

Listado de secuencias

<110> LLC "ANABION"

<120> Ácido nucleico optimizado por codones que codifica la proteína del factor de coagulación IX, y uso del mismo

<150> RU2021105703

<151> 2021-03-05

<160> 17

<170> BiSSAP 1.3.6

<210> 1

<211> 461

<212> PRT

<213> Secuencia natural

<220>

<223> Secuencia de aminoácidos de la proteína del factor de coagulación IX recombinante con la mutación R338L

<400> 1

Met Gln Arg Val Asn Met Ile Met Ala Glu Ser Pro Gly Leu Ile Thr

1. 5 10 15

Ile Cys Leu Leu Gly Tyr Leu Leu Ser Ala Glu Cys Thr Val Phe Leu

20 25 30

Asp His Glu Asn Ala Asn Lys Ile Leu Asn Arg Pro Lys Arg Tyr Asn

35 40 45

Ser Gly Lys Leu Glu Glu Phe Val Gln Gly Asn Leu Glu Arg Glu Cys

50 55 60

Met Glu Glu Lys Cys Ser Phe Glu Glu Ala Arg Glu Val Phe Glu Asn

65 70 75 80

Thr Glu Arg Thr Thr Glu Phe Trp Lys Gln Tyr Val Asp Gly Asp Gln

85 90 95

Cys Glu Ser Asn Pro Cys Leu Asn Gly Gly Ser Cys Lys Asp Asp Ile

100 105 110

Asn Ser Tyr Glu Cys Trp Cys Pro Phe Gly Phe Glu Gly Lys Asn Cys

115 120 125

Glu Leu Asp Val Thr Cys Asn Ile Lys Asn Gly Arg Cys Glu Gln Phe

130 135 140

Cys Lys Asn Ser Ala Asp Asn Lys Val Val Cys Ser Cys Thr Glu Gly

145 150 155 160

Tyr Arg Leu Ala Glu Asn Gln Lys Ser Cys Glu Pro Ala Val Pro Phe

165 170 175

Pro Cys Gly Arg Val Ser Val Ser Gln Thr Ser Lys Leu Thr Arg Ala

180 185 190

Glu Thr Val Phe Pro Asp Val Asp Tyr Val Asn Ser Thr Glu Ala Glu

195 200 205

Thr Ile Leu Asp Asn Ile Thr Gln Ser Thr Gln Ser Phe Asn Asp Phe

210 215 220

Thr Arg Val Val Gly Gly Glu Asp Ala Lys Pro Gly Gln Phe Pro Trp

225 230 235 240

Gln Val Val Leu Asn Gly Lys Val Asp Ala Phe Cys Gly Gly Ser Ile

245	250	255
Val Asn Glu Lys	Trp Ile Val Thr	Ala Ala His Cys Val Glu Thr Gly
260	265	270
Val Lys Ile Thr	Val Val Ala Gly	Glu His Asn Ile Glu Glu Thr Glu
275	280	285
His Thr Glu Gln	Lys Arg Asn Val	Ile Arg Ile Ile Pro His His Asn
290	295	300
Tyr Asn Ala Ala	Ile Asn Lys Tyr	Asn His Asp Ile Ala Leu Leu Glu
305	310	315
Leu Asp Glu Pro	Leu Val Leu Asn Ser	Tyr Val Thr Pro Ile Cys Ile
325	330	335
Ala Asp Lys Glu	Tyr Thr Asn Ile	Phe Leu Lys Phe Gly Ser Gly Tyr
340	345	350
Val Ser Gly Trp	Gly Arg Val Phe	His Lys Gly Arg Ser Ala Leu Val
355	360	365
Leu Gln Tyr Leu	Arg Val Pro Leu	Val Asp Arg Ala Thr Cys Leu Leu
370	375	380
Ser Thr Lys Phe	Thr Ile Tyr Asn	Asn Met Phe Cys Ala Gly Phe His
385	390	395
Glu Gly Gly Arg	Asp Ser Cys Gln	Gly Asp Ser Gly Gly Pro His Val
405	410	415
Thr Glu Val Glu	Gly Thr Ser Phe	Leu Thr Gly Ile Ile Ser Trp Gly
420	425	430
Glu Glu Cys Ala	Met Lys Gly Lys	Tyr Gly Ile Tyr Thr Lys Val Ser
435	440	445
Arg Tyr Val Asn	Trp Ile Lys Glu	Lys Thr Lys Leu Thr
450	455	460

<210> 2

<211> 1383

<212> ADN

<213> Secuencia artificial

<220>
 <223> Ácido nucleico optimizado por codones que codifica el factor IX de coagulación sanguínea recombinante (variante hFIXco-v1)

<400> 2

atgcagcggg tcaacatgat catggcggag tcgccgggcc tgatcacgat ctgcctctc	60
gggtacctgc tctccgccga gtgcaccgtg ttctggacc acgagaacgc caacaagatc	120
ctcaaccggc ccaagcgcta caactccggc aagctggagg agttcgtgca ggggaacctc	180
gagcgcgagt gcatggagga gaagtgctcg ttcgaggagg cgcgggaggt gttcgagAAC	240
accgagcgca ccacggagtt ctggaagcag tacgtggacg gggaccagtg cgagtcgaac	300
ccgtgcctca acgggggggtc gtgcaaggac gacatcaact cgtacgagtg ctggtgcccc	360
ttcggcttcg agggcaagaa ctgcgagctg gacgtgacct gcaacatcaa gaacgggCGC	420
tgcgagcagt tctgcaagaa cagcgccgac aacaaggTgG tctgtcctg caccgagggg	480
taccgcctcg cggagaacca gaagtctgc gagccggccg tgcccttccc ctgcggccgc	540

gtgtccgtca gccagacgtc gaagctgacg cgcgccgaga ccgtcttccc ggacgtggac 600
 tacgtgaact cgacggaggc cgagaccatc ctggacaaca tcaccagag caccagttcc 660
 ttcaacgact tcacgcgggt ggtcggcggc gaggacgcca agcccgggca gttcccgtgg 720
 caggctgtcc tcaacgggaa ggtcgacgcg ttctgcggcg ggagcatcgt gaacgagaag 780
 tggatcgtga ccgccgcgca ctgcgtcgag acgggctgta agatcaccgt ggtggccggg 840
 gagcacaaca tcgaggagac ggagcacacc gagcagaagc ggaacgtgat ccgcatcatc 900
 ccgcaccaca actacaacgc cgccatcaac aagtacaacc acgacatcgc gctcctcgag 960
 ctggacgagc cgctgggtct caactcctac gtcacgccga tctgcatcgc cgacaaggag 1020
 tacacgaaca tttcctgaa gttcgggagc ggctacgtct cgggctgggg ccgcgtgttc 1080
 cacaaggggc gcagcgcgct cgtgctccag tacctgcggg tcccctgggt cgaccgcgcg 1140
 acctgcctcc tctccacgaa gttcacgata tacaacaaca tgttctgcgc ggggttcac 1200
 gagggcgcc gccacagctg ccagggcgac agcgggggcc cgcacgtgac ggaggtggag 1260
 ggcacgagct tctgaccgg gatcatctcg tggggcgagg agtgcgcgat gaaggggaag 1320
 tacggcatct acaccaaggt cagccgttac gtgaactgga tcaaggagaa gacgaagctg 1380
 acg 1383

<210> 3

<211> 3122

<212> ADN

<213> Secuencia artificial

<220>

<223> casete de expresión con el gen del factor de coagulación IX recombinante optimizado por el codón (variante AAV5-hFIXco-v1)

