

pctgb2017051233-seq1 (1)
SEQUENCE LISTING

<110> CAMBRIDGE ENTERPRISE LIMITED
THE SYDNEY CHILDREN'S HOSPITALS NETWORK (RANDWICK AND
WESTMEAD) (INCORPORATING THE ROYAL ALEXANDRA HOSPITAL FOR
CHILDREN)

<120> Treatment of Complement-Mediated Disorders

<130> P/74985.W001

<150> GB1608046.7
<151> 2016-05-09

<160> 12

<170> PatentIn version 3.5

<210> 1
<211> 1752
<212> DNA
<213> Homo sapiens

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		20					25					30			

Cys	Leu	Ala	Lys	Lys	Tyr	Thr	His	Leu	Ser	Cys	Asp	Lys	Val	Phe	Cys
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pctgb2017051233-seql (1)

Gln Pro Trp Gln Arg Cys Ile Glu Gly Thr Cys Val Cys Lys Leu Pro
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Tyr Gln Cys Pro Lys Asn Gly Thr Ala Val Cys Ala Thr Asn Arg Arg
65 70 75 80

Ser Phe Pro Thr Tyr Cys Gln Gln Lys Ser Leu Glu Cys Leu His Pro
85 90 95

Gly Thr Lys Phe Leu Asn Asn Gly Thr Cys Thr Ala Glu Gly Lys Phe
100 105 110

Ser Val Ser Leu Lys His Gly Asn Thr Asp Ser Glu Gly Ile Val Glu
115 120 125

Val Lys Leu Val Asp Gln Asp Lys Thr Met Phe Ile Cys Lys Ser Ser
130 135 140

Trp Ser Met Arg Glu Ala Asn Val Ala Cys Leu Asp Leu Gly Phe Gln
145 150 155 160

Gln Gly Ala Asp Thr Gln Arg Arg Phe Lys Leu Ser Asp Leu Ser Ile
165 170 175

Asn Ser Thr Glu Cys Leu His Val His Cys Arg Gly Leu Glu Thr Ser
180 185 190

Leu Ala Glu Cys Thr Phe Thr Lys Arg Arg Thr Met Gly Tyr Gln Asp
195 200 205

Phe Ala Asp Val Val Cys Tyr Thr Gln Lys Ala Asp Ser Pro Met Asp
210 215 220

Asp Phe Phe Gln Cys Val Asn Gly Lys Tyr Ile Ser Gln Met Lys Ala
225 230 235 240

Cys Asp Gly Ile Asn Asp Cys Gly Asp Gln Ser Asp Glu Leu Cys Cys
245 250 255

pctgb2017051233-seql (1)

Lys Ala Cys Gln Gly Lys Gly Phe His Cys Lys Ser Gly Val Cys Ile
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Pro Ser Gln Tyr Gln Cys Asn Gly Glu Val Asp Cys Ile Thr Gly Glu
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 290 295 300

Glu Ile Leu Thr Ala Asp Met Asp Ala Glu Arg Arg Arg Ile Lys Ser
 305 310 315 320

Leu Leu Pro Lys Leu Ser Cys Gly Val Lys Asn Arg Met His Ile Arg
 325 330 335

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

Trp Gln Val Ala Ile Lys Asp Ala Ser Gly Ile Thr Cys Gly Gly Ile
 355 360 365

Tyr Ile Gly Gly Cys Trp Ile Leu Thr Ala Ala His Cys Leu Arg Ala
 370 375 380

Ser Lys Thr His Arg Tyr Gln Ile Trp Thr Thr Val Val Asp Trp Ile
 385 390 395 400

His Pro Asp Leu Lys Arg Ile Val Ile Glu Tyr Val Asp Arg Ile Ile
 405 410 415

Phe His Glu Asn Tyr Asn Ala Gly Thr Tyr Gln Asn Asp Ile Ala Leu
 420 425 430

Ile Glu Met Lys Lys Asp Gly Asn Lys Lys Asp Cys Glu Leu Pro Arg
 435 440 445

Ser Ile Pro Ala Cys Val Pro Trp Ser Pro Tyr Leu Phe Gln Pro Asn
 450 455 460

pctgb2017051233-seql (1)

