

SEQUENCE LISTING

<110> Research Institute at Nationwide Children's Hospital

<120> OPTIMIZED GENE THERAPY FOR TARGETING MUSCLE IN MUSCLE DISEASES

<130> 28335/54649

<150> US 62/951,564

<151> 2019-12-20

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<170> PatentIn version 3.5

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<211> 178

<212> PRT

<213> Homo Sapiens

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Gln Leu Leu Pro Leu Gly Gly Gly Arg Asp Arg Lys Val Arg Asp Leu
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Gln Glu Ala Asp Leu Asp Leu Leu Arg Val Thr Leu Ser Ser Lys Pro
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Gln Ala Leu Ala Thr Pro Asn Lys Glu Glu His Gly Lys Arg Lys Lys
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Lys Gly Lys Gly Leu Gly Lys Lys Arg Asp Pro Gly Pro Glu Thr Leu
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Cys Gly Ala Glu Leu Val Asp Ala Leu Gln Phe Val Cys Gly Asp Arg
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Gly Phe Tyr Phe Asn Lys Pro Thr Gly Tyr Gly Ser Ser Ser Arg Arg
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Glu Leu Ser Lys Glu Glu Cys Cys Ser Thr Gly Arg Leu Ser Thr Ser
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Trp Thr Glu Glu Asp Val Asn Asp Asn Thr Leu Phe Lys Trp Met Ile
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Phe Asn Gly Gly Ala Pro Asn Cys Ile Pro Cys Lys Glu Thr Cys Glu
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Asn Val Asp Cys Gly Pro Gly Lys Lys Cys Arg Met Asn Lys Lys Asn
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Gln Gly Arg Cys Lys Lys Thr Cys Arg Asp Val Phe Cys Pro Gly Ser
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Ser Thr Cys Val Val Asp Gln Thr Asn Asn Ala Tyr Cys Val Thr Cys
180 185 190

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195 200 205

Asn Asp Gly Val Thr Tyr Ser Ser Ala Cys His Leu Arg Lys Ala Thr
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gtgtgtgggtt tcactcagtt cttctcatgg ggtttctctg agctccattc ataccagaaa 180

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tcgcctcctt ttctgggagg acactccctt ctgatggtga atgggaactc ccttcctcct	300
cgagcagcct gcctgcagct gtcctggtag aacagtgtgg acattgcaga agctgtcact	360
gccccagaaa gaaagcaccc cagagccaag gcaaagagtc ttgaaagcgc cacaagcagc	420
tgctgag	427

<210> 31
 <211> 319
 <212> PRT
 <213> Homo Sapiens

 <400> 31

Met	Glu	Leu	Leu	Ser	Pro	Pro	Leu	Arg	Asp	Val	Asp	Leu	Thr	Ala	Pro
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Asp	Gly	Ser	Leu	Cys	Ser	Phe	Ala	Thr	Thr	Asp	Asp	Phe	Tyr	Asp	Asp
			20					25					30		

Pro	Cys	Phe	Asp	Ser	Pro	Asp	Leu	Arg	Phe	Phe	Glu	Asp	Leu	Asp	Pro
		35					40					45			

Arg	Leu	Met	His	Val	Gly	Ala	Leu	Leu	Lys	Pro	Glu	Glu	His	Ser	His
	50					55					60				

Phe	Pro	Ala	Ala	Val	His	Pro	Ala	Pro	Gly	Ala	Arg	Glu	Asp	Glu	His
65					70					75				80	

Val	Arg	Ala	Pro	Ser	Gly	His	His	Gln	Ala	Gly	Arg	Cys	Leu	Leu	Trp
				85					90					95	

Ala	Cys	Lys	Ala	Cys	Lys	Arg	Lys	Thr	Thr	Asn	Ala	Asp	Arg	Arg	Lys
			100						105				110		

Ala	Ala	Thr	Met	Arg	Glu	Arg	Arg	Arg	Leu	Ser	Lys	Val	Asn	Glu	Ala
		115					120						125		

