

Anti-hTfR scFv antibody clone	Amino acid sequence (Vk-3xG4S-Vh) SEQ ID NO
H1H12795B	SEQ ID NO: 492
H1H12798B	SEQ ID NO: 493
H1H12799B	SEQ ID NO: 494
H1H12801B	SEQ ID NO: 495
H1H12802B	SEQ ID NO: 496
H1H12808B	SEQ ID NO: 497
H1H12812B	SEQ ID NO: 498
H1H12816B	SEQ ID NO: 499
H1H12833B	SEQ ID NO: 500
H1H12834B	SEQ ID NO: 501
H1H12835B	SEQ ID NO: 502
H1H12839B	SEQ ID NO: 503
H1H12841B	SEQ ID NO: 504
H1H12843B	SEQ ID NO: 505
H1H12844B	SEQ ID NO: 506
H1H12845B	SEQ ID NO: 507
H1H12847B	SEQ ID NO: 508
H1H12848B	SEQ ID NO: 509
H1H12850B	SEQ ID NO: 510
H1H31863B	SEQ ID NO: 511
H1H31874B	SEQ ID NO: 512
PN69261	SEQ ID NO: 513
PN69263	SEQ ID NO: 514
PN69305	SEQ ID NO: 515
PN69307	SEQ ID NO: 516
PN69323	SEQ ID NO: 517
PN69326	SEQ ID NO: 518
PN69329	SEQ ID NO: 519
PN69331	SEQ ID NO: 520
PN69332	SEQ ID NO: 521
PN69340	SEQ ID NO: 522
PN69348	SEQ ID NO: 523
Anti-hTfR scFv:hGAA (12799B-2xG4S-GAA) with optional 29-aa N-terminal signal sequence (SEQ ID NO: 644)	
Anti-hTfR scFv:hGAA (12839B-2xG4S-GAA) with optional 29-aa N-terminal signal sequence (SEQ ID NO: 645)	
Anti-hTfR scFv:hGAA (12843B-2xG4S-GAA) with optional 29-aa N-terminal signal sequence (SEQ ID NO: 646)	
Anti-hTfR scFv:hGAA (12847B-2xG4S-GAA) with optional 29-aa N-terminal signal sequence (SEQ ID NO: 647)	

