

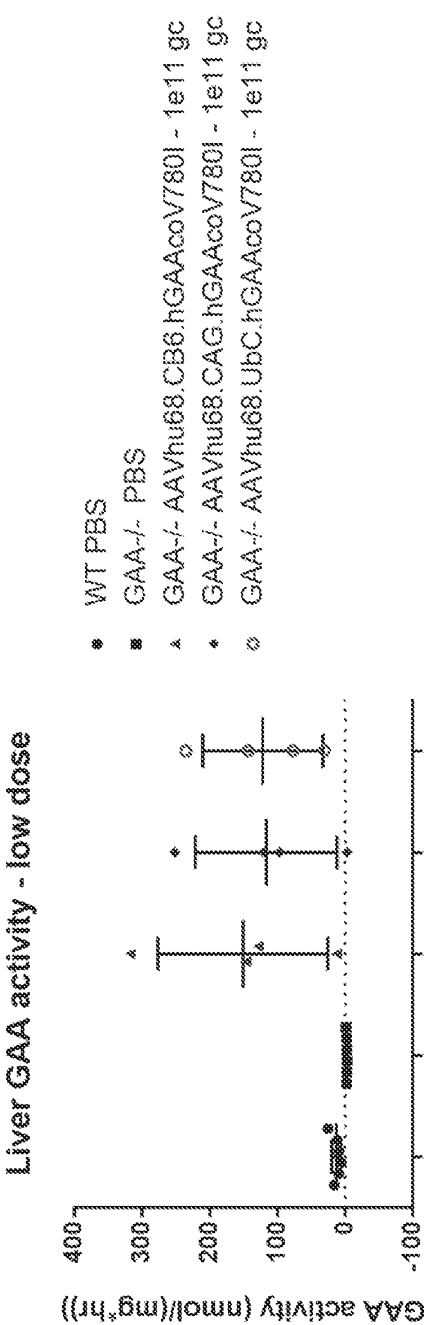


FIG. 1A

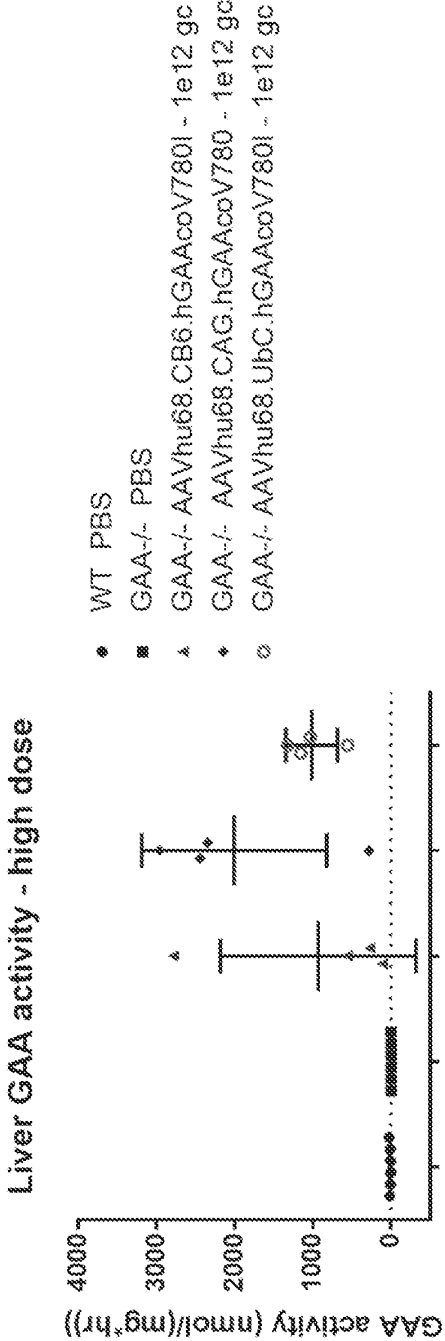


FIG. 1B

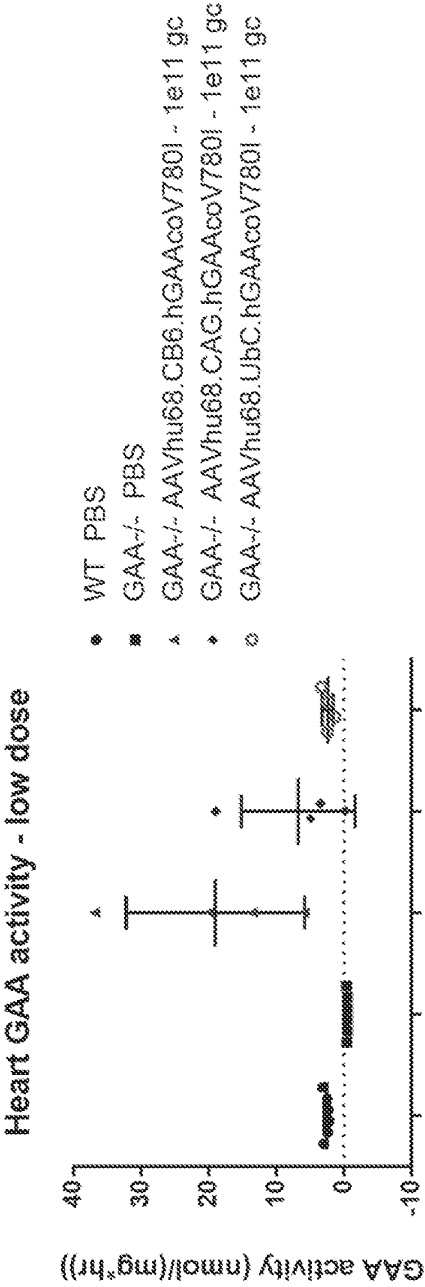


FIG. 2A



FIG. 2B

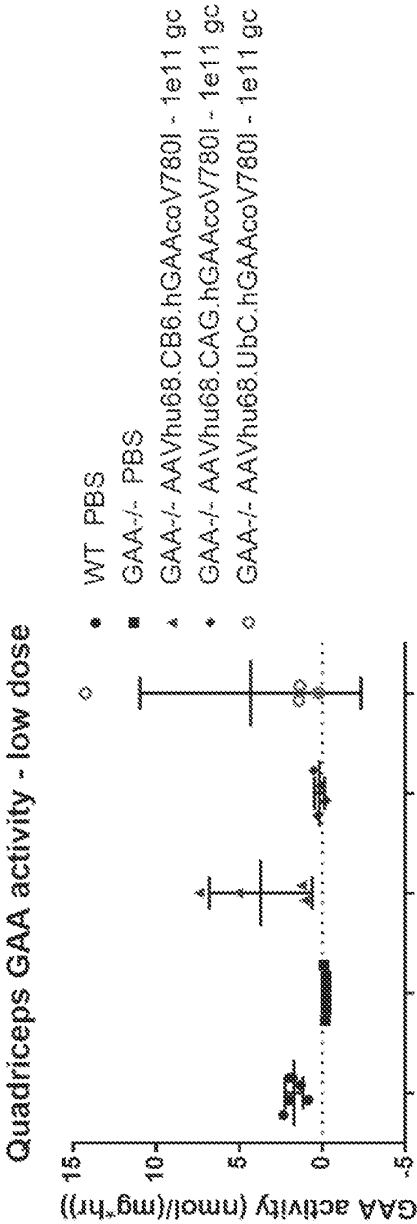


FIG. 3A

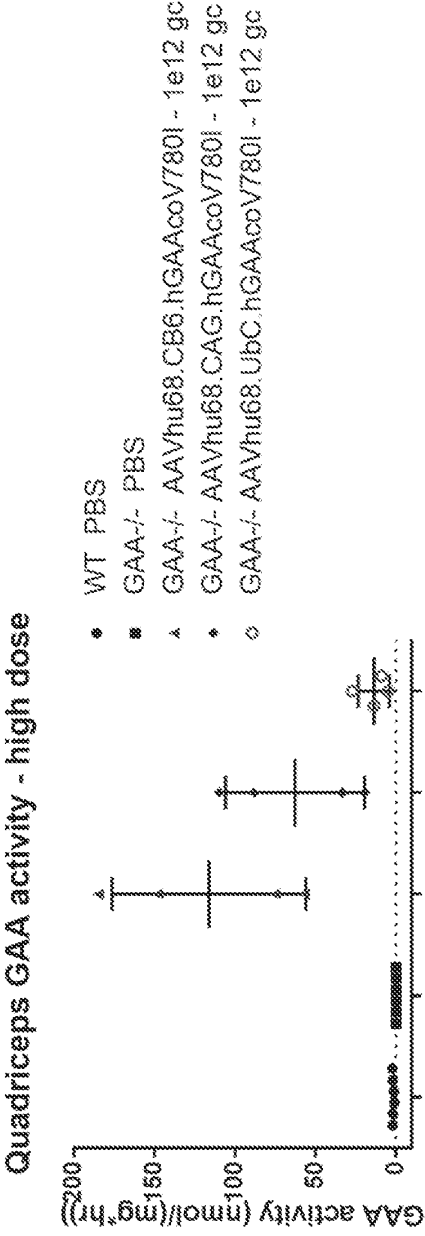


FIG. 3B

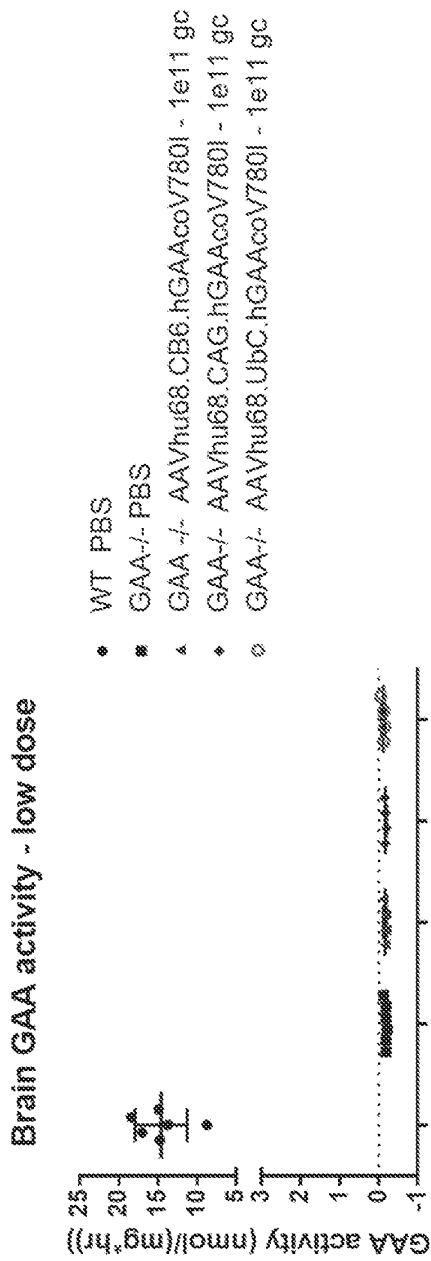


FIG. 4A

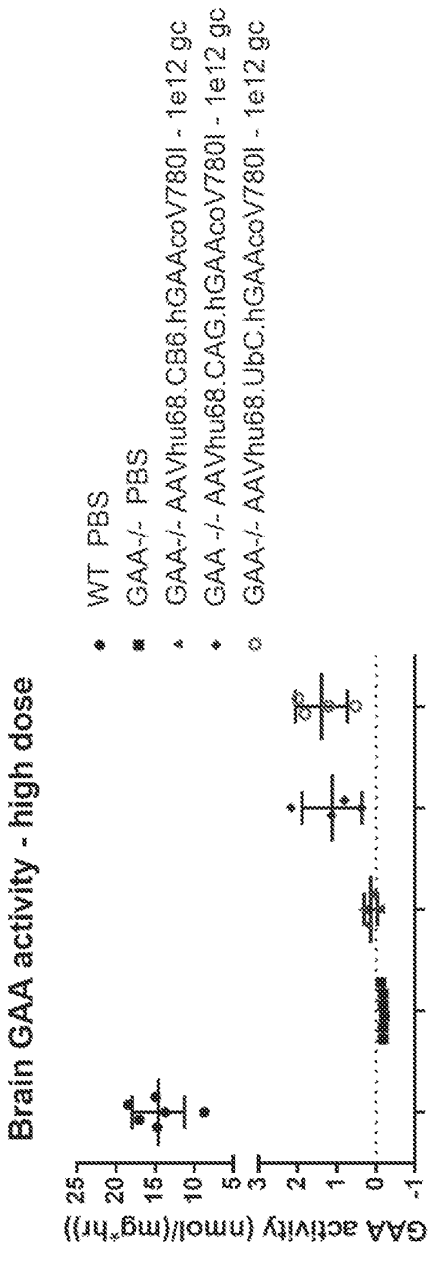


FIG. 4B

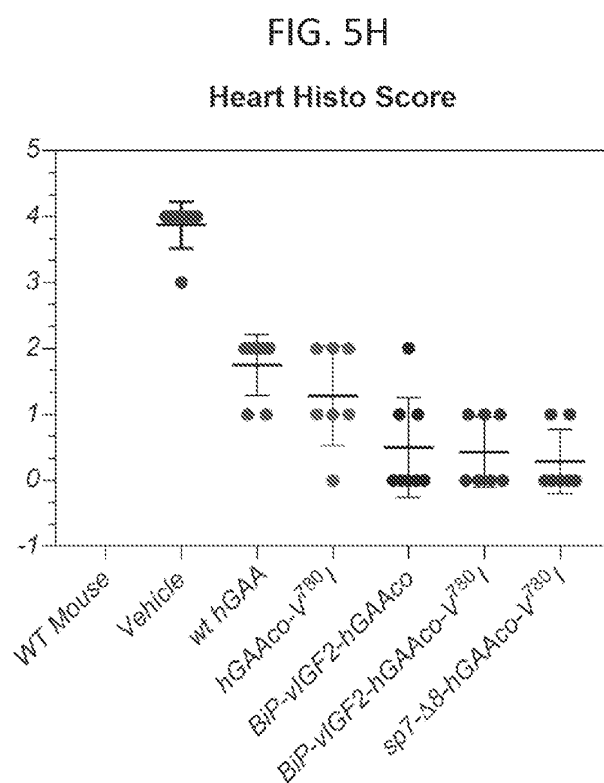
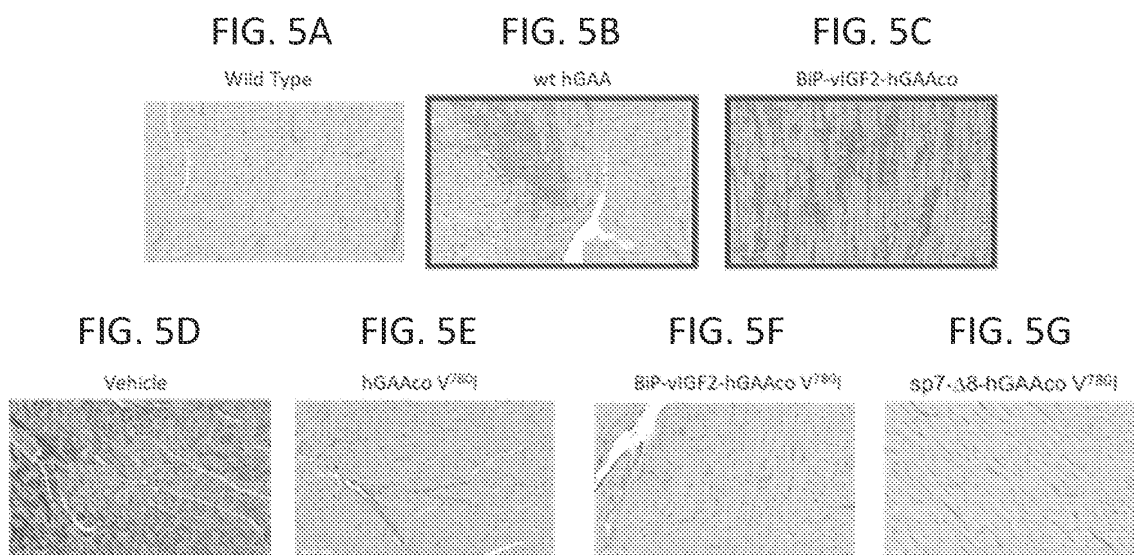


FIG. 6A

+/- PBS

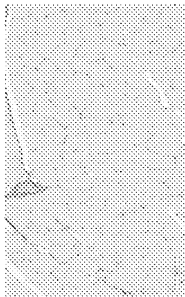


FIG. 6B

-/- hGAA

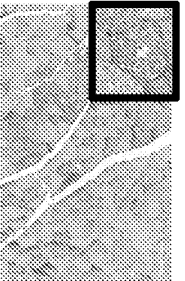


FIG. 6C

-/- BiP, VEGF2, hGAAco

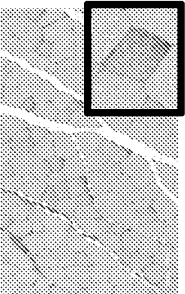


FIG. 6D

-/- PBS

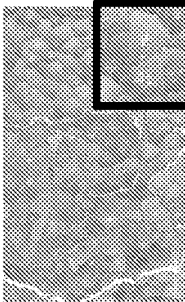


FIG. 6E

-/- hGAAcoV780I

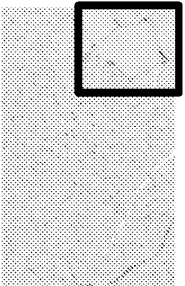


FIG. 6F

-/- Sp7, Δ8, hGAAcoV780I -/- BiP, VEGF2, hGAAcoV780I

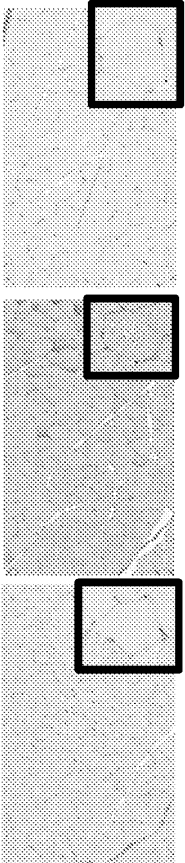


FIG. 6G

FIG. 6H

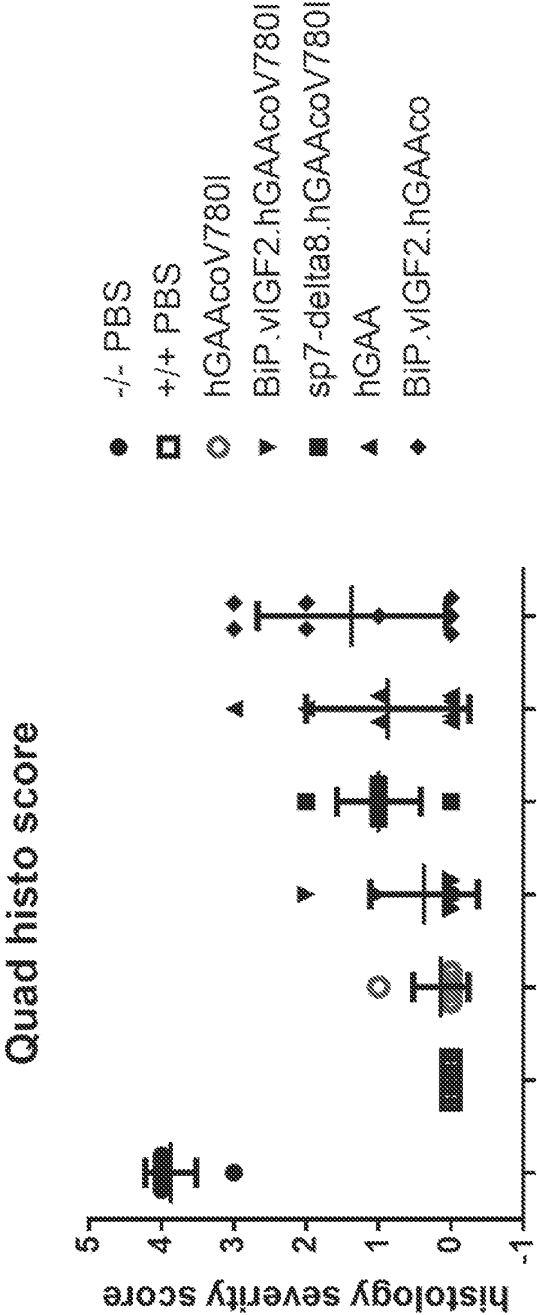


FIG. 7A

+/- PBS

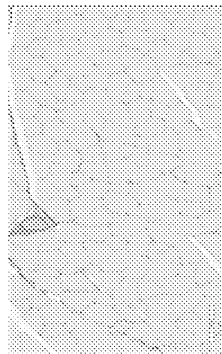


FIG. 7B

-/- hGAA

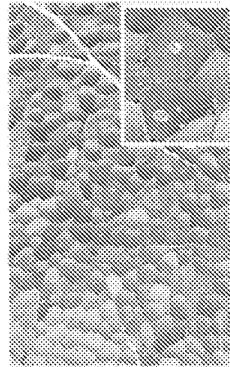
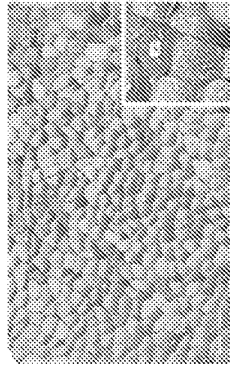
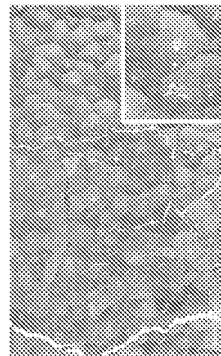


