

ПЕРЕЧЕНЬ ПОСЛЕДОВАТЕЛЬНОСТЕЙ

<110> ООО "АНАБИОН"

<120> Кодон-оптимизированная нуклеиновая кислота, которая кодирует белок SMN1, и ее применение

<160> 13

<170> BiSSAP 1.3.6

<210> 1

<211> 294

<212> PRT

<213> Природная последовательность

<220>

<223> Аминокислотная последовательность белка SMN1

<400> 1

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

Ala Asp Ala Leu Gly Ser Met Leu Ile Ser Trp Tyr Met Ser Gly Tyr
 260 265 270
 His Thr Gly Tyr Tyr Met Gly Phe Arg Gln Asn Gln Lys Glu Gly Arg
 275 280 285
 Cys Ser His Ser Leu Asn
 290

<210> 2

<211> 882

<212> DNA

<213> Искусственная последовательность

<220>

<223> Кодон-оптимизированная последовательность нуклеиновой кислоты,
 которая кодирует белок SMN1 (SMN1 GeneBeam)

<400> 2

atggccatga gcagcggcgg cagcggcggc ggcgtgcctg agcaagagga cagcgtgctg	60
ttcagaagag gcaccggcca gagcgacgac agcgacatct gggacgacac cgccctgac	120
aaggcctacg acaaggccgt ggccagcttc aagcacgccc tgaagaacgg cgacatctgc	180
gagaccagcg gcaagcccaa gaccaccccc aagagaaagc ccgccaagaa gaacaagagc	240
cagaagaaga acaccgccgc cagcctgcag cagtgggaagg tgggcgacaa gtgcagcgcc	300
atctggagcg aggacggctg catctacccc gccaccatcg ccagcatcga cttcaagaga	360
gagacctgcg tgggtggtgta caccggctac ggcaacagag aggagcagaa cctgagcgac	420
ctgctgagcc ccatctgcga ggtggccaac aacatcgagc agaacgccc agagaacgag	480
aacgagagcc aagtgagcac cgacgagagc gagaacagca gaagccccgg caacaagagc	540
gacaacatca agcccaagag cgccccctgg aacagcttcc tgccccctcc cccccctatg	600
cccggcccta gactgggccc tggcaagcct ggcctgaagt tcaacggccc cccccccct	660
cctcctcctc ctctcctca cctgctgagc tgctggctgc ccccttccc cagcggccct	720
cctatcatcc ctctcccc cccatctgc ccgacagcc tggacgacgc cgacgccctg	780
ggcagcatgc tgatcagctg gtacatgagc ggctaccaca ccggctacta catgggcttc	840
agacagaacc agaaggaggg ccggtgcagc cacagcctga ac	882

<210> 3

<211> 885

<212> DNA

<213> Природная последовательность

<220>

<223> Последовательность нуклеиновой кислоты гена SMN1 дикого типа
(SMN1-WT, SMN1-Wild type)

<400> 3

atggcgatga gcagcggcgg cagtgggtggc ggcgtcccgg agcaggagga ttccgtgctg	60
ttccggcgcg gcacaggcca gagcgatgat tctgacattt gggatgatac agcactgata	120
aaagcatatg ataaagctgt ggcttcattt aagcatgctc taaagaatgg tgacatttgt	180
gaaacttcgg gtaaaccaaa aaccacacct aaaagaaaac ctgctaagaa gaataaaagc	240
caaaagaaga atactgcagc ttccttaca cagtggaaag ttggggacaa atgttctgcc	300
atttggtcag aagacggttg catttaccca gctaccattg cttcaattga ttttaagaga	360
gaaacctgtg ttgtggttta cactggatat ggaaatagag aggagcaaaa tctgtccgat	420
ctactttccc caatctgtga agtagctaata aatatagaac aaaatgctca agagaatgaa	480
aatgaaagcc aagtttcaac agatgaaagt gagaactcca ggtctcctgg aaataaatca	540
gataacatca agcccaaadc tgctccatgg aactcttttc tccctccacc accccccatg	600
ccagggccaa gactgggacc aggaaagcca ggtctaaaat tcaatggccc accaccgcca	660
ccgccaccac caccacccca cttactatca tgctggctgc ctccatttcc ttctggacca	720
ccaataattc cccaccacc tcccatatgt ccagattctc ttgatgatgc tgatgctttg	780
ggaagtatgt taatttcatg gtacatgagt ggctatcata ctggctatta tatgggtttc	840
agacaaaatc aaaaagaagg aagggtgctca cattccttaa attaa	885

