

FIG. 1

```

1                                     50
AAV9 (1) MAADGYLPDWLEDNLSEGIREWWALKPGAPQPKANQQHQDNARGLVLPGY
AAVhu68(1) MAADGYLPDWLEDNLSEGIREWWALKPGAPQPKANQQHQDNARGLVLPGY
51                                     100
AAV9 (51) KYLGPGNGLDKGEPVNAADAAALEHDKAYDQQLKAGDNPYLKYNHADA EF
AAVhu68 (51) KYLGPGNGLDKGEPVNEADAAALEHDKAYDQQLKAGDNPYLKYNHADA EF
101                                     150
AAV9 (101) QERLKEDTSFGGNLGRAVFQAKKRLLLEPLGLVEEAAKTAPGKKRPVEQSP
AAVhu68 (101) QERLKEDTSFGGNLGRAVFQAKKRLLLEPLGLVEEAAKTAPGKKRPVEQSP
151                                     200
AAV9 (151) QEPDSSAGIGKSGAQPAKKRLNFGQTGDTESVPDPQPIGEPPAAPSGVGS
AAVhu68 (151) QEPDSSVGIGKSGAQPAKKRLNFGQTGDTESVPDPQPIGEPPAAPSGVGS
201                                     250
AAV9 (201) LTMASGGGAPVADNNEGADGVGSSSGNWHCD SQWL GDRVITTSTRTWALP
AAVhu68 (201) LTMASGGGAPVADNNEGADGVGSSSGNWHCD SQWL GDRVITTSTRTWALP
251                                     300
AAV9 (251) TYNNHLYKQISNSTSGGSSNDNAYFGYSTPWGYFDNRFHCHFS PRDWQR
AAVhu68 (251) TYNNHLYKQISNSTSGGSSNDNAYFGYSTPWGYFDNRFHCHFS PRDWQR
301                                     350
AAV9 (301) LINNNWGF RPKRLNFKLFNIQVKEVTDNNGVKTIANNLTSTVQVFTDSY
AAVhu68 (301) LINNNWGF RPKRLNFKLFNIQVKEVTDNNGVKTIANNLTSTVQVFTDSY
351                                     400
AAV9 (351) QLPYVLGSAHEGCLPPFPADVFMIPQYGYLTLNDGSQAVGRSSFYCLEYF
AAVhu68 (351) QLPYVLGSAHEGCLPPFPADVFMIPQYGYLTLNDGSQAVGRSSFYCLEYF
401                                     450
AAV9 (401) PSQMLRTGN NFQFSYEFENVPFHSSYAH SQSLDRLMNPLIDQYLYLSKT
AAVhu68 (401) PSQMLRTGN NFQFSYEFENVPFHSSYAH SQSLDRLMNPLIDQYLYLSKT
451                                     500
AAV9 (451) INGSQNQQTLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSE
AAVhu68 (451) INGSQNQQTLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSE
501                                     550
AAV9 (501) FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGR
AAVhu68 (501) FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGR
551                                     600
AAV9 (551) DNVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQAQTGWVQNQG
AAVhu68 (551) DNVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQAQTGWVQNQG
601                                     650
AAV9 (601) ILPGMVWQDRDVYLQGPIWAKIPHTDGNFHPSPLMGGFGMKHPPPPQILIK
AAVhu68 (601) ILPGMVWQDRDVYLQGPIWAKIPHTDGNFHPSPLMGGFGMKHPPPPQILIK
651                                     700
AAV9 (651) NTPVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENS KRWNPEIQ
AAVhu68 (651) NTPVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENS KRWNPEIQ
701                                     736
AAV9 (701) YTSNYYKSNNVEFAVNTEGVYSEPRPIGTRYLTRNL
AAVhu68 (701) YTSNYYKSNNVEFAVNTEGVYSEPRPIGTRYLTRNL

```

FIG. 2

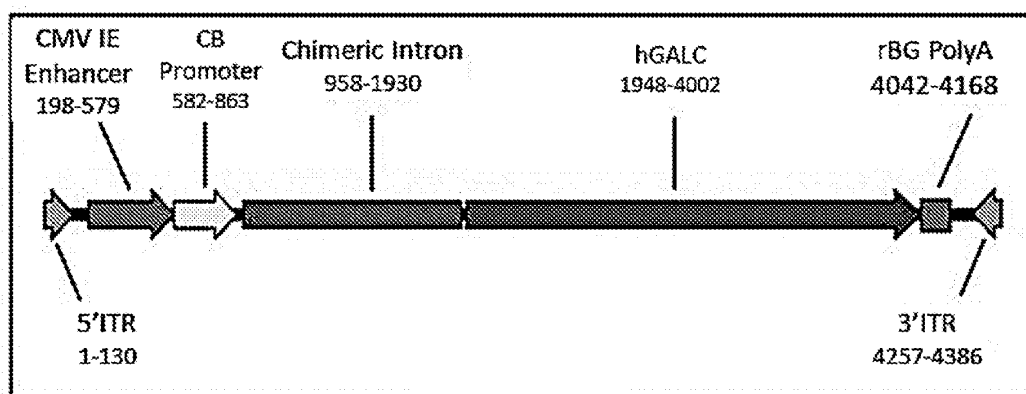
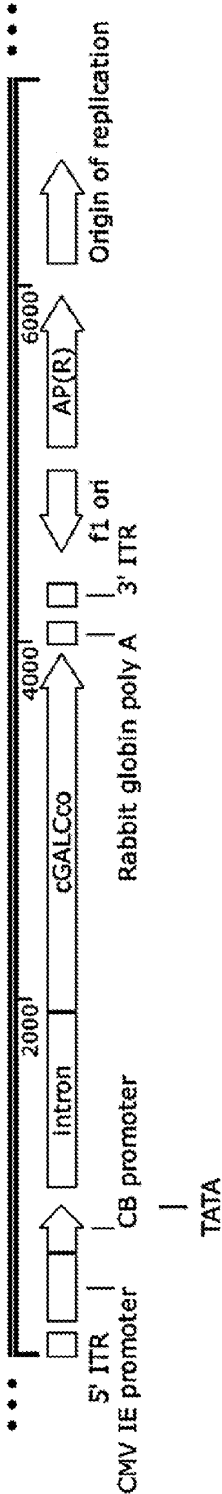


FIG. 3



pENN.AAV.CB7.CI.RBG (p1044) Q

FIG. 4

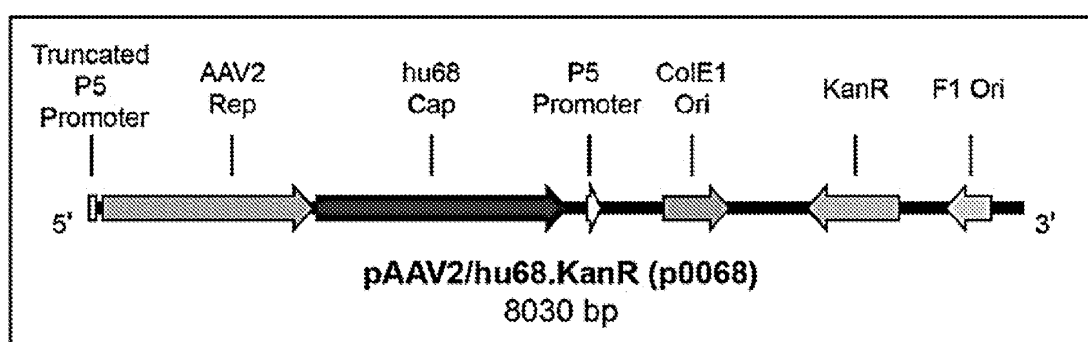


FIG. 5A

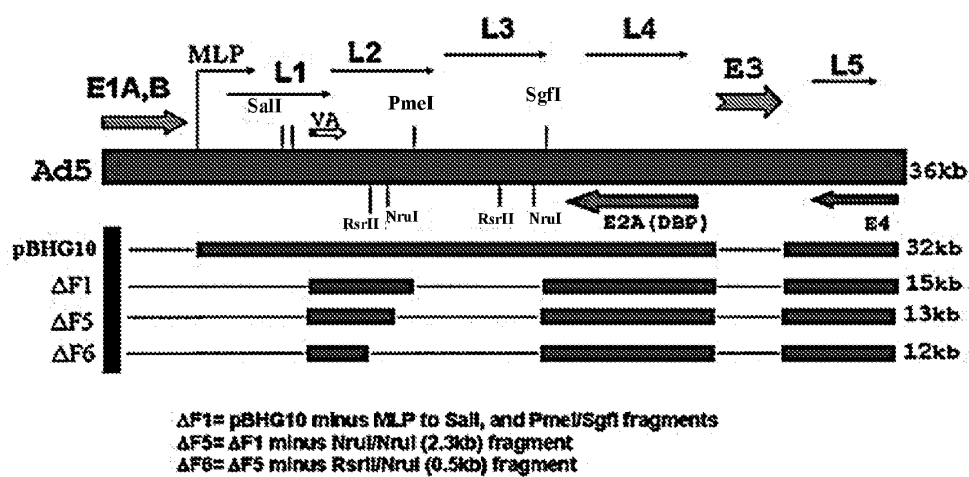


FIG. 5B

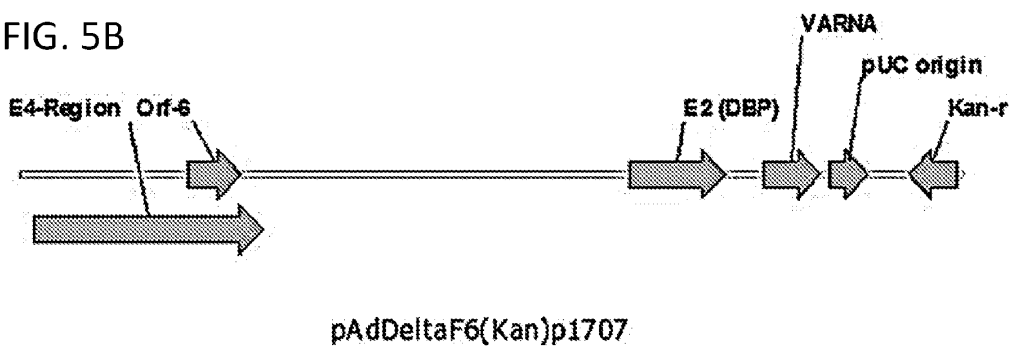


FIG. 6A

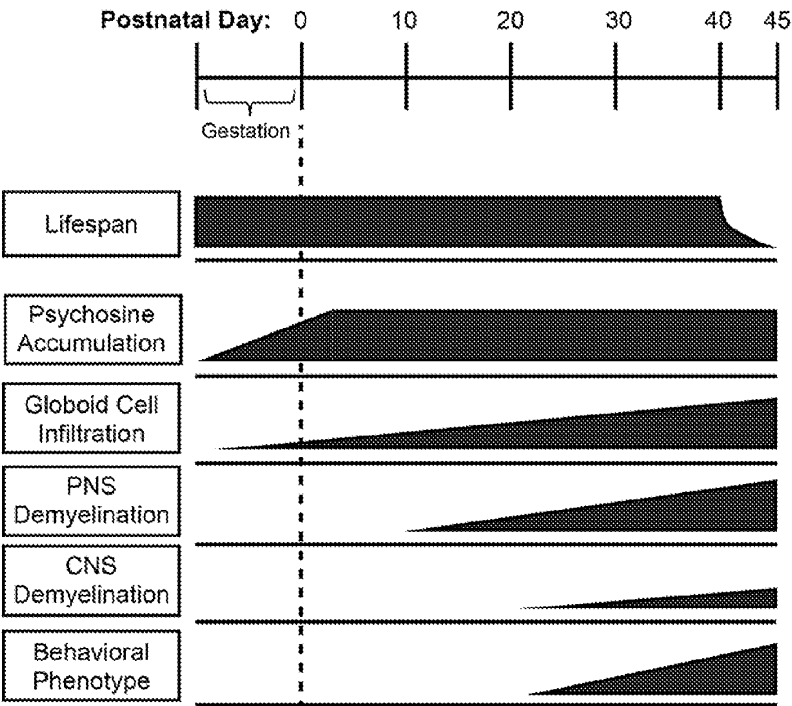


FIG. 6B

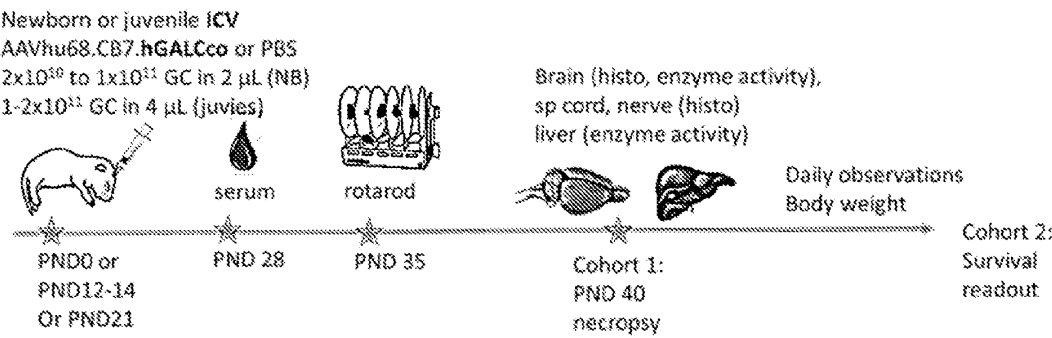


FIG. 7

Survival

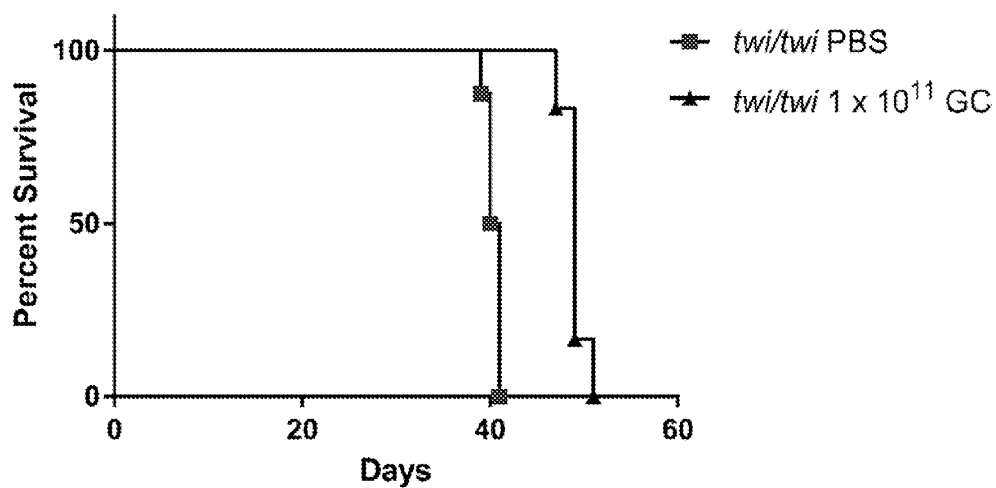


FIG. 8

Brain GALC Activity

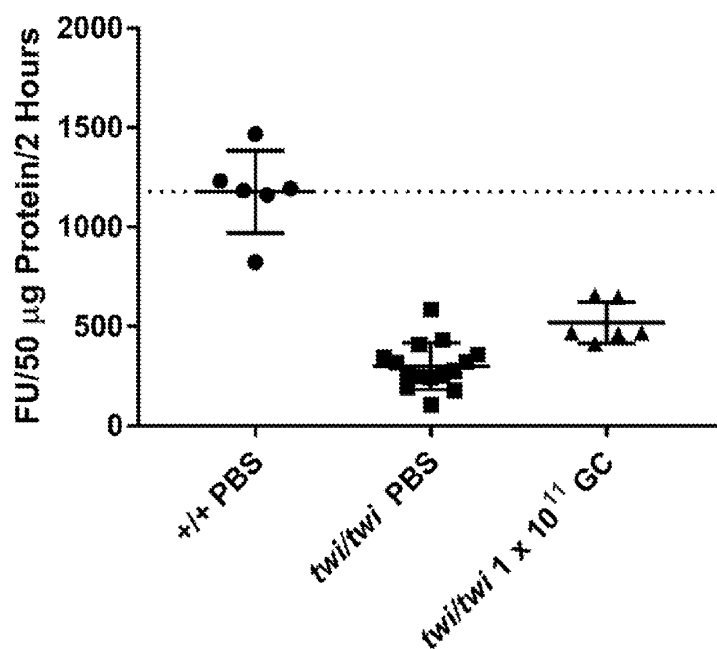


FIG. 9

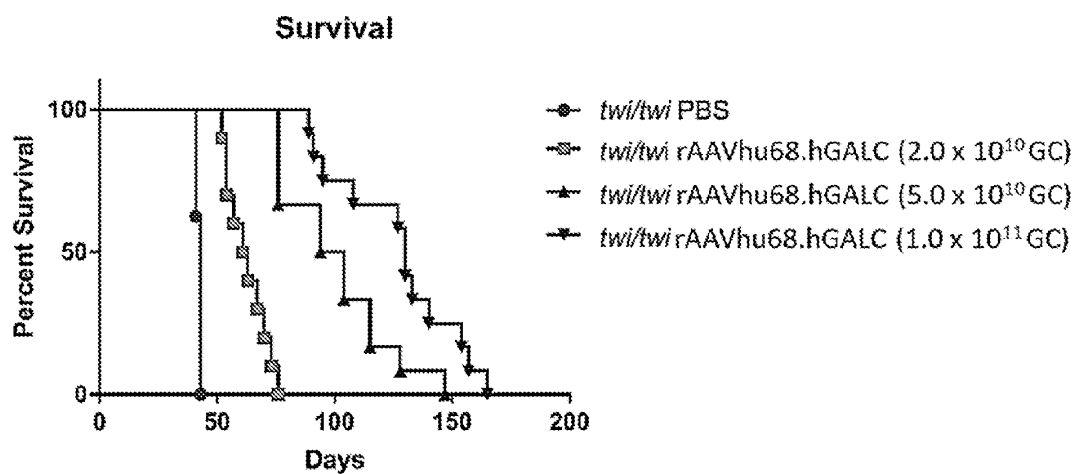


FIG. 10

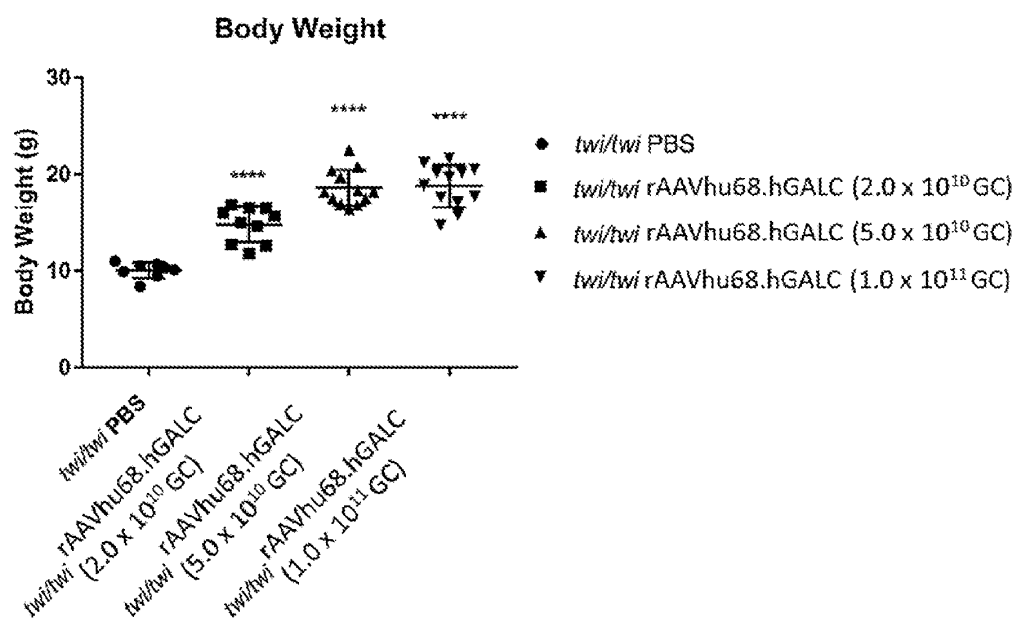


FIG. 11

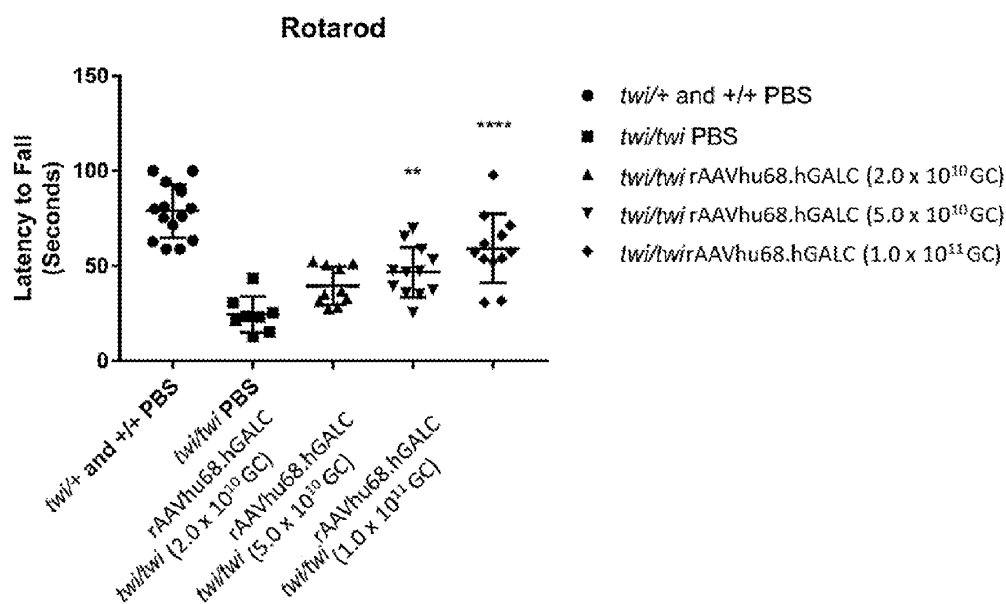


FIG. 12

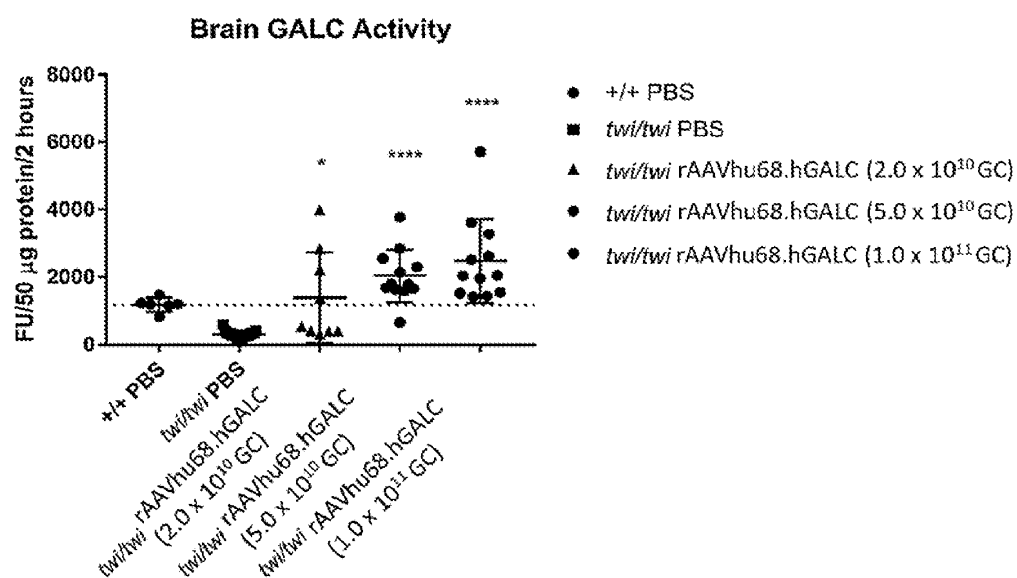


FIG. 13

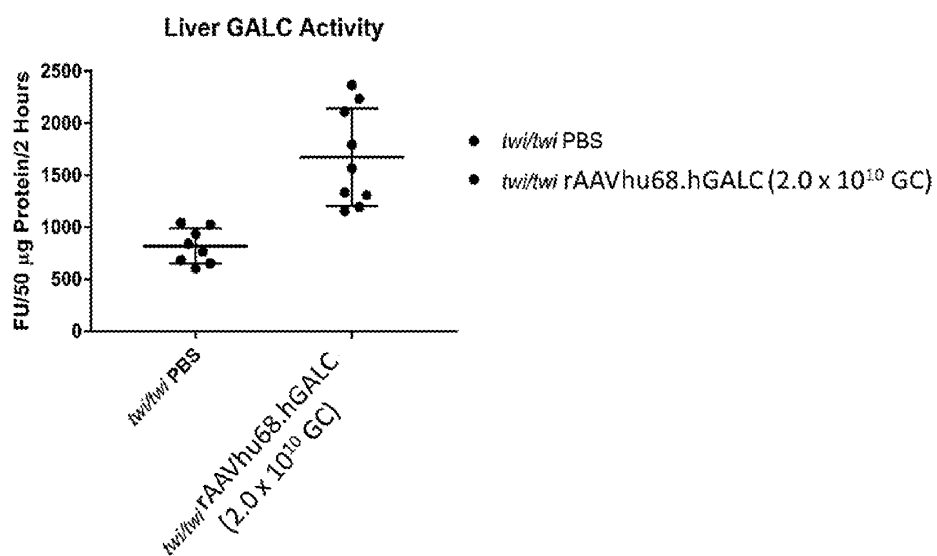


FIG. 14

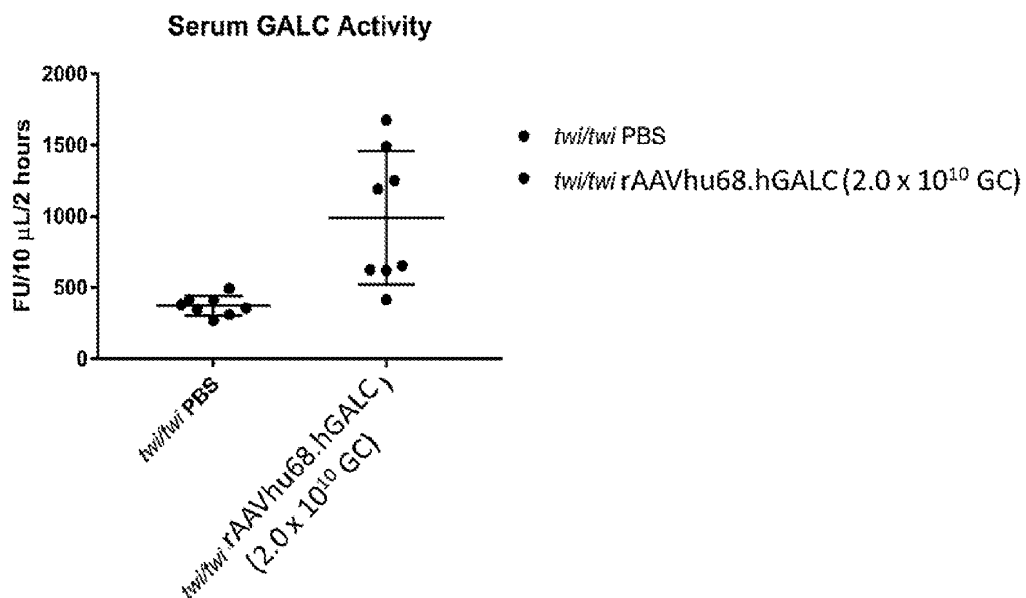
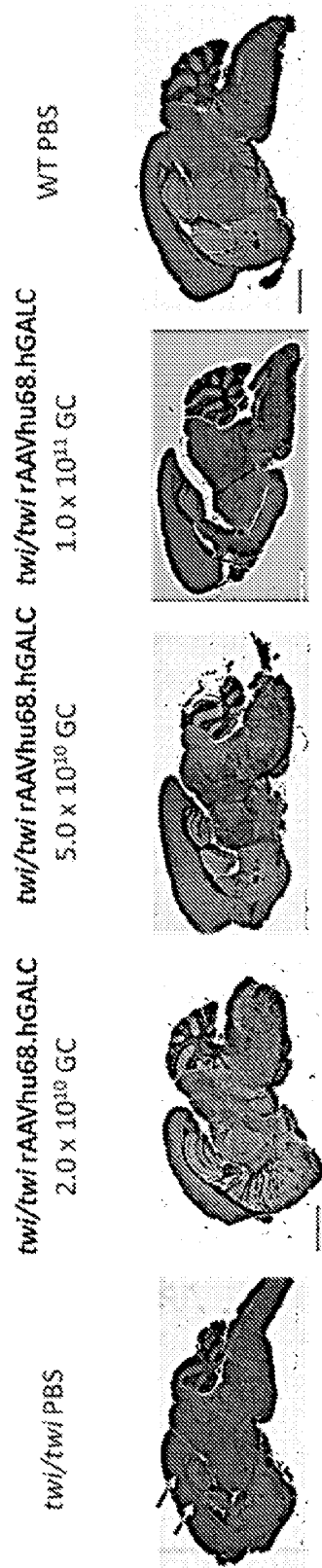