FIG. 7C

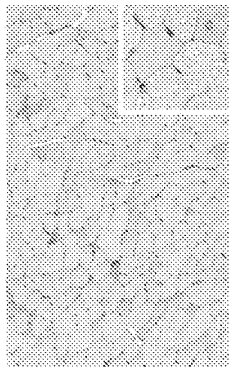
-/- BiP.vIGF2.hGAA^{co}



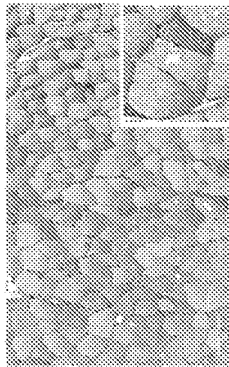
-/- PBS



-/- hGAA^{co}V780I



-/- Sp7.Δ8.hGAA^{co}V780I



-/- BiP.vIGF2.hGAA^{co}V780I

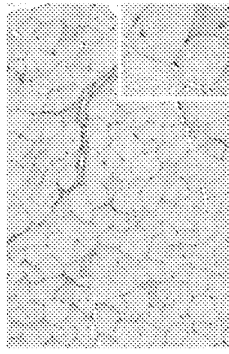


FIG. 7D

FIG. 7E

FIG. 7F

FIG. 7G

FIG. 7H

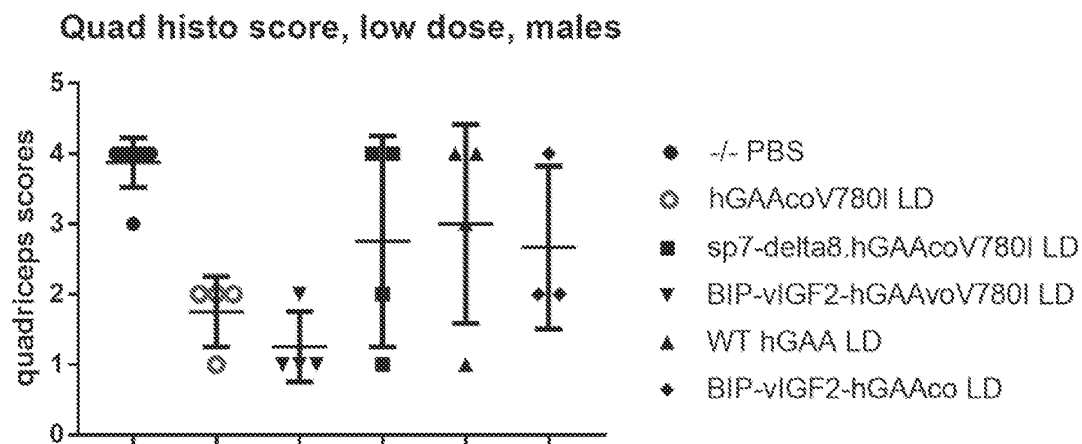


FIG. 8

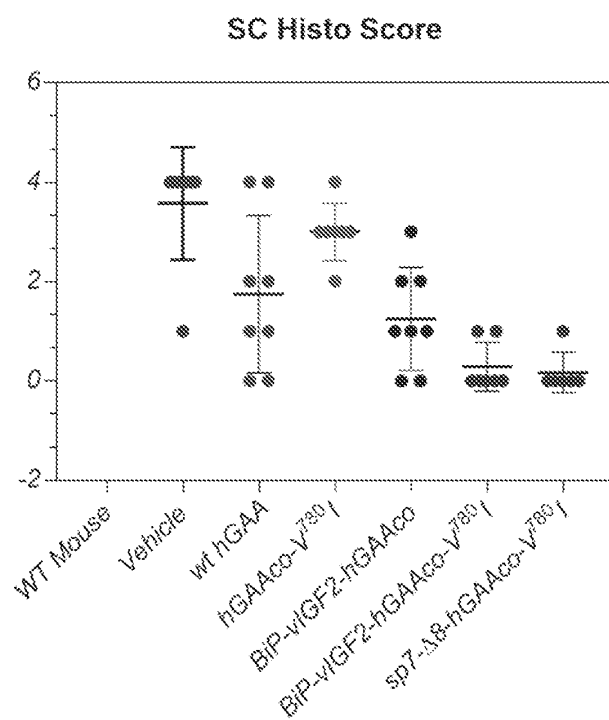


FIG. 9A

Activity

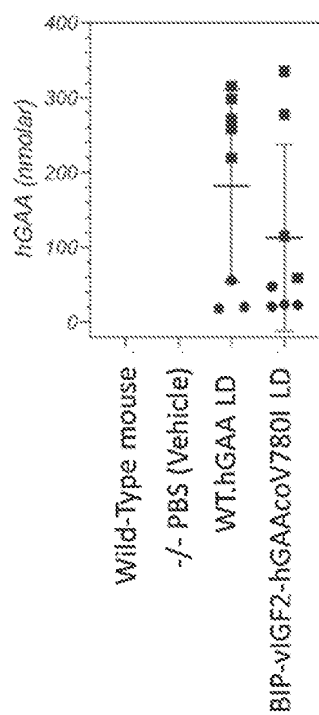


FIG. 9B

MS signature peptide

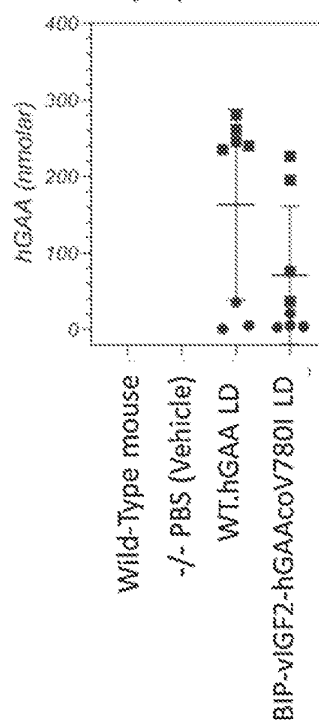


FIG. 9C

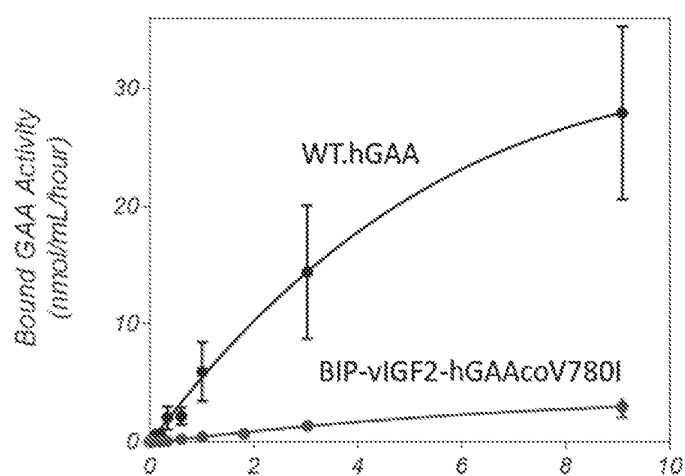


FIG. 10 LC3b (autophagic vacuole marker) and lectin (membrane)

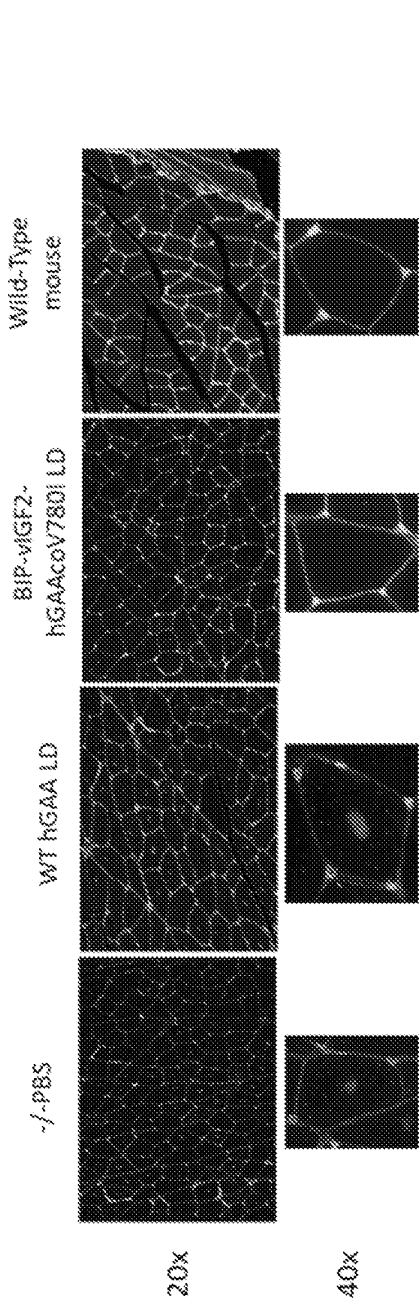


FIG. 11

BiP-vIGF2.hGAAcoV780I.4xmiR183

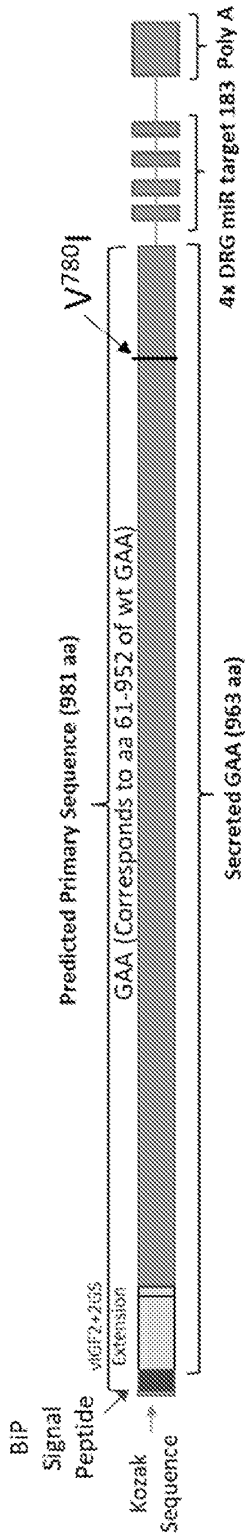


FIG. 12

Brainstem

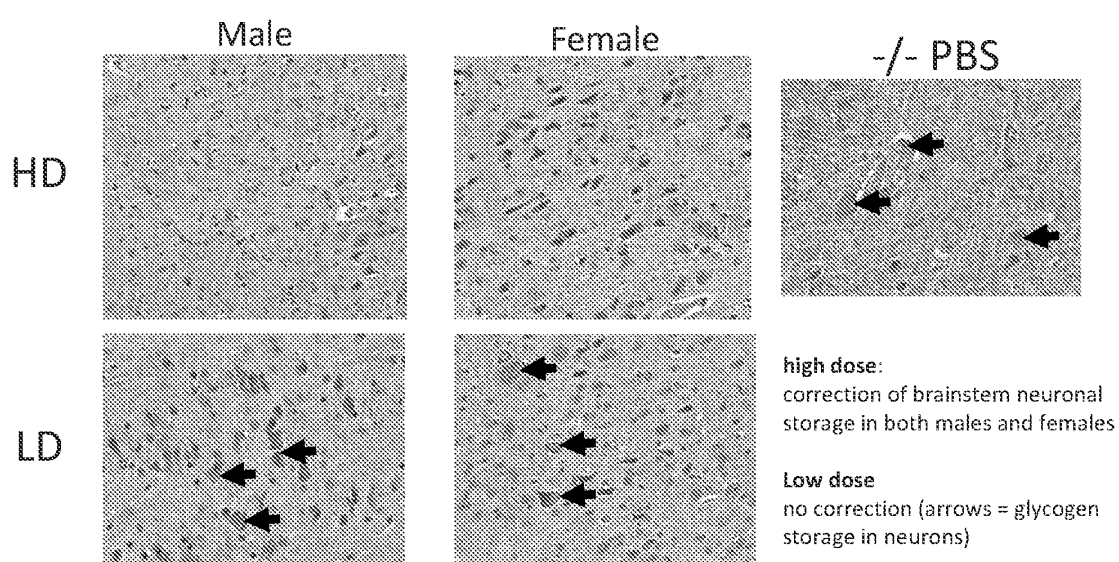


FIG. 13

Spinal cord

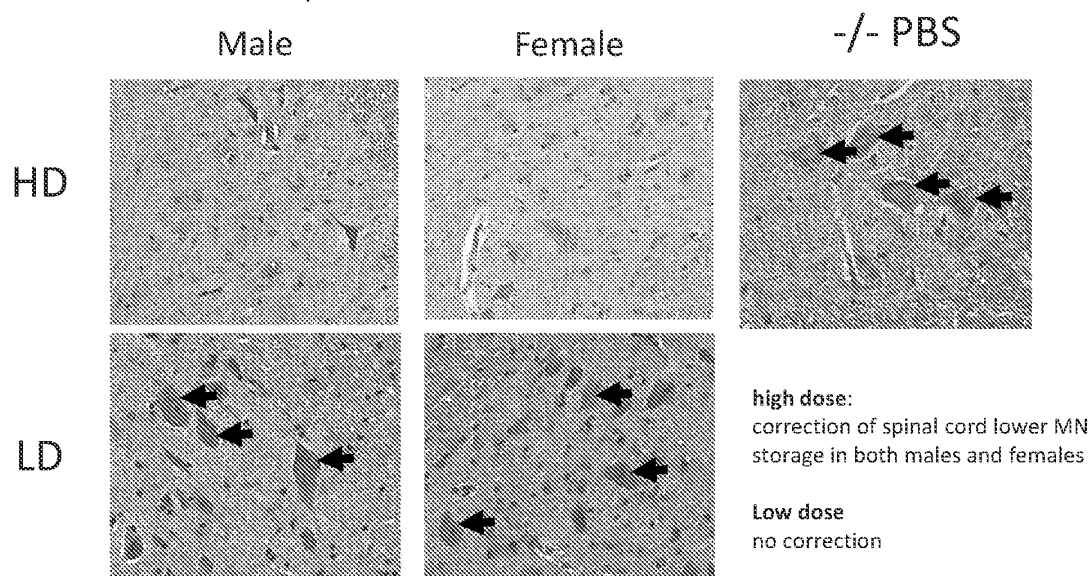


FIG. 14

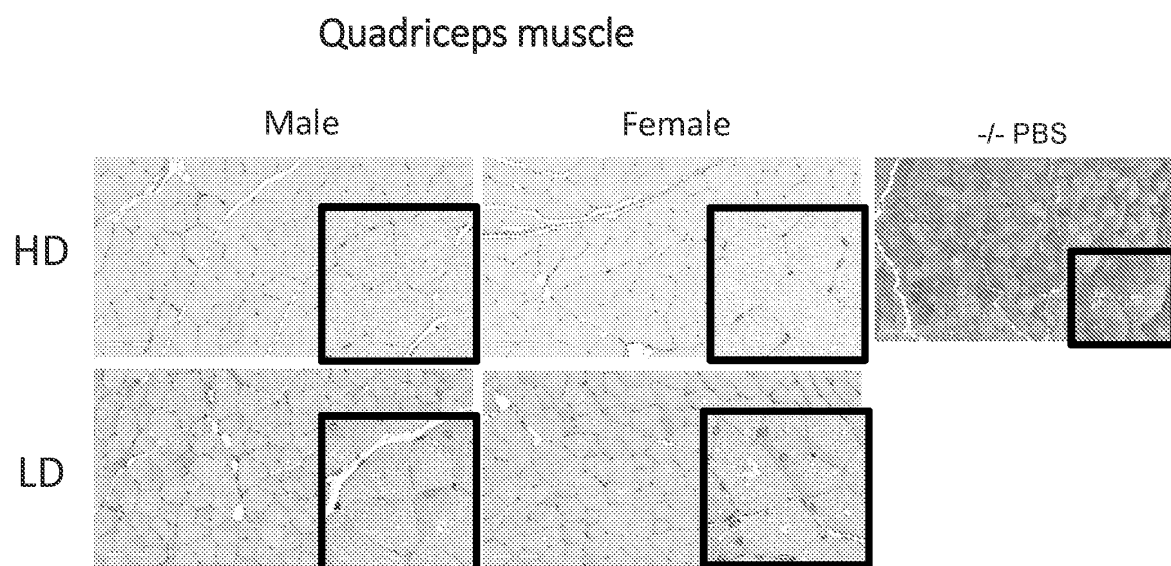
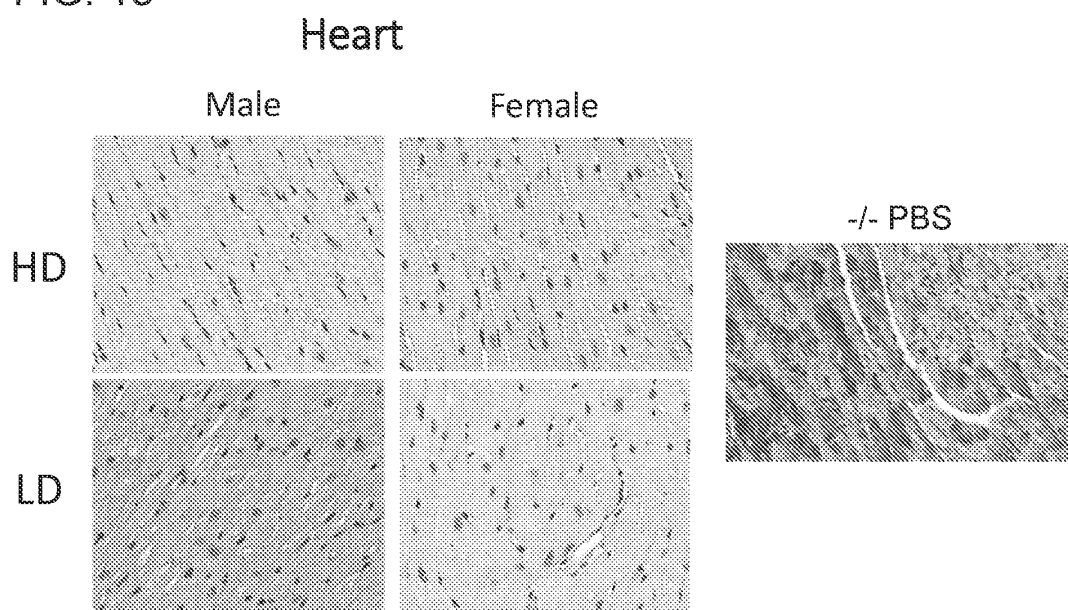