FIG. 1

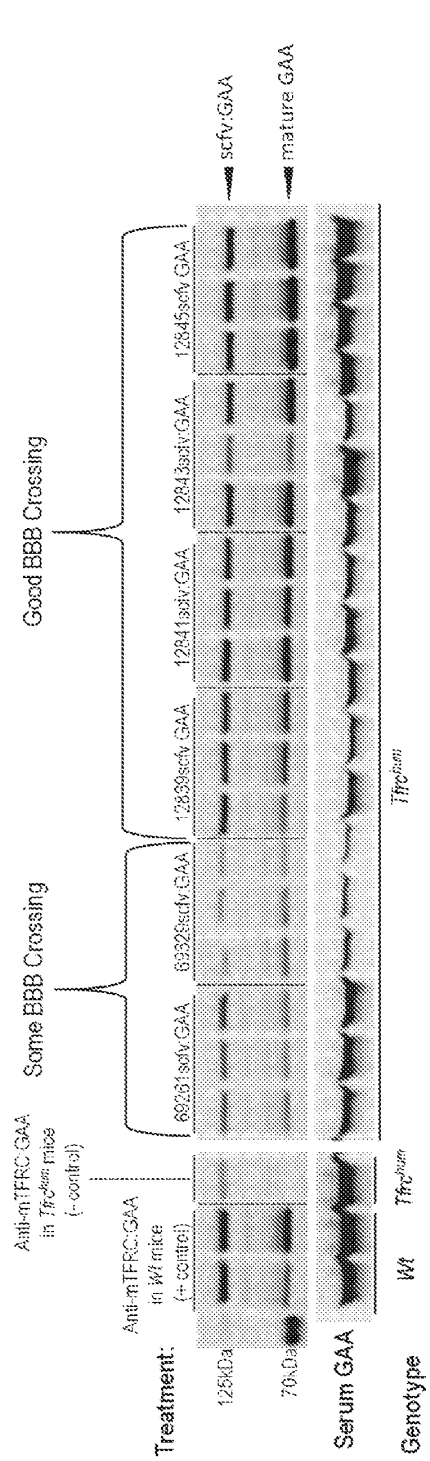


FIG. 2A

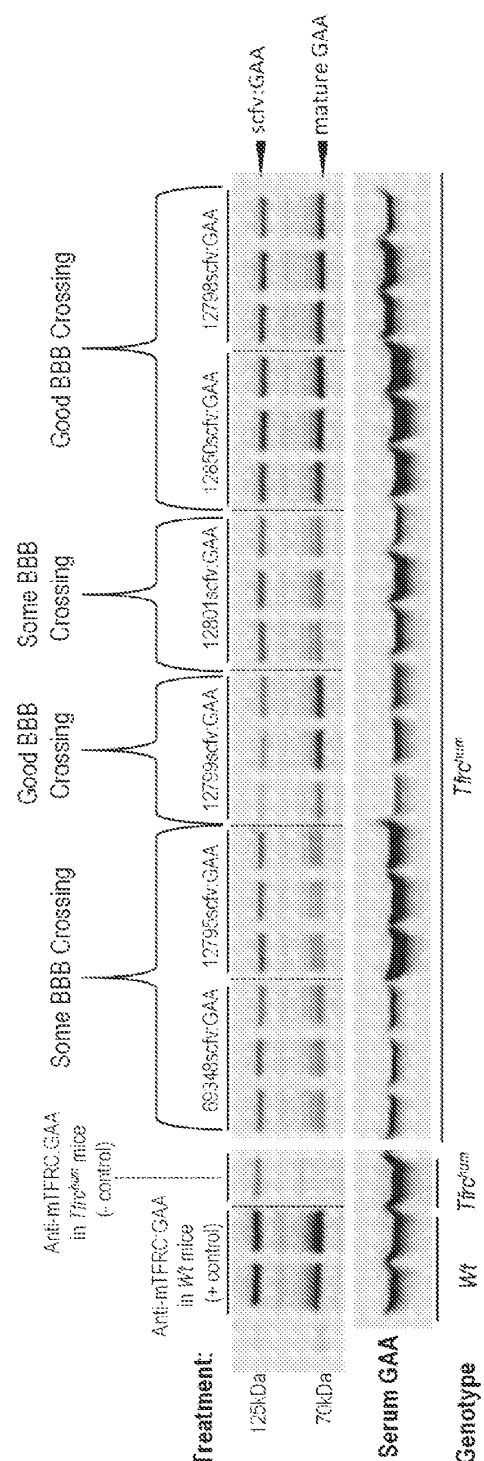


FIG. 2B

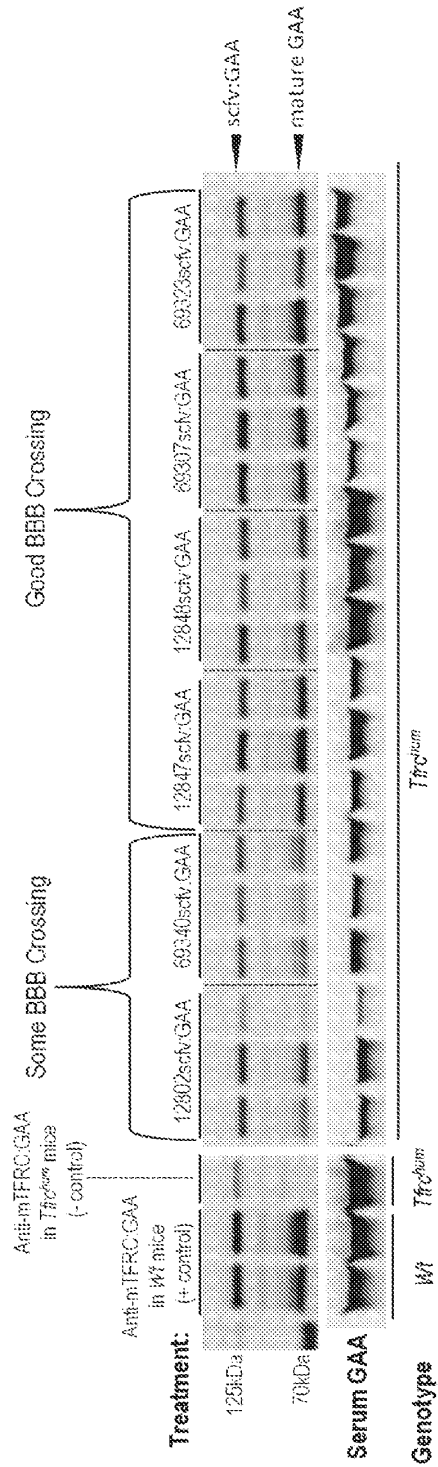


FIG. 2C

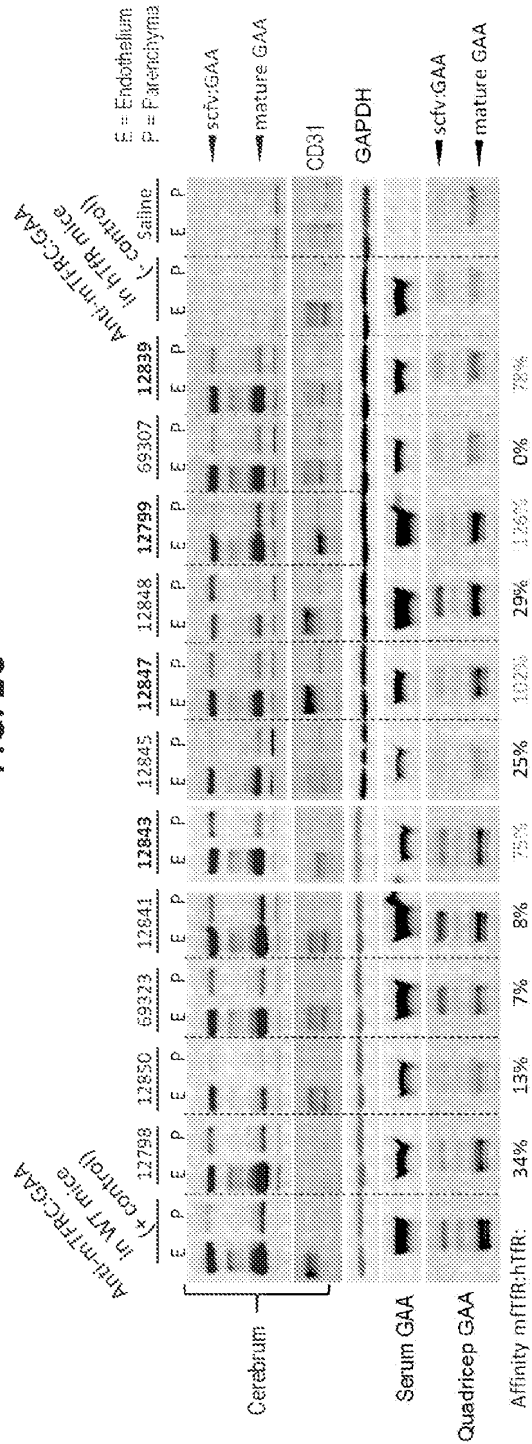


FIG. 3

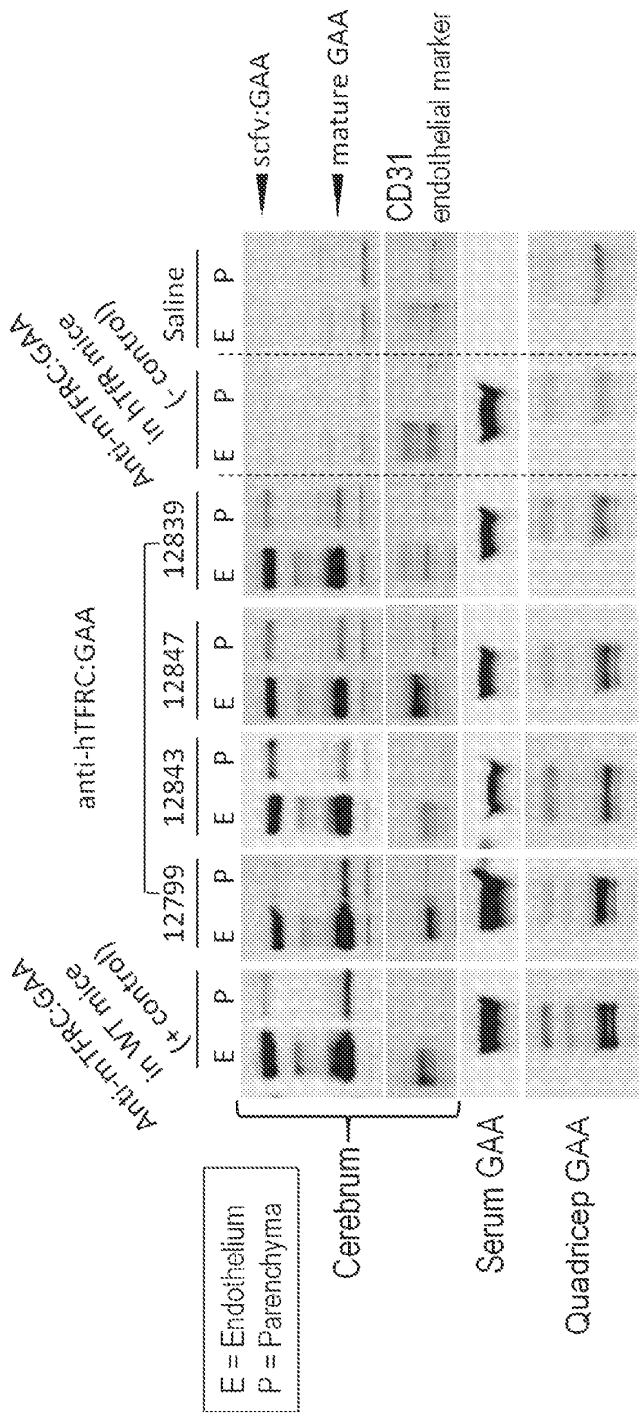


FIG. 4

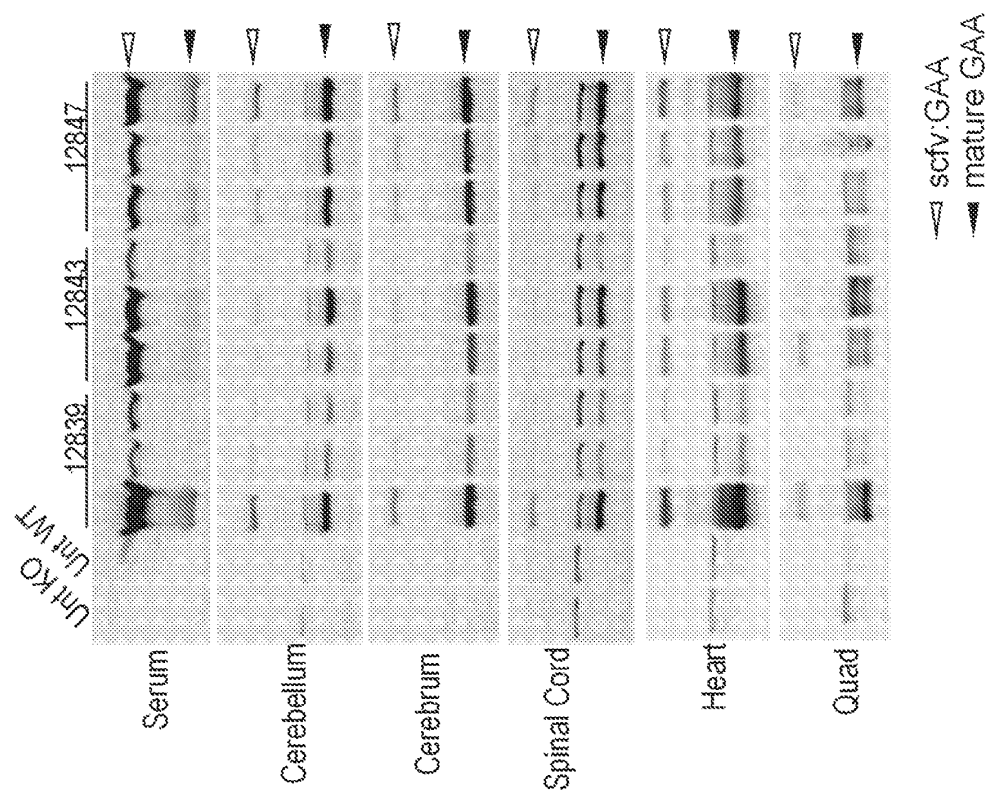


FIG. 5