<400> 3

cctgcaggca gctgcgcgct cgctcgtca ctgaggccgc ccgggcgtcg ggcgacctt 60
 ggtcggcccg cctcagttag cgagcgagcg cgagagagg gaggggccaa ctccatcact 120
 aggggttctt gcggccgcac gcgtgccgcc accatggtcg agcttgggct gcaggctgag 180
 ggcactggga ggaatgttag taagatggaa aactactgat gacccttgca gagacagagt 240
 attaggacat gtttgaacag gggccggcga tcagcaggta gctctagagg atccccgtct 300
 gtctgcacat ttcgtagagc gagggttccg atactctaata ctccctaggc aaggttcata 360
 tttgttagg ttacttattc tcctttgtt gactaagtca ataatacagaa tcagcagggt 420
 tggagtcagc ttggcaggga tcagcagcct ggggttgaag gaggggggtat aaaagcccct 480

tcaccaggag aagccgtcac acagatccac aagctcctga caggaagctc taggtgactc 540
 tcttaaggta gcctccgcgg attcgaatcc cggccgggaa cggtgcattg gaacgcggat 600
 tccccgtgcc aagagtgcg taagtaccgc ctatagagtc tataggccca caaaaaatgc 660
 tttctcttt taatatactt tttgtttat cttatttcta atactttccc taatctctt 720
 ctttcagggc aataatgata caatgtatca tgcctctttg caccattcta aagaataaca 780
 gtgataattt ctgggttaag gcaatagcaa tatttctgca tataaatatt tctgcatata 840
 aattgtaact gatgtaagag gtttcatatt gctaatagca gctacaatcc agctaccatt 900
 ctgcttttat ttatgggtg ggataaggct ggattattct gagtccaagc taggccctt 960
 tgctaatacat gttcatacct cttatcttcc tcccacagct cctgggcaac gtgctggtct 1020
 gtgtgctggc ccatcacttt ggcaaagaat tgggattcga acatcgatat gcagcgggctc 1080
 aacatgatca tggcggagtc gccgggcctg atcacgatct gcctctcgg gtacctgctc 1140
 tccgccgagt gcaccgtgtt cctggaccac gagaacgcca acaagatcct caaccggccc 1200
 aagcgctaca actccggcaa gctggaggag ttcgtgcagg ggaacctcga gcgcgagtg 1260
 atggaggaga agtgctcggt cgaggaggcg cgggaggtgt tcgagaacac cgagcgcacc 1320
 acggagttct ggaagcagta cgtggacggg gaccagtgcg agtcgaaccc gtgcctcaac 1380
 ggggggtcgt gcaaggacga catcaactcg tacgagtgcg ggtgcccctt cggcttcgag 1440
 ggcaagaact gcgagctgga cgtgacctgc aacatcaaga acgggcgctg cgagcagttc 1500
 tgcaagaaca gcgccgacaa caaggtggc tgctcctgca ccgaggggta ccgcctcgcg 1560
 gagaaccaga agtctcgca gccggccgtg ccctcccct gcggccgctg gtccgtcagc 1620
 cagacgtcga agctgacgcg cgccgagacc gtcttcccgg acgtggacta cgtgaactcg 1680
 acggaggccg agaccatcct ggacaacatc accagagca ccagtcctt caacgacttc 1740
 acgcgggtgg tcggcggcga ggacccaag cccgggcagt tccgtggca ggtcgtctc 1800
 aacgggaagg tcgacgcgtt ctgcggcggg agcatcgtga acgagaagtg gatcgtgacc 1860
 gccgcgcact gcgtcgagac gggcgtgaag atcaccgtgg tggccgggga gcacaacatc 1920
 gaggagacgg agcacaccga gcagaagcgg aacgtgatcc gcatcatccc gcaccacaac 1980
 tacaacgccg ccatcaacaa gtacaaccac gacatcgcg tcctcgagct ggacgagccg 2040
 ctggtcctca actctacgt cacgccgatc tgcatcgccg acaaggagta cacgaacatc 2100
 ttctgaagt tcgggagcgg ctacgtctcg ggctggggcc gcgtgttcca caaggggcgc 2160

agcgcgctcg tgctccagta cctgcggggtc ccctgggtcg accgcgcgac ctgcctcctc 2220
 tccacgaagt tcacgatcta caacaacatg ttctgcgcgg ggttcacga gggcggccgg 2280
 gacagctgcc agggcgacag cgggggccccg cacgtgacgg aggtggaggg cacgagcttc 2340
 ctgaccggga tcattctgtg gggcgaggag tgcgcgatga aggggaagta cggcatctac 2400
 accaaggta gccgggtacgt gaactggatc aaggagaaga cgaagctgac gtgatgaaga 2460
 tctacgggtg gcatccctgt gaccctccc cagtggcctt cctggcctg gaagttgcca 2520
 ctccagtgcc caccagcctt gtctaataa aattaagttg catcattttg tctgactagg 2580
 tgtccttcta taatattatg ggggtggaggg ggggtggtatg gagcaagggg caagttggga 2640
 agacaacctg tagggcctgc ggggtctatt gggaaccaag ctggagtga gtggcacaat 2700
 ctgggtcac tgcaatctcc gcctcctggg ttcaagcgat tctcctgcct cagcctcccg 2760
 agttgttggg attccaggca tgcattacca ggctcagcta attttgttt ttttggtaga 2820
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 caccttgcc tccaaattg ctgggattac aggcgtgaac cactgctcc ttcctgtcc 2940
 ttctgatttt gtaggaacc acgtgcggac cgagcggccg caggaacccc tagtgatgga 3000
 gttggccact ccctctctgc gcgctcgtc gctcactgag gccgggacgac caaaggtcgc 3060
 ccgacgcccg ggctttgccc gggcggcctc agtgagcgag cgagcgcgca gctgcctgca 3120
 gg 3122

<210> 4
 <211> 1383
 <212> ADN
 <213> Secuencia artificial

<220>
 <223> Ácido nucleico optimizado en codones que codifica el factor IX de coagulación sanguínea recombinante (variante hFIXco-v2)

<400> 4
 atgcagcggg tgaacatgat catggccgag tccccaggcc tgattaccat ctgtctgctg 60
 ggctacctgc tgagcgccga atgcaccgtg ttctggacc acgagaacgc caacaagatc 120
 ctgaaccgcc ctaagcggta caactccggc aagctggagg agtttggtgca gggcaatctg 180
 gagcgggagt gtatggagga gaagtgcagc ttcgaggagg ccaggagggt gttcgagaac 240
 accgagagga ccaccgagtt ctggaagcag tatgtggacg gcgaccagtg cgagtctaata 300
 cctgtctgta atggcgggag ctgcaaggac gacatcaaca gctacgagtg ctggtgcctt 360

ttcggcttcg agggcaagaa ttgcgagctg gacgtgacct gcaacatcaa gaacggccgg 420
tgtgagcagt tctgcaagaa cagcgccgac aacaagggtg tgtgctcctg caccgaggga 480
tacaggctgg ccgagaatca gaagagctgt gagcccgcg tgccattccc ctgtggcaga 540
gtgtctgtga gccagaccag caagctgacc agagccgaga ccgtgtttcc agacgtggac 600
tacgtgaaca gcaccgaggc cgagaccatc ctggataata tcaccagtc caccagagc 660
ttcaacgact tcaccagagt ggtgggaggc gaggatgcc aagccaggaca gtttcctgg 720
caggtggtgc tgaatggcaa ggtggacgcc ttctgcggag gcagcatcgt gaacgagaag 780
tggattgtga ccgagccca ctgctggag actggcgtga agattaccgt ggtcgccggc 840
gagcacaata tcgaagagac cgagcacacc gagcagaagc gcaacgtgat ccggatcatc 900
cctcaccaca actacaacgc agccatcaac aagtacaacc acgacatcgc cctgctggag 960
ctggacgagc cactggtgct gaactcttac gtgacccta tctgcatcgc cgacaaggag 1020
tacaccaaca tcttctgaa gttcggcagc ggctacgtga gcggatggg cagagtgtt 1080
cacaagggca ggagcgccct ggtgctgcag tatctgagag tgccactggt ggacagagct 1140
acctgctgc tgagcaccaa gttcaccatc tacaacaaca tgttctgccc cggttccac 1200
gaggggggaa gagactcttg ccagggcgat tccggcggac cacacgtgac cgaagtggag 1260
ggcaccagct tctgaccgg catcatctcc tggggcgagg aatgcgcat gaagggcaag 1320
tacggcatct acaccaaggt gagcaggtac gtgaactgga tcaaggagaa gaccaagctg 1380
acc 1383

