

FIG 1A



FIG 1B

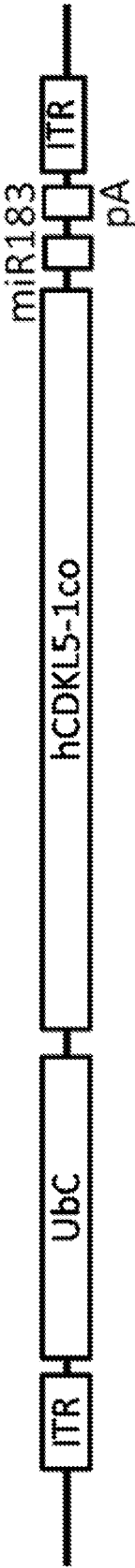


FIG 1C

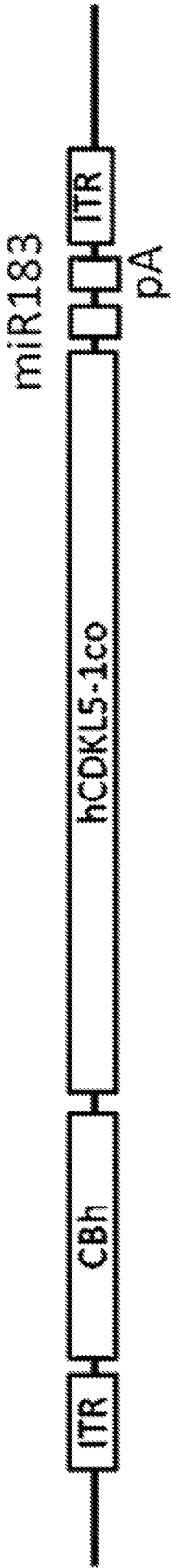


FIG 2A

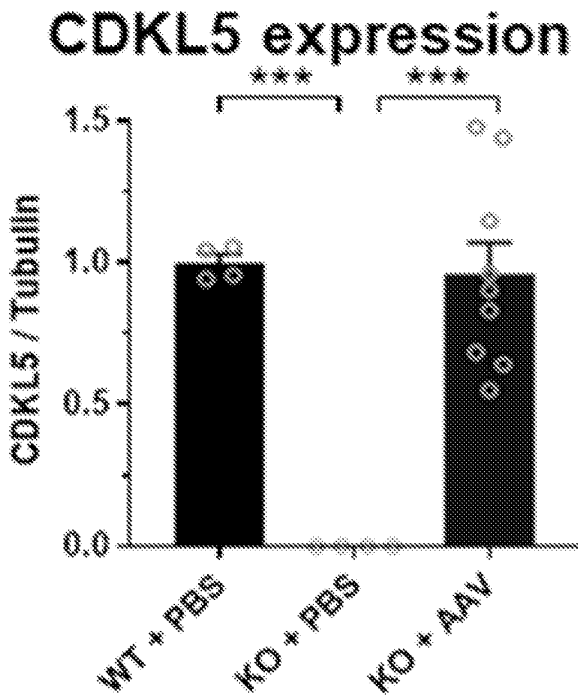


FIG 2B

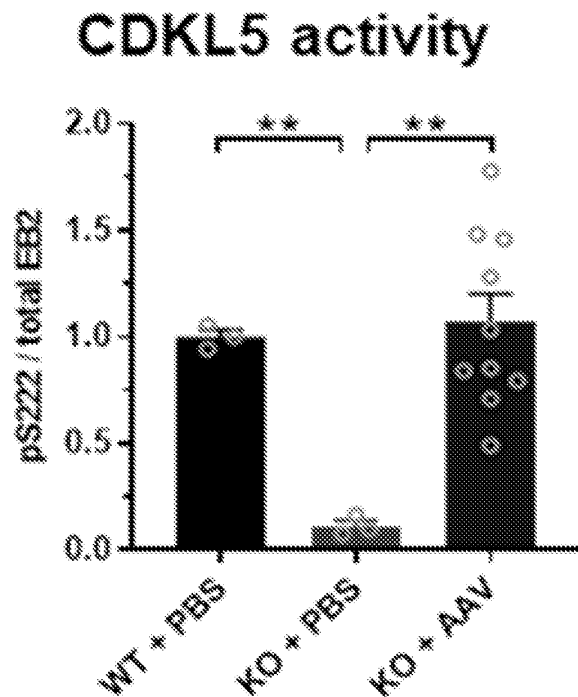


FIG 3