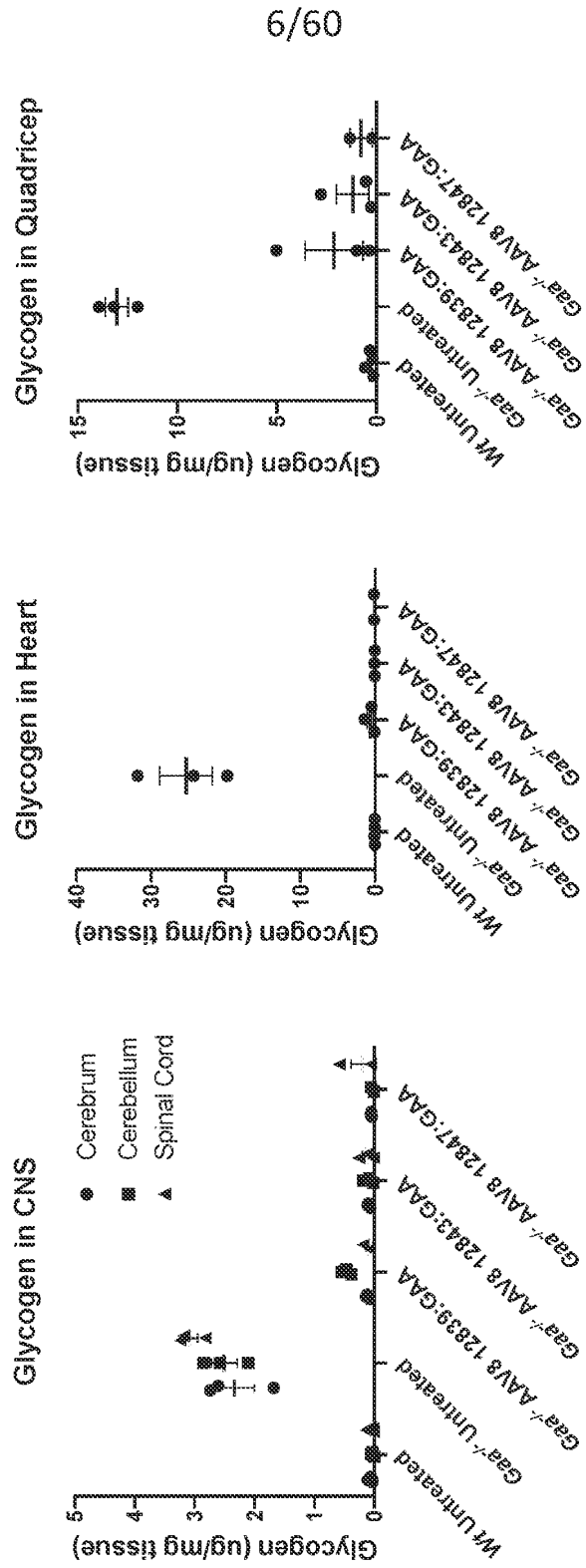


FIG. 6

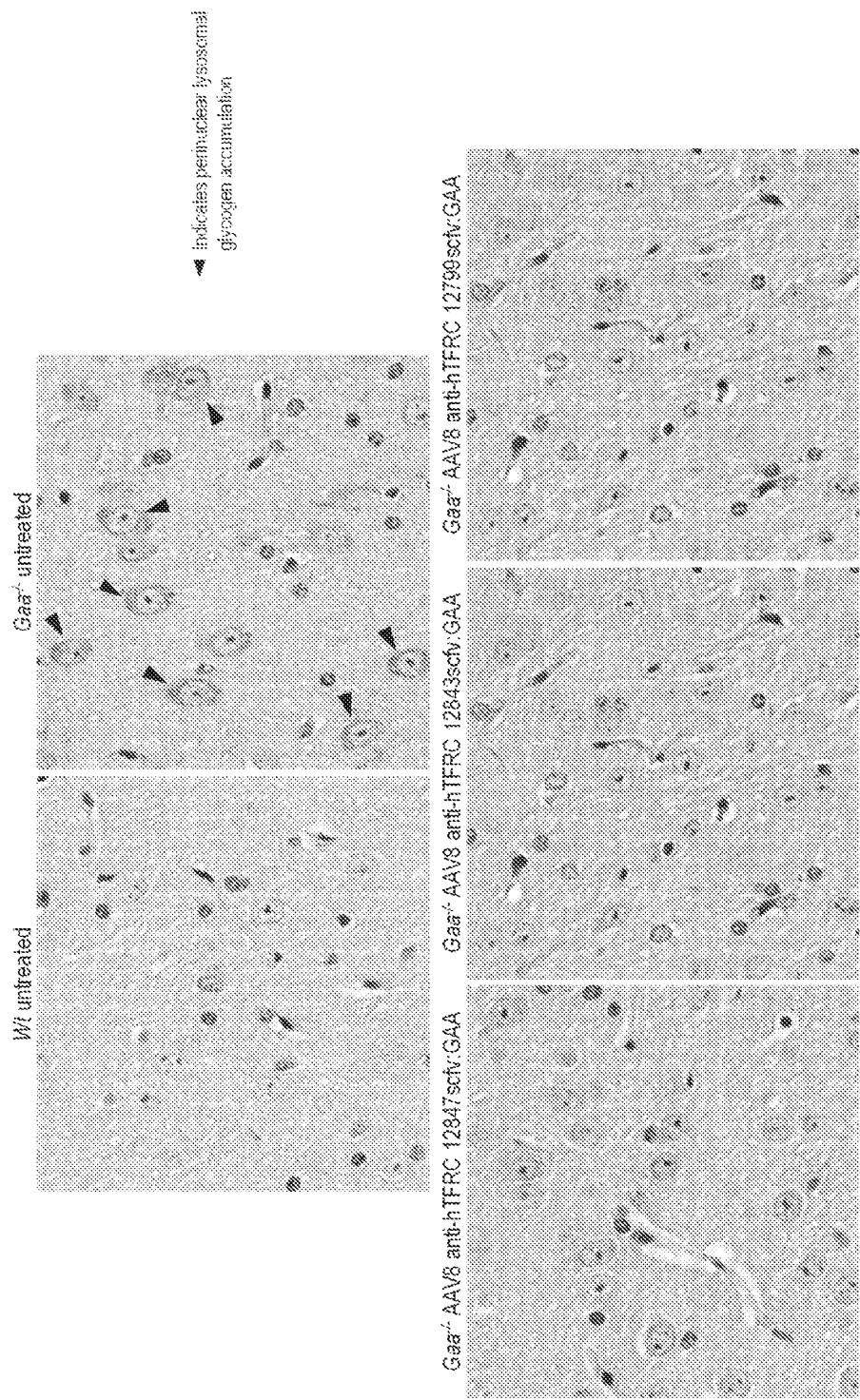


FIG. 7A

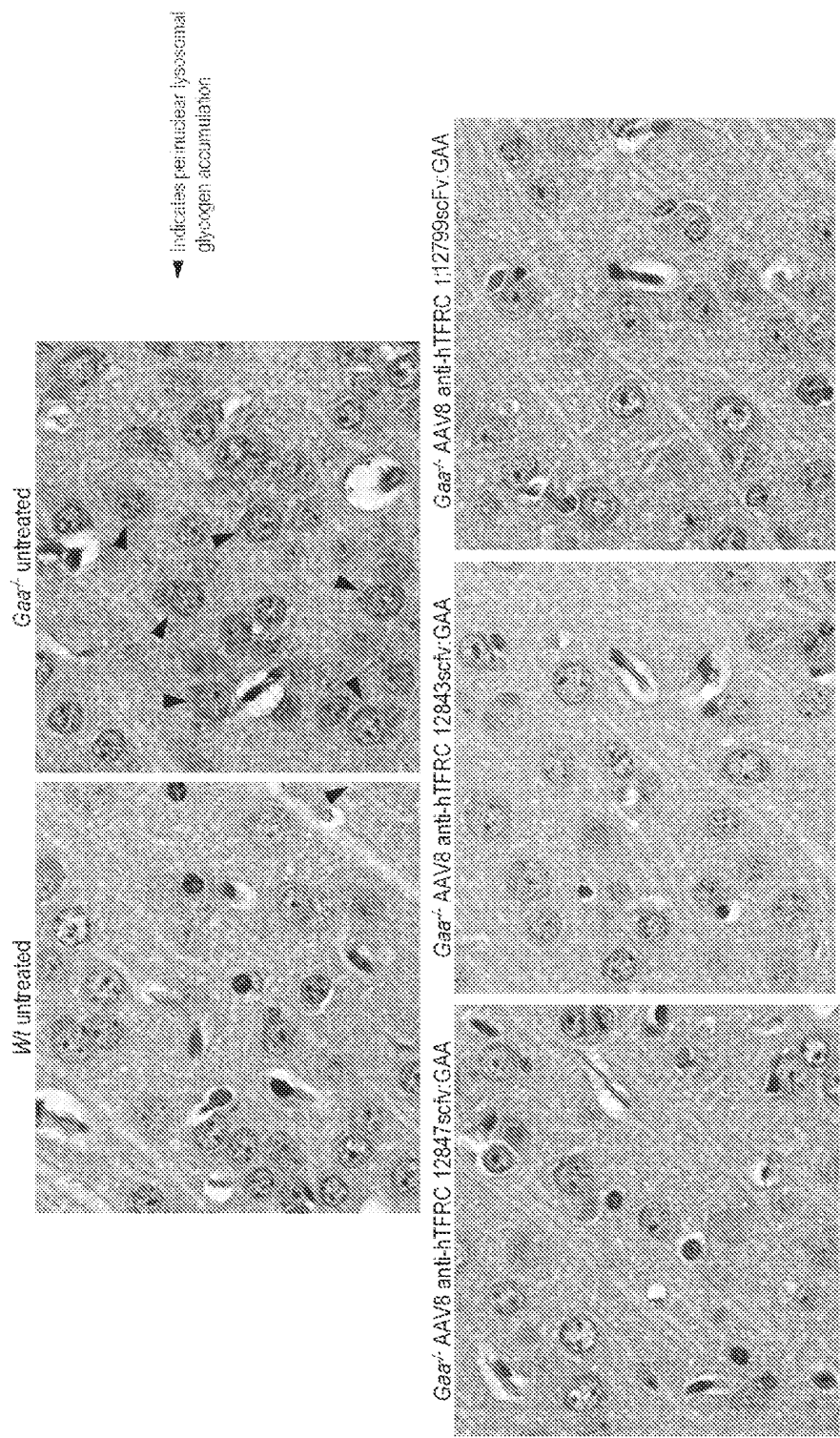


FIG. 7B

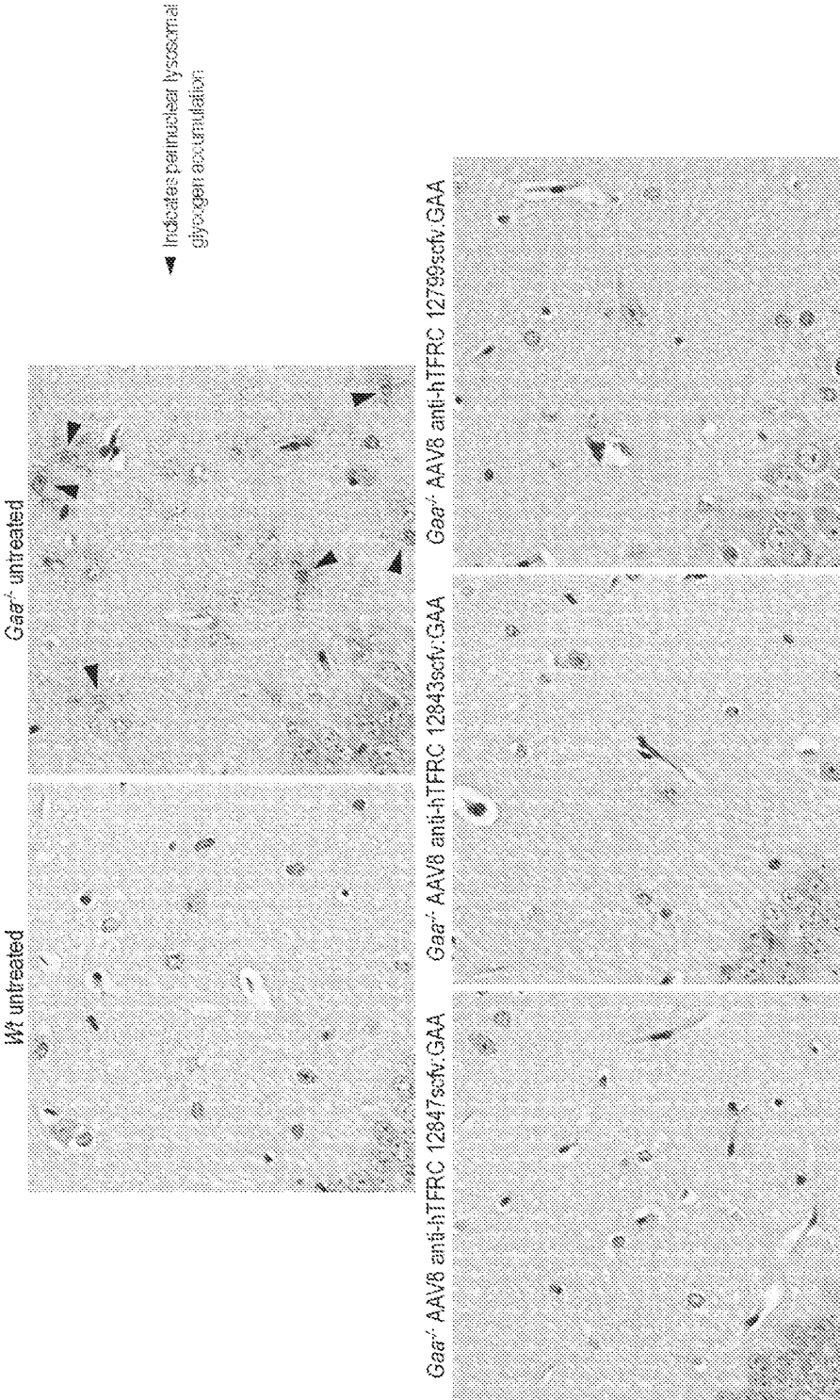


FIG. 7C

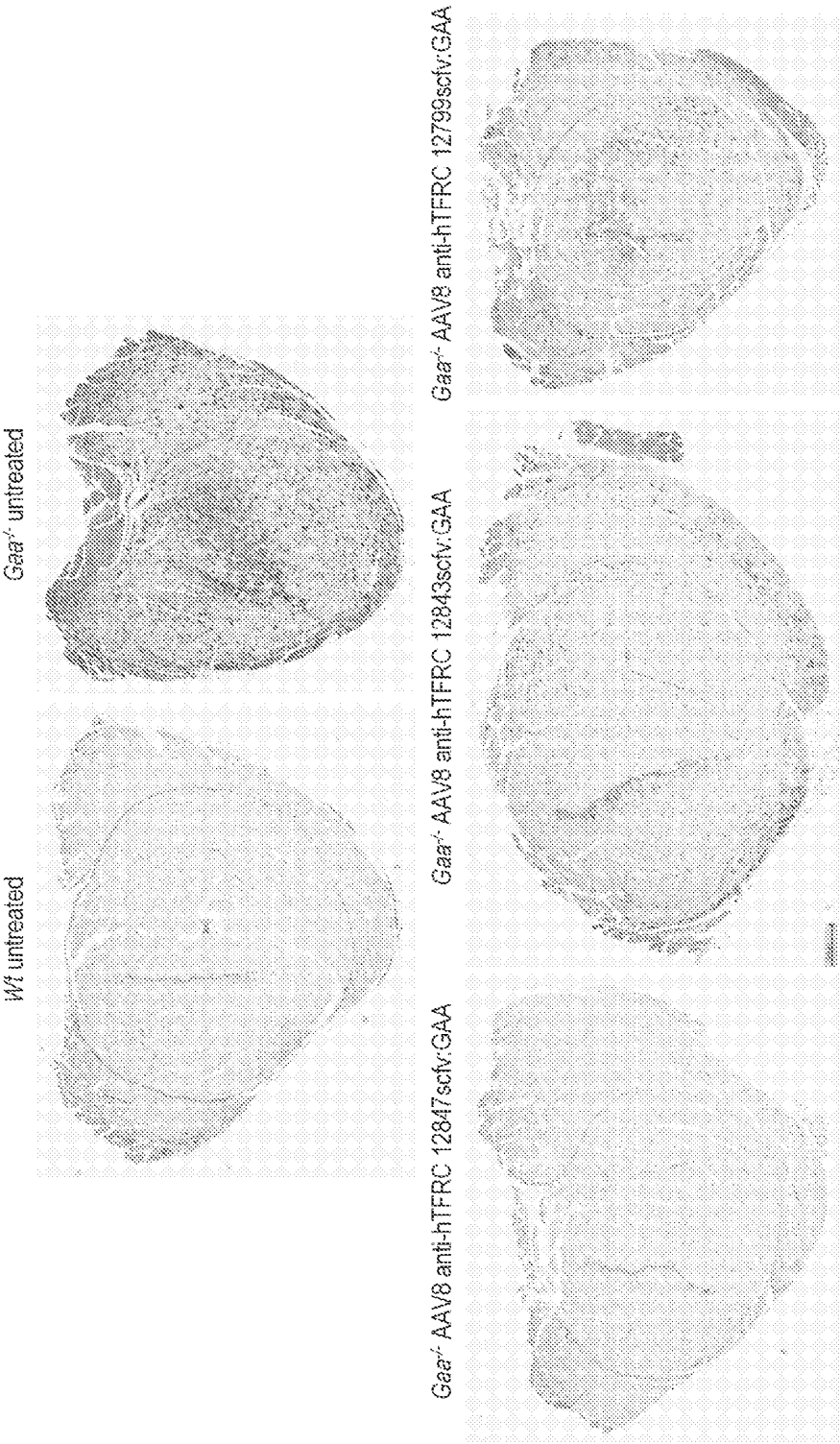


FIG. 7D

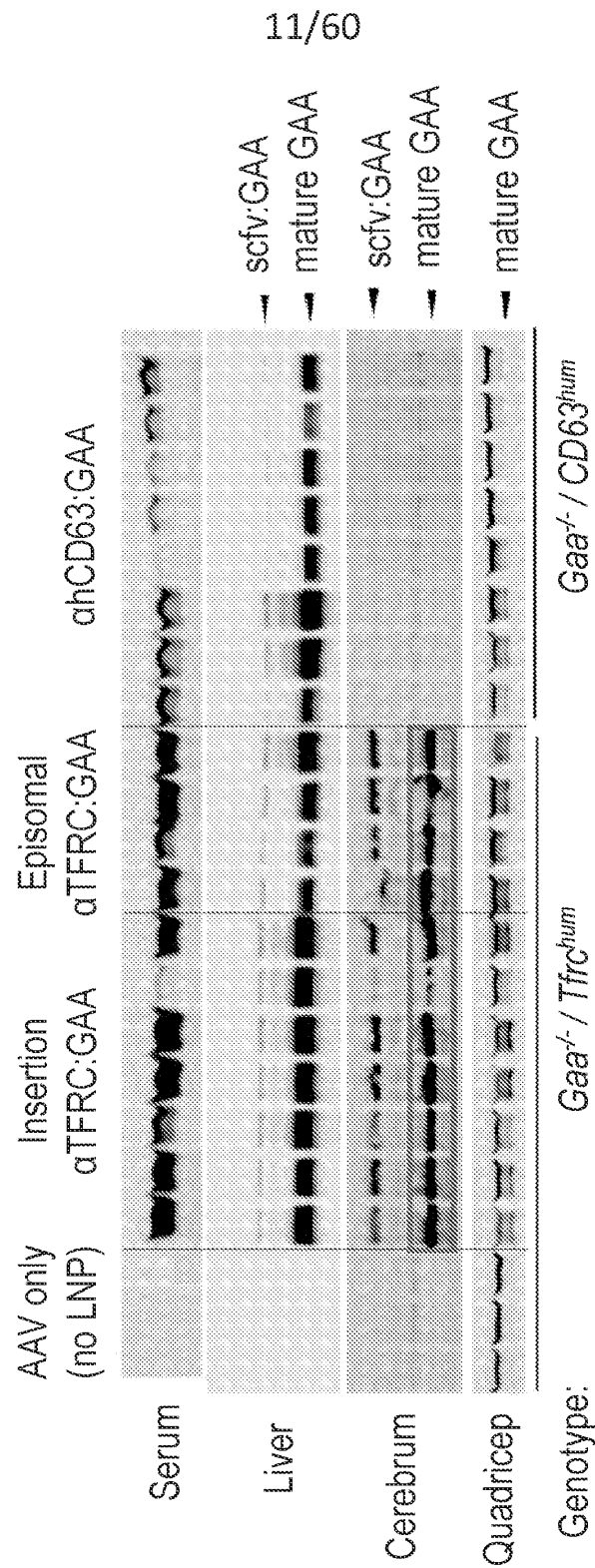
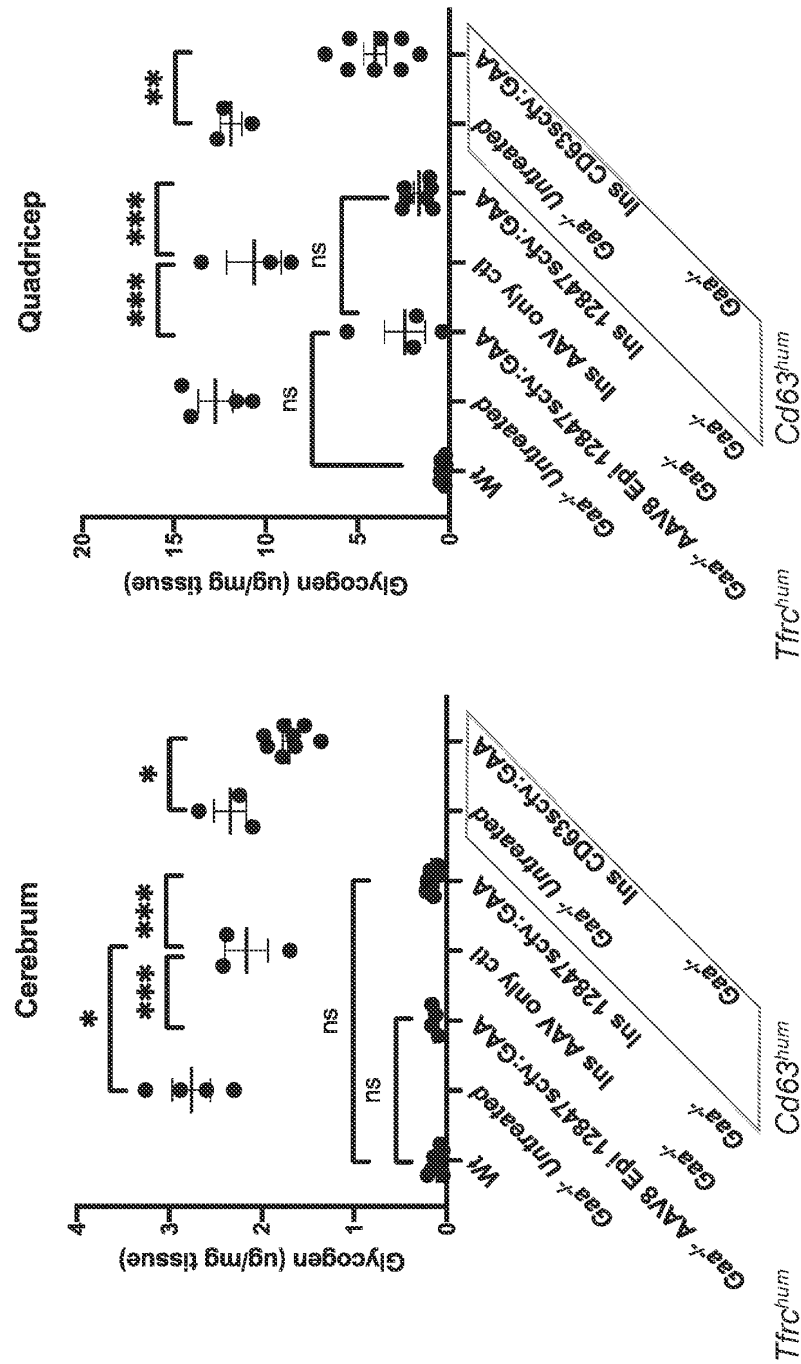