FIG. 15



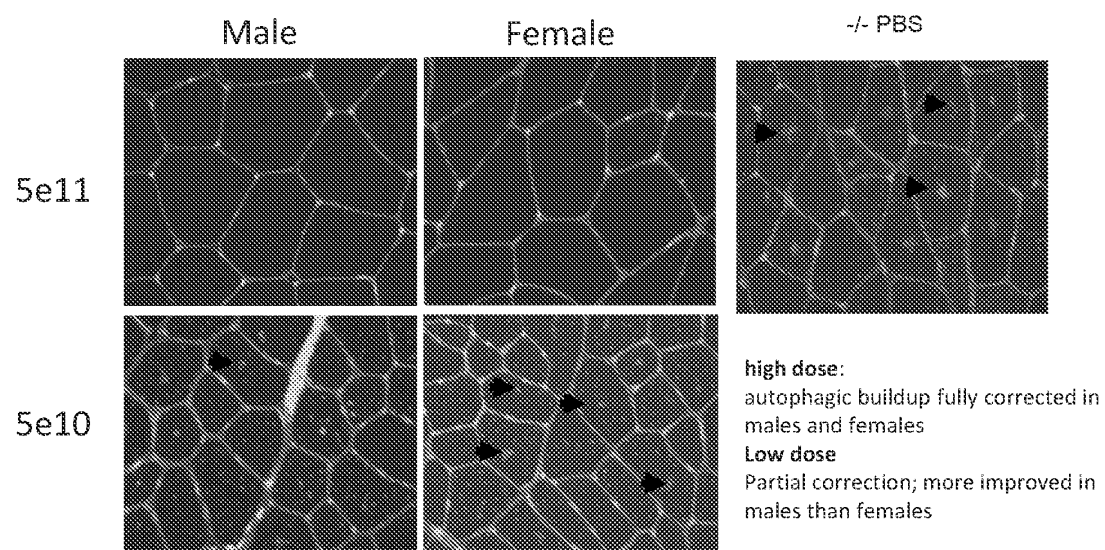


FIG. 16

FIG. 17 Cervical DRG hGAA IHC

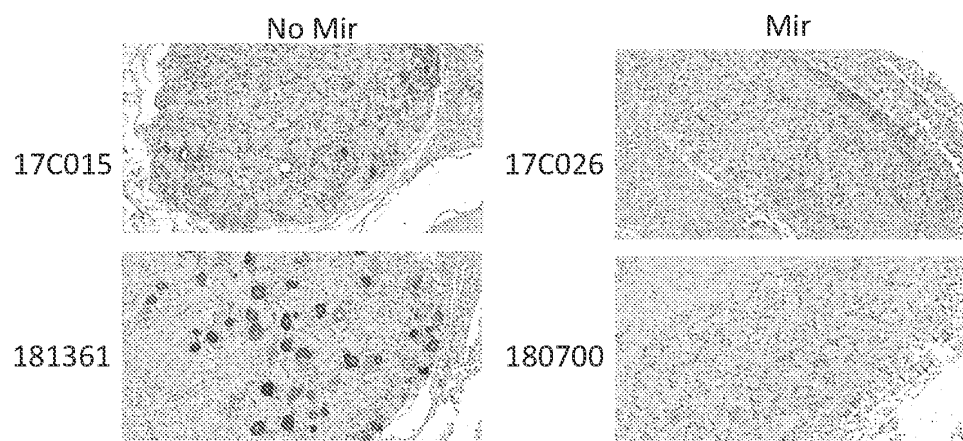


FIG. 18

Lumbar DRG hGAA IHC

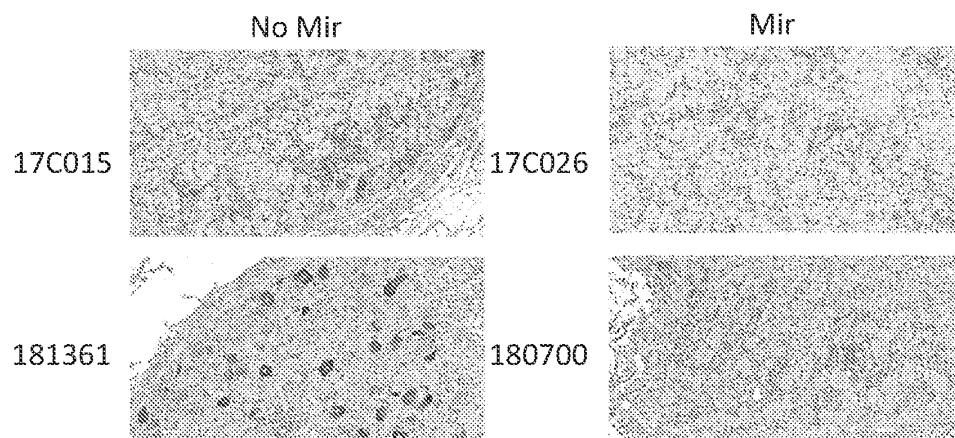


FIG. 19 cervical spinal cord
lower MN hGAA IHC

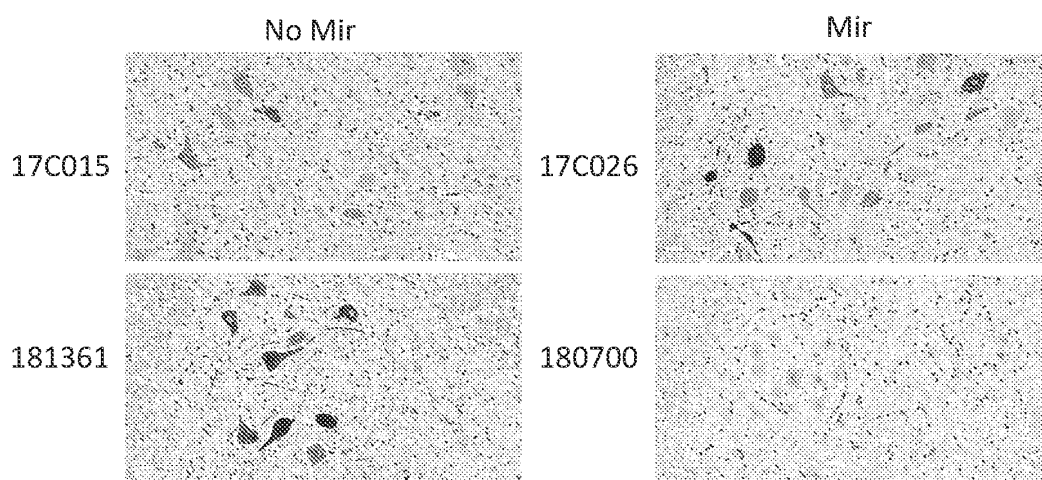


FIG. 20

heart hGAA IHC

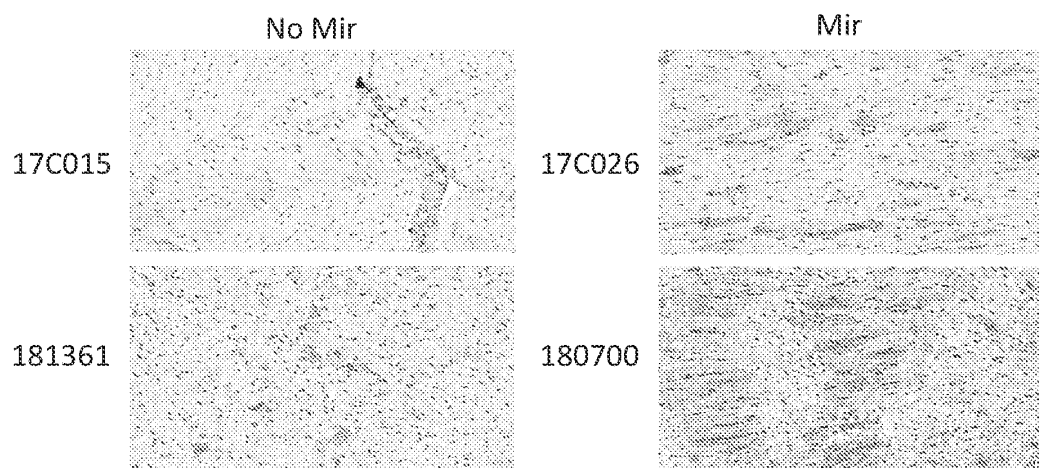


FIG. 21A

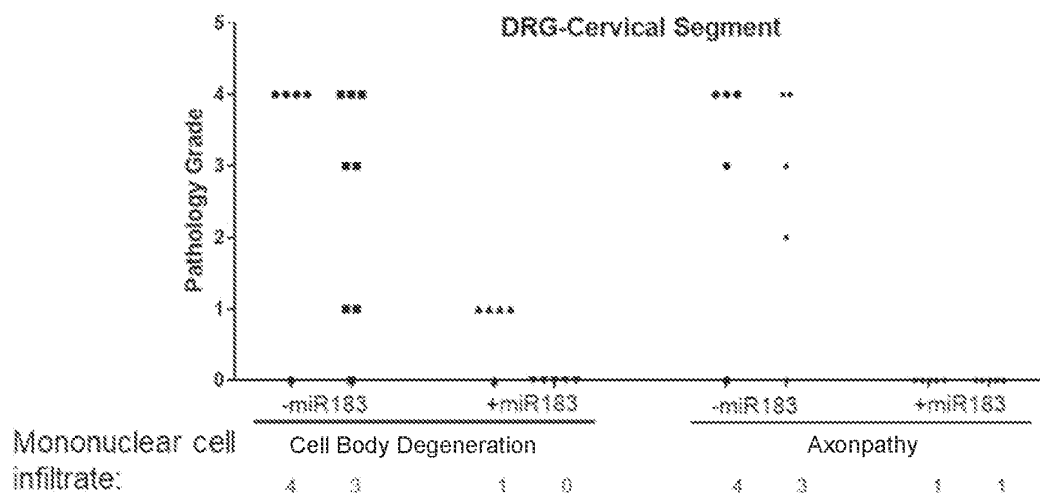


FIG. 21B

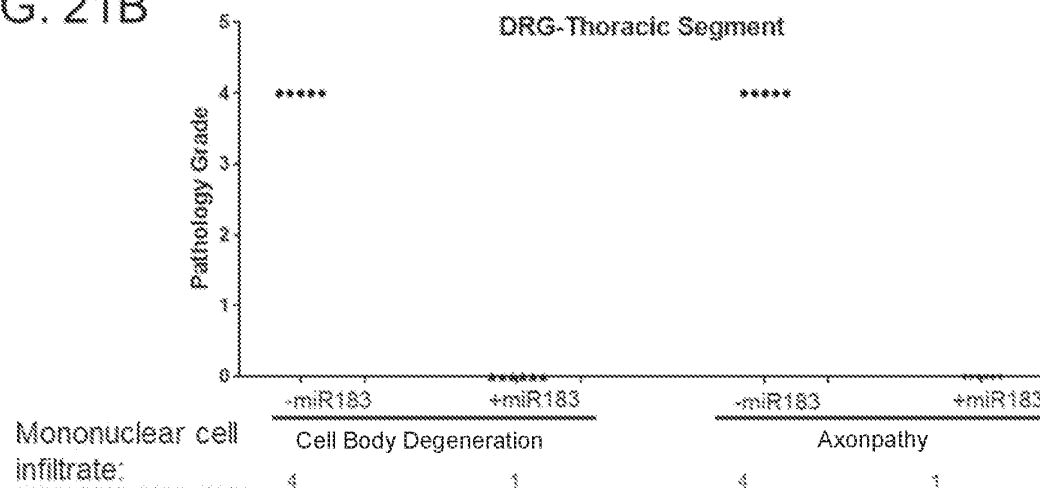


FIG. 21C

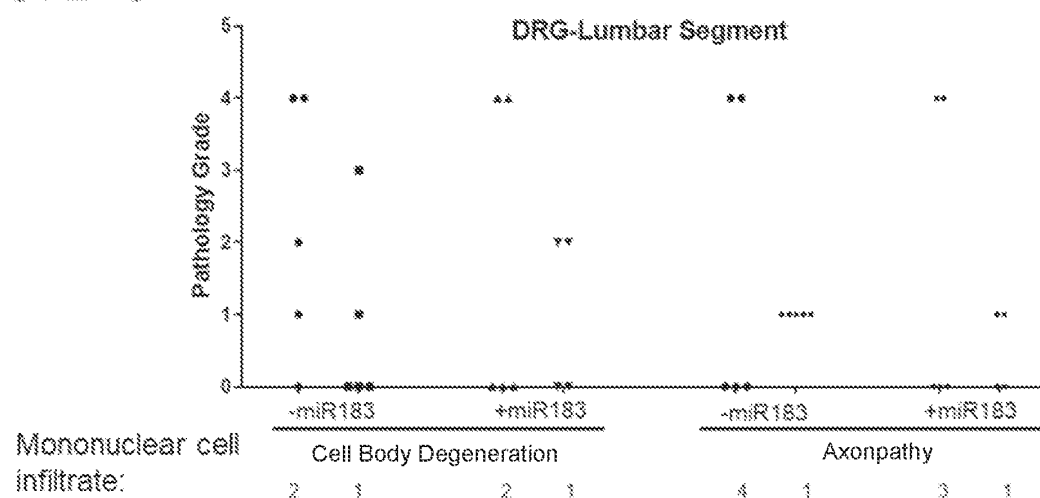


FIG. 22A

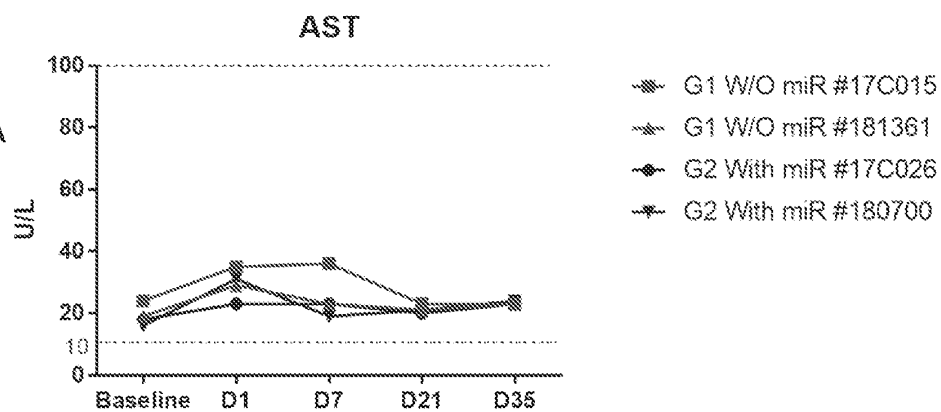


FIG. 22B

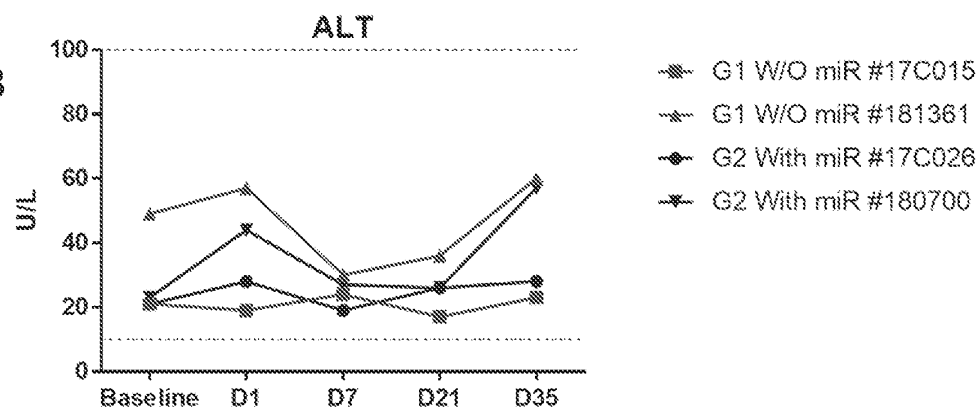


FIG. 22C

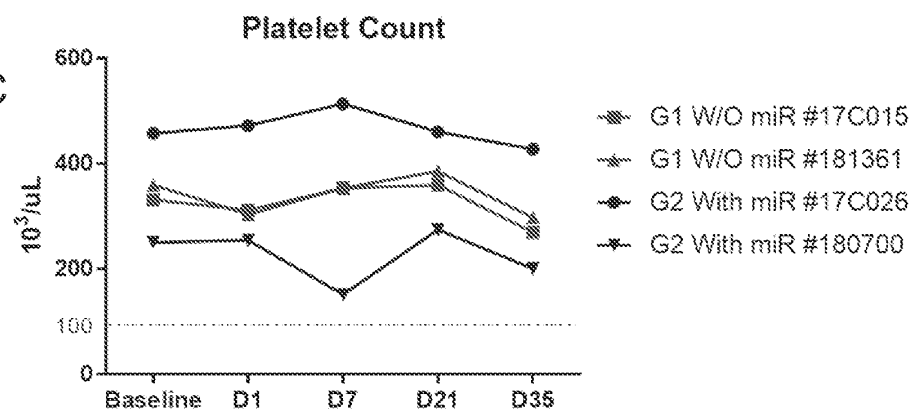


FIG. 23