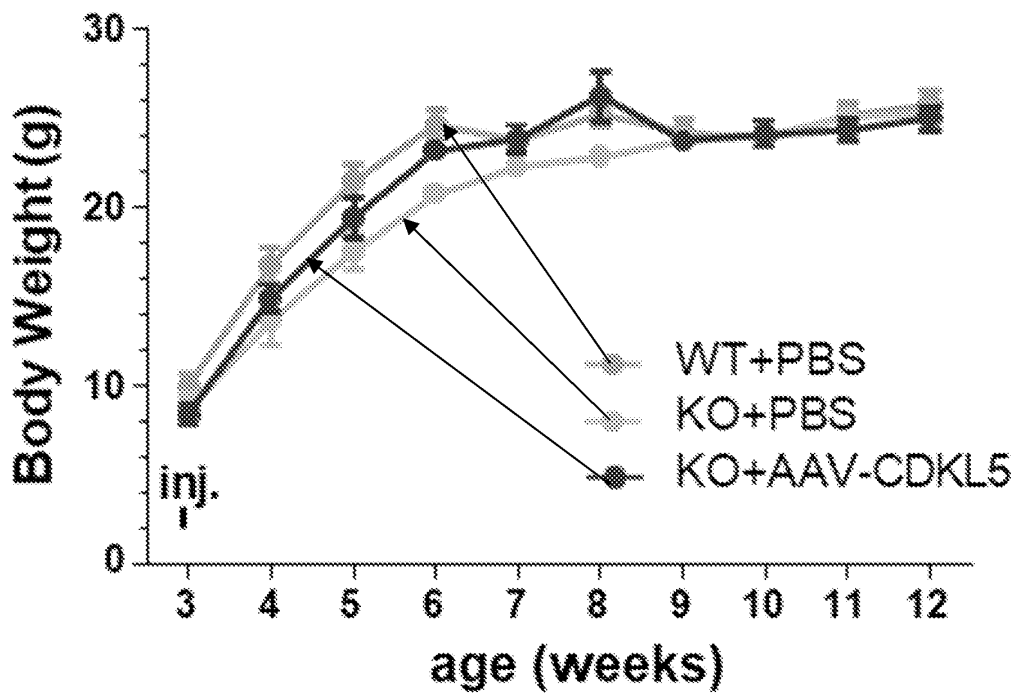


FIG 4

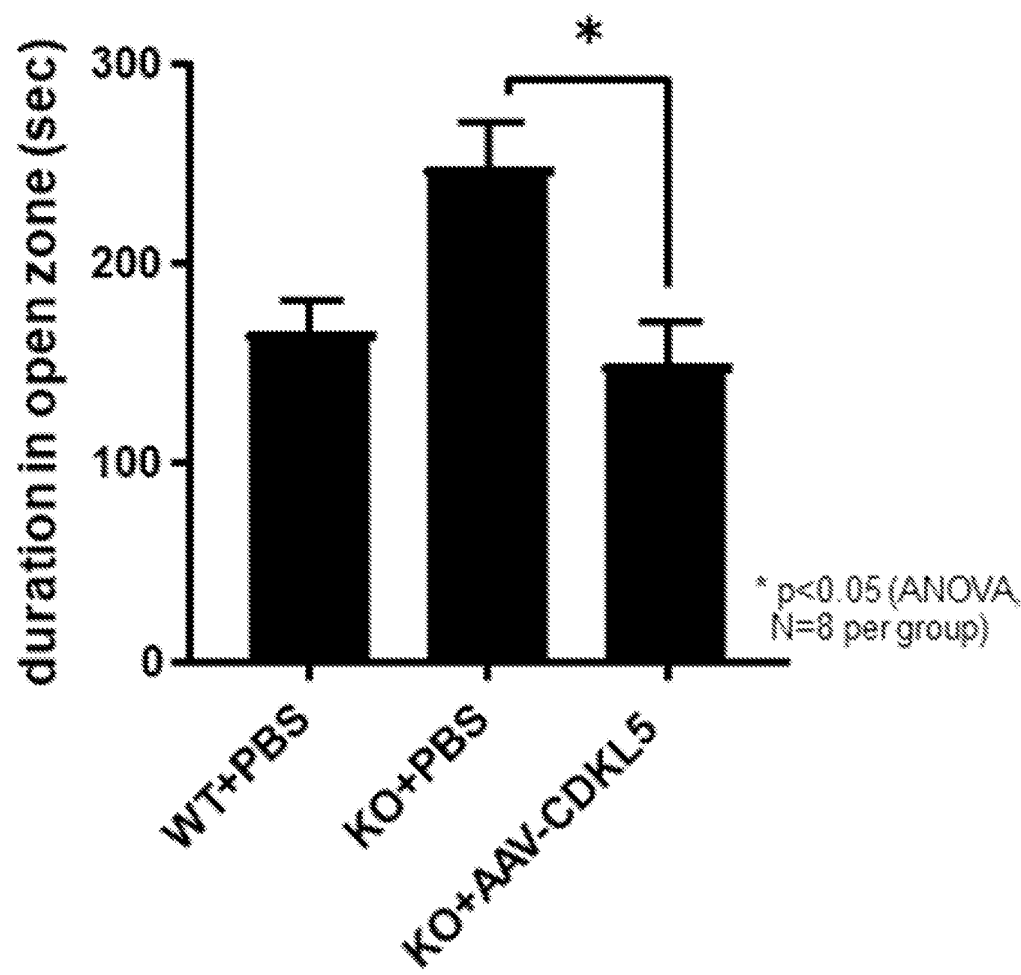


FIG 5A

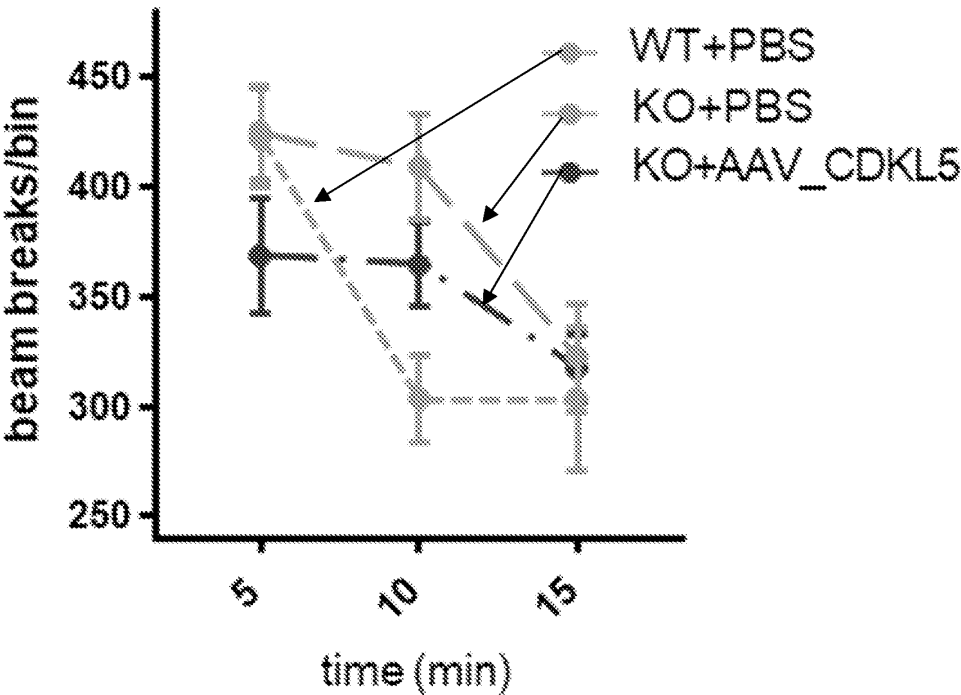


FIG 5B

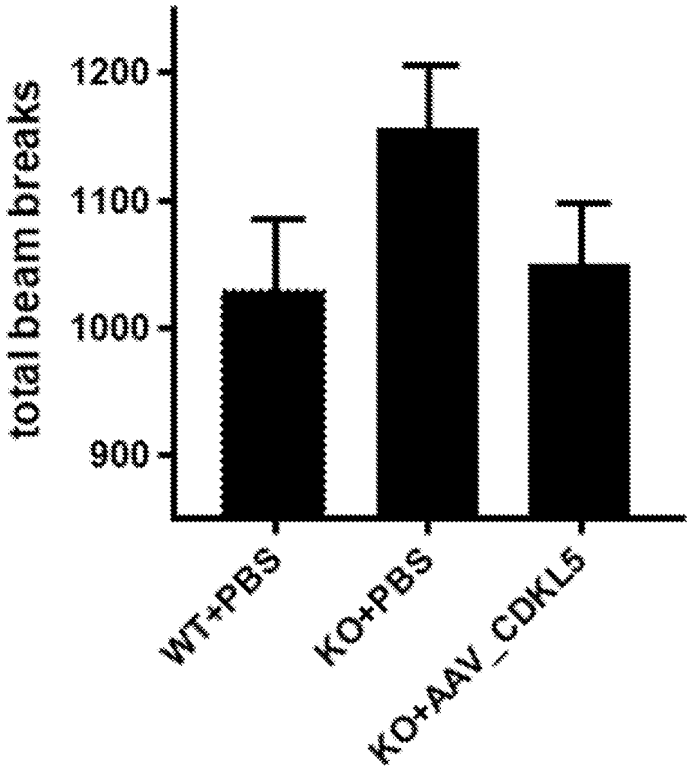


FIG 6

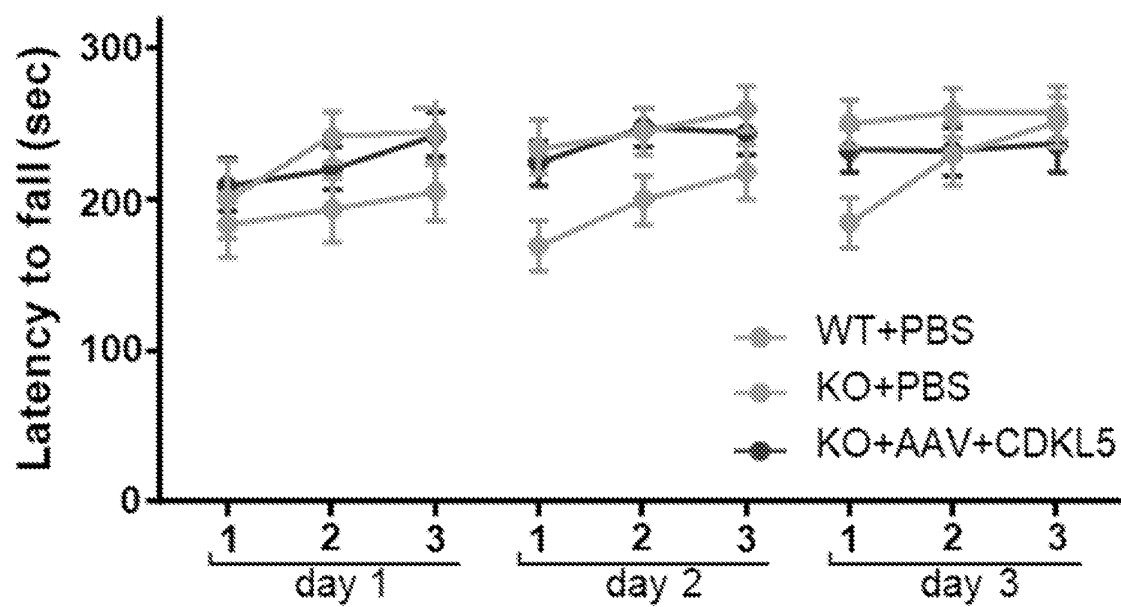


FIG 7A