Asp Thr Cys Ile Val Ser Gly Trp Gly Arg Glu Lys Asp Asn Glu Arg
465 470 475 480

Val Phe Ser Leu Gln Trp Gly Glu Val Lys Leu Ile Ser Asn Cys Ser
485 490 495

Lys Phe Tyr Gly Asn Arg Phe Tyr Glu Lys Glu Met Glu Cys Ala Gly
500 505 510

Thr Tyr Asp Gly Ser Ile Asp Ala Cys Lys Gly Asp Ser Gly Gly Pro
515 520 525

Leu Val Cys Met Asp Ala Asn Asn Val Thr Tyr Val Trp Gly Val Val
530 535 540

Ser Trp Gly Glu Asn Cys Gly Lys Pro Glu Phe Pro Gly Val Tyr Thr
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Lys Val Ala Asn Tyr Phe Asp Trp Ile Ser Tyr His Val Gly Arg Pro
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Phe Ile Ser Gln Tyr Asn Val
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<210> 3

<211> 1698

<212> DNA

<213> Homo sapiens

<400> 3

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<400> 4

pctgb2017051233-seql (1)

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Trp Gln Arg Cys Ile Glu Gly Thr Cys Val Cys Lys Leu Pro Tyr Gln
35 40 45

Cys Pro Lys Asn Gly Thr Ala Val Cys Ala Thr Asn Arg Arg Ser Phe
50 55 60

Pro Thr Tyr Cys Gln Gln Lys Ser Leu Glu Cys Leu His Pro Gly Thr
65 70 75 80

Lys Phe Leu Asn Asn Gly Thr Cys Thr Ala Glu Gly Lys Phe Ser Val
85 90 95

Ser Leu Lys His Gly Asn Thr Asp Ser Glu Gly Ile Val Glu Val Lys
100 105 110

Leu Val Asp Gln Asp Lys Thr Met Phe Ile Cys Lys Ser Ser Trp Ser
115 120 125

Met Arg Glu Ala Asn Val Ala Cys Leu Asp Leu Gly Phe Gln Gln Gly
130 135 140

Ala Asp Thr Gln Arg Arg Phe Lys Leu Ser Asp Leu Ser Ile Asn Ser
145 150 155 160

Thr Glu Cys Leu His Val His Cys Arg Gly Leu Glu Thr Ser Leu Ala
165 170 175

Glu Cys Thr Phe Thr Lys Arg Arg Thr Met Gly Tyr Gln Asp Phe Ala
180 185 190

Asp Val Val Cys Tyr Thr Gln Lys Ala Asp Ser Pro Met Asp Asp Phe
195 200 205

pctgb2017051233-seq1 (1)

Phe Gln Cys Val Asn Gly Lys Tyr Ile Ser Gln Met Lys Ala Cys Asp
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Gly Ile Asn Asp Cys Gly Asp Gln Ser Asp Glu Leu Cys Cys Lys Ala
225 230 235 240