Phe Glu Thr Leu Lys Arg Cys Thr Ser Ser Asn Pro Asn Gln Arg Leu
130 135 140

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

Gln Ala Leu Leu Arg Asp Gln Asp Ala Ala Pro Pro Gly Ala Ala Ala
165 170 175

Phe Tyr Ala Pro Gly Pro Leu Pro Pro Gly Arg Gly Gly Glu His Tyr
180 185 190

Ser Gly Asp Ser Asp Ala Ser Ser Pro Arg Ser Asn Cys Ser Asp Gly
195 200 205

Met Met Asp Tyr Ser Gly Pro Pro Ser Gly Ala Arg Arg Arg Asn Cys
210 215 220

Tyr Glu Gly Ala Tyr Tyr Asn Glu Ala Pro Ser Glu Pro Arg Pro Gly
225 230 235 240

Lys Ser Ala Ala Val Ser Ser Leu Asp Tyr Leu Ser Ser Ile Val Glu
245 250 255

Arg Ile Ser Thr Glu Ser Pro Ala Ala Pro Ala Leu Leu Leu Ala Asp
260 265 270

Val Pro Ser Glu Ser Pro Pro Arg Arg Gln Glu Ala Ala Ala Pro Ser
275 280 285

Glu Gly Glu Ser Ser Gly Asp Pro Thr Gln Ser Pro Asp Ala Ala Pro
290 295 300

Gln Cys Pro Ala Gly Ala Asn Pro Asn Pro Ile Tyr Gln Val Leu
305 310 315

<210> 32
<211> 2501

<212> DNA

<213> Homo Sapiens

<400> 32

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ggaaccccg ccccggtggt gtttgctggc tccagcaggg ccaggaggcc acctgcagcc	180
tggtgctcca gactgatgtc acccgggccc agtgctgtgc ctccggcaac attgacaccg	240
cctggtccaa cctcaccac ccggggaaca agatcaacct cctcggcttc ttgggccttg	300
tccactgcct tccctgcaaa gattcgtgcg acggcgtgga gtgcggcccg ggcaaggcgt	360
gccgcatgct gggggggccg ccgcgctgcg agtcgcgcc cgactgctcg gggctcccgg	420
cgcggtgca ggtctgcggc tcagacggcg ccacctaccg cgacgagtgc gagctgcgcg	480
ccgcgcgtg ccgcggccac ccggacctga gcgtcatgta ccggggccgc tgccgcaagt	540
cctgtgagca cgtggtgtgc ccgcggccac agtcgtgcgt cgtggaccag acgggcagcg	600
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tcctgggccc ctccatcggc gtgcgccacg cgggcagctg cgcaggcacc cctgaggagc	780
cgccaggtag tgagtctgca gaagaggaa agaaacttcgt gtgagcctgc aggacaggcc	840
tgggcctggt gcccagggcc ccccatcatc ccctgttatt tattgccaca gcagagtcta	900
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cctgggtgtg tacggagggt ctagcctggg tgagtacgga gggcttagcc tgggtgagta	1320
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<210> 33
 <211> 263
 <212> PRT
 <213> Homo Sapiens

<400> 33

Met	Arg	Pro	Gly	Ala	Pro	Gly	Pro	Leu	Trp	Pro	Leu	Pro	Trp	Gly	Ala
1				5				10						15	