FIG. 15



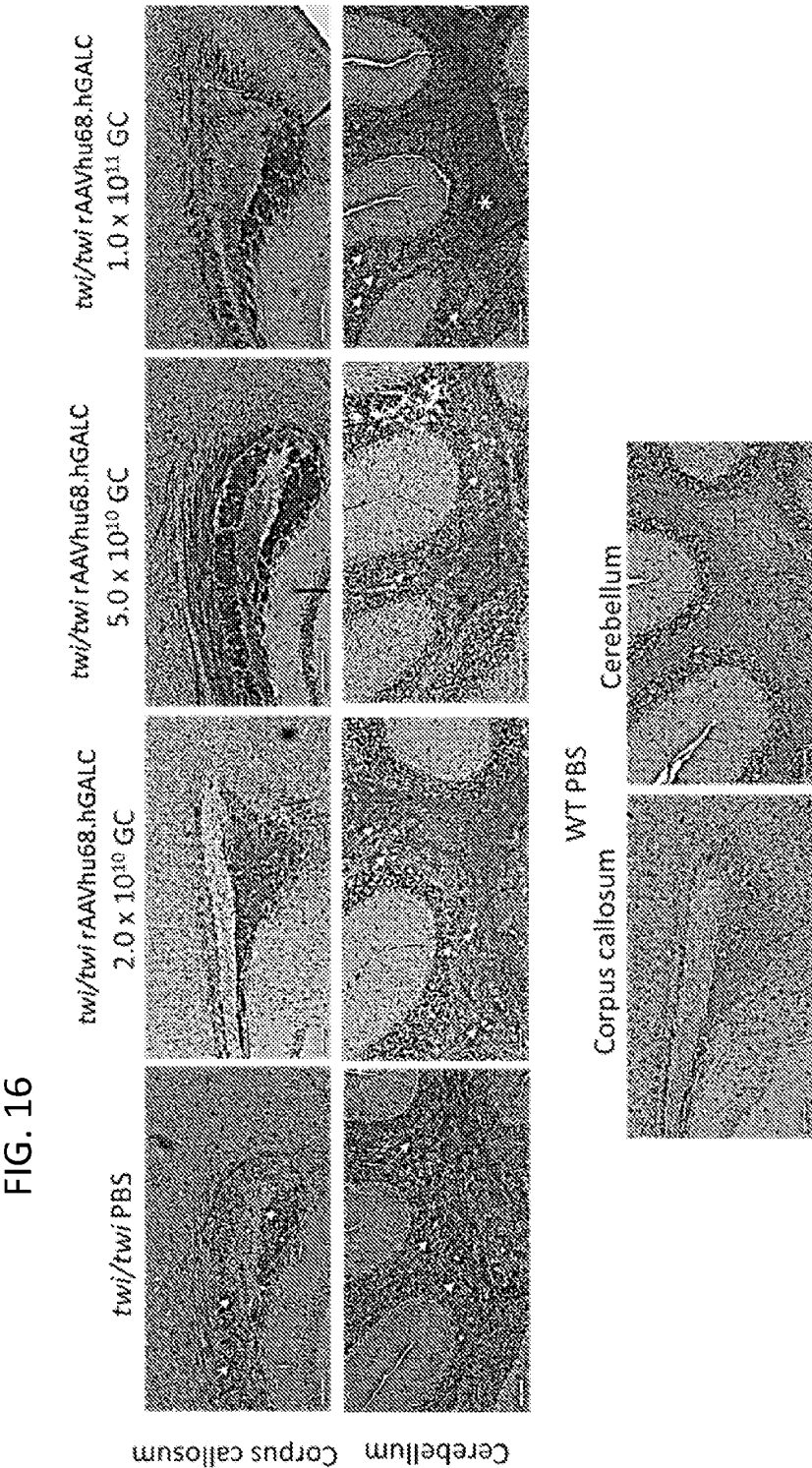


FIG. 17

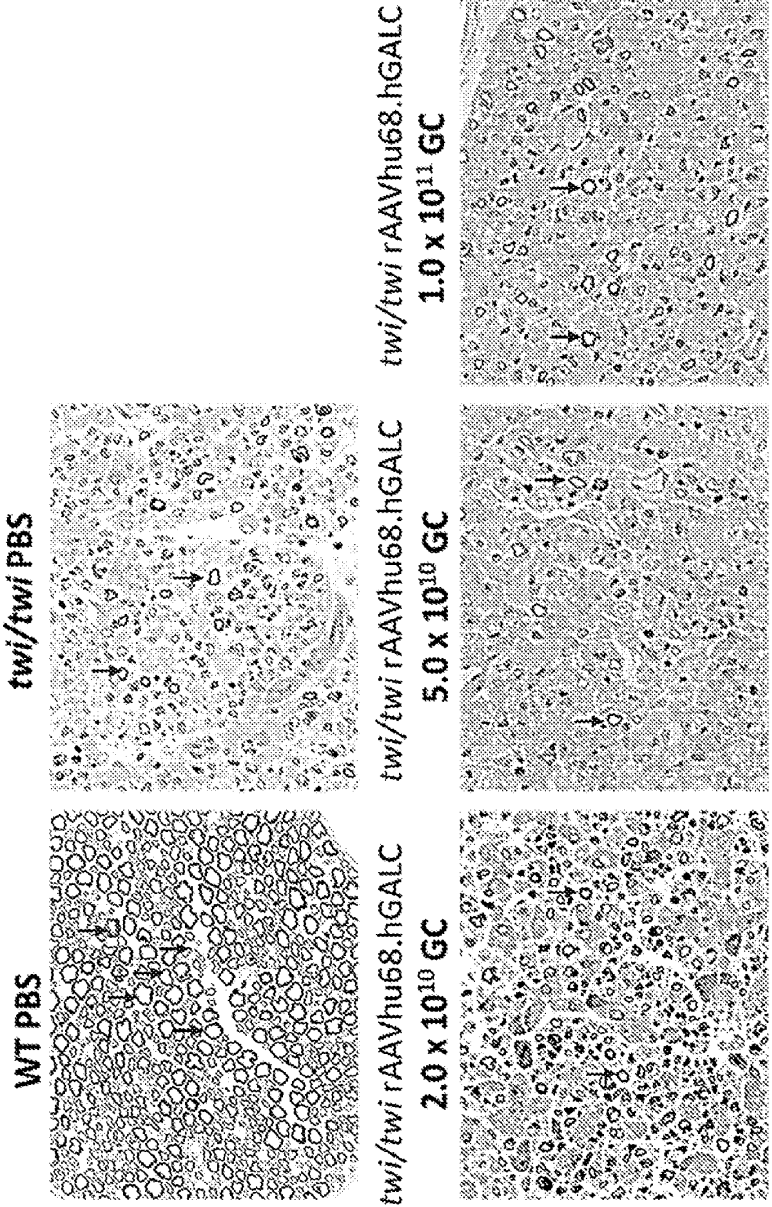


FIG. 18

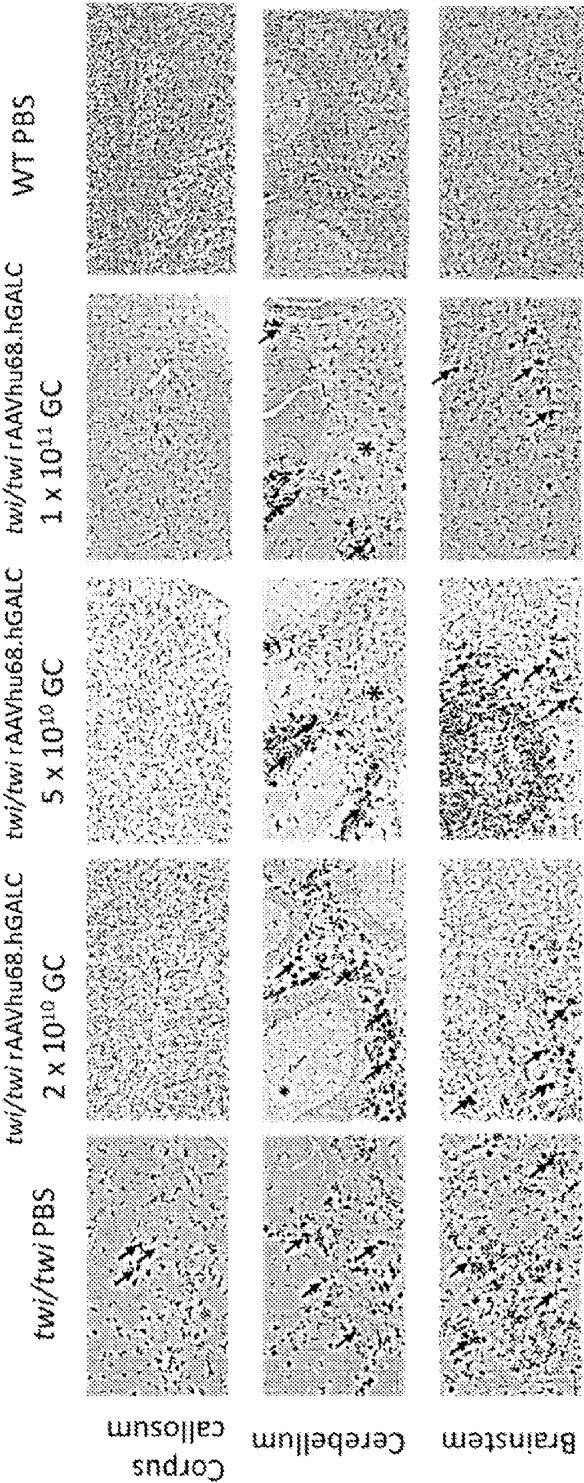


FIG. 19

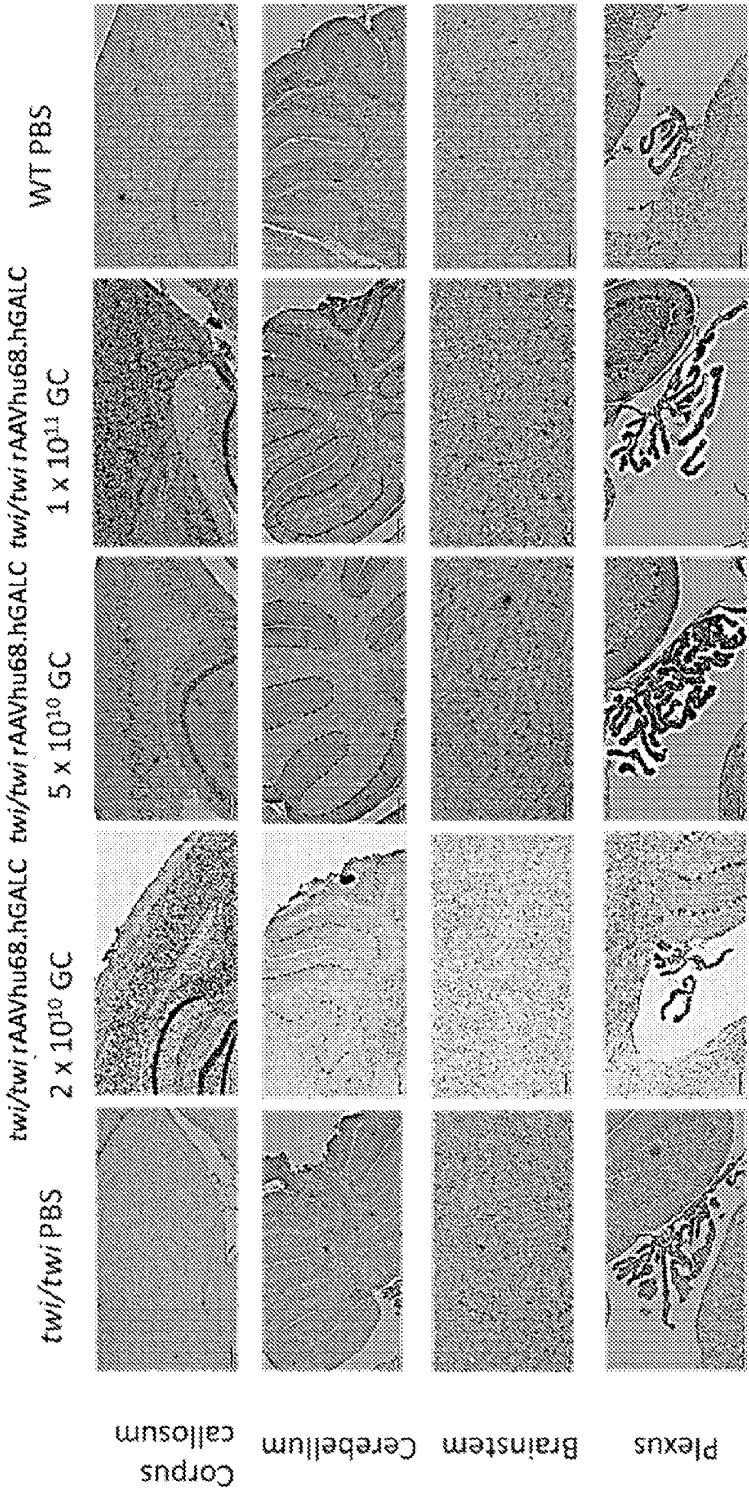


FIG. 20A

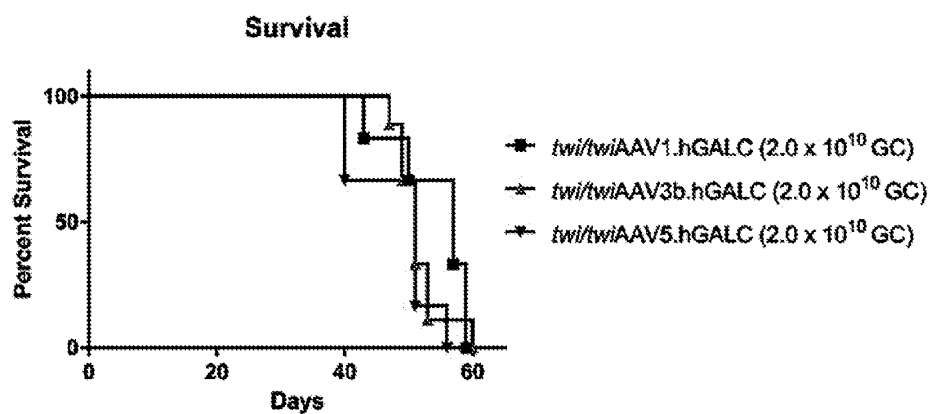


FIG. 20B

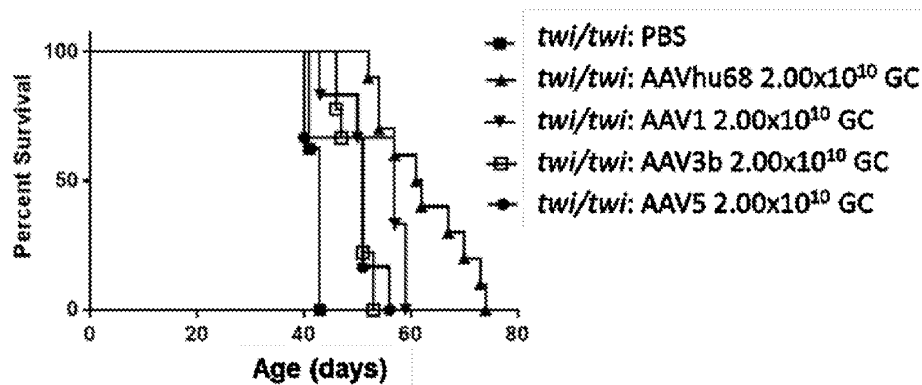


FIG. 21

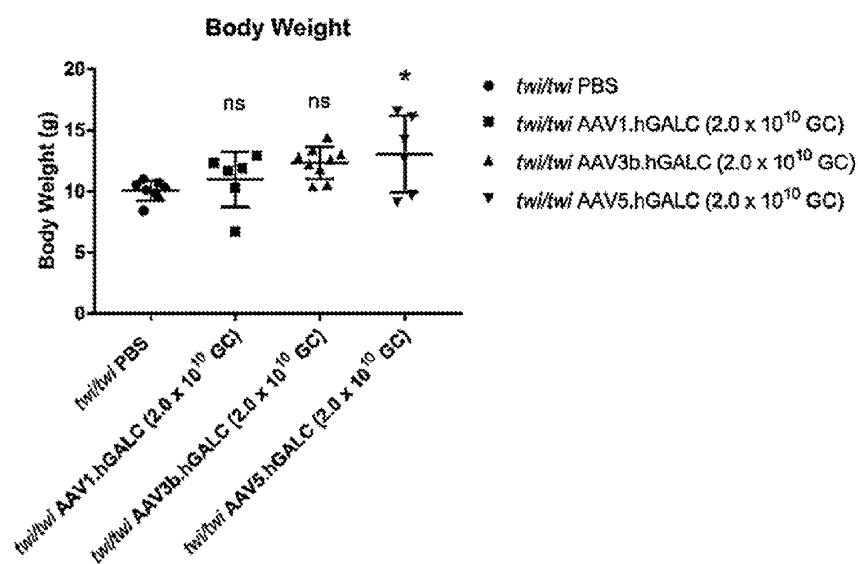


FIG. 22

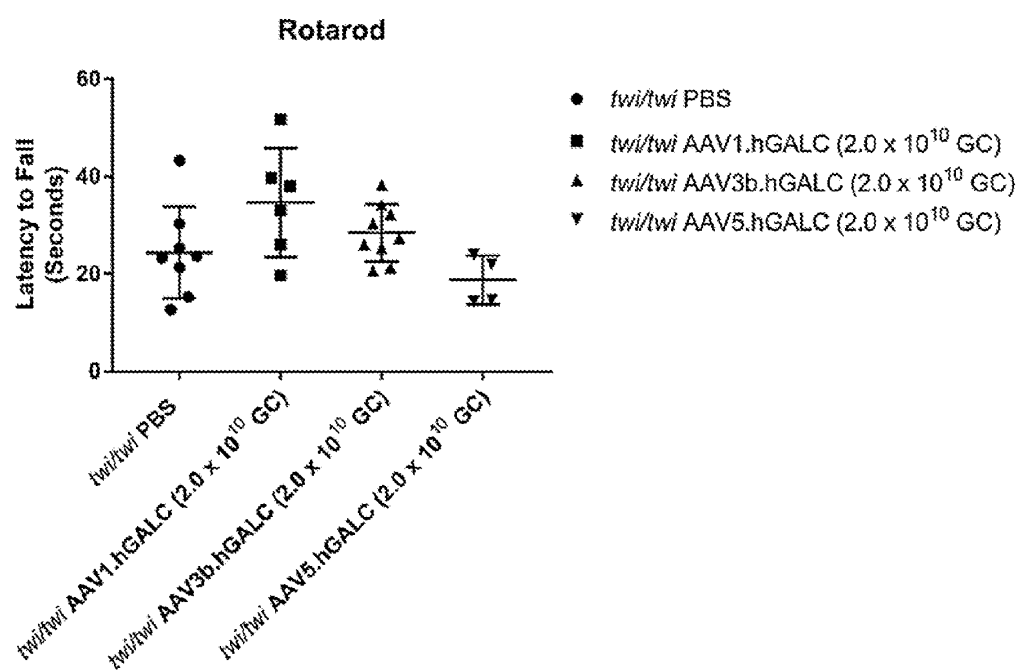
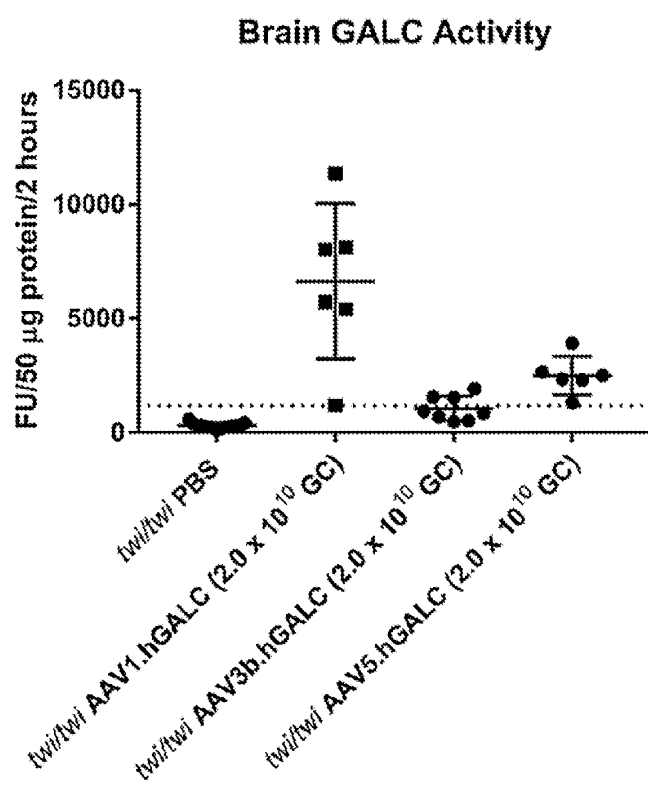


FIG. 23



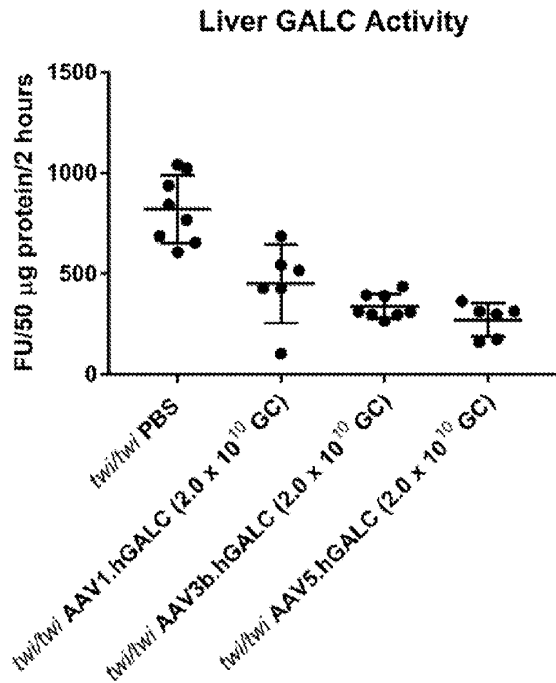


FIG. 24

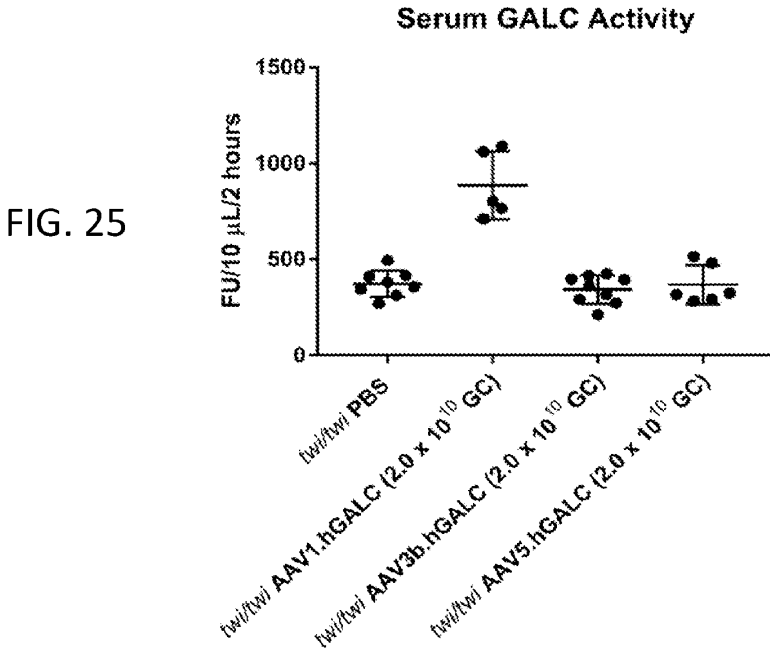


FIG. 25

FIG. 26

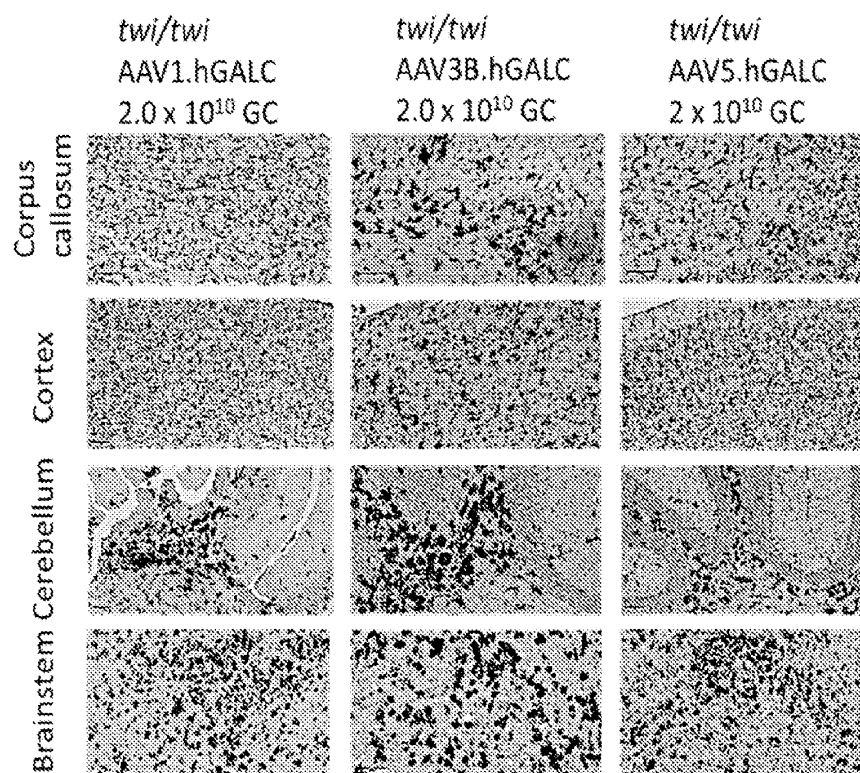
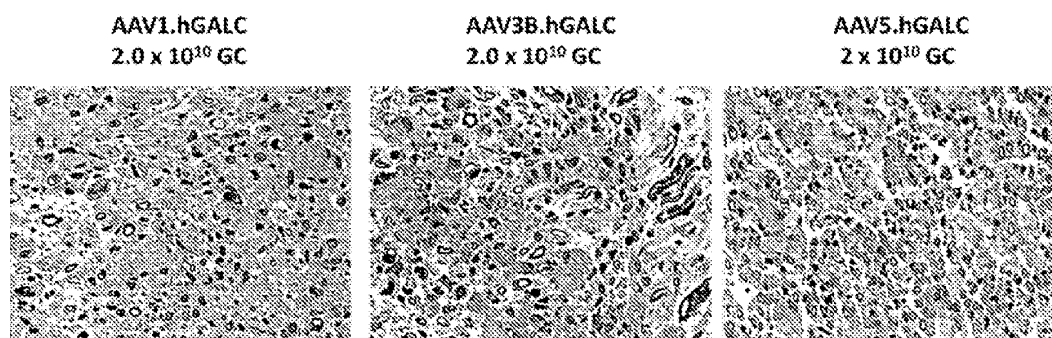