Cys Gln Gly Lys Gly Phe His Cys Lys Ser Gly Val Cys Ile Pro Ser
245 250 255

Gln Tyr Gln Cys Asn Gly Glu Val Asp Cys Ile Thr Gly Glu Asp Glu
260 265 270

Val Gly Cys Ala Gly Phe Ala Ser Val Ala Gln Glu Glu Thr Glu Ile
275 280 285

Leu Thr Ala Asp Met Asp Ala Glu Arg Arg Arg Ile Lys Ser Leu Leu
290 295 300

Pro Lys Leu Ser Cys Gly Val Lys Asn Arg Met His Ile Arg Arg Lys
305 310 315 320

Arg Ile Val Gly Gly Lys Arg Ala Gln Leu Gly Asp Leu Pro Trp Gln
325 330 335

Val Ala Ile Lys Asp Ala Ser Gly Ile Thr Cys Gly Gly Ile Tyr Ile
340 345 350

Gly Gly Cys Trp Ile Leu Thr Ala Ala His Cys Leu Arg Ala Ser Lys
355 360 365

Thr His Arg Tyr Gln Ile Trp Thr Thr Val Val Asp Trp Ile His Pro
370 375 380

Asp Leu Lys Arg Ile Val Ile Glu Tyr Val Asp Arg Ile Ile Phe His
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Glu Asn Tyr Asn Ala Gly Thr Tyr Gln Asn Asp Ile Ala Leu Ile Glu
405 410 415

pctgb2017051233-seq1 (1)

Met Lys Lys Asp Gly Asn Lys Lys Asp Cys Glu Leu Pro Arg Ser Ile
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Pro Ala Cys Val Pro Trp Ser Pro Tyr Leu Phe Gln Pro Asn Asp Thr
435 440 445

Cys Ile Val Ser Gly Trp Gly Arg Glu Lys Asp Asn Glu Arg Val Phe
450 455 460

Ser Leu Gln Trp Gly Glu Val Lys Leu Ile Ser Asn Cys Ser Lys Phe
465 470 475 480

Tyr Gly Asn Arg Phe Tyr Glu Lys Glu Met Glu Cys Ala Gly Thr Tyr
485 490 495

Asp Gly Ser Ile Asp Ala Cys Lys Gly Asp Ser Gly Gly Pro Leu Val
500 505 510

Cys Met Asp Ala Asn Asn Val Thr Tyr Val Trp Gly Val Val Ser Trp
515 520 525

Gly Glu Asn Cys Gly Lys Pro Glu Phe Pro Gly Val Tyr Thr Lys Val
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Ser Gln Tyr Asn Val
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<210> 5

<211> 27

<212> DNA

<213> Artificial

<220>

<223> Primer sequence: FI-AAV-F

<400> 5

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<212> DNA

<213> Artificial

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<223> Primer sequence: FI-AAV-R

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<211> 1752

<212> DNA

<213> Homo sapiens

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tggggagtgg tgtcctgggg agagaactgc ggcaagccag agttccccgg cgtatatacc	1680
aaggtggcca attattttga ttggatttcc taccacgtcg gcaggccctt tatttcccag	1740
tataatgtct aa	1752

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<220>
 <223> HLP liver-specific promoter sequence

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tggggtgacc ttggttaata ttcaccagca gcctcccccg ttgccctctt ggatccactg	180
cttaaatacg gacgaggaca gggccctgtc tcctcagctt caggcaccac cactgacctg	240
ggacagtga t	251

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 <211> 447
 <212> DNA
 <213> Artificial

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<220>

<223> LP1 liver-specific promoter sequence

<400> 9

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cactcgaccc cttggaattt cgggtggagag gagcagaggt tgtcctggcg tggtttaggt	180
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caccaccact gacctgggac agtgaat	447

<210> 10

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<212> DNA

<213> Artificial

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acacaggacg ctgtgggttc tgagccaggg ggcgactcag atcccagcca gtggacttag	180
cccctgtttg ctctccgat aactggggtg accttggtta atattcacca gcagcctccc	240
ccgttgcccc tctggatcca ctgcttaaata acggacgagg acagggcct gtctcctcag	300
cttcaggcac caccactgac ctgggacagt gaatgatccc cctgatctgc ggcc	354

<210> 11

<211> 736

<212> PRT

<213> Artificial

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<223> MutC capsid protein sequence