Leu Ala Trp Ala Val Gly Phe Val Ser Ser Met Gly Ser Gly Asn Pro
20 25 30

Ala Pro Gly Gly Val Cys Trp Leu Gln Gln Gly Gln Glu Ala Thr Cys
35 40 45

Ser Leu Val Leu Gln Thr Asp Val Thr Arg Ala Glu Cys Cys Ala Ser
50 55 60

Gly Asn Ile Asp Thr Ala Trp Ser Asn Leu Thr His Pro Gly Asn Lys
65 70 75 80

Ile Asn Leu Leu Gly Phe Leu Gly Leu Val His Cys Leu Pro Cys Lys
85 90 95

Asp Ser Cys Asp Gly Val Glu Cys Gly Pro Gly Lys Ala Cys Arg Met
100 105 110

Leu Gly Gly Arg Pro Arg Cys Glu Cys Ala Pro Asp Cys Ser Gly Leu
115 120 125

Pro Ala Arg Leu Gln Val Cys Gly Ser Asp Gly Ala Thr Tyr Arg Asp
130 135 140

Glu Cys Glu Leu Arg Ala Ala Arg Cys Arg Gly His Pro Asp Leu Ser
145 150 155 160

Val Met Tyr Arg Gly Arg Cys Arg Lys Ser Cys Glu His Val Val Cys
165 170 175

Pro Arg Pro Gln Ser Cys Val Val Asp Gln Thr Gly Ser Ala His Cys
180 185 190

Val Val Cys Arg Ala Ala Pro Cys Pro Val Pro Ser Ser Pro Gly Gln
195 200 205

Glu Leu Cys Gly Asn Asn Asn Val Thr Tyr Ile Ser Ser Cys His Met
210 215 220

Arg Gln Ala Thr Cys Phe Leu Gly Arg Ser Ile Gly Val Arg His Ala
 225 230 235 240

Gly Ser Cys Ala Gly Thr Pro Glu Glu Pro Pro Gly Gly Glu Ser Ala
 245 250 255

Glu Glu Glu Glu Asn Phe Val
 260

<210> 34
 <211> 3417
 <212> DNA
 <213> Homo Sapiens

<400> 34
 agaggcgcca gcagcctgcc tgtgacaggc atcaggttag ctggctccca ctcgggtggc 60
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 ggagagggag cttccttcta aagacctttc ttttatctga agccgcacag cccggcaggc 180
 tgtgctgact tgggtggaggc agcagcggca gagcagcctg agcagcagcc tgagcaggaa 240
 acctgctggg gtggggagggg cagggtgtctg cagcccctga gaagaaggcc ctggtggggc 300
 ccagaccctg gcatcgtttc aggggaggtc tctagccgcc ccagcctgca ccatgtgggc 360
 cccaaggtgt cgccggttct ggtctcgctg ggagcagggtg gcagcgctgc tgctgtgct 420
 gctactgctc ggggtgcccc cgccaagcct ggcgctgccg cccatccgct attcccacgc 480
 cggcatctgc cccaacgaca tgaatcccaa cctctgggtg gacgcacaga gcacctgcag 540
 gcgggagtgat gagacggacc aggagtgtga gacctatgag aagtgtgtcc ccaacgtatg 600
 tgggaccaag agctgcgtgg cggcccgccta catggacgtg aaagggaaga agggcccagt 660
 gggcatgccc aaggaggcca catgtgacca cttcatgtgt ctgcagcagg gctctgagt 720
 tgacatctgg gatggccagc ccgtgtgtaa gtgcaaagac cgctgtgaga aggagcccag 780
 ctttacctgc gcctcggacg gcctcaccta ctataaccgc tgctacatgg atgccgaggc 840
 ctgctccaaa ggcatcacac tggccgttgt aacctgccgc tatcacttca cctggcccaa 900

caccagcccc ccaccacctg agaccaccat gcaccccacc acagcctccc cagagacccc	960
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gggtgagaca gtgagcttcc tctgtgatgt ggtgggcccgg ccccgccctg agatcacctg	1080
ggagaagcag ttggaggatc gggagaatgt ggtcatgcgg cccaaccatg tgcgtggcaa	1140
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gatctacacc tgcacggccc ggaacgtggc tggggctcctg agggctgatt tcccgtgtc	1260
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ccacagaagg tctcagatca gcatctattc tttgggttca ataaggggtt catatctttt	2280
ttagctgagg gggacaagag gagaagtcag tggacacatg gaagttactc gtgaccacca	2340
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caggagaaat gaaggaagat gaaaaagcag gcagggaagg gggtagtaaa ggactgagag	3060
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cctcaggaac ccacgggaac ctggaaggag gctctttgtg agacctgggc aaaggatggg	3180
gcagctcgtc gatgattttt ttgtgtttcc aggcttcctg tgtgatcctg gccctccggc	3240
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tctgccctat tctgtccctg cccctccaag ctgaagaagg tccttggtgg gcgtcctcat	3360
ttcttcctca aatataagga ggaagatacc aattaaaagc tcatagtatc aactgcc	3417