FIG. 8



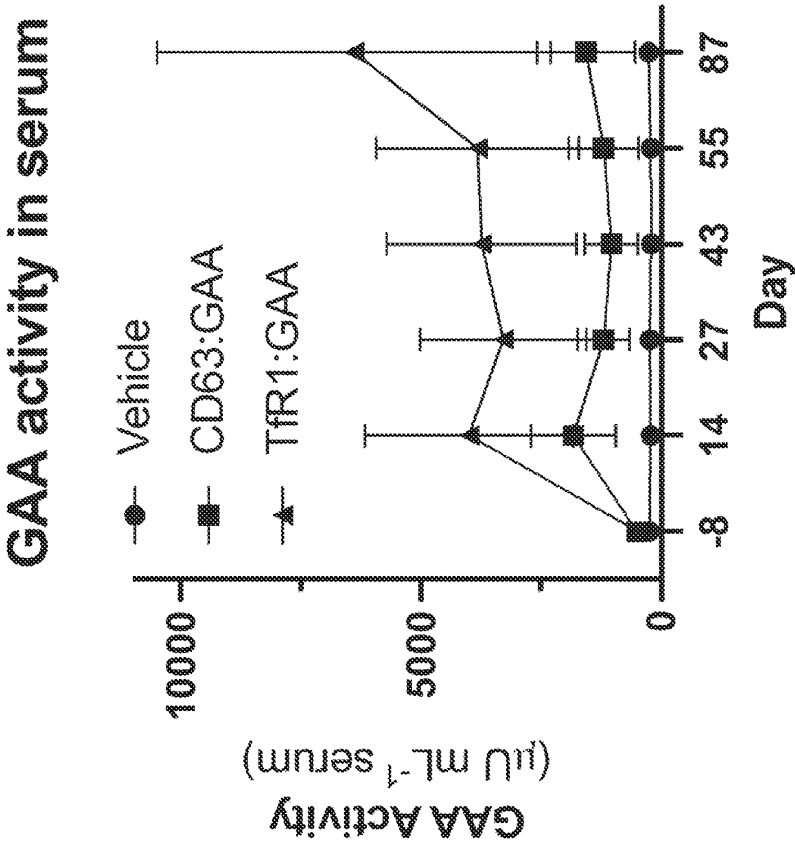


FIG. 10

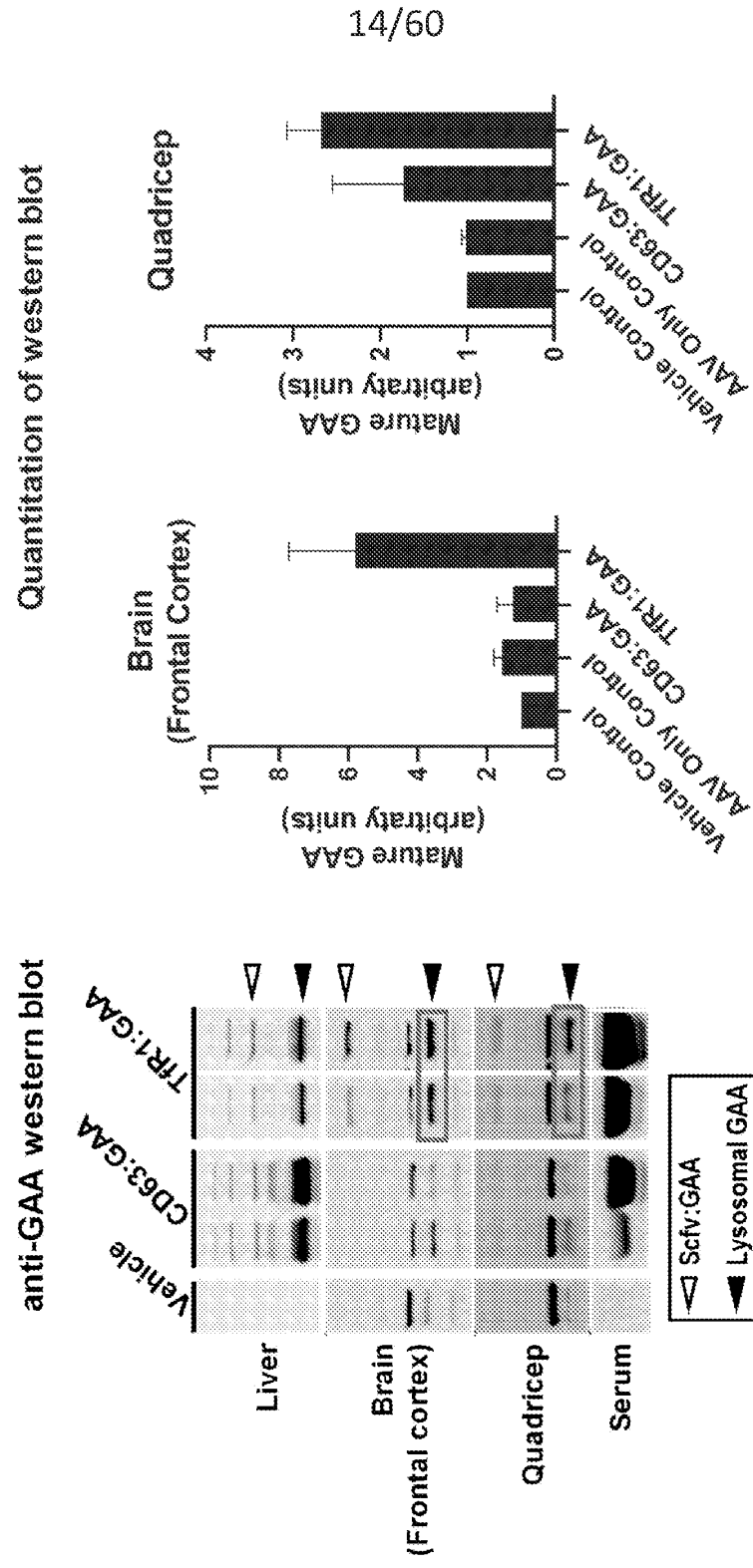


FIG. 11



FIG. 12

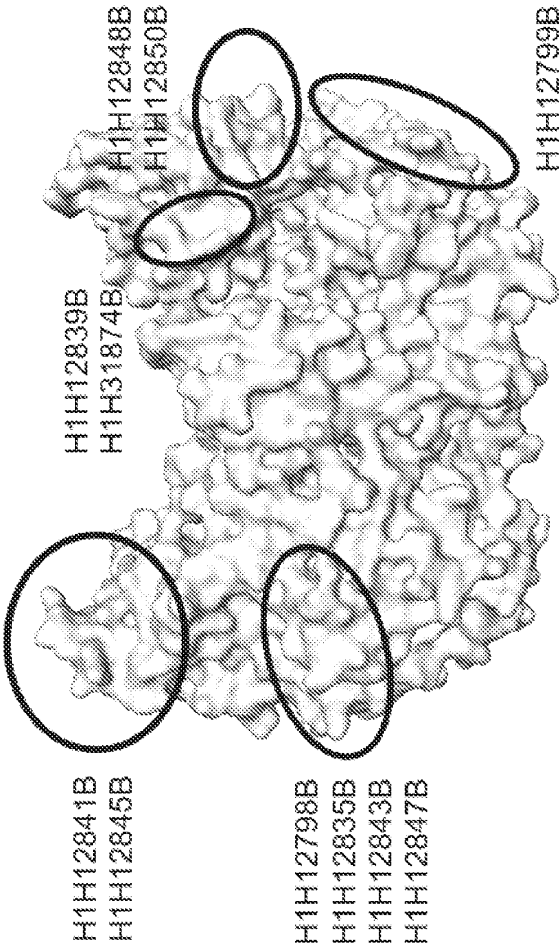


FIG. 13

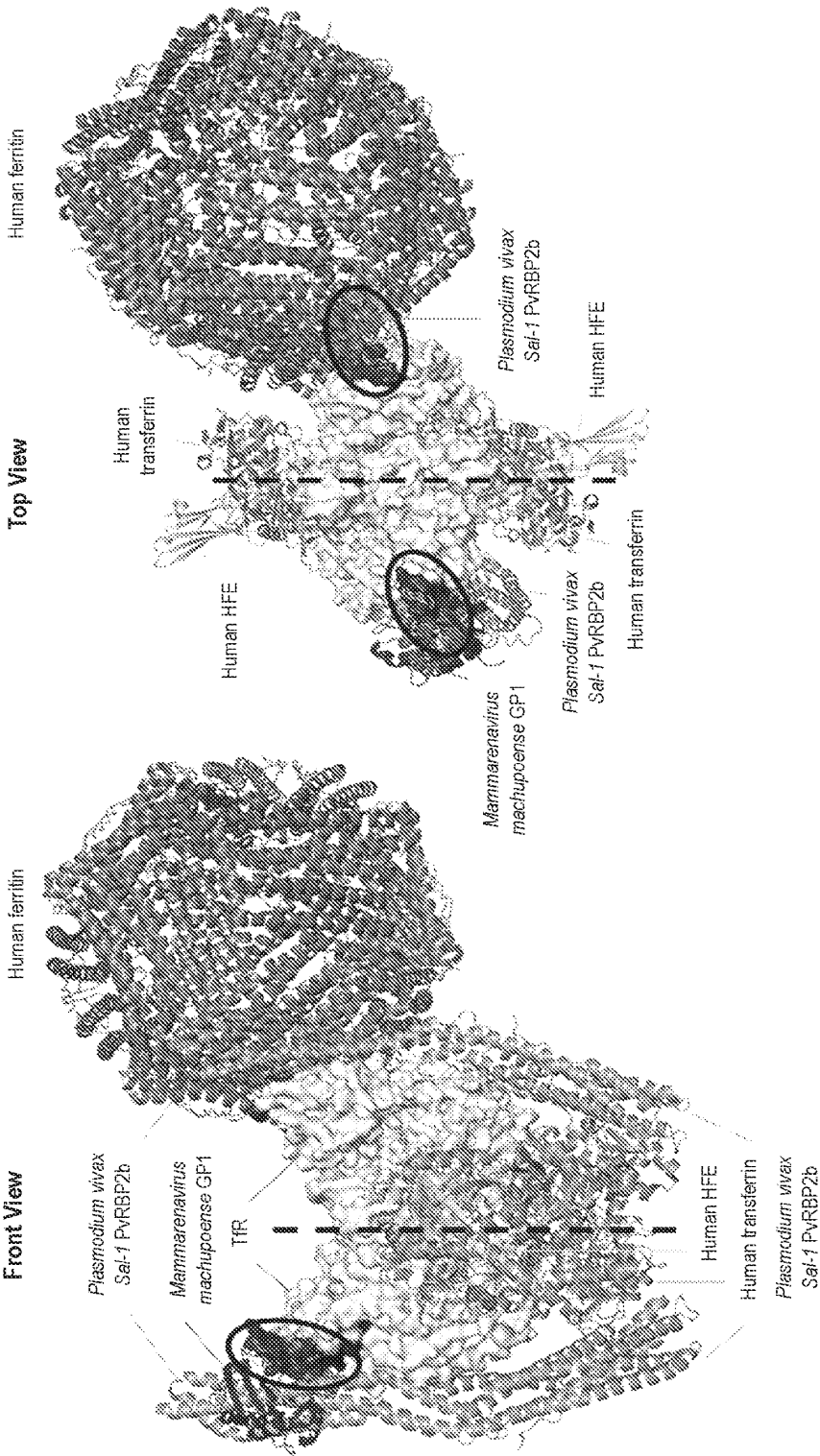


FIG. 14

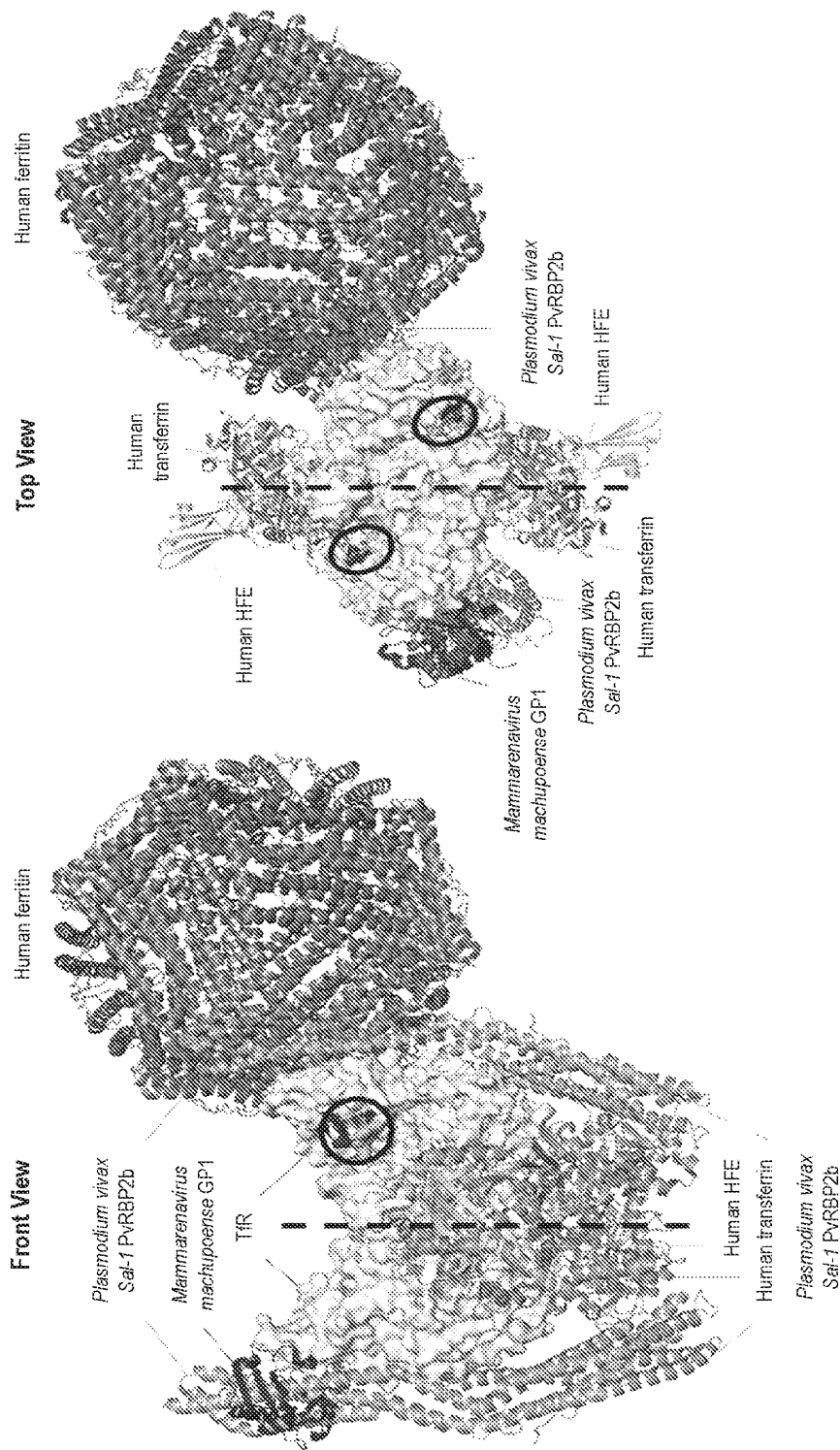


FIG. 15

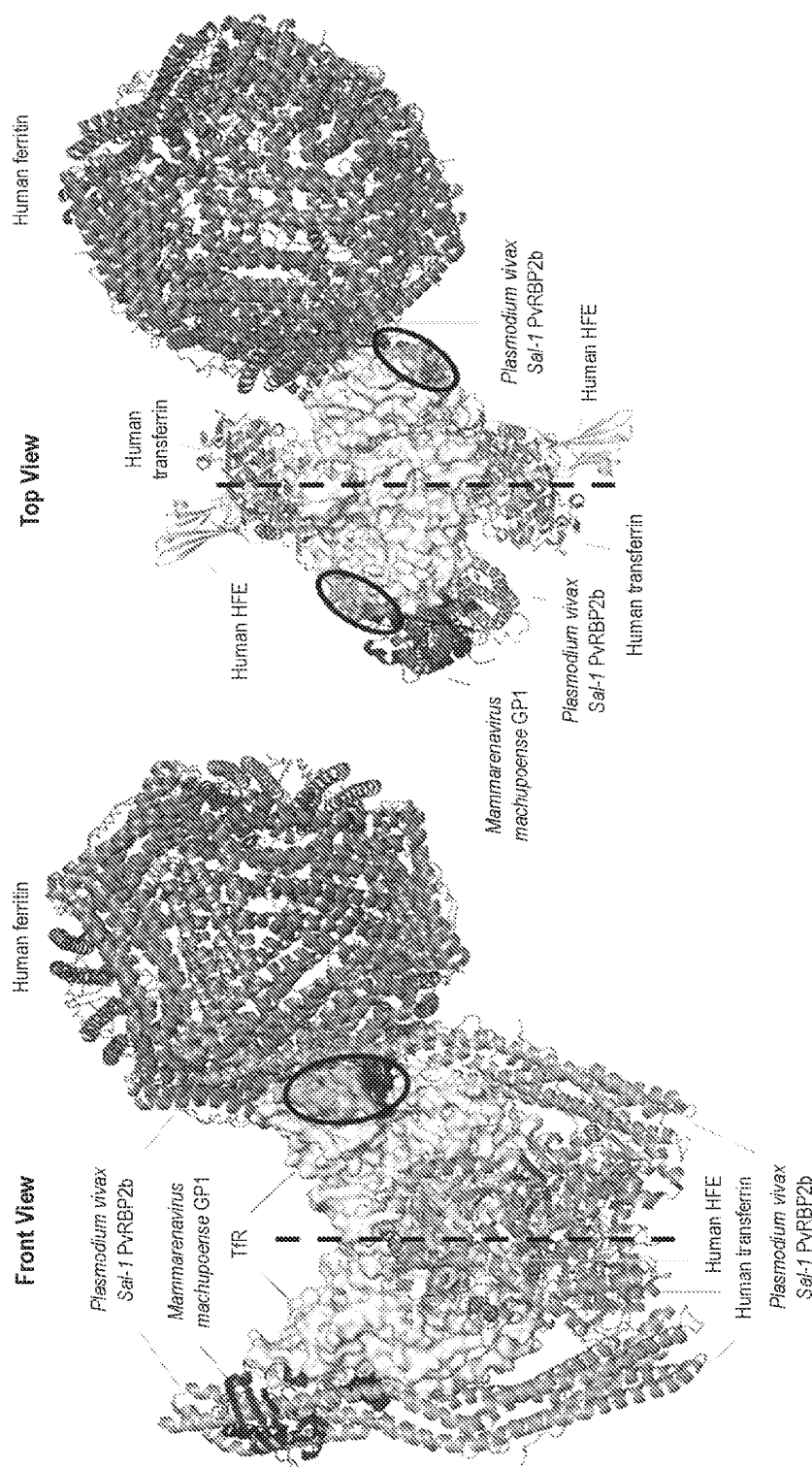


FIG. 16

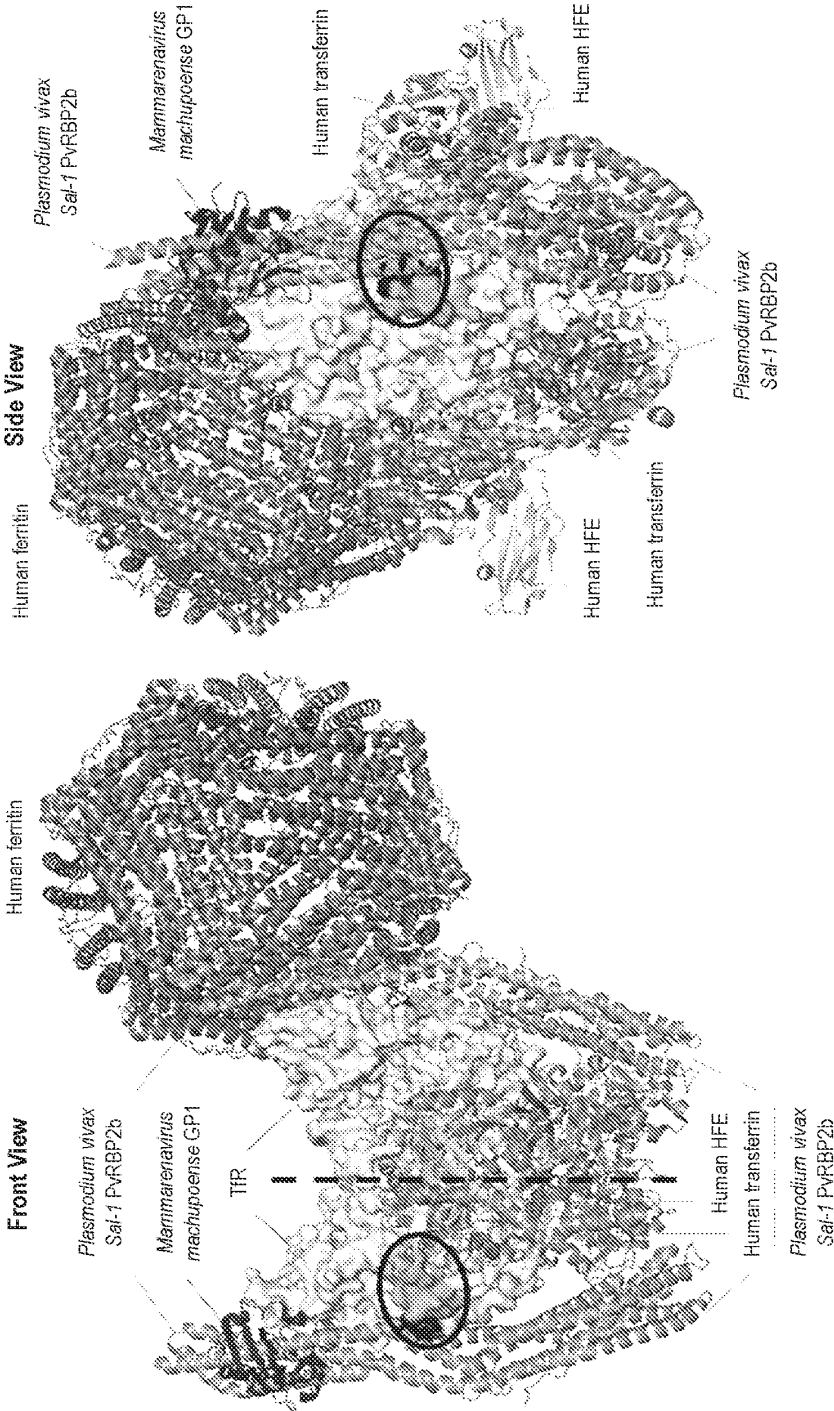


FIG. 17

