

FIG. 1

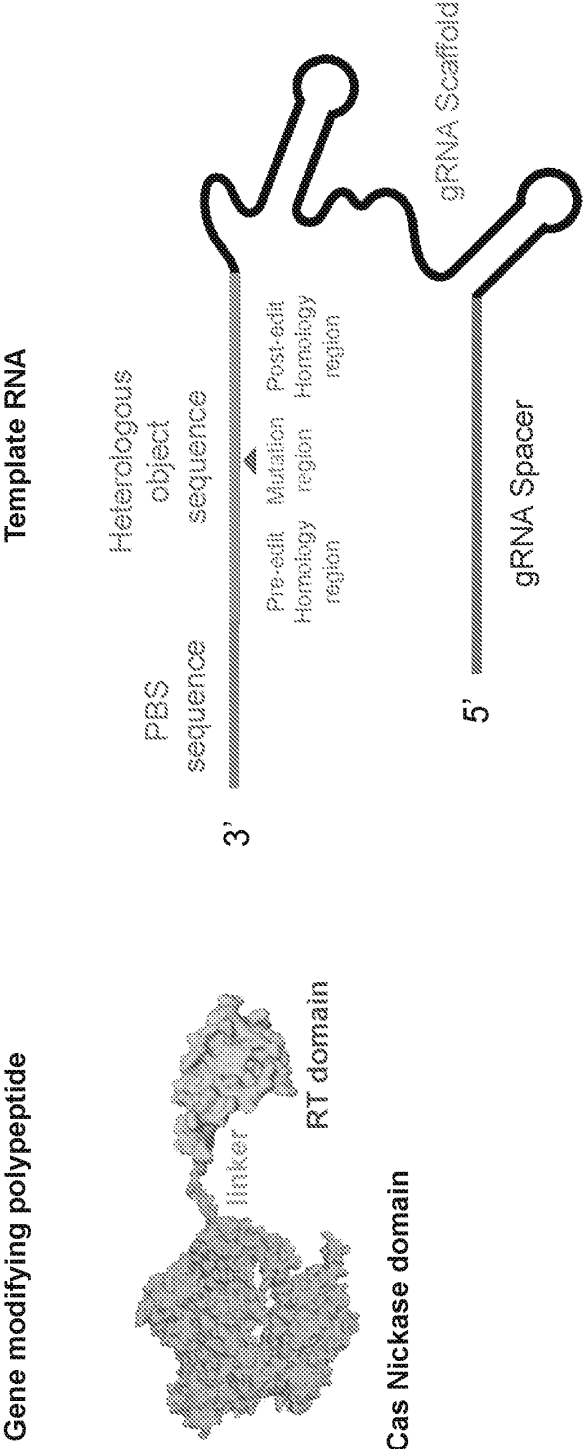


FIG. 2

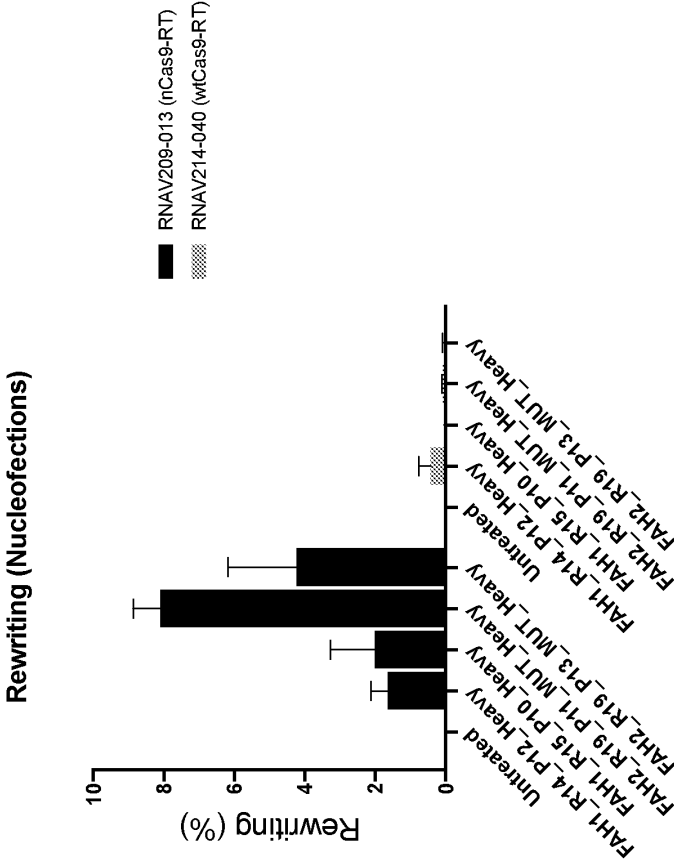


FIG. 3

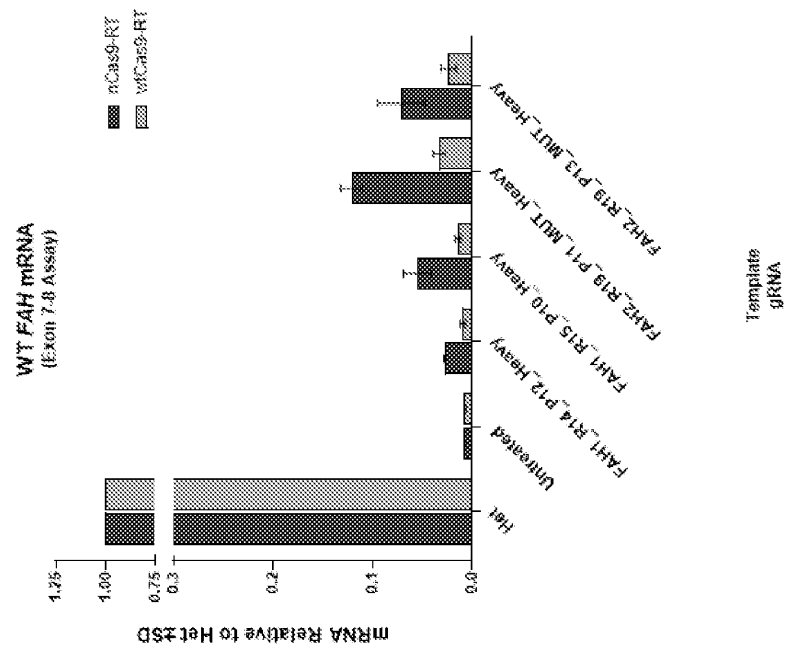


FIG. 4

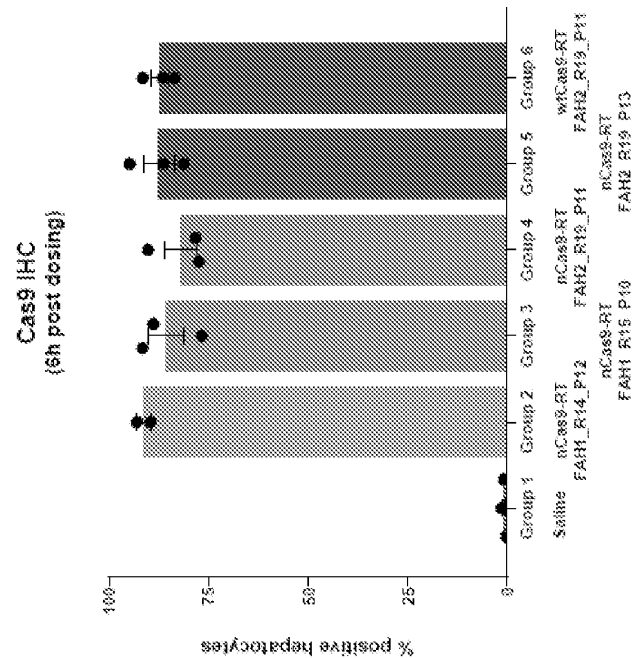


FIG. 5

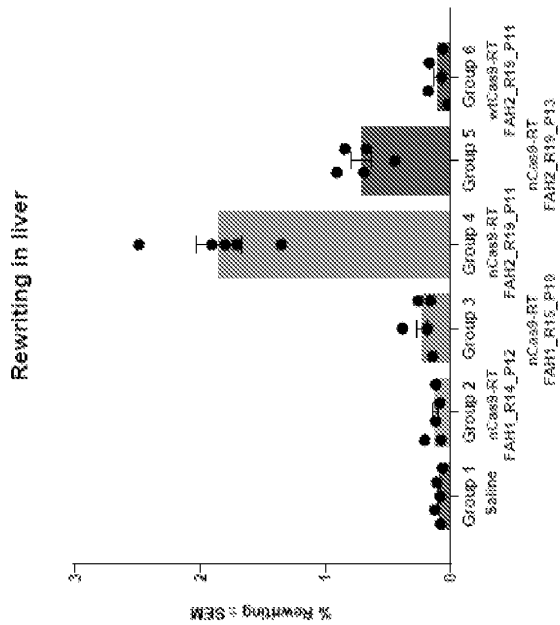


FIG. 6

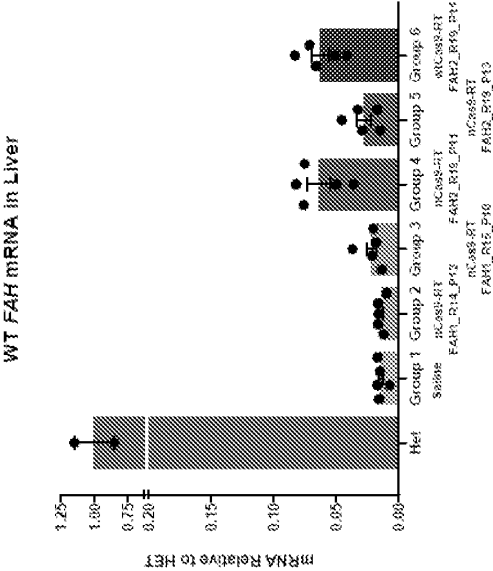