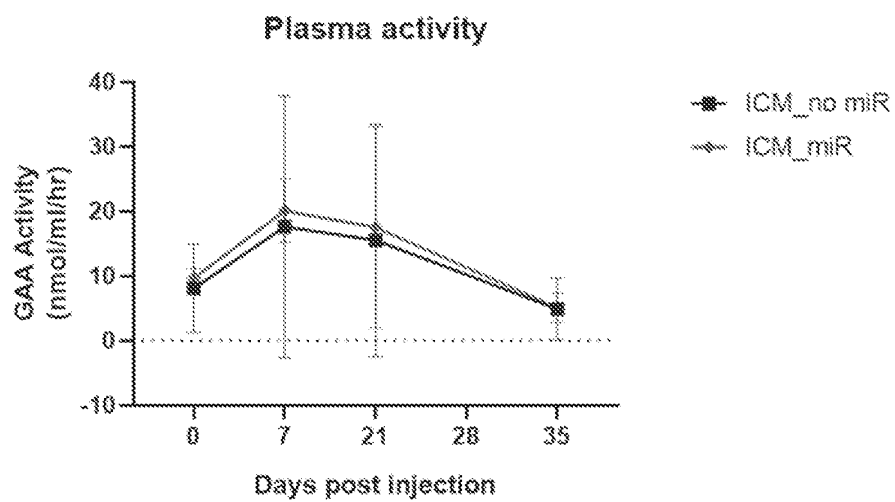


FIG. 24A

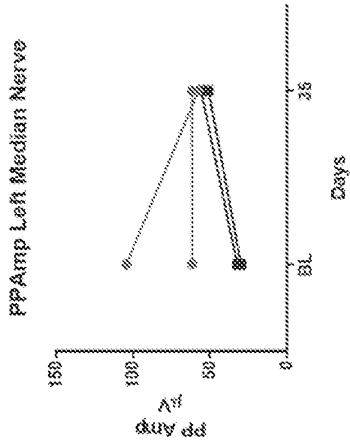


FIG. 24B

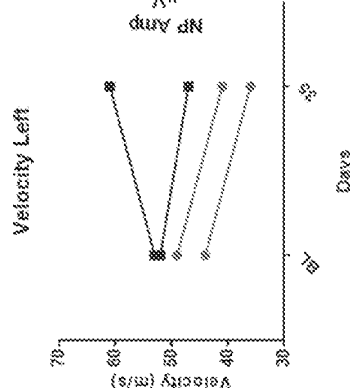


FIG. 24C

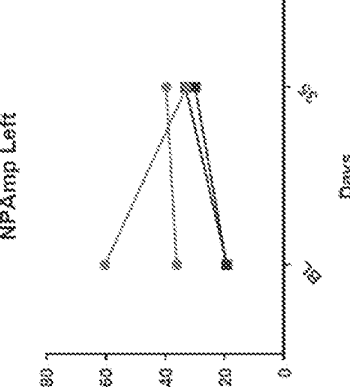


FIG. 24D

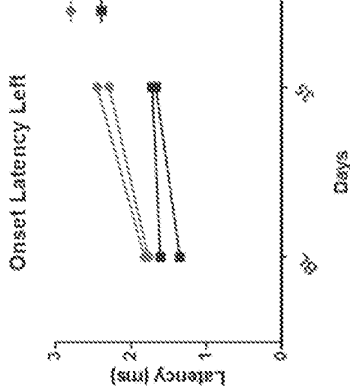


FIG. 24E

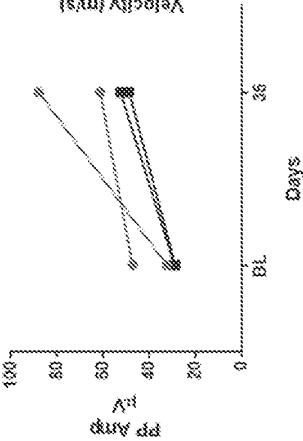


FIG. 24F

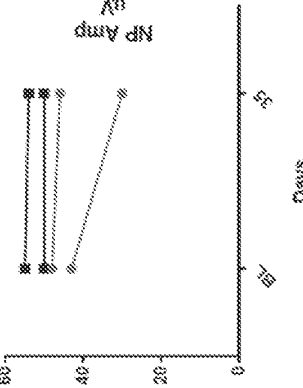


FIG. 24G

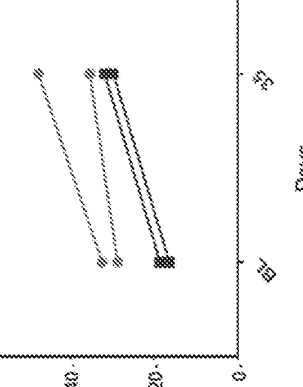


FIG. 24E

FIG. 24F

FIG. 24G

FIG. 25A

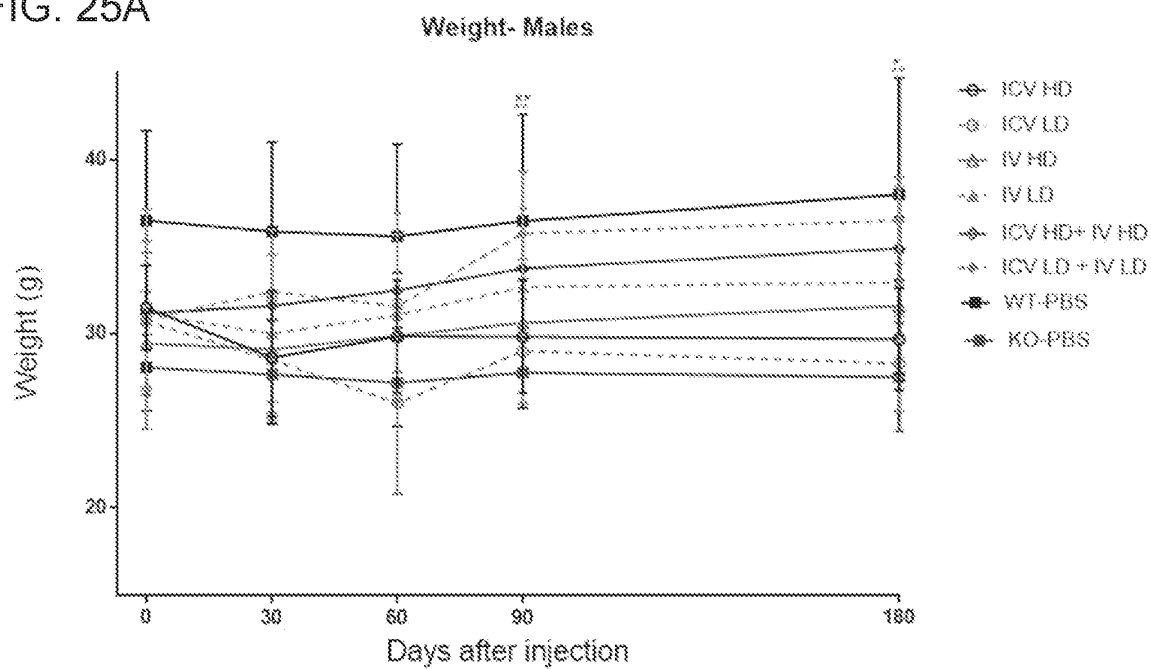


FIG. 25B

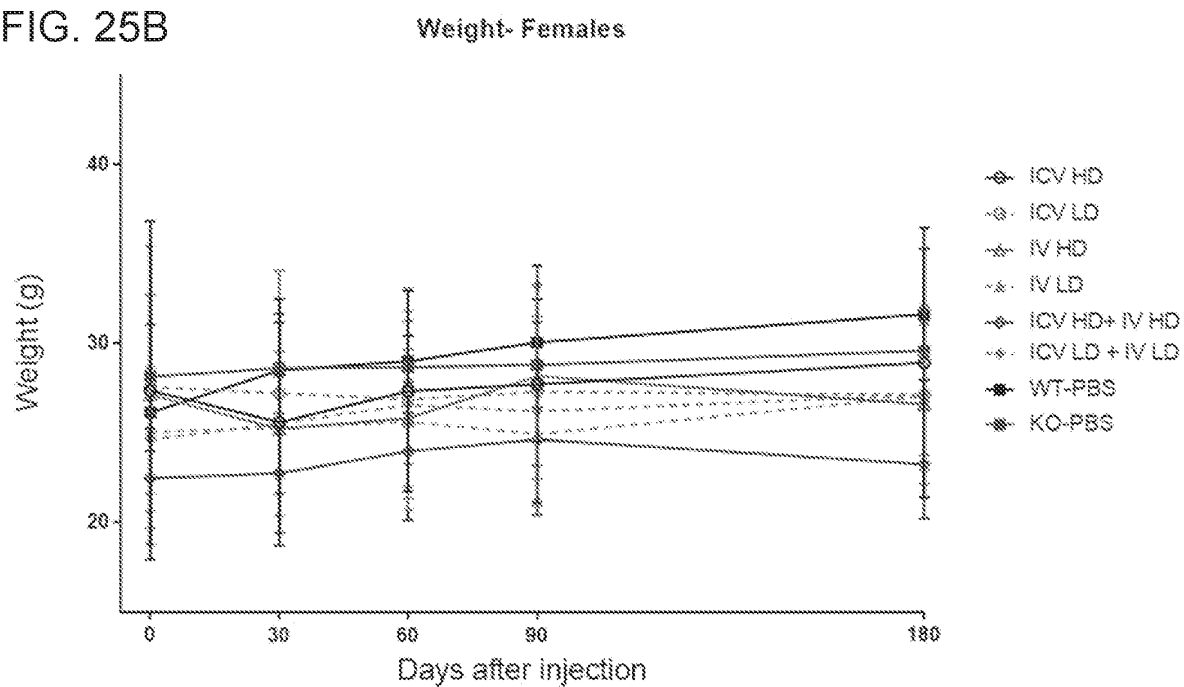


FIG. 26

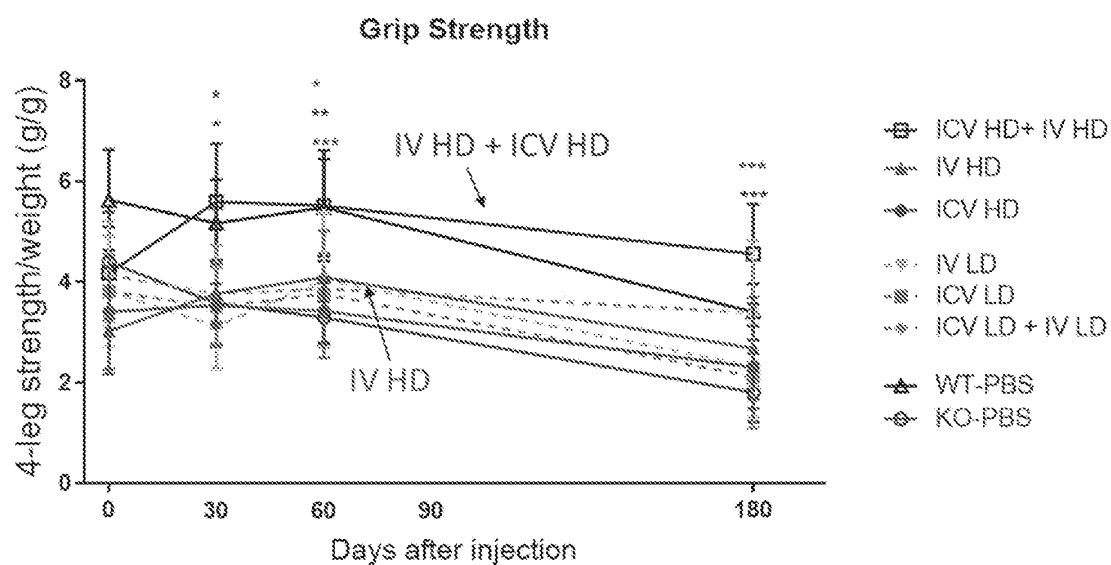


FIG. 27

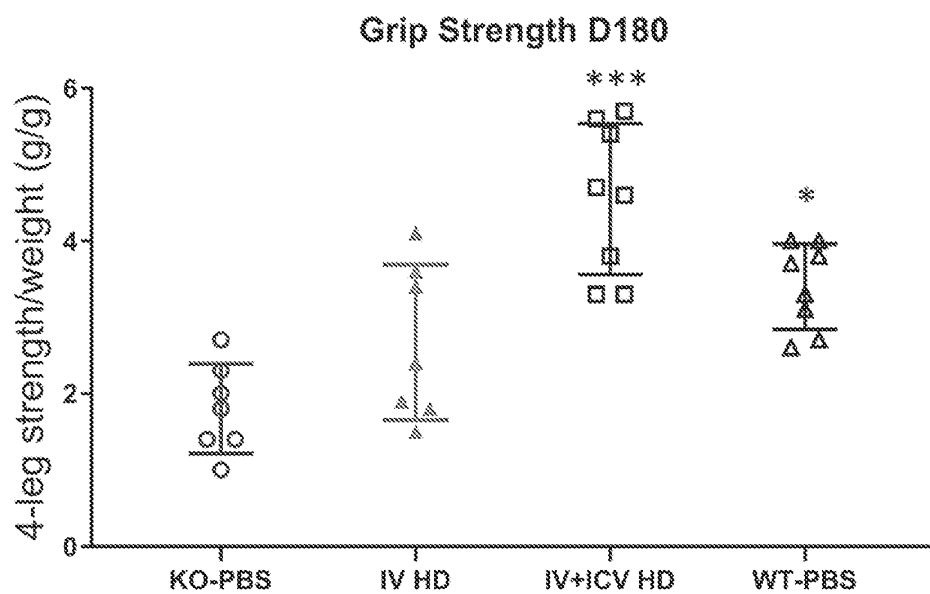
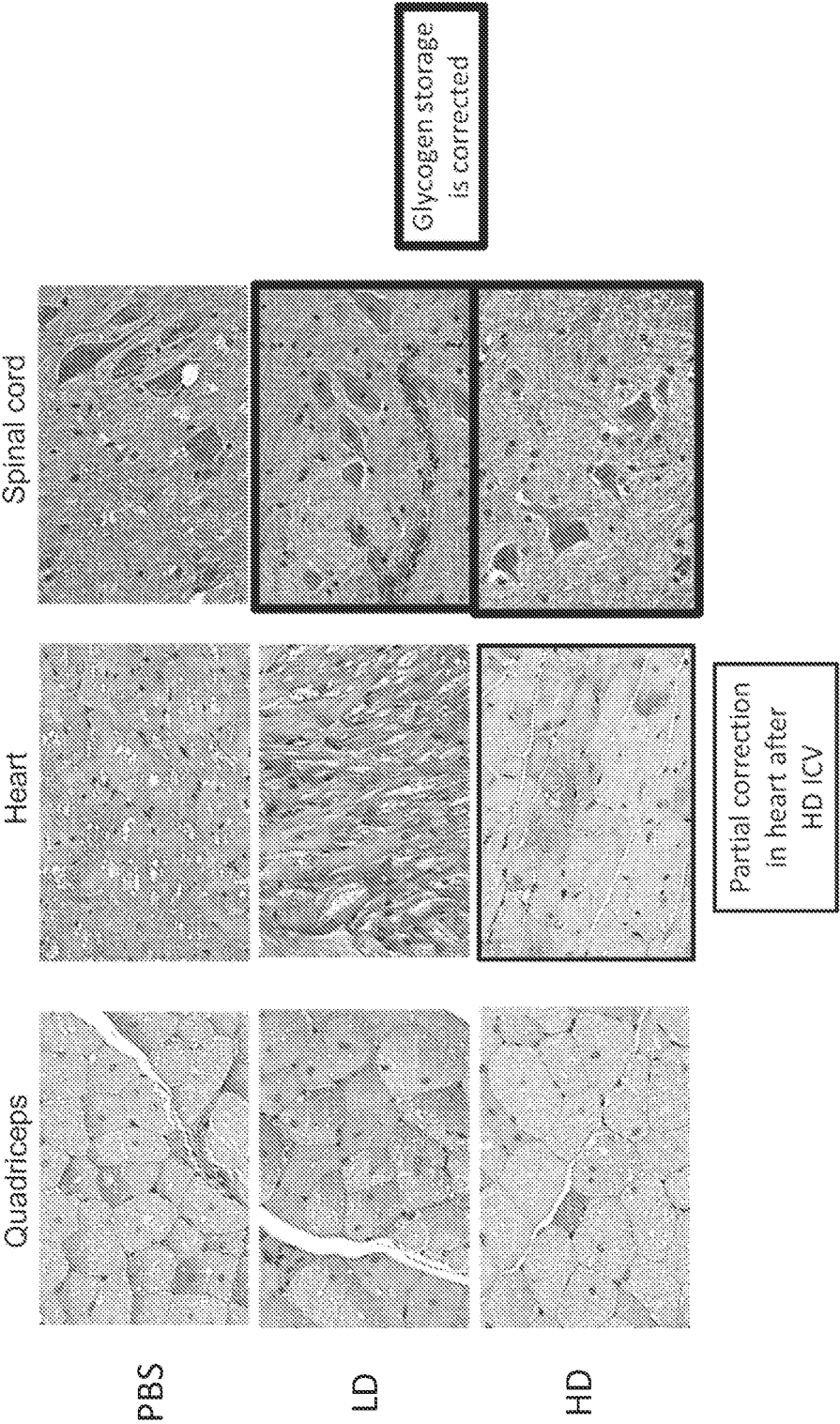
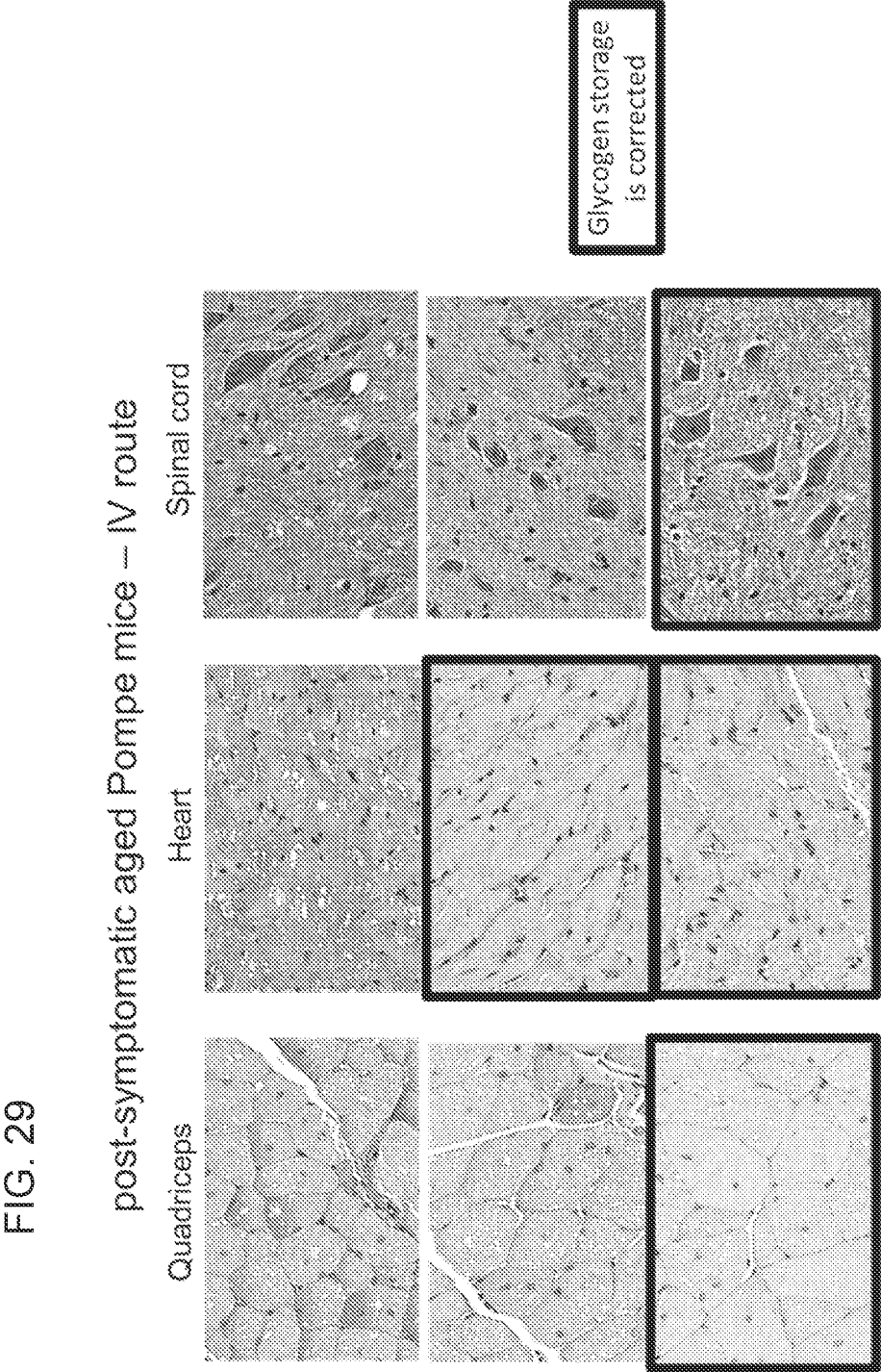