FIG. 27

FIG. 28

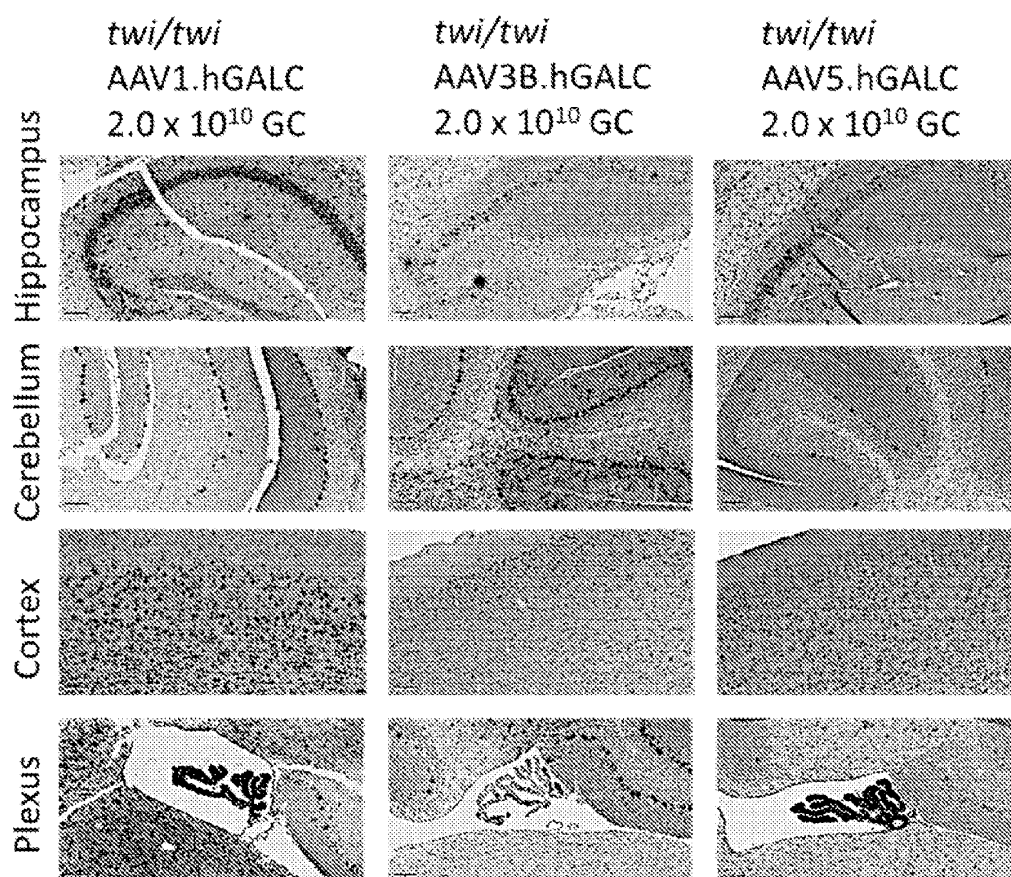


FIG. 29A

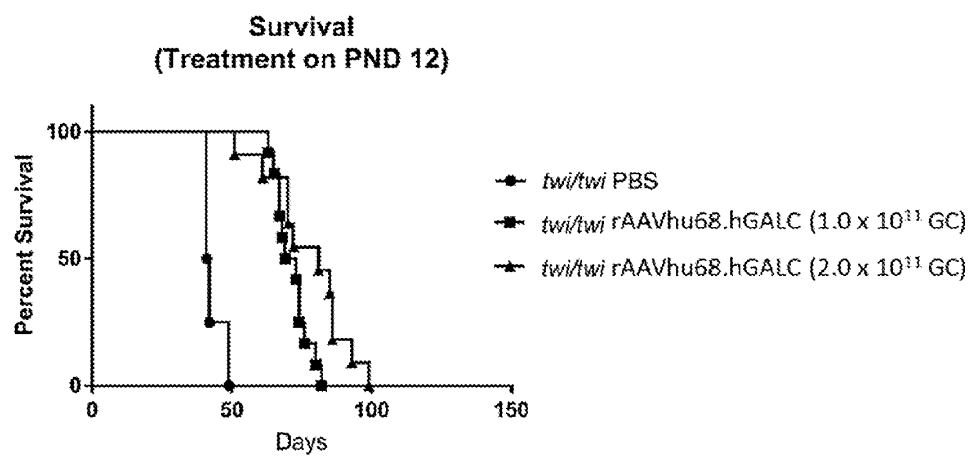


FIG. 29B

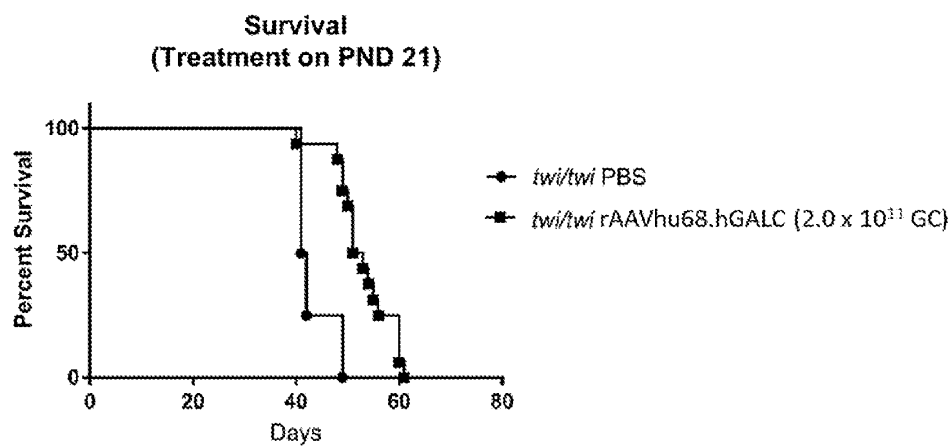


FIG. 30

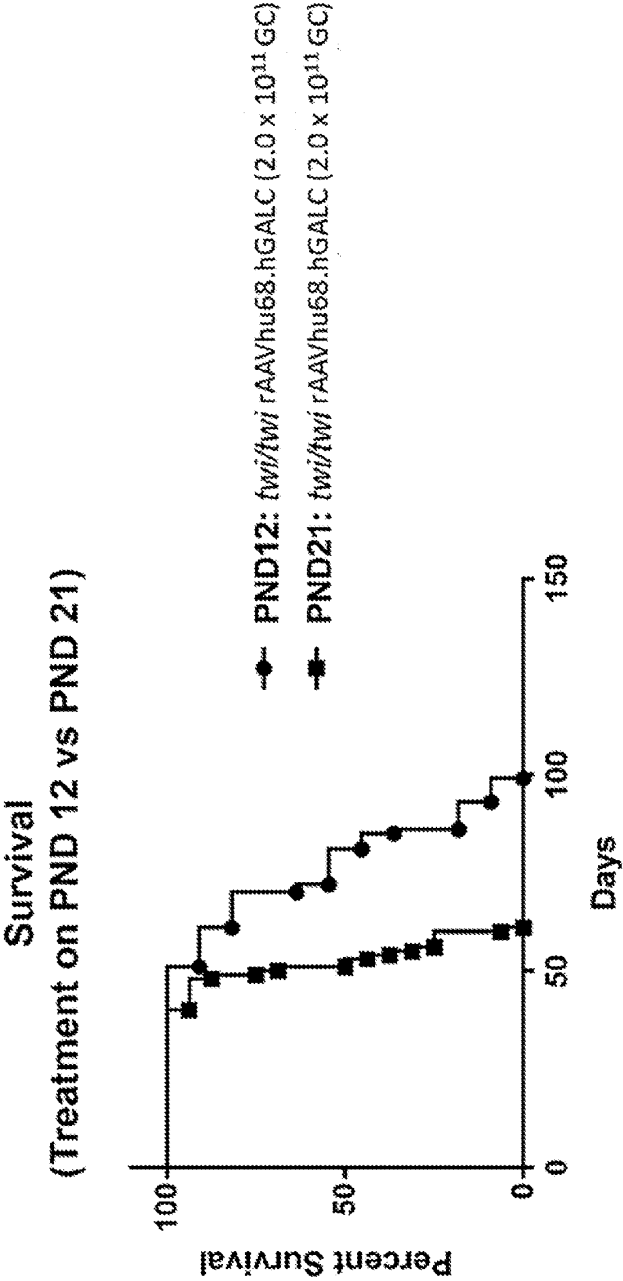


FIG. 31

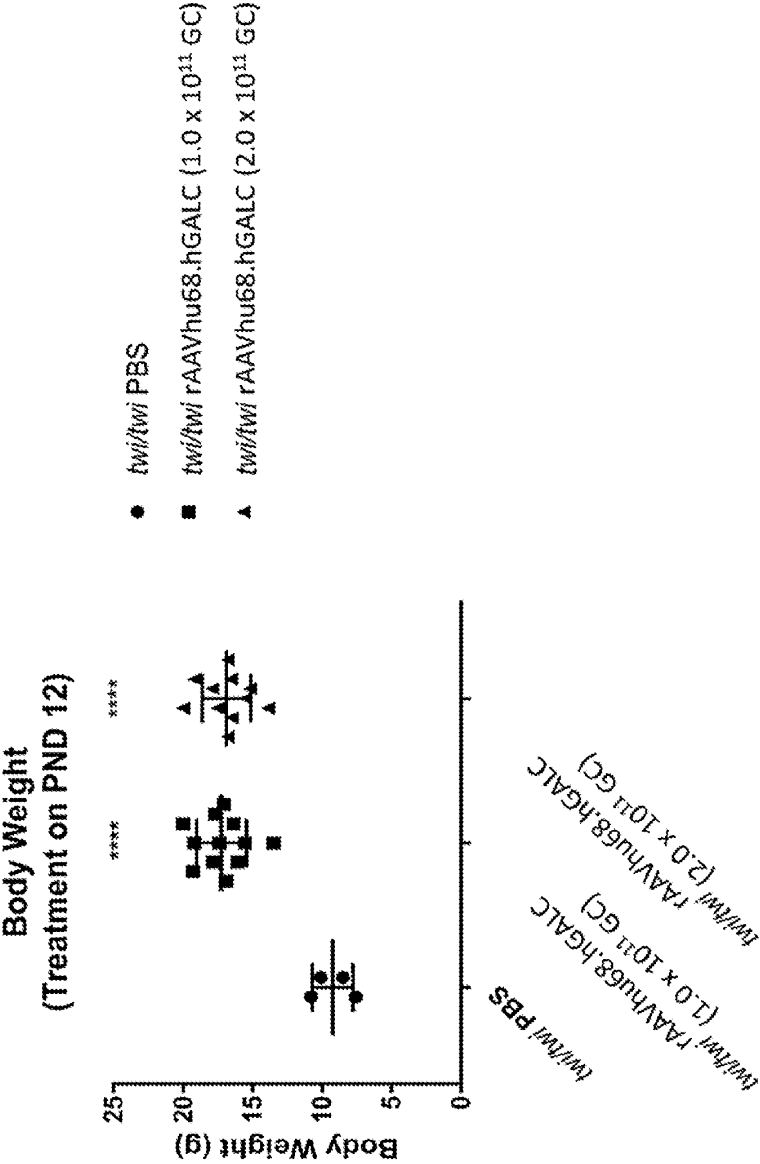


FIG. 32

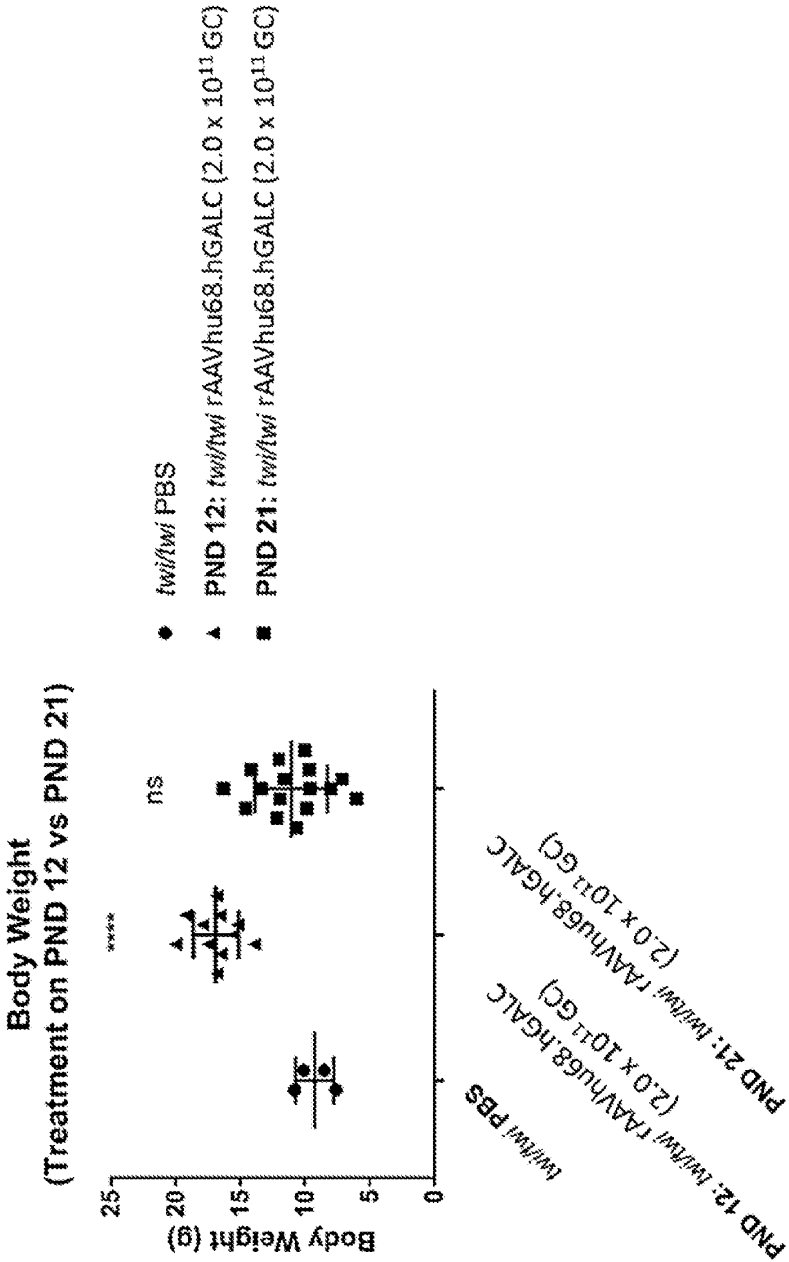


FIG. 33

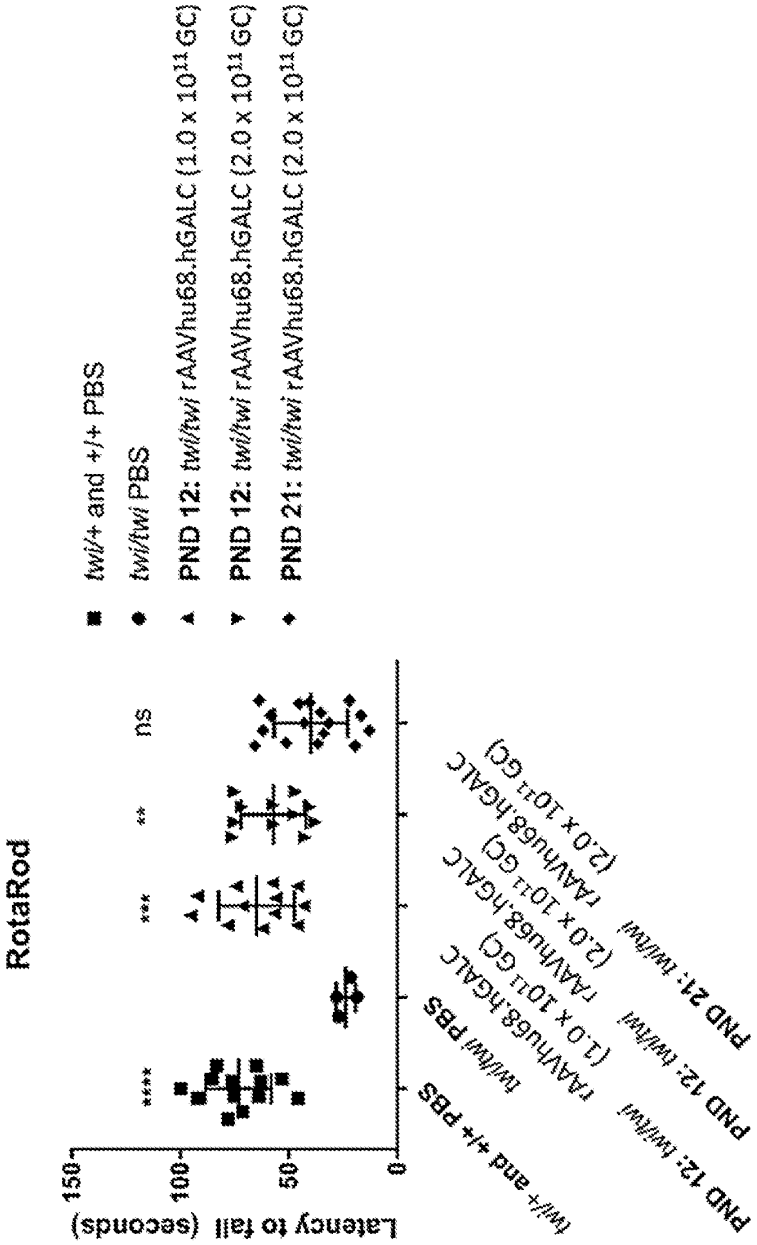


FIG. 34

RotaRod

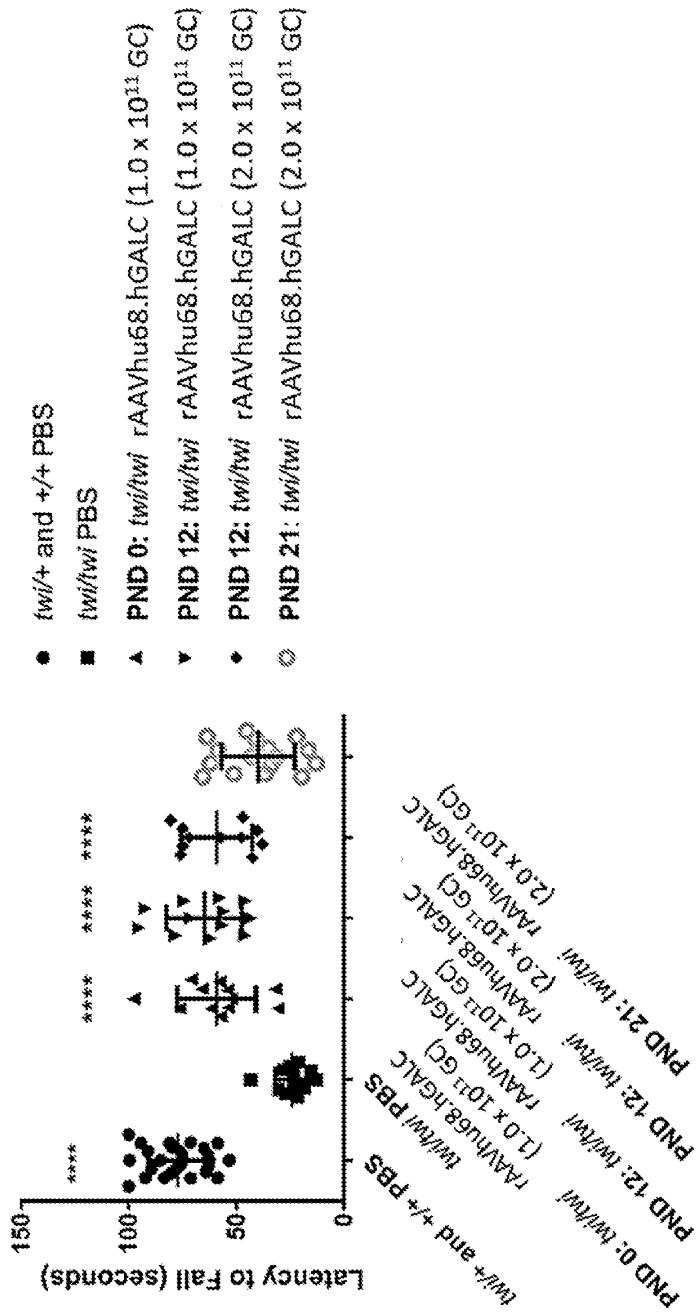


FIG. 35

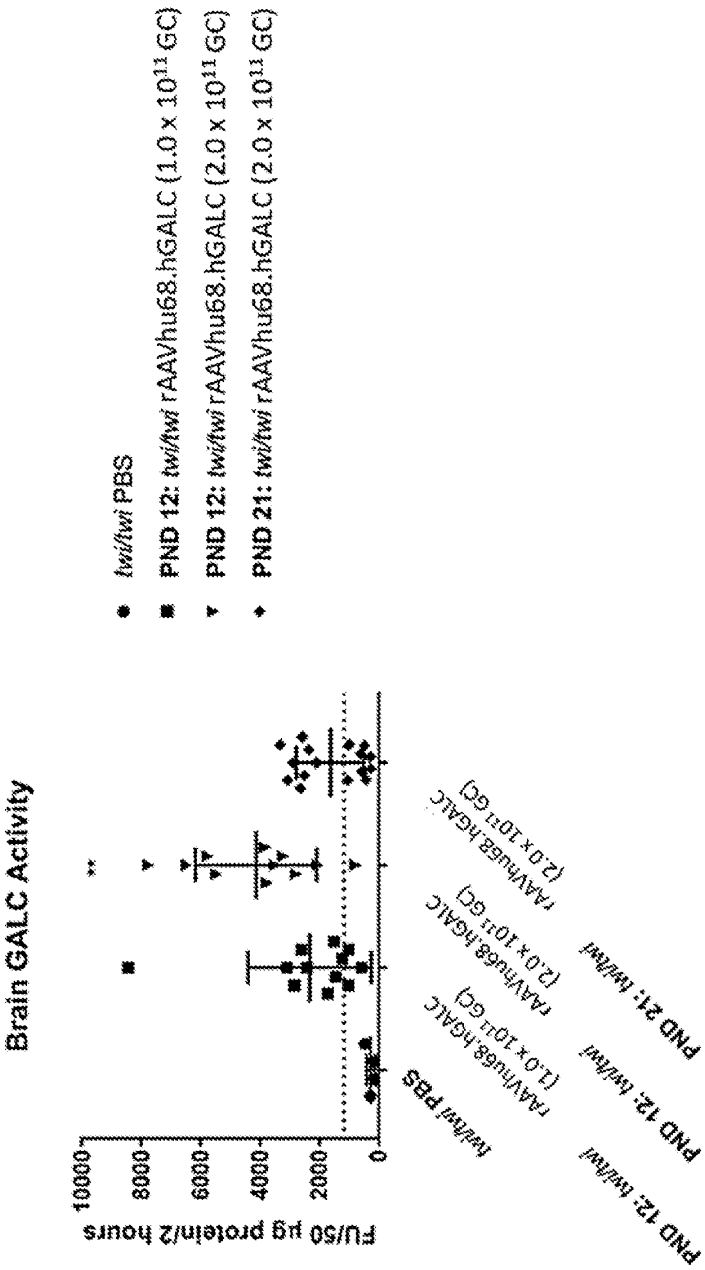


FIG. 36

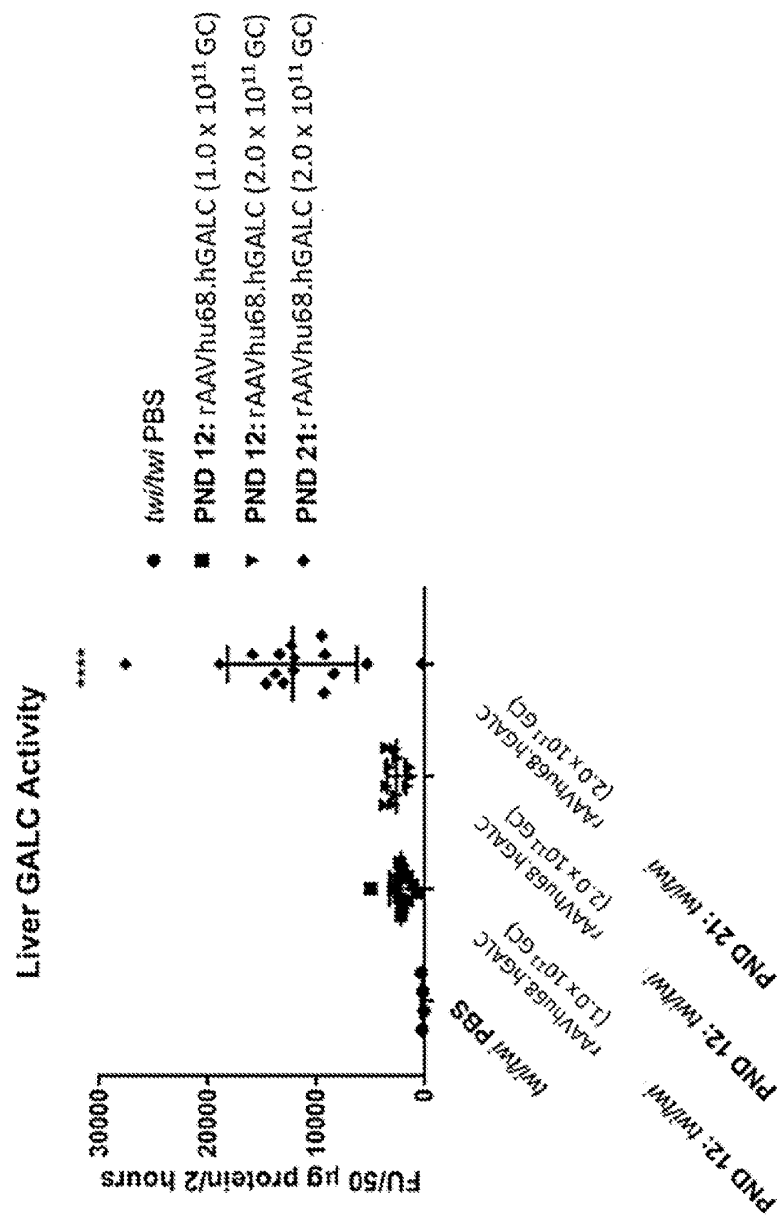


FIG. 37

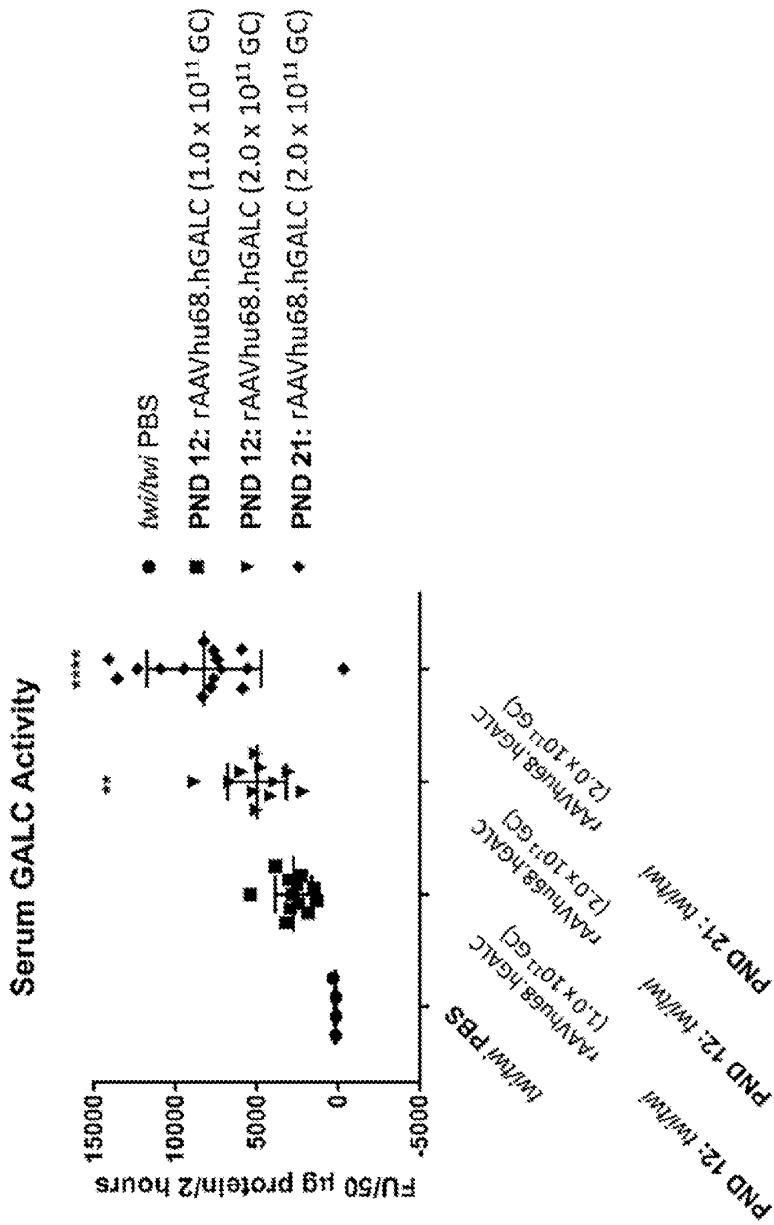


FIG. 38

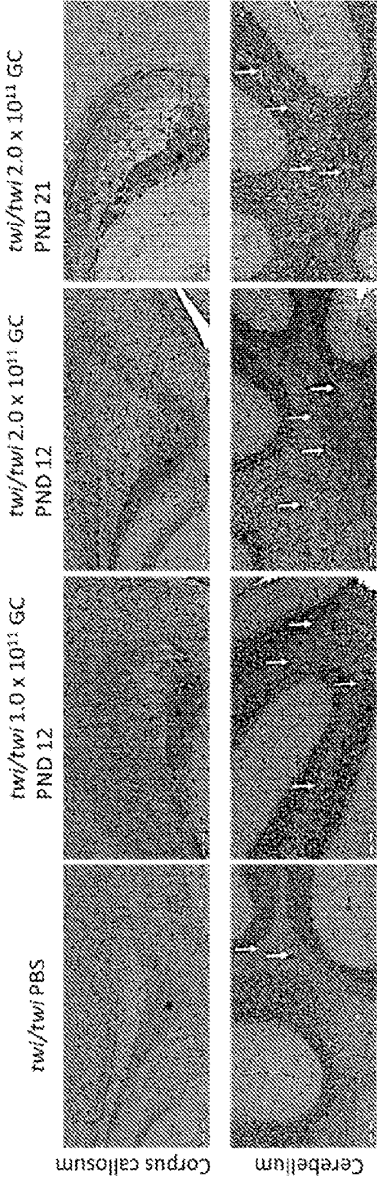


FIG. 39

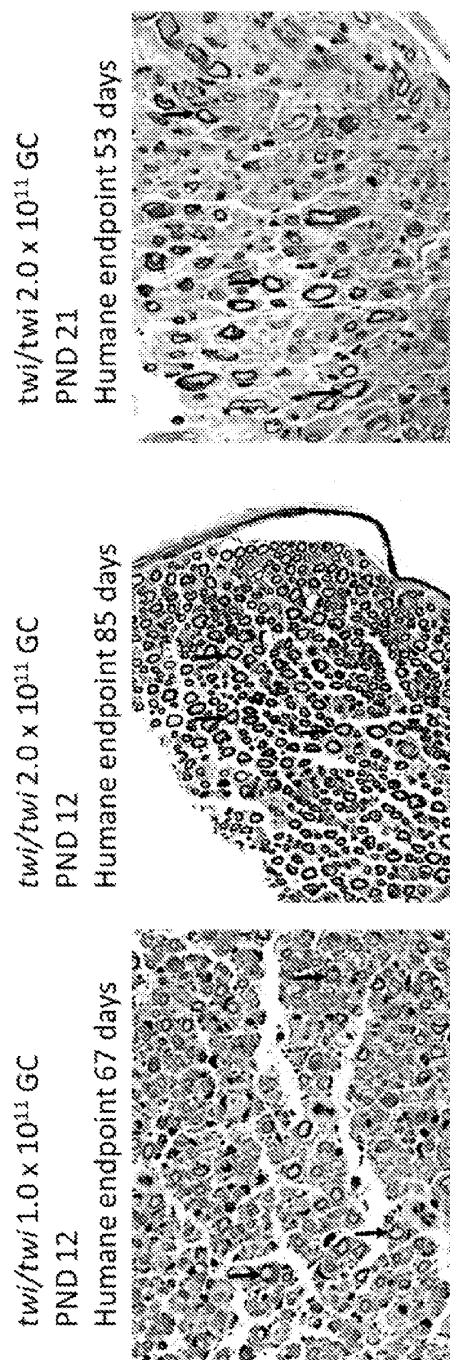


FIG. 40

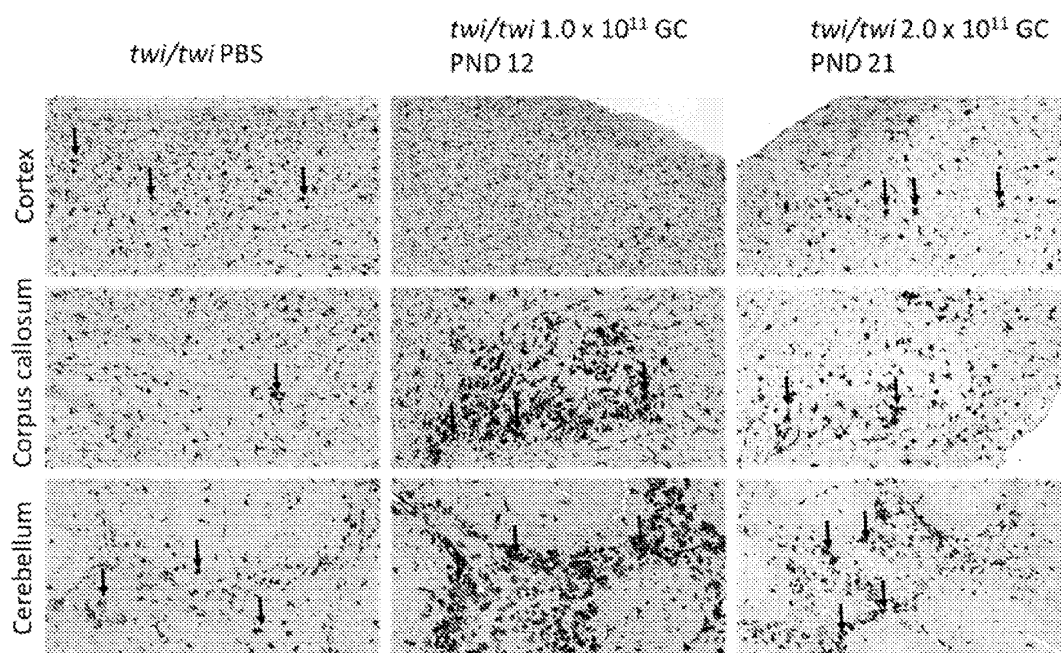


FIG. 41

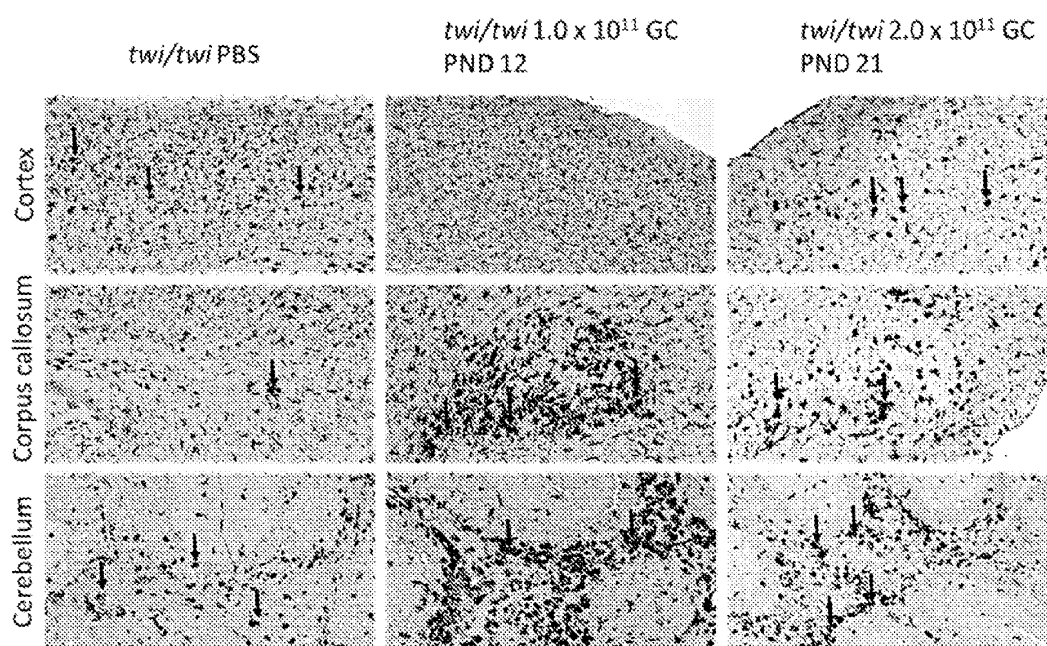


FIG. 42

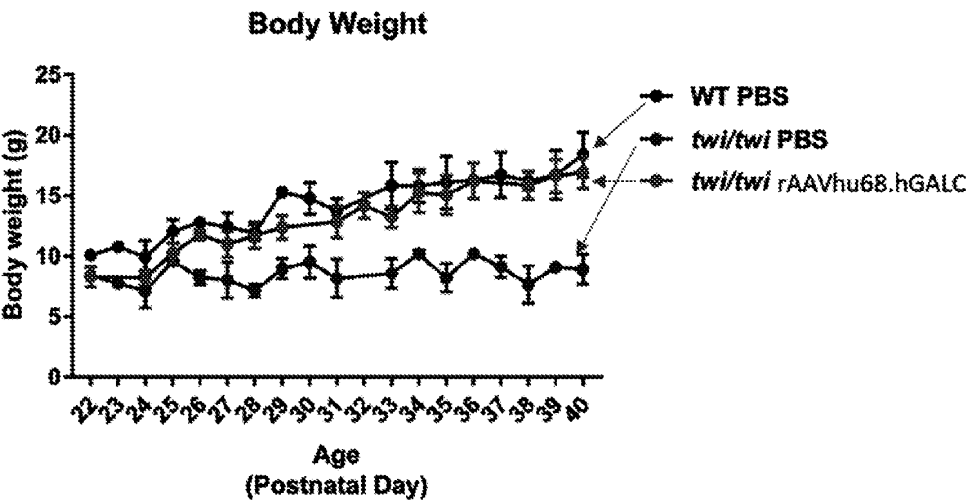


FIG. 43

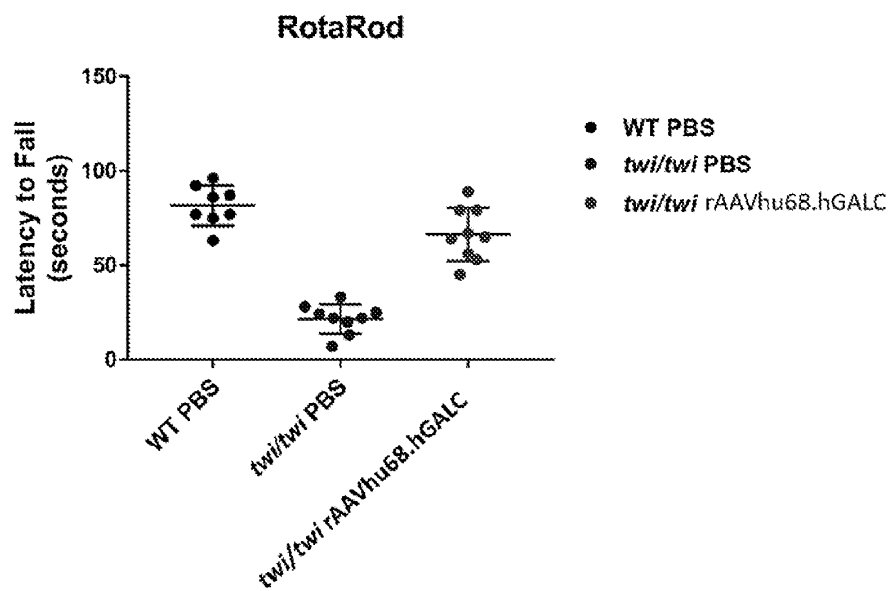


FIG. 44

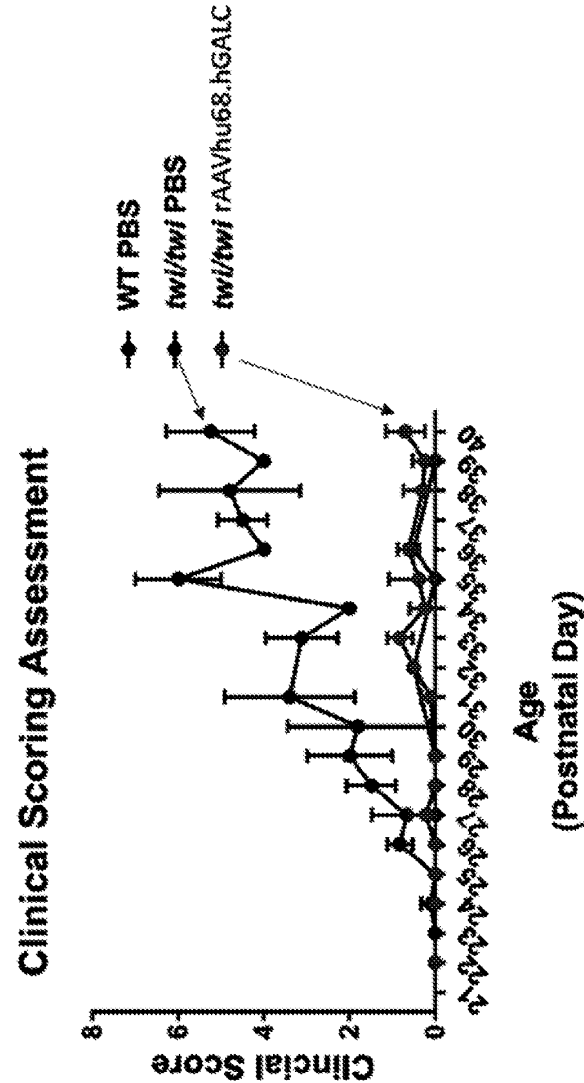


FIG. 45

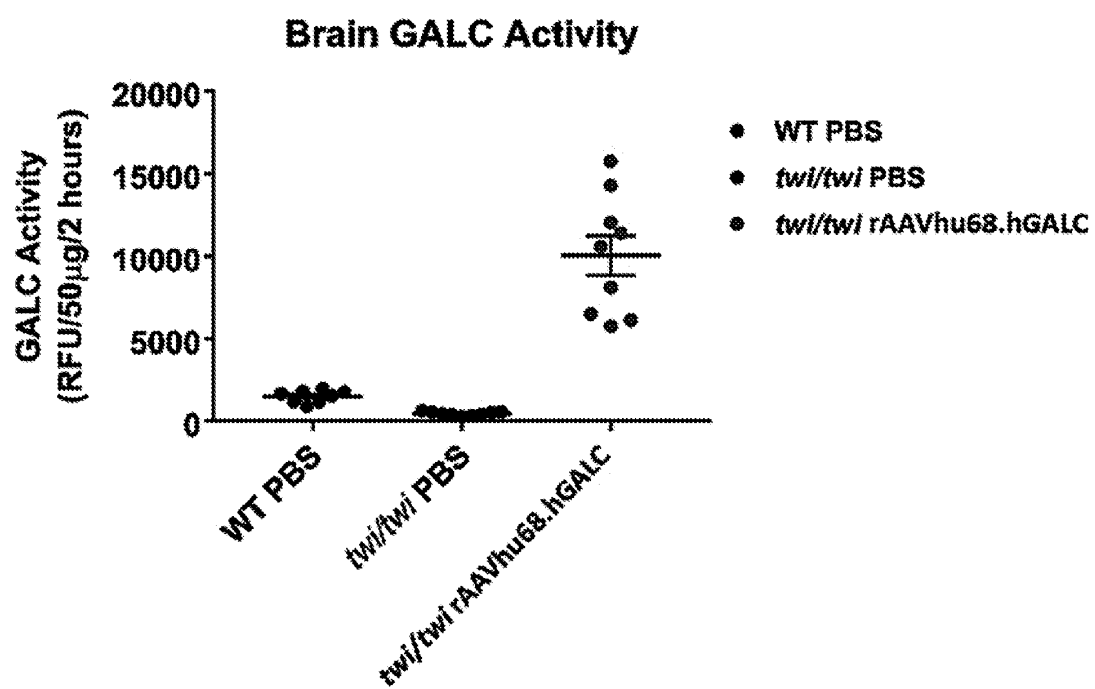


FIG. 46

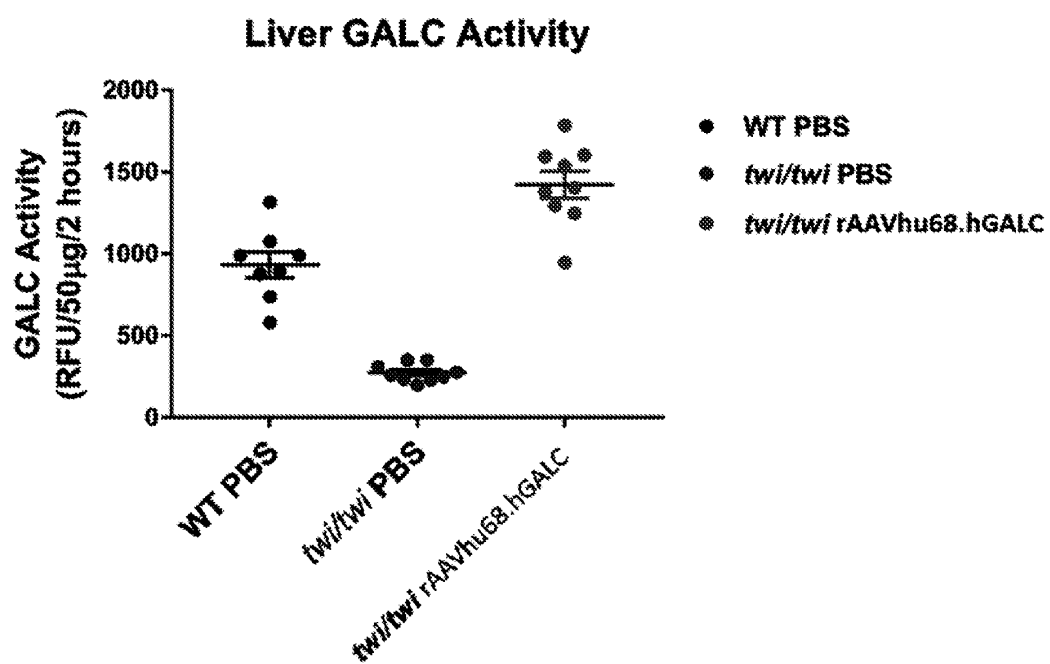