<210> 35
 <211> 576
 <212> PRT
 <213> Homo Sapiens

<400> 35

Met	Trp	Ala	Pro	Arg	Cys	Arg	Arg	Phe	Trp	Ser	Arg	Trp	Glu	Gln	Val
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Ala	Ala	Leu	Leu	Leu	Leu	Leu	Leu	Leu	Gly	Val	Pro	Pro	Arg	Ser
		20					25					30		

Leu Ala Leu Pro Pro Ile Arg Tyr Ser His Ala Gly Ile Cys Pro Asn
35 40 45

Asp Met Asn Pro Asn Leu Trp Val Asp Ala Gln Ser Thr Cys Arg Arg
50 55 60

Glu Cys Glu Thr Asp Gln Glu Cys Glu Thr Tyr Glu Lys Cys Cys Pro
65 70 75 80

Asn Val Cys Gly Thr Lys Ser Cys Val Ala Ala Arg Tyr Met Asp Val
85 90 95

Lys Gly Lys Lys Gly Pro Val Gly Met Pro Lys Glu Ala Thr Cys Asp
100 105 110

His Phe Met Cys Leu Gln Gln Gly Ser Glu Cys Asp Ile Trp Asp Gly
115 120 125

Gln Pro Val Cys Lys Cys Lys Asp Arg Cys Glu Lys Glu Pro Ser Phe
130 135 140

Thr Cys Ala Ser Asp Gly Leu Thr Tyr Tyr Asn Arg Cys Tyr Met Asp
145 150 155 160

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

Tyr His Phe Thr Trp Pro Asn Thr Ser Pro Pro Pro Pro Glu Thr Thr
180 185 190

Met His Pro Thr Thr Ala Ser Pro Glu Thr Pro Glu Leu Asp Met Ala
195 200 205

Ala Pro Ala Leu Leu Asn Asn Pro Val His Gln Ser Val Thr Met Gly
210 215 220

Glu Thr Val Ser Phe Leu Cys Asp Val Val Gly Arg Pro Arg Pro Glu
225 230 235 240

Ile Thr Trp Glu Lys Gln Leu Glu Asp Arg Glu Asn Val Val Met Arg
245 250 255

Pro Asn His Val Arg Gly Asn Val Val Val Thr Asn Ile Ala Gln Leu
260 265 270

Val Ile Tyr Asn Ala Gln Leu Gln Asp Ala Gly Ile Tyr Thr Cys Thr
275 280 285

Ala Arg Asn Val Ala Gly Val Leu Arg Ala Asp Phe Pro Leu Ser Val
290 295 300

Val Arg Gly His Gln Ala Ala Ala Thr Ser Glu Ser Ser Pro Asn Gly
305 310 315 320

Thr Ala Phe Pro Ala Ala Glu Cys Leu Lys Pro Pro Asp Ser Glu Asp
325 330 335

Cys Gly Glu Glu Gln Thr Arg Trp His Phe Asp Ala Gln Ala Asn Asn
340 345 350

Cys Leu Thr Phe Thr Phe Gly His Cys His Arg Asn Leu Asn His Phe
355 360 365

Glu Thr Tyr Glu Ala Cys Met Leu Ala Cys Met Ser Gly Pro Leu Ala
370 375 380

Ala Cys Ser Leu Pro Ala Leu Gln Gly Pro Cys Lys Ala Tyr Ala Pro
385 390 395 400

Arg Trp Ala Tyr Asn Ser Gln Thr Gly Gln Cys Gln Ser Phe Val Tyr
405 410 415

Gly Gly Cys Glu Gly Asn Gly Asn Asn Phe Glu Ser Arg Glu Ala Cys
420 425 430

Glu Glu Ser Cys Pro Phe Pro Arg Gly Asn Gln Arg Cys Arg Ala Cys
435 440 445

Lys Pro Arg Gln Lys Leu Val Thr Ser Phe Cys Arg Ser Asp Phe Val
450 455 460