FIG. 28

post-symptomatic Pompe mice – ICV route





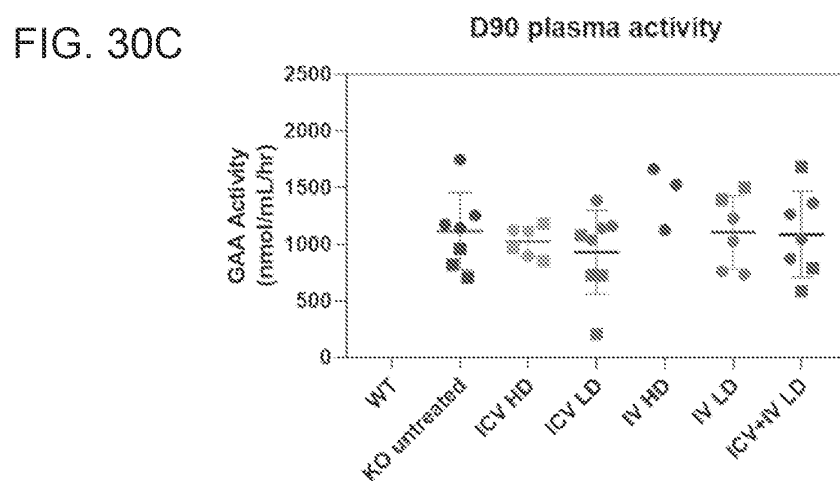
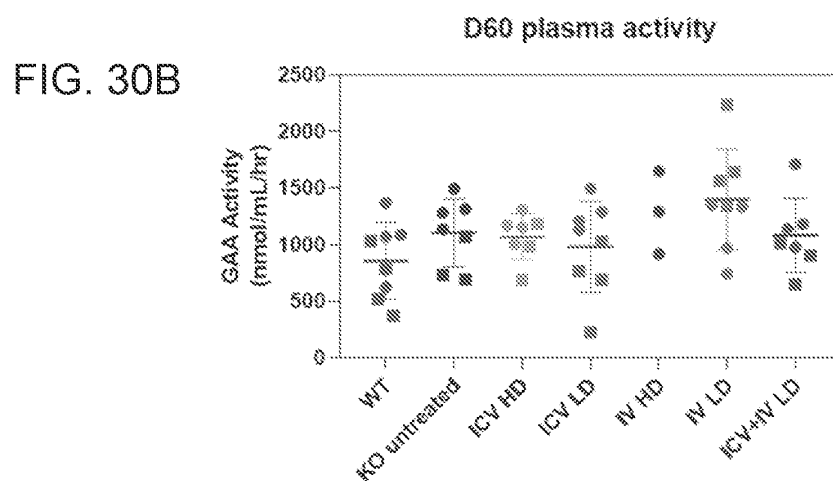
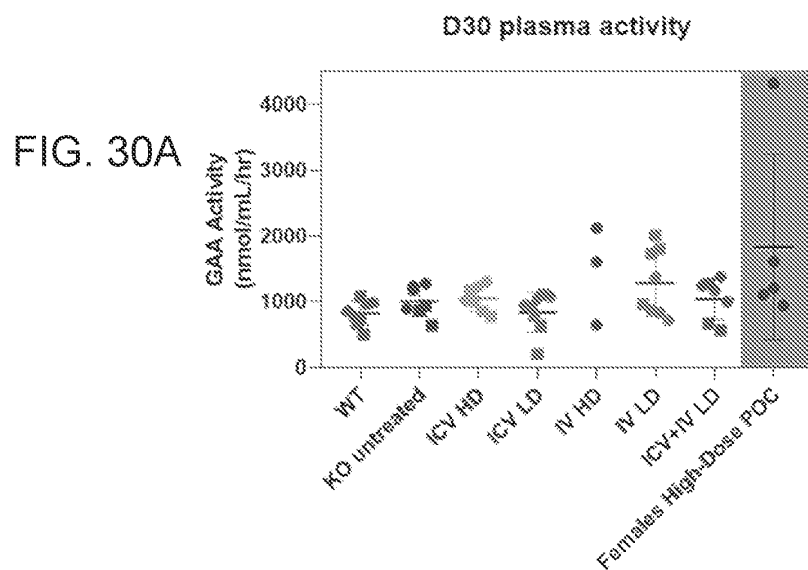


FIG. 31

Day									
-21	-10 (approx.)	0	3	7	21	29	35	60	
Baseline Clin Path NAB Neuro Exam	Pred	Vector dosing Clinpath Plasma / CSF NAB / PBMC	Clinpath Plasma	Clinpath Plasma / CSF	Clinpath Plasma / CSF	Pred Taper	Clinpath Plasma / CSF Neuro exam	Macropsy Clinpath Plasma / CSF NAB / PBMC Neuro exam	
Group Designation	1	2	3	4	5	6*			
N / Group	2	2	2	2	2	2			
Route of administration	IV LD	IV HD	ICM LD	ICM HD	IV HD ICM HD	IV HDD ICM LD			
Vector Dose	1e13 GC/kg	5e13 GC/kg	1e13 GC	3e13 GC	5E13 GC/kg 3e13 GC	1E14 GC/kg 1e13 GC			

FIG. 32A

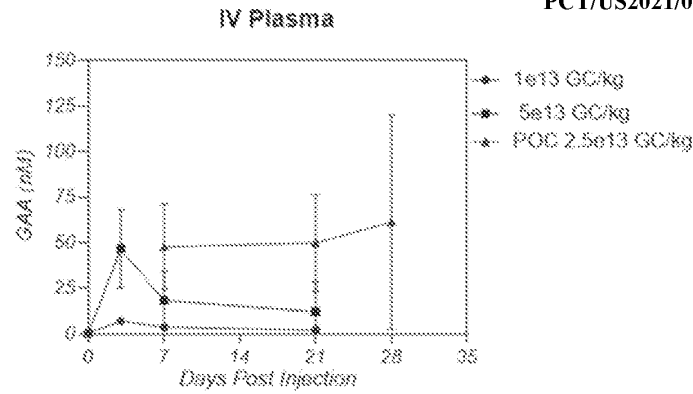


FIG. 32B

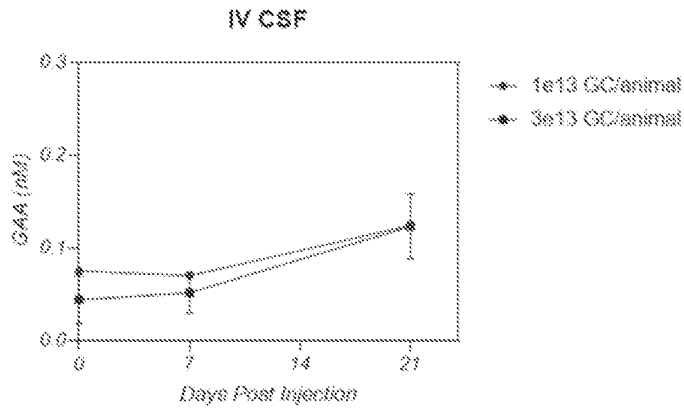


FIG. 32C

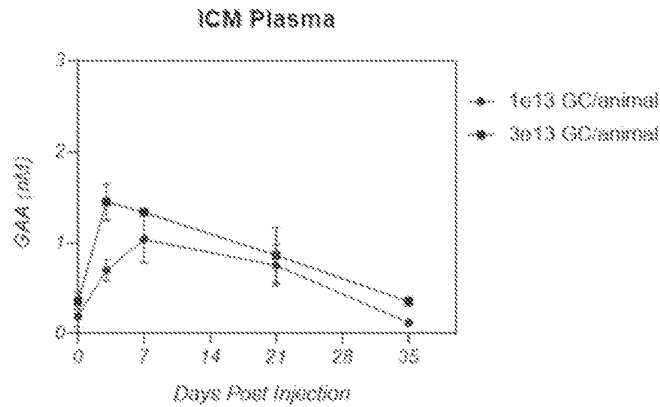


FIG. 32D

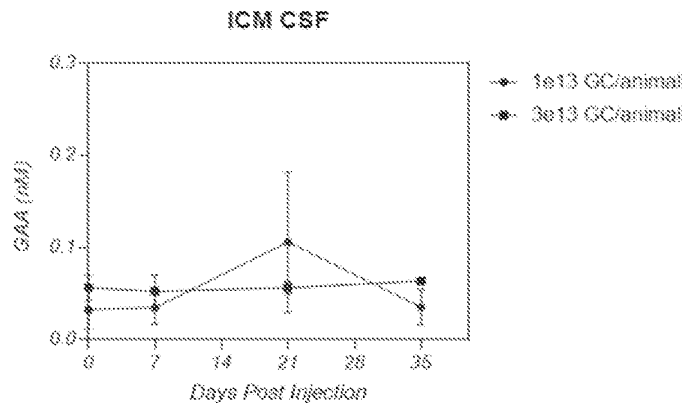


FIG. 32E

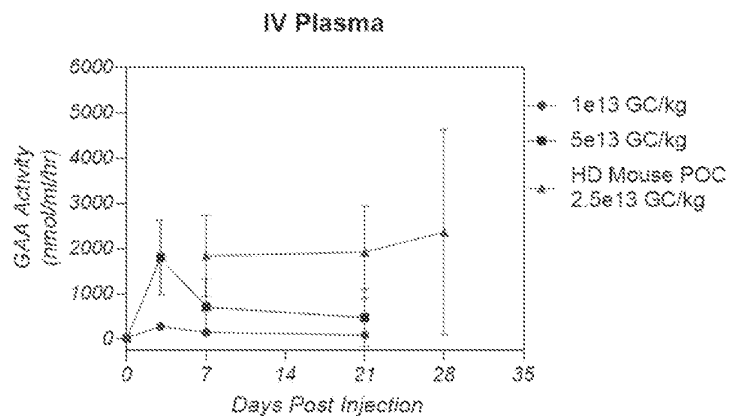


FIG. 32F

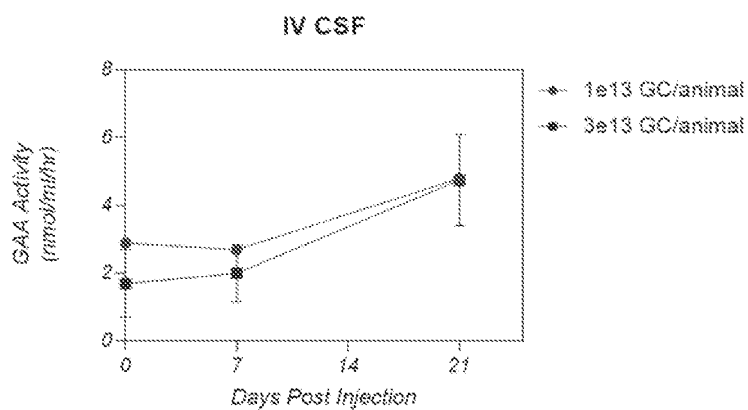


FIG. 32G

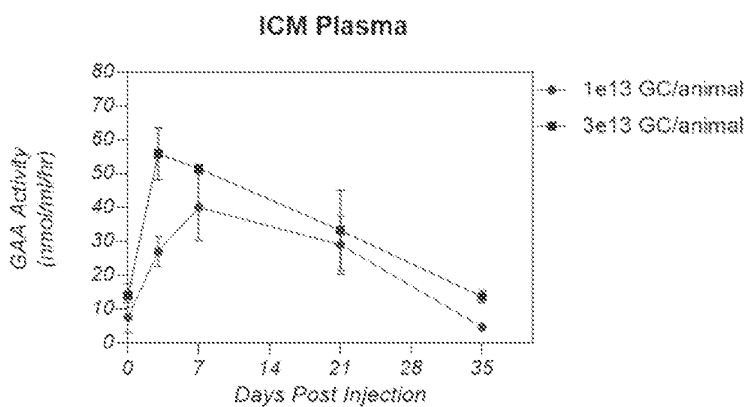
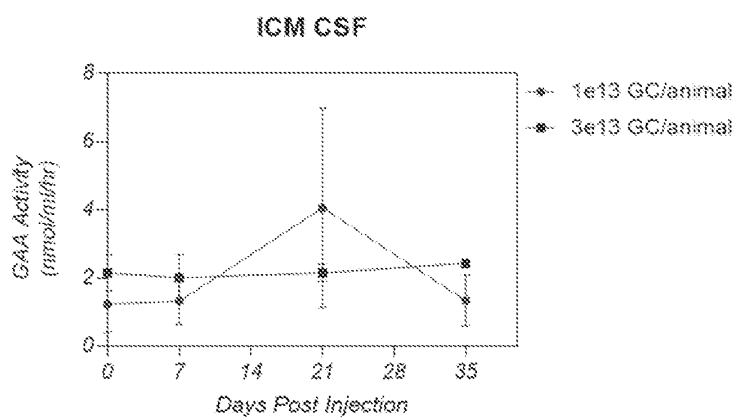
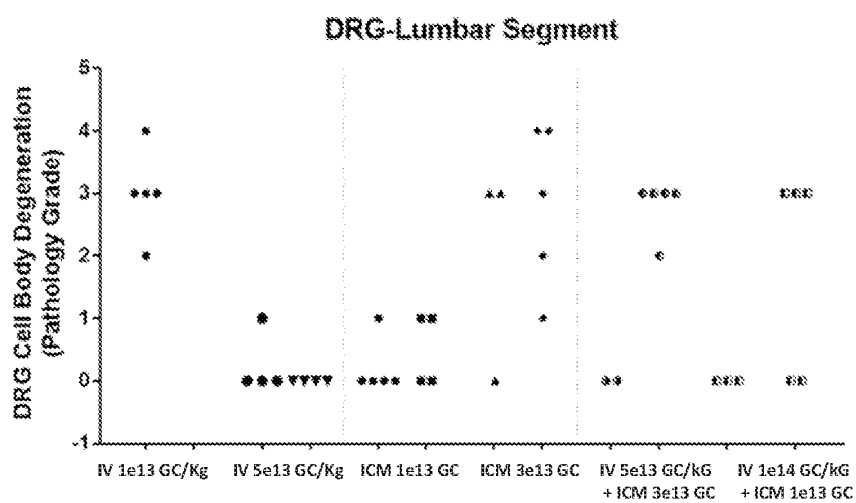
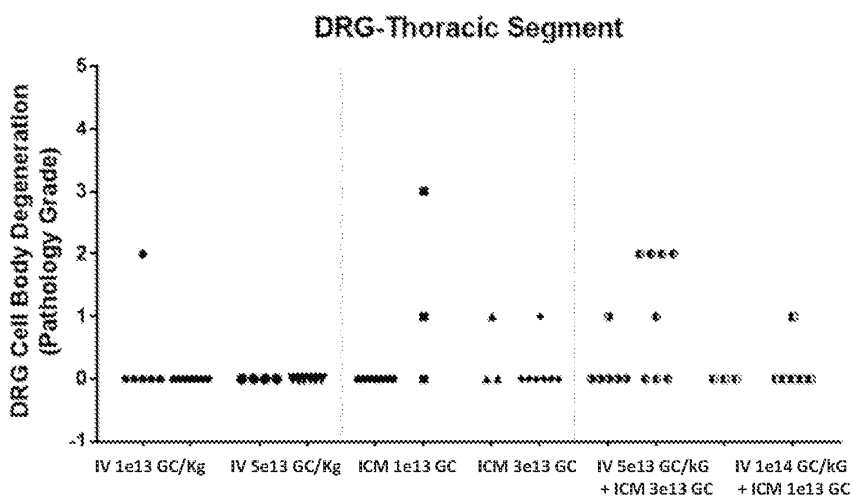
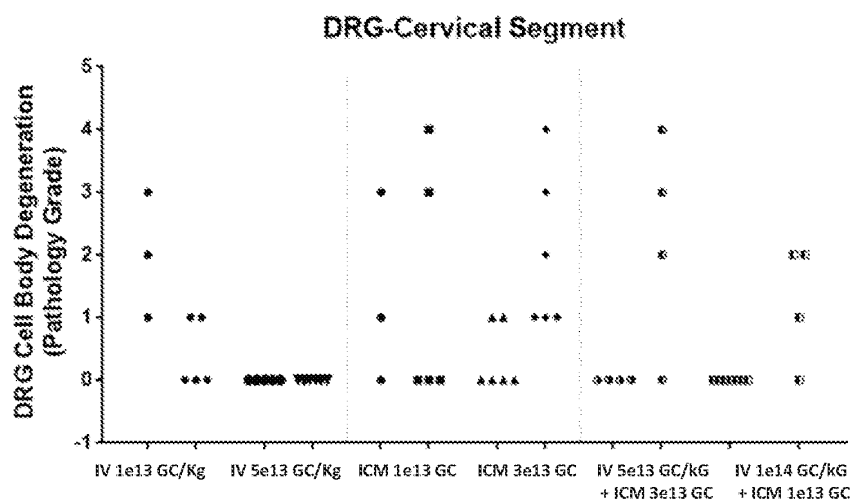