FIG. 7

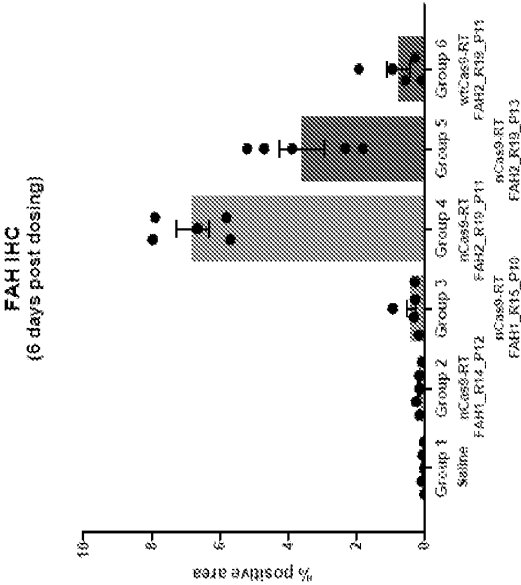


FIG. 8

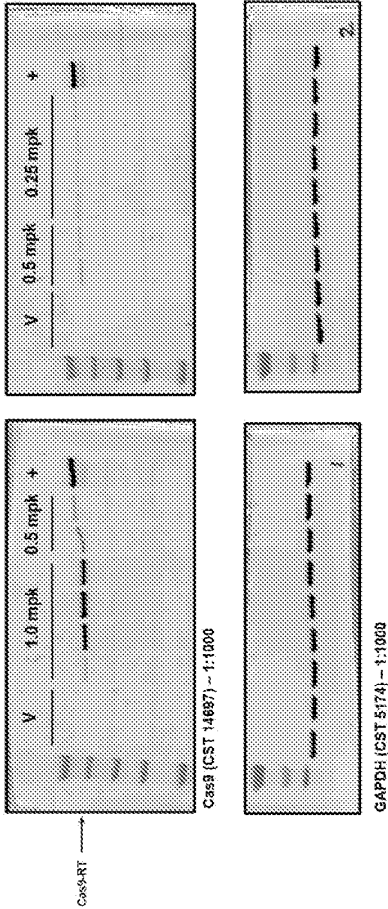


FIG. 9

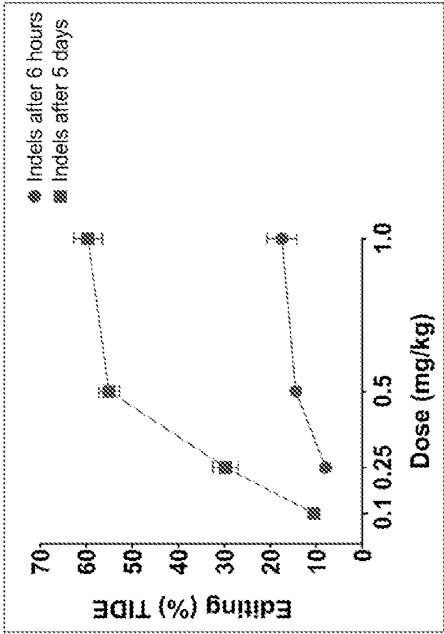


FIG. 10

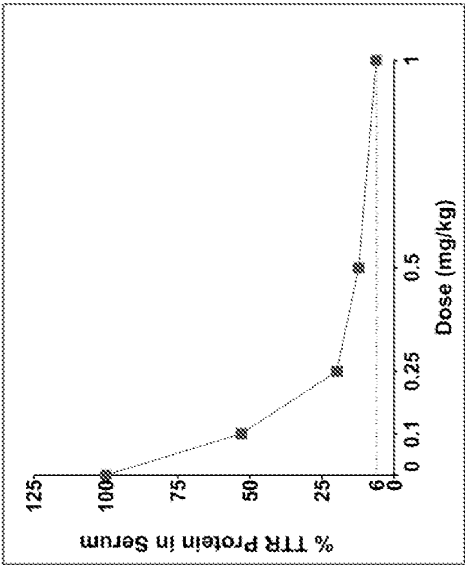


FIG. 11

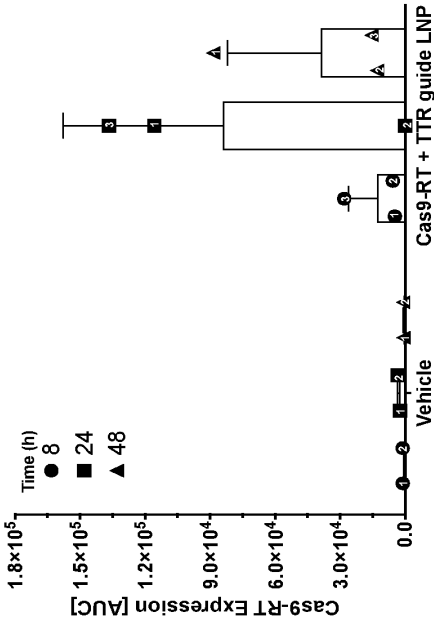
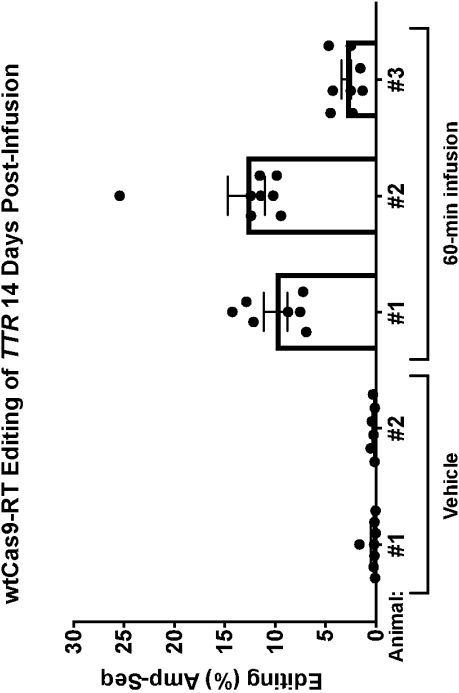