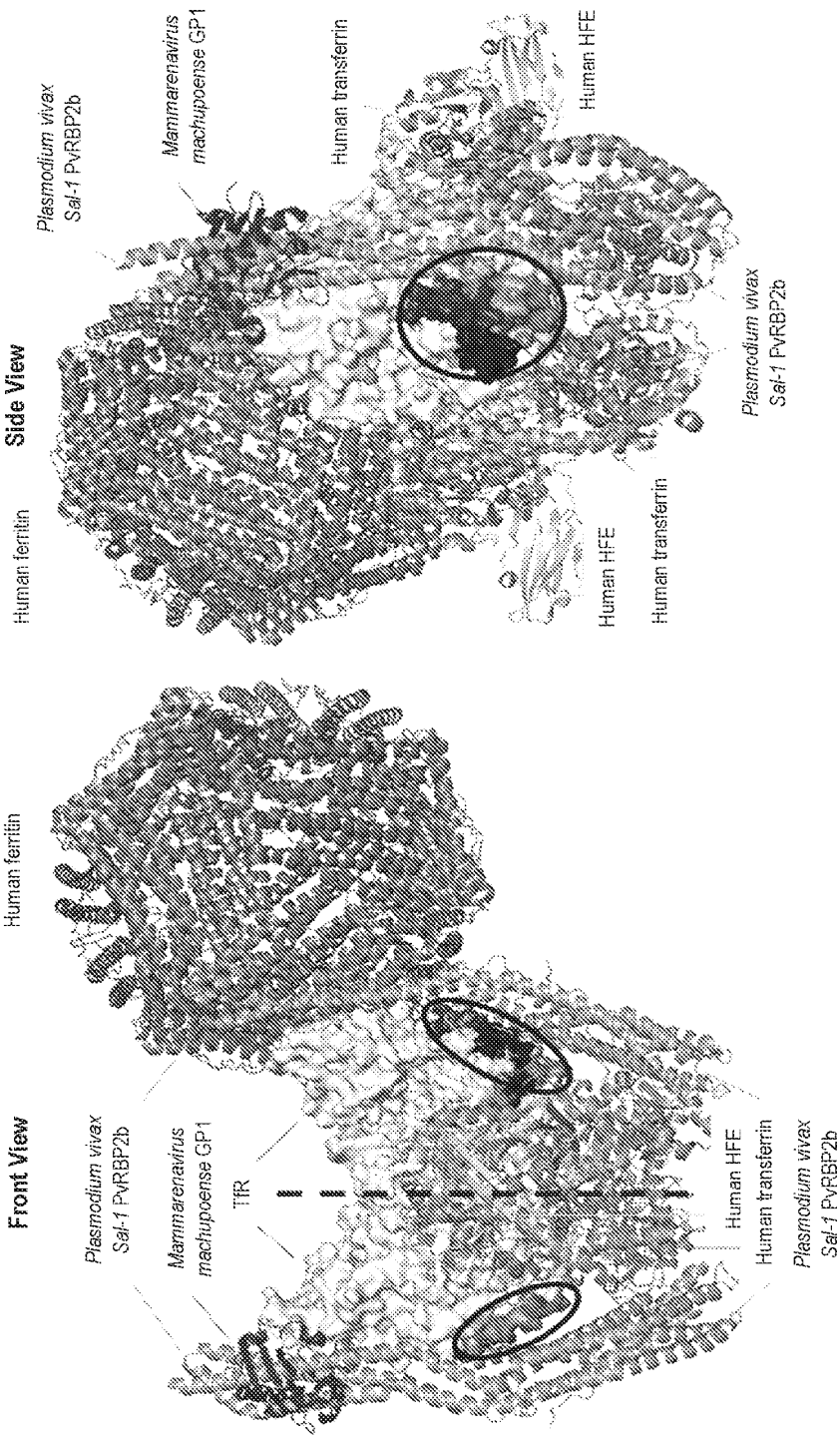


FIG. 18

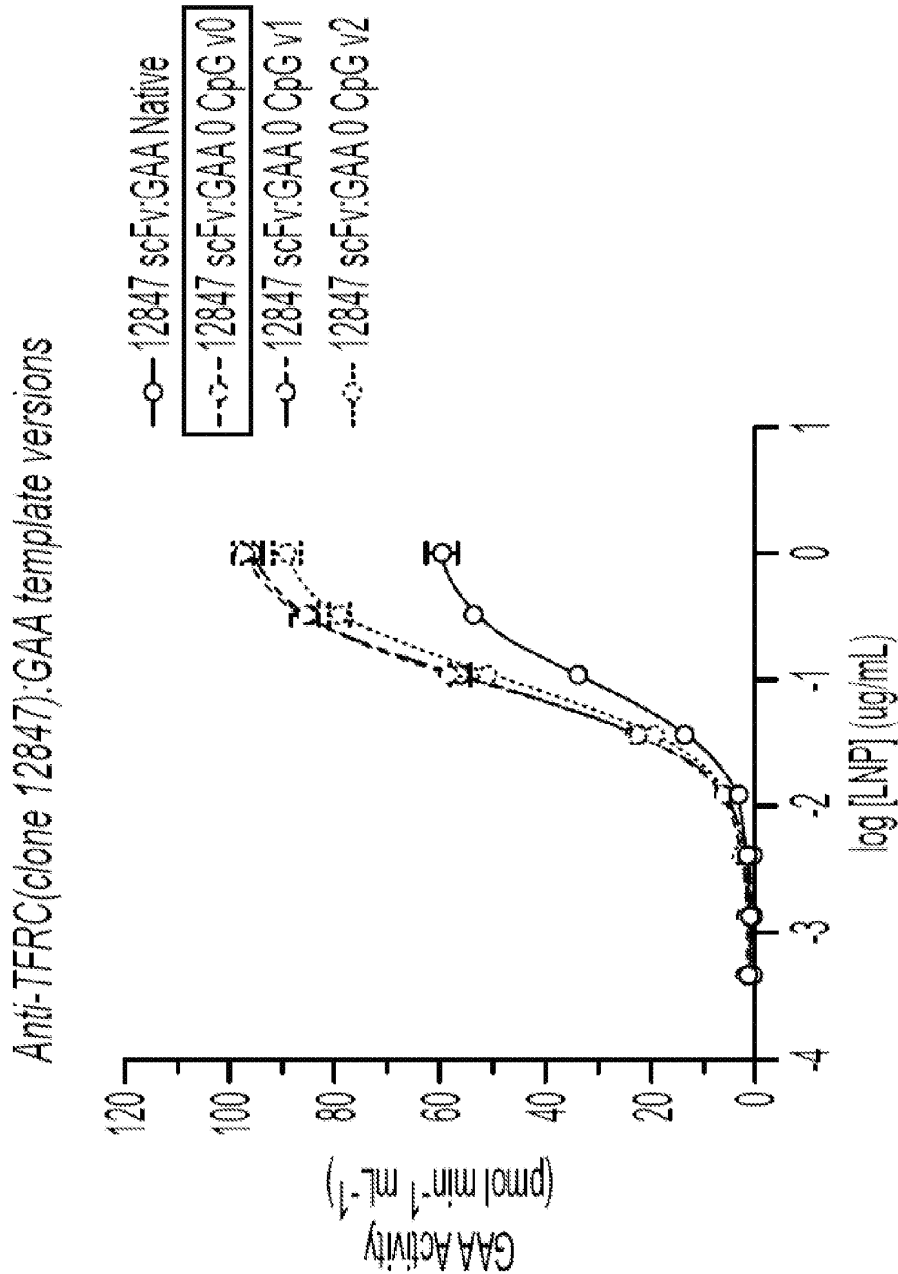


FIG. 19A

Anti-TFRC(12843):GAA template versions

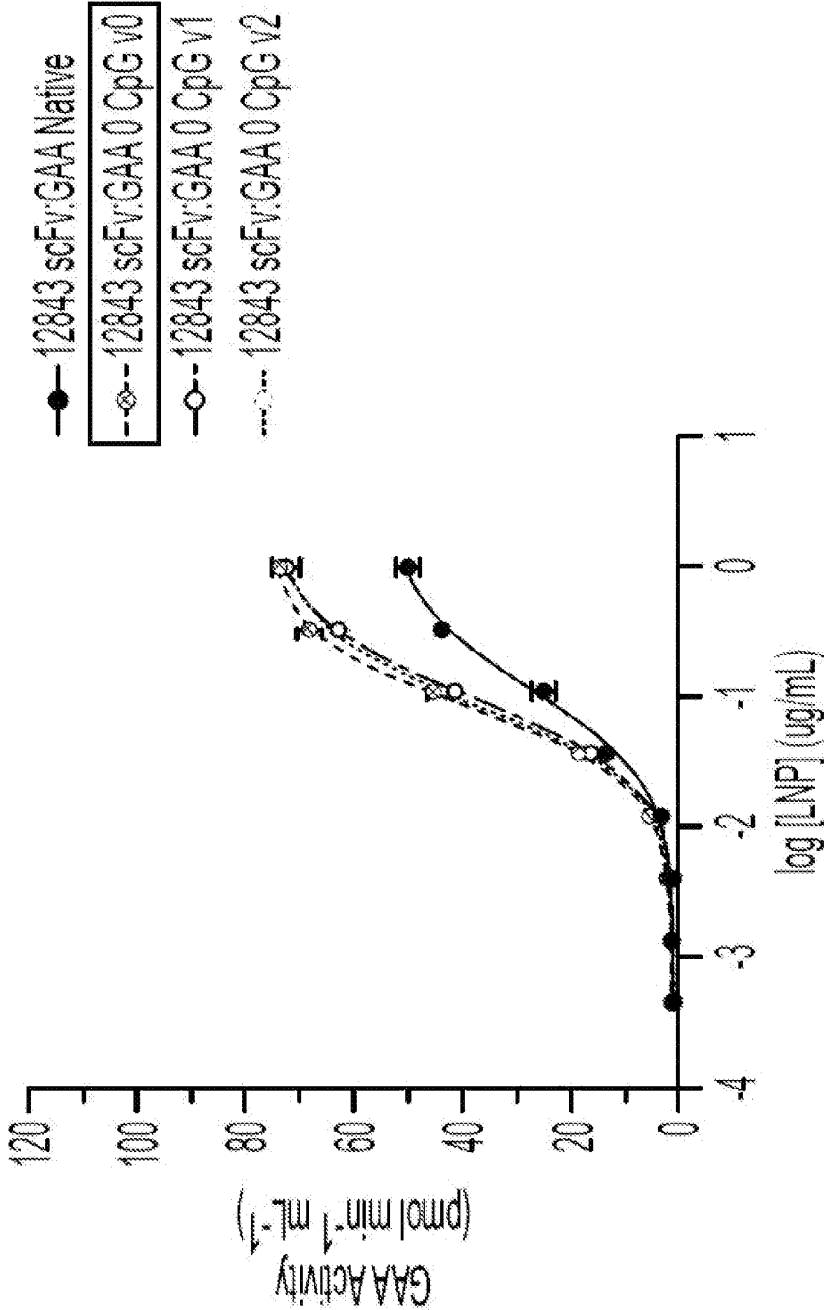


FIG. 19B

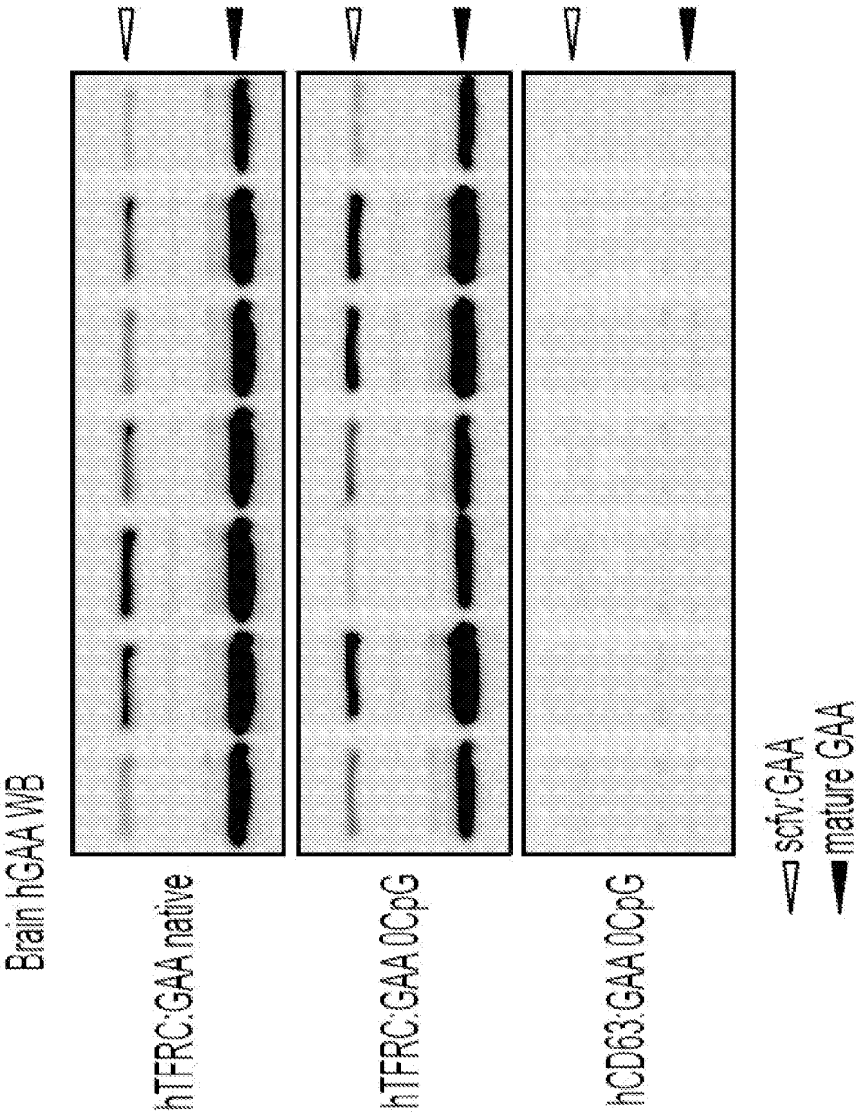


FIG. 20A

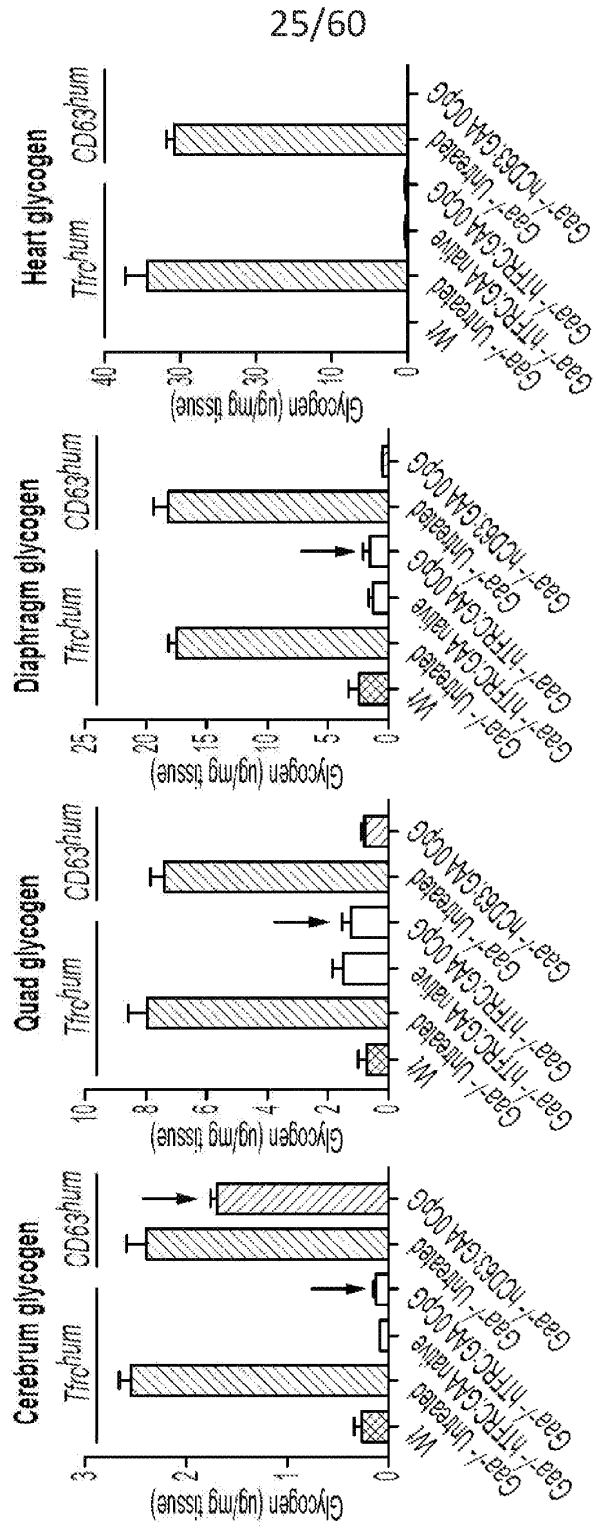


FIG. 20B

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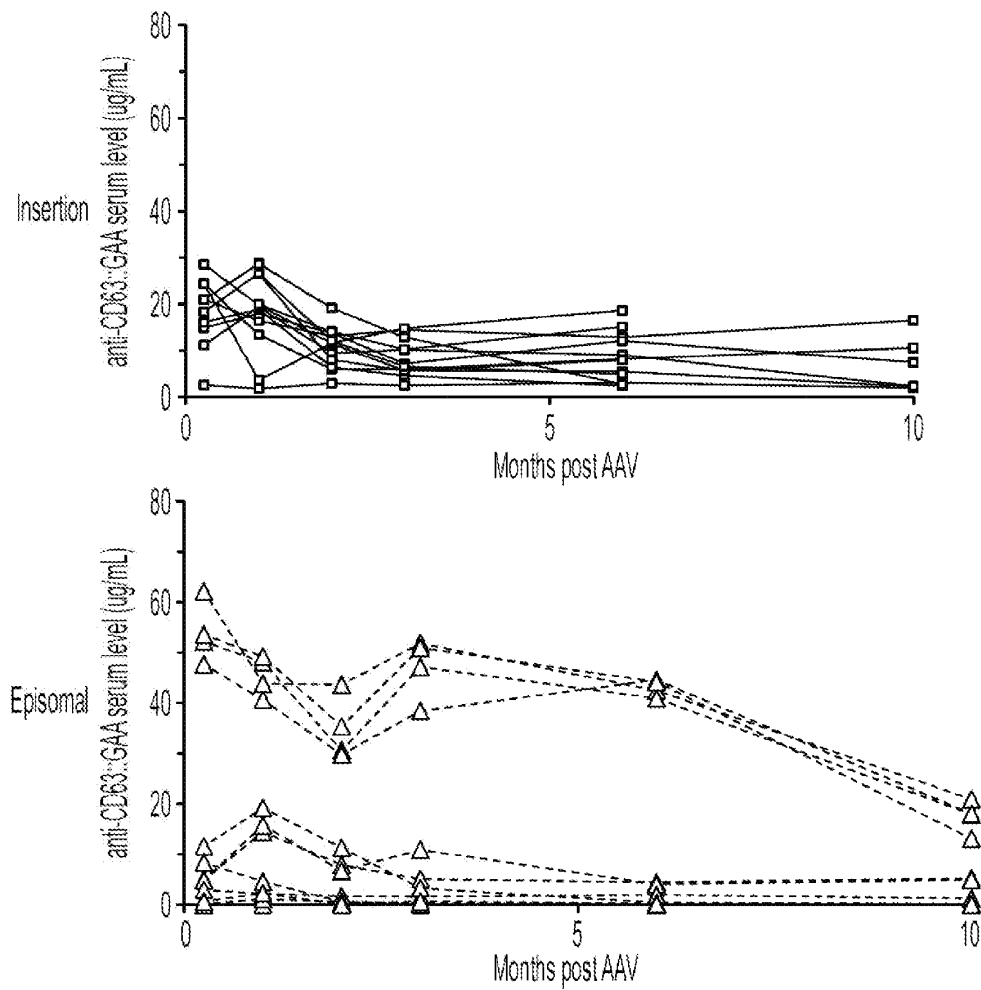