<400> 11

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20 25 30

Lys Ala Asn Gln Gln Lys Gln Asp Asp Gly Arg Gly Leu Val Leu Pro
35 40 45

Gly Tyr Lys Tyr Leu Gly Pro Phe Asn Gly Leu Asp Lys Gly Glu Pro
50 55 60

Val Asn Ala Ala Asp Ala Ala Ala Leu Glu His Asp Lys Ala Tyr Asp
65 70 75 80

Gln Gln Leu Gln Ala Gly Asp Asn Pro Tyr Leu Arg Tyr Asn His Ala
85 90 95

Asp Ala Glu Phe Gln Glu Arg Leu Gln Glu Asp Thr Ser Phe Gly Gly
100 105 110

Asn Leu Gly Arg Ala Val Phe Gln Ala Lys Lys Arg Val Leu Glu Pro
115 120 125

Leu Gly Leu Val Glu Glu Gly Ala Lys Thr Ala Pro Gly Lys Lys Arg
130 135 140

Pro Val Asp Gln Ser Pro Gln Glu Pro Asp Ser Ser Ser Gly Val Gly
145 150 155 160

Lys Ser Gly Lys Gln Pro Ala Arg Lys Arg Leu Asn Phe Gly Gln Thr
165 170 175

Gly Asp Ser Glu Ser Val Pro Asp Pro Gln Pro Leu Gly Glu Pro Pro
180 185 190

Ala Ala Pro Thr Ser Leu Gly Ser Asn Thr Met Ala Ser Gly Gly Gly
195 200 205

pctgb2017051233-seq1 (1)

Ala Pro Met Ala Asp Asn Asn Glu Gly Ala Asp Gly Val Gly Asn Ser
210 215 220

Ser Gly Asn Trp His Cys Asp Ser Gln Trp Leu Gly Asp Arg Val Ile
225 230 235 240

Thr Thr Ser Thr Arg Thr Trp Ala Leu Pro Thr Tyr Asn Asn His Leu
245 250 255

Tyr Lys Gln Ile Ser Ser Gln Ser Gly Ala Ser Asn Asp Asn His Tyr
260 265 270

Phe Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn Arg Phe His
275 280 285

Cys His Phe Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn Asn Asn Trp
290 295 300

Gly Phe Arg Pro Lys Lys Leu Ser Phe Lys Leu Phe Asn Ile Gln Val
305 310 315 320

Lys Glu Val Thr Gln Asn Asp Gly Thr Thr Thr Ile Ala Asn Asn Leu
325 330 335

Thr Ser Thr Val Gln Val Phe Thr Asp Ser Glu Tyr Gln Leu Pro Tyr
340 345 350

Val Leu Gly Ser Ala His Gln Gly Cys Leu Pro Pro Phe Pro Ala Asp
355 360 365

Val Phe Met Val Pro Gln Tyr Gly Tyr Leu Thr Leu Asn Asn Gly Ser
370 375 380

Gln Ala Val Gly Arg Ser Ser Phe Tyr Cys Leu Glu Tyr Phe Pro Ser
385 390 395 400

Gln Met Leu Arg Thr Gly Asn Asn Phe Gln Phe Ser Tyr Thr Phe Glu
405 410 415

pctgb2017051233-seql (1)