FIG. 12



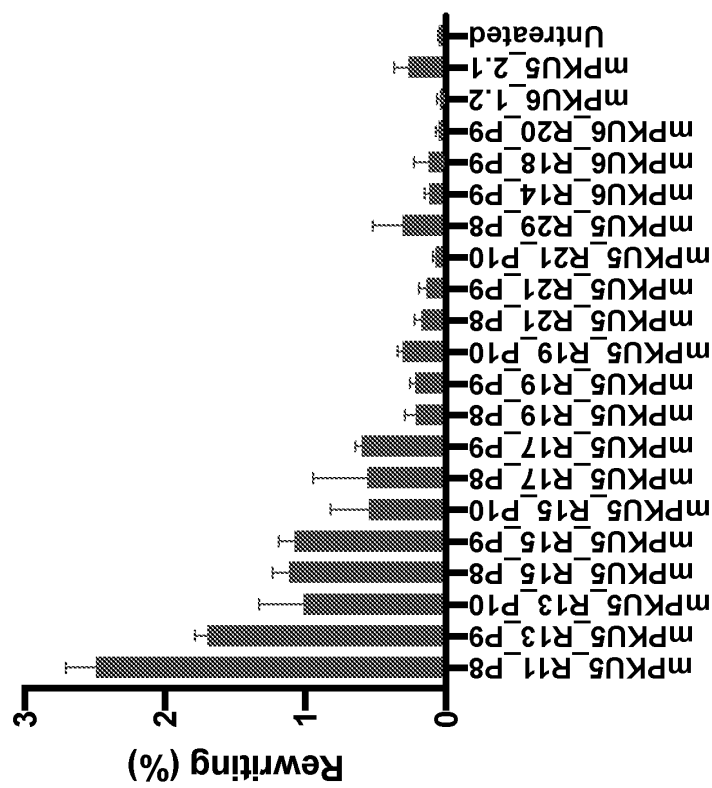


FIG. 13

FIG. 14

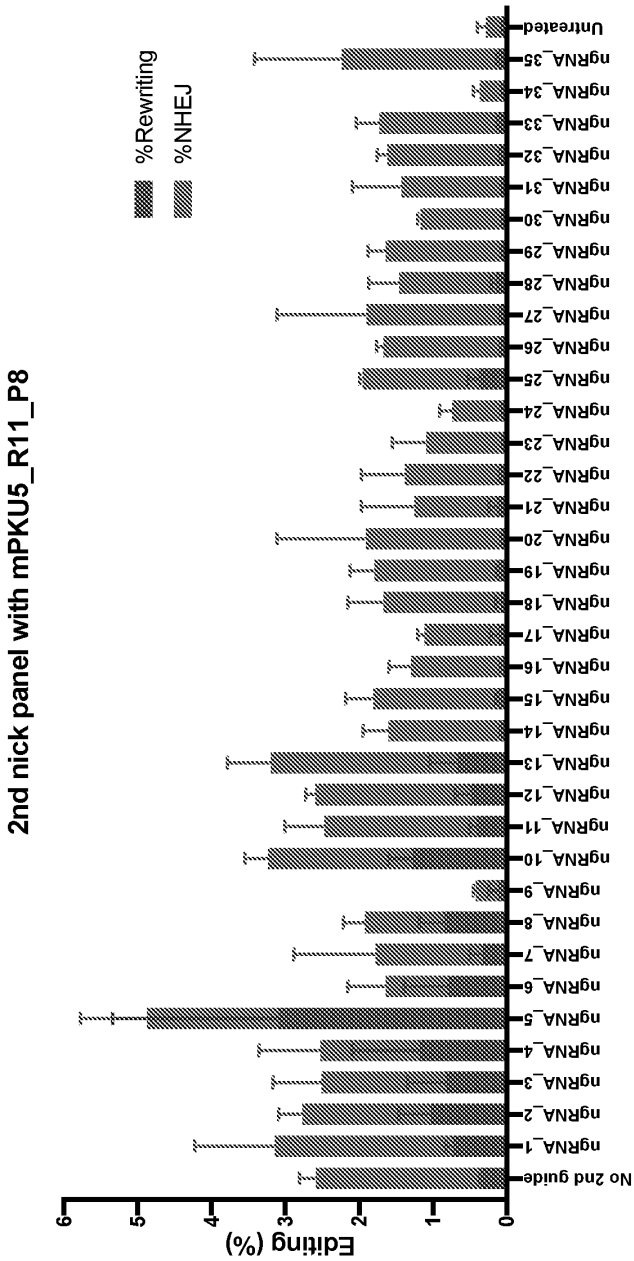
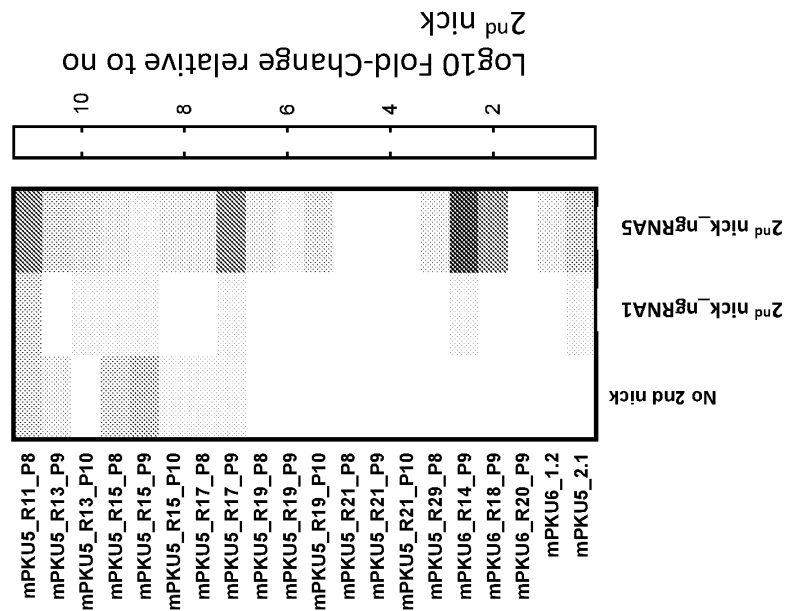