FIG. 47A

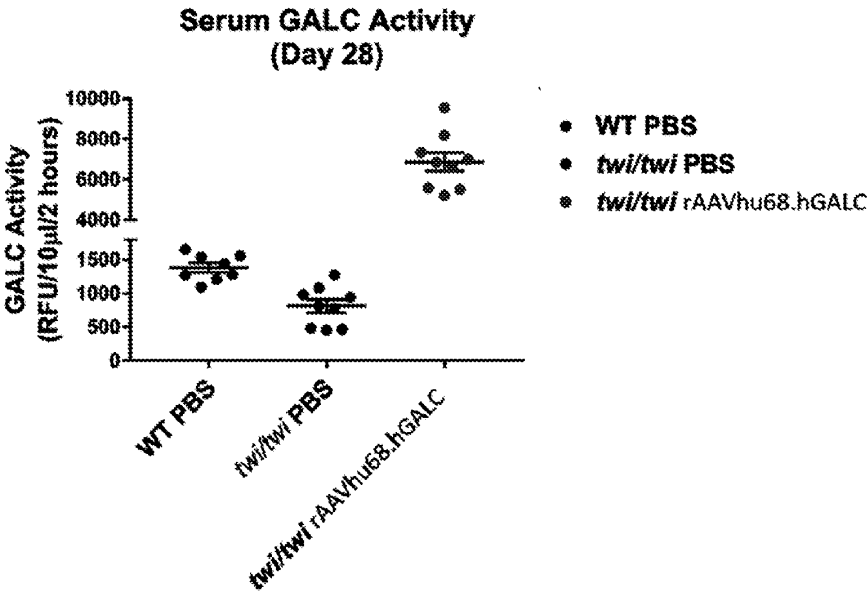


FIG. 47B

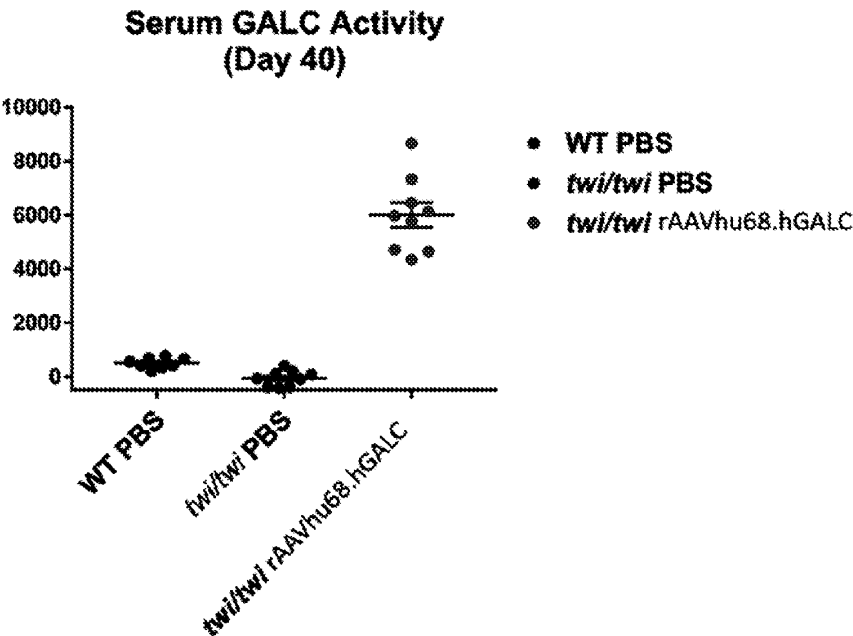


FIG. 48A

WT PBS

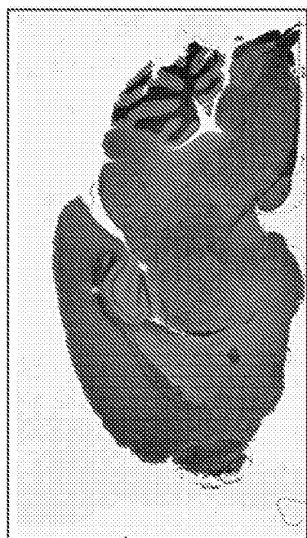


FIG. 48B

***twi/twi* PBS**



FIG. 48C

***twi/twi* rAAVhu68.hGALC**



FIG. 49

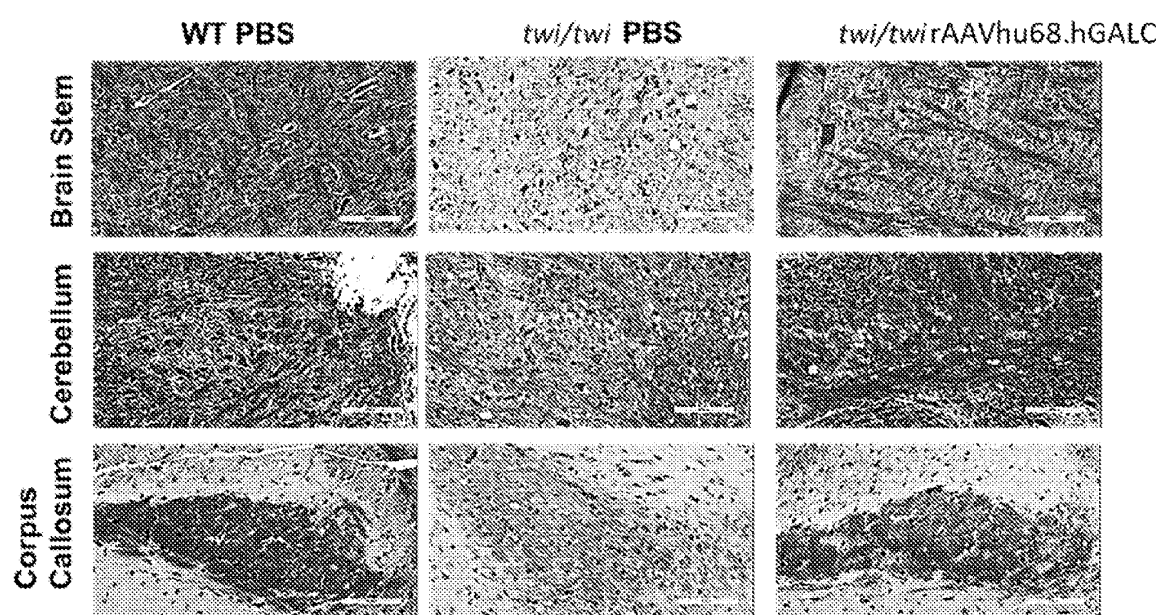


FIG. 50

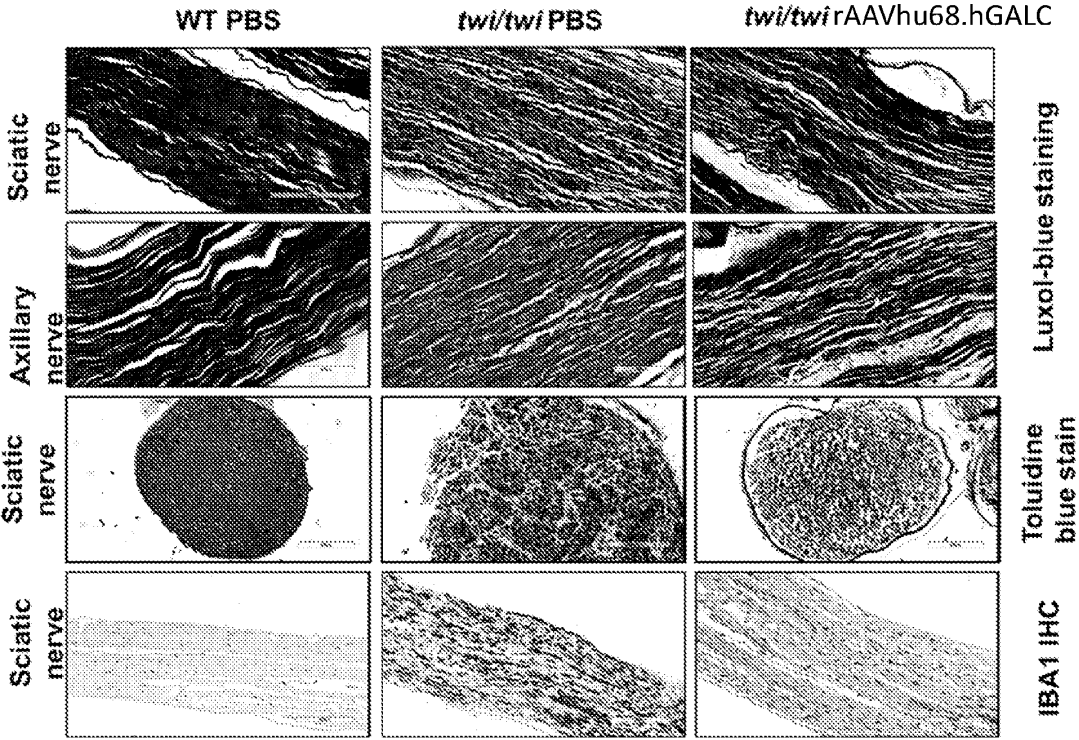


FIG. 51

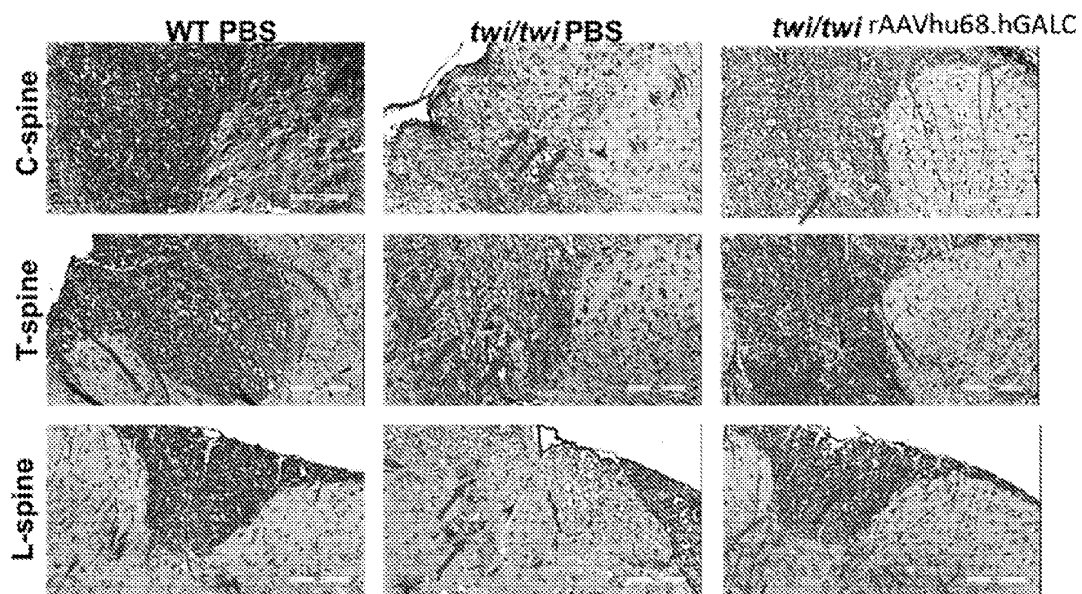


FIG. 52A

WT PBS



FIG. 52B

twi/twi PBS

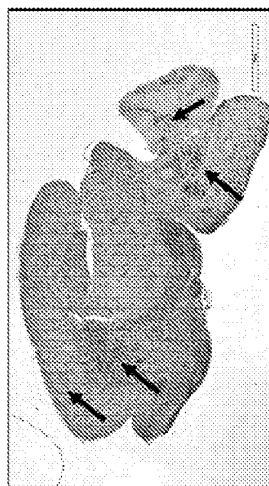


FIG. 52C

twi/twi rAAVhu68.hGALC

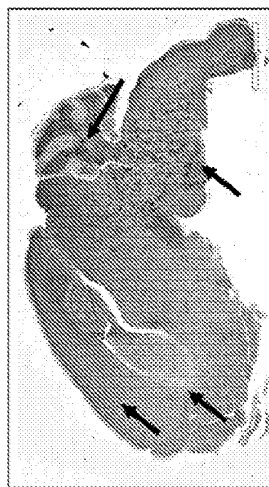


FIG. 53

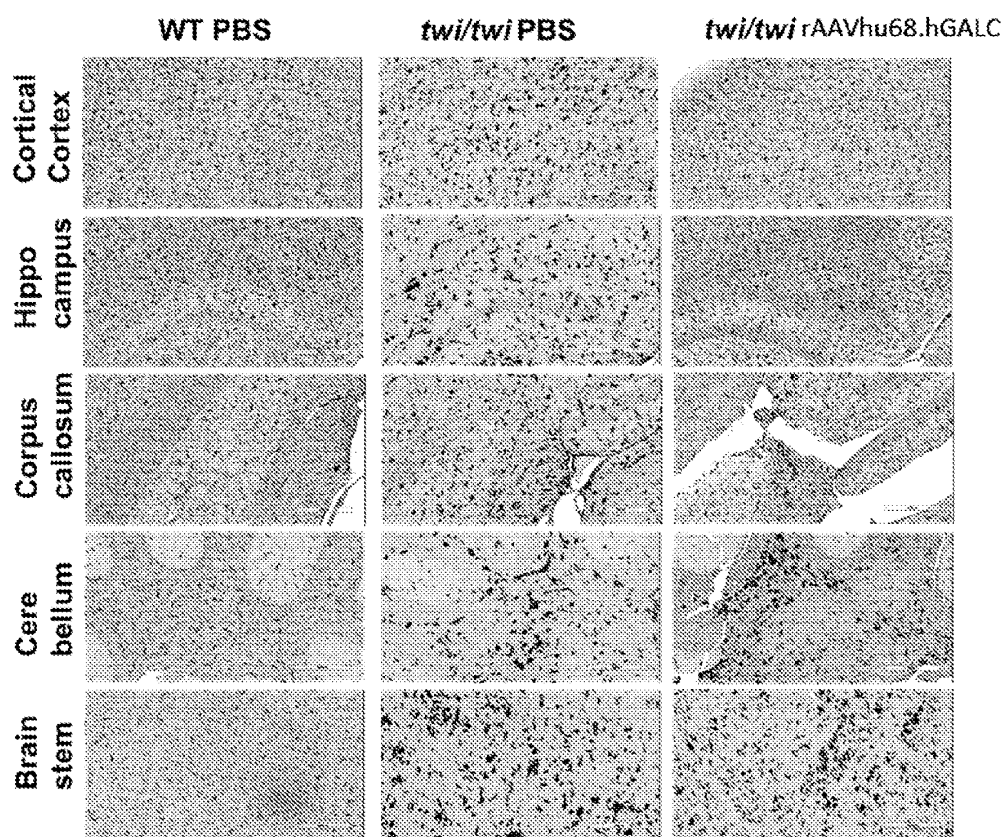


FIG. 54

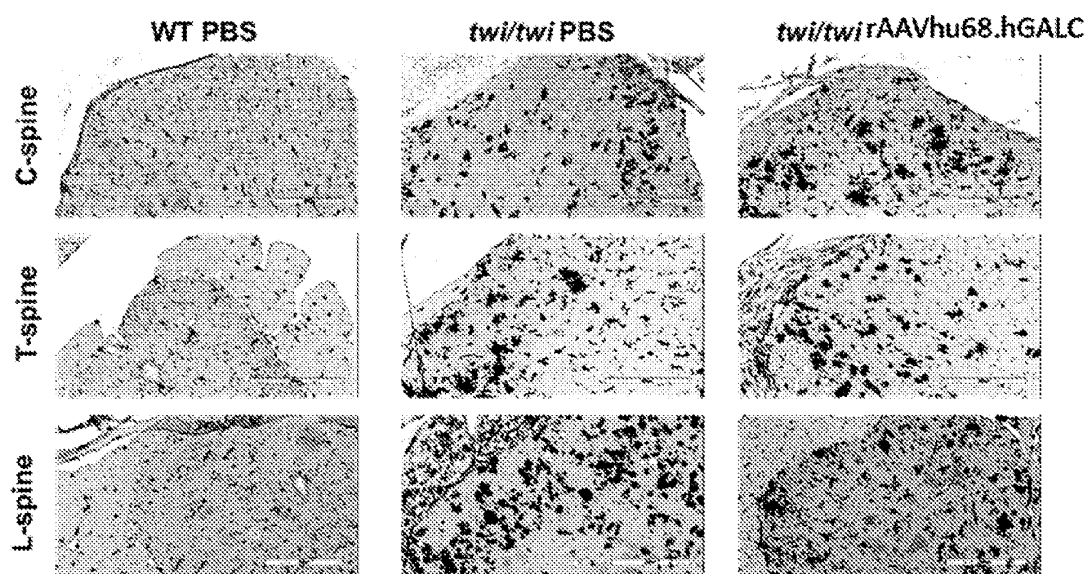


FIG. 55

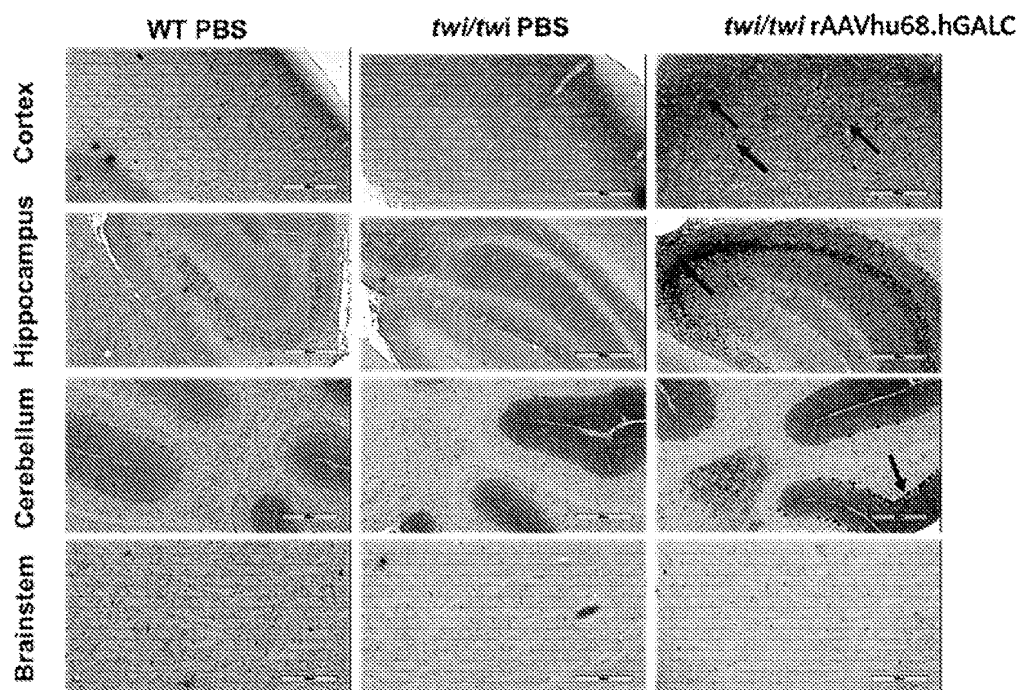


FIG. 56A

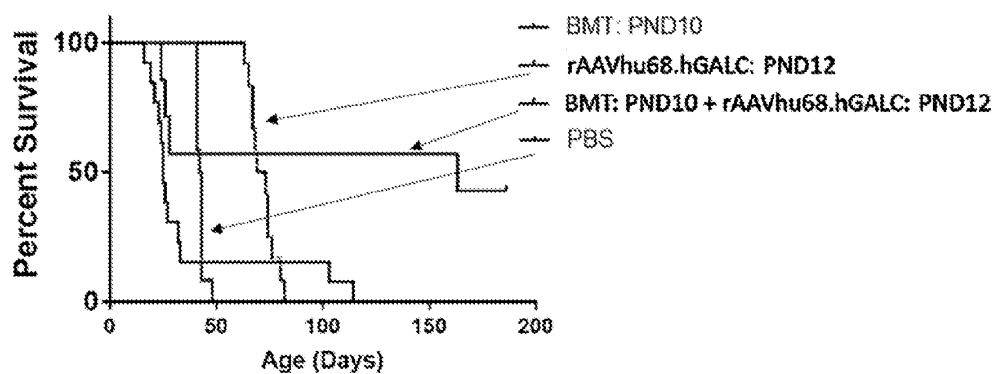


FIG. 56B

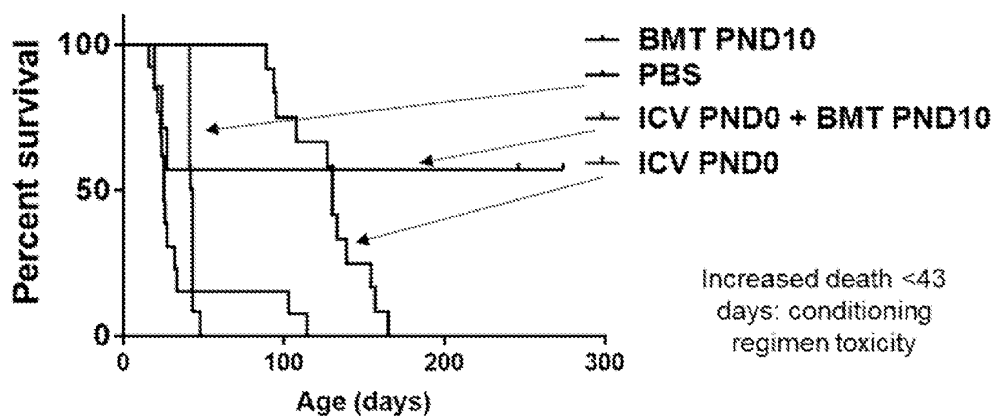


FIG. 56C

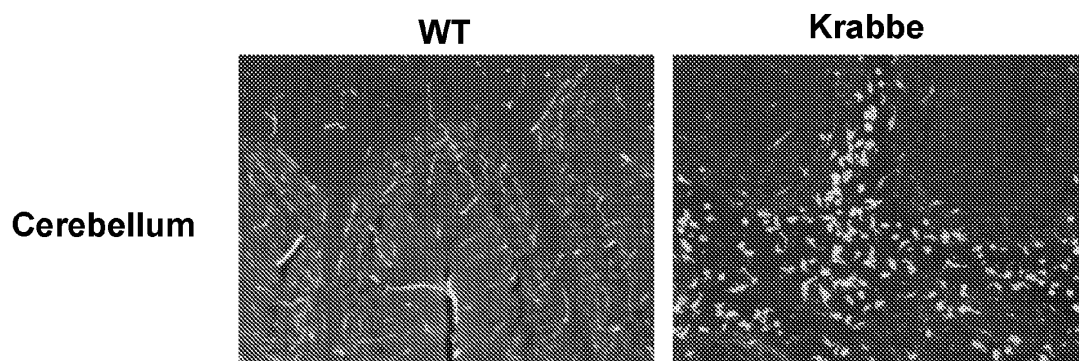


FIG. 57

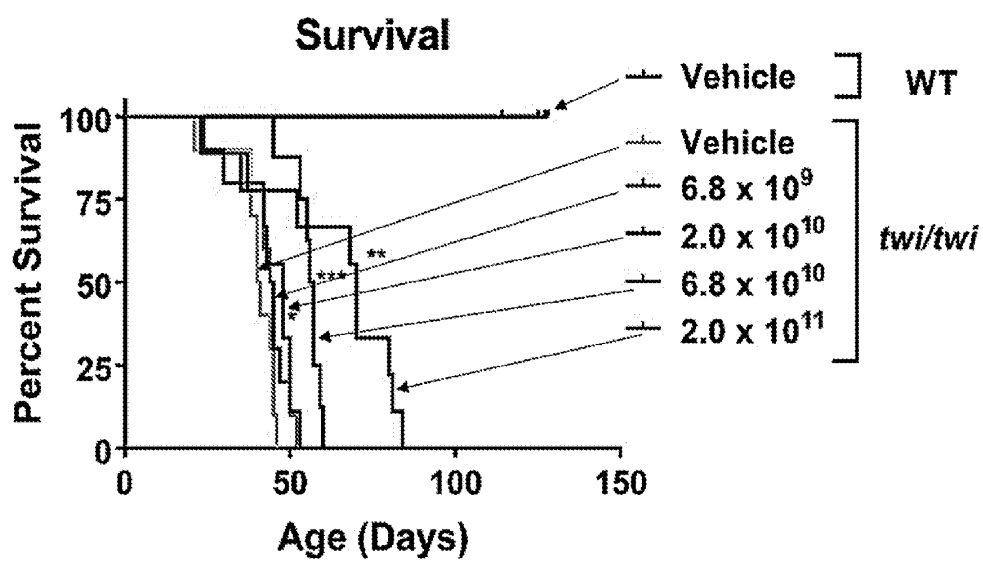


FIG. 58A

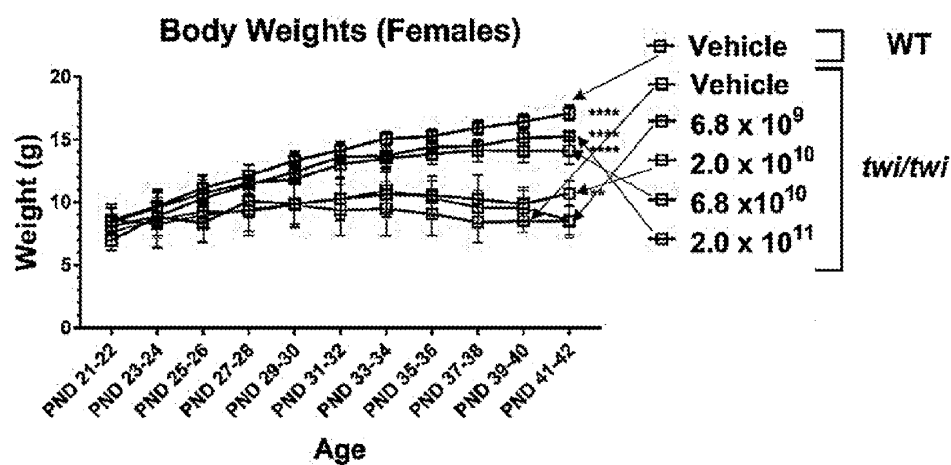
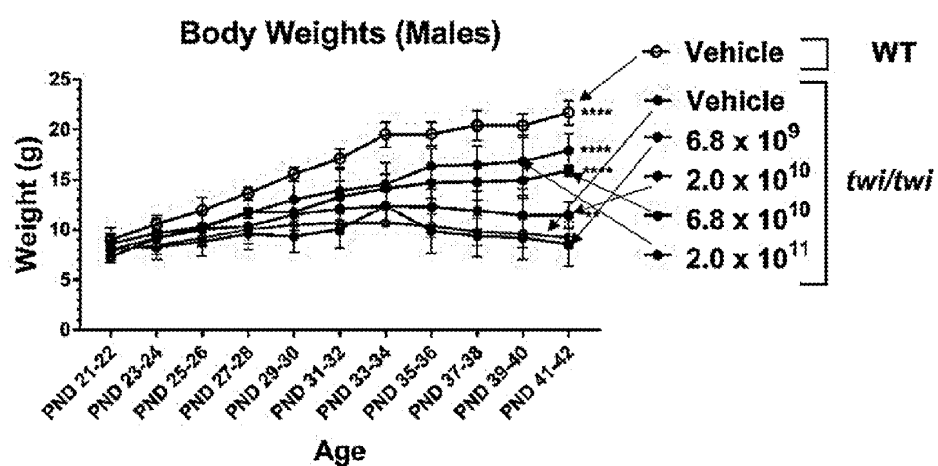