<210> 5
<211> 3123
<212> ADN
<213> Secuencia artificial

<220>
<223> casete de expresión con un gen optimizado en codones para el factor IX de coagulación sanguínea recombinante (variante AAV5-hFIXco-v2)

<400> 5
cctgcaggca gctgcgcgct cgctcgctca ctgaggccgc ccgggcgtcg ggcgacctt 60
ggtcgcccgg cctcagtgag cgagcgagcg cgagagagg gagtggccaa ctccatcact 120
aggggttcct gcggccgcac gcgtgccgcc accatggctg agcttgggct gcaggtcgag 180
ggcactggga ggatgttgag taagatggaa aactactgat gacccttgca gagacagagt 240

attaggacat gtttgaacag gggccggcga tcagcaggta gctctagagg atccccgtct 300
 gtctgcacat ttcgtagagc gagtggtccg atactcta atccttaggc aaggttcata 360
 tttgtgtagg ttacttattc tcctttgtt gactaagtca ataatcagaa tcagcagggt 420
 tggagtcagc ttggcaggga tcagcagcct ggggtggaag gaggggggtat aaaagcccct 480
 tcaccaggag aagccgtcac acagatccac aagctcctga caggaagctc taggtgactc 540
 tcttaaggta gcctccgagg attcgaatcc cggccgggaa cgggtgcattg gaacgcggat 600
 tccccgtgcc aagagtgcg taagtaccgc ctatagagtc tataggccca caaaaaatgc 660
 tttctcttt taatatactt tttgtttat ctatttcta atactttccc taatctctt 720
 cttcagggc aataatgata caatgtatca tgcctcttg caccattcta aagaataaca 780
 gtgataattt ctgggttaag gcaatagcaa ttttctgca tataaatatt tctgcatata 840
 aattgtaact gatgtaagag gtttcatatt gctaatagca gctacaatcc agctaccatt 900
 ctgctttat tttatgggtg ggataaggct ggattattct gagtccaagc taggccctt 960
 tgctaatacat gttcatacct ctatcttcc tcccacagct cctgggcaac gtgctggtct 1020
 gtgtgctggc ccatcactt ggcaaagaat tgggattcga acatcgatat aaatgcagcg 1080
 ggtgaacatg atcatggccg agtccccagg cctgattacc atctgtctgc tgggctacct 1140
 gctgagcgcc gaatgcaccg tgtttctgga ccacgagaac gccaacaaga tcctgaaccg 1200
 ccctaagcgg tacaactccg gcaagctgga ggagttgtg cagggaatc tggagcggga 1260
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 gaccaccgag ttctggaagc agtatgtgga cggcgaccag tgcgagtcta atcctgtct 1380
 gaatggcggg agctgcaagg acgacatcaa cagctacgag tgctggtgcc cttcggctt 1440
 cgagggaag aattgcgagc tggacgtgac ctgcaacatc aagaacggcc ggtgtgagca 1500
 gttctgcaag aacagcgccg acaacaagg ggtgtgctcc tgcaccgagg gatacaggct 1560
 ggccgagaat cagaagagct gtgagccgc cgtgccattc ccctgtggca gagtgtctgt 1620
 gagccagacc agcaagctga ccagagccga gaccgtgtt ccagacgtgg actacgtgaa 1680
 cagcaccgag gccgagacca tcctggataa tatcaccag tccaccaga gttcaacga 1740
 cttcaccaga gtggtgggag gcgaggatgc caagccagga cagtttcct ggcagggtgt 1800
 gctgaatggc aaggtggacg cttctgcgg aggcagcatc gtgaacgaga agtggattgt 1860
 gaccgcagcc cactgcgtgg agactggcgt gaagattacc gtggtcgccg gcgagcacia 1920
 tatcgaagag accgagcaca ccgagcagaa gcgcaacgtg atccggatca tccctacca 1980

caactacaac gcagccatca acaagtacaa ccacgacatc gccctgctgg agctggacga 2040
gccactggtg ctgaactctt acgtgacccc tatctgcatc gccgacaagg agtacaccaa 2100
catcttctctg aagttcggca gcggtctacgt gagcggatgg ggcagagtgt ttcacaaggg 2160
caggagcgcc ctggtgctgc agtatctgag agtgccactg gtggacagag ctacctgcct 2220
gctgagcacc aagttcacca tctacaacaa catgttctgc gccggcttc acgagggggg 2280
aagagactct tgccagggcg attccggcg accacacgtg accgaagtgg agggcaccag 2340
cttctgacc ggcacatct cctggggcga ggaatgcgcc atgaaggga agtacggcat 2400
ctacaccaag gtgagcaggt acgtgaactg gatcaaggag aagaccaagc tgacctgaag 2460
atctacgggt ggcacccctg tgacccctcc ccagtgcgcc tctggccct ggaagttgcc 2520
actccagtgc ccaccagcct tgtcctaata aaattaagtt gcatcattt gtctgactag 2580
gtgtcttct ataataattat ggggtggagg ggggtggtat ggagcaagg gcaagttggg 2640
aagacaacct gtagggcctg cgggtctat tgggaaccaa gctggagtgc agtggcaca 2700
tcttggtca ctgcaatctc cgctctctgg gttcaagcga ttctctgcc tcagcctccc 2760
gagttgttg gattccaggc atgcatgacc aggctcagct aattttgtt ttttggtag 2820
agacgggggt tcacatatt ggccaggctg gtctccaact cctaattca ggtgatctac 2880
ccacctggc ctccaaatt gctgggatta caggcgtgaa cactgctcc cttcctgtc 2940
cttctgatt tgtaggtaac cacgtgcgga ccgagcggcc gcaggaacc ctagtgatgg 3000
agttggccac tccctctctg cgcgctcgt cgctcactga ggccgggcga ccaaaggctc 3060
cccagcggc gggcttgcc cgggcggcct cagtgagcga gcgagcgcgc agctgcctgc 3120
agg 3123

<210> 6
<211> 130
<212> ADN
<213> Secuencia artificial

<220>
<223> ITR (repeticiones terminales invertidas) de lado izquierdo (primera)

<400> 6
cctgcaggca gctgcgcgt cgctcgtca ctgaggccgc ccgggcgtcg ggcgacctt 60
ggtcgcccgg cctcagttag cgagcgagcg cgcagagagg gagtggccaa ctccatcact 120
aggggttcct 130

<210> 7
<211> 372
<212> ADN
<213> Secuencia natural

<220>
<223> Secuencia promotora de la TTR (promotor de la transtiretina)