FIG. 15



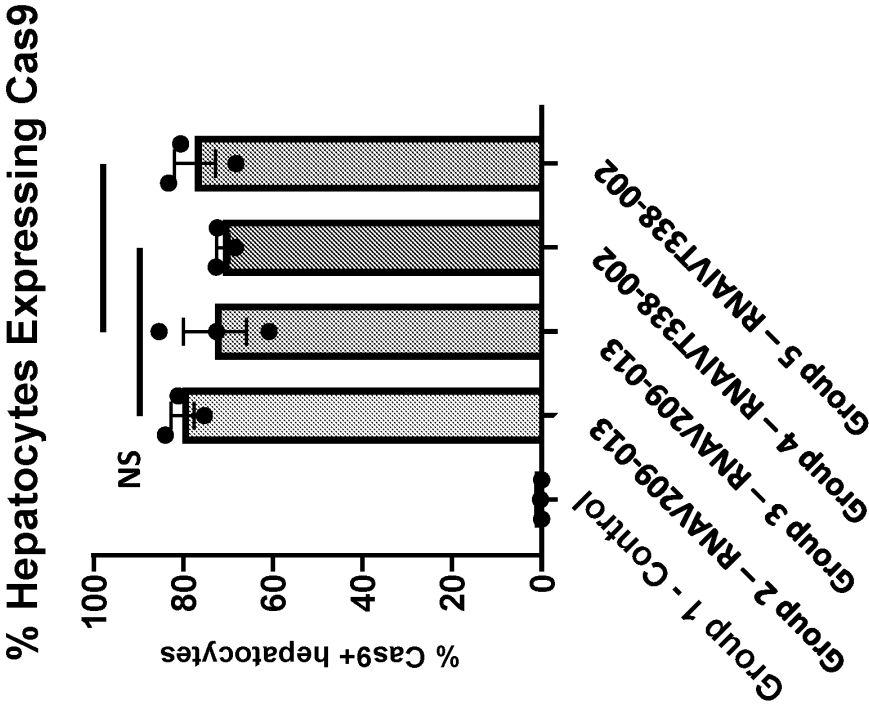


FIG. 16

FIG. 17

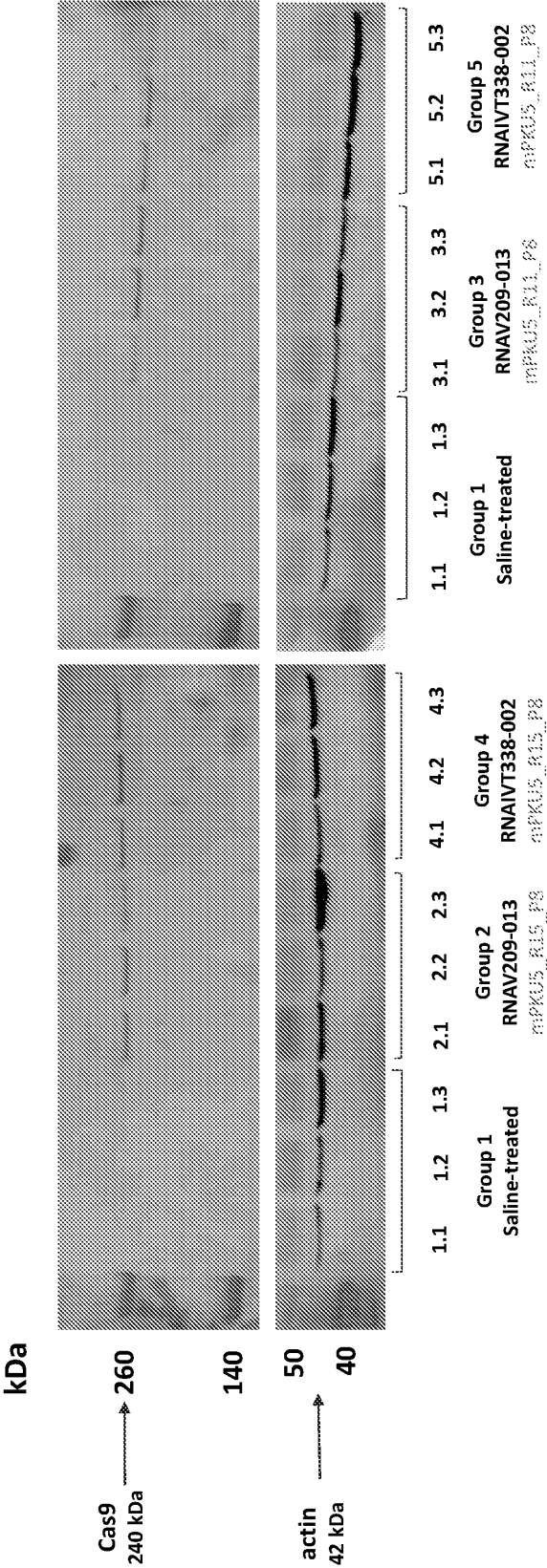


FIG. 18

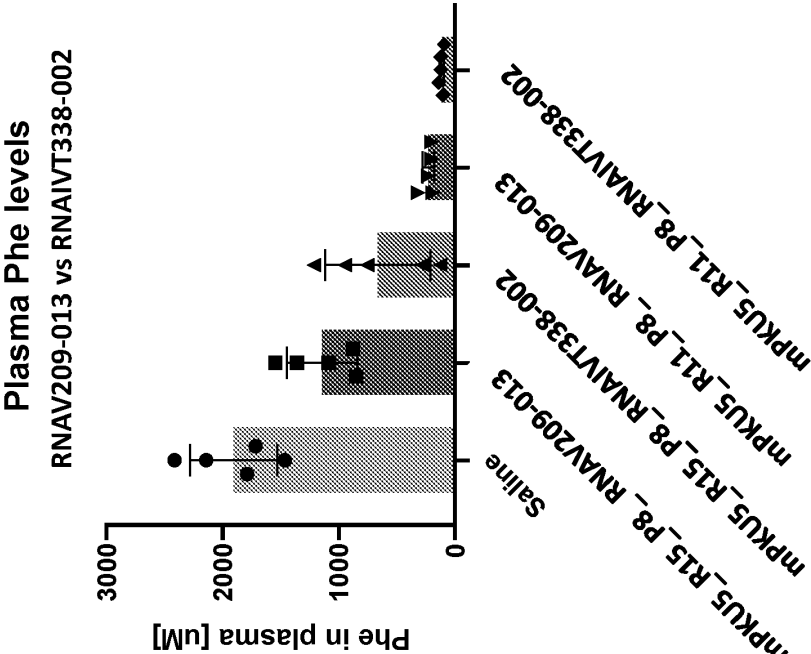


FIG. 19A

Rewriting in mouse liver
RNAV209-013 vs RNAVT338-002

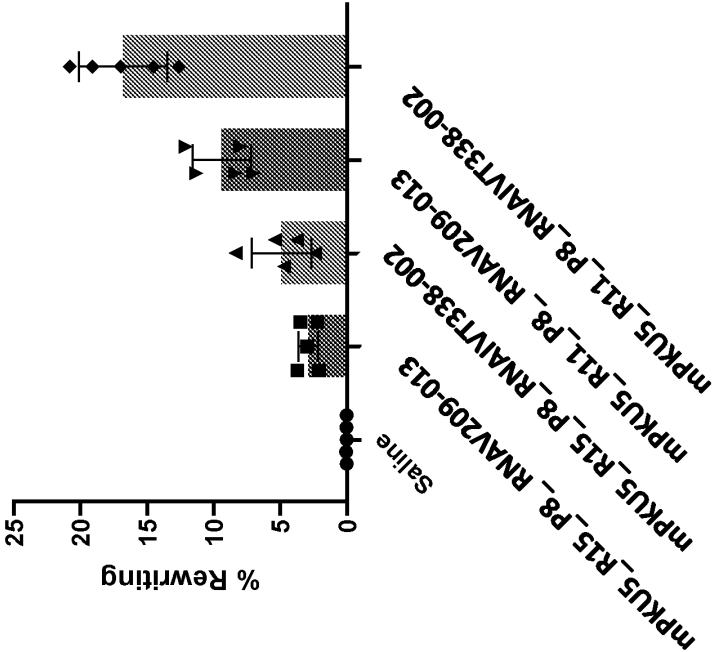


FIG. 19B

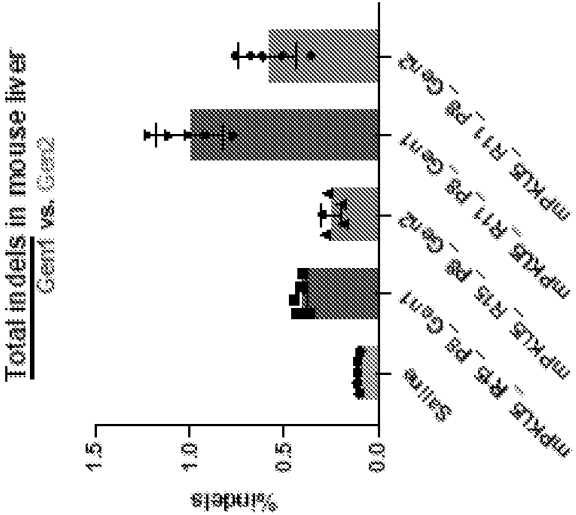


FIG. 20B

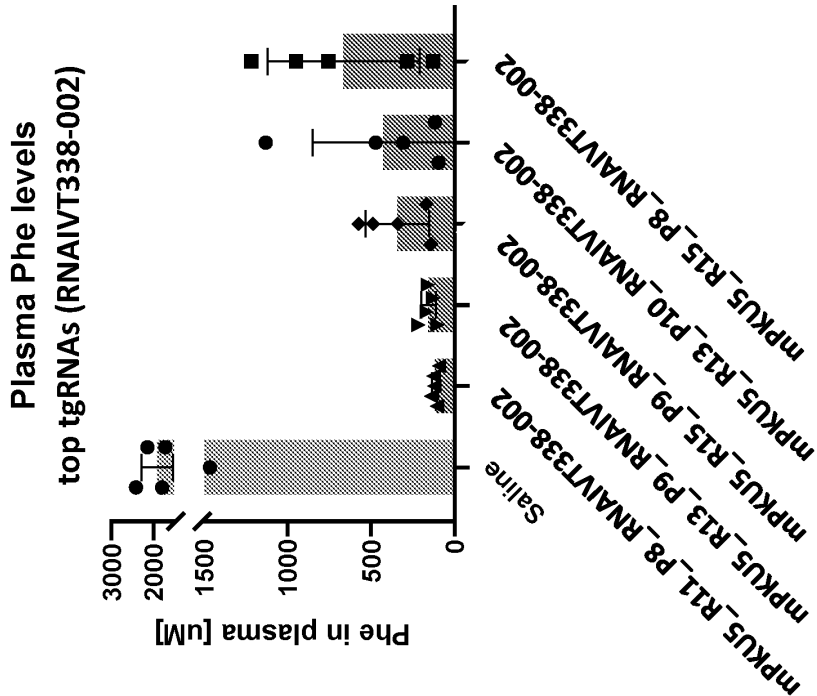


FIG. 20A

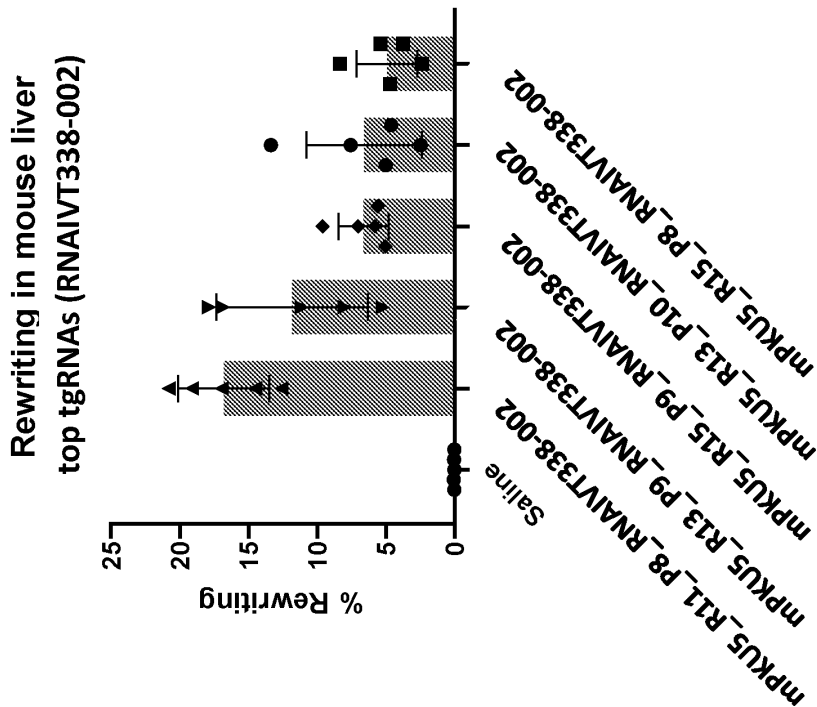
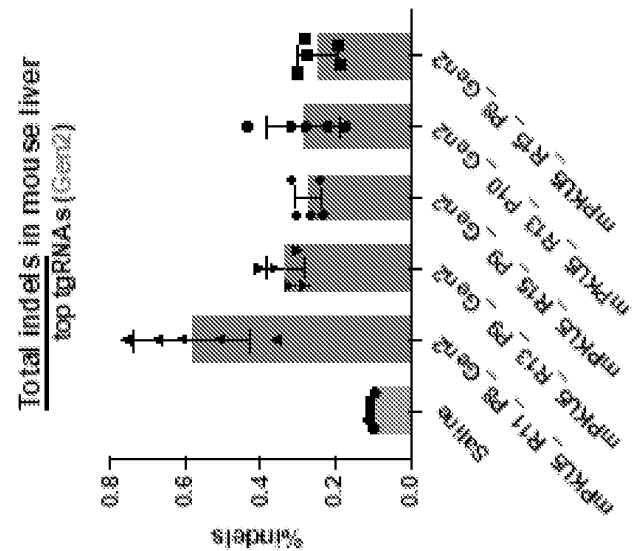