<210> 4

<211> 2924

<212> DNA

<213> Искусственная последовательность

<220>

<223> Последовательность нуклеиновой кислоты экспрессионной кассеты
(полная)

<400> 4

cctgcaggca gctgcgcgct cgctcgctca ctgaggccgc ccgggcgctg ggcgaccttt	60
ggctgcccgg cctcagttag cgagcgagcg cgacagagagg gaggggccaa ctccatcact	120

aggggttcct gcggccgcac gcgctctagtt attaatagta atcaattacg gggtcattag	180
ttcatagccc atatatggag ttccgcgtta cataacttac ggtaaatggc ccgcctggct	240
gaccgcccac cgacccccgc ccattgacgt caataatgac gtatgttccc atagtaacgc	300
caatagggac tttccattga cgtcaatggg tggagtattt acggtaaact gcccacttgg	360
cagtacatca agtgtatcat atgccaagta cggccccctat tgacgtcaat gacggtaa	420
ggcccgccctg gcattatgcc cagtacatga ccttatggga ctttcctact tggcagtaca	480
tctacgtatt agtcatcgct attaccatgg tgatgcgggt ttggcagtac atcaatgggc	540
gtggatagcg gtttgactca cggggatttc caagtctcca cccattgac gtcaatggga	600
gtttgttttg gcacaaaaat caacgggact ttccaaaatg tcgtaacaac tccgccccat	660
tgacgcaaat gggcggtagg cgtgtacggg gggagggtcta tataagcaga gctcgtttag	720
tgaaccgtca gatcgccctgg agacgccatc cacgctgttt tgacctccat agaagacacc	780
gggaccgatc cagcctccgc ggattcgaat cccggccggg aacgggtgcat tggaacgcgg	840
attccccgtg ccaagagtga cgtaagtacc gcctatagag tctataggcc cacaaaaat	900
gctttcttct tttaatatac ttttttgttt atcttatttc taatactttc cctaattctt	960
ttctttcagg gcaataatga tacaatgtat catgcctctt tgcaccattc taaagaataa	1020
cagtgataat ttctgggtta aggcaatagc aatatttctg catataaata tttctgcata	1080
taaattgtaa ctgatgtaag aggtttcata ttgctaatag cagctacaat ccagctacca	1140
ttctgctttt attttatggg tgggataagg ctggattatt ctgagtccaa gctaggccct	1200
tttgctaatac atgttcatac ctcttatctt cctcccacag ctcttgggca acgtgctggg	1260
ctgtgtgctg gcccatactt ttggcaaaga attgggattc gaacatcgat tgtaattcat	1320
gagccaccat ggccatgagc agcggcggca gcggcggcgg cgtgcctgag caagaggaca	1380
gcgtgctgtt cagaagaggc accggccaga gcgacgacag cgacatctgg gacgacaccg	1440
ccctgatcaa ggcctacgac aaggccgtgg ccagcttcaa gcacgccctg aagaacggcg	1500
acatctgcga gaccagcggc aagcccaaga ccacccccaa gagaaagccc gccagaaga	1560
acaagagcca gaagaagaac accgccgcca gcctgcagca gtggaagggt ggcgacaagt	1620
gcagcgccat ctggagcgag gacggctgca tctaccccg caccatcgcc agcatcgact	1680
tcaagagaga gacctgcgtg gtggtgtaca ccggctacgg caacagagag gagcagaacc	1740