FIG. 58B

FIG. 59A

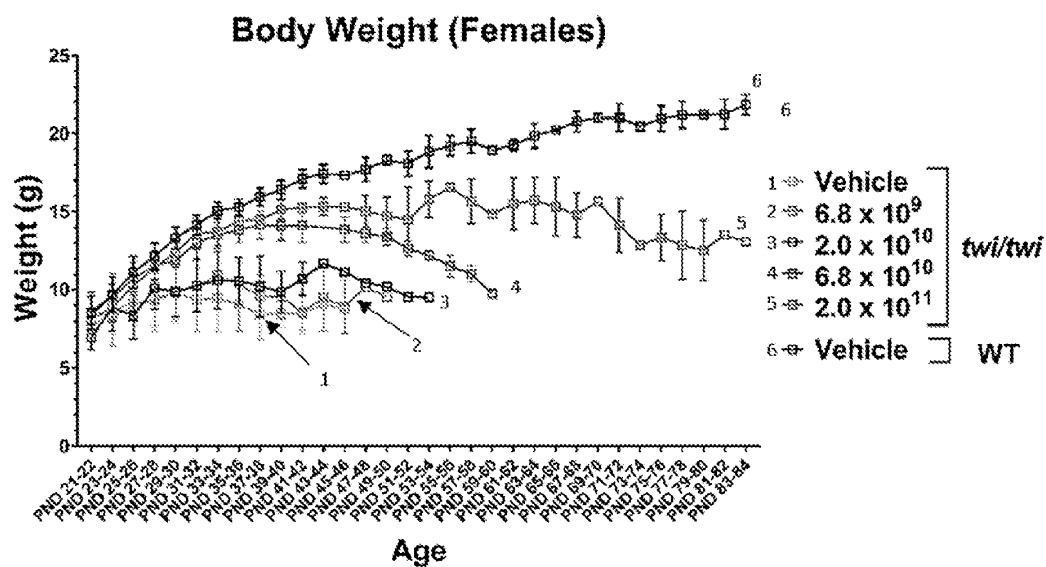
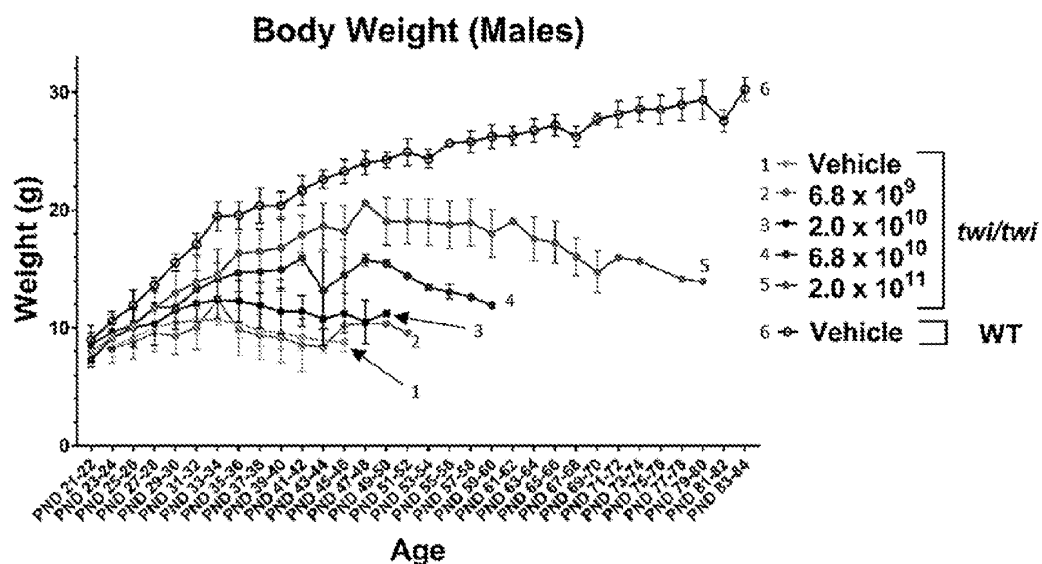


FIG. 59B

FIG. 60

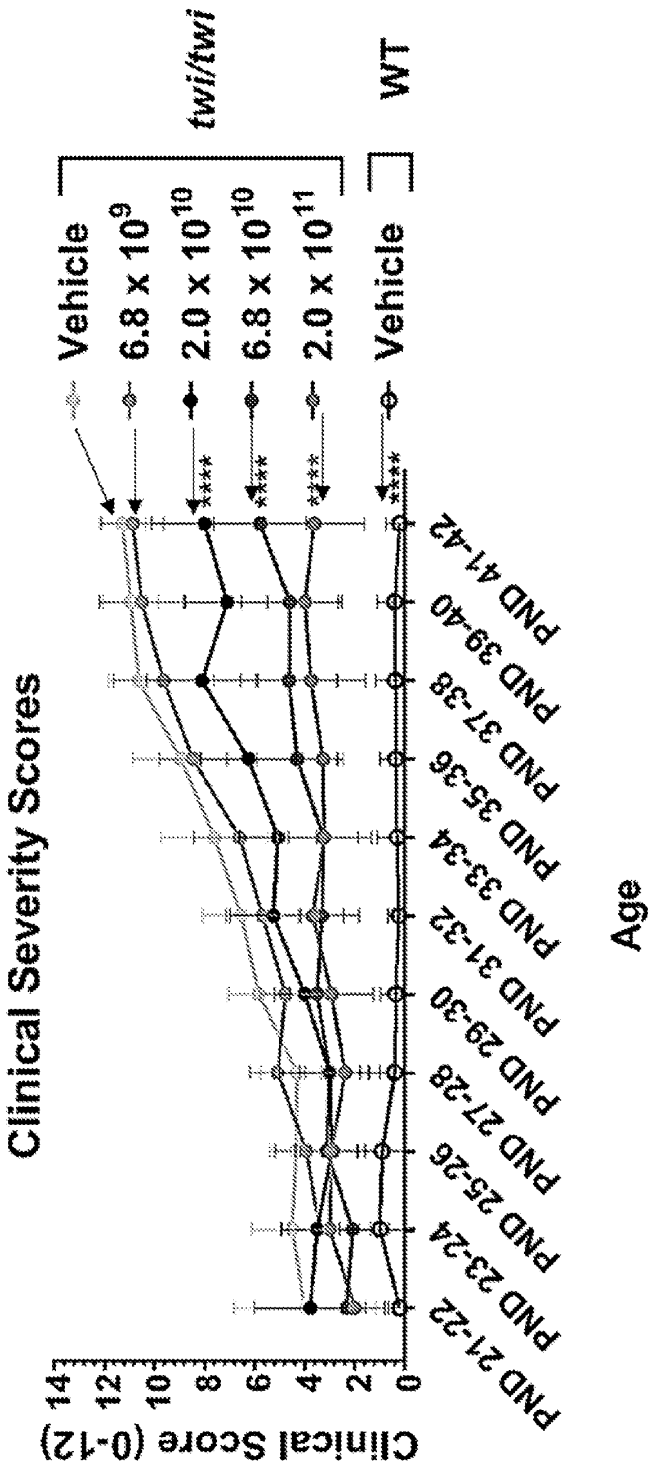


FIG. 61

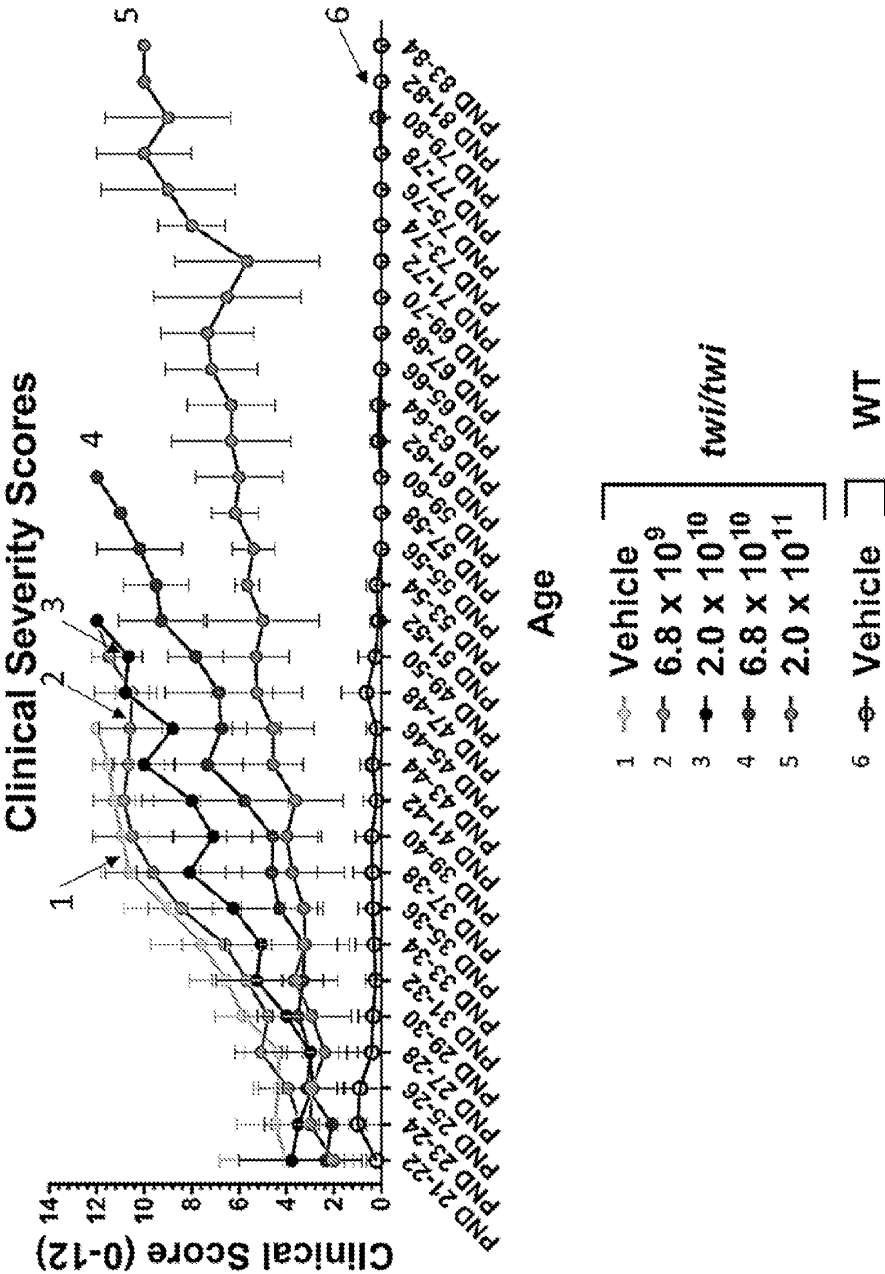
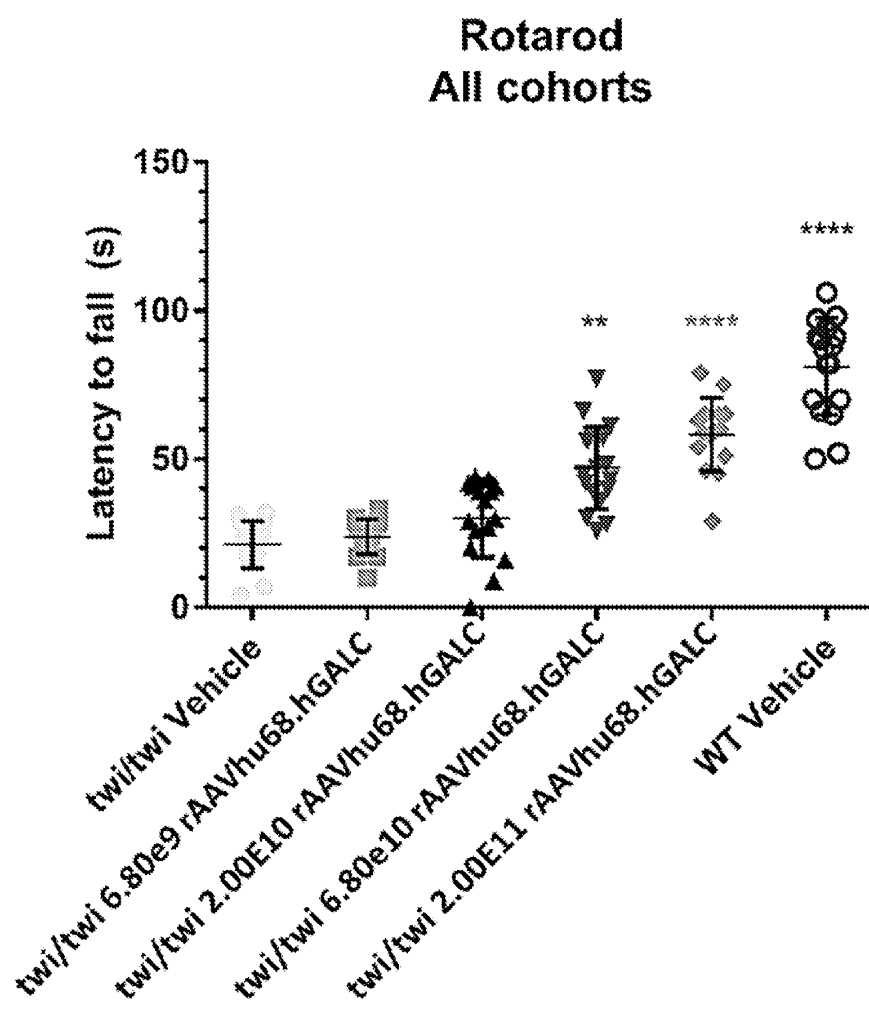


FIG. 62



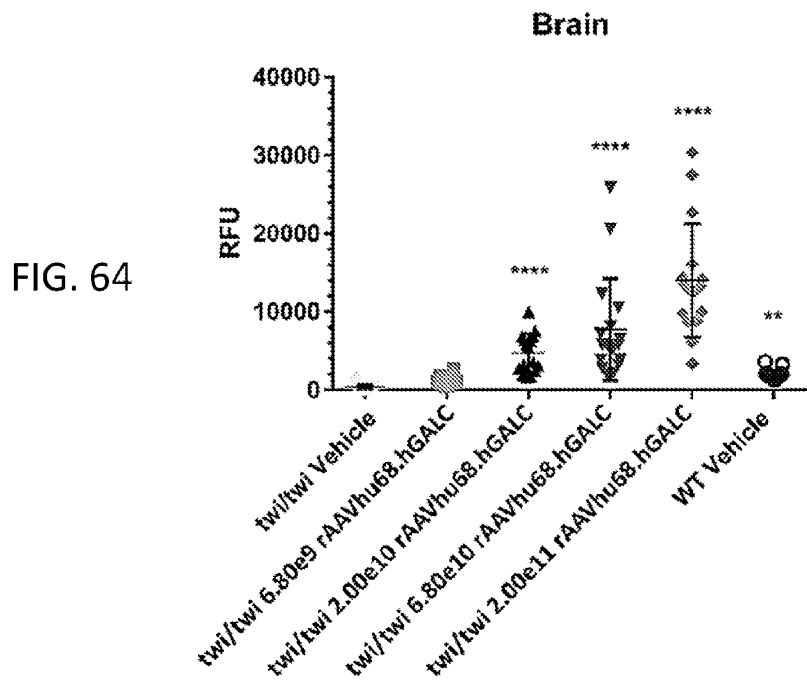
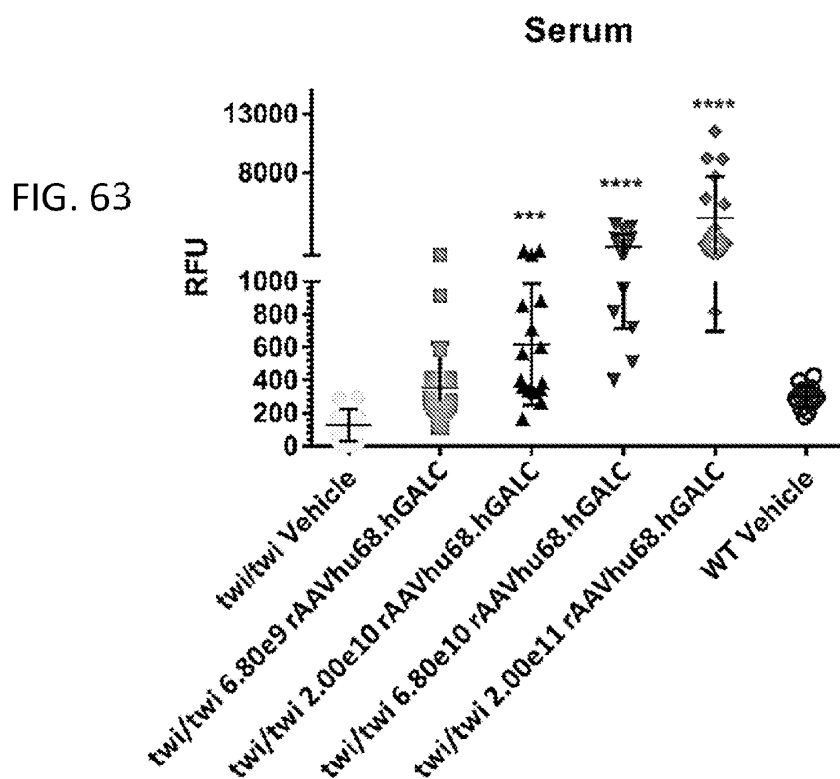


FIG. 65A

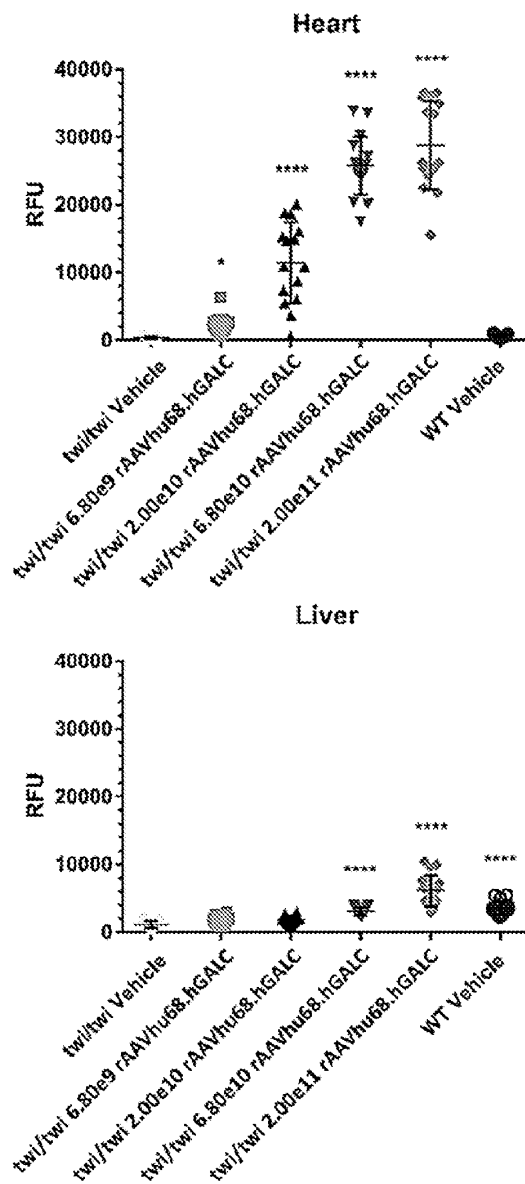


FIG. 65C

FIG. 65B

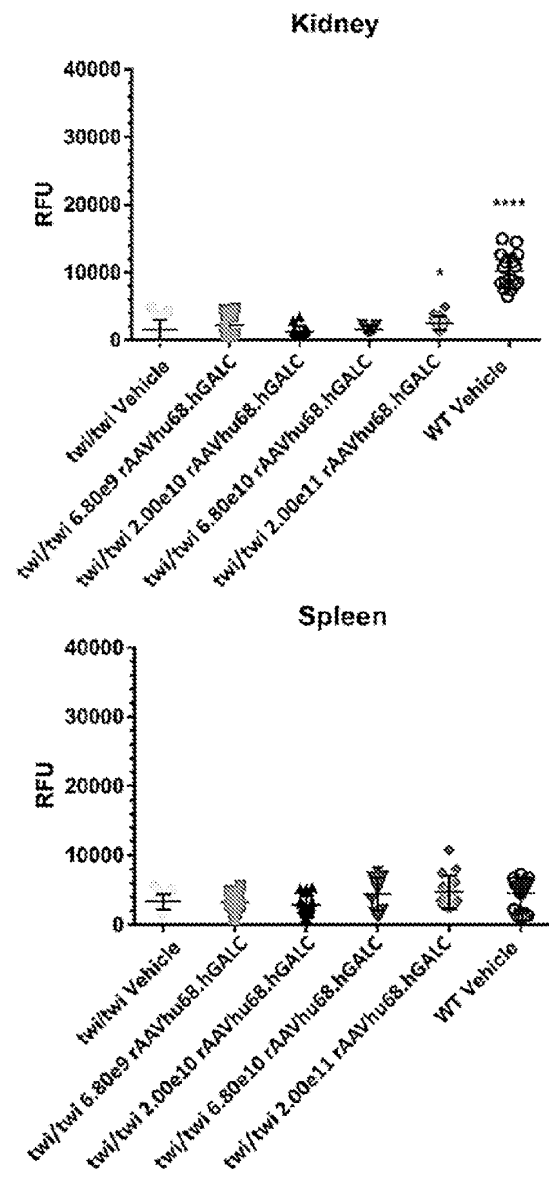


FIG. 65D

FIG. 66A

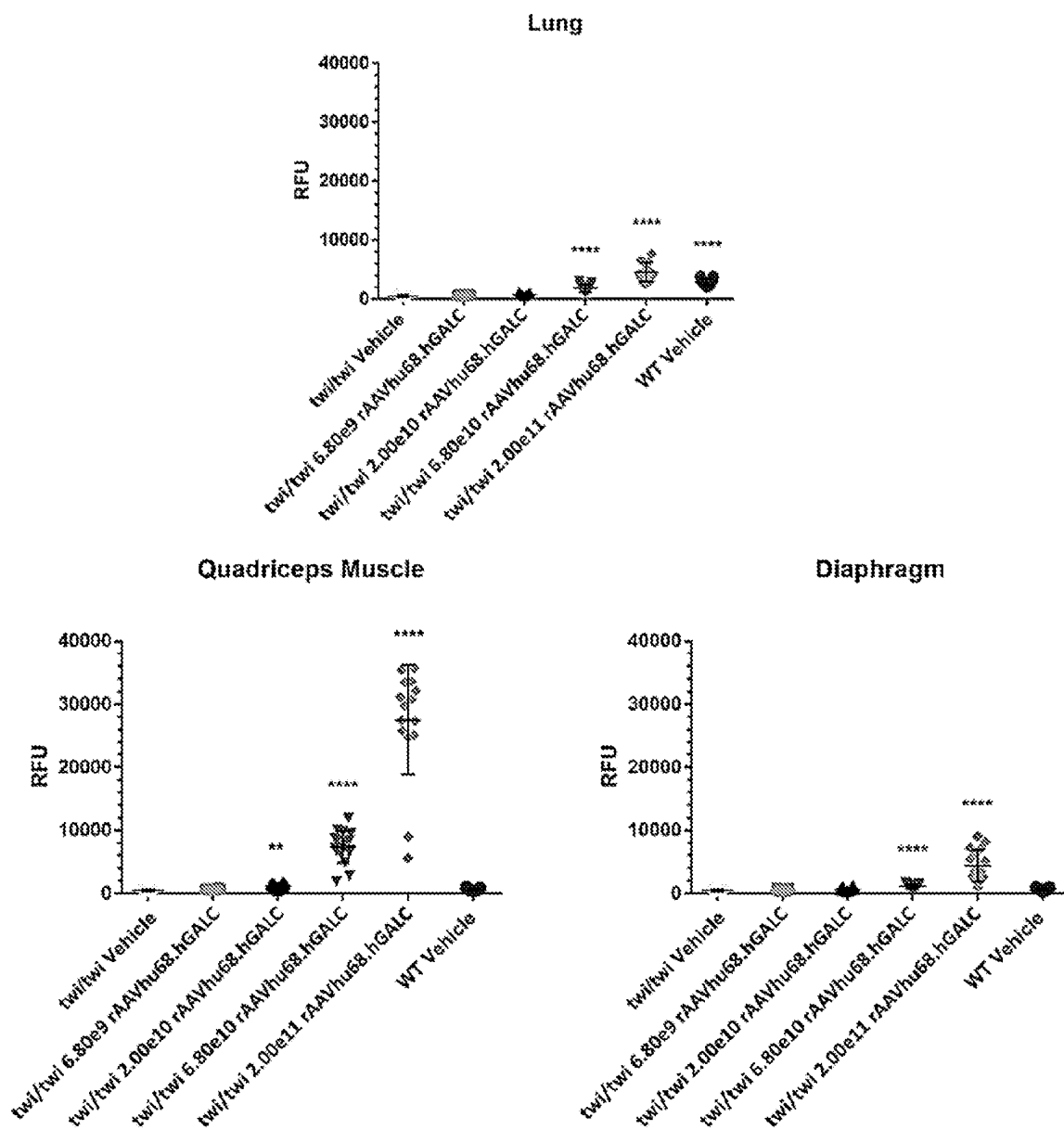


FIG. 66B

FIG. 66C

FIG. 67B

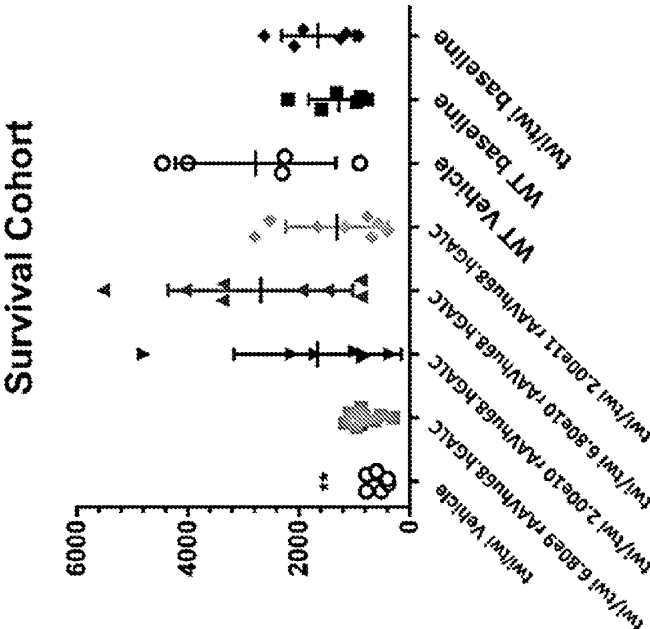


FIG. 67A

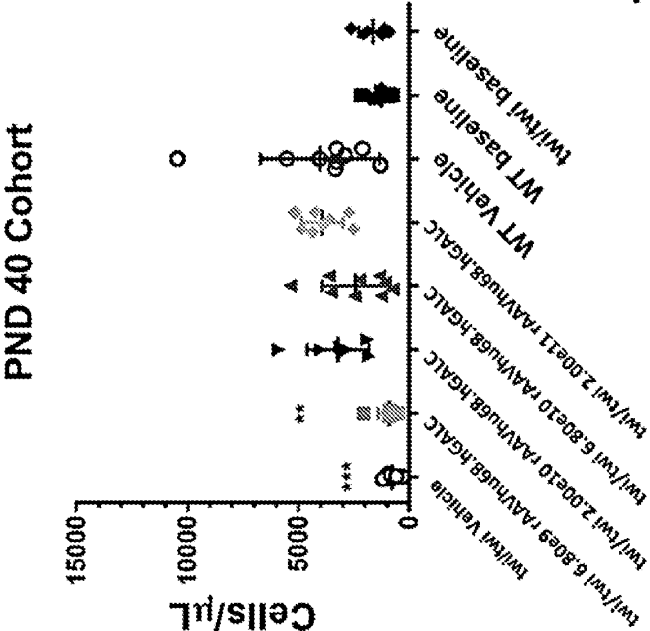


FIG. 68A

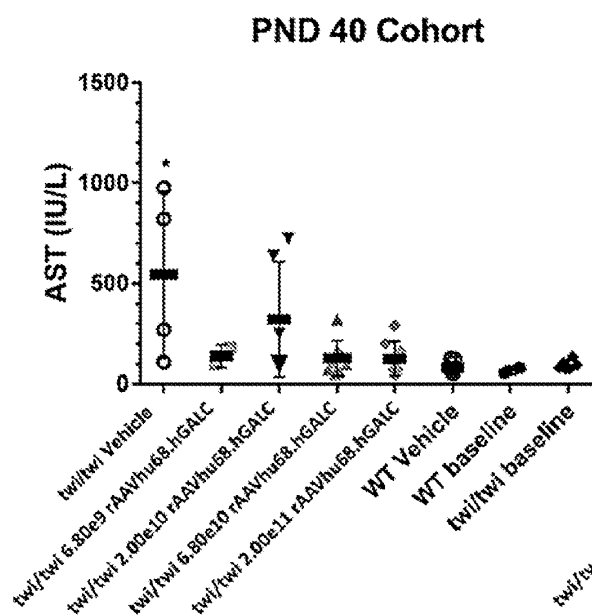


FIG. 68B

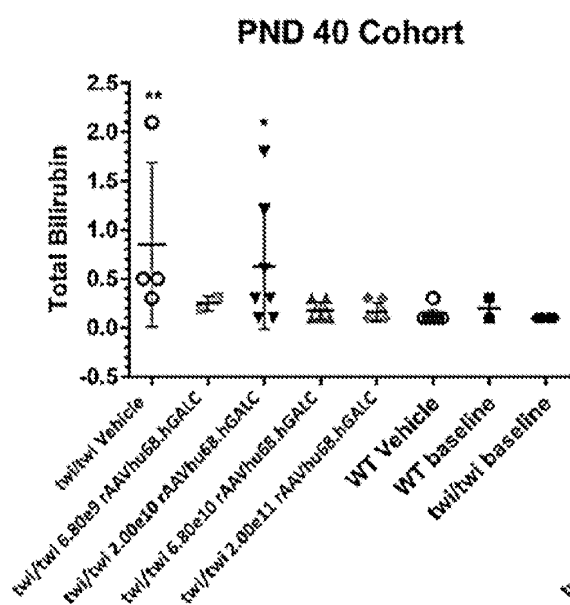
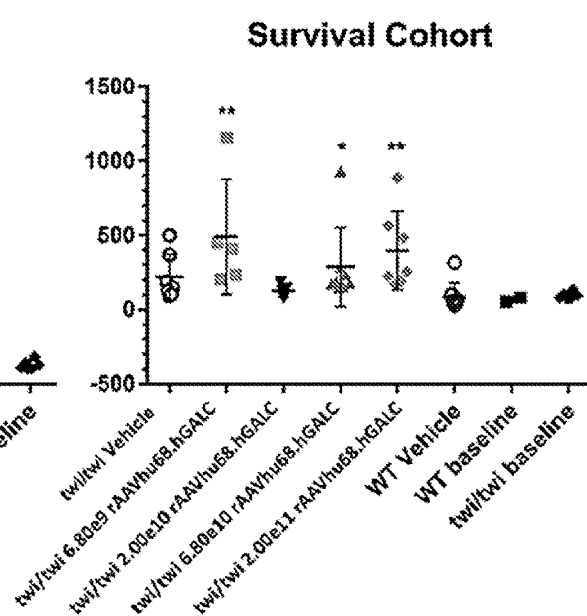


