

SEQUENCE LISTING

<110> California Institute of Technology
Deverman, Benjamin E.
Gradinaru, Viviana
Chan, Ken Y.

<120> TARGETING PEPTIDES FOR DIRECTING
ADENO-ASSOCIATED VIRUSES (AAVs)

<130> CALTE.118W0

<150> 62/266184
<151> 2015-12-11

<150> 62/421891
<151> 2016-11-14

<160> 93

<170> FastSEQ for Windows Version 4.0

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<400> 1
Ala Gln Thr Leu Ala Val Pro Phe Lys Ala Gln
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Ala Gln Ser Val Ser Lys Pro Phe Leu Ala Gln
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Ala	Gln	Phe	Thr	Leu	Thr	Thr	Pro	Lys	Ala	Gln
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Glu	Ser	Thr	Leu	Ala	Val	Pro	Phe	Lys	Ala	Gln
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Gly	Gly	Thr	Leu	Ala	Val	Pro	Phe	Lys	Ala	Gln
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Ala	Gln	Thr	Leu	Ala	Thr	Pro	Phe	Lys	Ala	Gln
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<223> PHP.B-ATT-T

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Ala	Thr	Thr	Leu	Ala	Thr	Pro	Phe	Lys	Ala	Gln
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Asp	Gly	Thr	Leu	Ala	Thr	Pro	Phe	Lys	Ala	Gln
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<223> PHP.B-GGT-T

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Gly	Gly	Thr	Leu	Ala	Thr	Pro	Phe	Lys	Ala	Gln
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Ser Gly Ser Leu Ala Val Pro Phe Lys Ala Gln
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<400> 12
Ala Gln Thr Leu Ala Gln Pro Phe Lys Ala Gln
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<400> 14
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Gln Gly Thr Leu Ala Val Pro Phe Lys Ala Gln
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<400> 17
Asn Gln Thr Leu Ala Val Pro Phe Lys Ala Gln
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Glu Gly Ser Leu Ala Val Pro Phe Lys Ala Gln
1 5 10

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Ser Gly Asn Leu Ala Val Pro Phe Lys Ala Gln
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<400> 20
Glu Gly Thr Leu Ala Val Pro Phe Lys Ala Gln
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<400> 21
Pro His Pro Asx Asp Ser Thr
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Ala Gln Thr Leu Gln Leu Pro Phe Lys Ala Gln
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Ala Gln Thr Leu Thr Met Pro Phe Lys Ala Gln
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Ala	Gln	Tyr	Thr	Leu	Ser	Gln	Gly	Trp	Ala	Gln
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Ala	Gln	Met	Asn	Ala	Thr	Lys	Asn	Val	Ala	Gln
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<400> 31

Ala	Gln	Val	Ser	Gly	Gly	His	His	Ser	Ala	Gln
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<400> 32

Ala	Gln	Thr	Leu	Pro	Gln	Pro	Phe	Lys	Ala	Gln
1				5					10	

<210> 33

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<400> 33

Ala	Gln	Thr	Leu	Ala	Thr	Pro	Phe	Lys	Ala	Gln
1				5					10	

<210> 34

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<400> 34

Ala	Gln	Thr	Leu	Thr	Met	Pro	Phe	Lys	Ala	Gln
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Ala	Gln	Thr	Leu	Thr	Met	Pro	Phe	Lys	Ala	Gln
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Ala Gln Thr Leu Ser Lys Pro Phe Lys Ala Gln
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<400> 37
Gln Ala Val Arg Thr Ser Leu
1 5

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<400> 38
Tyr Thr Leu Ser Gln Gly Trp
1 5

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<400> 39
Leu Ala Lys Glu Arg Leu Ser
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<400> 40

Thr Leu Ala Val Pro Phe Lys
1 5

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<400> 41
Ser Val Ser Lys Pro Phe Leu
1 5

<210> 42
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<400> 42
Phe Thr Leu Thr Thr Pro Lys
1 5

<210> 43
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<400> 43
Met Asn Ser Thr Lys Asn Val
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<400> 44
Val Ser Gly Gly His His Ser
1 5

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 Glu Gly Ile Arg Glu Trp Trp Ala Leu Lys Pro Gly Ala Pro Gln 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
 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

<213> Artificial Sequence

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<223> AAV-PHP.N VP1 Capsid Sequence