FIG. 32H





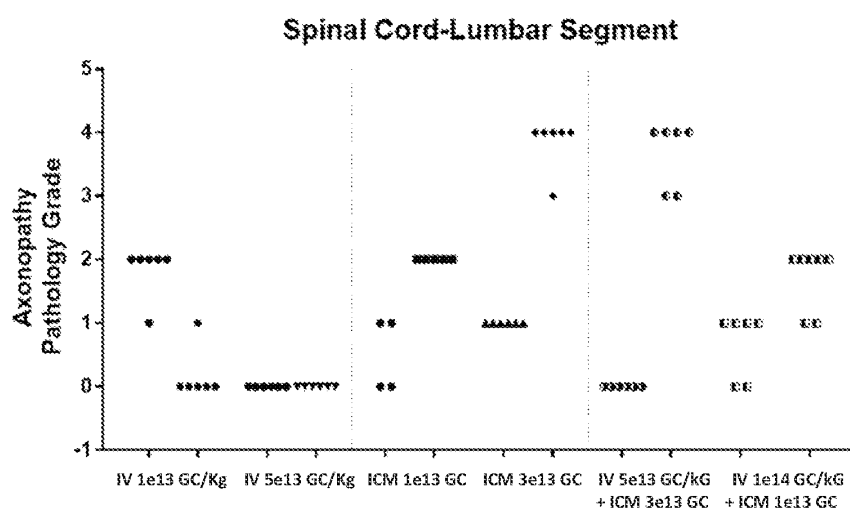
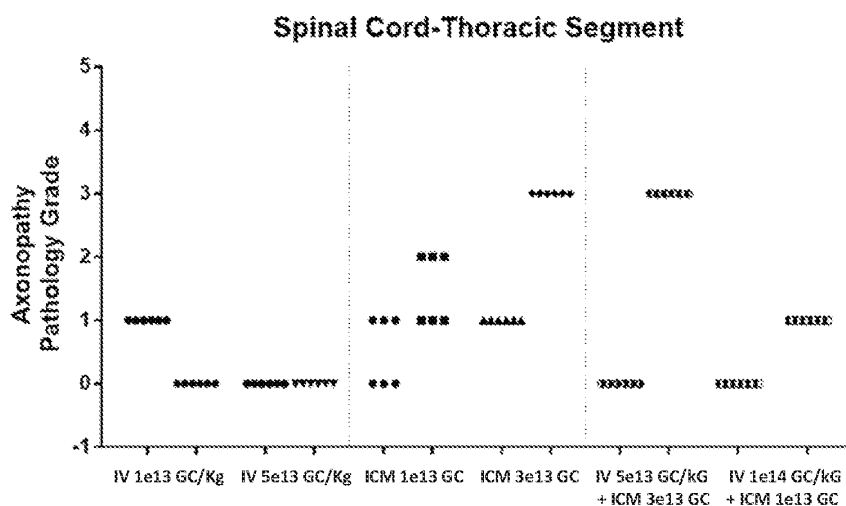
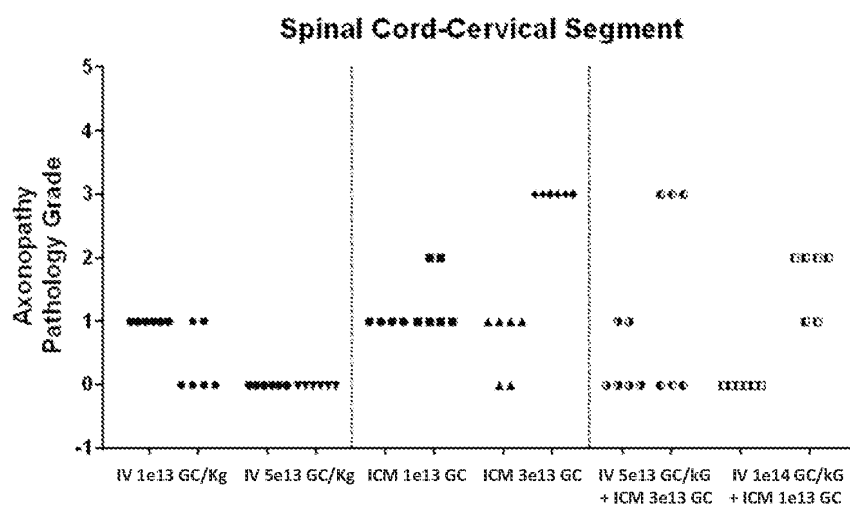
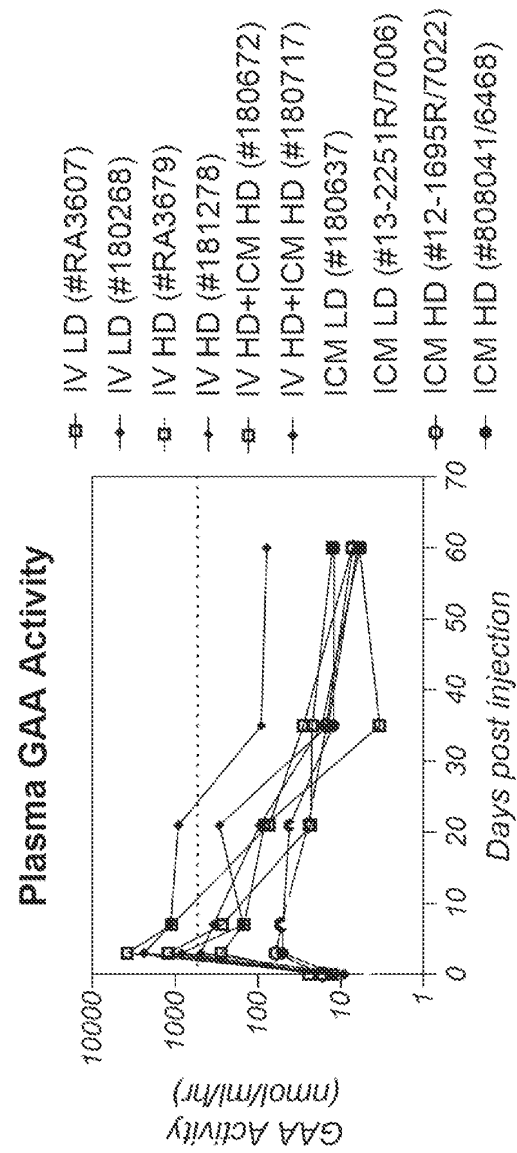
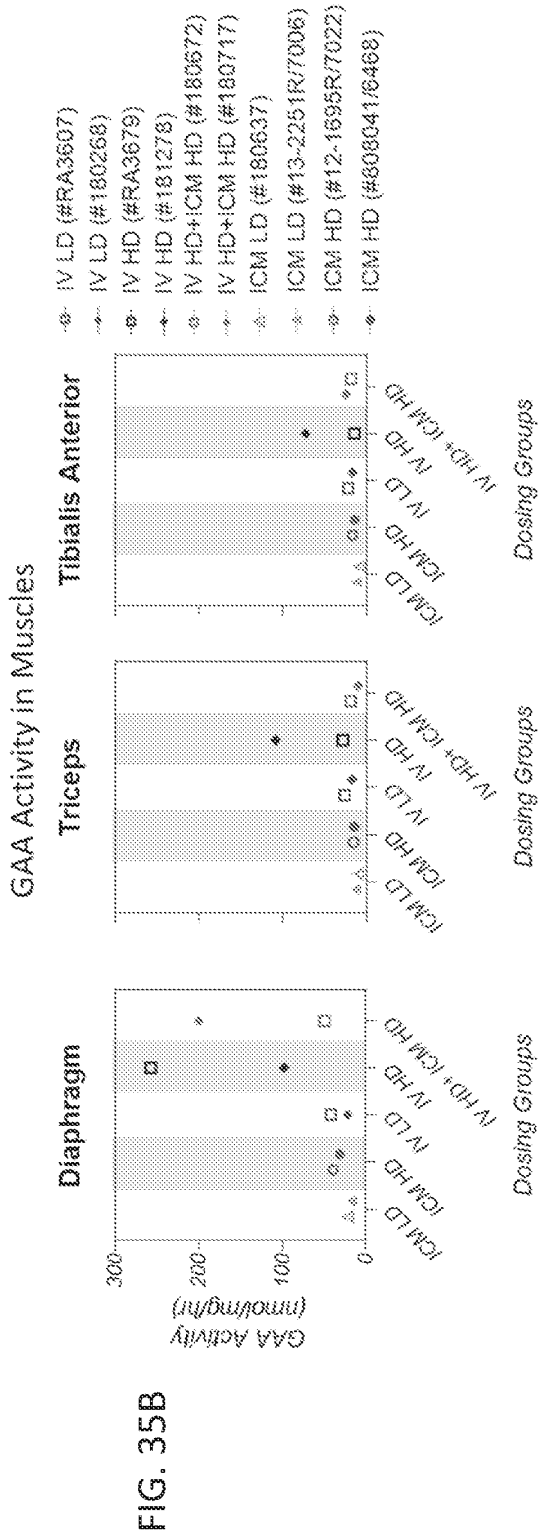
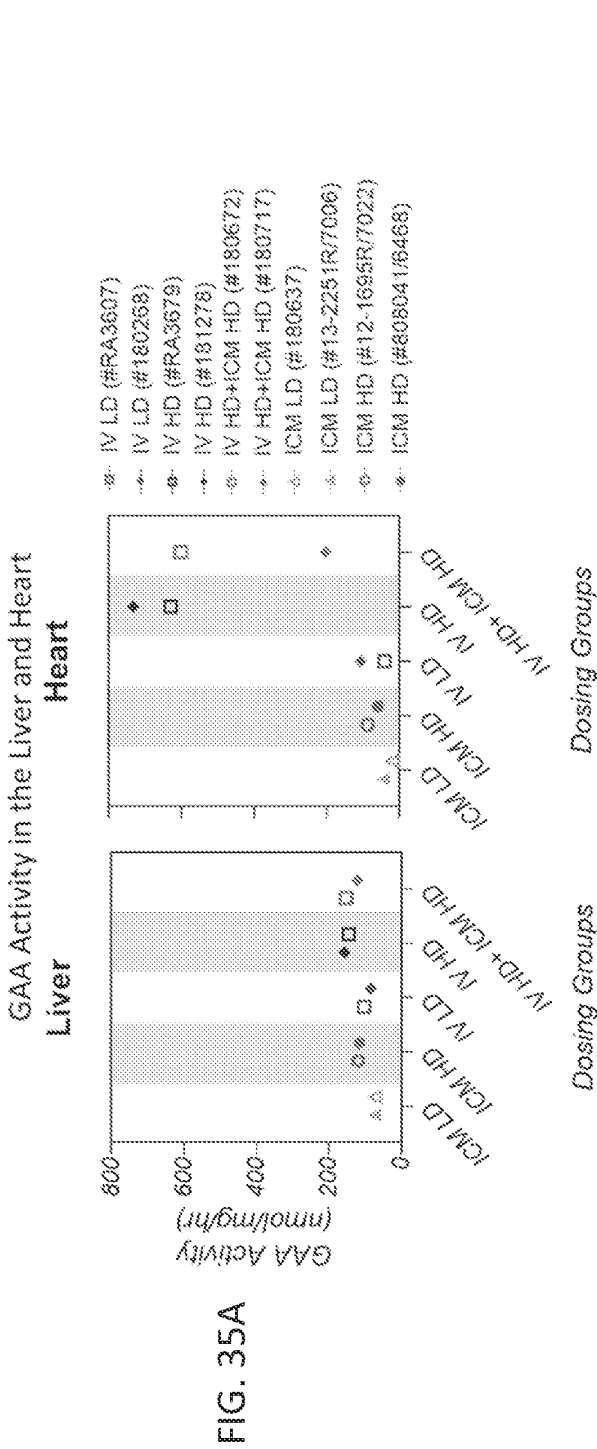


FIG. 34





GAA Activity in the Spinal Cord

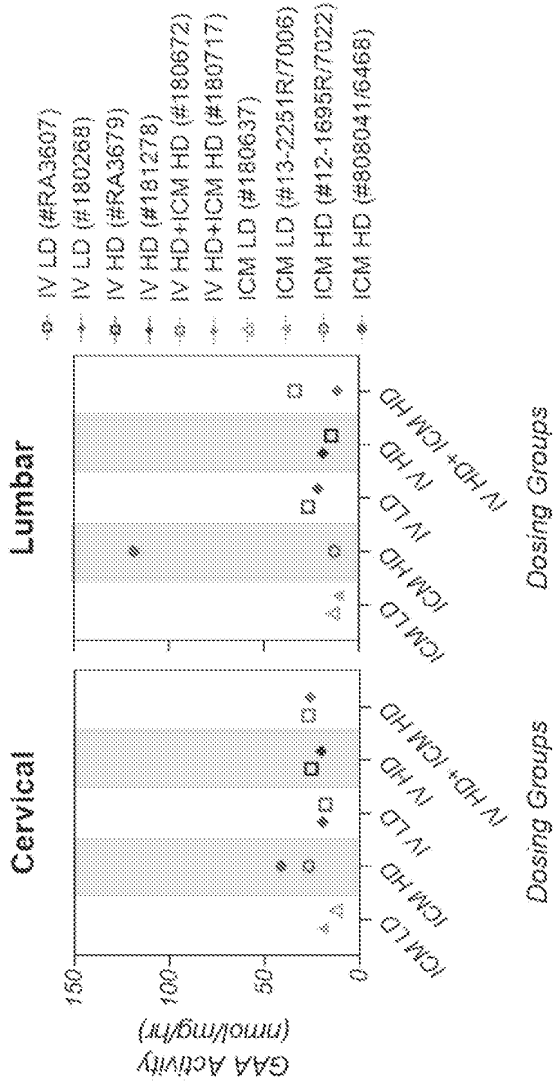


FIG. 35C

GAA Activity in the Brain

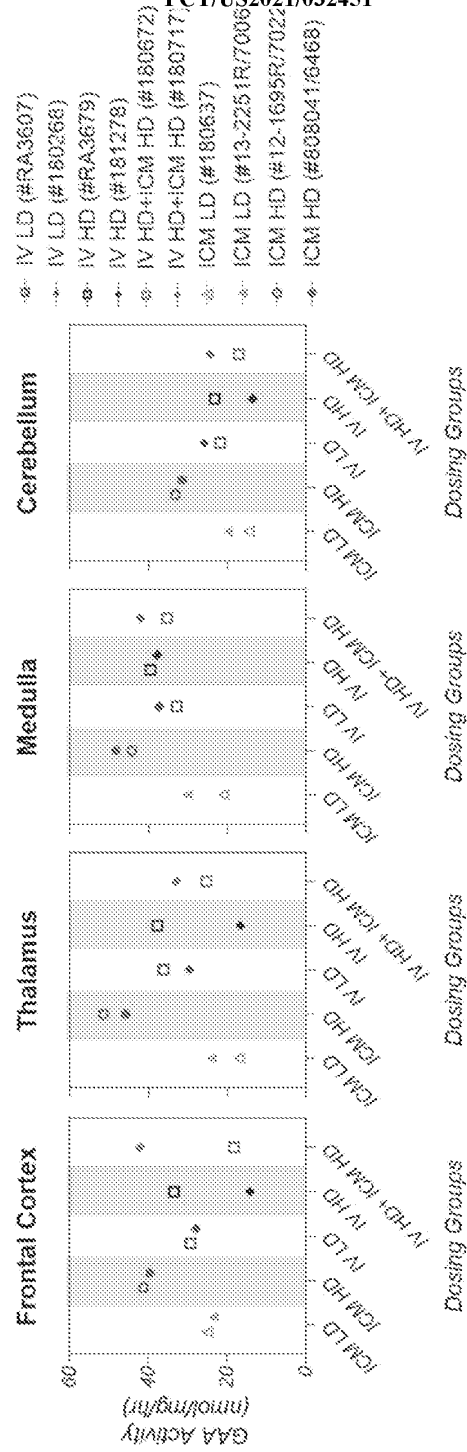


FIG. 35D

FIG. 36

Serial dilution from 1:50 to 1:51200

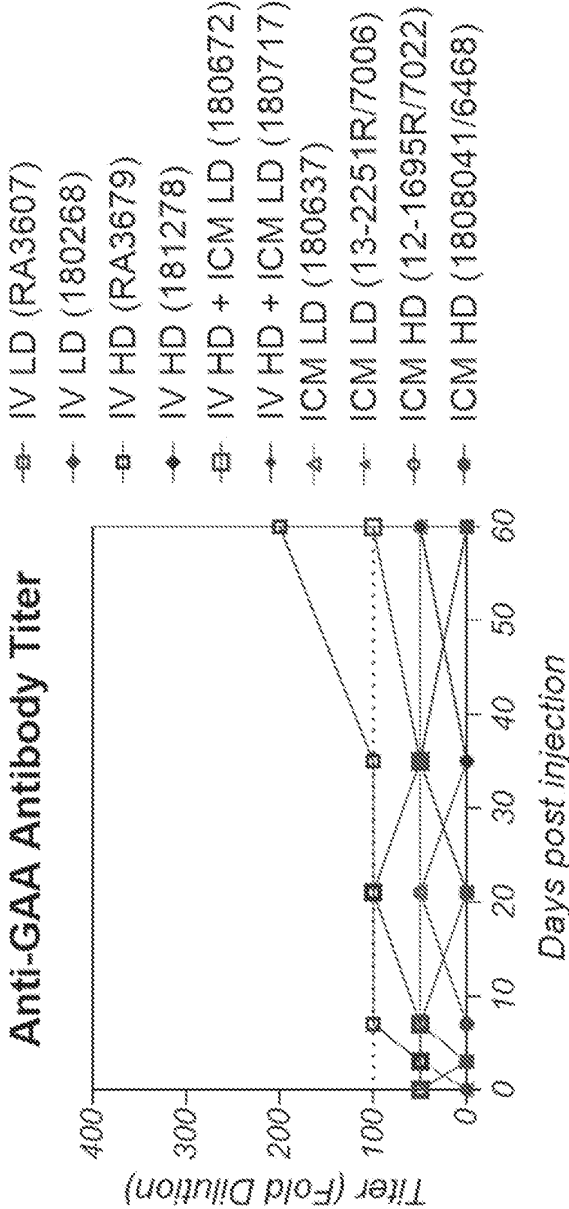
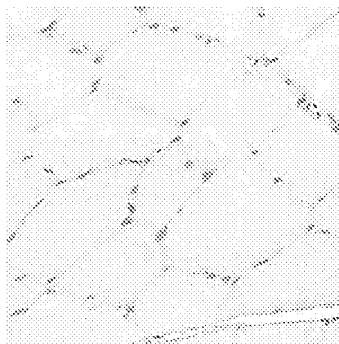