FIG. 20C



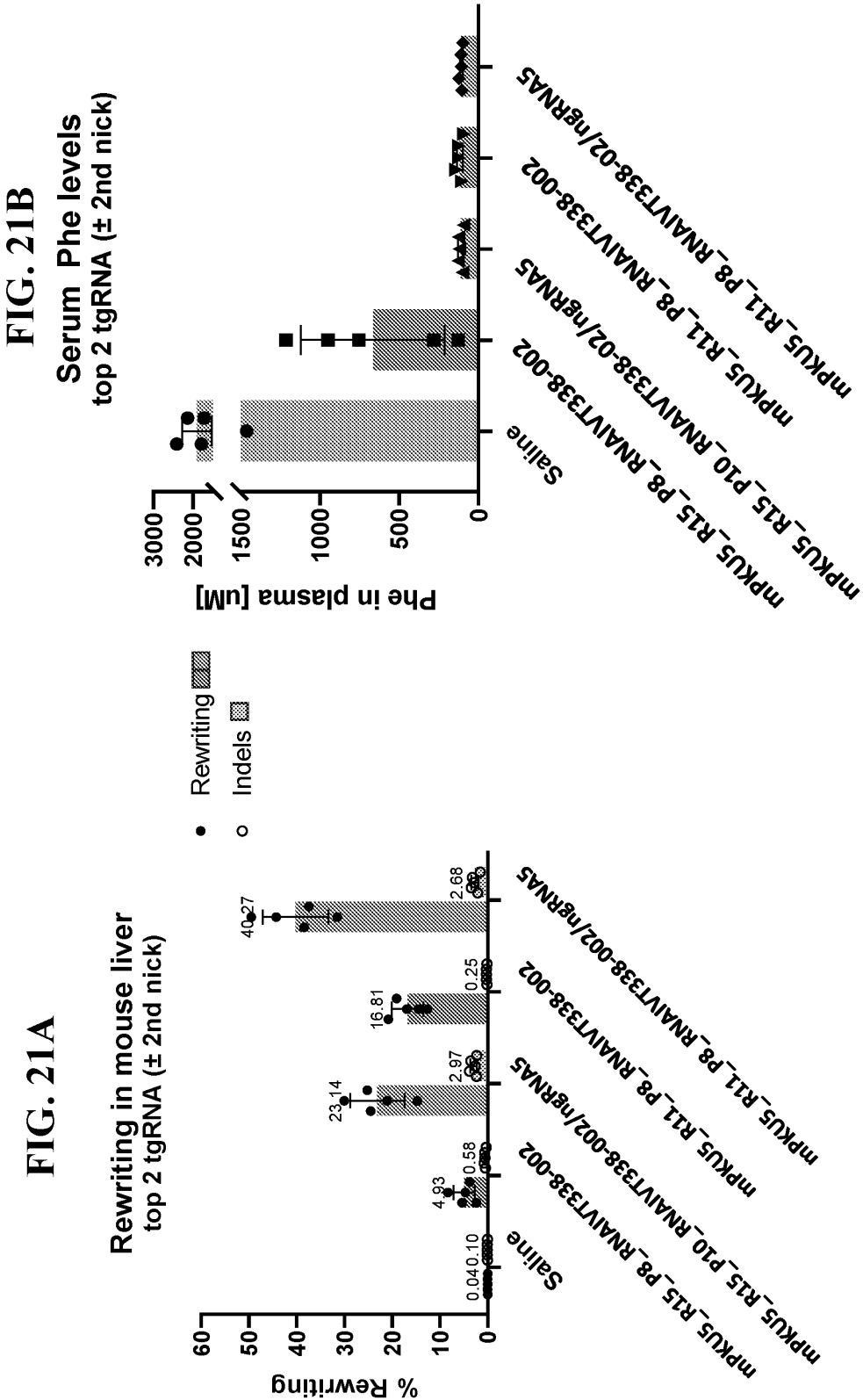
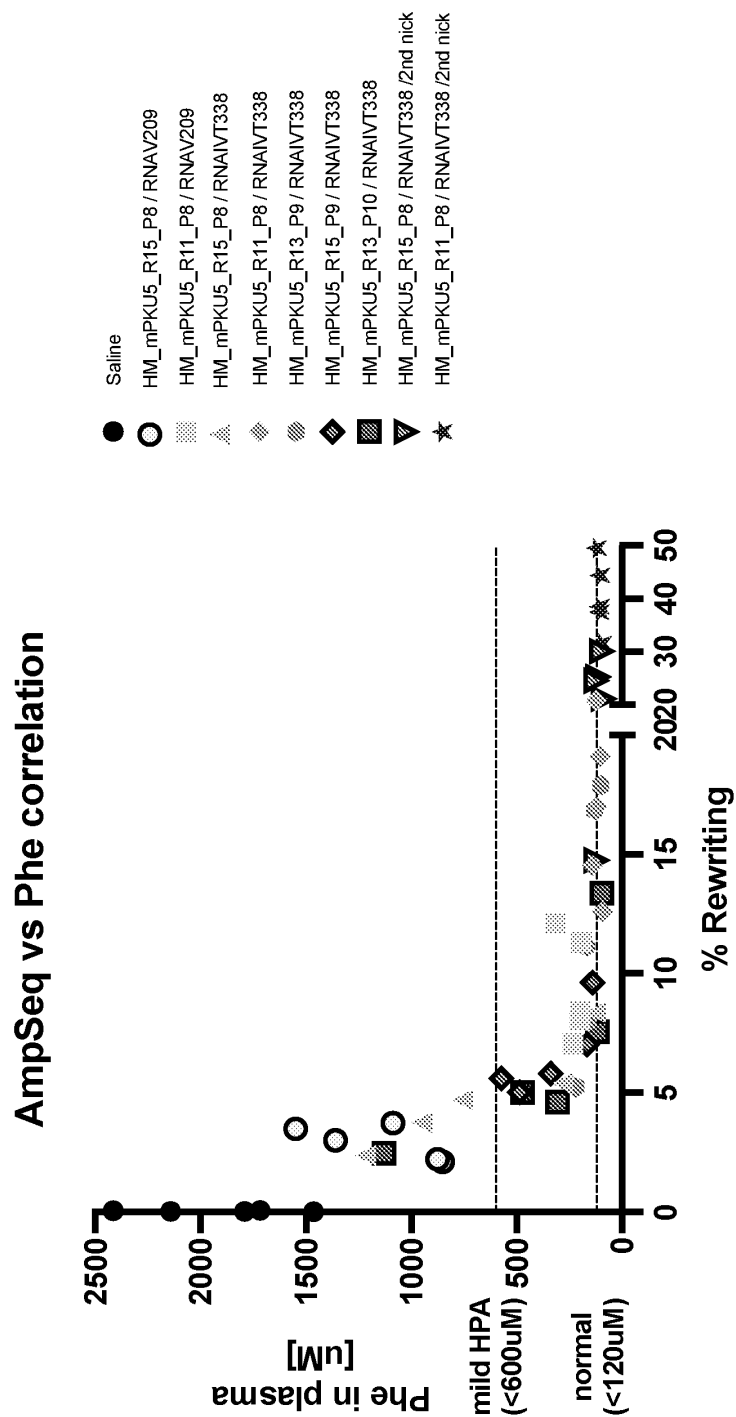


FIG. 22



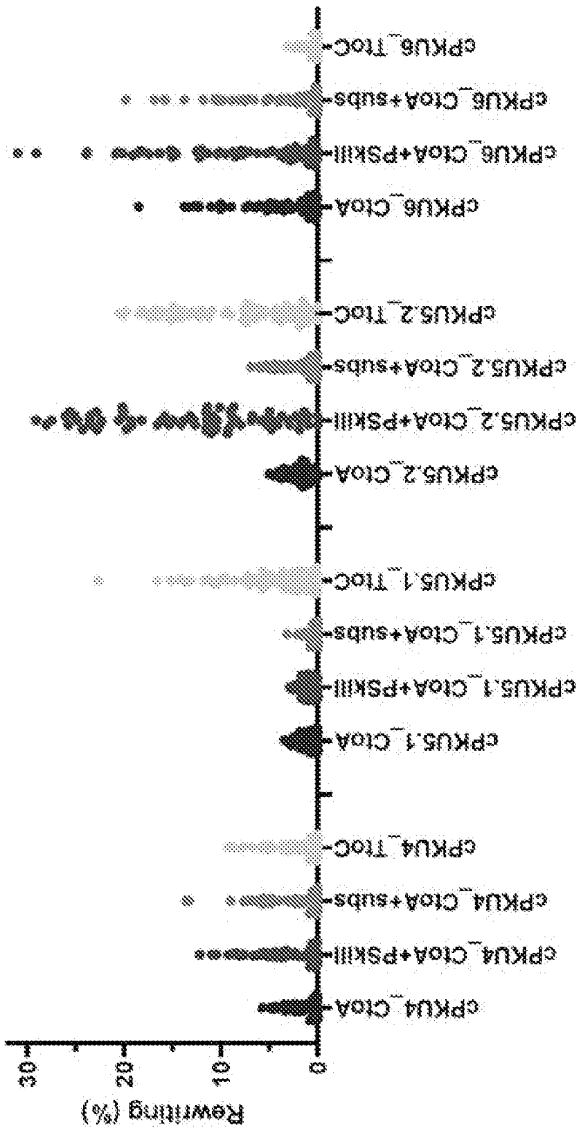


FIG. 23

FIG. 25A

hPKU11 install to 100% in PHH
(Running hPKU3.4.5 with AAVE RNAV1328.017)

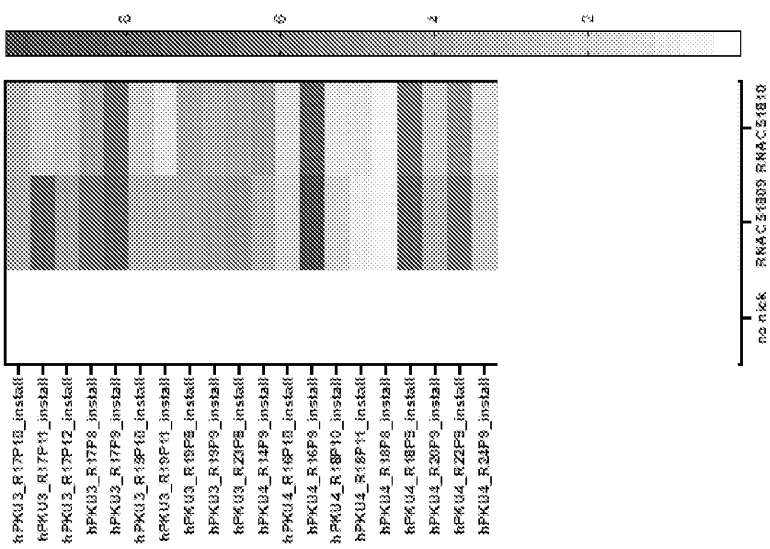


FIG. 25B

hPKU1 correct to 100% in PAH PSC-heps
(Running hPKU3.4.5 with AAVE RNAV1328.017)