tgagcgacct gctgagcccc atctgagagg tggccaacaa catcgagcag aacgccccag	1800
agaacgagaa cgagagccaa gtgagcaccg acgagagcga gaacagcaga agccccggca	1860
acaagagcga caacatcaag cccaagagcg ccccttgga cagcttcctg cccctcccc	1920
cccctatgcc cggccctaga ctgggcccctg gcaagcctgg cctgaagtgc aacggccccc	1980
ccccccctcc tcctcctcct cctcctcacc tgctgagctg ctggctgccc cccttcccca	2040
gcggccctcc tatcatccct cctccccccc ccatctgccc cgacagcctg gacgagccg	2100
acgccctggg cagcatgctg atcagctggg acatgagcgg ctaccacacc ggctactaca	2160
tgggcttcag acagaaccag aaggagggcc ggtgcagcca cagcctgaac tgatctagag	2220
tcgacctgca gaagcttgcc tcgagcagcg ctgctcgaga gatctacggg tggcatccct	2280
gtgacccttc cccagtgcct ctcttgcccc tgggaagttgc cactccagtg cccaccagcc	2340
ttgtcctaataaaaattaagt tgcattcattt tgtctgacta ggtgtccttc tataatatta	2400
tgggggtggag ggggggtggta tggagcaagg ggcaagttgg gaagacaacc tgtagggcct	2460
gcgggggtcta ttgggaacca agctggagtg cagtggcaca atcttggtc actgcaatct	2520
ccgcctcctg ggttcaagcg attctcctgc ctgagcctcc cgagttgttg ggattccagg	2580
catgcatgac caggctcagc taatttttgt ttttttggtg gagacggggg ttcaccatat	2640
tggccaggct ggtctccaac tcctaattct aggtgatcta cccaccttg cctcccaaat	2700
tgctgggatt acaggcgtga accactgctc ccttcctgt ccttctgatt ttgtaggtaa	2760
ccacgtgcgg accgagcggc cgcaggaacc cctagtgatg gatttgcca ctccctctct	2820
gcgcgctcgc tcgctcactg aggcggggcg accaaaggtc gccgacgcc cgggctttgc	2880
ccgggcggcc tcagtgagcg agcgagcgcg cagctgcctg cagg	2924

<210> 5

<211> 736

<212> PRT

<213> Природная последовательность

<220>

<223> Аминокислотная последовательность белка VP1 AAV9

<400> 5

Met Ala Ala Asp Gly Tyr Leu Pro Asp Trp Leu Glu Asp Asn Leu Ser

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			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		
		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	Glu	Gln	Ser	Pro	Gln	Glu	Pro	Asp	Ser	Ser	Ala	Gly	Ile	Gly		
145				150					155					160			
Lys	Ser	Gly	Ala	Gln	Pro	Ala	Lys	Lys	Arg	Leu	Asn	Phe	Gly	Gln	Thr		
			165						170					175			
Gly	Asp	Thr	Glu	Ser	Val	Pro	Asp	Pro	Gln	Pro	Ile	Gly	Glu	Pro	Pro		
		180					185					190					
Ala	Ala	Pro	Ser	Gly	Val	Gly	Ser	Leu	Thr	Met	Ala	Ser	Gly	Gly	Gly		
		195					200					205					
Ala	Pro	Val	Ala	Asp	Asn	Asn	Glu	Gly	Ala	Asp	Gly	Val	Gly	Ser	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	Asn	Ser	Thr	Ser	Gly	Gly	Ser	Ser	Asn	Asp	Asn		
		260					265						270				
Ala	Tyr	Phe	Gly	Tyr	Ser	Thr	Pro	Trp	Gly	Tyr	Phe	Asp	Phe	Asn	Arg		
		275					280					285					
Phe	His	Cys	His	Phe	Ser	Pro	Arg	Asp	Trp	Gln	Arg	Leu	Ile	Asn	Asn		
290					295					300							
Asn	Trp	Gly	Phe	Arg	Pro	Lys	Arg	Leu	Asn	Phe	Lys	Leu	Phe	Asn	Ile		
305				310					315					320			
Gln	Val	Lys	Glu	Val	Thr	Asp	Asn	Asn	Gly	Val	Lys	Thr	Ile	Ala	Asn		
			325						330					335			
Asn	Leu	Thr	Ser	Thr	Val	Gln	Val	Phe	Thr	Asp	Ser	Asp	Tyr	Gln	Leu		
		340					345					350					
Pro	Tyr	Val	Leu	Gly	Ser	Ala	His	Glu	Gly	Cys	Leu	Pro	Pro	Phe	Pro		
		355				360						365					
Ala	Asp	Val	Phe	Met	Ile	Pro	Gln	Tyr	Gly	Tyr	Leu	Thr	Leu	Asn	Asp		
370					375						380						
Gly	Ser	Gln	Ala	Val	Gly	Arg	Ser	Ser	Phe	Tyr	Cys	Leu	Glu	Tyr	Phe		
385				390					395					400			
Pro	Ser	Gln	Met	Leu	Arg	Thr	Gly	Asn	Asn	Phe	Gln	Phe	Ser	Tyr	Glu		
			405					410					415				
Phe	Glu	Asn	Val	Pro	Phe	His	Ser	Ser	Tyr	Ala	His	Ser	Gln	Ser	Leu		
		420					425						430				