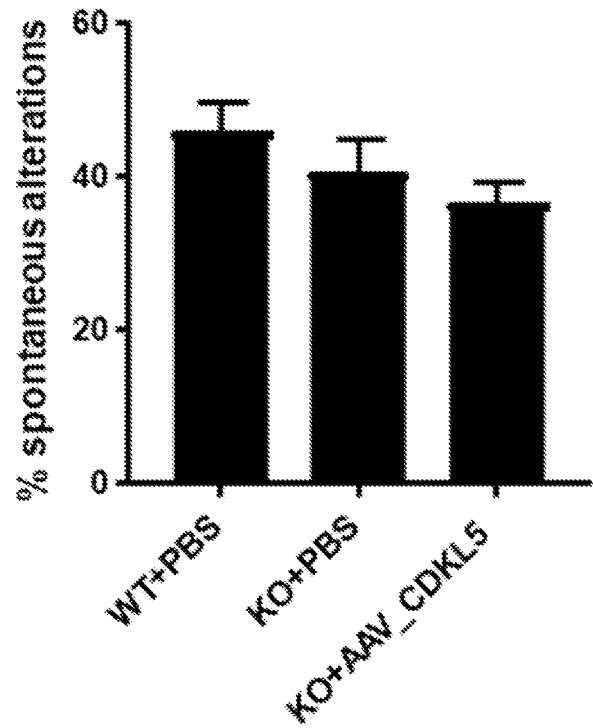


FIG 7B

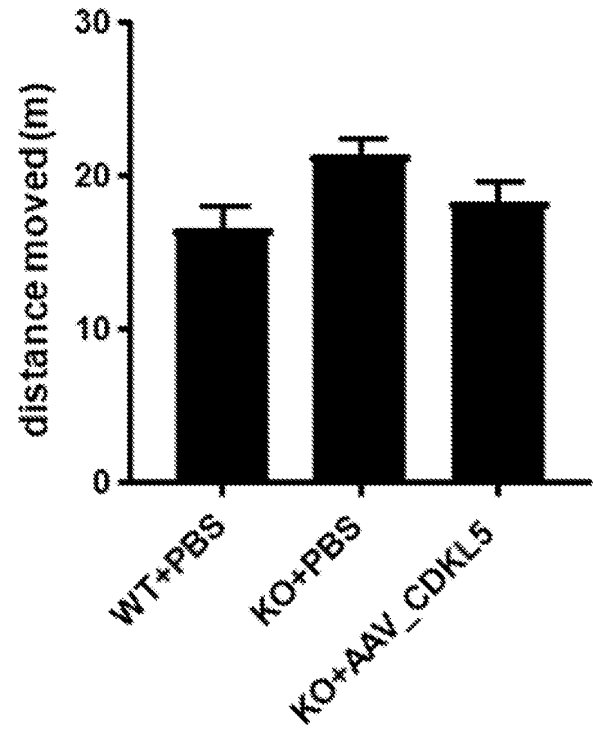


FIG 8A

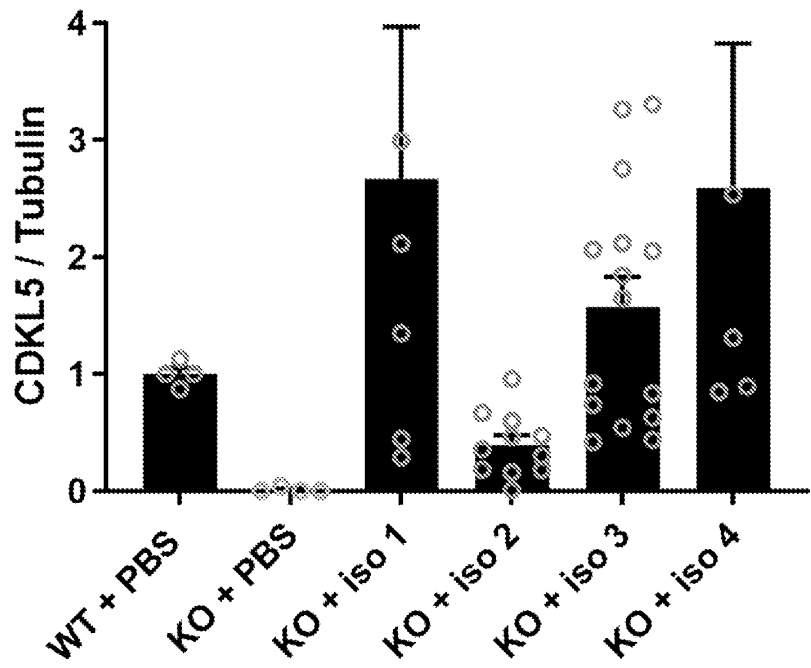


FIG 8B

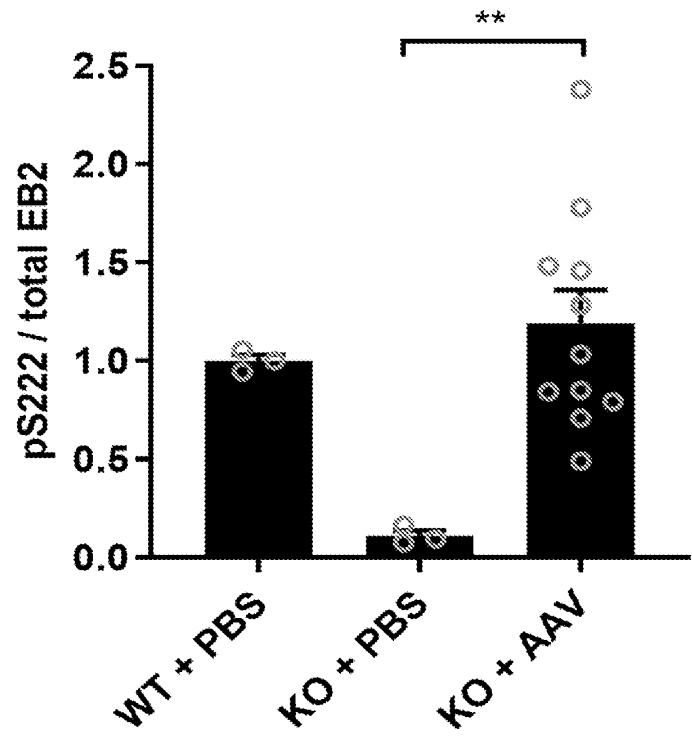
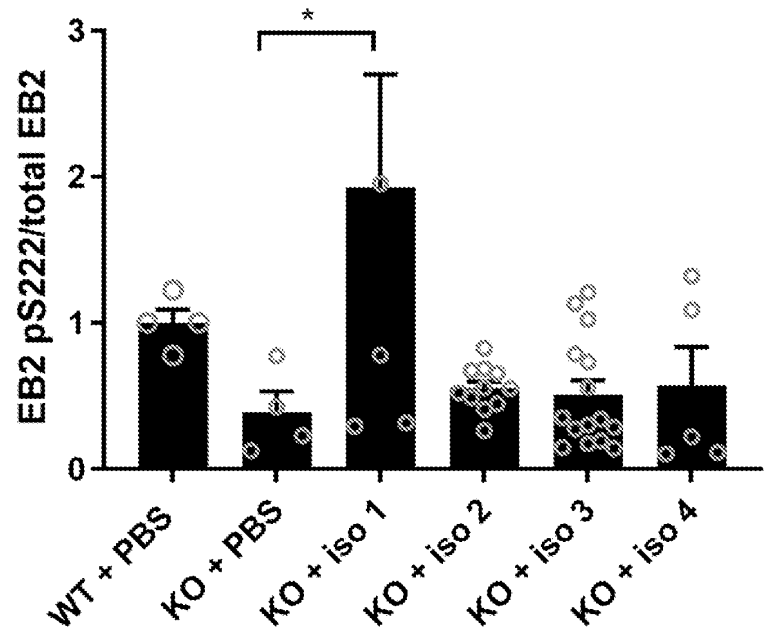