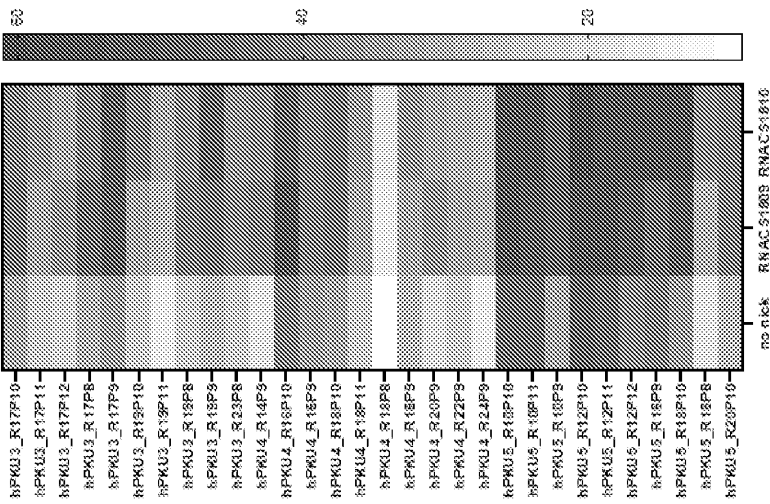


FIG. 25C

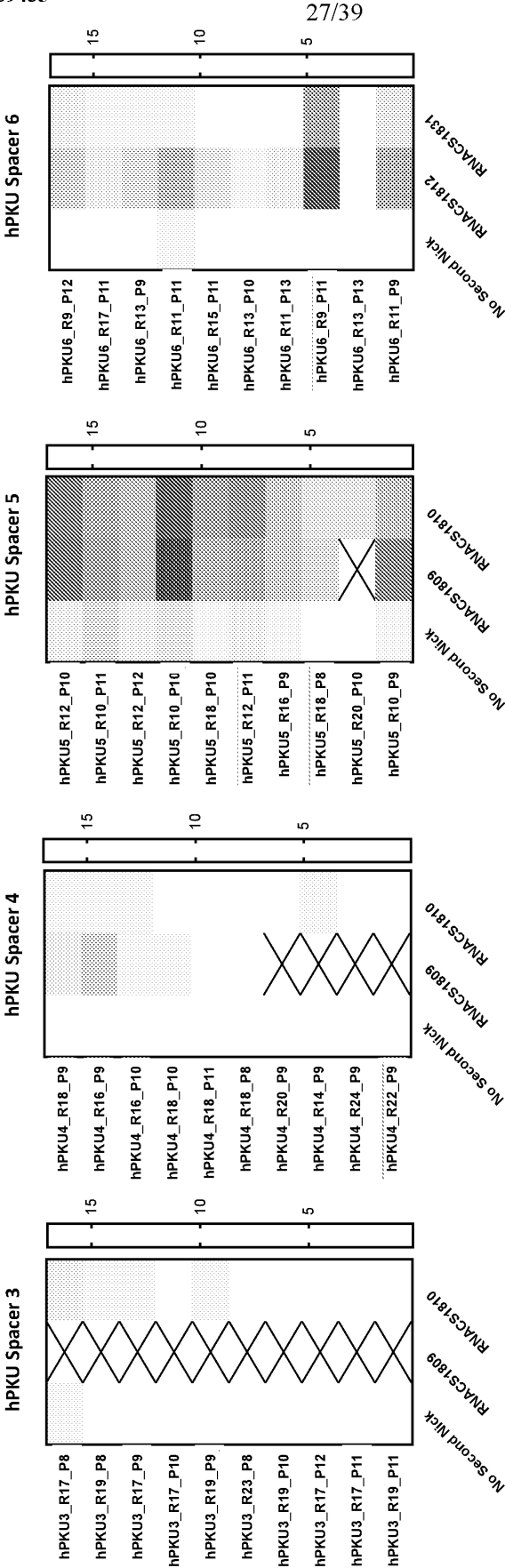


FIG. 26A

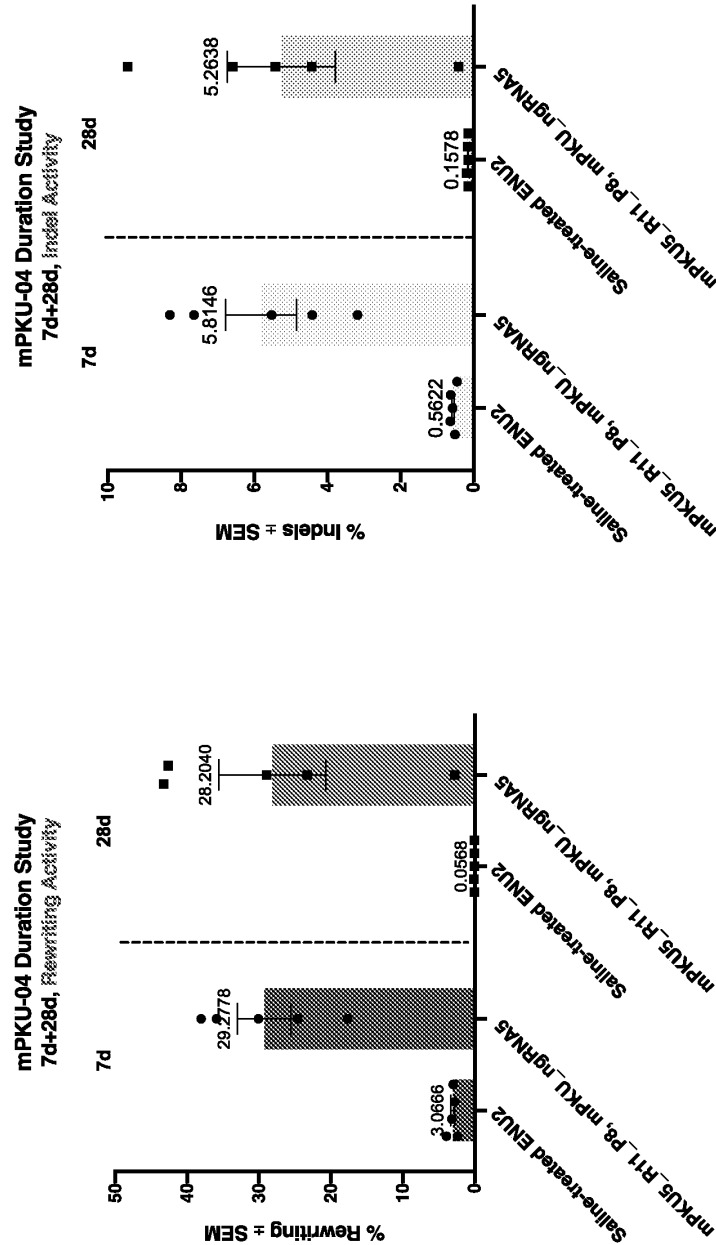


FIG. 26B

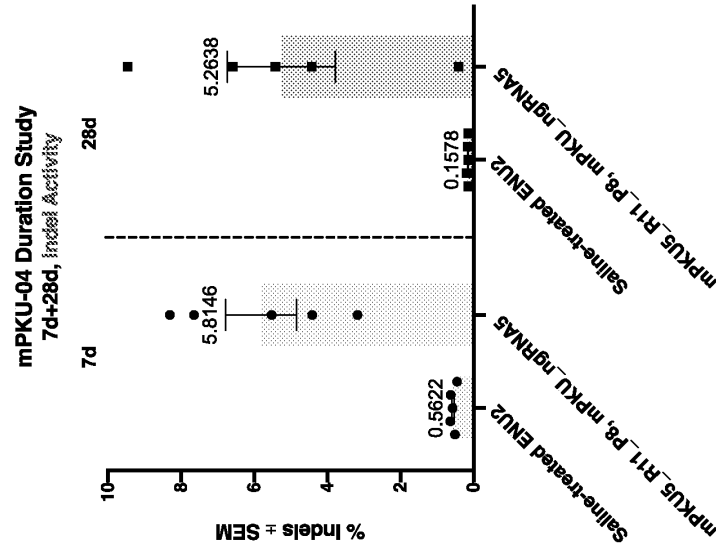


FIG. 27

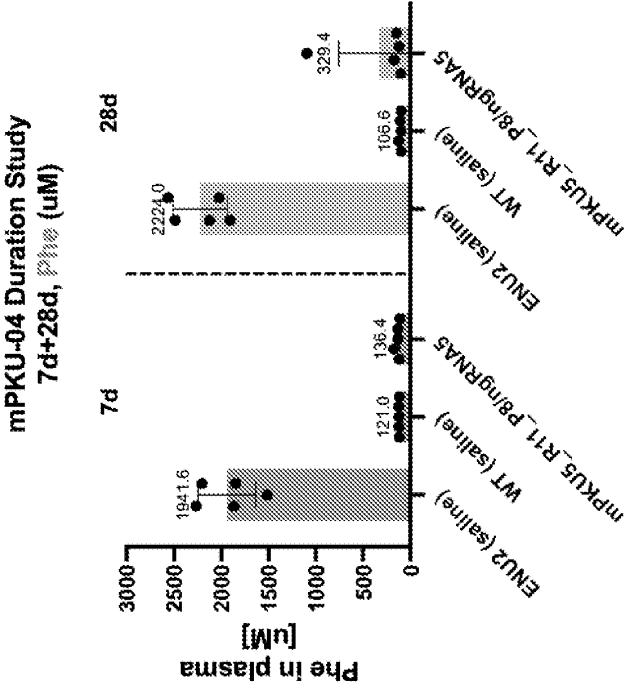


FIG. 28
mPKU-04 Duration Study
7d+28d, Brain Phe

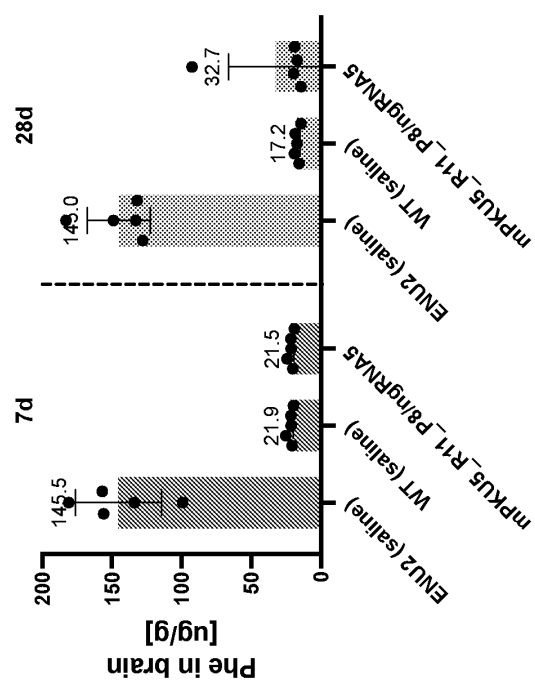
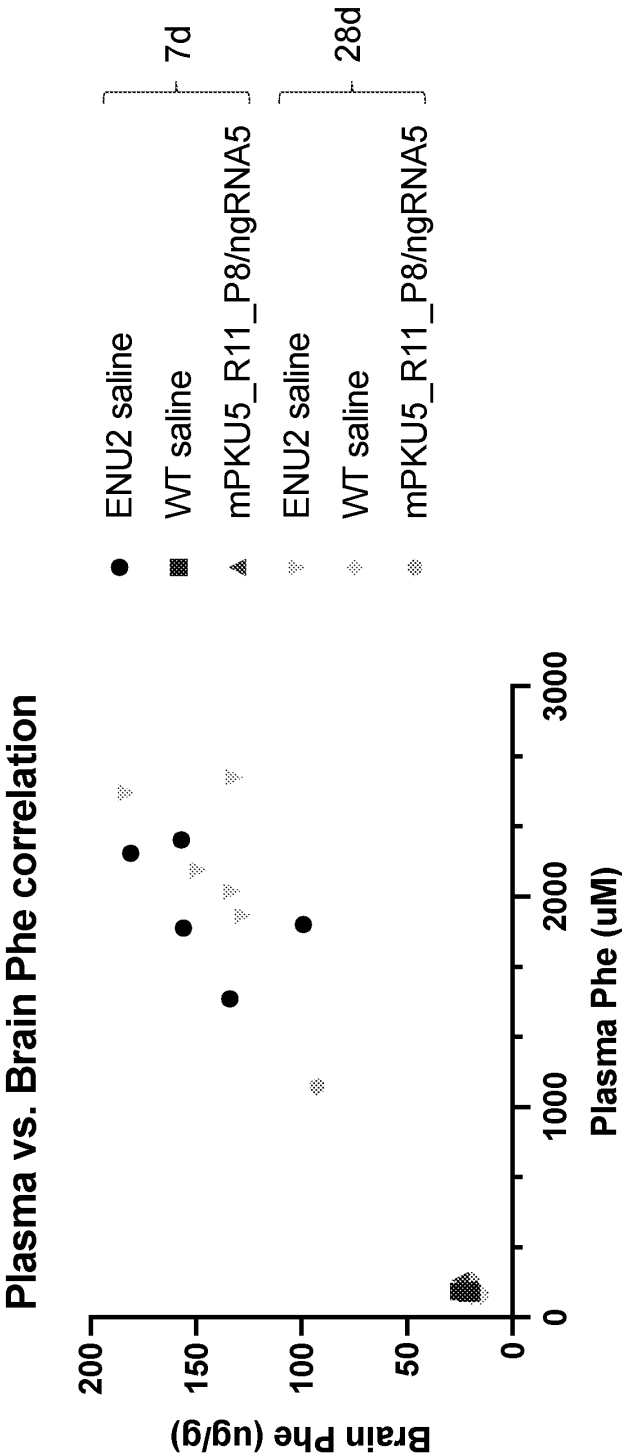
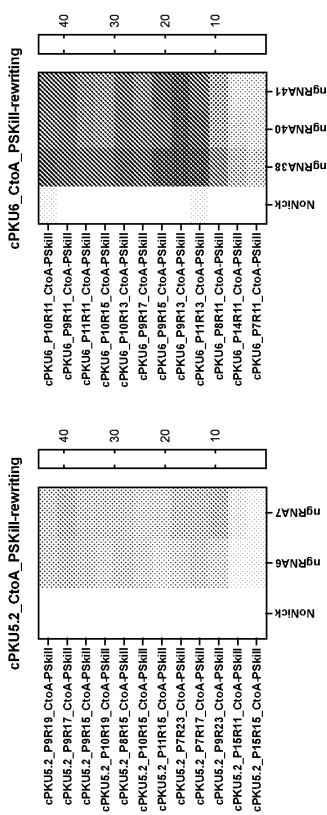
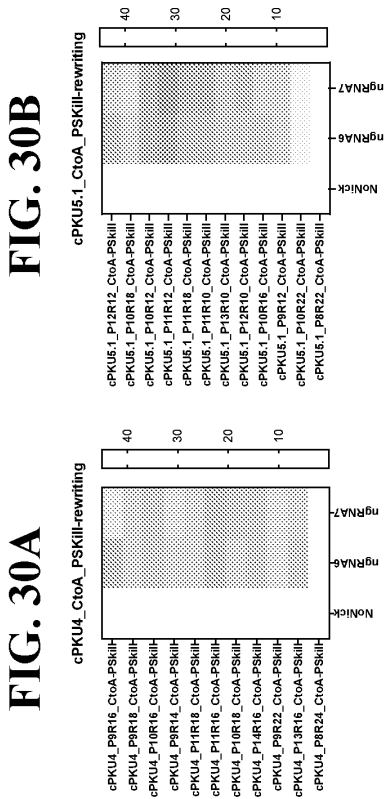
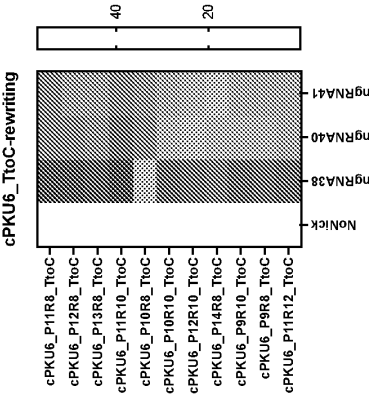