FIG. 37

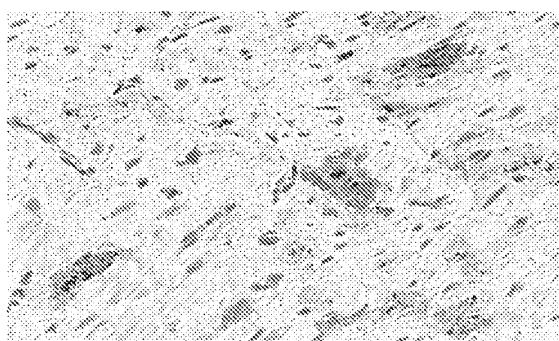
Quadriceps
Non injected NHP



Quadriceps
IV 1e13 GC/Kg



Heart
IV 1e13 GC/Kg



Spinal cord
ICM 1e13 GC

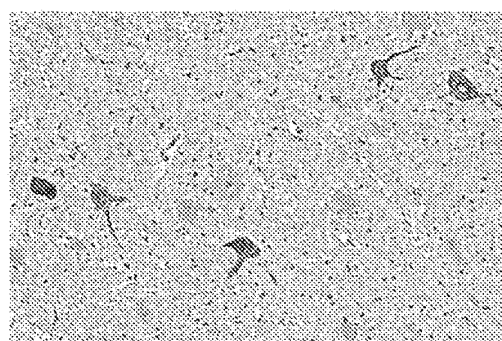


FIG. 38

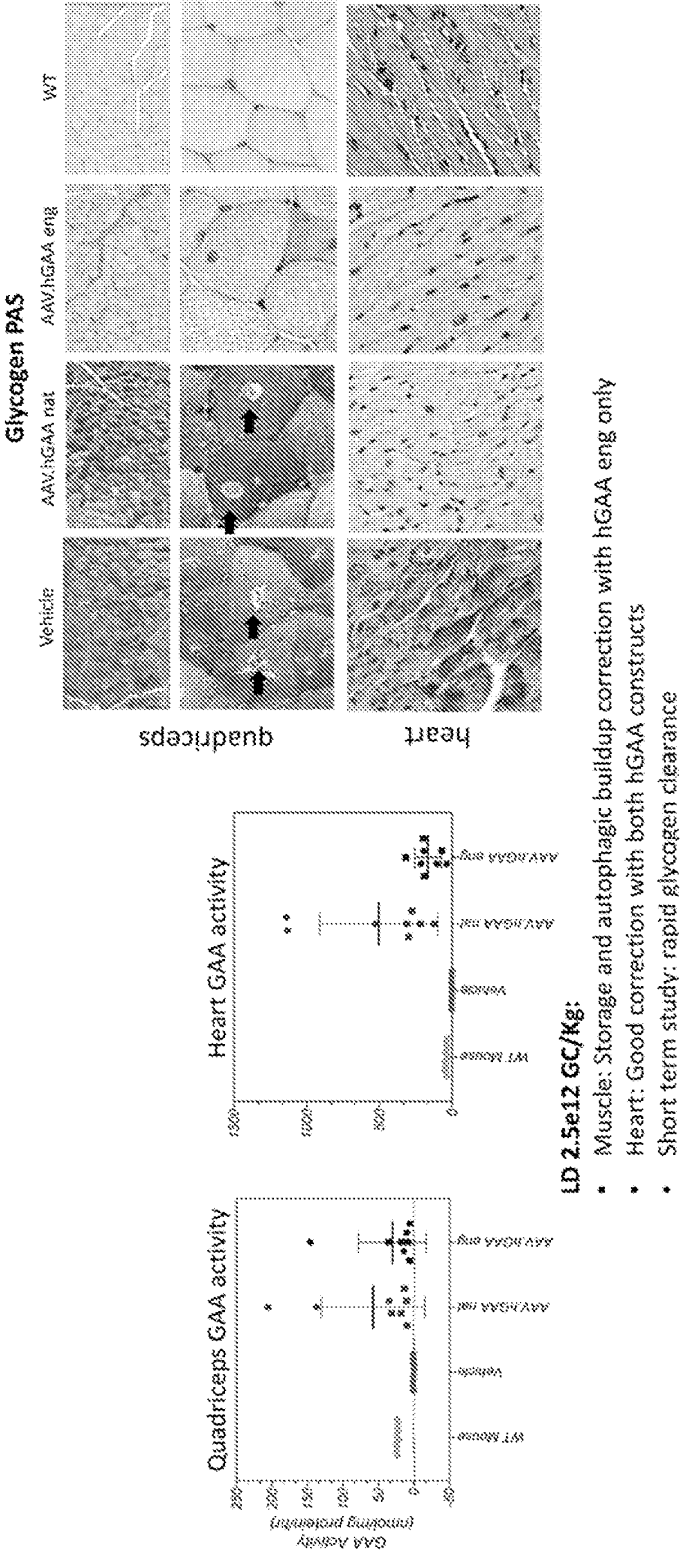
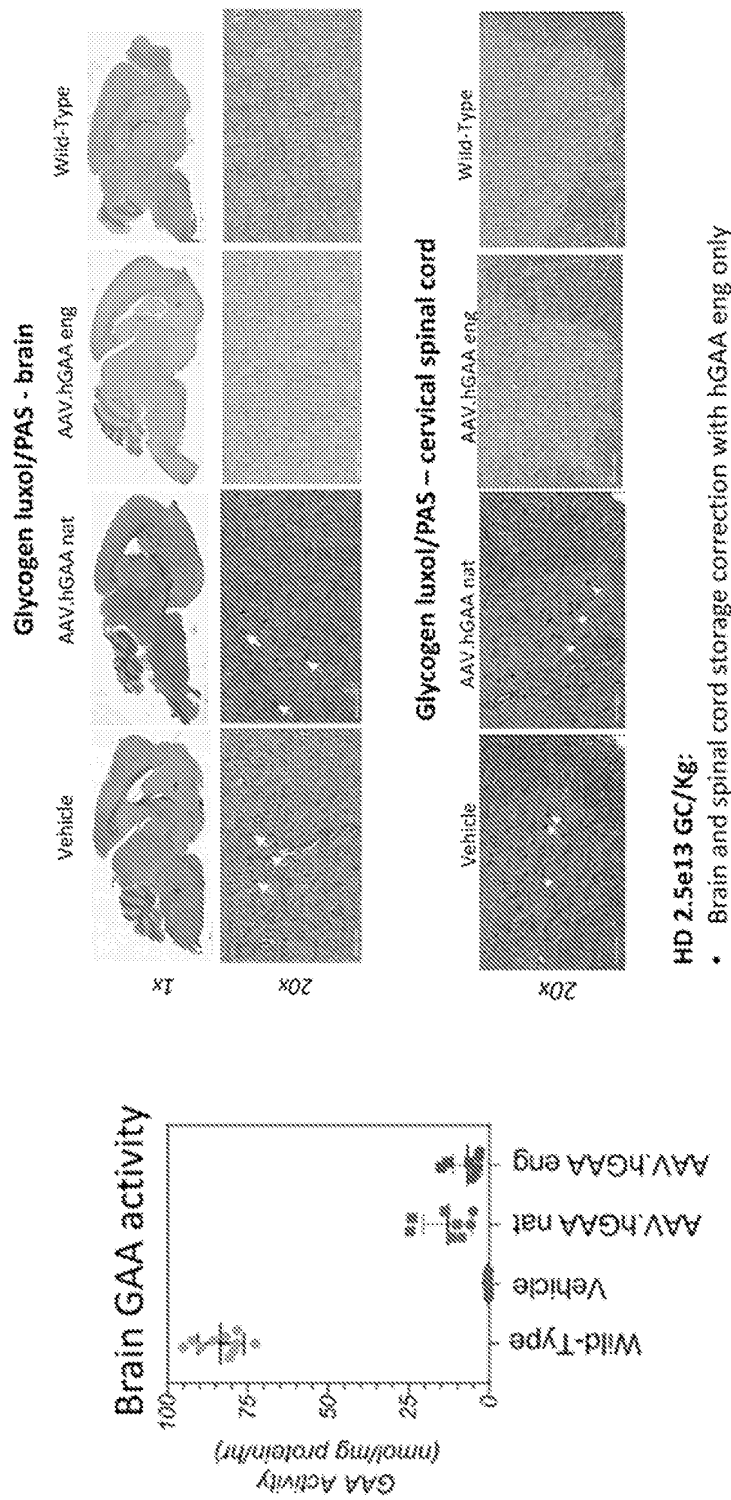


FIG. 39



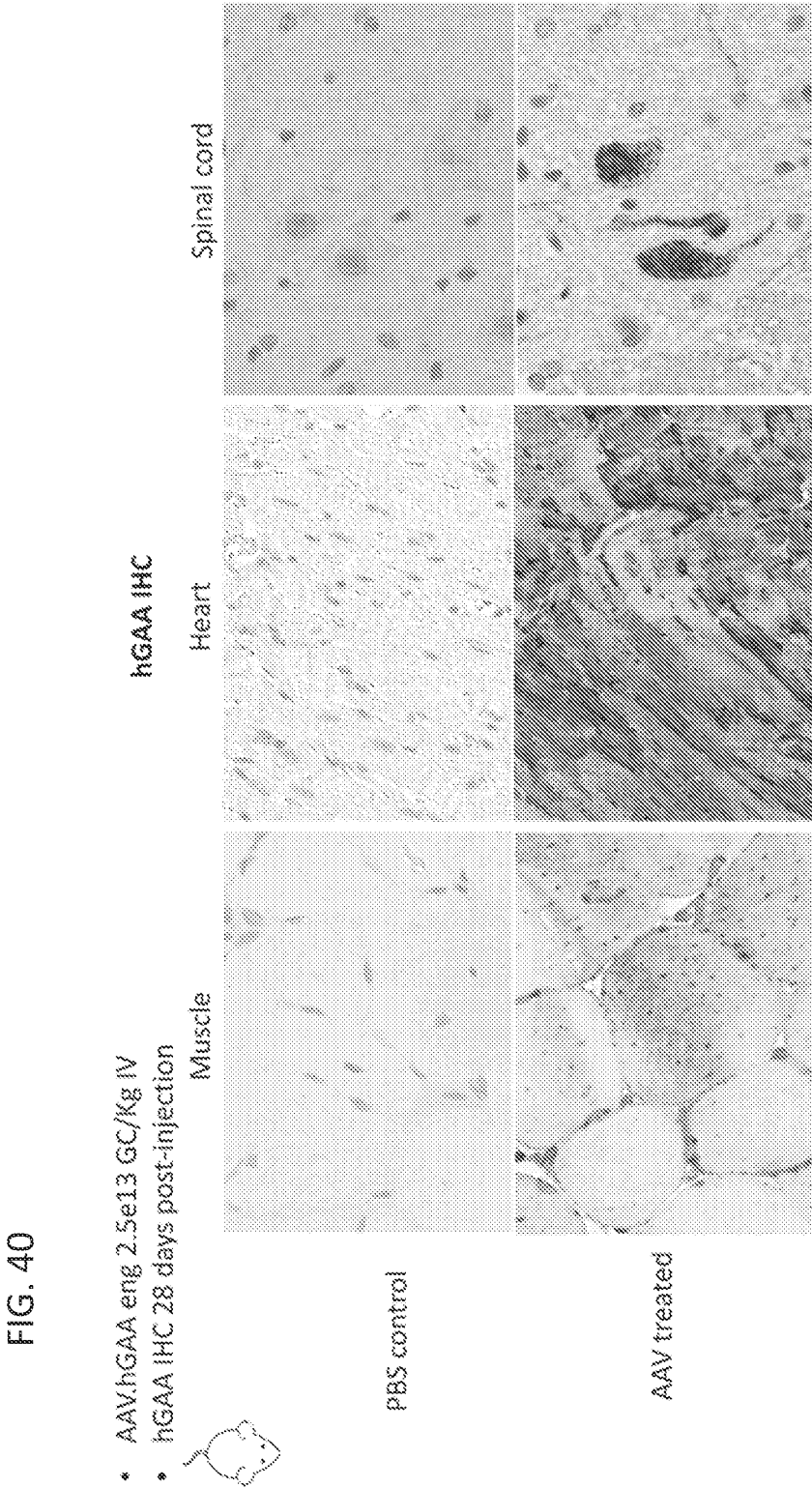


FIG. 41

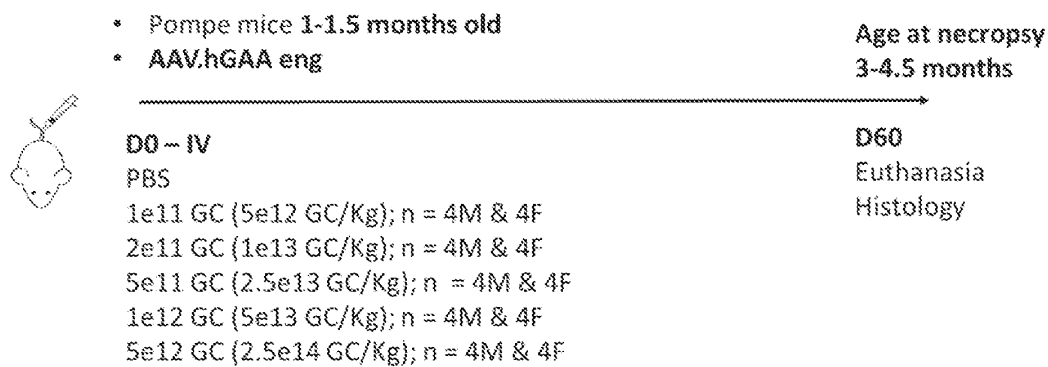
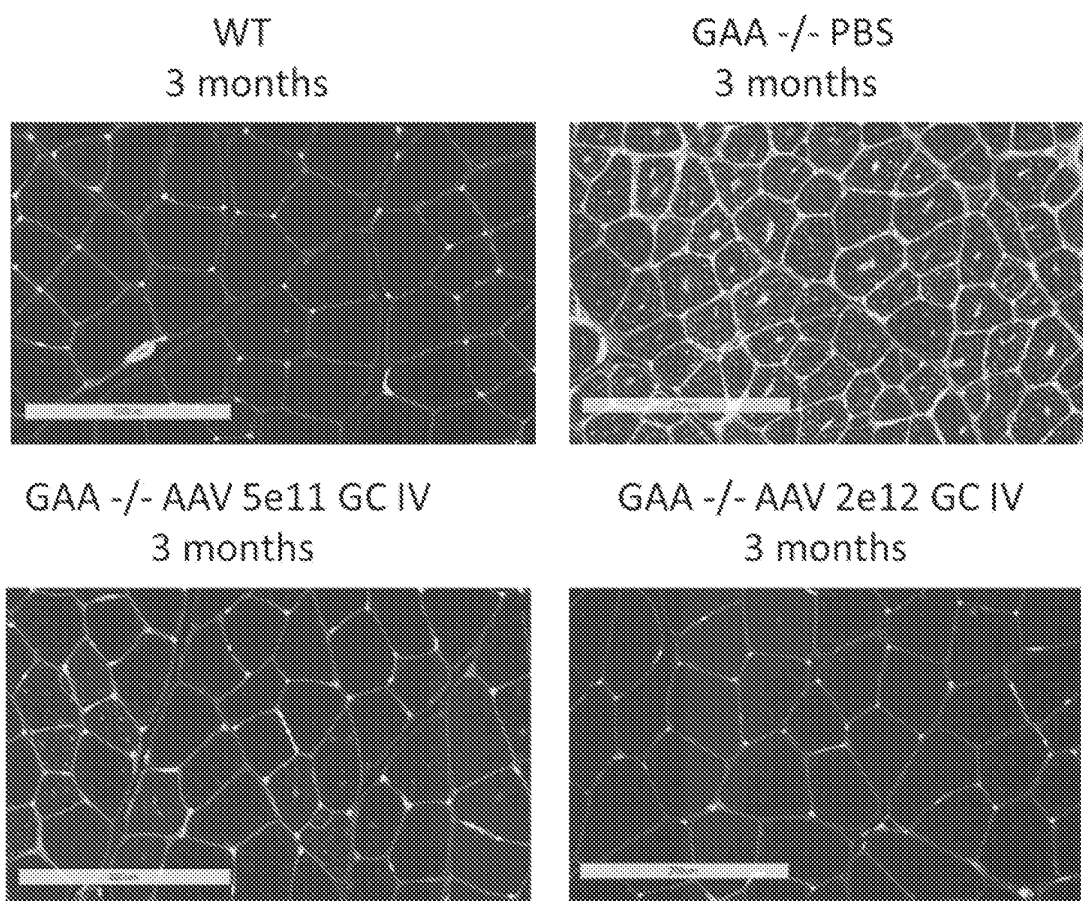