Asp	Arg	Leu	Met	Asn	Pro	Leu	Ile	Asp	Gln	Tyr	Leu	Tyr	Tyr	Leu	Ser
		435					440					445			
Lys	Thr	Ile	Asn	Gly	Ser	Gly	Gln	Asn	Gln	Gln	Thr	Leu	Lys	Phe	Ser
		450				455					460				
Val	Ala	Gly	Pro	Ser	Asn	Met	Ala	Val	Gln	Gly	Arg	Asn	Tyr	Ile	Pro
465					470					475					480
Gly	Pro	Ser	Tyr	Arg	Gln	Gln	Arg	Val	Ser	Thr	Thr	Val	Thr	Gln	Asn
				485					490					495	
Asn	Asn	Ser	Glu	Phe	Ala	Trp	Pro	Gly	Ala	Ser	Ser	Trp	Ala	Leu	Asn
			500					505					510		
Gly	Arg	Asn	Ser	Leu	Met	Asn	Pro	Gly	Pro	Ala	Met	Ala	Ser	His	Lys
		515					520					525			
Glu	Gly	Glu	Asp	Arg	Phe	Phe	Pro	Leu	Ser	Gly	Ser	Leu	Ile	Phe	Gly
		530				535					540				
Lys	Gln	Gly	Thr	Gly	Arg	Asp	Asn	Val	Asp	Ala	Asp	Lys	Val	Met	Ile
545					550					555					560
Thr	Asn	Glu	Glu	Glu	Ile	Lys	Thr	Thr	Asn	Pro	Val	Ala	Thr	Glu	Ser
				565					570					575	
Tyr	Gly	Gln	Val	Ala	Thr	Asn	His	Gln	Ser	Ala	Gln	Ala	Gln	Ala	Gln
			580					585					590		
Thr	Gly	Trp	Val	Gln	Asn	Gln	Gly	Ile	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	Asn	Phe	His	Pro	Ser	Pro	Leu	Met	Gly	Gly	Phe	Gly	Met
625					630					635					640
Lys	His	Pro	Pro	Pro	Gln	Ile	Leu	Ile	Lys	Asn	Thr	Pro	Val	Pro	Ala
				645					650					655	
Asp	Pro	Pro	Thr	Ala	Phe	Asn	Lys	Asp	Lys	Leu	Asn	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	Tyr	Lys	Ser	Asn	Asn	Val	Glu	Phe	Ala	Val	Asn	Thr	Glu	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> 6

<211> 599

<212> PRT

<213> Природная последовательность

<220>

<223> Аминокислотная последовательность белка VP2 AAV9

<400> 6

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

435 440 445
 Ser Ala Gln Ala Gln Ala Gln Thr Gly Trp Val Gln Asn Gln Gly Ile
 450 455 460
 Leu Pro Gly Met Val Trp Gln Asp Arg Asp Val Tyr Leu Gln Gly Pro
 465 470 475 480
 Ile Trp Ala Lys Ile Pro His Thr Asp Gly Asn Phe His Pro Ser Pro
 485 490 495
 Leu Met Gly Gly Phe Gly Met Lys His Pro Pro Pro Gln Ile Leu Ile
 500 505 510
 Lys Asn Thr Pro Val Pro Ala Asp Pro Pro Thr Ala Phe Asn Lys Asp
 515 520 525
 Lys Leu Asn Ser Phe Ile Thr Gln Tyr Ser Thr Gly Gln Val Ser Val
 530 535 540
 Glu Ile Glu Trp Glu Leu Gln Lys Glu Asn Ser Lys Arg Trp Asn Pro
 545 550 555 560
 Glu Ile Gln Tyr Thr Ser Asn Tyr Tyr Lys Ser Asn Asn Val Glu Phe
 565 570 575
 Ala Val Asn Thr Glu Gly Val Tyr Ser Glu Pro Arg Pro Ile Gly Thr
 580 585 590
 Arg Tyr Leu Thr Arg Asn Leu
 595