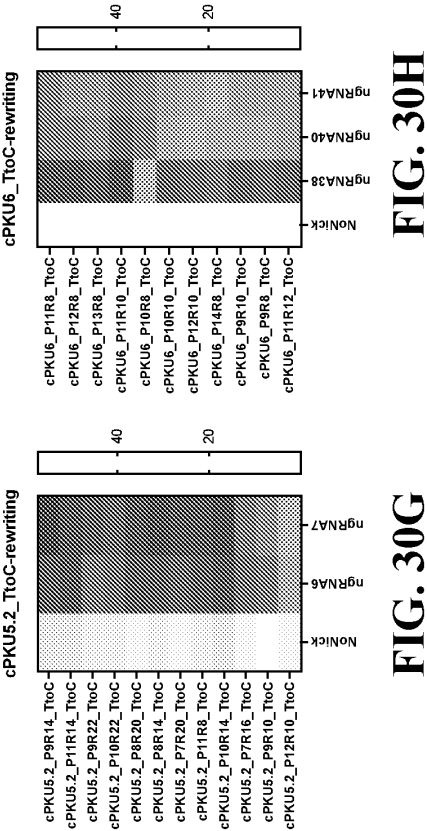
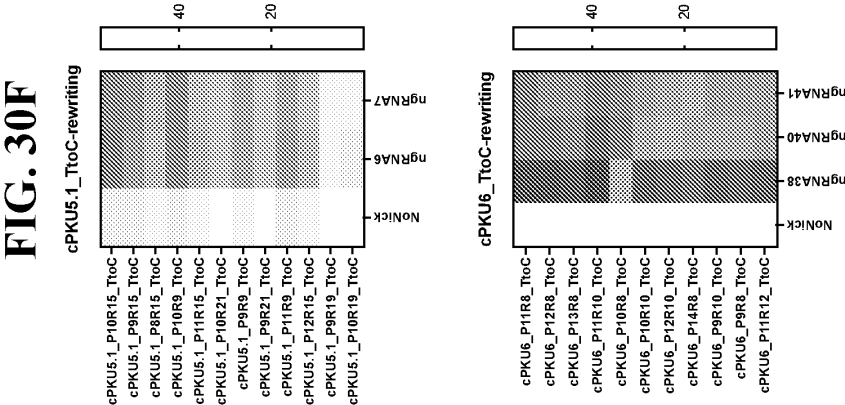
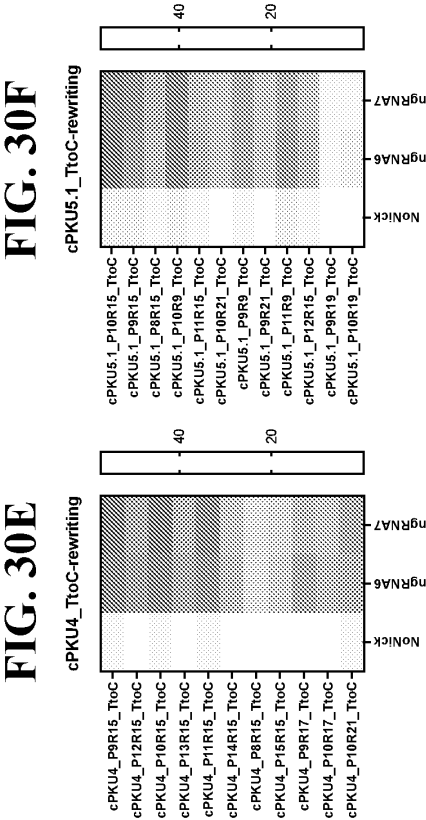


FIG. 29







	Ile				Pro				Arg				Pro				Nick			
Reference	A	T	A	A	C	C	T	T	G	G	C	C	C	C	T	T	C	T	C	T
sub0	A	T	A	C	C	T	C	G	G	C	C	C	C	C	T	T	C	T	C	T
sub1	A	T	A	C	C	G	C	G	G	C	C	C	C	C	T	T	C	T	C	T
sub2	A	T	C	C	C	T	C	G	G	C	C	C	C	C	T	T	C	T	C	T
sub4	A	T	A	C	C	T	C	G	C	C	C	C	C	C	T	T	C	T	C	T
sub7	A	T	A	C	C	G	C	G	C	C	C	C	C	C	T	T	C	T	C	T
sub8	A	T	A	C	C	G	C	G	C	C	C	C	C	C	A	T	T	C	T	C