Asp Val Pro Phe His Ser Ser Tyr Ala His Ser Gln Ser Leu Asp Arg
420 425 430

Leu Met Asn Pro Leu Ile Asp Gln Tyr Leu Tyr Tyr Leu Asn Arg Thr
435 440 445

Gln Gly Thr Thr Ser Gly Thr Thr Asn Gln Ser Arg Leu Leu Phe Ser
450 455 460

Gln Ala Gly Pro Gln Ser Met Ser Leu Gln Ala Arg Asn Trp Leu Pro
465 470 475 480

Gly Pro Cys Tyr Arg Gln Gln Arg Leu Ser Lys Thr Ala Asn Asp Asn
485 490 495

Asn Asn Ser Asn Phe Pro Trp Thr Ala Ala Ser Lys Tyr His Leu Asn
500 505 510

Gly Arg Asp Ser Leu Val Asn Pro Gly Pro Ala Met Ala Ser His Lys
515 520 525

Asp Asp Glu Glu Lys Phe Phe Pro Met His Gly Asn Leu Ile Phe Gly
530 535 540

Lys Glu Gly Thr Thr Ala Ser Asn Ala Glu Leu Asp Asn Val Met Ile
545 550 555 560

Thr Asp Glu Glu Glu Ile Arg Thr Thr Asn Pro Val Ala Thr Glu Gln
565 570 575

Tyr Gly Thr Val Ala Asn Asn Leu Gln Ser Ser Asn Thr Ala Pro Thr
580 585 590

Thr Arg Thr Val Asn Asp Gln Gly Ala Leu Pro Gly Met Val Trp Gln
595 600 605

Asp Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile Pro His
610 615 620

pctgb2017051233-seql (1)

Thr Asp Gly His Phe His Pro Ser Pro Leu Met Gly Gly Phe Gly Leu
625 630 635 640

Lys His Pro Pro Pro Gln Ile Met Ile Lys Asn Thr Pro Val Pro Ala
645 650 655

Asn Pro Pro Thr Thr Phe Ser Pro Ala Lys Phe Ala Ser Phe Ile Thr
660 665 670

Gln Tyr Ser Thr Gly Gln Val Ser Val Glu Ile Glu Trp Glu Leu Gln
675 680 685

Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr Ser Asn
690 695 700

Tyr Asn Lys Ser Val Asn Val Asp Phe Thr Val Asp Thr Asn Gly Val
705 710 715 720

Tyr Ser Glu Pro Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg Asn Leu
725 730 735

<210> 12

<211> 736

<212> PRT

<213> Artificial

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<223> LK03 capsid protein sequence

<400> 12

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Glu Gly Ile Arg Glu Trp Trp Ala Leu Gln Pro Gly Ala Pro Lys Pro
20 25 30

Lys Ala Asn Gln Gln His Gln Asp Asn Ala Arg Gly Leu Val Leu Pro
35 40 45

Gly Tyr Lys Tyr Leu Gly Pro Gly Asn Gly Leu Asp Lys Gly Glu Pro

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50

55

60

Val Asn Ala Ala Asp Ala Ala Ala Leu Glu His Asp Lys Ala Tyr Asp
 65 70 75 80

Gln Gln Leu Lys Ala Gly Asp Asn Pro Tyr Leu Lys Tyr Asn His Ala
 85 90 95

Asp Ala Glu Phe Gln Glu Arg Leu Lys Glu Asp Thr Ser Phe Gly Gly
 100 105 110

Asn Leu Gly Arg Ala Val Phe Gln Ala Lys Lys Arg Leu Leu Glu Pro
 115 120 125

Leu Gly Leu Val Glu Glu Ala Ala Lys Thr Ala Pro Gly Lys Lys Arg
 130 135 140

Pro Val Asp Gln Ser Pro Gln Glu Pro Asp Ser Ser Ser Gly Val Gly
 145 150 155 160

Lys Ser Gly Lys Gln Pro Ala Arg Lys Arg Leu Asn Phe Gly Gln Thr
 165 170 175

Gly Asp Ser Glu Ser Val Pro Asp Pro Gln Pro Leu Gly Glu Pro Pro
 180 185 190

Ala Ala Pro Thr Ser Leu Gly Ser Asn Thr Met Ala Ser Gly Gly Gly
 195 200 205

Ala Pro Met Ala Asp Asn Asn Glu Gly Ala Asp Gly Val Gly Asn Ser
 210 215 220

Ser Gly Asn Trp His Cys Asp Ser Gln Trp Leu Gly Asp Arg Val Ile
 225 230 235 240

Thr Thr Ser Thr Arg Thr Trp Ala Leu Pro Thr Tyr Asn Asn His Leu
 245 250 255

Tyr Lys Gln Ile Ser Ser Gln Ser Gly Ala Ser Asn Asp Asn His Tyr

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260

265

270

Phe Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn Arg Phe His
 275 280 285