<210> 7

<211> 534

<212> PRT

<213> Природная последовательность

<220>

<223> Аминокислотная последовательность белка VP3 AAV9

<400> 7

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

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

<210> 8

<211> 130

<212> DNA

<213> Природная последовательность

<220>

<223> Левый (первый) ITR (инвертированные концевые повторы)

<400> 8

cctgcaggca gctgcgctcg cgctcgctca ctgaggccgc ccgggcgtcg ggcgaccttt 60

ggtcgcccgg cctcagtgag cgagcgagcg cgagagagg gaggggccaa ctccatcact 120

aggggttcct 130

<210> 9

<211> 304

<212> DNA

<213> Природная последовательность

<220>

<223> CMV (Цитомегаловирусный) энхансер

<400> 9

cgttacataa cttacggtaa atggcccgcc tggctgaccg cccaacgacc cccgcccatt 60

gacgtcaata atgacgtatg ttcccatagt aacgccaata gggactttcc attgacgtca 120

atgggtggag tatttacggg aaactgcccc cttggcagta catcaagtgt atcatatgcc 180

aagtagcccc cctattgacg tcaatgacgg taaatggccc gcctggcatt atgcccagta 240

catgacctta tgggactttc ctacttggca gtacatctac gtattagtca tcgctattac 300

catg 304

<210> 10

<211> 204

<212> DNA

<213> Природная последовательность

<220>

<223> CMV (Цитомегаловирусный) промотер

<400> 10

gtgatgcggt tttggcagta catcaatggg cgtggatagc gggttgactc acggggattt 60

ccaagtctcc acccattga cgtcaatggg agtttgtttt ggcaccaaaa tcaacgggac 120

tttccaaaat gtcgtaacaa ctccgcccc ttgacgcaaa tgggcggtag gcgtgtacgg 180

tgggaggtct atataagcag agct 204

<210> 11
 <211> 493
 <212> DNA
 <213> Природная последовательность

<220>
 <223> Интрон гена hBG1 (Субъединица гемоглобина гамма-1)

<400> 11
 cgaatccccgg ccgggaacgg tgcattggaa cgcggattcc ccgtgccaaag agtgacgtaa 60
 gtaccgccta tagagtctat aggcccacaa aaaatgcttt cttcttttaa tatacttttt 120
 tgtttatctt atttctaata ctttccttaa tctctttctt tcagggaat aatgatacaa 180
 tgtatcatgc ctctttgcac cattctaaag aataacagtg ataatttctg ggtaaggca 240
 atagcaatat ttctgcatat aaatatttct gcatataaat tgtaactgat gtaagagggt 300
 tcatattgct aatagcagct acaatccagc taccattctg cttttatttt atggttggga 360
 taaggctgga ttattctgag tccaagctag gcccttttgc taatcatgtt catacctctt 420
 atcttcctcc cacagctcct gggcaacgtg ctggctctgtg tgctggccca tcactttggc 480
 aaagaattgg gat 493

<210> 12
 <211> 479
 <212> DNA
 <213> Природная последовательность

<220>
 <223> Сигнал полиаденилирования hGH1 (сигнал полиаденилирования (поли(А)) гормона роста человека)

<400> 12
 acgggtggca tccctgtgac ccctccccag tgcctctcct ggccctggaa gttgccactc 60
 cagtgccac cagccttgct ctaataaaat taagttgcat cttttgtct gactaggtgt 120
 ctttctataa tattatgggg tggagggggg tggatggag caaggggcaa gttgggaaga 180
 caacctgtag ggcctgcggg gtctattggg aaccaagctg gagtgcagtg gcacaatctt 240
 ggctcactgc aatctccgc tcctgggttc aagcgattct cctgcctcag cctcccgagt 300
 tgttgggatt ccaggcatgc atgaccaggc tcagctaatt tttgtttttt tggtagagac 360

ggggttttcac catattggcc aggctggtct ccaactccta atctcaggtg atctacccac 420

cttggcctcc caaatgctg ggattacagg cgtgaaccac tgctcccttc cctgtcctt 479

<210> 13

<211> 141

<212> DNA

<213> Природная последовательность

<220>

<223> Правый (второй) ITR

<400> 13

aggaacccct agtgatggag ttggccactc cctctctgcg cgctcgctcg ctactgagg 60

ccgggcgacc aaaggctgcc cgacgcccgg gctttgcccg ggcggcctca gtgagcgagc 120

gagcgcgcag ctgcctgcag g 141