FIG. 31B

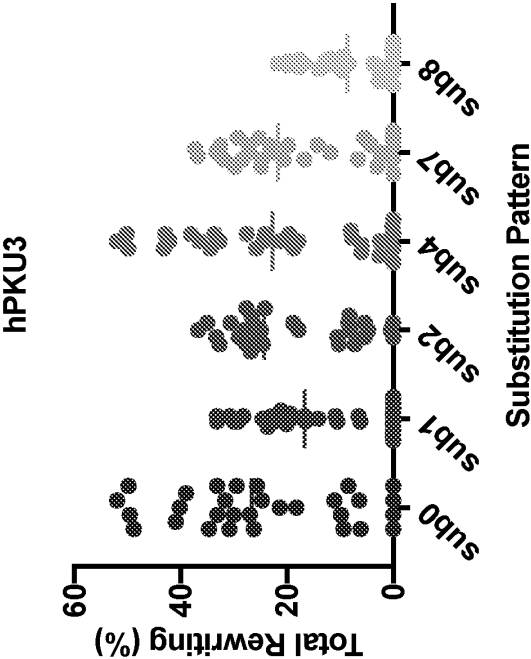


FIG. 31A

	Pro			Arg			Pro			Phe			Nick
Reference	C	C	T	T	G	G	C	C	C	T	T	C	
sub0	C	C	T	C	G	G	C	C	C	T	T	C	
sub1	C	C	G	C	G	G	C	C	C	T	T	C	
sub4	C	C	T	C	G	C	C	C	C	T	T	C	
sub5	C	C	T	C	G	C	C	A	C	T	T	C	
sub7	C	C	G	C	G	C	C	C	C	T	T	C	
sub8	C	C	G	C	G	C	C	A	C	T	T	C	

FIG. 32B

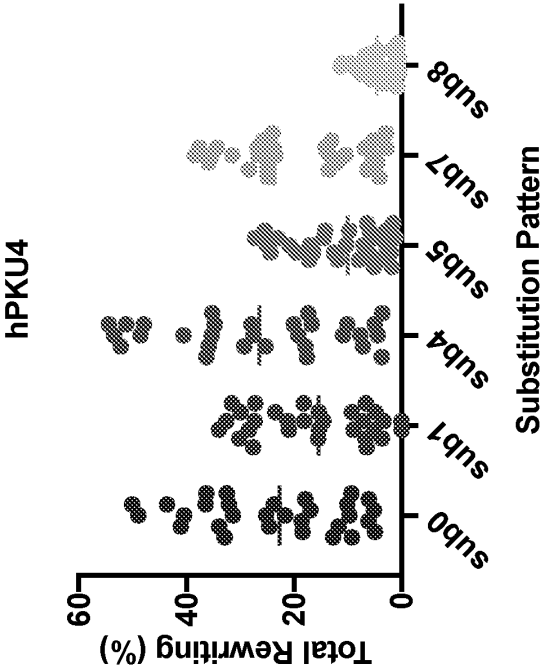


FIG. 32A

	Thr				Ile		Pro		Arg		Nick
	A	C	A	A	T	A	C	C	T	T	
Reference	A	C	A	A	T	A	C	C	T	T	G
sub0	A	C	A	A	T	A	C	C	T	C	G
sub1	A	C	A	A	T	A	C	C	G	C	G
sub2	A	C	A	A	T	C	C	C	T	C	G
sub3	A	C	A	A	T	C	C	C	G	C	G
sub4	A	C	A	A	T	A	C	C	T	C	C
sub7	A	C	G	A	T	A	C	C	T	C	G

FIG. 33B

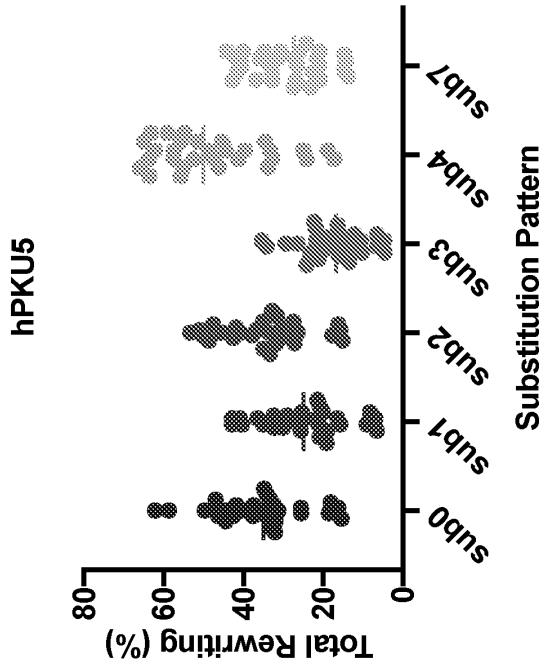


FIG. 33A

Nick	←			Pro			Arg			Pro			→
	←			Pro			Arg			Pro			
	Reference	G	G	G	C	C	C	A	A	G	G		
	sub0	G	G	G	C	C	C	A	A	G	G		
	sub1	G	G	G	C	C	C	G	G	G	G		
	sub4	C	G	G	C	C	C	G	G	G	G		
	sub5	C	G	G	A	C	G	G	G	G	G		
	sub6	G	G	G	C	C	C	C	C	G	G		
	sub7	G	G	G	A	C	G	C	C	G	G		

FIG. 34B

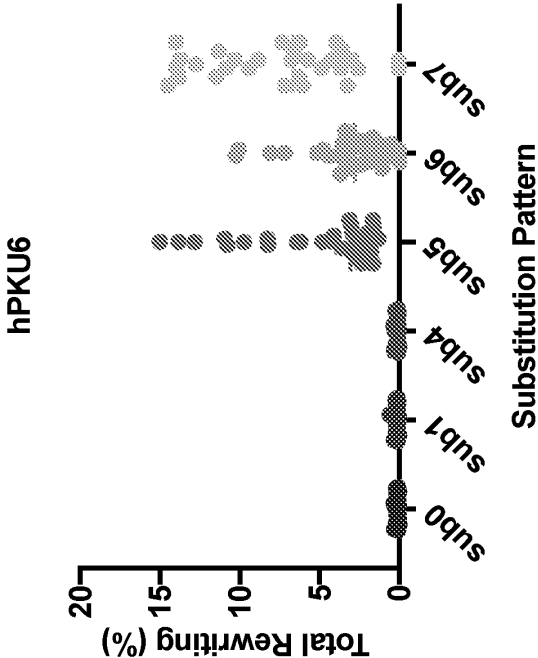


FIG. 34A

FIG. 35

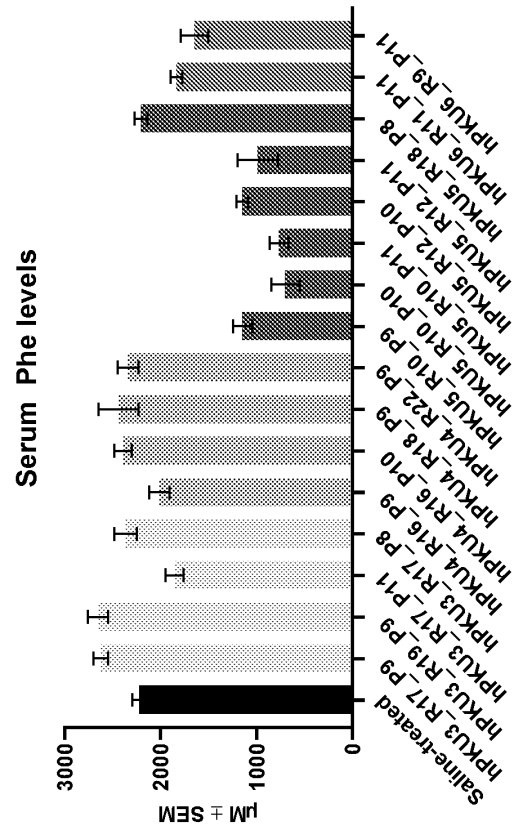


FIG. 36B

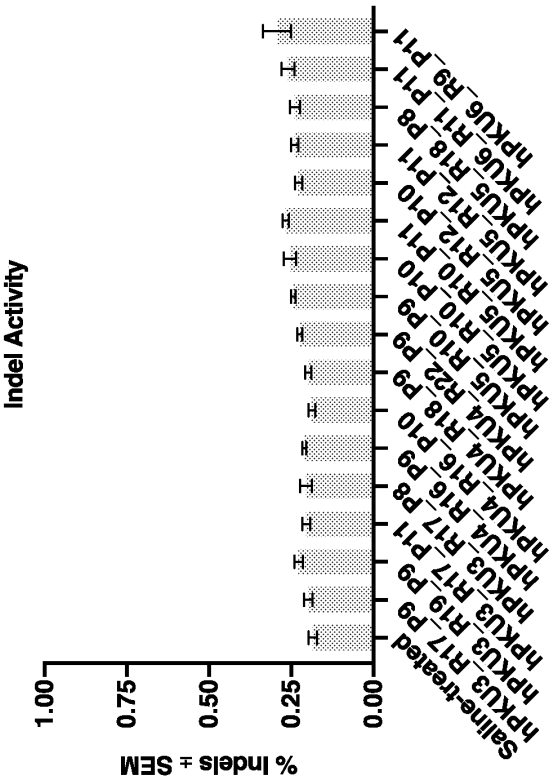


FIG. 36A