<400> 7
tcgagcttgg gctgcaggtc gagggcactg ggaggatgtt gagtaagatg gaaaactact 60
gatgaccctt gcagagacag agtattagga catgtttgaa caggggccgg cgatcagcag 120
gtagctctag aggatccccg tctgtctgca catttcgtag agcgagtgtt ccgatactct 180
aatctcccta ggcaagggtc atatttgtgt aggttactta ttctccttt gttgactaag 240
tcaataatca gaatcagcag gtttgagatc agcttggcag ggatcagcag cctgggttgg 300
aaggaggggg tataaaagcc ccttcaccag gagaagccgt cacacagatc cacaagctcc 360
tgacaggaag ct 372

<210> 8
<211> 493
<212> ADN
<213> Secuencia natural

<220>
<223> intrón del gen hBG1 (un fragmento del gen de la β globina humana que lleva el intrón)

<400> 8
cgaatcccgg ccgggaacgg tgcattggaa cgcgattcc ccgtgccaag agtgacgtaa 60
gtaccgccta tagagtctat agggccacaa aaaatgcttt cttcttttaa tatactttt 120
tgtttatctt atttctaata cttccctaa tctcttctt tcagggaat aatgatacaa 180
tgtatcatgc ctcttgcac cattctaaag aataacagtg ataatttctg ggtaaggca 240
atagcaatat ttctgcatat aaatatttct gcatataaat tgtaactgat gtaagaggtt 300
tcatattgct aatagcagct acaatccagc taccattctg cttttattt atggttggga 360
taaggctgga ttattctgag tccaagctag gccctttgc taatcatgtt catacctctt 420
atcttctcc cacagctctt gggcaacgtg ctggtctgtg tgctggcca tcactttggc 480
aaagaattgg gat 493

<210> 9

<211> 479
<212> ADN
<213> Secuencia natural

<220>
<223> Señal de poliadenilación hGH1 (señal de poliadenilación del gen de la hormona de crecimiento humana)

<400> 9
acgggtggca tccctgtgac ccctccccag tgccctcct gccctggaa gttgccactc 60
cagtgtccac cagcctgtc ctaataaaat taagttgcat cattttgtct gactaggtgt 120
ccttctataa tattatgggg tggagggggg tggtatggag caaggggcaa gttgggaaga 180
caacctgtag ggctgcggg gtctattggg aaccaagctg gagtgagtg gcacaatctt 240
ggctcactgc aatctccgcc tctgggttc aagcgattct cctgcctcag cctcccagat 300
tgttgggatt ccaggcatgc atgaccaggc tcagctaatt ttgtttttt tggtagagac 360
ggggtttcac catattggcc aggtgtgtct ccaactccta atctcagtg atctaccac 420
cttggcctcc caaattgctg ggattacagg cgtgaaccac tgctccctc cctgtcctt 479

<210> 10
<211> 141
<212> ADN
<213> Secuencia artificial

<220>
<223> ITR derecho (segundo)

<400> 10
aggaaccct agtgatggag ttggccactc cctctctgcg cgctcgctcg ctactgagg 60
ccgggcgacc aaaggctgcc cgacggccgg gctttgcccg ggcggcctca gtgagcgagc 120
gagcgcgag ctgcctgcag g 141

<210> 11
<211> 724
<212> PRT
<213> Secuencia natural

<220>
<223> Secuencia natural de la proteína VP1 de la cápside del AAV5 de tipo salvaje

<400> 11
Met Ser Phe Val Asp His Pro Pro Asp Trp Leu Glu Glu Val Gly Glu
1. 5 10 15
Gly Leu Arg Glu Phe Leu Gly Leu Glu Ala Gly Pro Pro Lys Pro Lys

20	25	30	
Pro Asn Gln Gln His Gln Asp Gln Ala Arg Gly Leu Val Leu Pro Gly			
35	40	45	
Tyr Asn Tyr Leu Gly Pro Gly Asn Gly Leu Asp Arg Gly Glu Pro Val			
50	55	60	
Asn Arg Ala Asp Glu Val Ala Arg Glu His Asp Ile Ser Tyr Asn Glu			
65	70	75	80
Gln Leu Glu Ala Gly Asp Asn Pro Tyr Leu Lys Tyr Asn His Ala Asp			
85	90	95	
Ala Glu Phe Gln Glu Lys Leu Ala Asp Asp Thr Ser Phe Gly Gly Asn			
100	105	110	
Leu Gly Lys Ala Val Phe Gln Ala Lys Lys Arg Val Leu Glu Pro Phe			
115	120	125	
Gly Leu Val Glu Glu Gly Ala Lys Thr Ala Pro Thr Gly Lys Arg Ile			
130	135	140	
Asp Asp His Phe Pro Lys Arg Lys Lys Ala Arg Thr Glu Glu Asp Ser			
145	150	155	160
Lys Pro Ser Thr Ser Ser Asp Ala Glu Ala Gly Pro Ser Gly Ser Gln			
165	170	175	
Gln Leu Gln Ile Pro Ala Gln Pro Ala Ser Ser Leu Gly Ala Asp Thr			
180	185	190	
Met Ser Ala Gly Gly Gly Gly Pro Leu Gly Asp Asn Asn Gln Gly Ala			
195	200	205	
Asp Gly Val Gly Asn Ala Ser Gly Asp Trp His Cys Asp Ser Thr Trp			
210	215	220	
Met Gly Asp Arg Val Val Thr Lys Ser Thr Arg Thr Trp Val Leu Pro			
225	230	235	240
Ser Tyr Asn Asn His Gln Tyr Arg Glu Ile Lys Ser Gly Ser Val Asp			
245	250	255	
Gly Ser Asn Ala Asn Ala Tyr Phe Gly Tyr Ser Thr Pro Trp Gly Tyr			
260	265	270	
Phe Asp Phe Asn Arg Phe His Ser His Trp Ser Pro Arg Asp Trp Gln			
275	280	285	
Arg Leu Ile Asn Asn Tyr Trp Gly Phe Arg Pro Arg Ser Leu Arg Val			
290	295	300	
Lys Ile Phe Asn Ile Gln Val Lys Glu Val Thr Val Gln Asp Ser Thr			
305	310	315	320
Thr Thr Ile Ala Asn Asn Leu Thr Ser Thr Val Gln Val Phe Thr Asp			
325	330	335	
Asp Asp Tyr Gln Leu Pro Tyr Val Val Gly Asn Gly Thr Glu Gly Cys			
340	345	350	
Leu Pro Ala Phe Pro Pro Gln Val Phe Thr Leu Pro Gln Tyr Gly Tyr			
355	360	365	
Ala Thr Leu Asn Arg Asp Asn Thr Glu Asn Pro Thr Glu Arg Ser Ser			
370	375	380	
Phe Phe Cys Leu Glu Tyr Phe Pro Ser Lys Met Leu Arg Thr Gly Asn			
385	390	395	400
Asn Phe Glu Phe Thr Tyr Asn Phe Glu Glu Val Pro Phe His Ser Ser			
405	410	415	
Phe Ala Pro Ser Gln Asn Leu Phe Lys Leu Ala Asn Pro Leu Val Asp			
420	425	430	
Gln Tyr Leu Tyr Arg Phe Val Ser Thr Asn Asn Thr Gly Gly Val Gln			
435	440	445	
Phe Asn Lys Asn Leu Ala Gly Arg Tyr Ala Asn Thr Tyr Lys Asn Trp			
450	455	460	
Phe Pro Gly Pro Met Gly Arg Thr Gln Gly Trp Asn Leu Gly Ser Gly			
465	470	475	480