FIG. 21

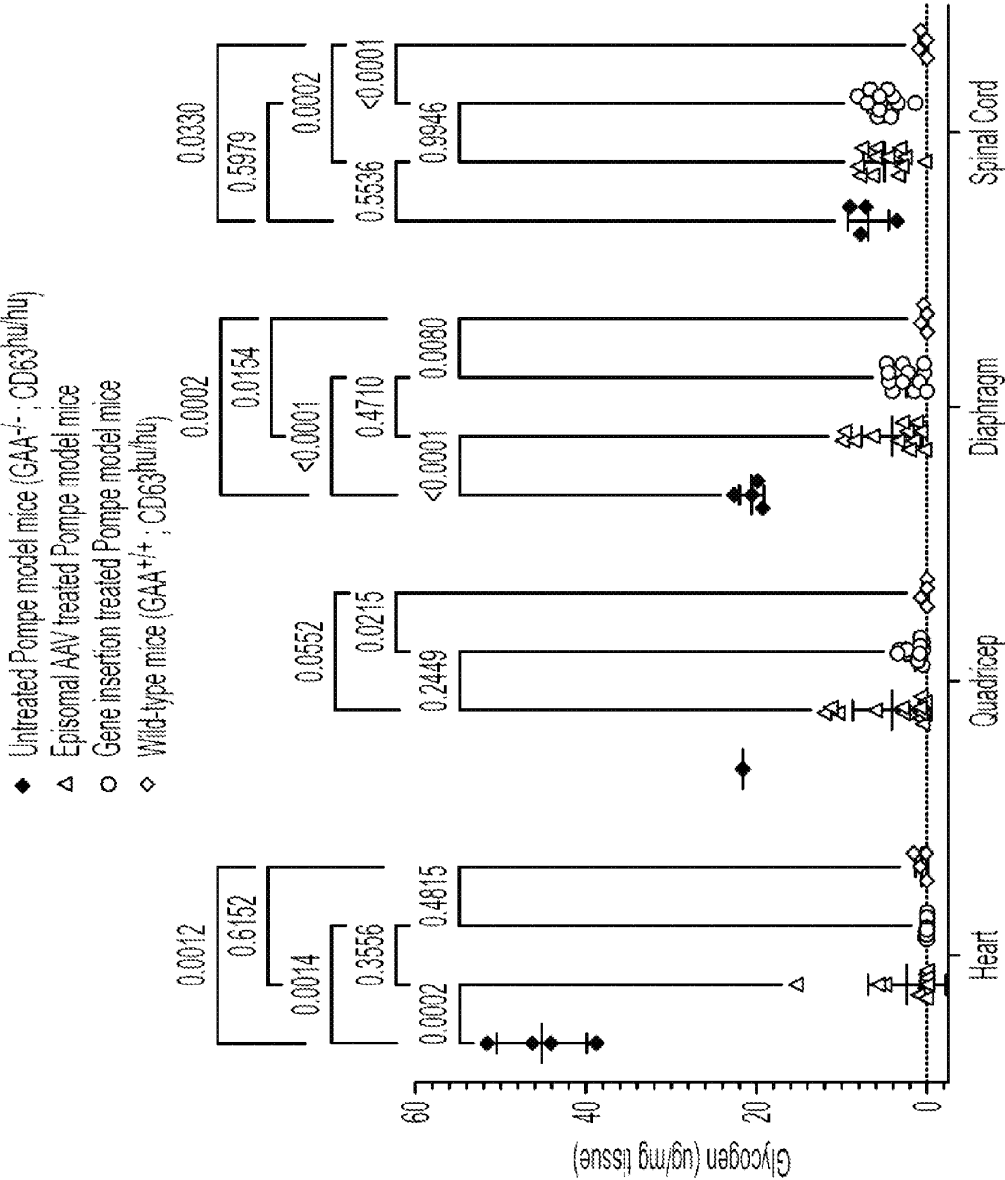


FIG. 22

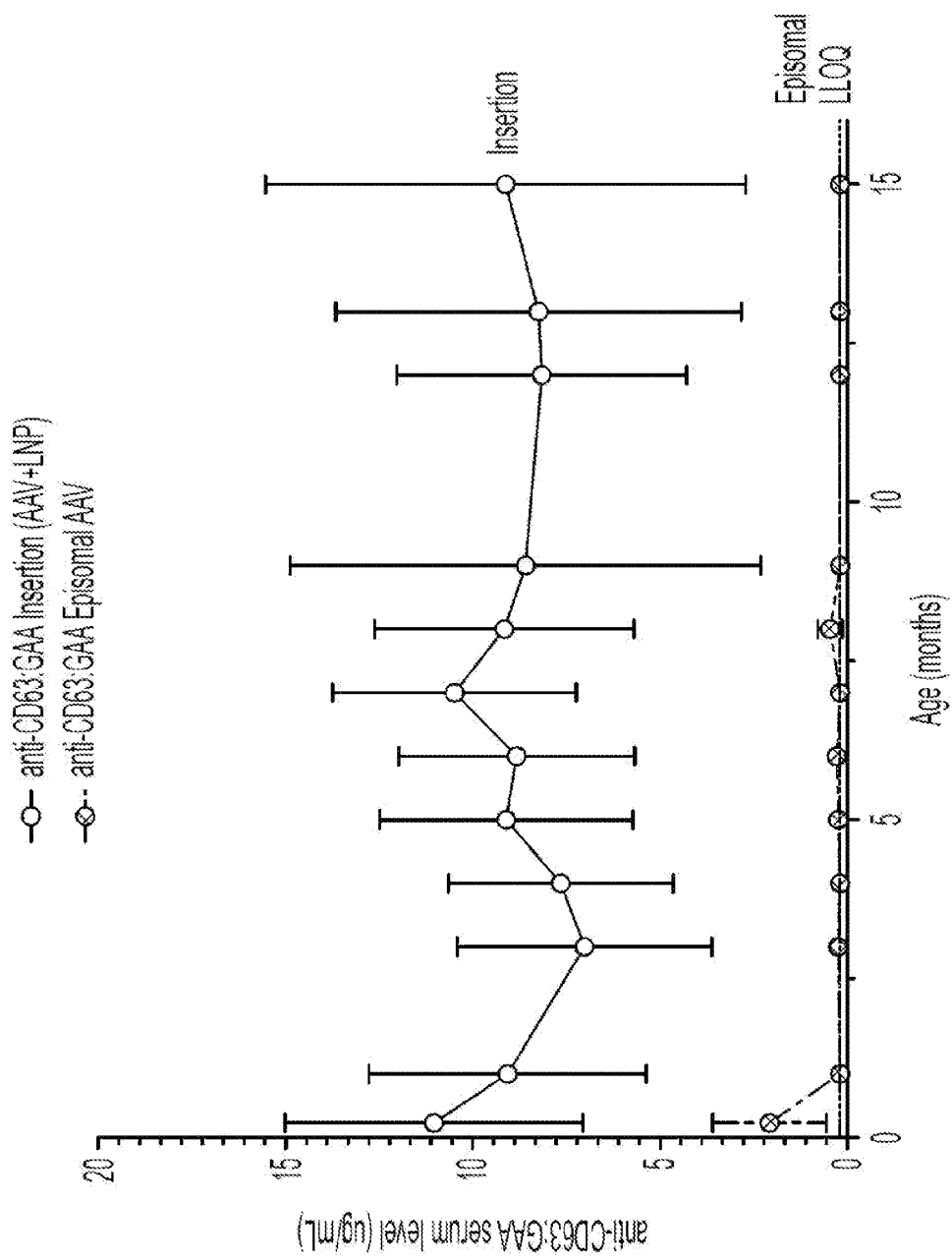


FIG. 23A

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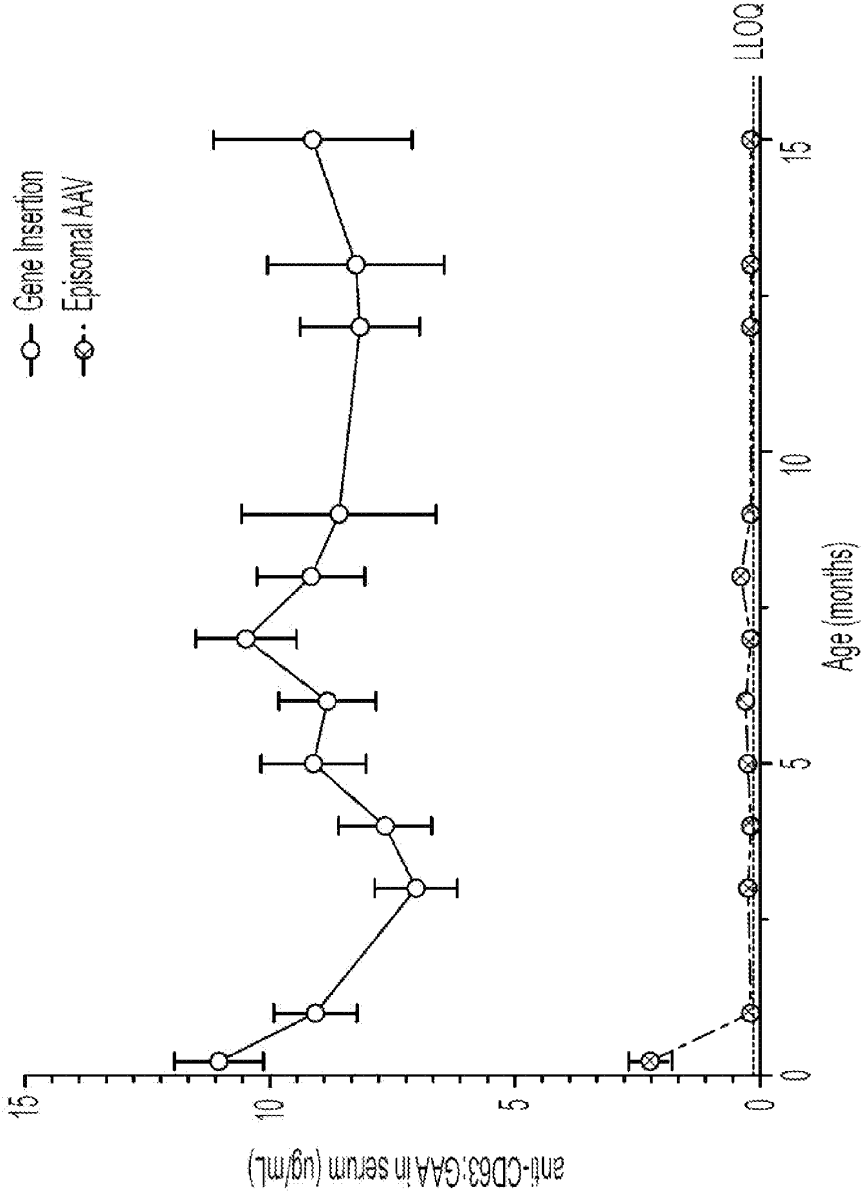


FIG. 23B

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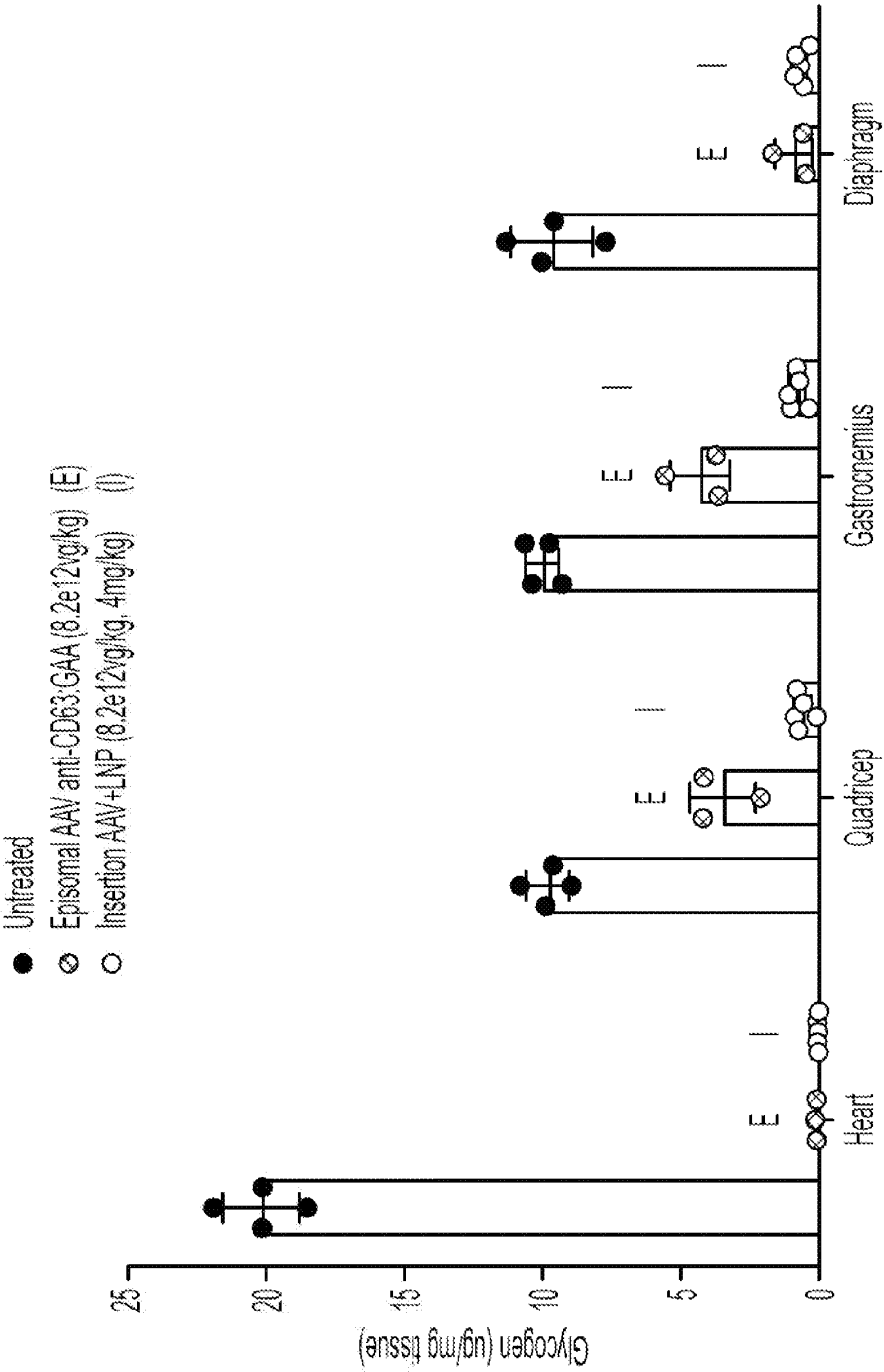


FIG. 24A

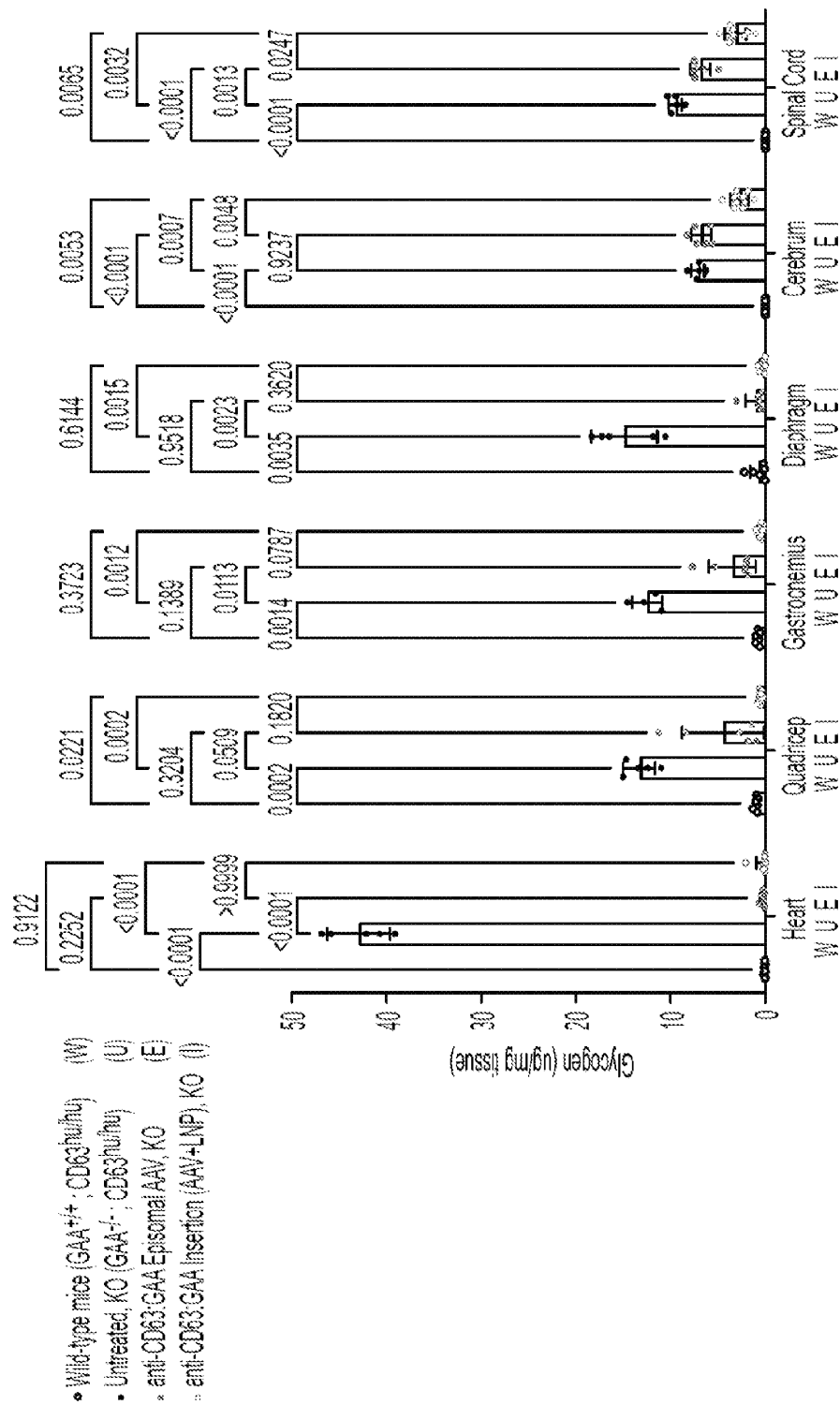


FIG. 24B

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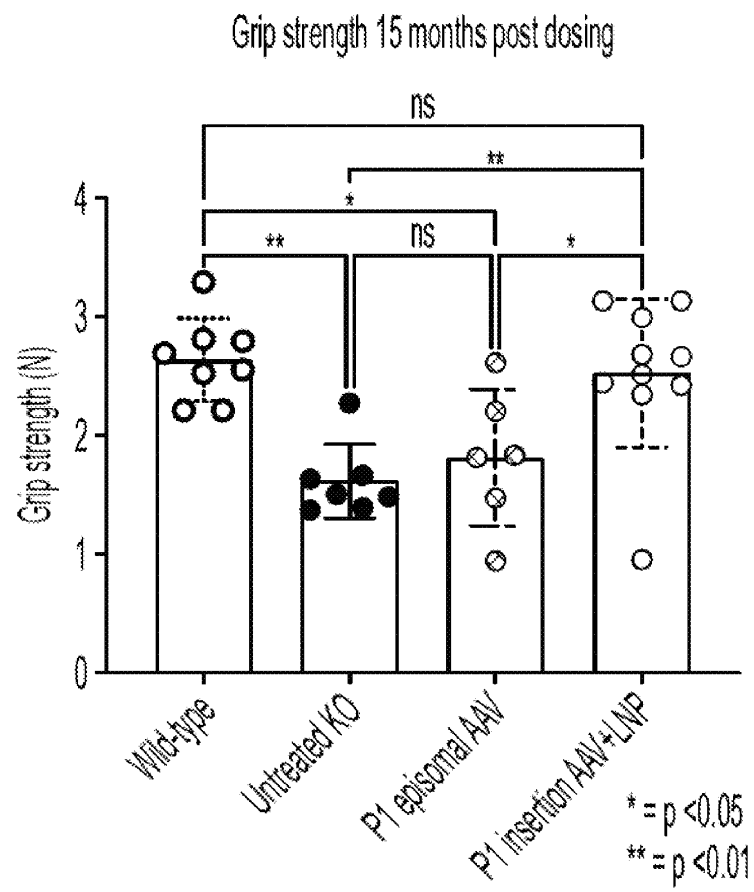


FIG. 25

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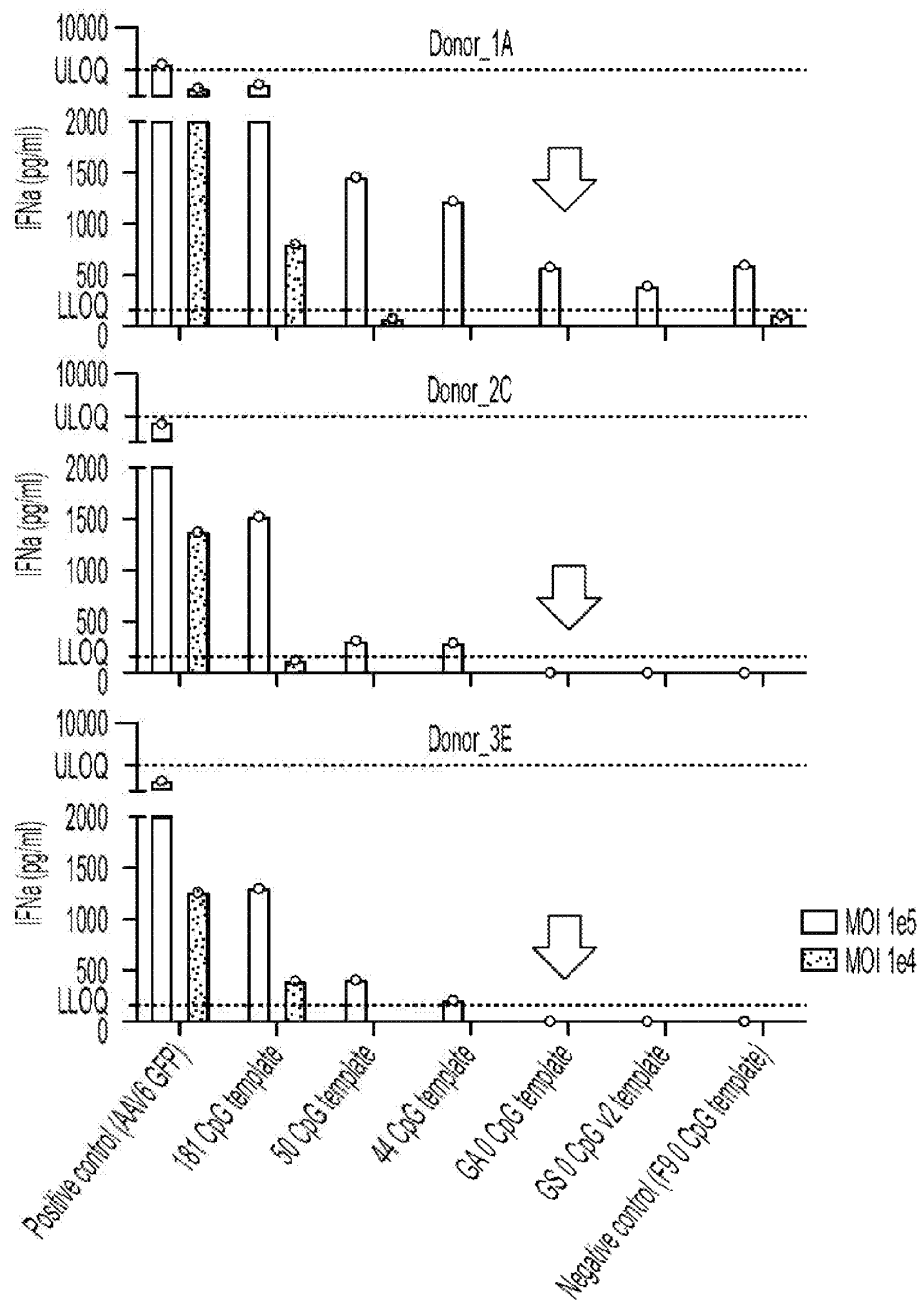


FIG. 26

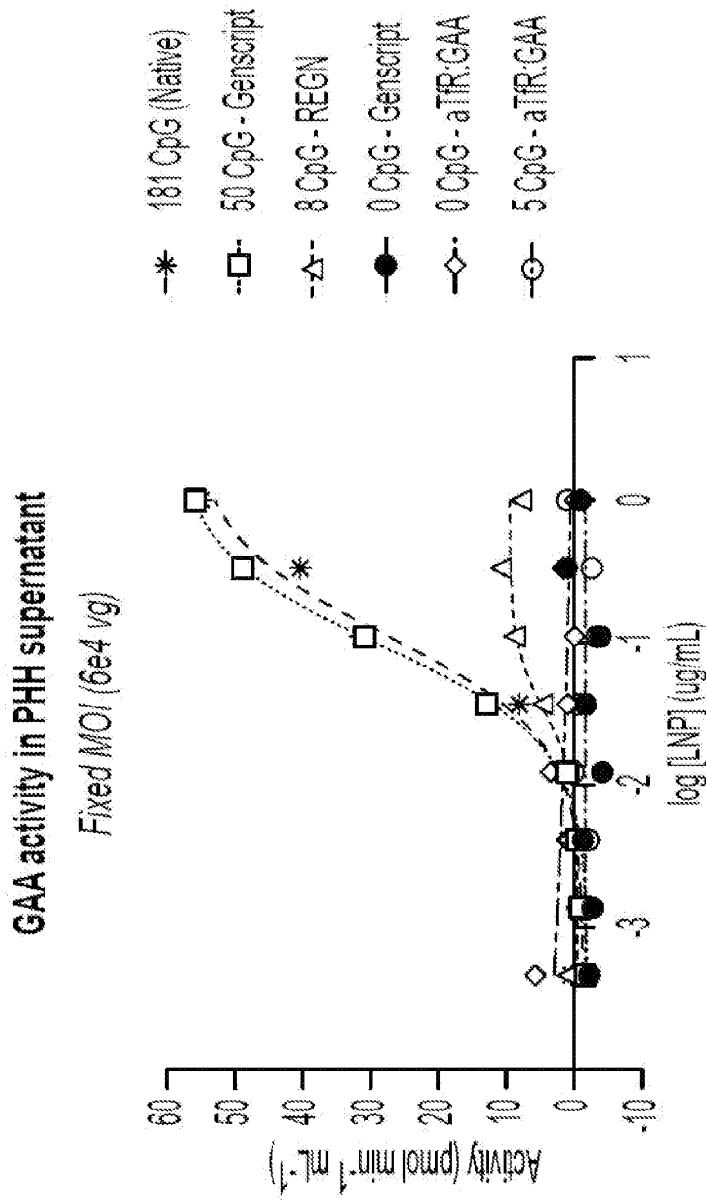


FIG. 27

GAA activity in supernatant from primary human hepatocytes
treated with anti-CD63:GAA insertion

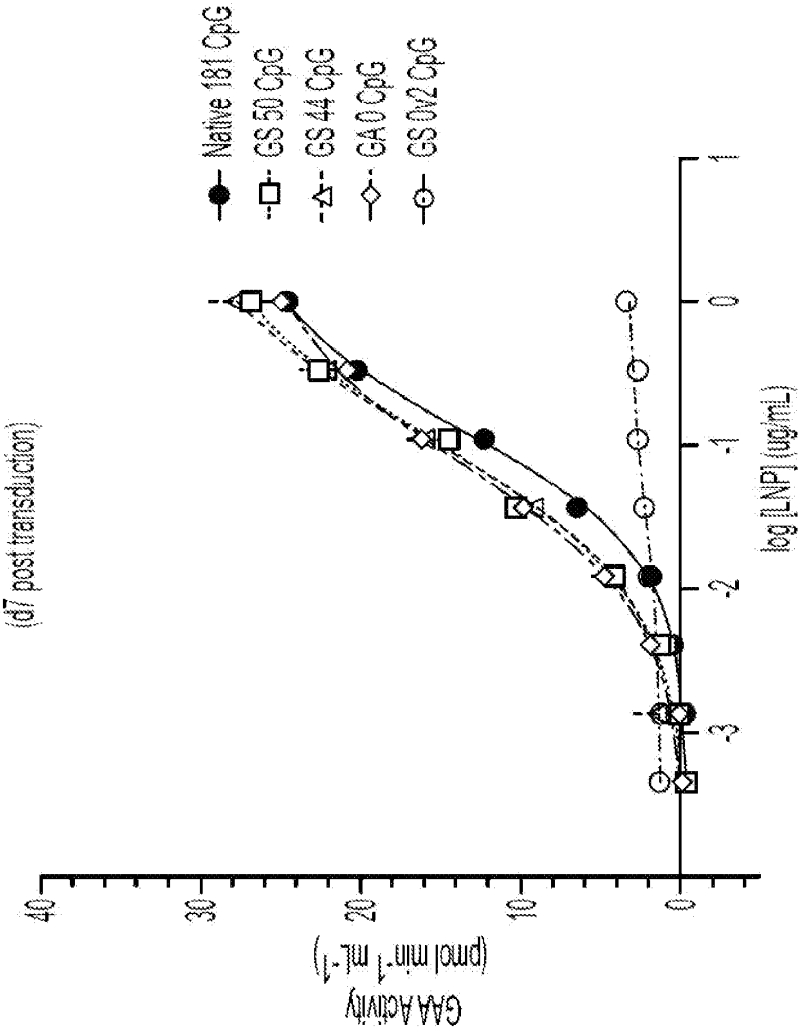


FIG. 28

Serum hCD63 scFv:GAA levels in adult male GAA KO mice treated with anti-hCD63:GAA insertion