Cys His Phe Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn Asn Asn Trp
 290 295 300

Gly Phe Arg Pro Lys Lys Leu Ser Phe Lys Leu Phe Asn Ile Gln Val
 305 310 315 320

Lys Glu Val Thr Gln Asn Asp Gly Thr Thr Thr Ile Ala Asn Asn Leu
 325 330 335

Thr Ser Thr Val Gln Val Phe Thr Asp Ser Glu Tyr Gln Leu Pro Tyr
 340 345 350

Val Leu Gly Ser Ala His Gln Gly Cys Leu Pro Pro Phe Pro Ala Asp
 355 360 365

Val Phe Met Val Pro Gln Tyr Gly Tyr Leu Thr Leu Asn Asn Gly Ser
 370 375 380

Gln Ala Val Gly Arg Ser Ser Phe Tyr Cys Leu Glu Tyr Phe Pro Ser
 385 390 395 400

Gln Met Leu Arg Thr Gly Asn Asn Phe Gln Phe Ser Tyr Thr Phe Glu
 405 410 415

Asp Val Pro Phe His Ser Ser Tyr Ala His Ser Gln Ser Leu Asp Arg
 420 425 430

Leu Met Asn Pro Leu Ile Asp Gln Tyr Leu Tyr Tyr Leu Asn Arg Thr
 435 440 445

Gln Gly Thr Thr Ser Gly Thr Thr Asn Gln Ser Arg Leu Leu Phe Ser
 450 455 460

Gln Ala Gly Pro Gln Ser Met Ser Leu Gln Ala Arg Asn Trp Leu Pro

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465 470 475 480
 Gly Pro Cys Tyr Arg Gln Gln Arg Leu Ser Lys Thr Ala Asn Asp Asn
 485 490 495
 Asn Asn Ser Asn Phe Pro Trp Thr Ala Ala Ser Lys Tyr His Leu Asn
 500 505 510
 Gly Arg Asp Ser Leu Val Asn Pro Gly Pro Ala Met Ala Ser His Lys
 515 520 525
 Asp Asp Glu Glu Lys Phe Phe Pro Met His Gly Asn Leu Ile Phe Gly
 530 535 540
 Lys Glu Gly Thr Thr Ala Ser Asn Ala Glu Leu Asp Asn Val Met Ile
 545 550 555 560
 Thr Asp Glu Glu Glu Ile Arg Thr Thr Asn Pro Val Ala Thr Glu Gln
 565 570 575
 Tyr Gly Thr Val Ala Asn Asn Leu Gln Ser Ser Asn Thr Ala Pro Thr
 580 585 590
 Thr Arg Thr Val Asn Asp Gln Gly Ala Leu Pro Gly Met Val Trp Gln
 595 600 605
 Asp Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile Pro His
 610 615 620
 Thr Asp Gly His Phe His Pro Ser Pro Leu Met Gly Gly Phe Gly Leu
 625 630 635 640
 Lys His Pro Pro Pro Gln Ile Met Ile Lys Asn Thr Pro Val Pro Ala
 645 650 655
 Asn Pro Pro Thr Thr Phe Ser Pro Ala Lys Phe Ala Ser Phe Ile Thr
 660 665 670
 Gln Tyr Ser Thr Gly Gln Val Ser Val Glu Ile Glu Trp Glu Leu Gln

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675

680

685

Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr Ser Asn
690 695 700

Tyr Asn Lys Ser Val Asn Val Asp Phe Thr Val Asp Thr Asn Gly Val
705 710 715 720

Tyr Ser Glu Pro Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg Pro Leu
725 730 735