FIG 8C



One-way ANOVA with Sidak's multiple comparison's test
*p<0.5

FIG 8D

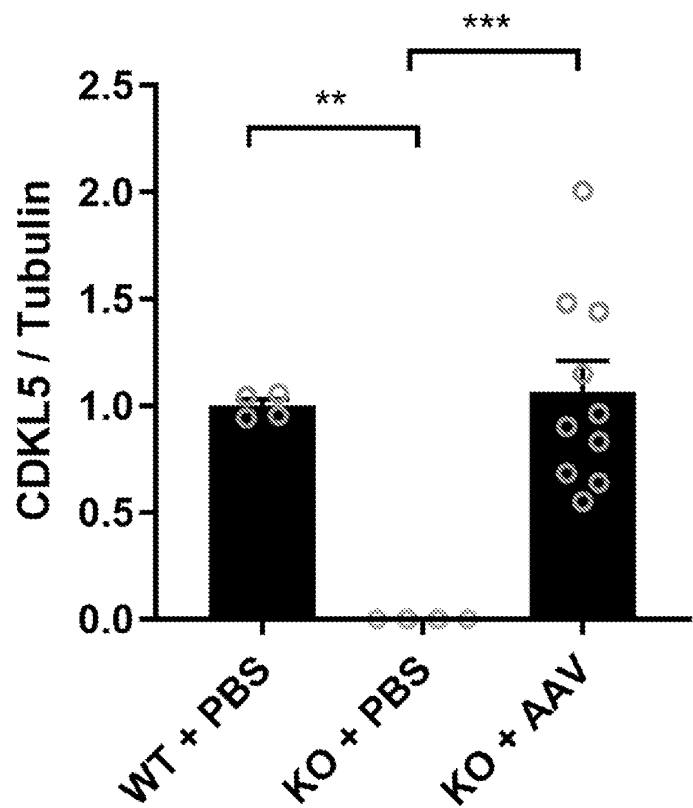


FIG 9A

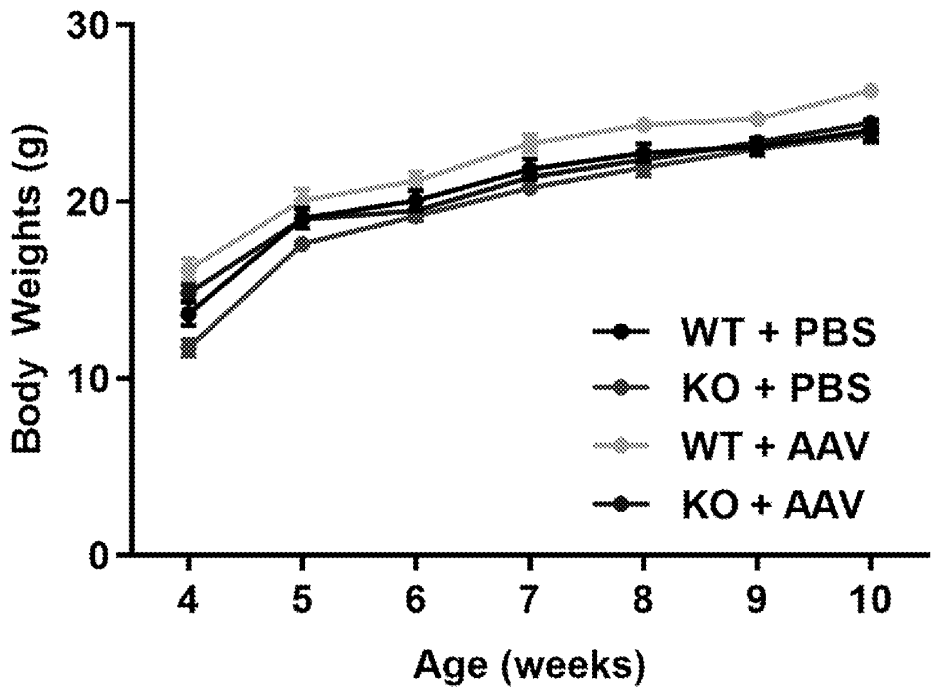


FIG 9B

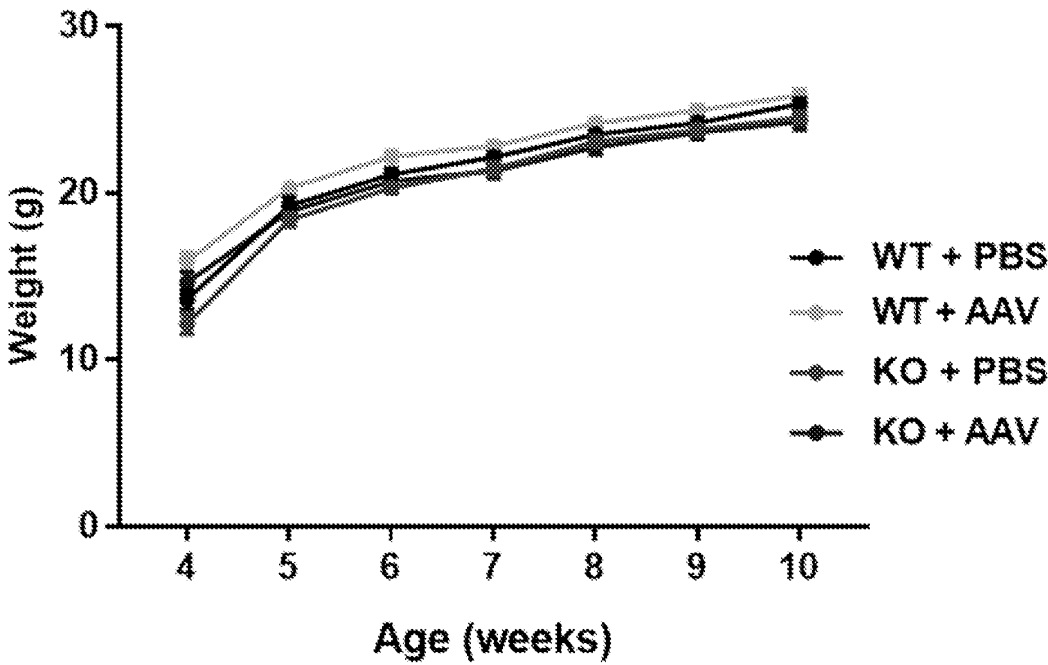


FIG 9C