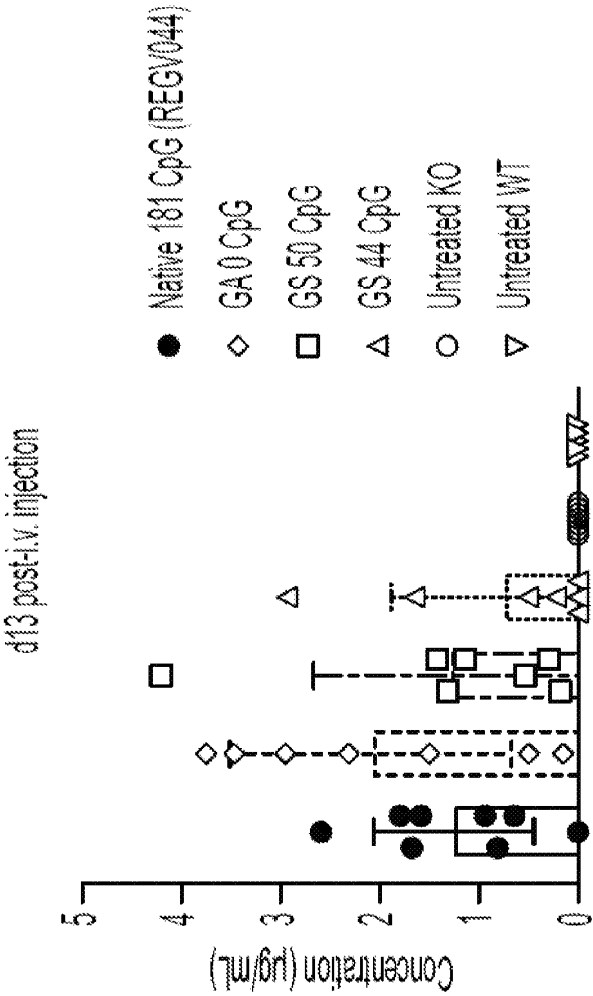


FIG. 29A

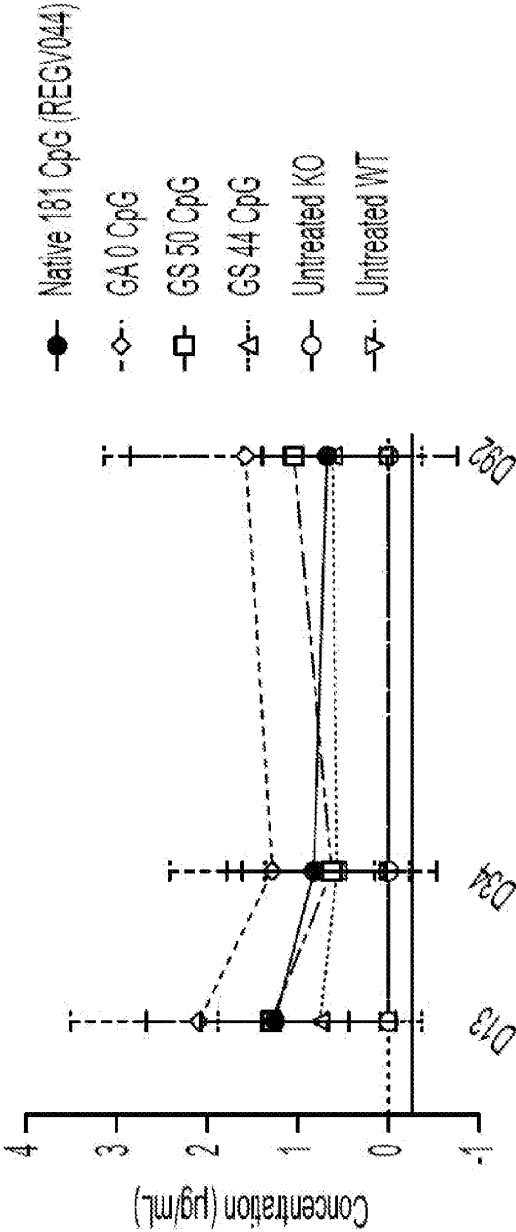


FIG. 29B

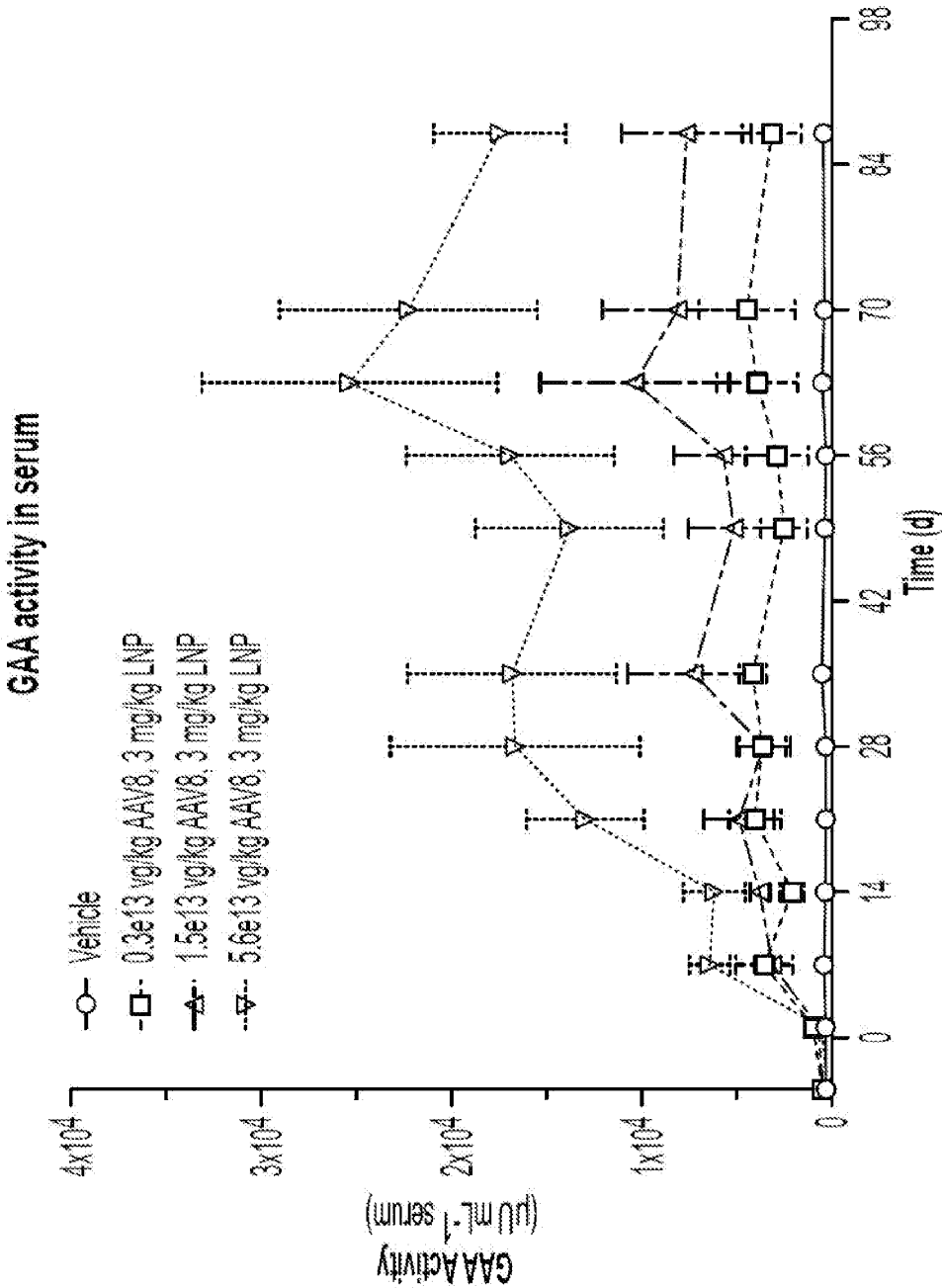


FIG. 30A

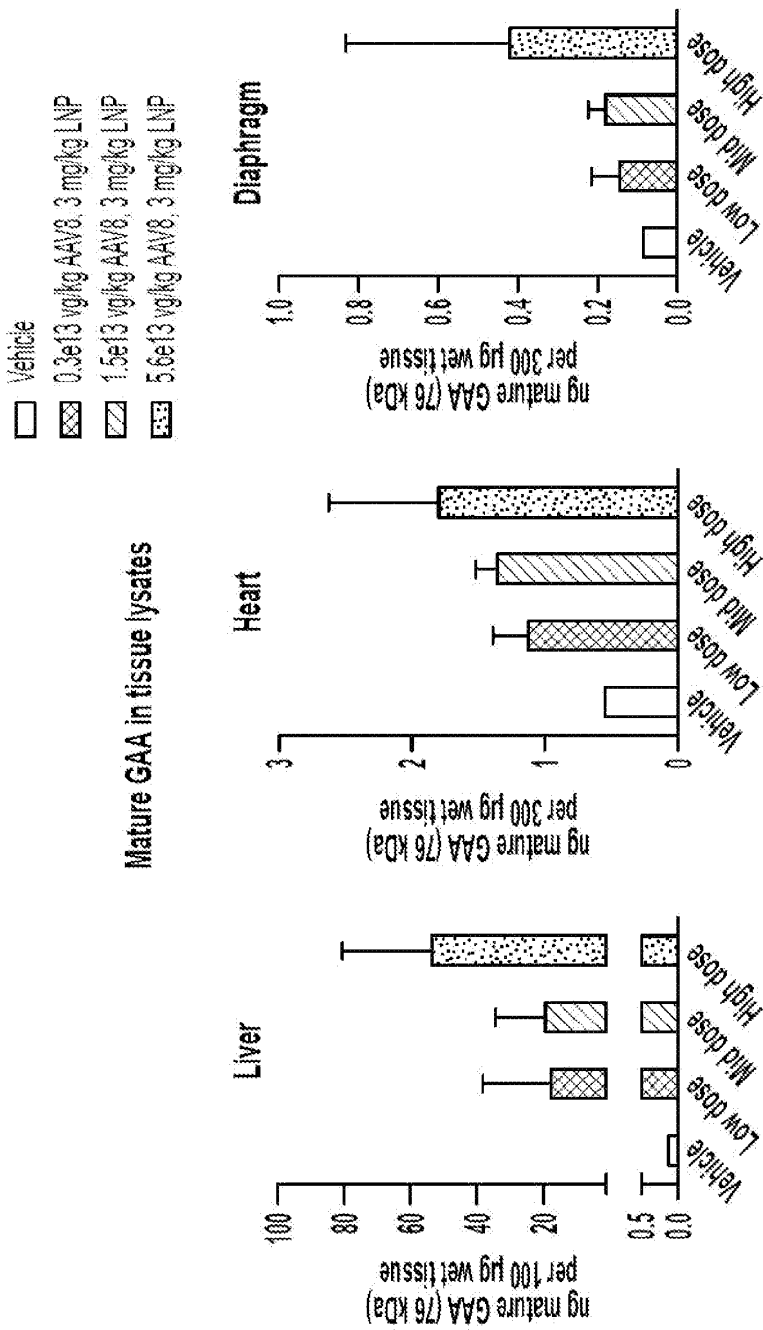


FIG. 30B

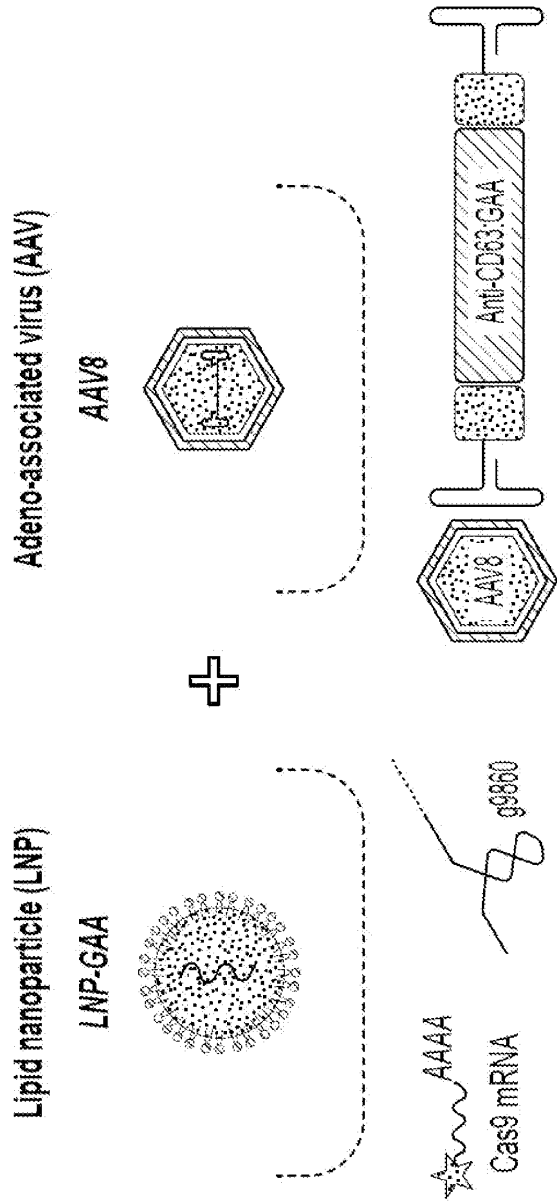


FIG. 31

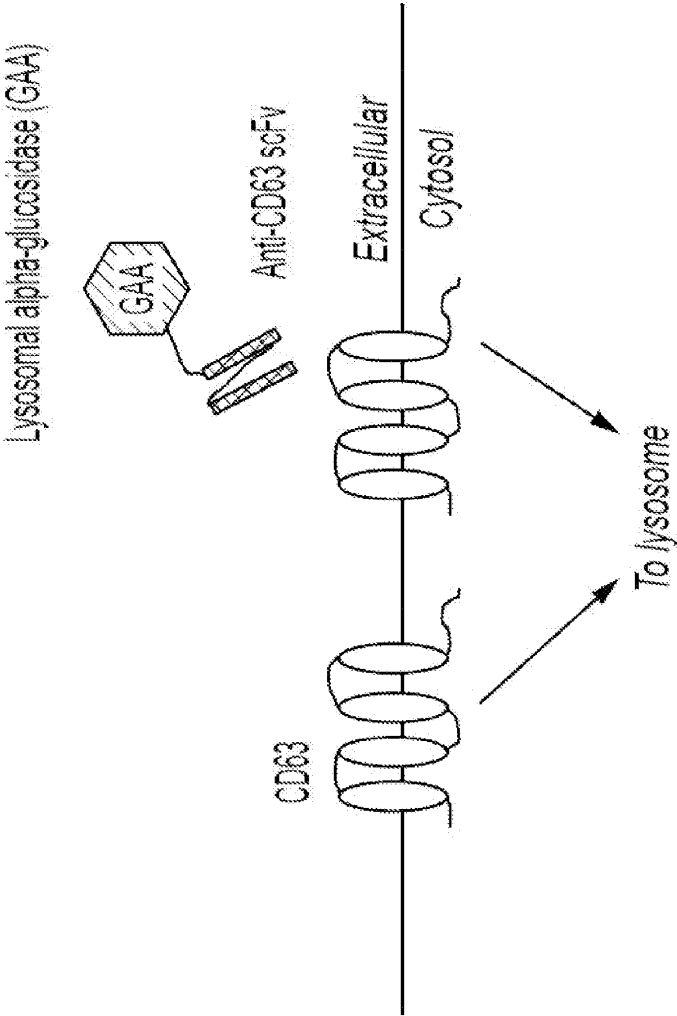


FIG. 32

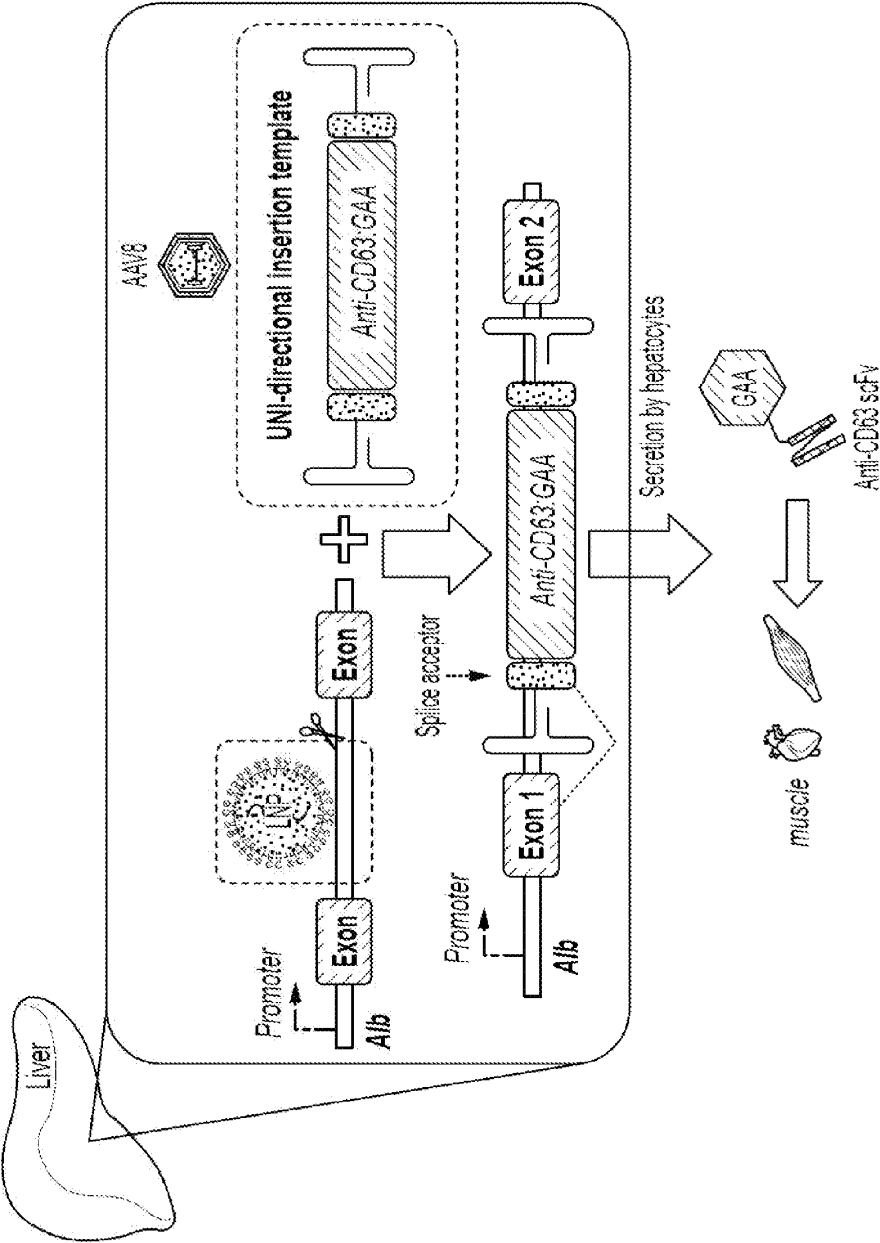


FIG. 33

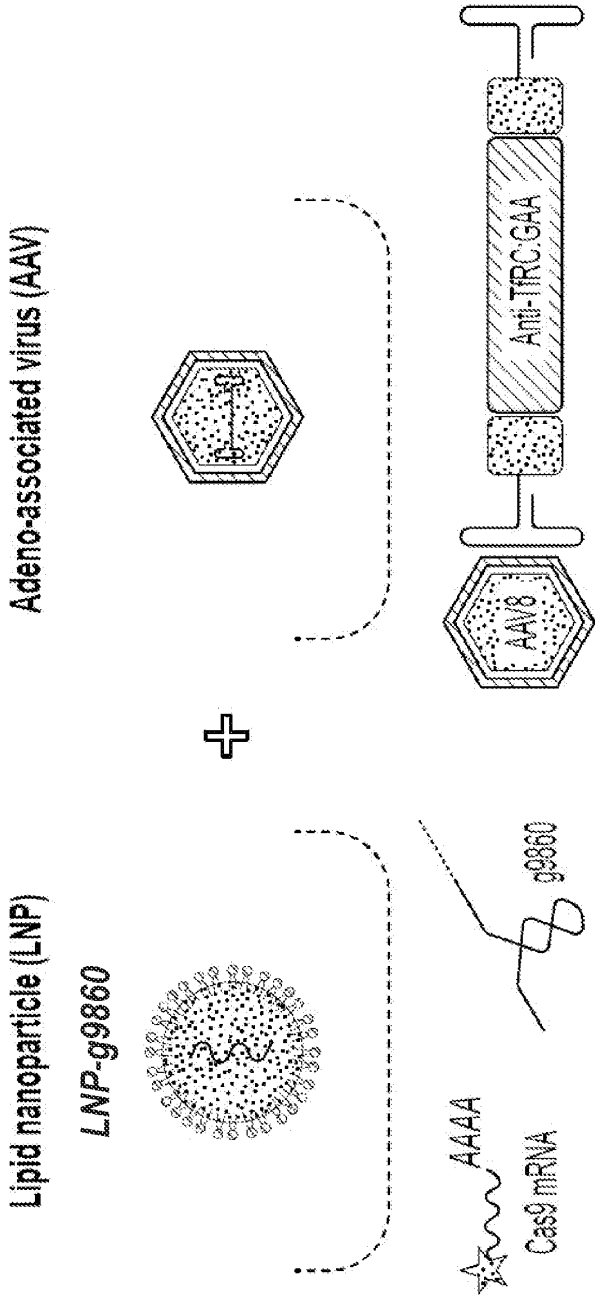


FIG. 34

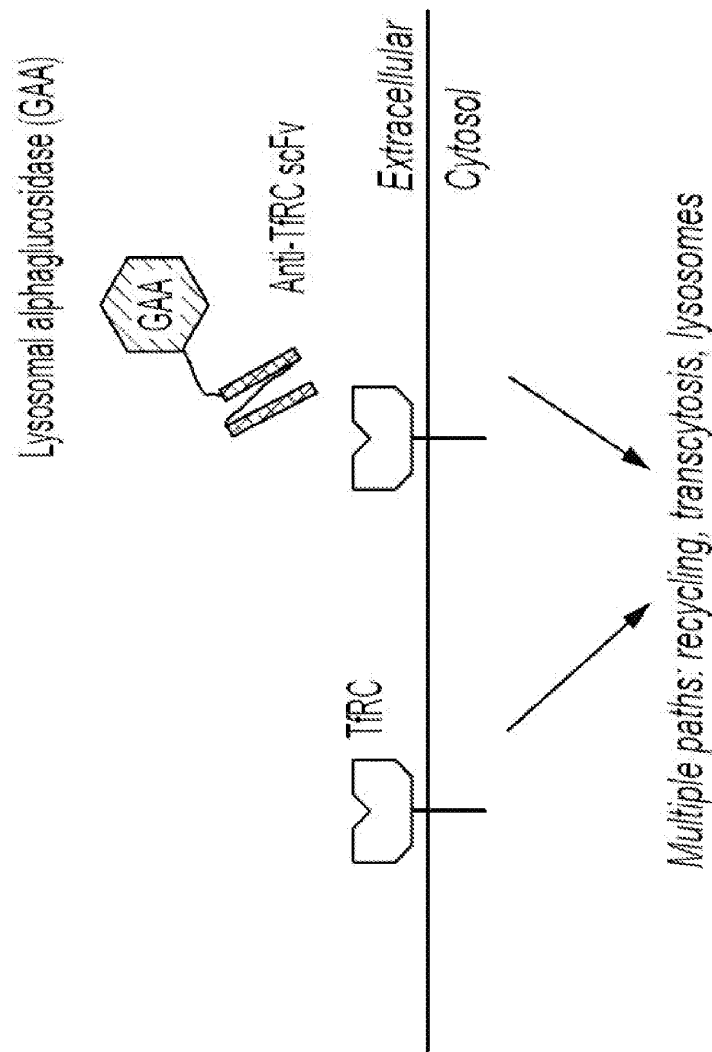


FIG. 35

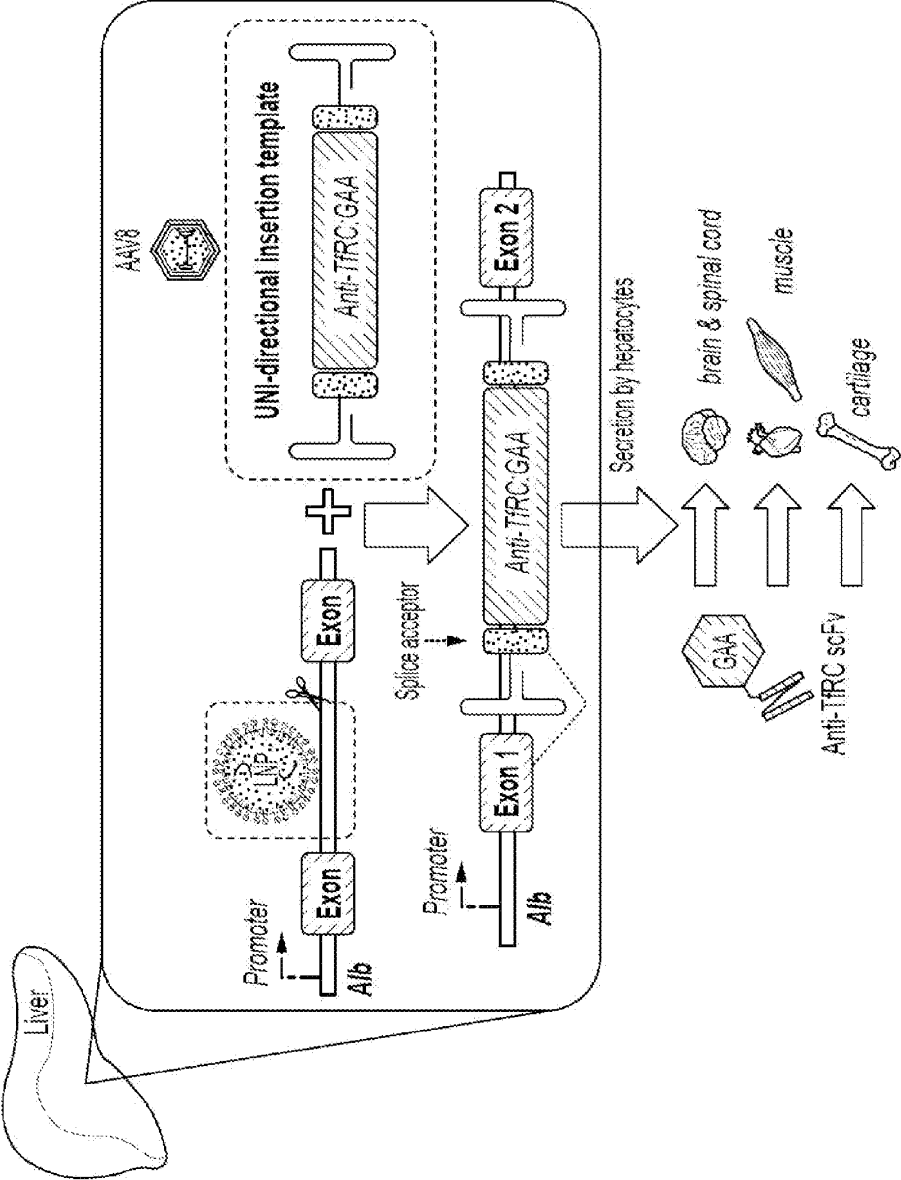


FIG. 36

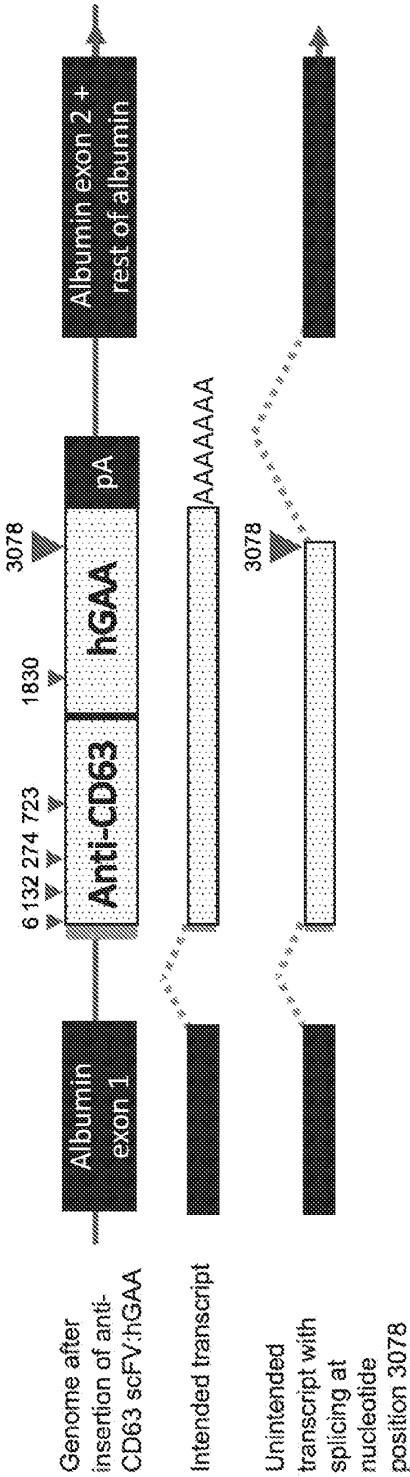
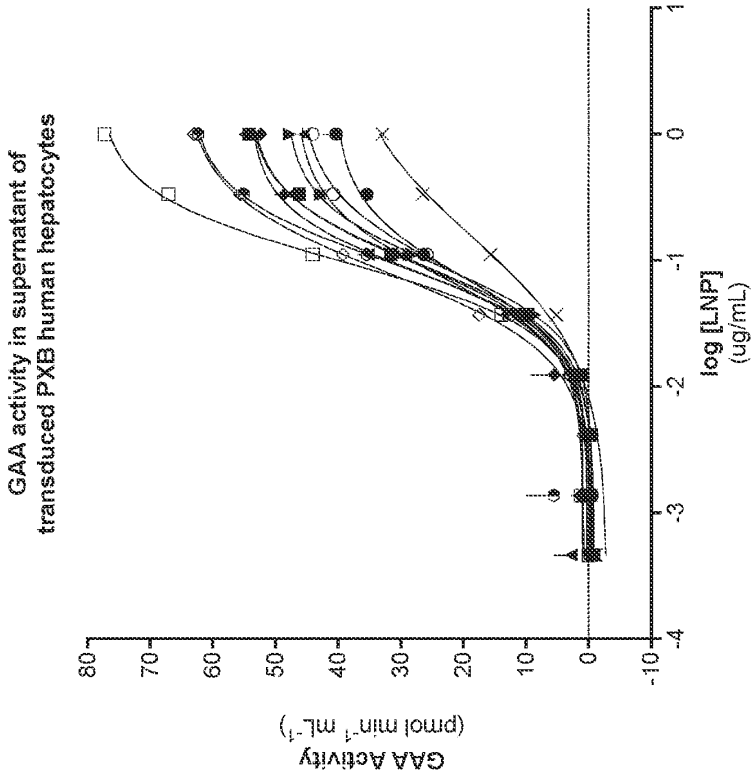


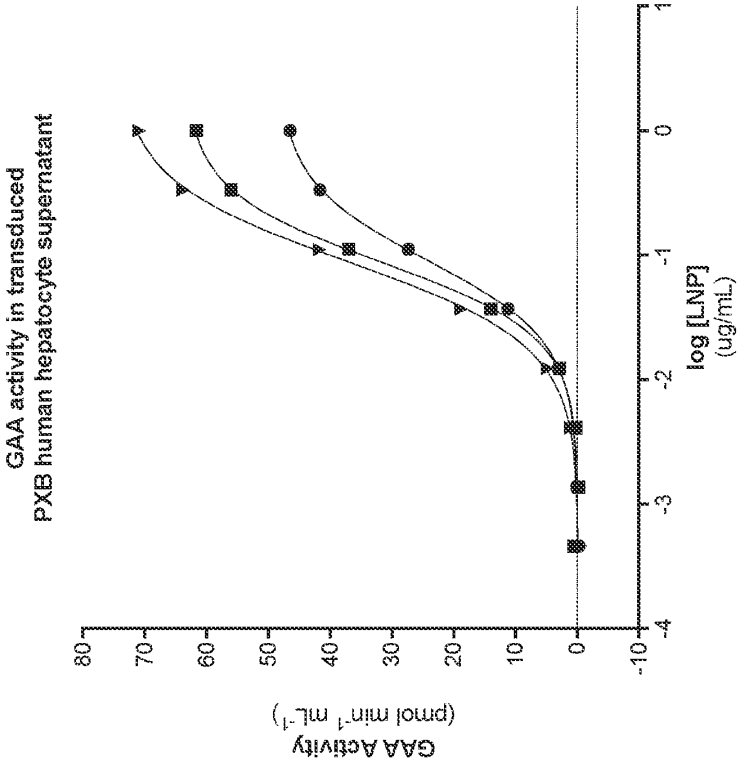
FIG. 37



Symbol	#	Genome
▼	VVT1118	CD63GAA-3078fix-bGHpA
●	VVT1119	CD63GAA-bGHpA SPA swap
■	VVT1120	CD63GAA-bGHpA stuffer SPA
▲	VVT1121	CD63GAA-4Xfix-bGH sv40LuniPA
◆	VVT1122	CD63GAA-4Xfix-bGHpA
◊	VVT1123	CD63GAA-3078fix-bGH sv40LuniPA (1CpG)
×	VVT1124	CD63GAA-bGHpA swap
○	VVT1127	CD63GAA-SV40E-MAZ
□	VVT1128	CD63GAA-6Xfix-bGH sv40LuniPA
◇	VVT1138	CD63GAA-bGHpA-MAZ
★	VVT1139	CD63GAA-6Xfix-bGHpA

FIG. 38

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Symbol	#	Genome
▼	VVT1125	TFR:GAA 12847GAA(GS0v2)-2Xfix-bGH.sv40LunIPA
●	VVT1126	TFR:GAA 12847GAA(GS0v2)-bGHpA swap
■	VVT1129	TFR:GAA 12847GAA(GS0v2)-2Xfix-bGHpA

