys Met Ser Val Ile Leu Gly Val Val
    530                 535                 540

His Met Ala Phe Gly Val Val Leu Gly Val Phe Asn His Val His Phe
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Gly Gln Arg His Arg Leu Leu Leu Glu Thr Leu Pro Glu Leu Thr Phe
                565                 570                 575

Leu Leu Gly Leu Phe Gly Tyr Leu Val Phe Leu Val Ile Tyr Lys Trp
            580                 585                 590

Leu Cys Val Trp Ala Ala Arg Ala Ala Ser Ala Pro Ser Ile Leu Ile
        595                 600                 605

His Phe Ile Asn Met Phe Leu Phe Ser His Ser Pro Ser Asn Arg Leu
    610                 615                 620

Leu Tyr Pro Arg Gln Glu Val Val Gln Ala Thr Leu Val Val Leu Ala
625                 630                 635                 640

Leu Ala Met Val Pro Ile Leu Leu Leu Gly Thr Pro Leu His Leu Leu
                645                 650                 655
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His Arg His Arg Arg Arg Leu Arg Arg Arg Pro Ala Asp Arg Gln Glu
            660                 665                 670

Glu Asn Lys Ala Gly Leu Leu Asp Leu Pro Asp Ala Ser Val Asn Gly
        675                 680                 685

Trp Ser Ser Asp Glu Glu Lys Ala Gly Gly Leu Asp Asp Glu Glu Glu
    690                 695                 700

Ala Glu Leu Val Pro Ser Glu Val Leu Met His Gln Ala Ile His Thr
705                 710                 715                 720

Ile Glu Phe Cys Leu Gly Cys Val Ser Asn Thr Ala Ser Tyr Leu Arg
                725                 730                 735

Leu Trp Ala Leu Ser Leu Ala His Ala Gln Leu Ser Glu Val Leu Trp
            740                 745                 750

Ala Met Val Met Arg Ile Gly Leu Gly Leu Gly Arg Glu Val Gly Val
        755                 760                 765

Ala Ala Val Val Leu Val Pro Ile Phe Ala Ala Phe Ala Val Met Thr
    770                 775                 780

Val Ala Ile Leu Leu Val Met Glu Gly Leu Ser Ala Phe Leu His Ala
785                 790                 795                 800

Leu Arg Leu His Trp Val Glu Phe Gln Asn Lys Phe Tyr Ser Gly Thr
                805                 810                 815

Gly Tyr Lys Leu Ser Pro Phe Thr Phe Ala Ala Thr Asp Asp
            820                 825                 830
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