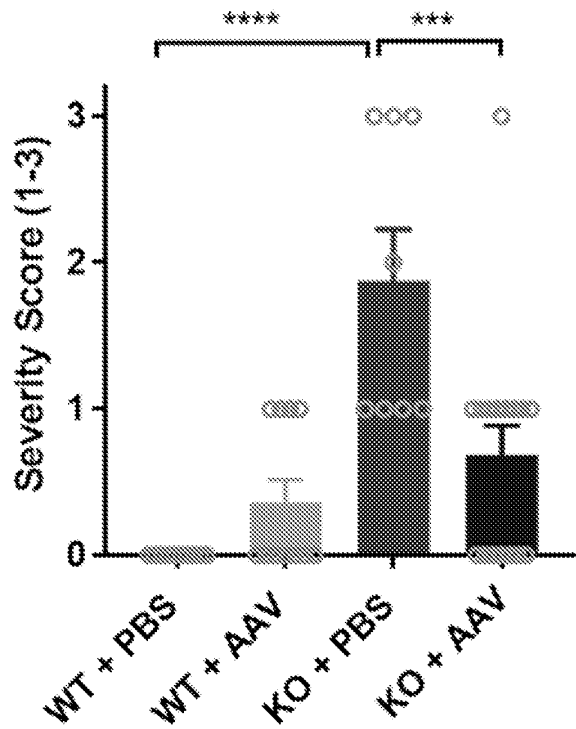


FIG 9D

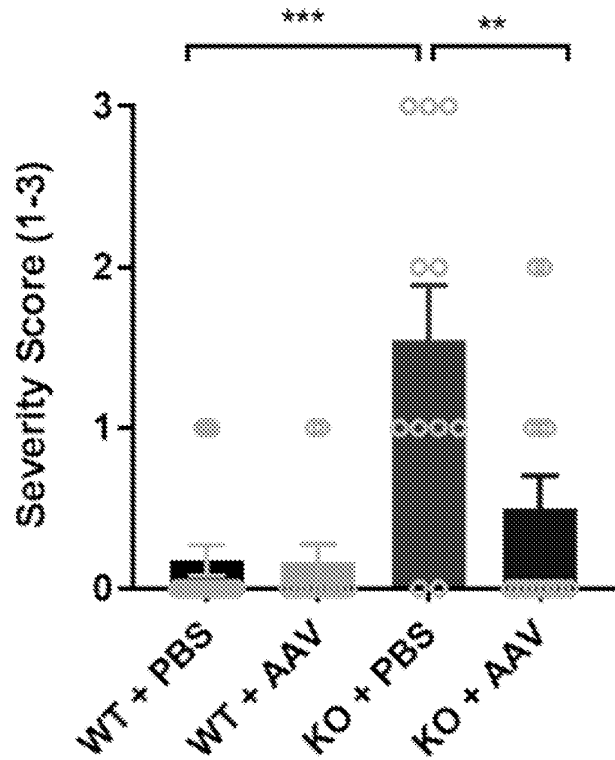


FIG 9E

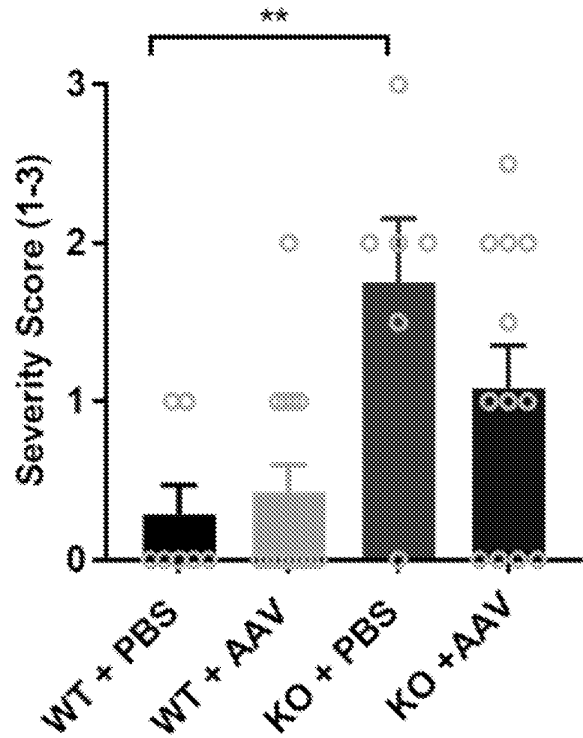


FIG 9F

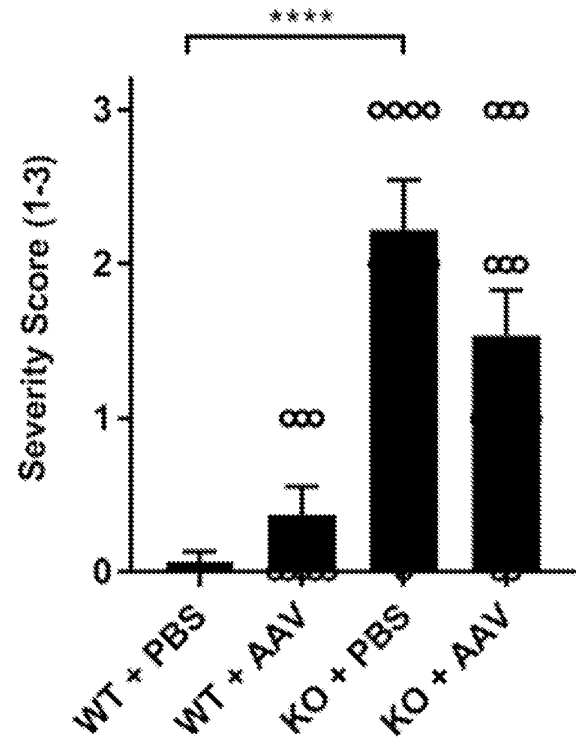


FIG 10A

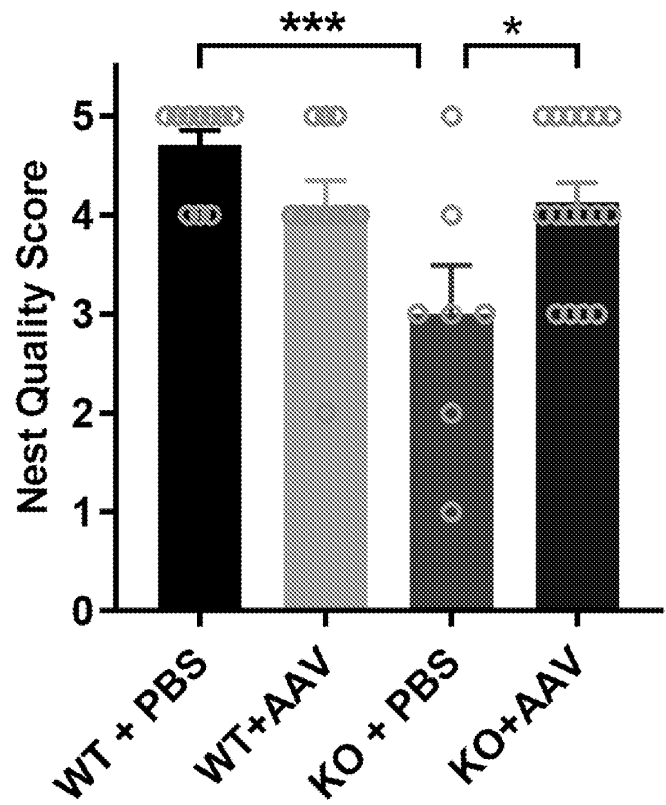


FIG 10B