Val Asn Arg Ala Ser Val Ser Ala Phe Ala Thr Thr Asn Arg Met Glu
 485 490 495
 Leu Glu Gly Ala Ser Tyr Gln Val Pro Pro Gln Pro Asn Gly Met Thr
 500 505 510
 Asn Asn Leu Gln Gly Ser Asn Thr Tyr Ala Leu Glu Asn Thr Met Ile
 515 520 525
 Phe Asn Ser Gln Pro Ala Asn Pro Gly Thr Thr Ala Thr Tyr Leu Glu
 530 535 540
 Gly Asn Met Leu Ile Thr Ser Glu Ser Glu Thr Gln Pro Val Asn Arg
 545 550 555 560
 Val Ala Tyr Asn Val Gly Gly Gln Met Ala Thr Asn Asn Gln Ser Ser
 565 570 575
 Thr Thr Ala Pro Ala Thr Gly Thr Tyr Asn Leu Gln Glu Ile Val Pro
 580 585 590
 Gly Ser Val Trp Met Glu Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp
 595 600 605
 Ala Lys Ile Pro Glu Thr Gly Ala His Phe His Pro Ser Pro Ala Met
 610 615 620
 Gly Gly Phe Gly Leu Lys His Pro Pro Pro Met Met Leu Ile Lys Asn
 625 630 635 640
 Thr Pro Val Pro Gly Asn Ile Thr Ser Phe Ser Asp Val Pro Val Ser
 645 650 655
 Ser Phe Ile Thr Gln Tyr Ser Thr Gly Gln Val Thr Val Glu Met Glu
 660 665 670
 Trp Glu Leu Lys Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln
 675 680 685
 Tyr Thr Asn Asn Tyr Asn Asp Pro Gln Phe Val Asp Phe Ala Pro Asp
 690 695 700
 Ser Thr Gly Glu Tyr Arg Thr Thr Arg Pro Ile Gly Thr Arg Tyr Leu
 705 710 715 720
 Thr Arg Pro Leu

<210> 12

<211> 588

<212> PRT

<213> Secuencia natural

<220>

<223> Secuencia natural de la proteína VP2 de la cápside del AAV5 de tipo salvaje

<400> 12

Thr Ala Pro Thr Gly Lys Arg Ile Asp Asp His Phe Pro Lys Arg Lys
 1. 5 10 15
 Lys Ala Arg Thr Glu Glu Asp Ser Lys Pro Ser Thr Ser Ser Asp Ala
 20 25 30
 Glu Ala Gly Pro Ser Gly Ser Gln Gln Leu Gln Ile Pro Ala Gln Pro
 35 40 45
 Ala Ser Ser Leu Gly Ala Asp Thr Met Ser Ala Gly Gly Gly Gly Pro
 50 55 60
 Leu Gly Asp Asn Asn Gln Gly Ala Asp Gly Val Gly Asn Ala Ser Gly
 65 70 75 80
 Asp Trp His Cys Asp Ser Thr Trp Met Gly Asp Arg Val Val Thr Lys
 85 90 95
 Ser Thr Arg Thr Trp Val Leu Pro Ser Tyr Asn Asn His Gln Tyr Arg
 100 105 110

Glu Ile Lys Ser Gly Ser Val Asp Gly Ser Asn Ala Asn Ala Tyr Phe
 115 120 125
 Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn Arg Phe His Ser
 130 135 140
 His Trp Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn Asn Tyr Trp Gly
 145 150 155 160
 Phe Arg Pro Arg Ser Leu Arg Val Lys Ile Phe Asn Ile Gln Val Lys
 165 170 175
 Glu Val Thr Val Gln Asp Ser Thr Thr Thr Ile Ala Asn Asn Leu Thr
 180 185 190
 Ser Thr Val Gln Val Phe Thr Asp Asp Asp Tyr Gln Leu Pro Tyr Val
 195 200 205
 Val Gly Asn Gly Thr Glu Gly Cys Leu Pro Ala Phe Pro Pro Gln Val
 210 215 220
 Phe Thr Leu Pro Gln Tyr Gly Tyr Ala Thr Leu Asn Arg Asp Asn Thr
 225 230 235 240
 Glu Asn Pro Thr Glu Arg Ser Ser Phe Phe Cys Leu Glu Tyr Phe Pro
 245 250 255
 Ser Lys Met Leu Arg Thr Gly Asn Asn Phe Glu Phe Thr Tyr Asn Phe
 260 265 270
 Glu Glu Val Pro Phe His Ser Ser Phe Ala Pro Ser Gln Asn Leu Phe
 275 280 285
 Lys Leu Ala Asn Pro Leu Val Asp Gln Tyr Leu Tyr Arg Phe Val Ser
 290 295 300
 Thr Asn Asn Thr Gly Gly Val Gln Phe Asn Lys Asn Leu Ala Gly Arg
 305 310 315 320
 Tyr Ala Asn Thr Tyr Lys Asn Trp Phe Pro Gly Pro Met Gly Arg Thr
 325 330 335
 Gln Gly Trp Asn Leu Gly Ser Gly Val Asn Arg Ala Ser Val Ser Ala
 340 345 350
 Phe Ala Thr Thr Asn Arg Met Glu Leu Glu Gly Ala Ser Tyr Gln Val
 355 360 365
 Pro Pro Gln Pro Asn Gly Met Thr Asn Asn Leu Gln Gly Ser Asn Thr
 370 375 380
 Tyr Ala Leu Glu Asn Thr Met Ile Phe Asn Ser Gln Pro Ala Asn Pro
 385 390 395 400
 Gly Thr Thr Ala Thr Tyr Leu Glu Gly Asn Met Leu Ile Thr Ser Glu
 405 410 415
 Ser Glu Thr Gln Pro Val Asn Arg Val Ala Tyr Asn Val Gly Gly Gln
 420 425 430
 Met Ala Thr Asn Asn Gln Ser Ser Thr Thr Ala Pro Ala Thr Gly Thr
 435 440 445
 Tyr Asn Leu Gln Glu Ile Val Pro Gly Ser Val Trp Met Glu Arg Asp
 450 455 460
 Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile Pro Glu Thr Gly Ala
 465 470 475 480
 His Phe His Pro Ser Pro Ala Met Gly Gly Phe Gly Leu Lys His Pro
 485 490 495
 Pro Pro Met Met Leu Ile Lys Asn Thr Pro Val Pro Gly Asn Ile Thr
 500 505 510
 Ser Phe Ser Asp Val Pro Val Ser Ser Phe Ile Thr Gln Tyr Ser Thr
 515 520 525
 Gly Gln Val Thr Val Glu Met Glu Trp Glu Leu Lys Lys Glu Asn Ser
 530 535 540
 Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr Asn Asn Tyr Asn Asp Pro
 545 550 555 560
 Gln Phe Val Asp Phe Ala Pro Asp Ser Thr Gly Glu Tyr Arg Thr Thr

565 570 575
 Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg Pro Leu
 580 585

<210> 13
 <211> 532
 <212> PRT
 <213> Secuencia natural

<220>
 <223> Secuencia natural de la proteína VP3 de la cápside del AAV5 de tipo salvaje