FIG. 42



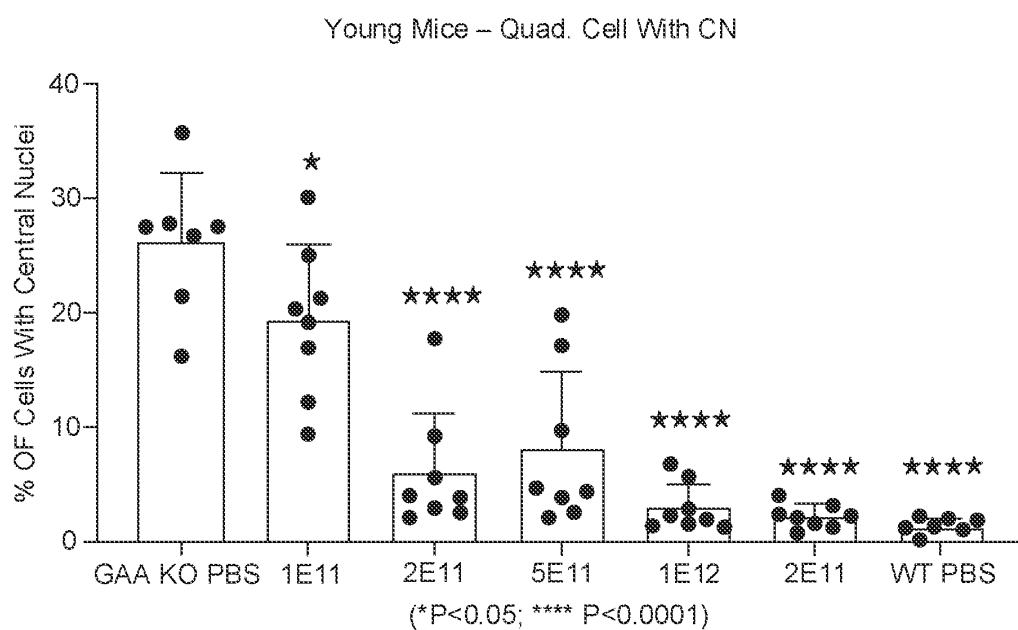


FIG.43

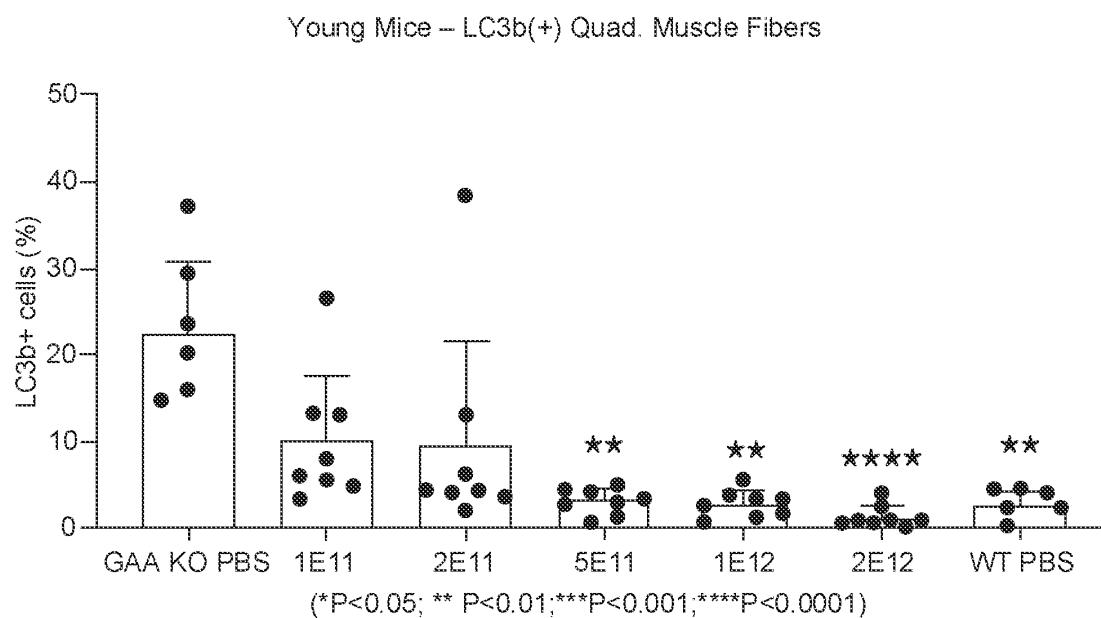


FIG.44

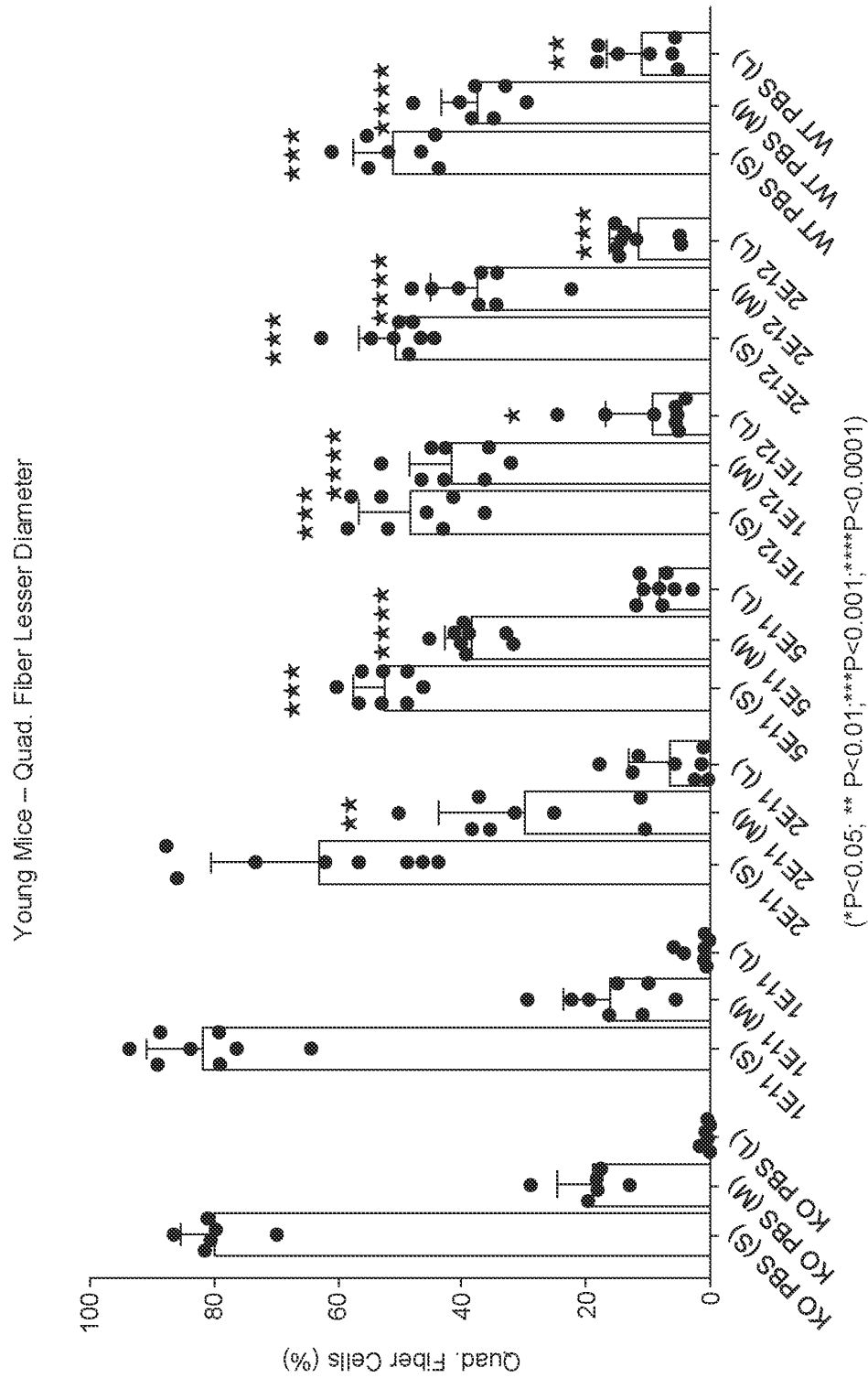


FIG.45

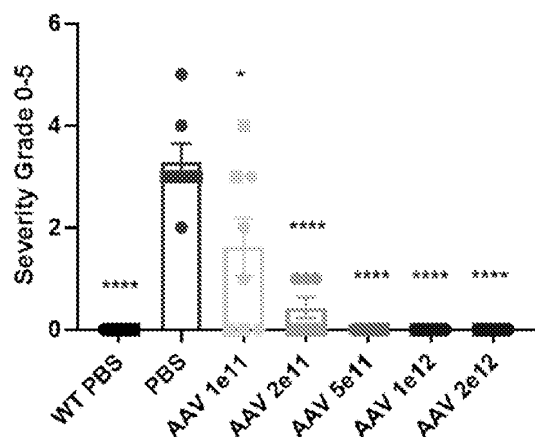
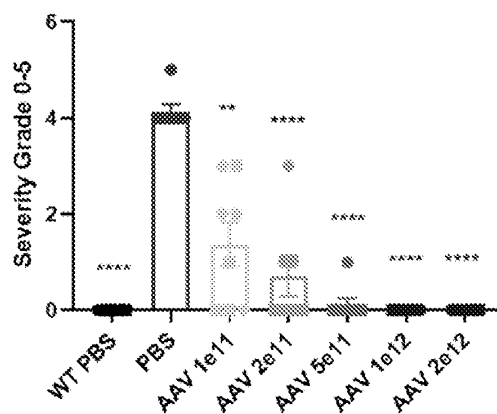
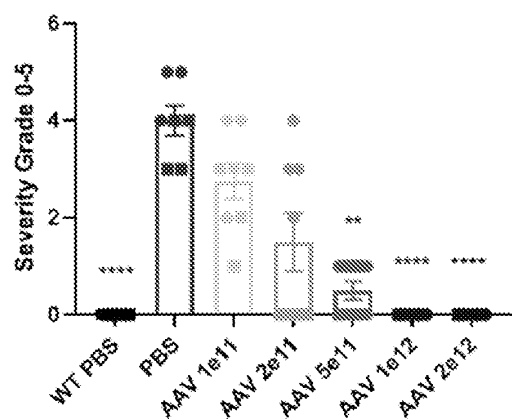
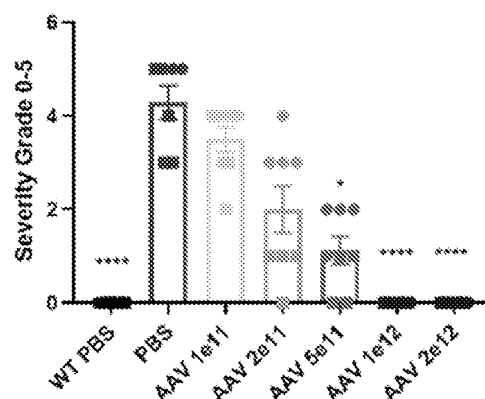
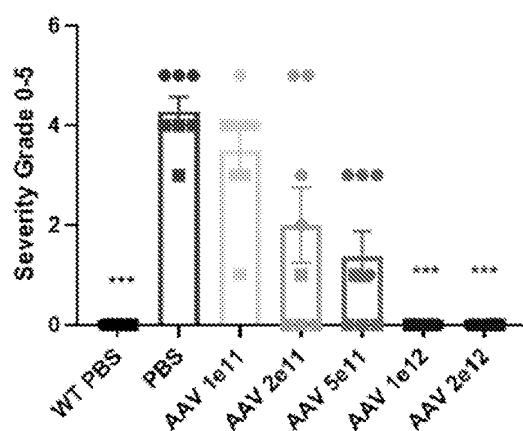
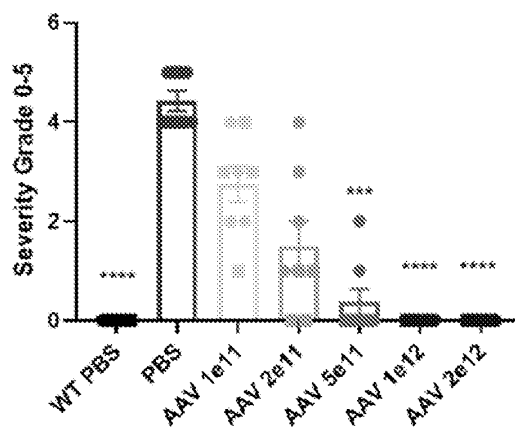
FIG. 46A
SoleusFIG. 46B
DiaphragmFIG. 46C
QuadricepsFIG. 46D
TricepsFIG. 46E
GastrocnemiusFIG. 46F
Tibialis anterior

FIG. 47

- Pompe mice 6-7 months old
- AAV.hGAA eng

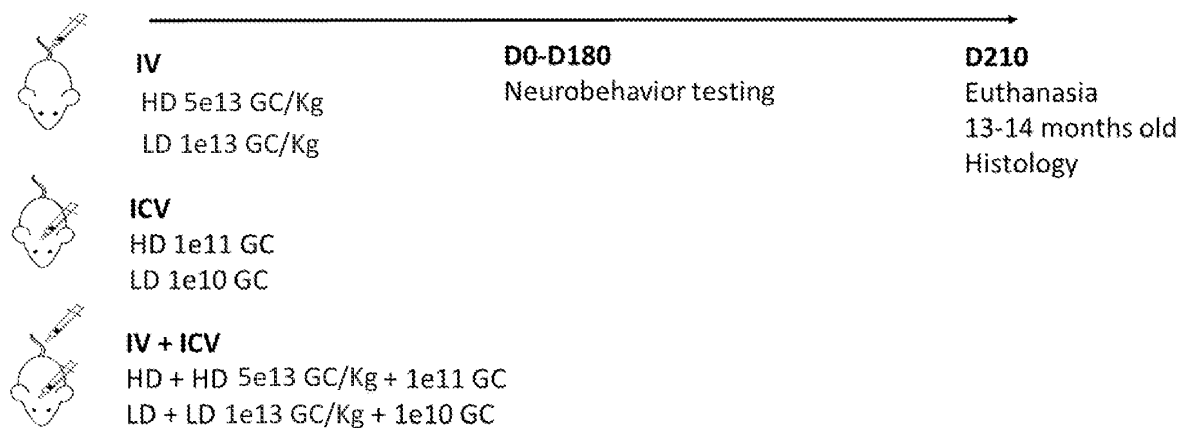
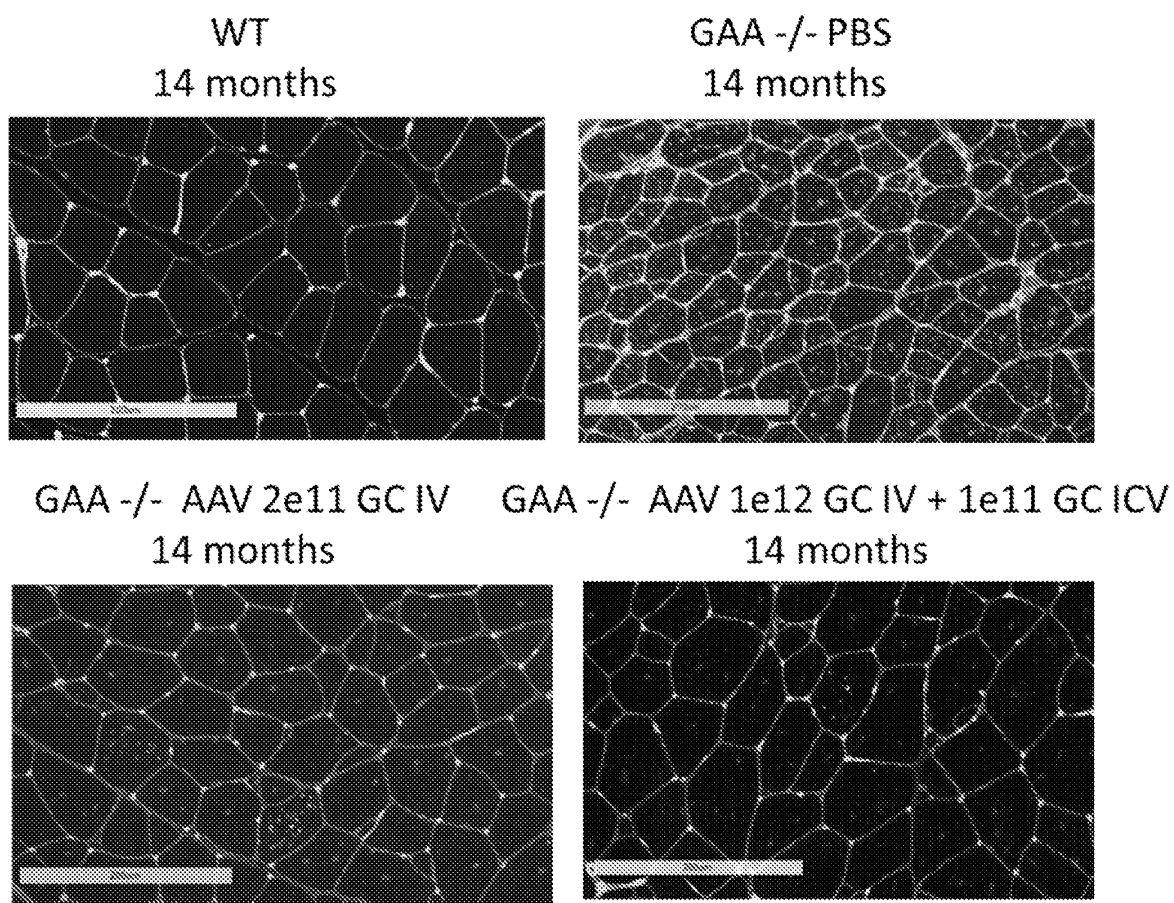
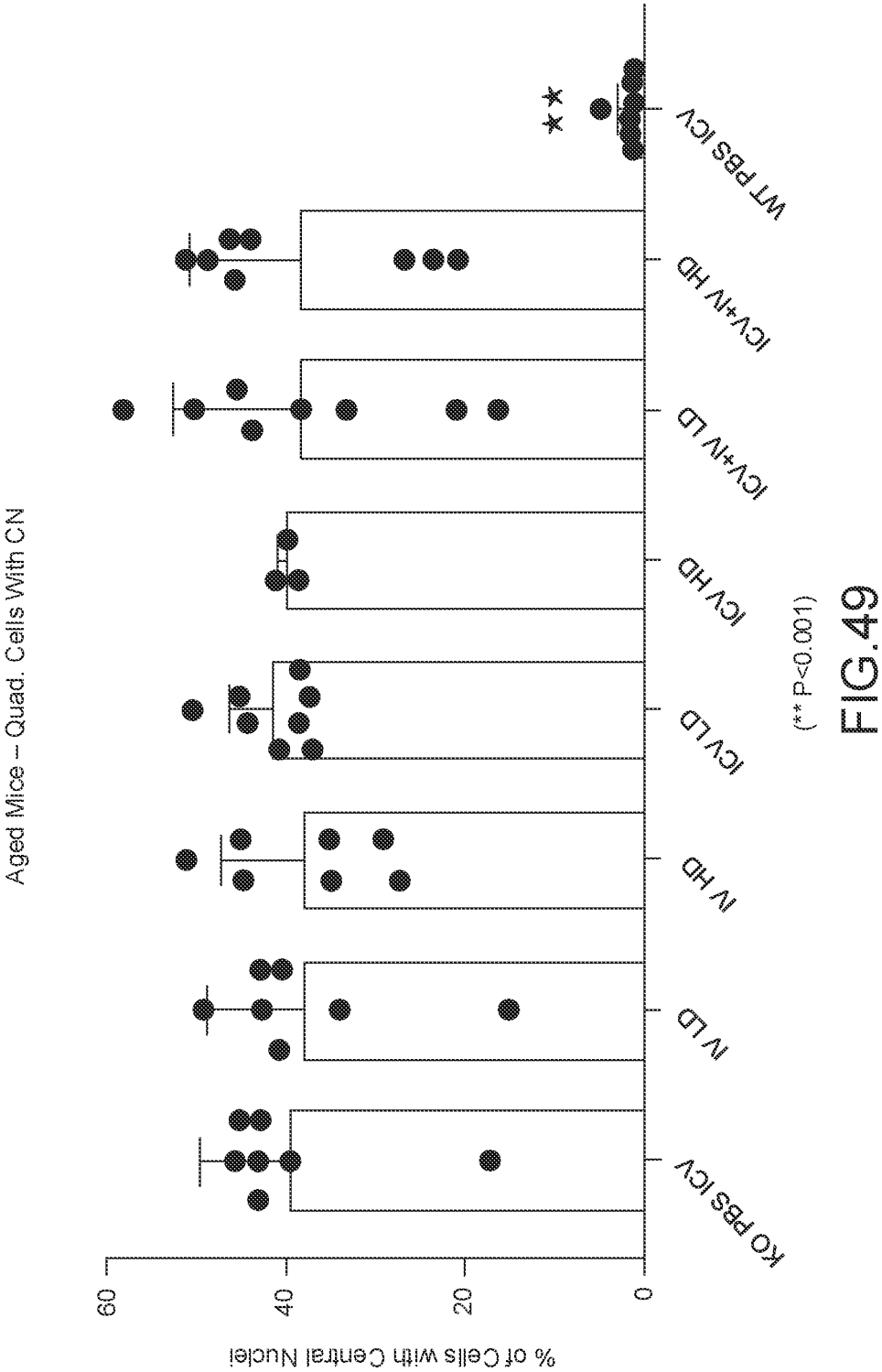


FIG. 48





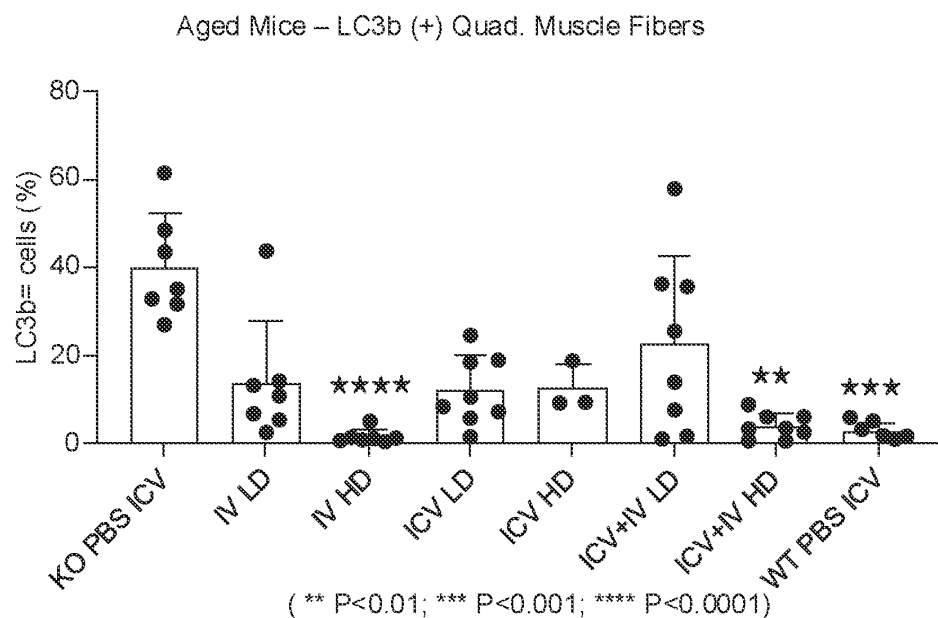


FIG.50

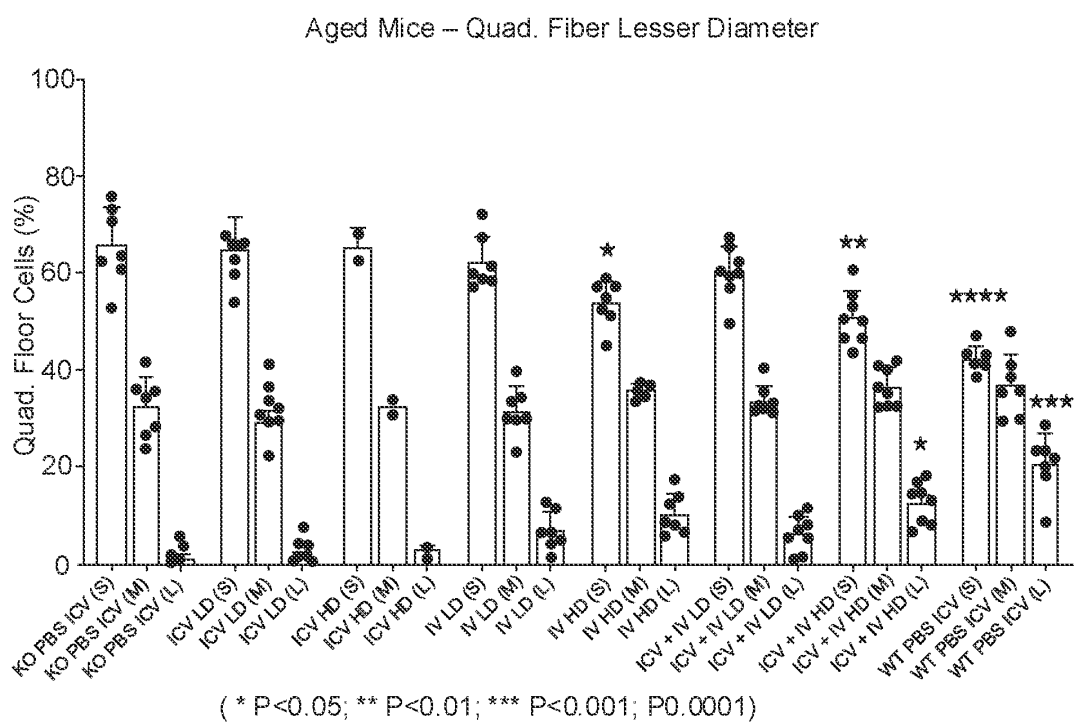


FIG.51

FIG. 52