FIG. 39

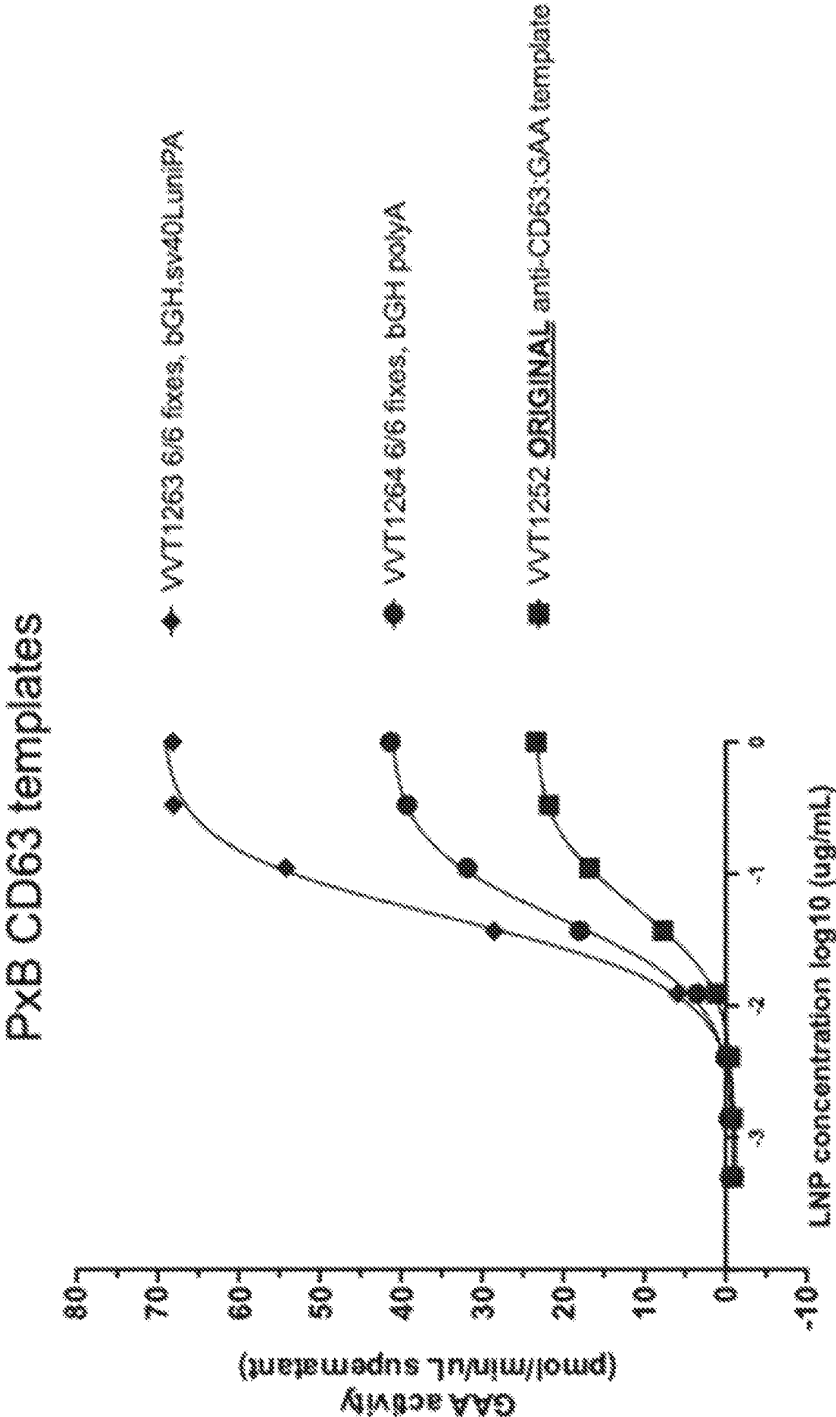


FIG. 40

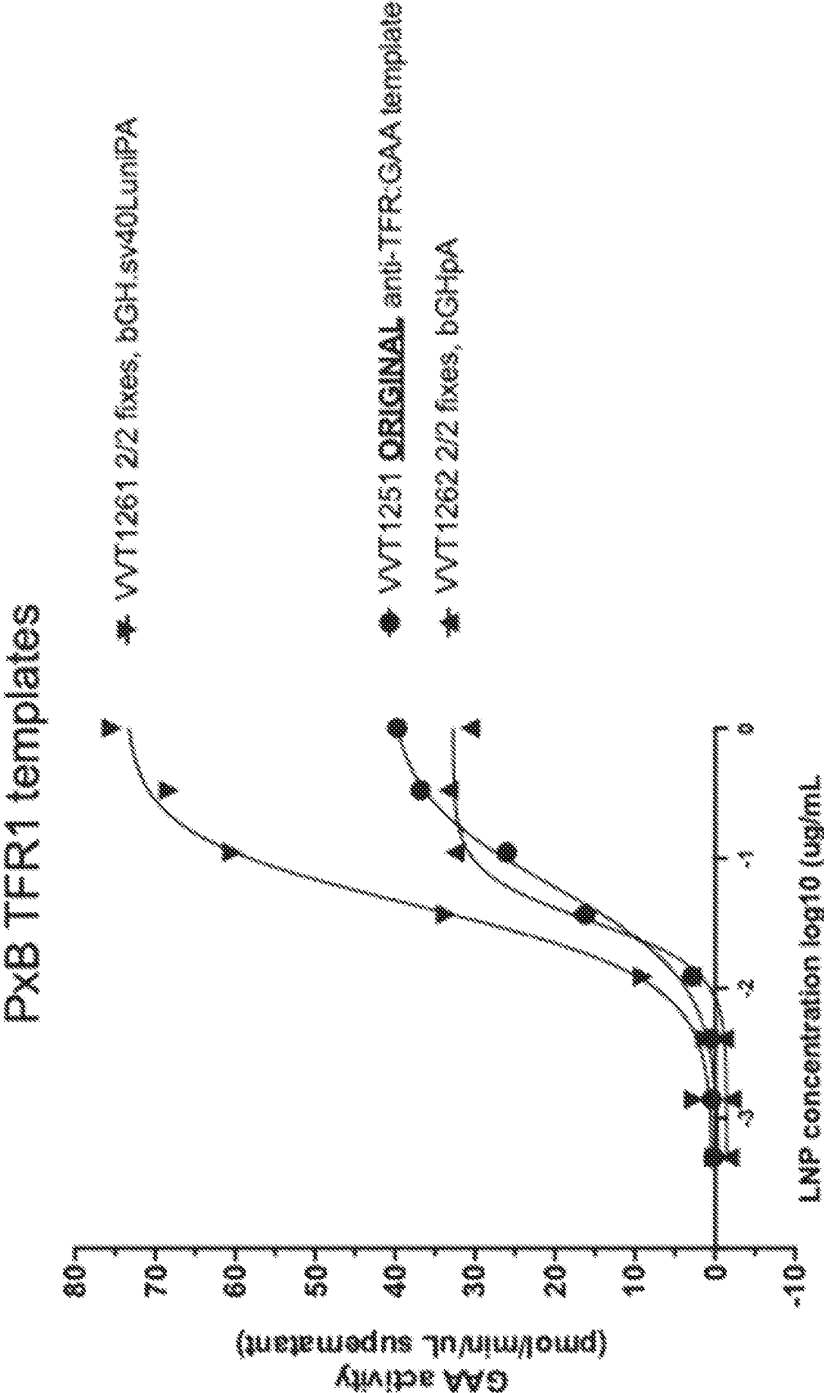


FIG. 41

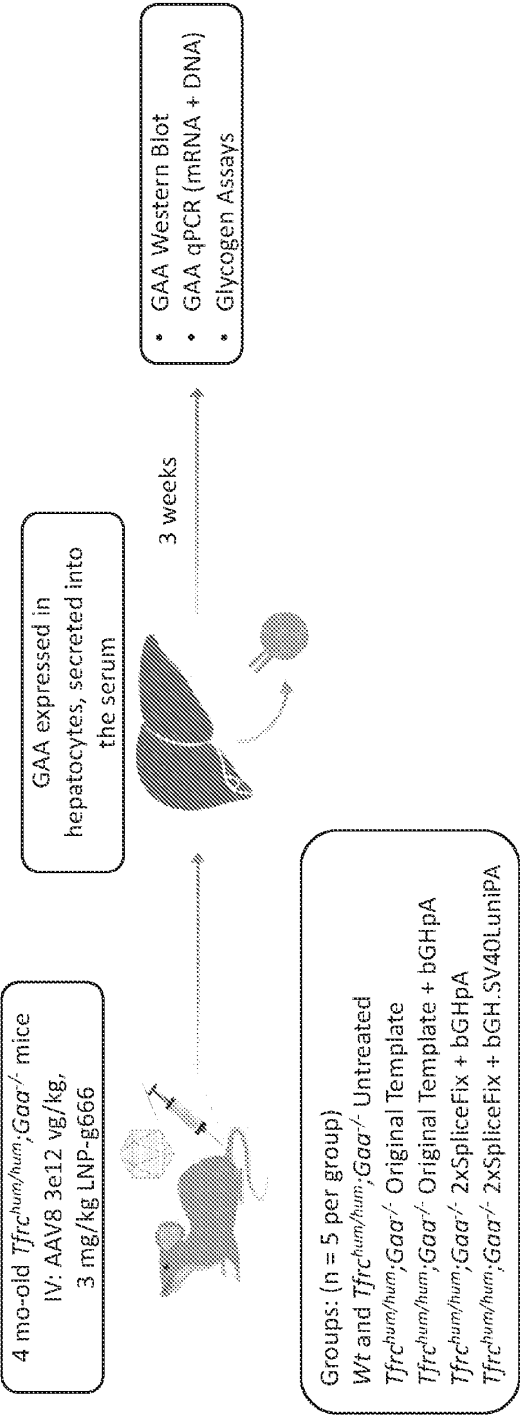


FIG. 42

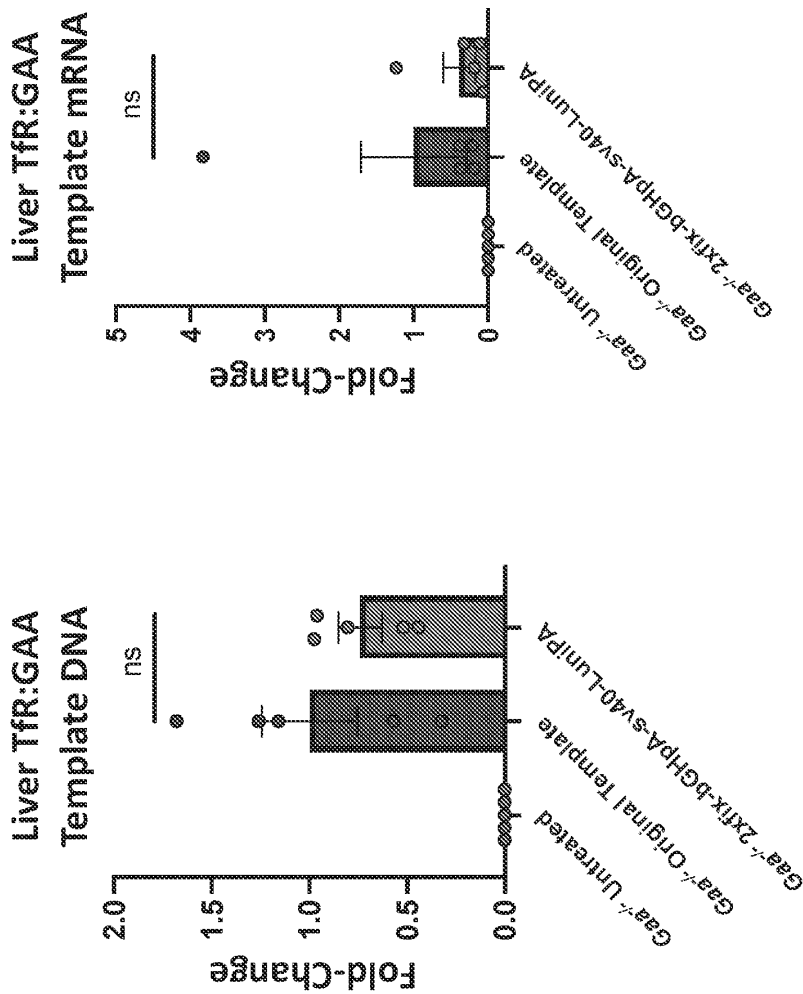


FIG. 43

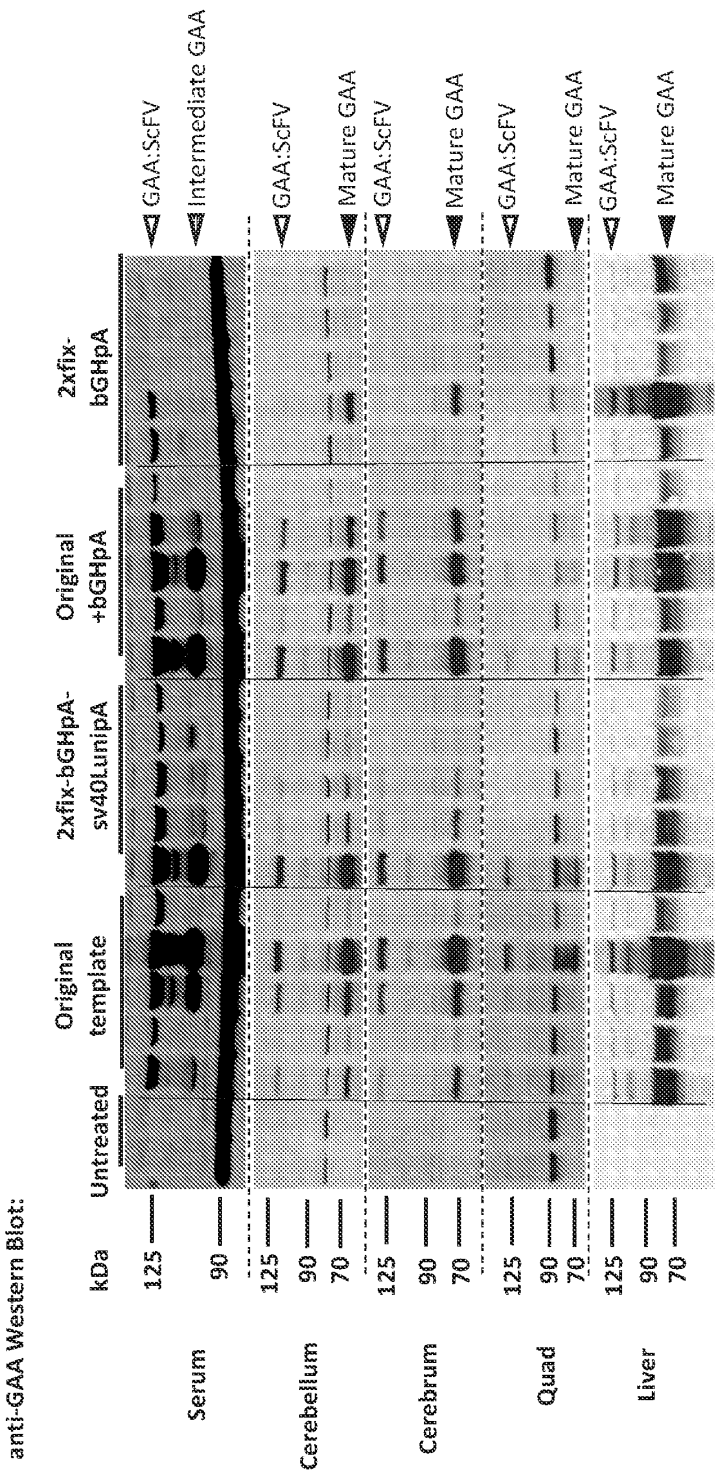


FIG. 44

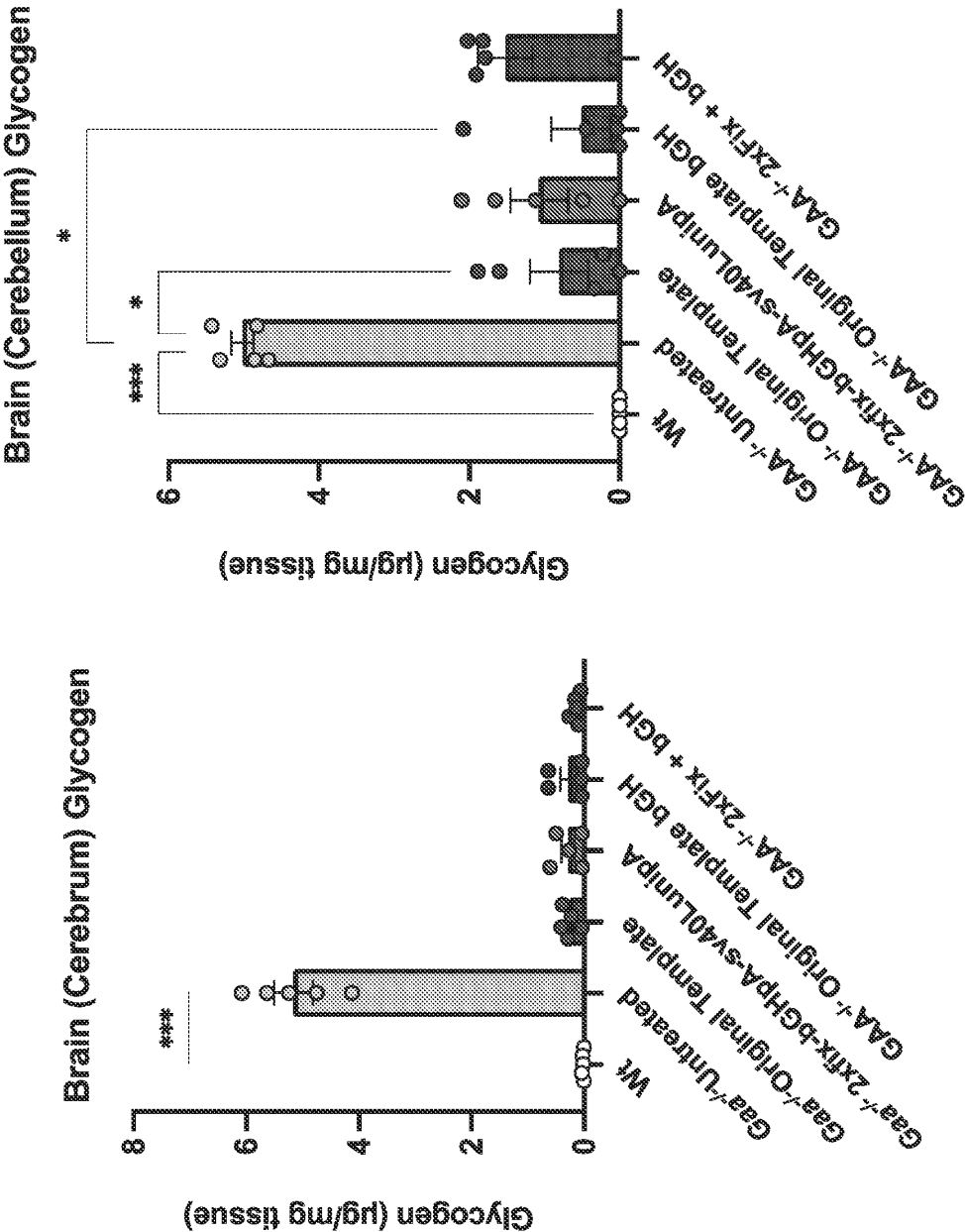


FIG. 45A

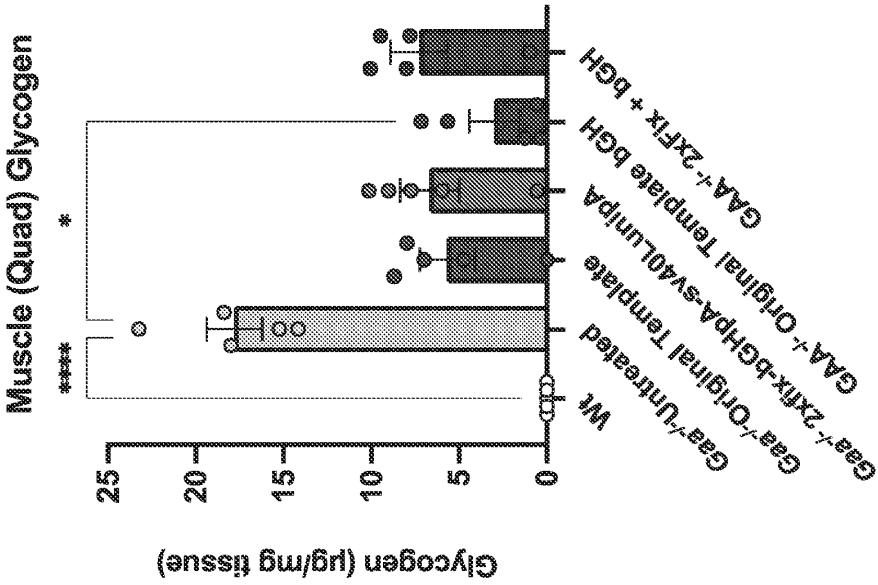


FIG. 45B

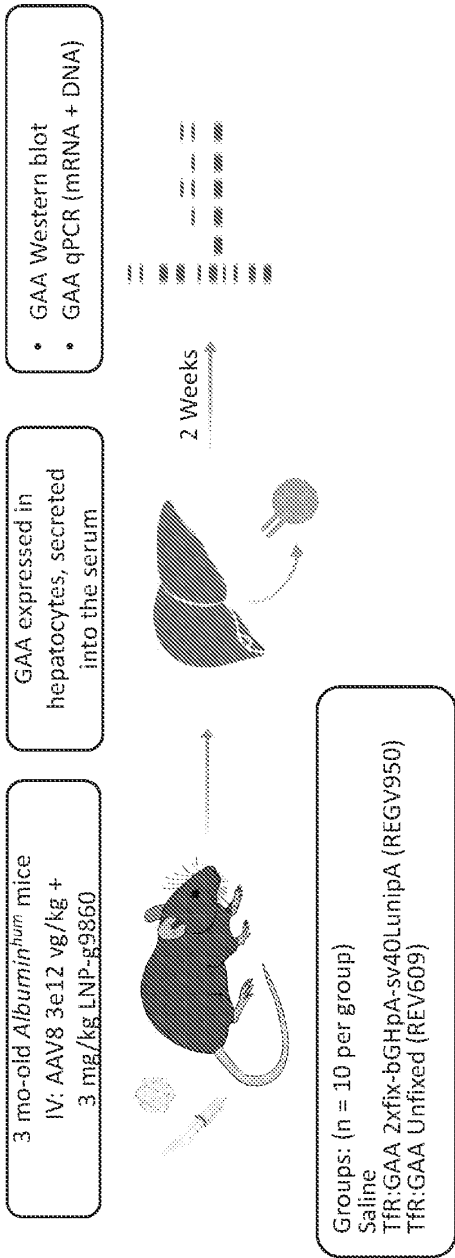


FIG. 46

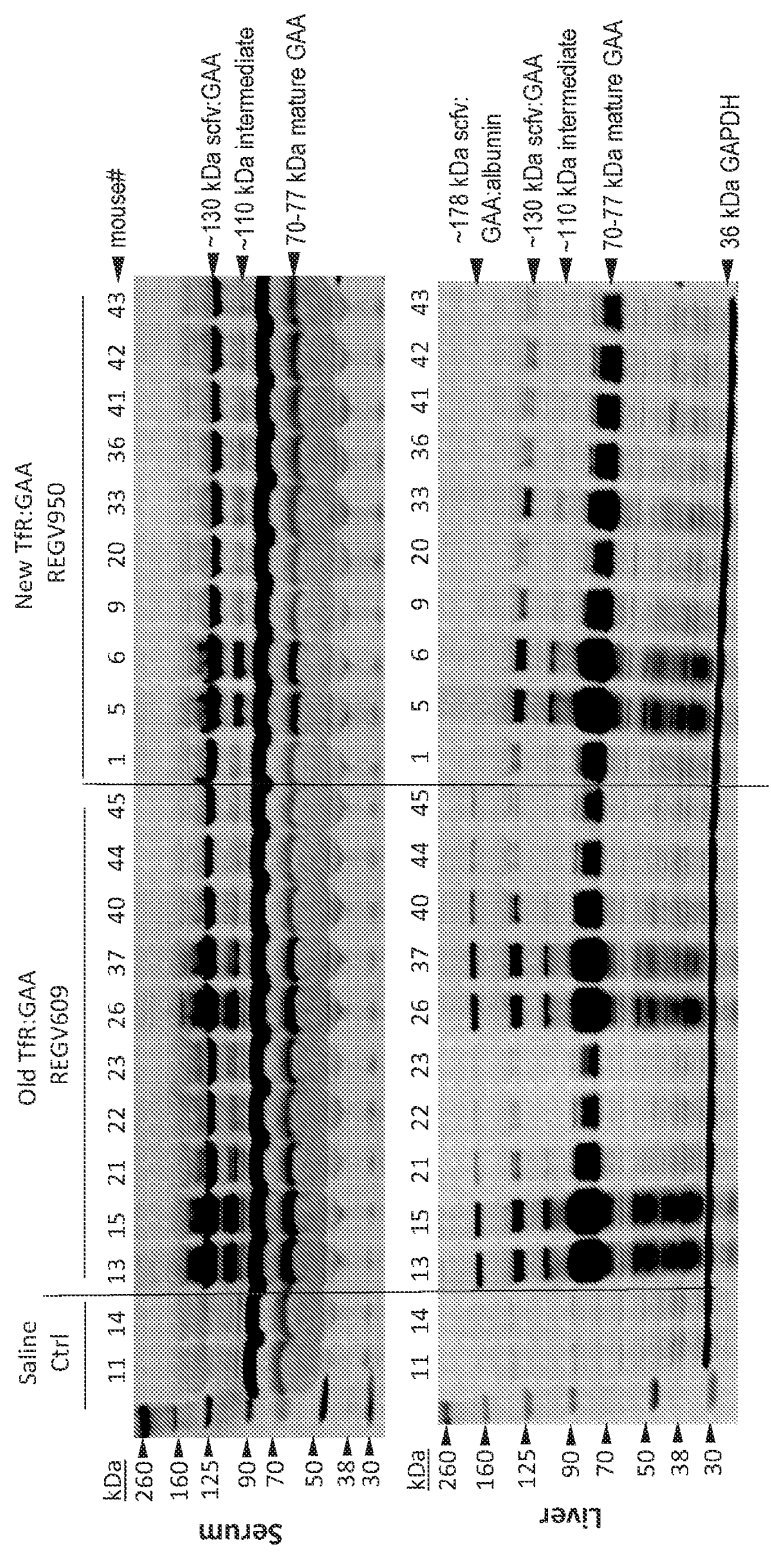


FIG. 47A

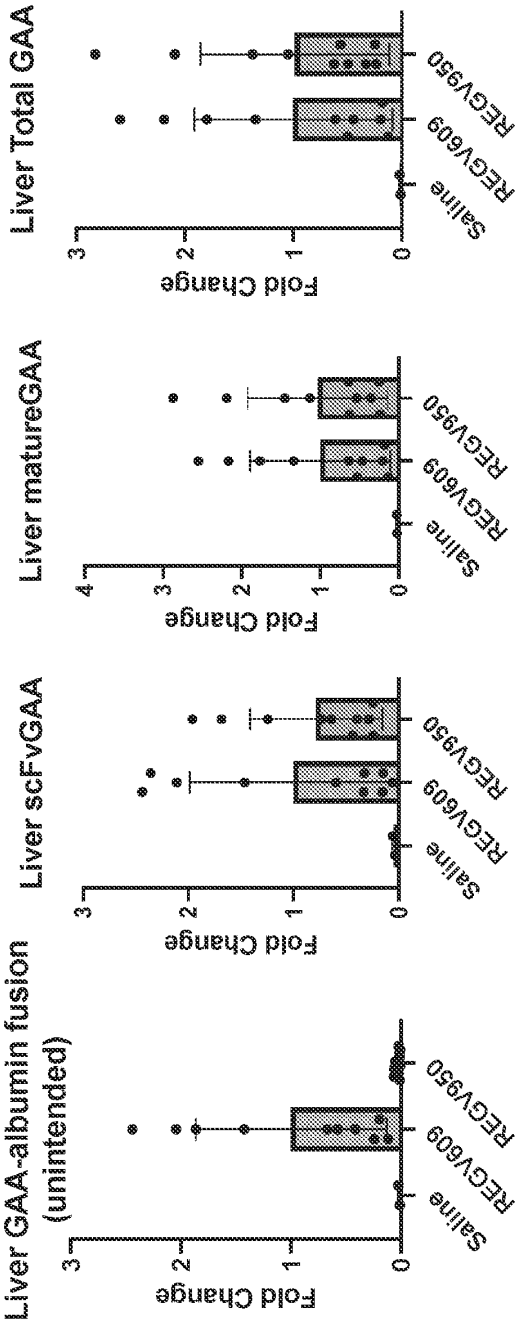


FIG. 47B

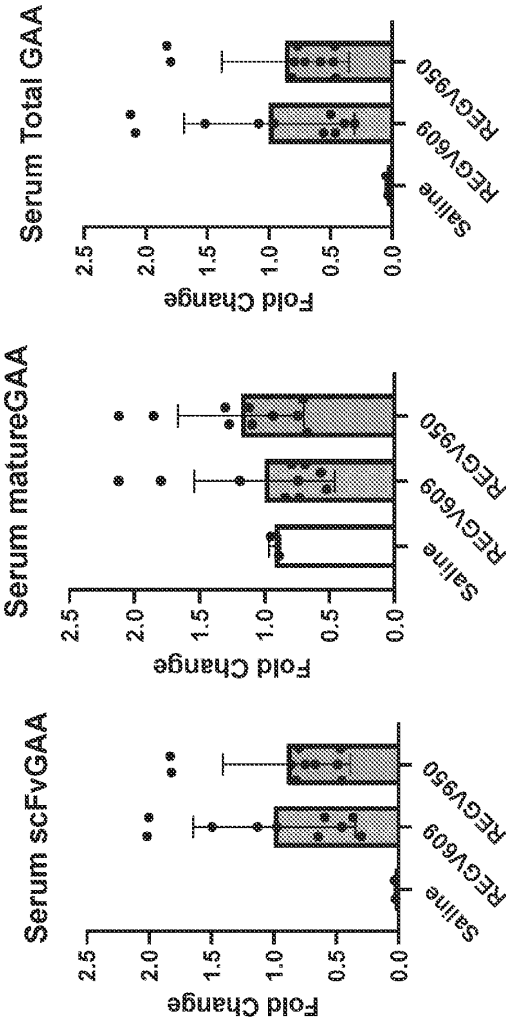


FIG. 47C

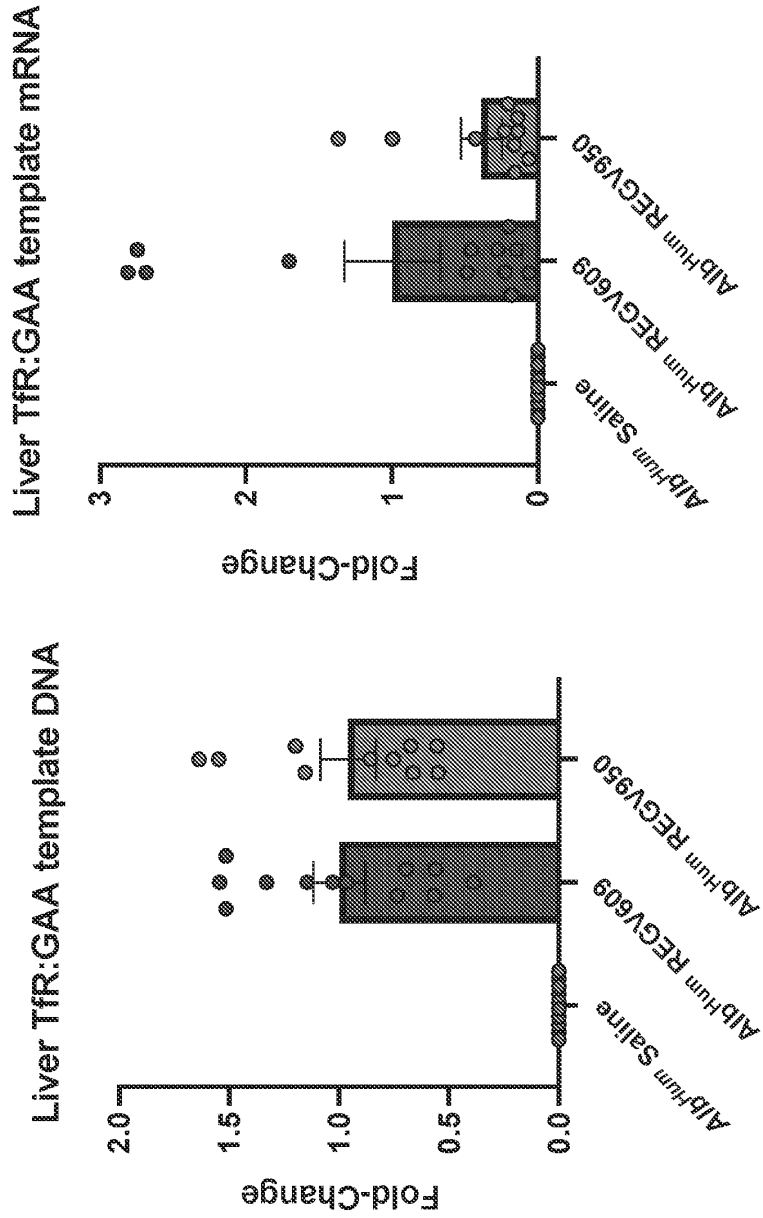


FIG. 48