<400> 13
 Met Ser Ala Gly Gly Gly Gly Pro Leu Gly Asp Asn Asn Gln Gly Ala
 1. 5 10 15
 Asp Gly Val Gly Asn Ala Ser Gly Asp Trp His Cys Asp Ser Thr Trp
 20 25 30
 Met Gly Asp Arg Val Val Thr Lys Ser Thr Arg Thr Trp Val Leu Pro
 35 40 45
 Ser Tyr Asn Asn His Gln Tyr Arg Glu Ile Lys Ser Gly Ser Val Asp
 50 55 60
 Gly Ser Asn Ala Asn Ala Tyr Phe Gly Tyr Ser Thr Pro Trp Gly Tyr
 65 70 75 80
 Phe Asp Phe Asn Arg Phe His Ser His Trp Ser Pro Arg Asp Trp Gln
 85 90 95
 Arg Leu Ile Asn Asn Tyr Trp Gly Phe Arg Pro Arg Ser Leu Arg Val
 100 105 110
 Lys Ile Phe Asn Ile Gln Val Lys Glu Val Thr Val Gln Asp Ser Thr
 115 120 125
 Thr Thr Ile Ala Asn Asn Leu Thr Ser Thr Val Gln Val Phe Thr Asp
 130 135 140
 Asp Asp Tyr Gln Leu Pro Tyr Val Val Gly Asn Gly Thr Glu Gly Cys
 145 150 155 160
 Leu Pro Ala Phe Pro Pro Gln Val Phe Thr Leu Pro Gln Tyr Gly Tyr
 165 170 175
 Ala Thr Leu Asn Arg Asp Asn Thr Glu Asn Pro Thr Glu Arg Ser Ser
 180 185 190
 Phe Phe Cys Leu Glu Tyr Phe Pro Ser Lys Met Leu Arg Thr Gly Asn
 195 200 205
 Asn Phe Glu Phe Thr Tyr Asn Phe Glu Glu Val Pro Phe His Ser Ser
 210 215 220
 Phe Ala Pro Ser Gln Asn Leu Phe Lys Leu Ala Asn Pro Leu Val Asp
 225 230 235 240
 Gln Tyr Leu Tyr Arg Phe Val Ser Thr Asn Asn Thr Gly Gly Val Gln
 245 250 255
 Phe Asn Lys Asn Leu Ala Gly Arg Tyr Ala Asn Thr Tyr Lys Asn Trp
 260 265 270
 Phe Pro Gly Pro Met Gly Arg Thr Gln Gly Trp Asn Leu Gly Ser Gly
 275 280 285
 Val Asn Arg Ala Ser Val Ser Ala Phe Ala Thr Thr Asn Arg Met Glu
 290 295 300
 Leu Glu Gly Ala Ser Tyr Gln Val Pro Pro Gln Pro Asn Gly Met Thr
 305 310 315 320
 Asn Asn Leu Gln Gly Ser Asn Thr Tyr Ala Leu Glu Asn Thr Met Ile
 325 330 335
 Phe Asn Ser Gln Pro Ala Asn Pro Gly Thr Thr Ala Thr Tyr Leu Glu

340 345 350
 Gly Asn Met Leu Ile Thr Ser Glu Ser Glu Thr Gln Pro Val Asn Arg
 355 360 365
 Val Ala Tyr Asn Val Gly Gly Gln Met Ala Thr Asn Asn Gln Ser Ser
 370 375 380
 Thr Thr Ala Pro Ala Thr Gly Thr Tyr Asn Leu Gln Glu Ile Val Pro
 385 390 395 400
 Gly Ser Val Trp Met Glu Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp
 405 410 415
 Ala Lys Ile Pro Glu Thr Gly Ala His Phe His Pro Ser Pro Ala Met
 420 425 430
 Gly Gly Phe Gly Leu Lys His Pro Pro Pro Met Met Leu Ile Lys Asn
 435 440 445
 Thr Pro Val Pro Gly Asn Ile Thr Ser Phe Ser Asp Val Pro Val Ser
 450 455 460
 Ser Phe Ile Thr Gln Tyr Ser Thr Gly Gln Val Thr Val Glu Met Glu
 465 470 475 480
 Trp Glu Leu Lys Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln
 485 490 495
 Tyr Thr Asn Asn Tyr Asn Asp Pro Gln Phe Val Asp Phe Ala Pro Asp
 500 505 510
 Ser Thr Gly Glu Tyr Arg Thr Thr Arg Pro Ile Gly Thr Arg Tyr Leu
 515 520 525
 Thr Arg Pro Leu
 530

<210> 14

<211> 724

<212> PRT

<213> Secuencia artificial

<220>

<223> proteína VP1 de la cápside de AAV5 modificada que incluye las sustituciones S2A y T711S

<400> 14

Met Ala Phe Val Asp His Pro Pro Asp Trp Leu Glu Glu Val Gly Glu
 1. 5 10 15
 Gly Leu Arg Glu Phe Leu Gly Leu Glu Ala Gly Pro Pro Lys Pro Lys
 20 25 30
 Pro Asn Gln Gln His Gln Asp Gln Ala Arg Gly Leu Val Leu Pro Gly
 35 40 45
 Tyr Asn Tyr Leu Gly Pro Gly Asn Gly Leu Asp Arg Gly Glu Pro Val
 50 55 60
 Asn Arg Ala Asp Glu Val Ala Arg Glu His Asp Ile Ser Tyr Asn Glu
 65 70 75 80
 Gln Leu Glu Ala Gly Asp Asn Pro Tyr Leu Lys Tyr Asn His Ala Asp
 85 90 95
 Ala Glu Phe Gln Glu Lys Leu Ala Asp Asp Thr Ser Phe Gly Gly Asn
 100 105 110
 Leu Gly Lys Ala Val Phe Gln Ala Lys Lys Arg Val Leu Glu Pro Phe
 115 120 125
 Gly Leu Val Glu Glu Gly Ala Lys Thr Ala Pro Thr Gly Lys Arg Ile
 130 135 140
 Asp Asp His Phe Pro Lys Arg Lys Lys Ala Arg Thr Glu Glu Asp Ser
 145 150 155 160
 Lys Pro Ser Thr Ser Ser Asp Ala Glu Ala Gly Pro Ser Gly Ser Gln