Ile Leu Gly Arg Val Ser Glu Leu Thr Glu Glu Pro Asp Ser Gly Arg
465 470 475 480

Ala Leu Val Thr Val Asp Glu Val Leu Lys Asp Glu Lys Met Gly Leu
485 490 495

Lys Phe Leu Gly Gln Glu Pro Leu Glu Val Thr Leu Leu His Val Asp
500 505 510

Trp Ala Cys Pro Cys Pro Asn Val Thr Val Ser Glu Met Pro Leu Ile
515 520 525

Ile Met Gly Glu Val Asp Gly Gly Met Ala Met Leu Arg Pro Asp Ser
530 535 540

Phe Val Gly Ala Ser Ser Ala Arg Arg Val Arg Lys Leu Arg Glu Val
545 550 555 560

Met His Lys Lys Thr Cys Asp Val Leu Lys Glu Phe Leu Gly Leu His
565 570 575

<210> 36
<211> 1521
<212> DNA
<213> Homo Sapiens

<400> 36
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cttggcattg ttggatttat gttcggaagc atgttccttc aagcagtgtt cagcagcccc 120
aagccagaac tcccaagtcc tgccccgggt gtccagaagc tgaagcttct gcctgaggaa 180
cgtctcagga acctcttttc ctacgatgga atctggctgt tcccgaataa tcagtgcaaa 240

tgtgaagcca acaaagagca gggagggttac aactttcagg atgcctatgg ccagagcgac	300
ctcccagcgg tgaaagcgag gagacaggct gaatttgaac actttcagag gagagaaggg	360
ctgccccgcc cactgcccct gctgggtccag cccaacctcc cttttgggta cccagtccac	420
ggagtggagg tgatgcccct gcacacgggt cccatcccag gcctccagtt tgaaggaccc	480
gatgcccccg tctatgaggt caccctgaca gcttctctgg ggacactgaa cacccttgct	540
gatgtcccag acagtgtggt gcagggcaga ggccagaagc agctgatcat ttctaccagt	600
gaccggaagc tgttgaagtt cattcttcag cacgtgacat acaccagcac ggggtaccag	660
caccagaagg tagacatagt gagtctggag tccaggctct cagtggccaa gtttccagtg	720
accatccgcc atcctgtcat acccaagcta tacgacctg gaccagagag gaagctcaga	780
aacctgggta ccattgctac caagactttc ctccgcccc acaagctcat gatcatgctc	840
cggagtattc gagagtatta cccagacttg accgtaatag tggctgatga cagccagaag	900
cccctggaaa ttaaagacaa ccacgtggag tattacacta tgccctttgg gaagggttgg	960
tttgctggta ggaacctggc catatctcag gtcaccacca aatacgttct ctgggtggac	1020
gatgattttc tcttcaacga ggagaccaag attgaggtgc tgggtgatgt cctggagaaa	1080
acagaactgg acgtggtagg cggcagtgtg ctgggaaatg tgttccagtt taagttgttg	1140
ctggaacaga gtgagaatgg ggcctgcctt cacaagagga tgggattttt ccaacccctg	1200
gatggcttcc ccagctgcgt ggtgaccagt ggcgtgggtca acttcttcct ggcccacacg	1260
gagcgactcc aaagagttagg ctttgatccc cgctgcaac gagtgggtca ctcagaattc	1320
ttcattgatg ggctagggac cctactcgtg gggtcatgcc cagaagtgat tataggtcac	1380
cagtctcggg ctccagtggg ggactcagaa ctggctgccc tagagaagac ctacaataca	1440
taccgggtcca acaccctcac ccgggtccag ttcaagctgg ccctccacta cttcaagaac	1500
catctccaat gtgccgata a	1521