FIG. 68C

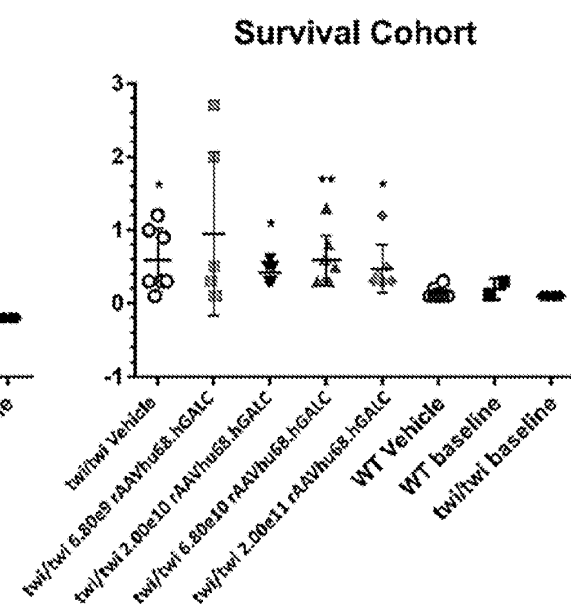


FIG. 68D

FIG. 69A

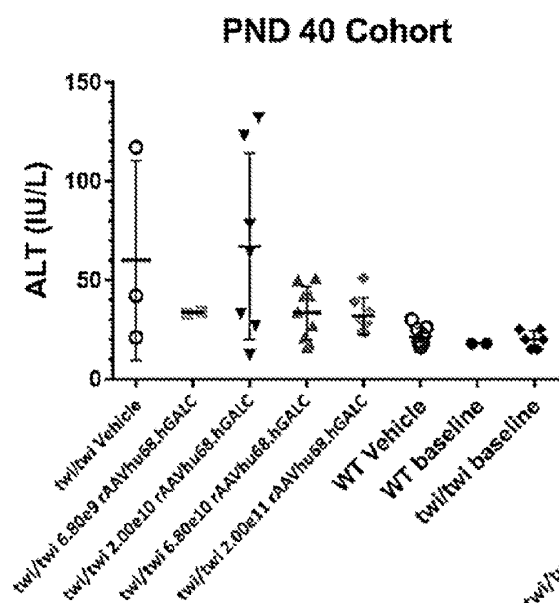


FIG. 69B

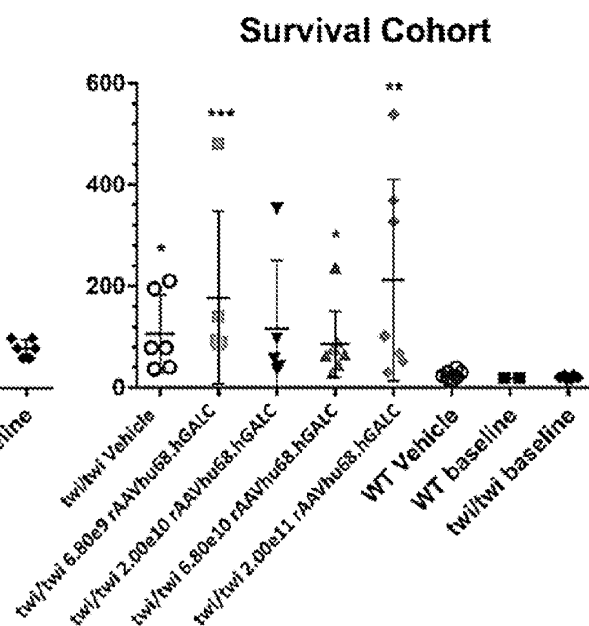


FIG. 70A

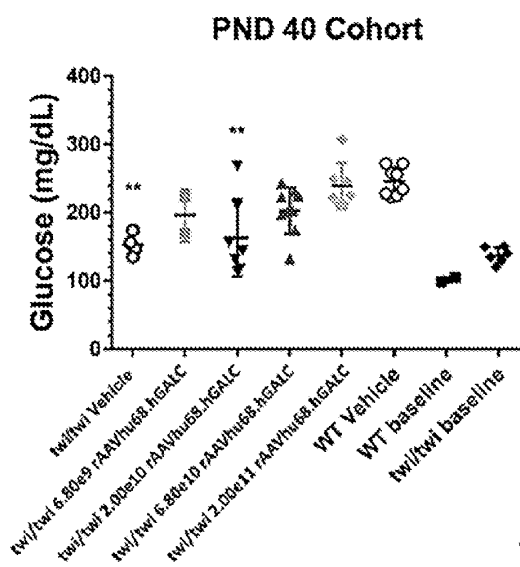


FIG. 70B

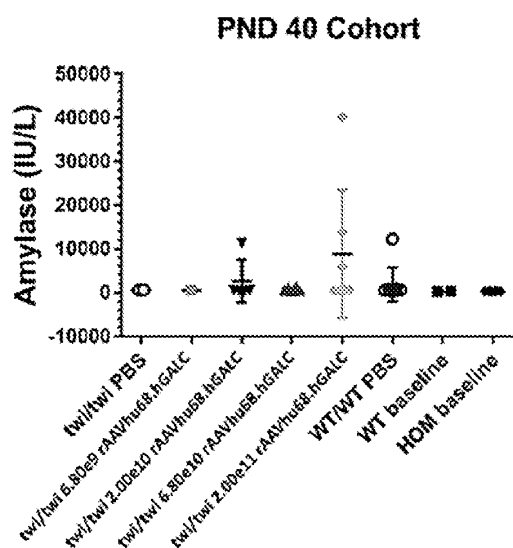
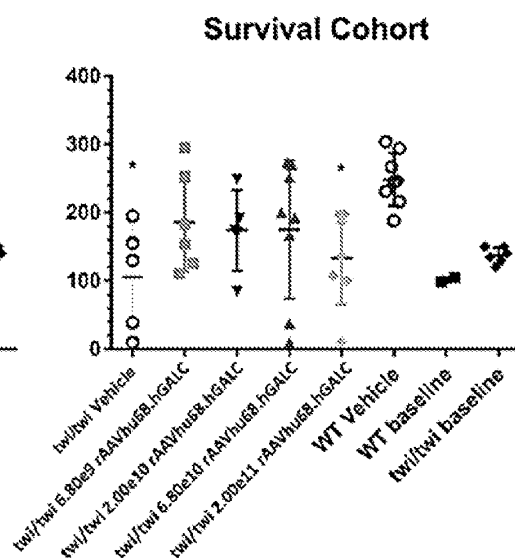


FIG. 70C

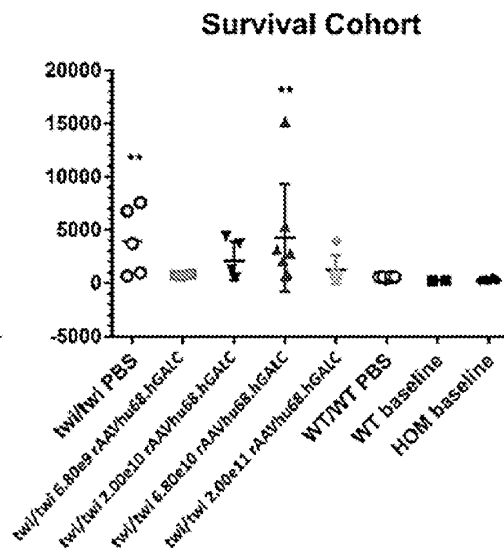


FIG. 70D

FIG. 71

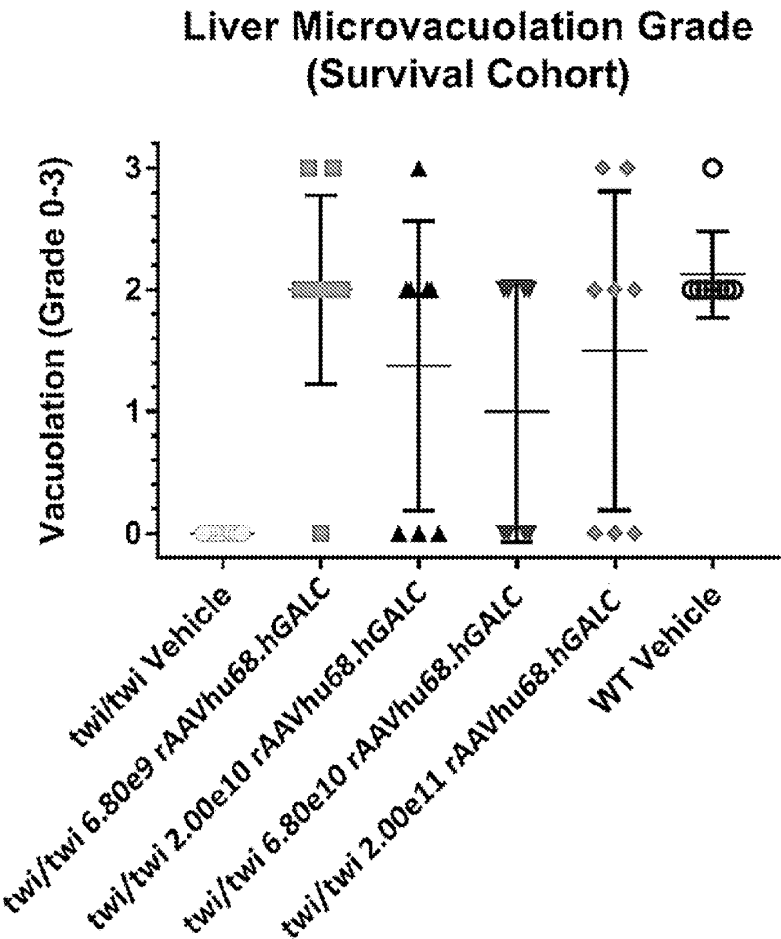


FIG. 72A

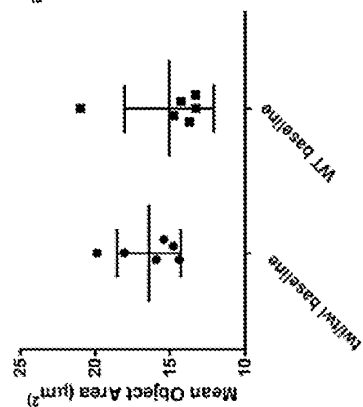


FIG. 72B

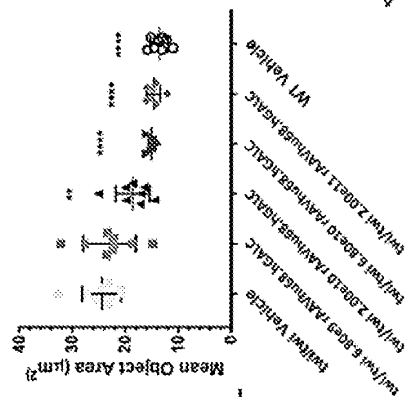
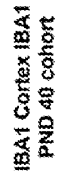
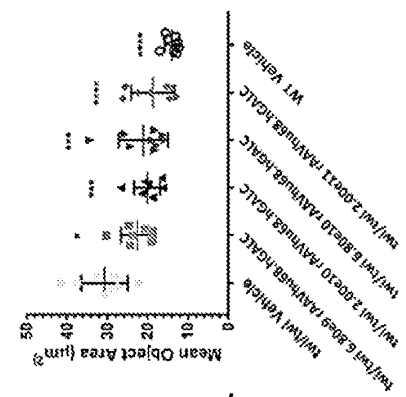
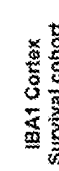
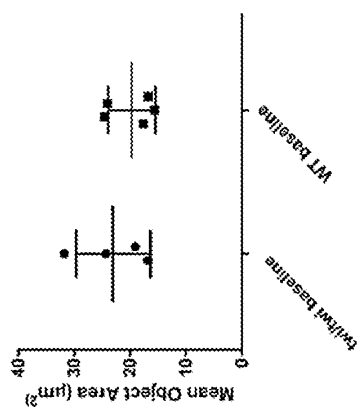
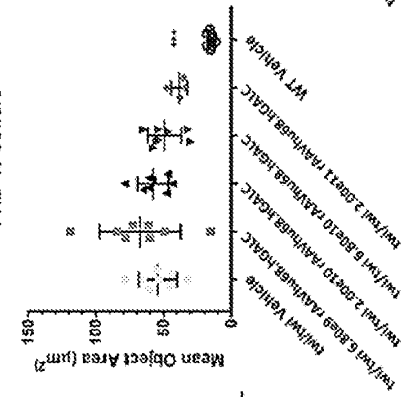


FIG. 72C



IBA1 cerebellum
Baseline

**IBA1 Cerebellum
PND 40 cohort**

IBA1 Cerebellum
Survival cohort

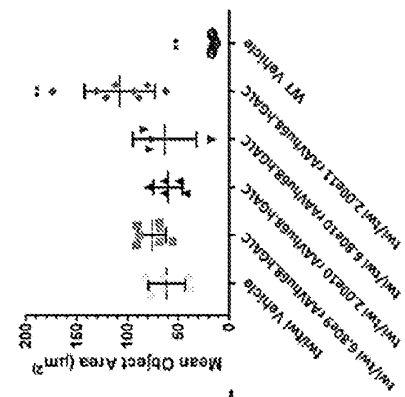


FIG. 72D

FIG. 72E

FIG. 72F

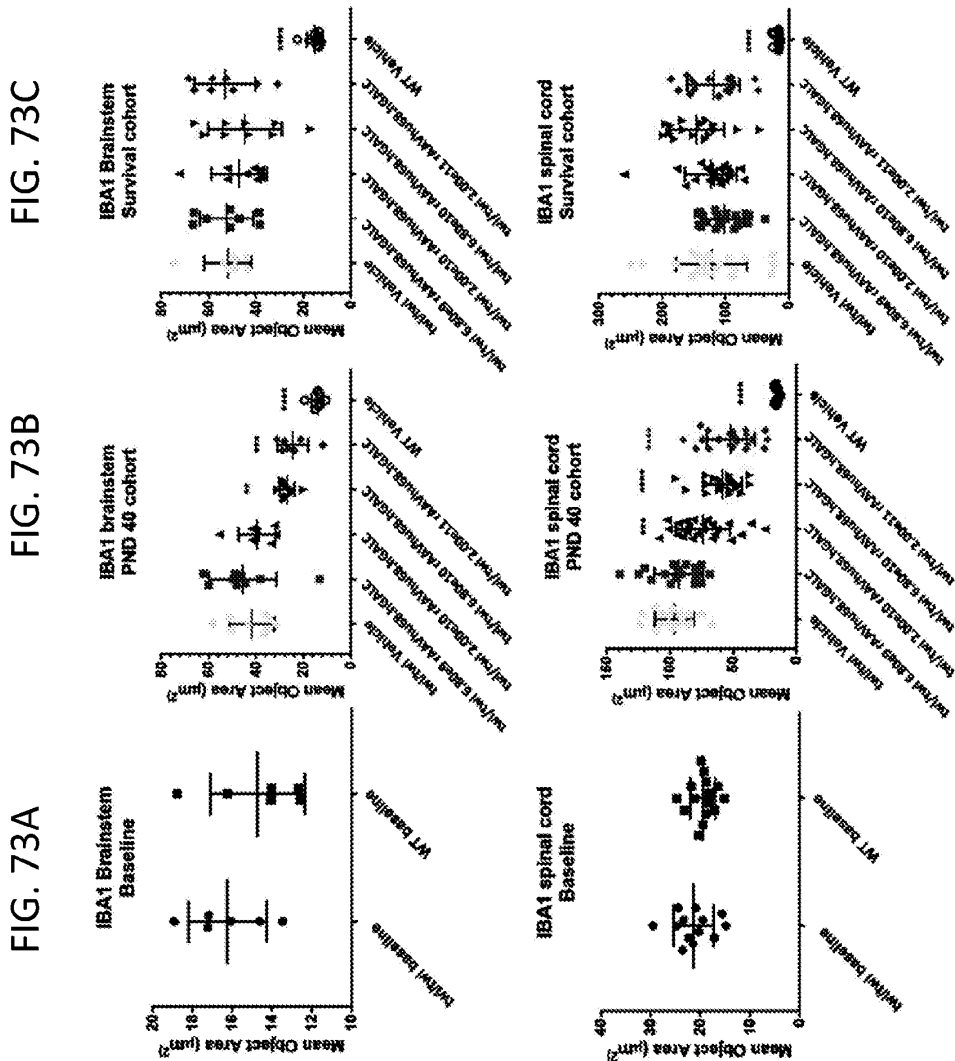


FIG. 73A

FIG. 73B

FIG. 73C

FIG. 73D

FIG. 73E

FIG. 73F

FIG. 74C

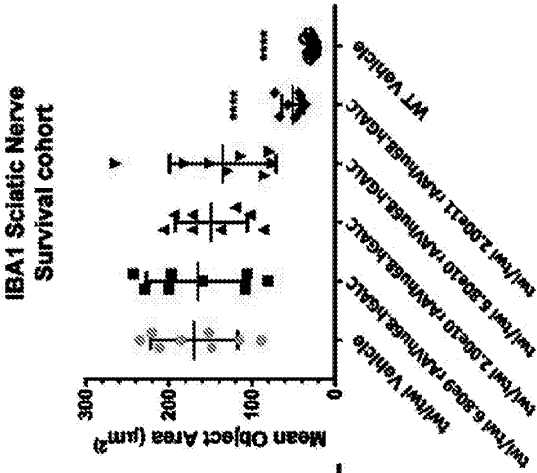


FIG. 74B

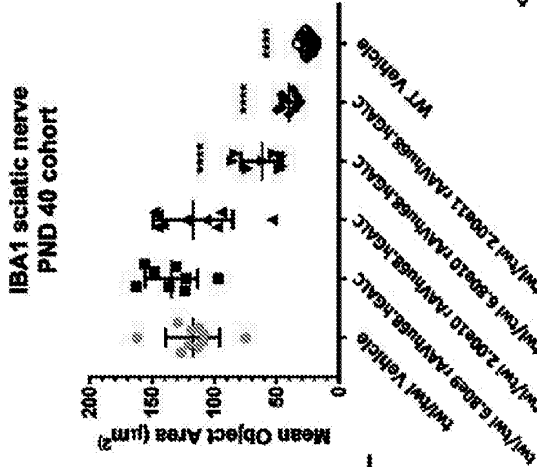


FIG. 74A

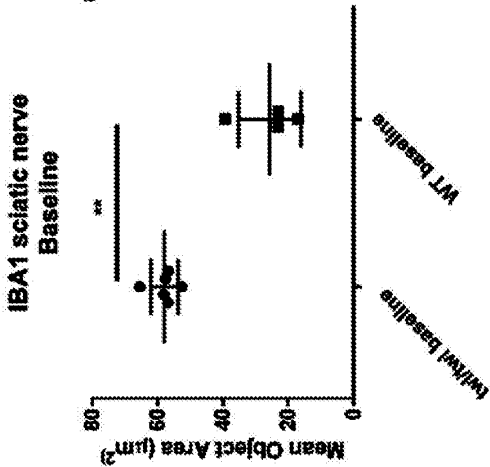


FIG. 75

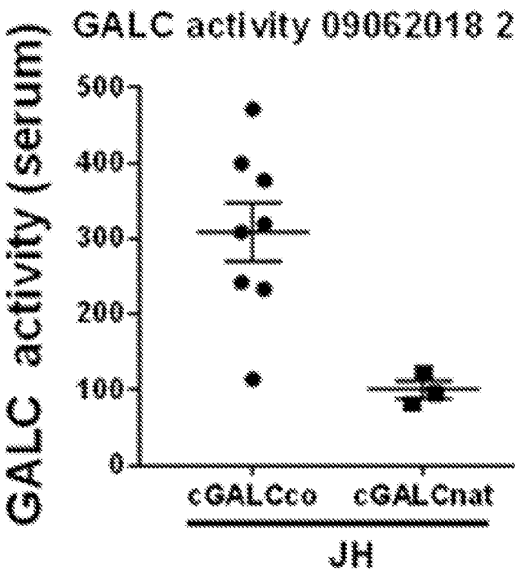


FIG. 76

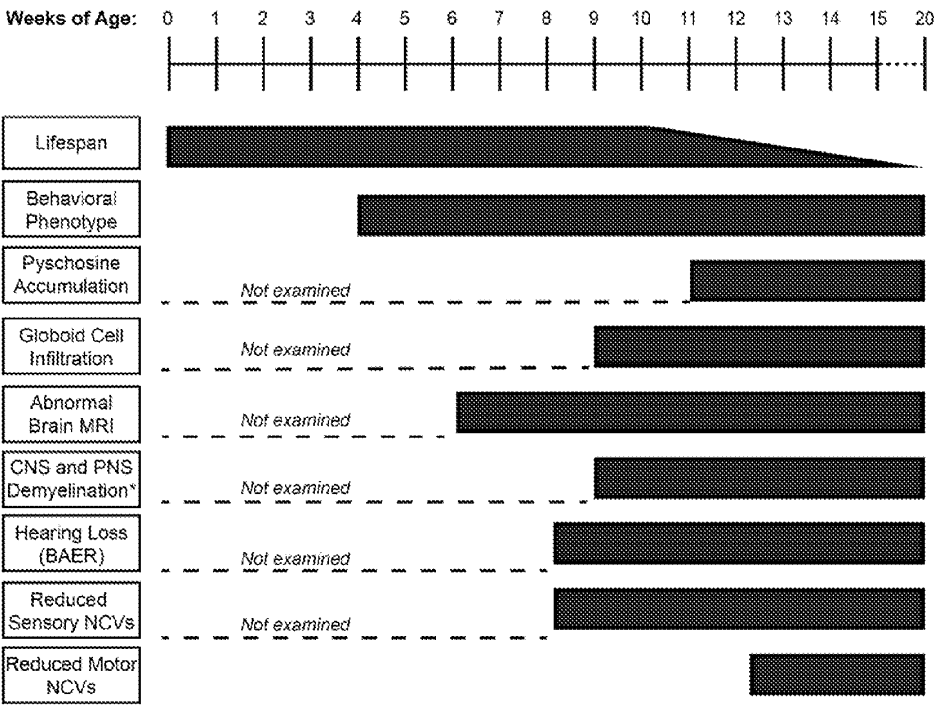


FIG. 77

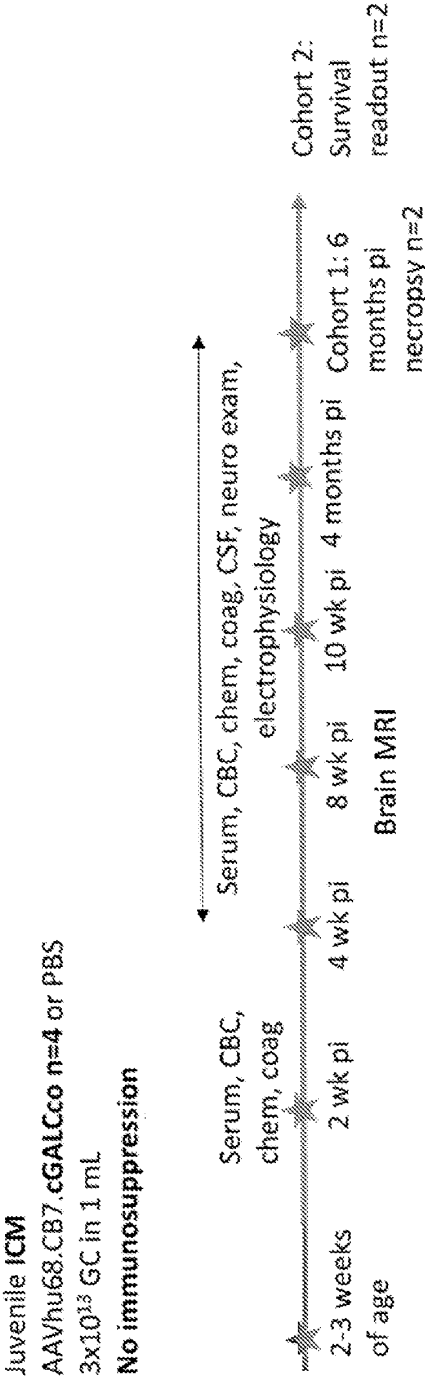


FIG. 78

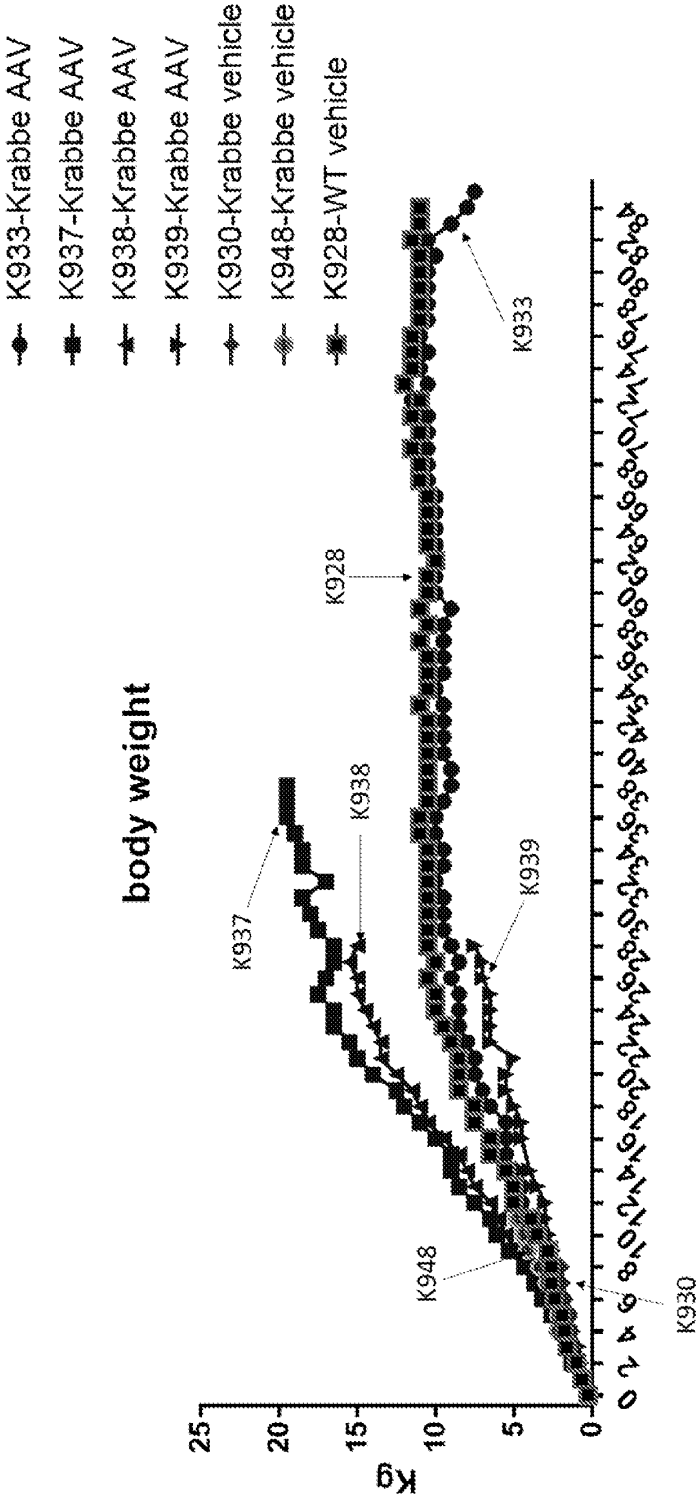


FIG. 79A

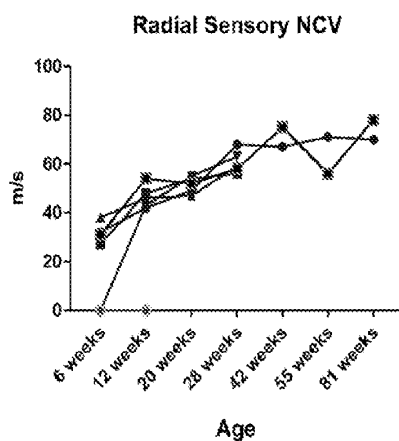


FIG. 79B

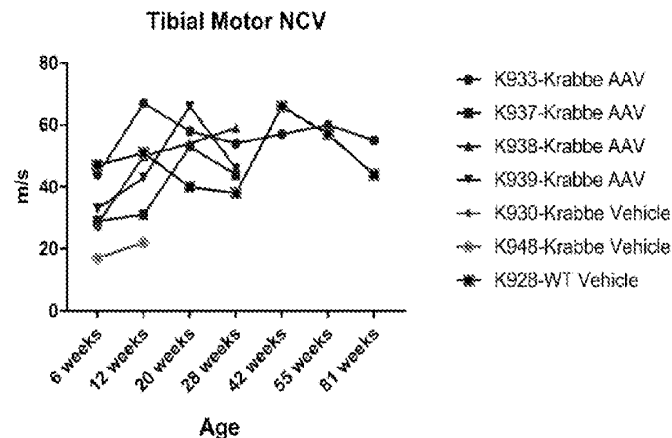
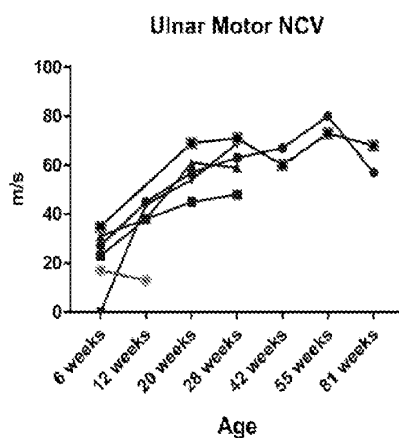
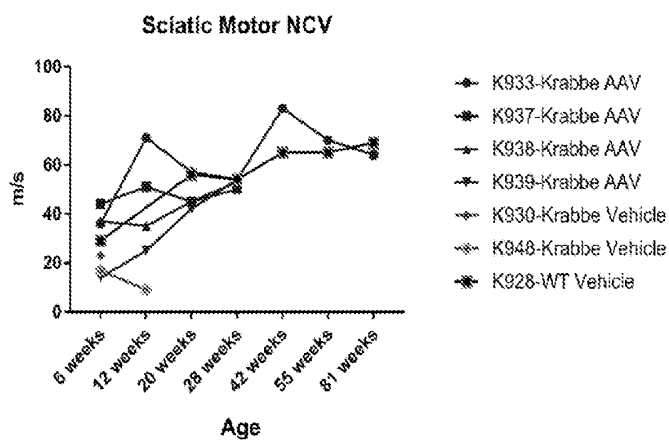