165	170	175
Gln Leu Gln Ile	Pro Ala Gln Pro	Ala Ser Ser Leu Gly Ala Asp Thr
180	185	190
Met Ser Ala Gly	Gly Gly Gly Pro	Leu Gly Asp Asn Asn Gln Gly Ala
195	200	205
Asp Gly Val Gly	Asn Ala Ser Gly	Asp Trp His Cys Asp Ser Thr Trp
210	215	220
Met Gly Asp Arg	Val Val Thr Lys Ser	Thr Arg Thr Trp Val Leu Pro
225	230	235
Ser Tyr Asn Asn	His Gln Tyr Arg	Glu Ile Lys Ser Gly Ser Val Asp
245	250	255
Gly Ser Asn Ala	Asn Ala Tyr Phe	Gly Tyr Ser Thr Pro Trp Gly Tyr
260	265	270
Phe Asp Phe Asn	Arg Phe His Ser	His Trp Ser Pro Arg Asp Trp Gln
275	280	285
Arg Leu Ile Asn	Asn Tyr Trp Gly	Phe Arg Pro Arg Ser Leu Arg Val
290	295	300
Lys Ile Phe Asn	Ile Gln Val Lys	Glu Val Thr Val Gln Asp Ser Thr
305	310	315
Thr Thr Ile Ala	Asn Asn Leu Thr	Ser Thr Val Gln Val Phe Thr Asp
325	330	335
Asp Asp Tyr Gln	Leu Pro Tyr Val	Val Gly Asn Gly Thr Glu Gly Cys
340	345	350
Leu Pro Ala Phe	Pro Pro Gln Val	Phe Thr Leu Pro Gln Tyr Gly Tyr
355	360	365
Ala Thr Leu Asn	Arg Asp Asn Thr	Glu Asn Pro Thr Glu Arg Ser Ser
370	375	380
Phe Phe Cys Leu	Glu Tyr Phe Pro	Ser Lys Met Leu Arg Thr Gly Asn
385	390	395
Asn Phe Glu Phe	Thr Tyr Asn Phe	Glu Glu Val Pro Phe His Ser Ser
405	410	415
Phe Ala Pro Ser	Gln Asn Leu Phe	Lys Leu Ala Asn Pro Leu Val Asp
420	425	430
Gln Tyr Leu Tyr	Arg Phe Val Ser	Thr Asn Asn Thr Gly Gly Val Gln
435	440	445
Phe Asn Lys Asn	Leu Ala Gly Arg	Tyr Ala Asn Thr Tyr Lys Asn Trp
450	455	460
Phe Pro Gly Pro	Met Gly Arg Thr	Gln Gly Trp Asn Leu Gly Ser Gly
465	470	475
Val Asn Arg Ala	Ser Val Ser Ala	Phe Ala Thr Thr Asn Arg Met Glu
485	490	495
Leu Glu Gly Ala	Ser Tyr Gln Val	Pro Pro Gln Pro Asn Gly Met Thr
500	505	510
Asn Asn Leu Gln	Gly Ser Asn Thr	Tyr Ala Leu Glu Asn Thr Met Ile
515	520	525
Phe Asn Ser Gln	Pro Ala Asn Pro	Gly Thr Thr Ala Thr Tyr Leu Glu
530	535	540
Gly Asn Met Leu	Ile Thr Ser Glu	Ser Glu Thr Gln Pro Val Asn Arg
545	550	555
Val Ala Tyr Asn	Val Gly Gly Gln	Met Ala Thr Asn Asn Gln Ser Ser
565	570	575
Thr Thr Ala Pro	Ala Thr Gly Thr	Tyr Asn Leu Gln Glu Ile Val Pro
580	585	590
Gly Ser Val Trp	Met Glu Arg Asp	Val Tyr Leu Gln Gly Pro Ile Trp
595	600	605
Ala Lys Ile Pro	Glu Thr Gly Ala	His Phe His Pro Ser Pro Ala Met
610	615	620

Gly Gly Phe Gly Leu Lys His Pro Pro Pro Met Met Leu Ile Lys Asn
 625 630 635 640
 Thr Pro Val Pro Gly Asn Ile Thr Ser Phe Ser Asp Val Pro Val Ser
 645 650 655
 Ser Phe Ile Thr Gln Tyr Ser Thr Gly Gln Val Thr Val Glu Met Glu
 660 665 670
 Trp Glu Leu Lys Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln
 675 680 685
 Tyr Thr Asn Asn Tyr Asn Asp Pro Gln Phe Val Asp Phe Ala Pro Asp
 690 695 700
 Ser Thr Gly Glu Tyr Arg Ser Thr Arg Pro Ile Gly Thr Arg Tyr Leu
 705 710 715 720
 Thr Arg Pro Leu

<210> 15

<211> 588

<212> PRT

<213> Secuencia artificial

<220>

<223> proteína VP2 de la cápside de AAV5 modificada que incluye la sustitución T575S

<400> 15

Thr Ala Pro Thr Gly Lys Arg Ile Asp Asp His Phe Pro Lys Arg Lys
 1. 5 10 15
 Lys Ala Arg Thr Glu Glu Asp Ser Lys Pro Ser Thr Ser Ser Asp Ala
 20 25 30
 Glu Ala Gly Pro Ser Gly Ser Gln Gln Leu Gln Ile Pro Ala Gln Pro
 35 40 45
 Ala Ser Ser Leu Gly Ala Asp Thr Met Ser Ala Gly Gly Gly Gly Pro
 50 55 60
 Leu Gly Asp Asn Asn Gln Gly Ala Asp Gly Val Gly Asn Ala Ser Gly
 65 70 75 80
 Asp Trp His Cys Asp Ser Thr Trp Met Gly Asp Arg Val Val Thr Lys
 85 90 95
 Ser Thr Arg Thr Trp Val Leu Pro Ser Tyr Asn Asn His Gln Tyr Arg
 100 105 110
 Glu Ile Lys Ser Gly Ser Val Asp Gly Ser Asn Ala Asn Ala Tyr Phe
 115 120 125
 Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn Arg Phe His Ser
 130 135 140
 His Trp Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn Asn Tyr Trp Gly
 145 150 155 160
 Phe Arg Pro Arg Ser Leu Arg Val Lys Ile Phe Asn Ile Gln Val Lys
 165 170 175
 Glu Val Thr Val Gln Asp Ser Thr Thr Thr Ile Ala Asn Asn Leu Thr
 180 185 190
 Ser Thr Val Gln Val Phe Thr Asp Asp Asp Tyr Gln Leu Pro Tyr Val
 195 200 205
 Val Gly Asn Gly Thr Glu Gly Cys Leu Pro Ala Phe Pro Pro Gln Val
 210 215 220
 Phe Thr Leu Pro Gln Tyr Gly Tyr Ala Thr Leu Asn Arg Asp Asn Thr
 225 230 235 240
 Glu Asn Pro Thr Glu Arg Ser Ser Phe Phe Cys Leu Glu Tyr Phe Pro
 245 250 255

Ser Lys Met Leu Arg Thr Gly Asn Asn Phe Glu Phe Thr Tyr Asn Phe
 260 265 270
 Glu Glu Val Pro Phe His Ser Ser Phe Ala Pro Ser Gln Asn Leu Phe
 275 280 285
 Lys Leu Ala Asn Pro Leu Val Asp Gln Tyr Leu Tyr Arg Phe Val Ser
 290 295 300
 Thr Asn Asn Thr Gly Gly Val Gln Phe Asn Lys Asn Leu Ala Gly Arg
 305 310 315 320
 Tyr Ala Asn Thr Tyr Lys Asn Trp Phe Pro Gly Pro Met Gly Arg Thr
 325 330 335
 Gln Gly Trp Asn Leu Gly Ser Gly Val Asn Arg Ala Ser Val Ser Ala
 340 345 350
 Phe Ala Thr Thr Asn Arg Met Glu Leu Glu Gly Ala Ser Tyr Gln Val
 355 360 365
 Pro Pro Gln Pro Asn Gly Met Thr Asn Asn Leu Gln Gly Ser Asn Thr
 370 375 380
 Tyr Ala Leu Glu Asn Thr Met Ile Phe Asn Ser Gln Pro Ala Asn Pro
 385 390 395 400
 Gly Thr Thr Ala Thr Tyr Leu Glu Gly Asn Met Leu Ile Thr Ser Glu
 405 410 415
 Ser Glu Thr Gln Pro Val Asn Arg Val Ala Tyr Asn Val Gly Gly Gln
 420 425 430
 Met Ala Thr Asn Asn Gln Ser Ser Thr Thr Ala Pro Ala Thr Gly Thr
 435 440 445
 Tyr Asn Leu Gln Glu Ile Val Pro Gly Ser Val Trp Met Glu Arg Asp
 450 455 460
 Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile Pro Glu Thr Gly Ala
 465 470 475 480
 His Phe His Pro Ser Pro Ala Met Gly Gly Phe Gly Leu Lys His Pro
 485 490 495
 Pro Pro Met Met Leu Ile Lys Asn Thr Pro Val Pro Gly Asn Ile Thr
 500 505 510
 Ser Phe Ser Asp Val Pro Val Ser Ser Phe Ile Thr Gln Tyr Ser Thr
 515 520 525
 Gly Gln Val Thr Val Glu Met Glu Trp Glu Leu Lys Lys Glu Asn Ser
 530 535 540
 Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr Asn Asn Tyr Asn Asp Pro
 545 550 555 560
 Gln Phe Val Asp Phe Ala Pro Asp Ser Thr Gly Glu Tyr Arg Ser Thr
 565 570 575
 Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg Pro Leu
 580 585