<210> 37
 <211> 506
 <212> PRT
 <213> Homo Sapiens

<400> 37

Met Thr Ser Gly Gly Ser Arg Phe Leu Trp Leu Leu Lys Ile Leu Val
1 5 10 15

Ile Ile Leu Val Leu Gly Ile Val Gly Phe Met Phe Gly Ser Met Phe
20 25 30

Leu Gln Ala Val Phe Ser Ser Pro Lys Pro Glu Leu Pro Ser Pro Ala
35 40 45

Pro Gly Val Gln Lys Leu Lys Leu Leu Pro Glu Glu Arg Leu Arg Asn
50 55 60

Leu Phe Ser Tyr Asp Gly Ile Trp Leu Phe Pro Lys Asn Gln Cys Lys
65 70 75 80

Cys Glu Ala Asn Lys Glu Gln Gly Gly Tyr Asn Phe Gln Asp Ala Tyr
85 90 95

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

Glu His Phe Gln Arg Arg Glu Gly Leu Pro Arg Pro Leu Pro Leu Leu
115 120 125

Val Gln Pro Asn Leu Pro Phe Gly Tyr Pro Val His Gly Val Glu Val
130 135 140

Met Pro Leu His Thr Val Pro Ile Pro Gly Leu Gln Phe Glu Gly Pro
145 150 155 160

Asp Ala Pro Val Tyr Glu Val Thr Leu Thr Ala Ser Leu Gly Thr Leu
165 170 175

Asn Thr Leu Ala Asp Val Pro Asp Ser Val Val Gln Gly Arg Gly Gln
180 185 190

Lys Gln Leu Ile Ile Ser Thr Ser Asp Arg Lys Leu Leu Lys Phe Ile
195 200 205

Leu Gln His Val Thr Tyr Thr Ser Thr Gly Tyr Gln His Gln Lys Val
210 215 220

Asp Ile Val Ser Leu Glu Ser Arg Ser Ser Val Ala Lys Phe Pro Val
225 230 235 240

Thr Ile Arg His Pro Val Ile Pro Lys Leu Tyr Asp Pro Gly Pro Glu
245 250 255

Arg Lys Leu Arg Asn Leu Val Thr Ile Ala Thr Lys Thr Phe Leu Arg
260 265 270

Pro His Lys Leu Met Ile Met Leu Arg Ser Ile Arg Glu Tyr Tyr Pro
275 280 285

Asp Leu Thr Val Ile Val Ala Asp Asp Ser Gln Lys Pro Leu Glu Ile
290 295 300

Lys Asp Asn His Val Glu Tyr Tyr Thr Met Pro Phe Gly Lys Gly Trp
305 310 315 320

Phe Ala Gly Arg Asn Leu Ala Ile Ser Gln Val Thr Thr Lys Tyr Val
325 330 335

Leu Trp Val Asp Asp Asp Phe Leu Phe Asn Glu Glu Thr Lys Ile Glu
340 345 350

Val Leu Val Asp Val Leu Glu Lys Thr Glu Leu Asp Val Val Gly Gly
355 360 365

Ser Val Leu Gly Asn Val Phe Gln Phe Lys Leu Leu Leu Glu Gln Ser
370 375 380

Glu Asn Gly Ala Cys Leu His Lys Arg Met Gly Phe Phe Gln Pro Leu
385 390 395 400

Asp Gly Phe Pro Ser Cys Val Val Thr Ser Gly Val Val Asn Phe Phe
405 410 415

Leu Ala His Thr Glu Arg Leu Gln Arg Val Gly Phe Asp Pro Arg Leu
420 425 430

Gln Arg Val Ala His Ser Glu Phe Phe Ile Asp Gly Leu Gly Thr Leu
435 440 445

Leu Val Gly Ser Cys Pro Glu Val Ile Ile Gly His Gln Ser Arg Ser
450 455 460

Pro Val Val Asp Ser Glu Leu Ala Ala Leu Glu Lys Thr Tyr Asn Thr
465 470 475 480

Tyr Arg Ser Asn Thr Leu Thr Arg Val Gln Phe Lys Leu Ala Leu His
485 490 495

Tyr Phe Lys Asn His Leu Gln Cys Ala Ala
500 505

<210> 38
<211> 7887
<212> DNA
<213> Homo Sapiens

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