FIG. 79C

FIG. 79D

FIG. 80

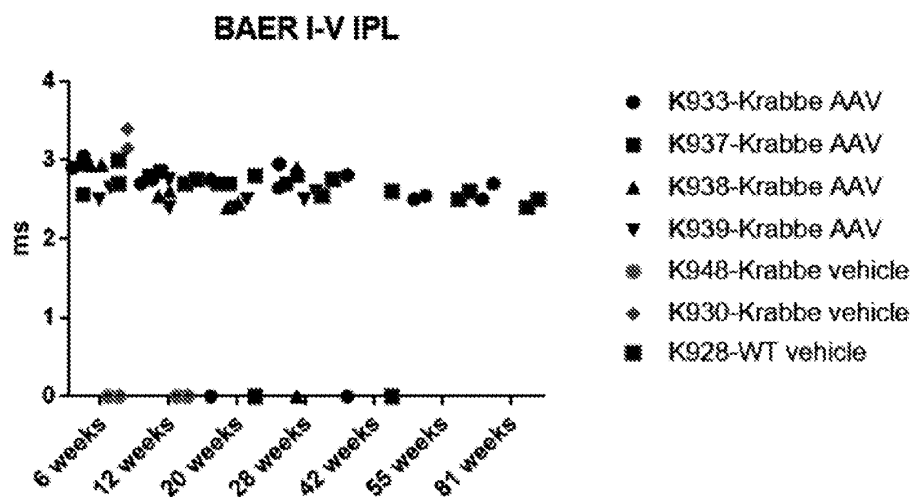


FIG. 81

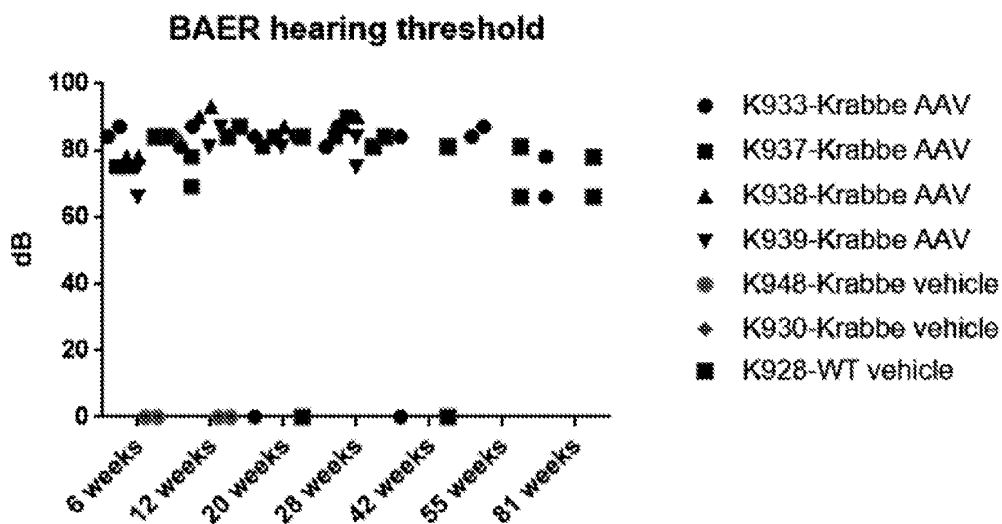


FIG. 82A

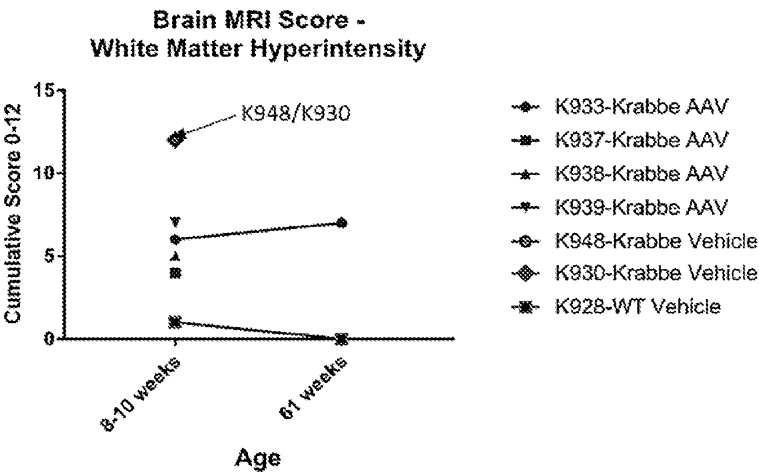
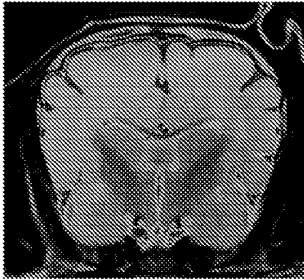


FIG. 82B
Krabbe sham



Hypertintense WM

FIG. 82C
Krabbe AAV



Isointense WM

FIG. 82D

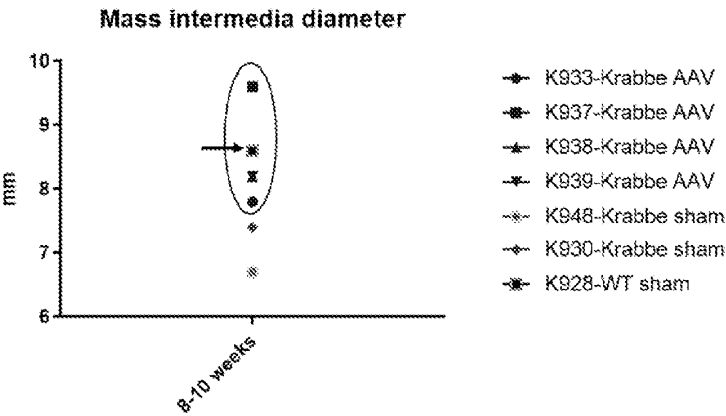


FIG. 83

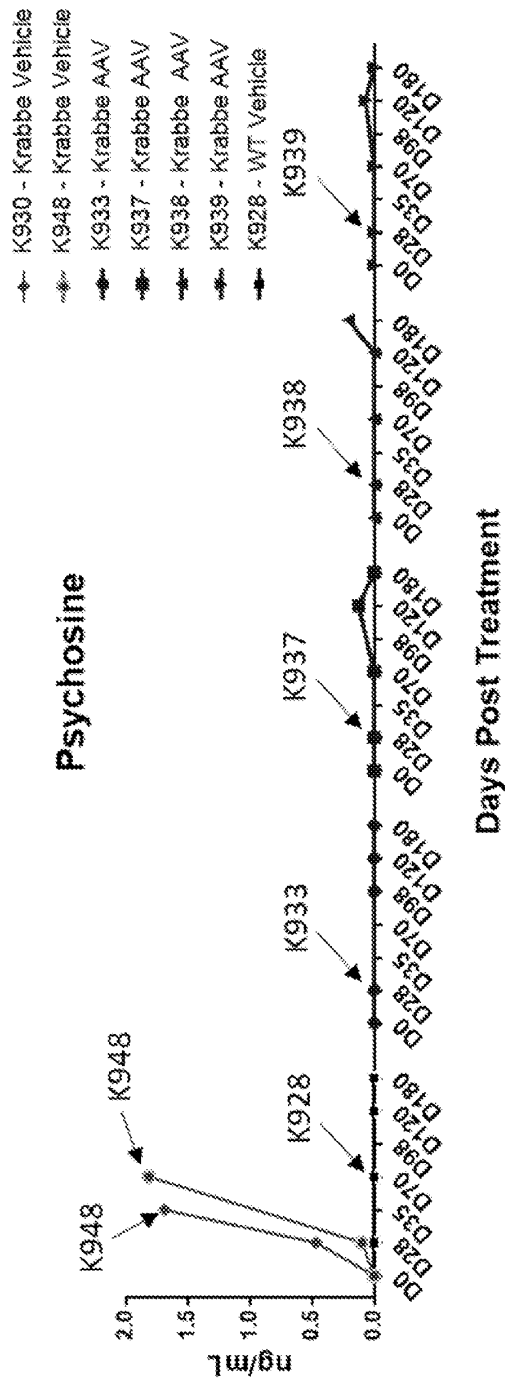


FIG. 84

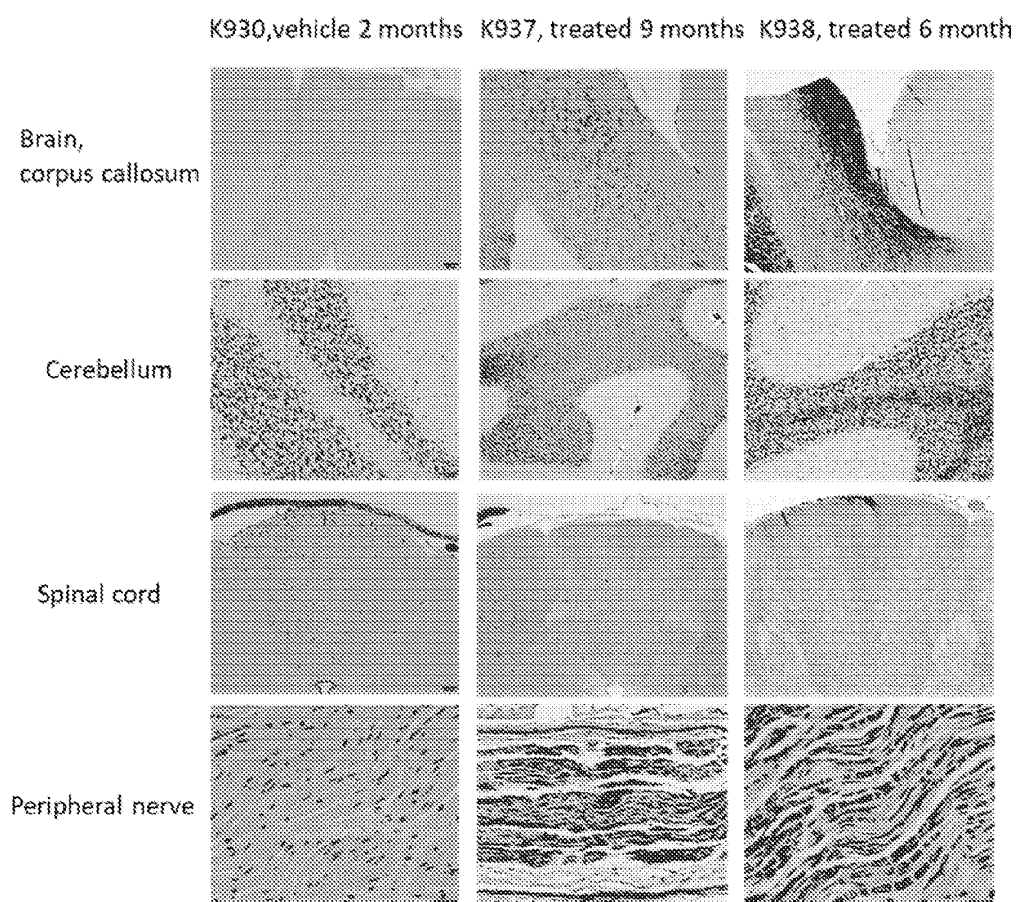


FIG. 85A

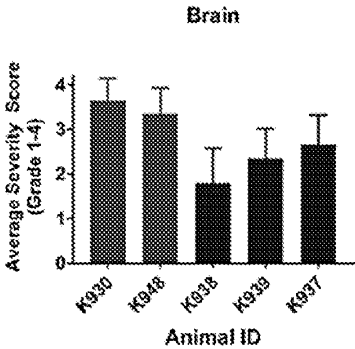


FIG. 85B

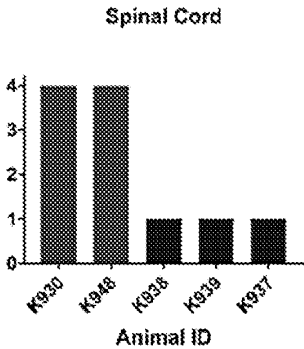


FIG. 85C

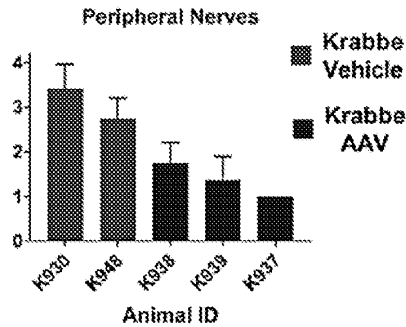


FIG. 85D

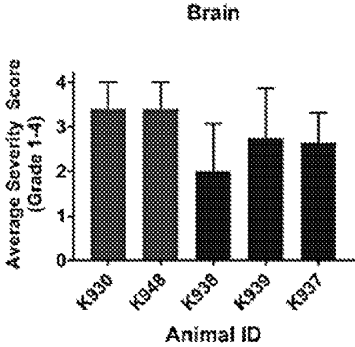


FIG. 85E

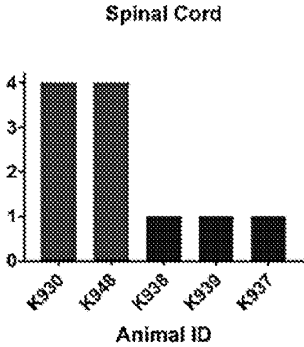


FIG. 85F

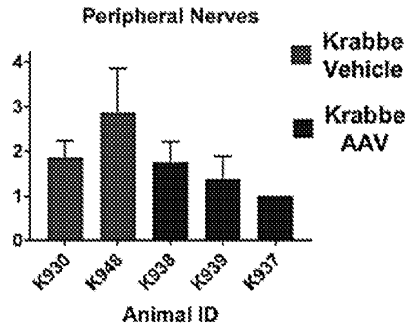


FIG. 86A

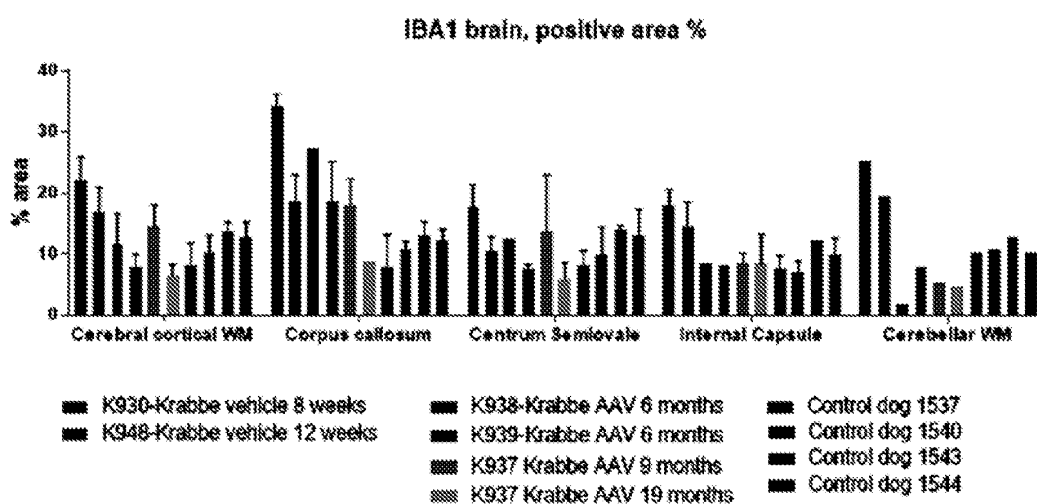


FIG. 86B

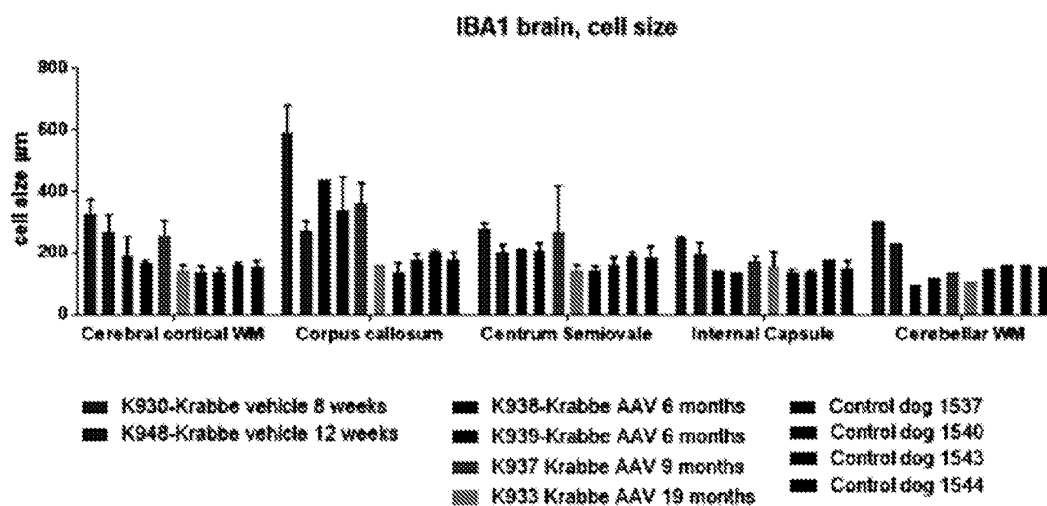


FIG. 87A

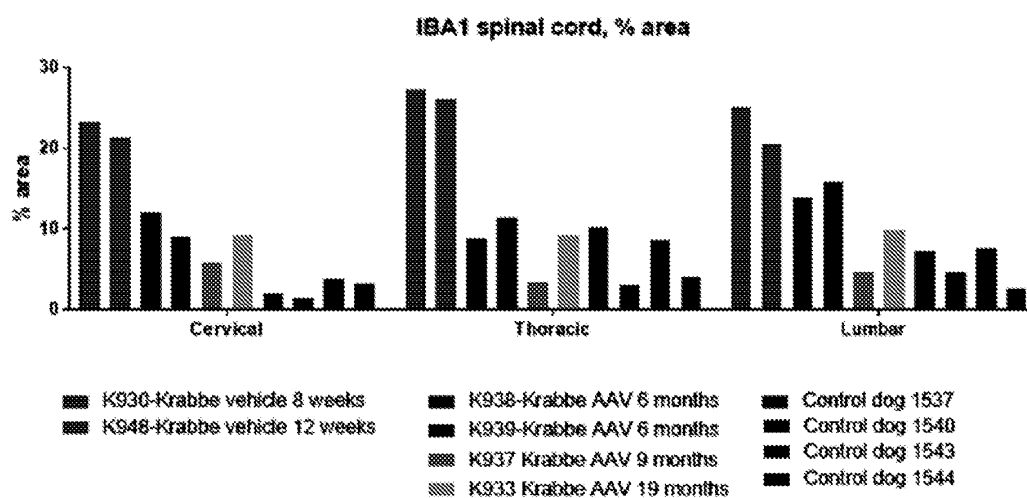


FIG. 87B

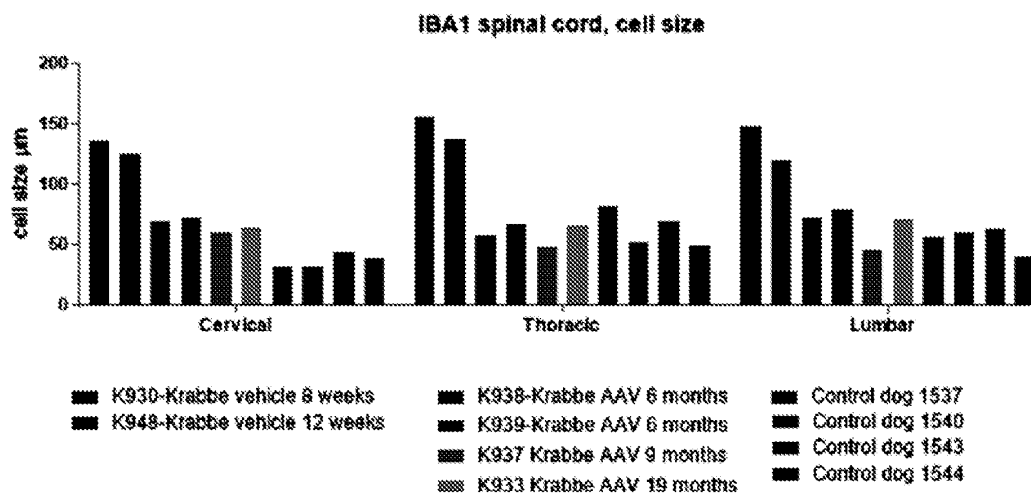
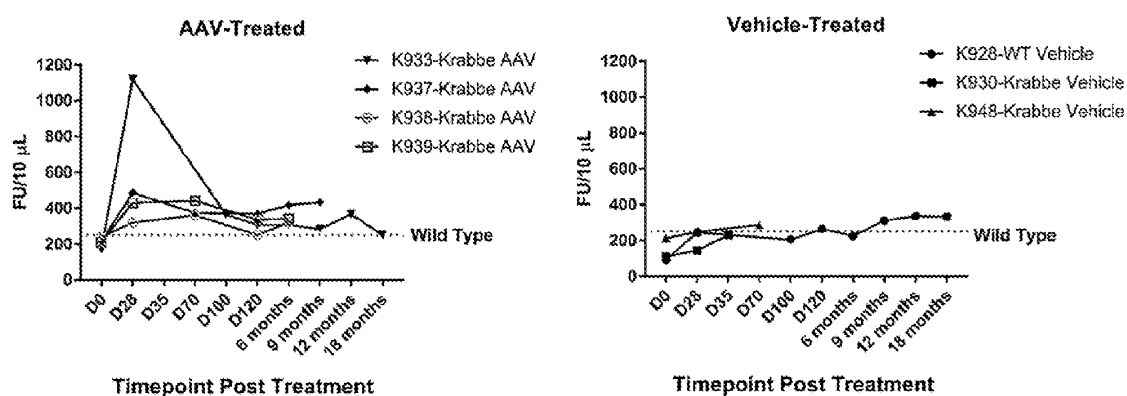


FIG. 88A

FIG. 88B

GALC Activity - CSF



GALC Activity - Serum

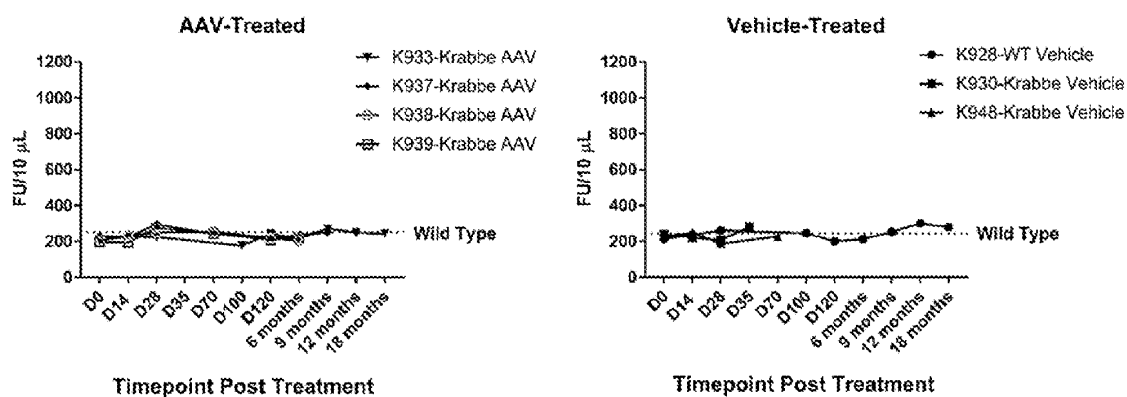


FIG. 88C

FIG. 88D

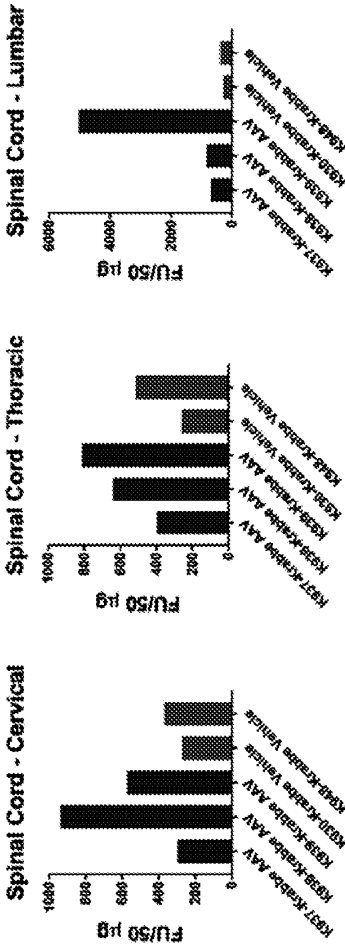
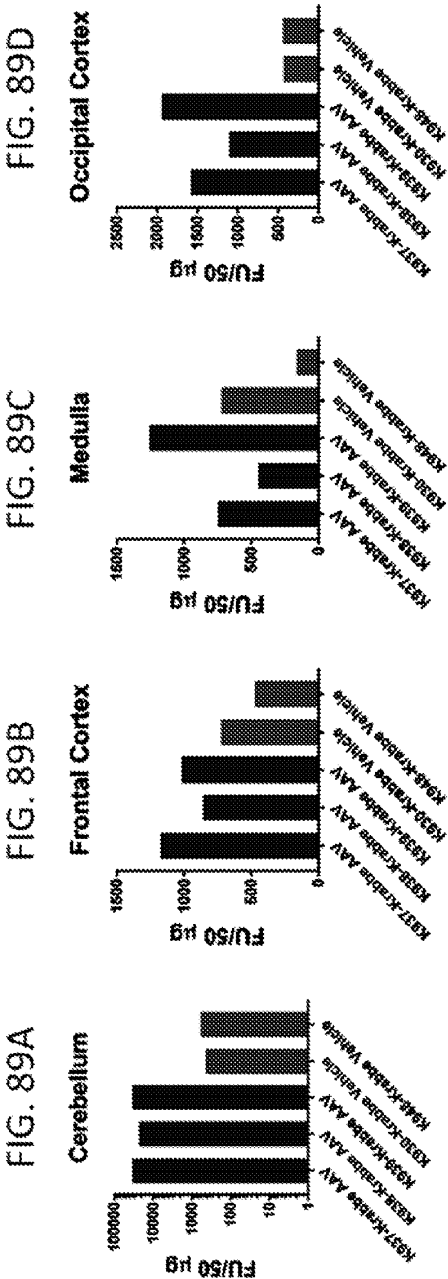


FIG. 90A

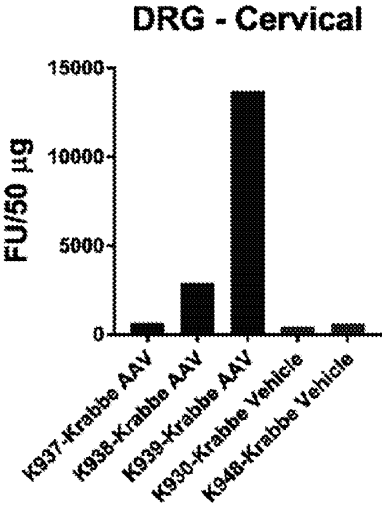
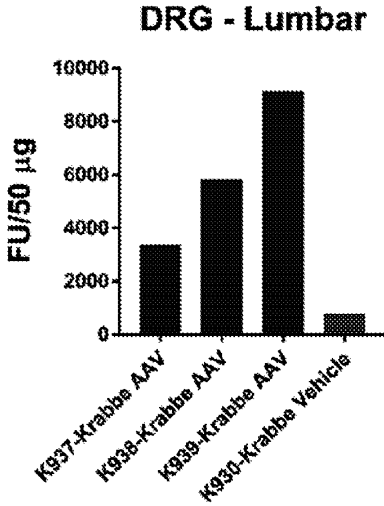
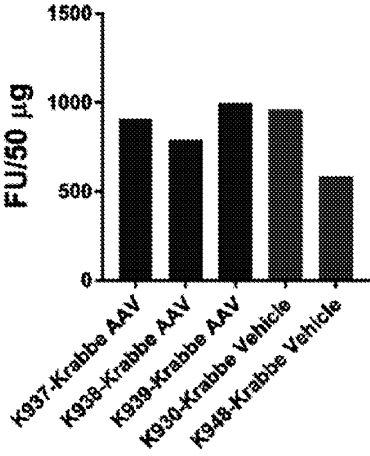


FIG. 90B



Sciatic Nerve



Median Nerve

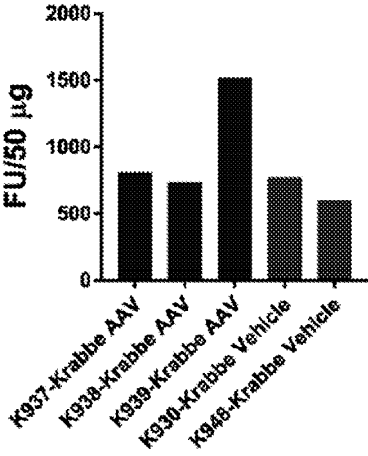


FIG. 90C

FIG. 90D

FIG. 91A

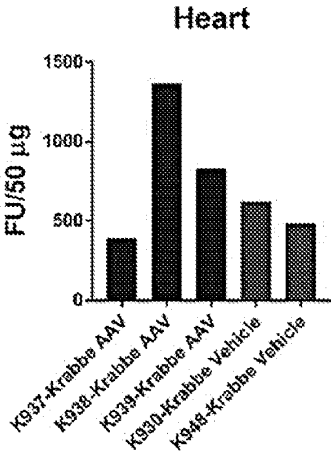


FIG. 91B

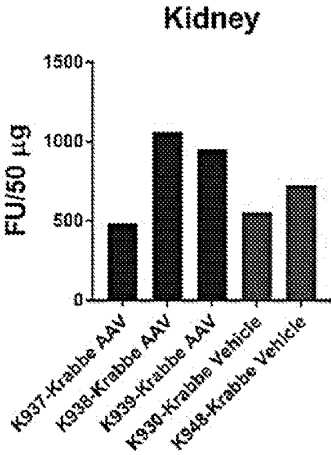
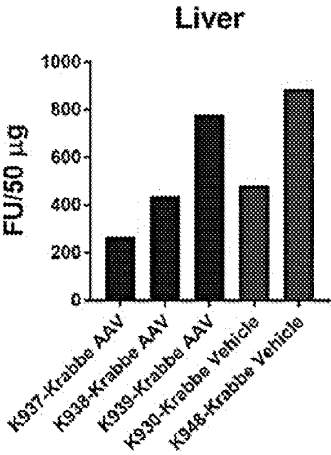
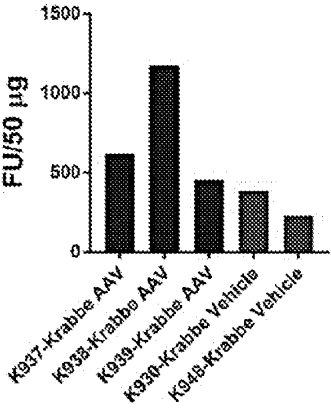