<210> 16

<211> 532

<212> PRT

<213> Secuencia artificial

<220>

<223> proteína VP3 de la cápside de AAV5 modificada que incluye la sustitución T519S

<400> 16

Met Ser Ala Gly Gly Gly Gly Pro Leu Gly Asp Asn Asn Gln Gly Ala

1. 5 10 15

Asp Gly Val Gly Asn Ala Ser Gly Asp Trp His Cys Asp Ser Thr Trp

20 25 30

Met Gly Asp Arg Val Val Thr Lys Ser Thr Arg Thr Trp Val Leu Pro
 35 40 45
 Ser Tyr Asn Asn His Gln Tyr Arg Glu Ile Lys Ser Gly Ser Val Asp
 50 55 60
 Gly Ser Asn Ala Asn Ala Tyr Phe Gly Tyr Ser Thr Pro Trp Gly Tyr
 65 70 75 80
 Phe Asp Phe Asn Arg Phe His Ser His Trp Ser Pro Arg Asp Trp Gln
 85 90 95
 Arg Leu Ile Asn Asn Tyr Trp Gly Phe Arg Pro Arg Ser Leu Arg Val
 100 105 110
 Lys Ile Phe Asn Ile Gln Val Lys Glu Val Thr Val Gln Asp Ser Thr
 115 120 125
 Thr Thr Ile Ala Asn Asn Leu Thr Ser Thr Val Gln Val Phe Thr Asp
 130 135 140
 Asp Asp Tyr Gln Leu Pro Tyr Val Val Gly Asn Gly Thr Glu Gly Cys
 145 150 155 160
 Leu Pro Ala Phe Pro Pro Gln Val Phe Thr Leu Pro Gln Tyr Gly Tyr
 165 170 175
 Ala Thr Leu Asn Arg Asp Asn Thr Glu Asn Pro Thr Glu Arg Ser Ser
 180 185 190
 Phe Phe Cys Leu Glu Tyr Phe Pro Ser Lys Met Leu Arg Thr Gly Asn
 195 200 205
 Asn Phe Glu Phe Thr Tyr Asn Phe Glu Glu Val Pro Phe His Ser Ser
 210 215 220
 Phe Ala Pro Ser Gln Asn Leu Phe Lys Leu Ala Asn Pro Leu Val Asp
 225 230 235 240
 Gln Tyr Leu Tyr Arg Phe Val Ser Thr Asn Asn Thr Gly Gly Val Gln
 245 250 255
 Phe Asn Lys Asn Leu Ala Gly Arg Tyr Ala Asn Thr Tyr Lys Asn Trp
 260 265 270
 Phe Pro Gly Pro Met Gly Arg Thr Gln Gly Trp Asn Leu Gly Ser Gly
 275 280 285
 Val Asn Arg Ala Ser Val Ser Ala Phe Ala Thr Thr Asn Arg Met Glu
 290 295 300
 Leu Glu Gly Ala Ser Tyr Gln Val Pro Pro Gln Pro Asn Gly Met Thr
 305 310 315 320
 Asn Asn Leu Gln Gly Ser Asn Thr Tyr Ala Leu Glu Asn Thr Met Ile
 325 330 335
 Phe Asn Ser Gln Pro Ala Asn Pro Gly Thr Thr Ala Thr Tyr Leu Glu
 340 345 350
 Gly Asn Met Leu Ile Thr Ser Glu Ser Glu Thr Gln Pro Val Asn Arg
 355 360 365
 Val Ala Tyr Asn Val Gly Gly Gln Met Ala Thr Asn Asn Gln Ser Ser
 370 375 380
 Thr Thr Ala Pro Ala Thr Gly Thr Tyr Asn Leu Gln Glu Ile Val Pro
 385 390 395 400
 Gly Ser Val Trp Met Glu Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp
 405 410 415
 Ala Lys Ile Pro Glu Thr Gly Ala His Phe His Pro Ser Pro Ala Met
 420 425 430
 Gly Gly Phe Gly Leu Lys His Pro Pro Met Met Leu Ile Lys Asn
 435 440 445
 Thr Pro Val Pro Gly Asn Ile Thr Ser Phe Ser Asp Val Pro Val Ser
 450 455 460
 Ser Phe Ile Thr Gln Tyr Ser Thr Gly Gln Val Thr Val Glu Met Glu
 465 470 475 480
 Trp Glu Leu Lys Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln

485 490 495
Tyr Thr Asn Asn Tyr Asn Asp Pro Gln Phe Val Asp Phe Ala Pro Asp
500 505 510
Ser Thr Gly Glu Tyr Arg Ser Thr Arg Pro Ile Gly Thr Arg Tyr Leu
515 520 525
Thr Arg Pro Leu
530

<210> 17
<211> 1383
<212> ADN
<213> Secuencia natural

<220>
<223> ácido nucleico que codifica el factor de coagulación IX recombinante de tipo salvaje (hFIX-wt) con la mutación R338L

<400> 17
atgcagcgcg tgaacatgat catggcagaa tcaccaggcc tcatcaccat ctgccttta 60

ggatatctac tcagtgtga atgtacagtt tttcttgatc atgaaaacgc caacaaaatt 120

ctgaatcggc caaagaggta taattcaggt aaattggaag agtttgttca agggaacctt 180

gagagagaat gtatggaaga aaagtgtagt ttgaagaag cacgagaagt tttgaaaac 240

actgaaagaa caactgaatt ttggaagcag tatgttgatg gagatcagtg tgagtccaat 300

ccatgtttaa atggcggcag ttgcaaggat gacattaatt cctatgaatg ttggtgtccc 360

tttgatttg aaggaaagaa ctgtgaatta gatgtaacat gtaacattaa gaatggcaga 420

tgcgagcagt ttgtaaaaa tagtgctgat aacaagggtg ttgctcctg tactgaggga 480

tatcgacttg cagaaaacca gaagtcctgt gaaccagcag tgccatttcc atgtggaaga 540

gtttctgttt caaaacttc taagctcacc cgtgctgaga ctgttttcc tgatgtggac 600

tatgtaaatt ctactgaagc tgaaccatt ttggataaca tcaactaaag cacccaatca 660

ttaatgact tcactcgggt tgttggtgga gaagatgcca aaccagggtca attcccttg 720

caggtgttt tgaatgtaa agttgatgca ttctgtggag gctctatcgt taatgaaaa 780

tgattgtaa ctgtgccca ctgtgtgaa actggtgta aaattacagt tgcgcaggt 840

gaacataata ttgaggagac agaacataga gagcaaaagc gaaatgtgat tcgaattatt 900

cctcaccaca actacaatgc agctattaat aagtacaacc atgacattgc ccttctggaa 960

ctggacgaac ccttagtgct aaacagctac gttacaccta ttgcattgc tgacaaggaa 1020

tacacgaaca tcttctcaa atttgatct ggctatgtaa gtggctgggg aagagtcttc 1080

cacaaggga gatcagcttt agttcttcag taccttagag ttccacttgt tgaccgagcc 1140

acatgtcttc tatctacaaa gttcaccatc tataacaaca tggtctgtgc tggcttccat 1200
gaaggaggta gagattcatg tcaaggagat agtggggggac cccatgttac tgaagtggaa 1260
gggaccagtt tcttaactgg aattattagc tgggggtgaag agtgtgcaat gaaaggcaaa 1320
tatggaatat ataccaaggt atcccgggtat gtcaactgga ttaaggaaaa aacaaagctc 1380
act 1383