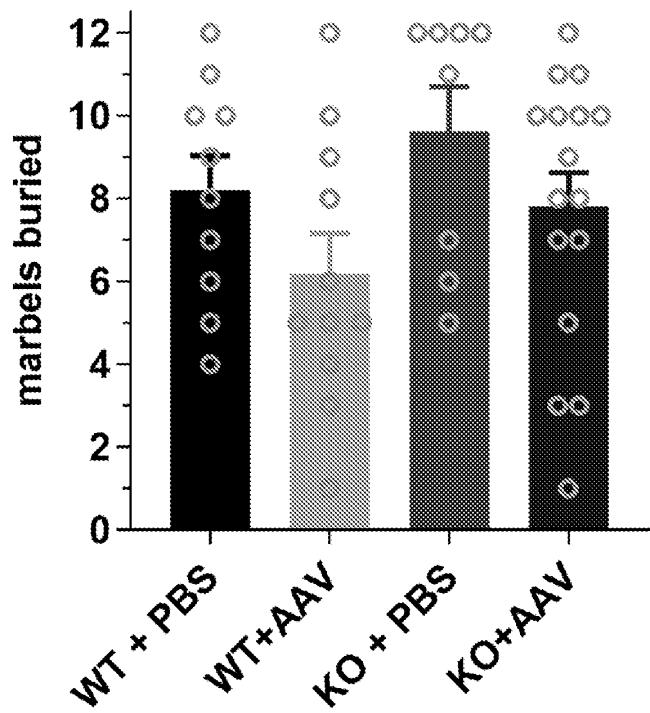


FIG 10C

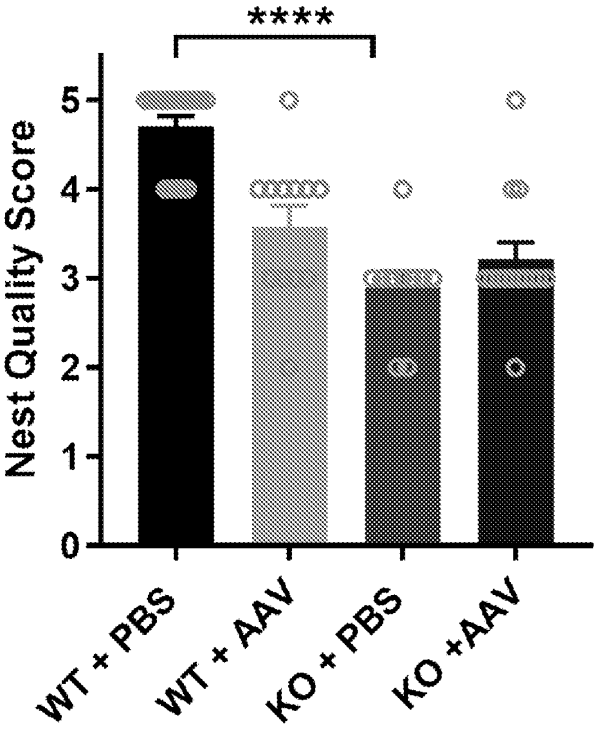


FIG 10D

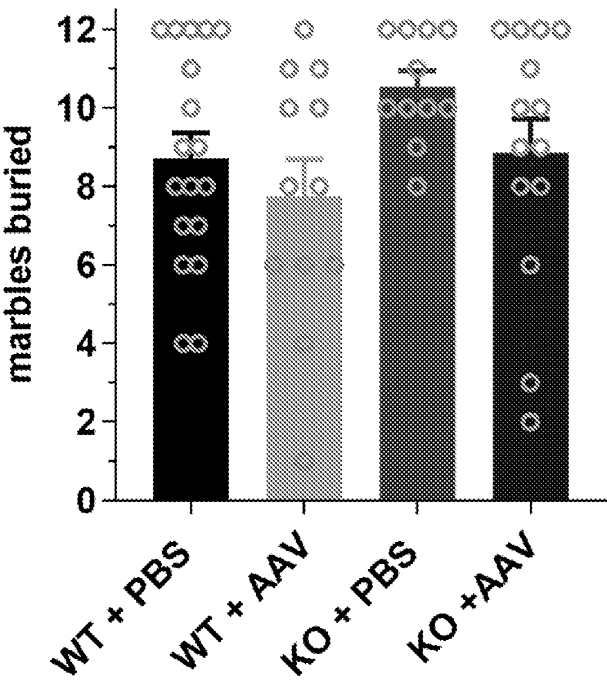


FIG 10E

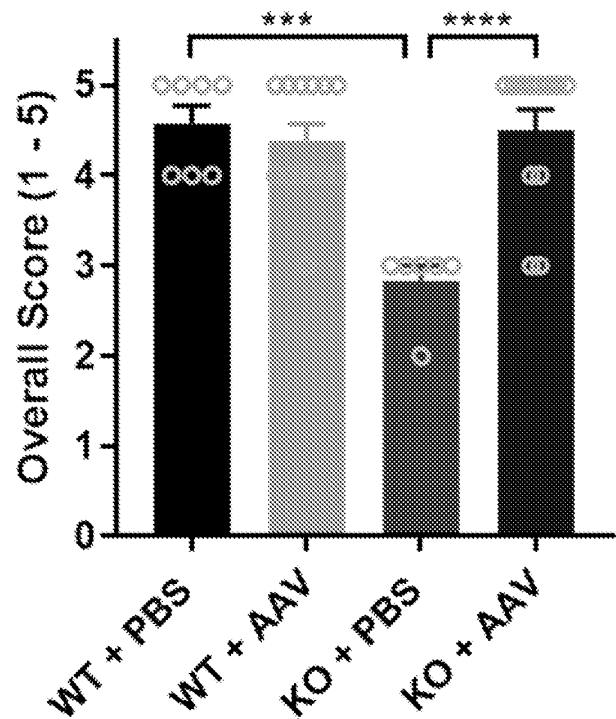
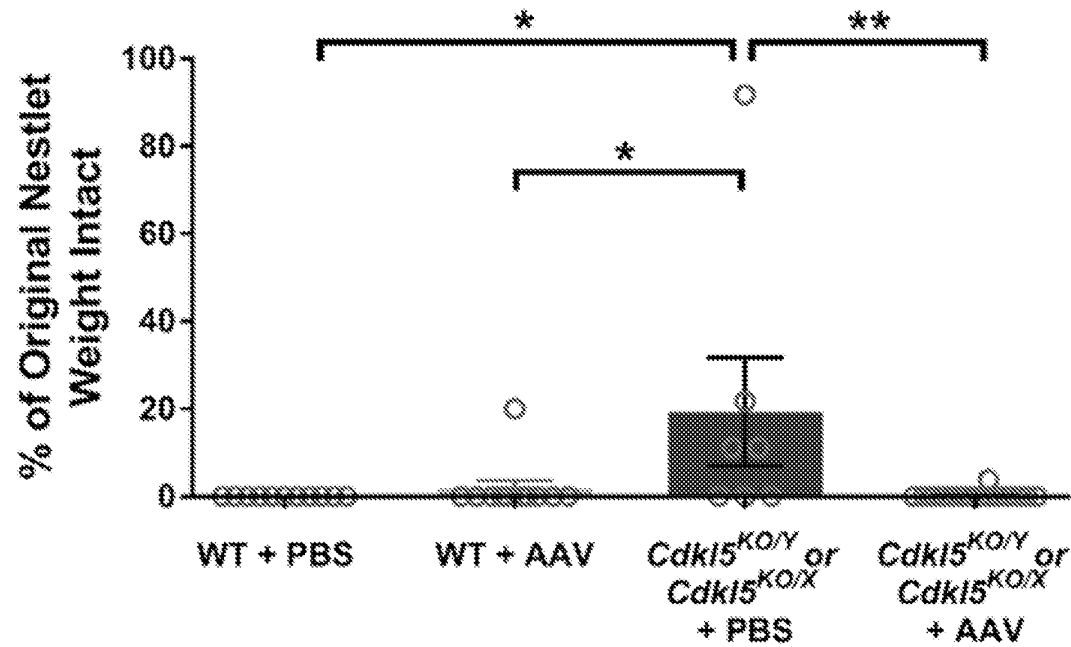