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Glu	Gly	Ile	Arg	Glu	Trp	Trp	Ala	Leu	Lys	Pro	Gly	Ala	Pro	Gln	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
	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	
Arg	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	Asp	Gly	Thr	Leu	Ala	Val	580	585	590	
Pro	Phe	Lys	Ala	Gln	Ala	Gln	Thr	Gly	Trp	Val	Gln	Asn	Gln	Gly	Ile	595	600	605	
Leu	Pro	Gly	Met	Val	Trp	Gln	Asp	Arg	Asp	Val	Tyr	Leu	Gln	Gly	Pro	610	615	620	
Ile	Trp	Ala	Lys	Ile	Pro	His	Thr	Asp	Gly	Asn	Phe	His	Pro	Ser	Pro	625	630	635	640
Leu	Met	Gly	Gly	Phe	Gly	Met	Lys	His	Pro	Pro	Pro	Gln	Ile	Leu	Ile	645	650	655	
Lys	Asn	Thr	Pro	Val	Pro	Ala	Asp	Pro	Pro	Thr	Ala	Phe	Asn	Lys	Asp	660	665	670	
Lys	Leu	Asn	Ser	Phe	Ile	Thr	Gln	Tyr	Ser	Thr	Gly	Gln	Val	Ser	Val	675	680	685	
Glu	Ile	Glu	Trp	Glu	Leu	Gln	Lys	Glu	Asn	Ser	Lys	Arg	Trp	Asn	Pro	690	695	700	
Glu	Ile	Gln	Tyr	Thr	Ser	Asn	Tyr	Tyr	Lys	Ser	Asn	Asn	Val	Glu	Phe	705	710	715	720
Ala	Val	Asn	Thr	Glu	Gly	Val	Tyr	Ser	Glu	Pro	Arg	Pro	Ile	Gly	Thr	725	730	735	
Arg	Tyr	Leu	Thr	Arg	Asn	Leu										740			

<210> 47

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<212> PRT

<213> Artificial Sequence

<220>

<223> AAV-PHP.S VP1 Capsid Sequence

<400> 47

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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

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				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				
Arg	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	Gln	Ala	Val	Arg		
	580					585							590				
Thr	Ser	Leu	Ala	Gln	Ala	Gln	Thr	Gly	Trp	Val	Gln	Asn	Gln	Gly	Ile		
	595					600						605					
Leu	Pro	Gly	Met	Val	Trp	Gln	Asp	Arg	Asp	Val	Tyr	Leu	Gln	Gly	Pro		
	610					615				620							
Ile	Trp	Ala	Lys	Ile	Pro	His	Thr	Asp	Gly	Asn	Phe	His	Pro	Ser	Pro		
625					630					635					640		
Leu	Met	Gly	Gly	Phe	Gly	Met	Lys	His	Pro	Pro	Pro	Gln	Ile	Leu	Ile		
			645						650					655			
Lys	Asn	Thr	Pro	Val	Pro	Ala	Asp	Pro	Pro	Thr	Ala	Phe	Asn	Lys	Asp		
		660						665					670				
Lys	Leu	Asn	Ser	Phe	Ile	Thr	Gln	Tyr	Ser	Thr	Gly	Gln	Val	Ser	Val		
	675						680					685					
Glu	Ile	Glu	Trp	Glu	Leu	Gln	Lys	Glu	Asn	Ser	Lys	Arg	Trp	Asn	Pro		
	690					695					700						
Glu	Ile	Gln	Tyr	Thr	Ser	Asn	Tyr	Tyr	Lys	Ser	Asn	Asn	Val	Glu	Phe		
705					710					715					720		
Ala	Val	Asn	Thr	Glu	Gly	Val	Tyr	Ser	Glu	Pro	Arg	Pro	Ile	Gly	Thr		
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Arg	Tyr	Leu	Thr	Arg	Asn	Leu											
		740															

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<223> Xaa = any amino acid

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<210> 50

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<210> 51

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<222> 8-10

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gatgggacgt tggcgggtgcc ttttaaggca cag

33

<210> 56

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<212> DNA

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<223> Synthetic nucleotide sequence

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21

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27

<210> 58

<211> 32

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caagtaaaac ctctacaaat gtggtaaaat cg

32

<210> 59

<211> 36

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<400> 59

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36

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 <223> n = a, c, t or g

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 nttgggcact ctggtggttt gtc 83

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<400> 66
Ala Gln Ala Gln Thr Gly Trp
1 5

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Asp Gly Thr Leu Ala Thr Pro Phe Lys
1 5

<210> 68
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tttg 64

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<223> n = a, c, t or g

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<210> 72

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 <223> n = a, c, t or g

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 AAV-PHP.B-XXX libraries

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 <223> n = a, c, t or g

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<210> 76

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tatactttgt cgcagggttg g 21

<210> 78

<211> 21

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<400> 79

cttgcaagg agcggctttc g 21

<210> 80

<211> 8

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Leu Ala Val Pro Phe Lys Ala Gln

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<210> 81

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Phe Lys Ala Gln

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<400> 83

Ala Gln Thr Leu Ala Val

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5

<210> 84

<211> 8

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Ala Gln Thr Leu Ala Val Pro Phe

<210> 85

<211> 4

<212> PRT

<213> Artificial Sequence

<220>

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<400> 85

Gln Ala Val Arg

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<212> PRT

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<400> 86

Ala Val Arg Thr

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<210> 87

<211> 4

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Val Arg Thr Ser

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<210> 88

<211> 4

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<220>

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Arg Thr Ser Leu

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Ala Val Arg Thr Ser
1 5

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Val Arg Thr Ser Leu
1 5

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Gln Ala Val Arg Thr Ser
1 5

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Ala Val Arg Thr Ser Leu

1

5