FIG. 91C



Diaphragm



Quadriceps Femoris

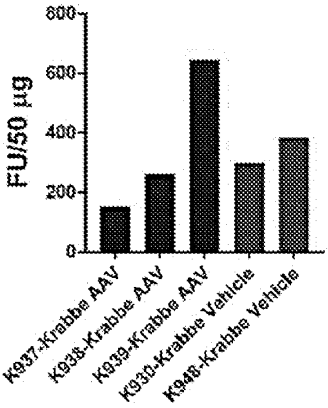


FIG. 91D

FIG. 91E

FIG. 92

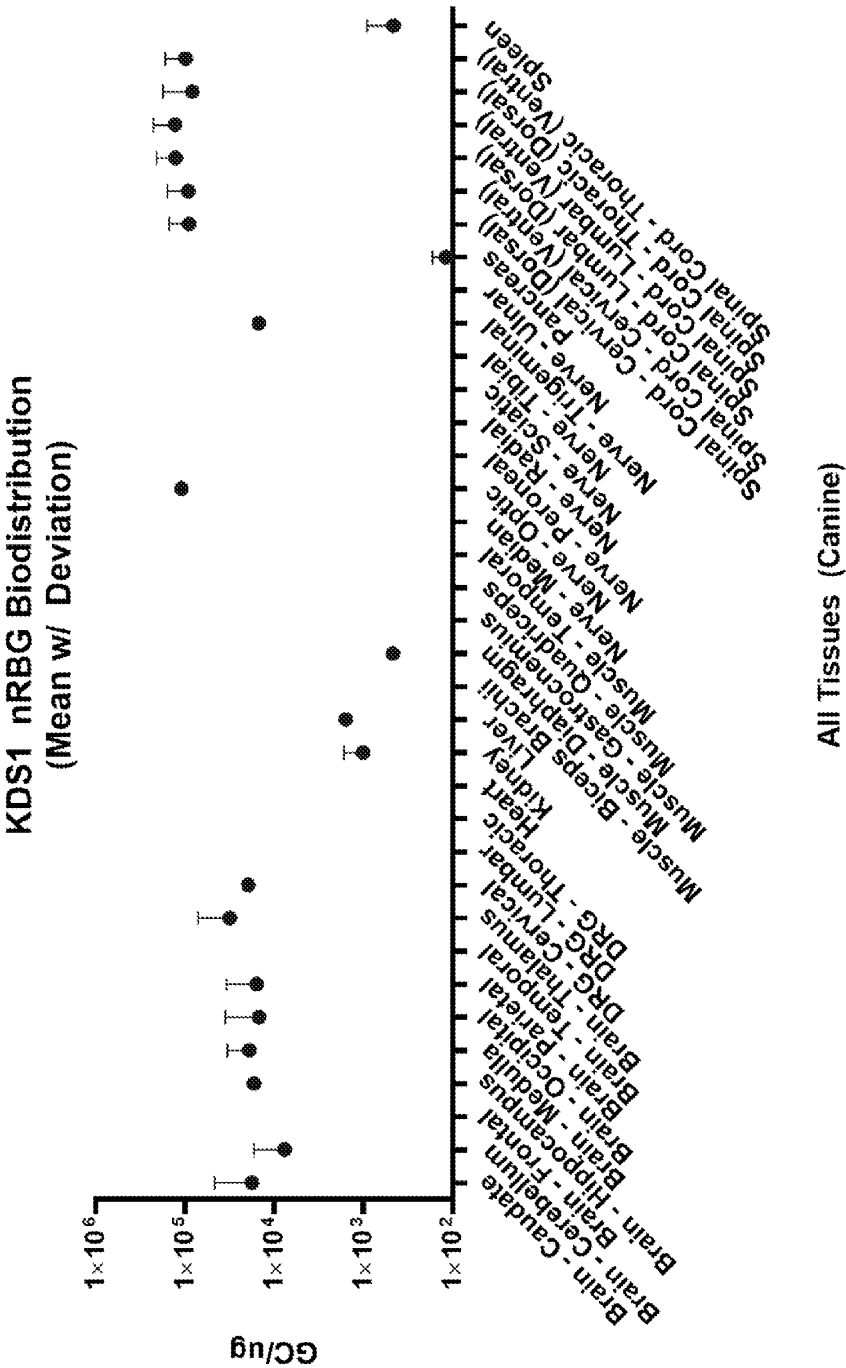
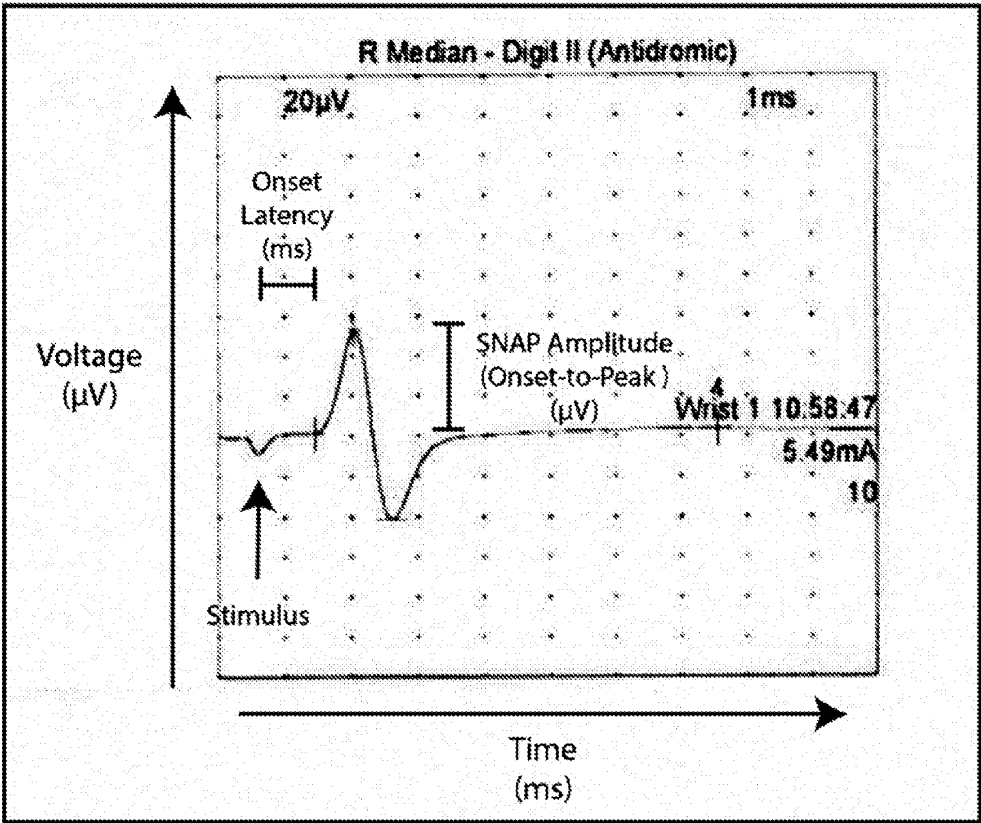


FIG. 93



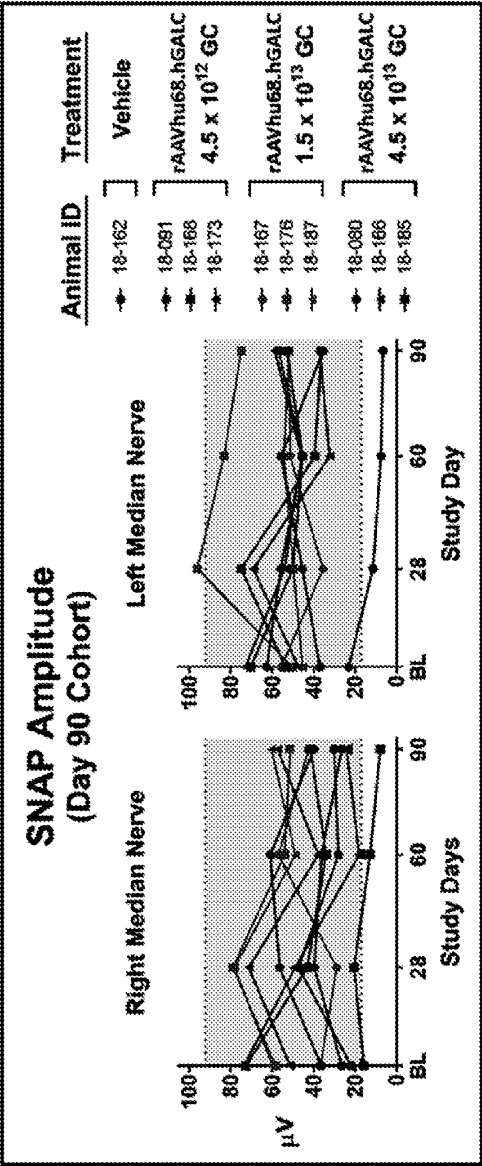


FIG. 94A

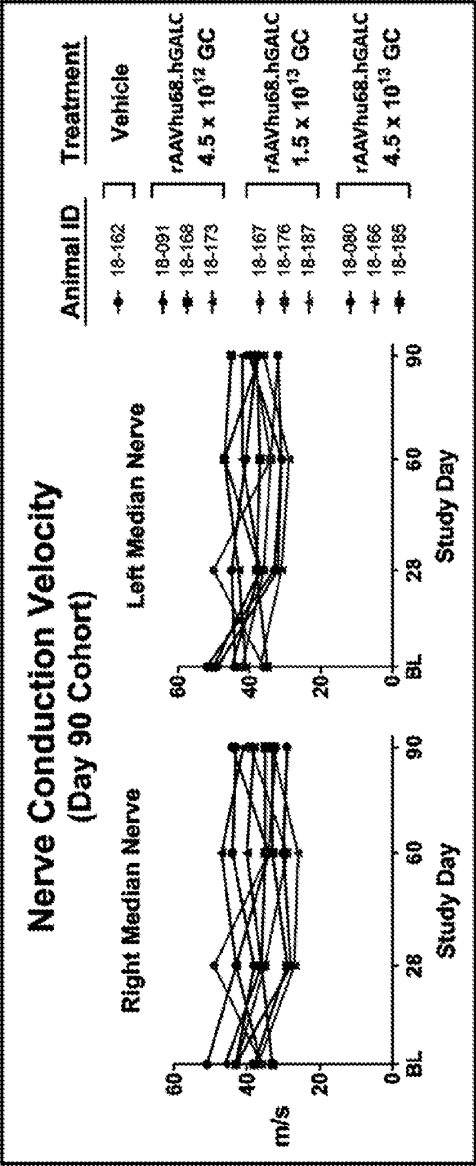


FIG. 94B

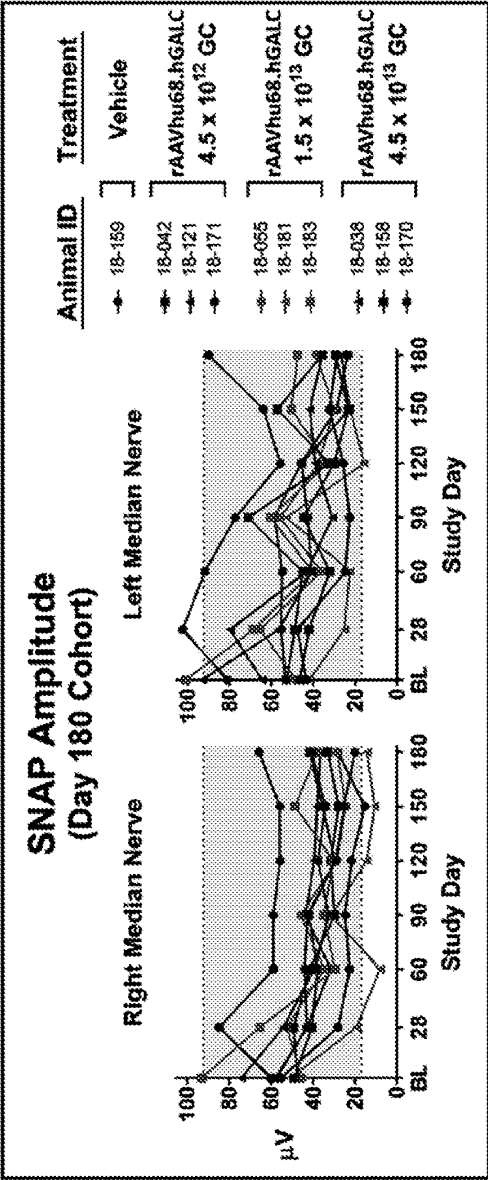


FIG. 95A

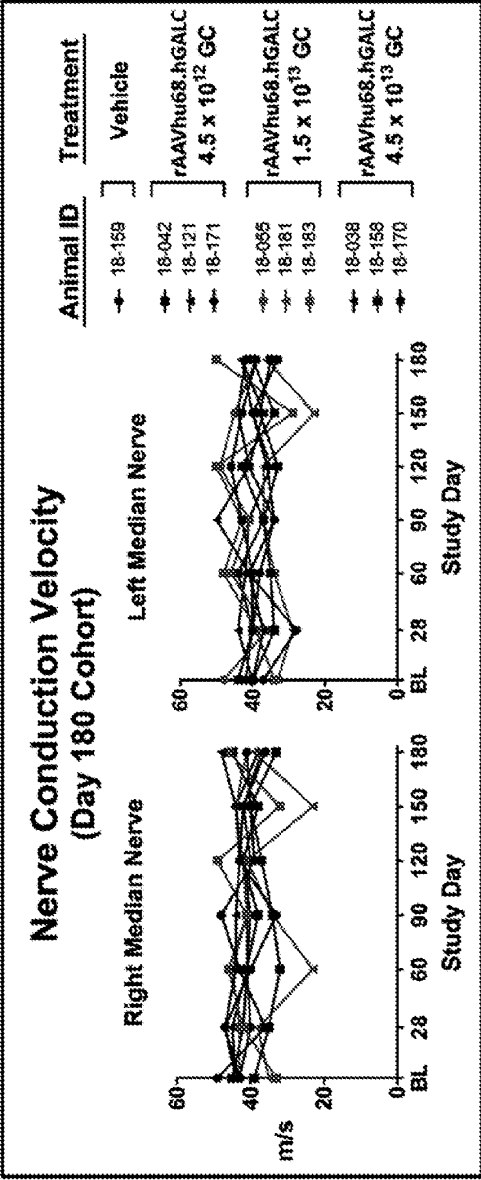


FIG. 95B

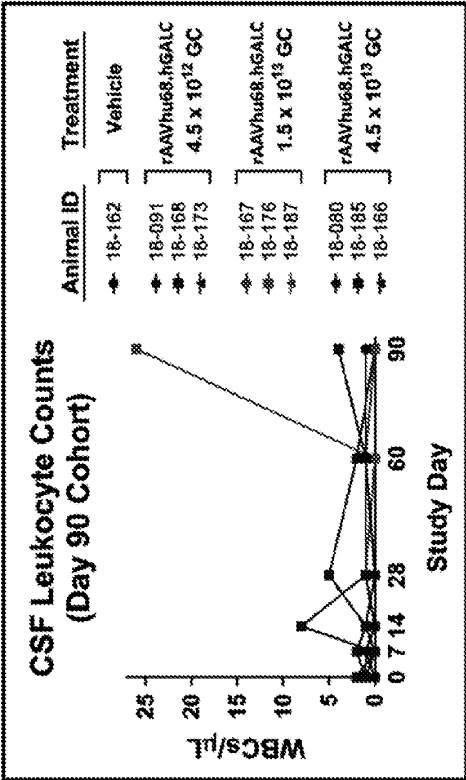


FIG. 96A

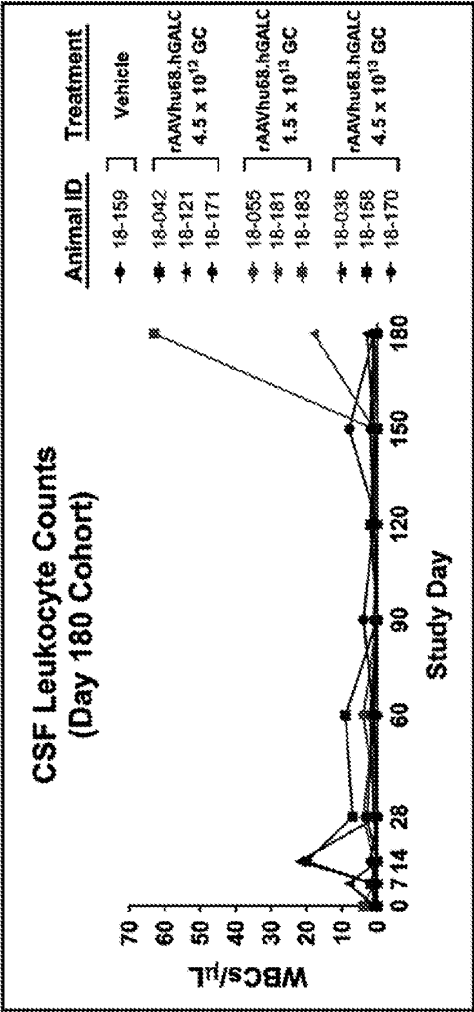


FIG. 96B

FIG. 96C

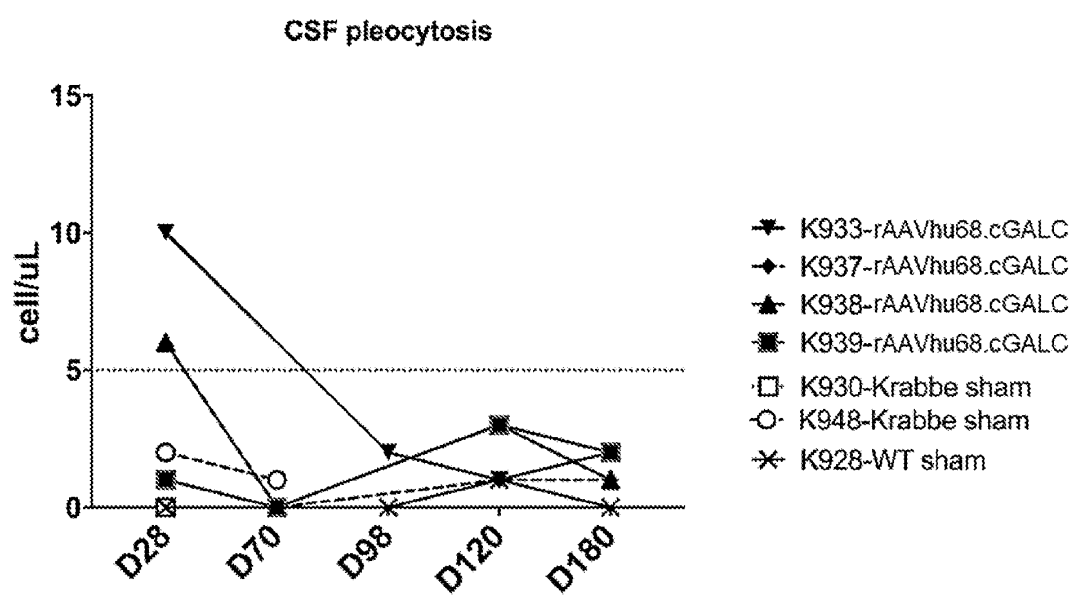
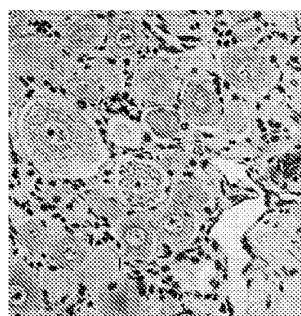


FIG. 96D



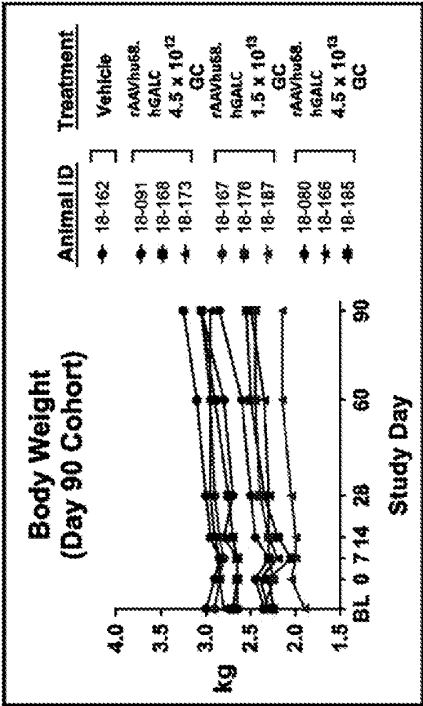


FIG. 97A

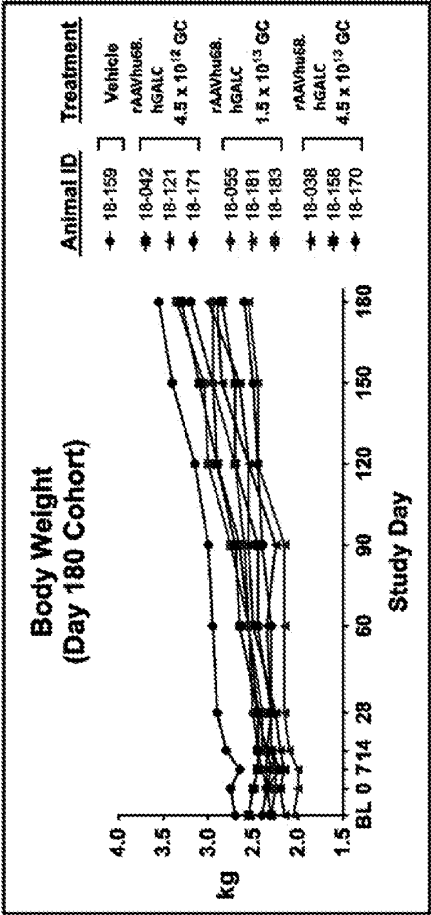


FIG. 97B

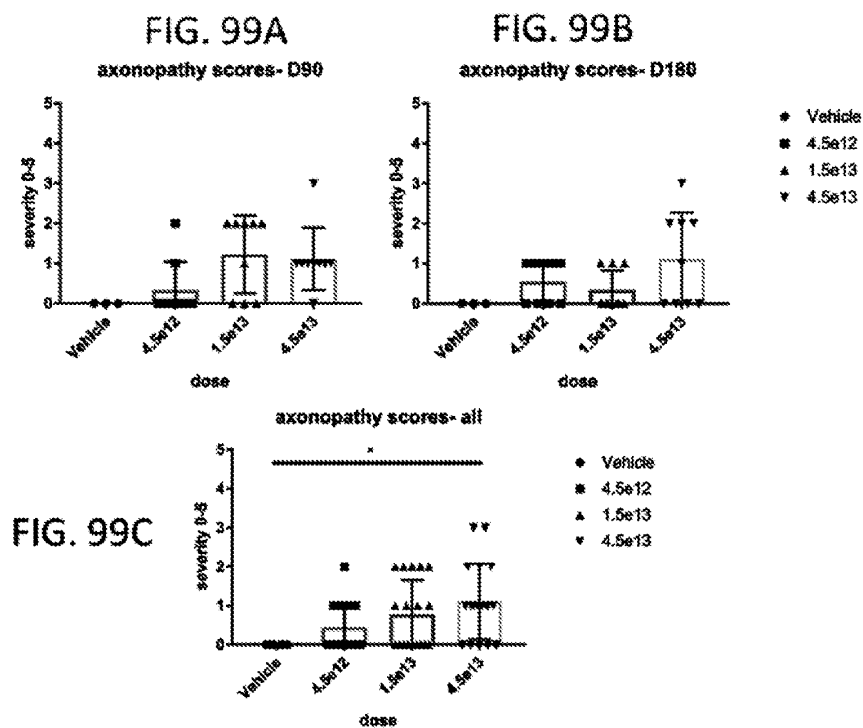
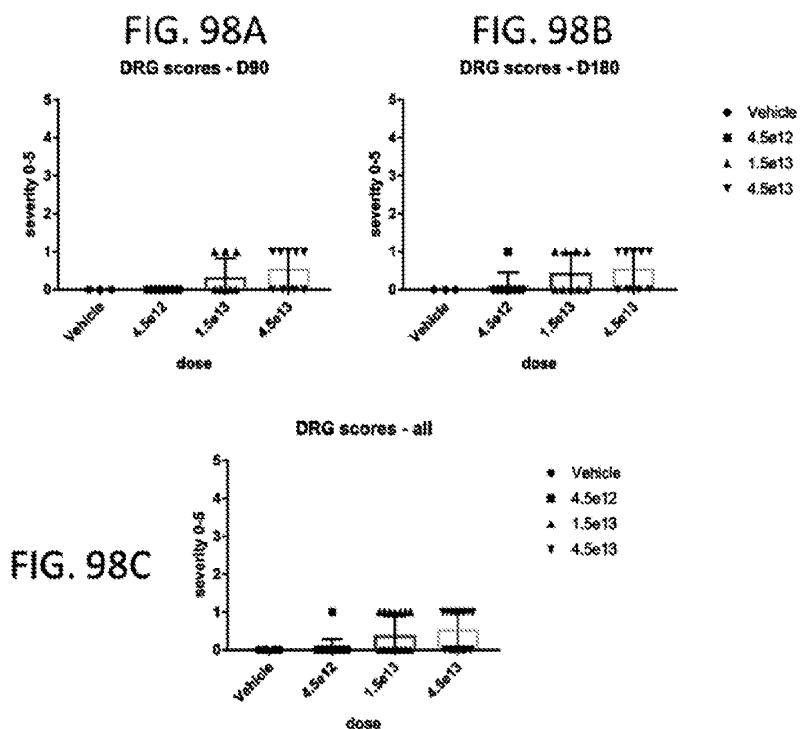


FIG. 100A

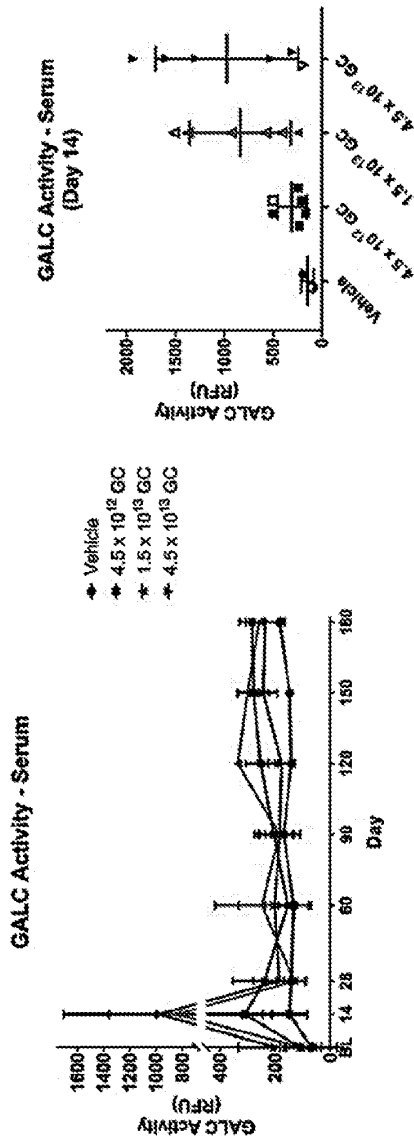


FIG. 100B

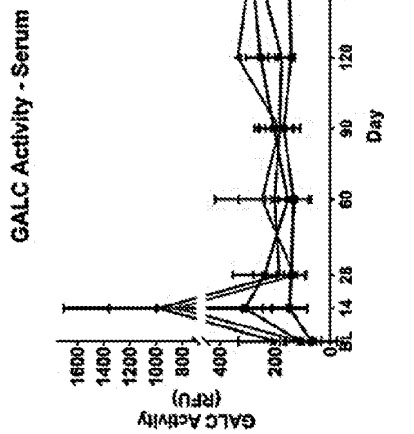


FIG. 100C

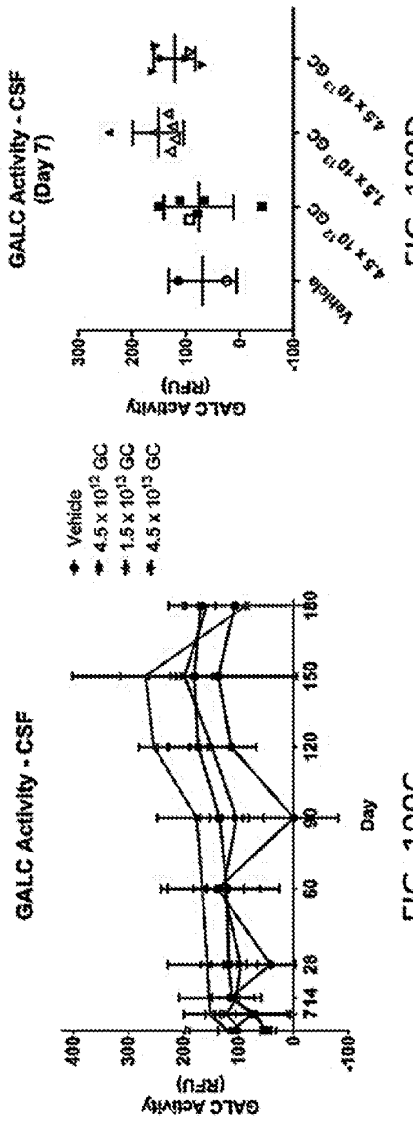


FIG. 100D

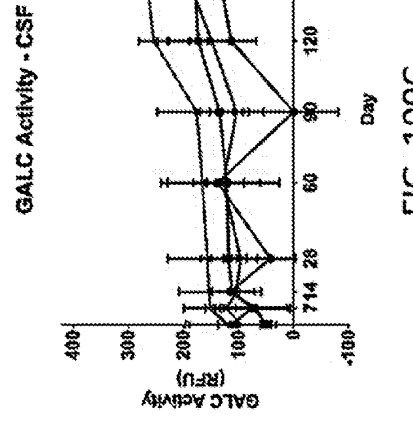


FIG. 101A
Anti-Human GALC Antibodies

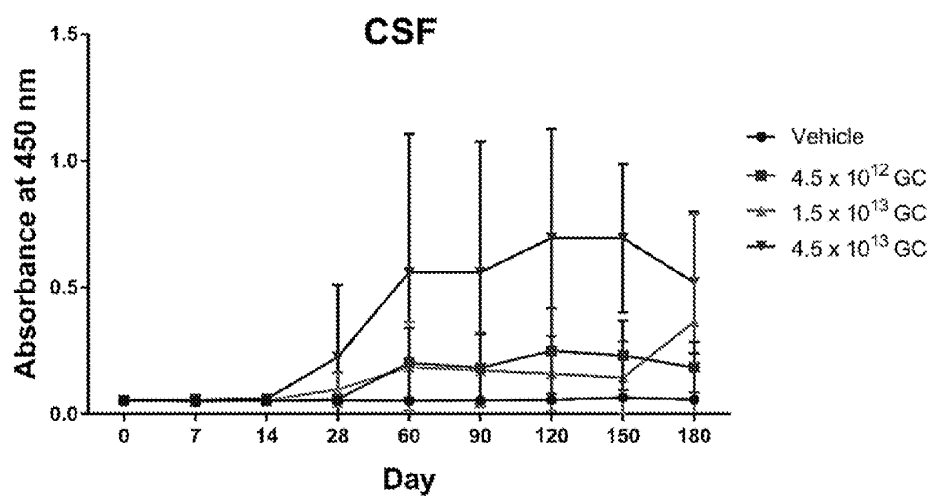
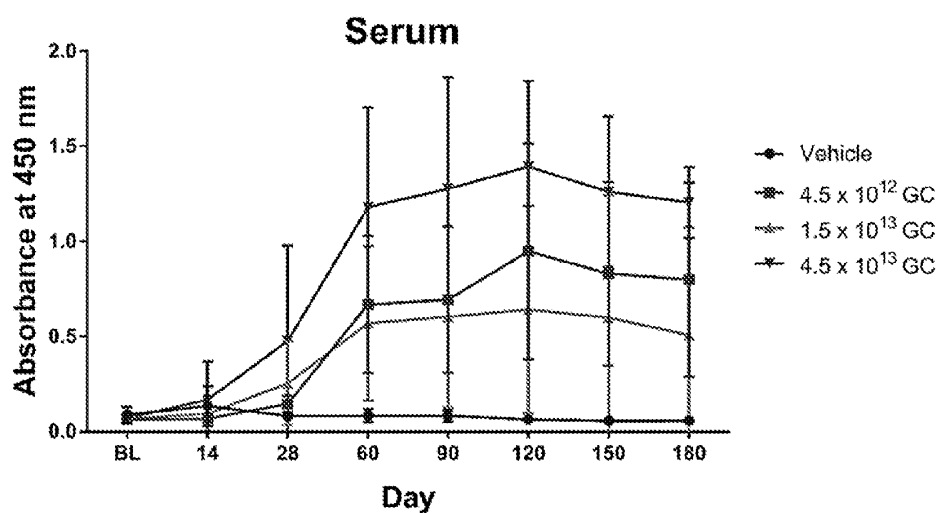


FIG. 101B