FIG 10F



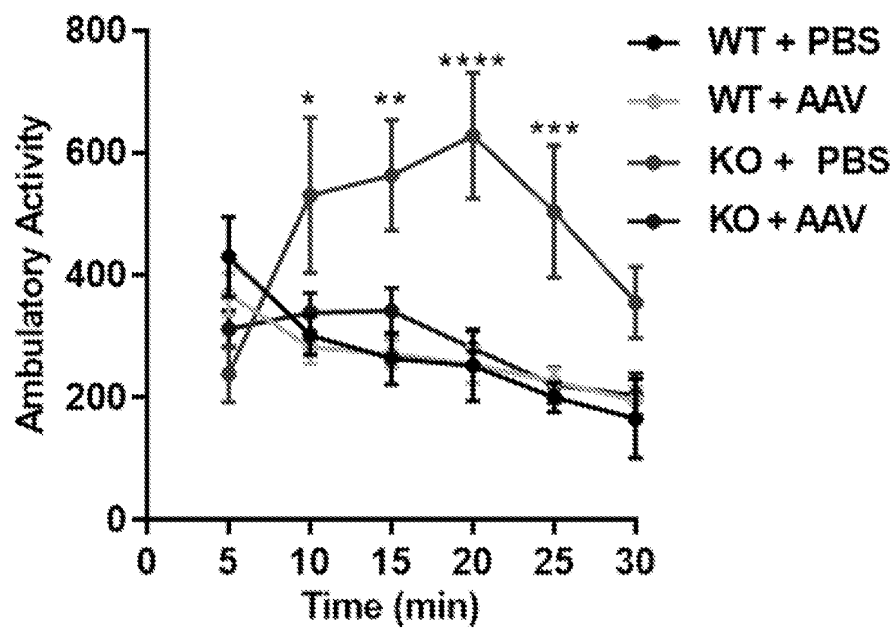


FIG 11B

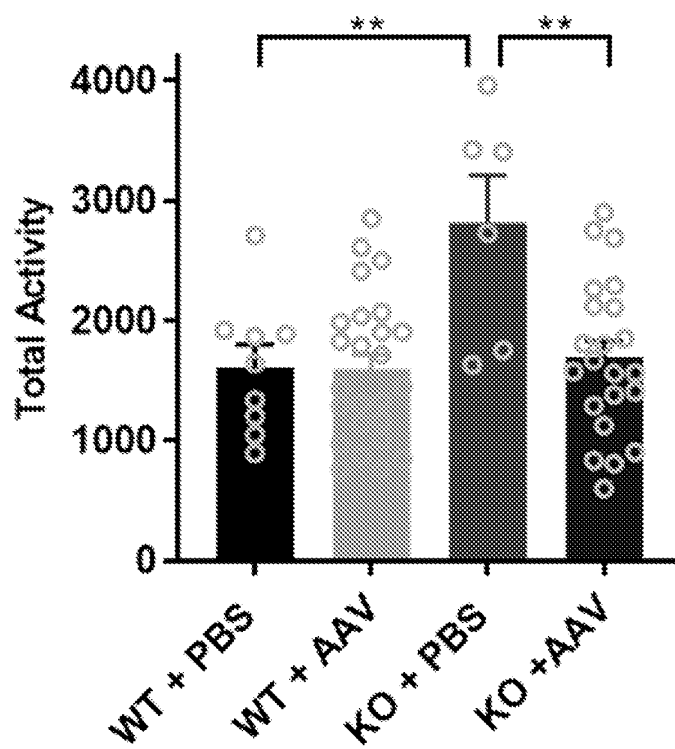


FIG 11C

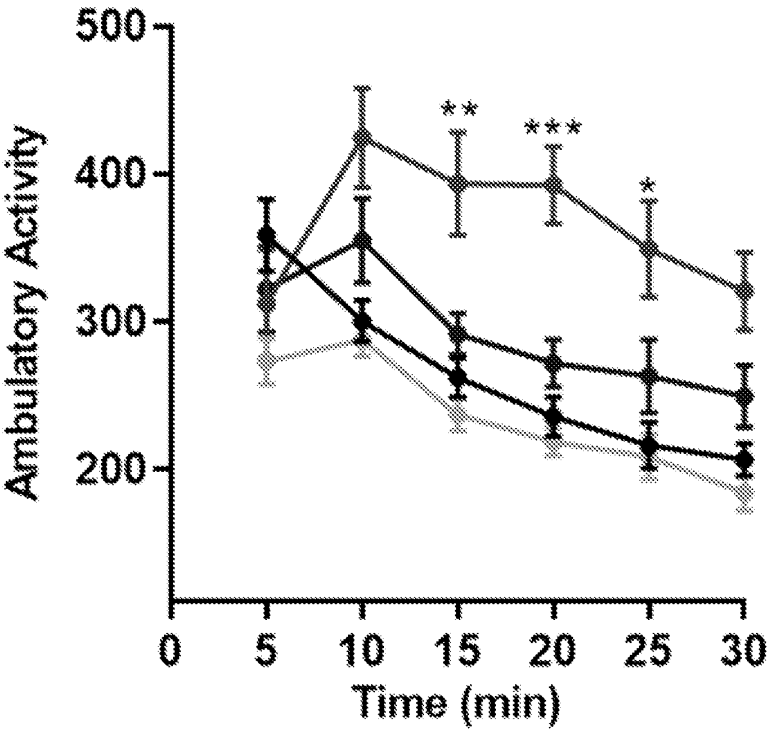
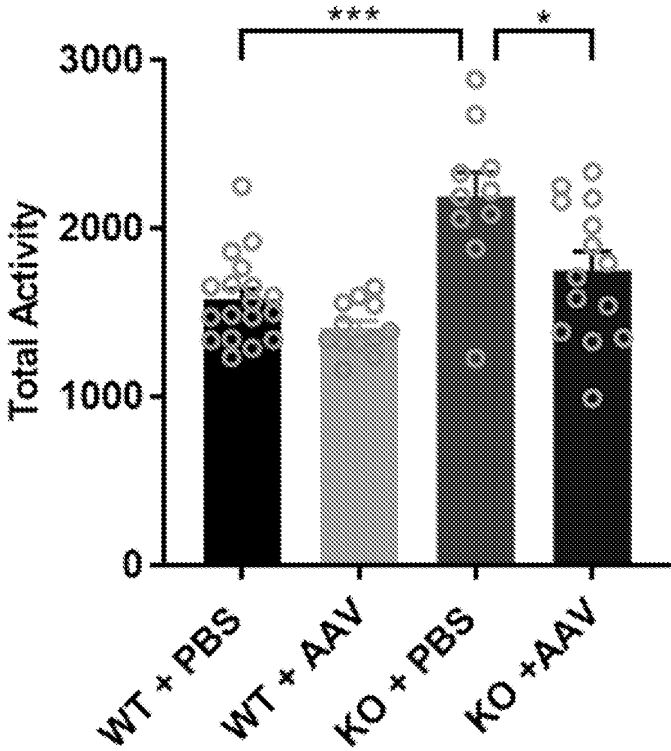


FIG 11D



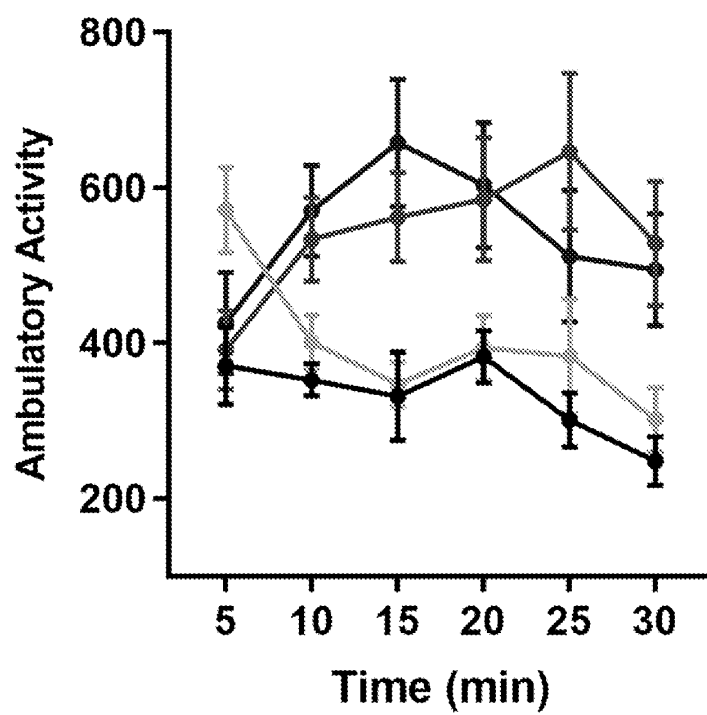


FIG 11F

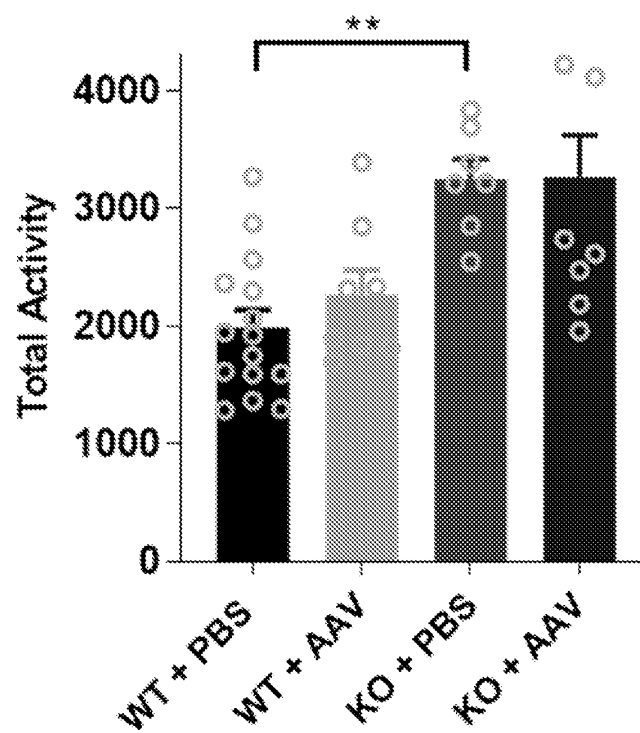


FIG 12

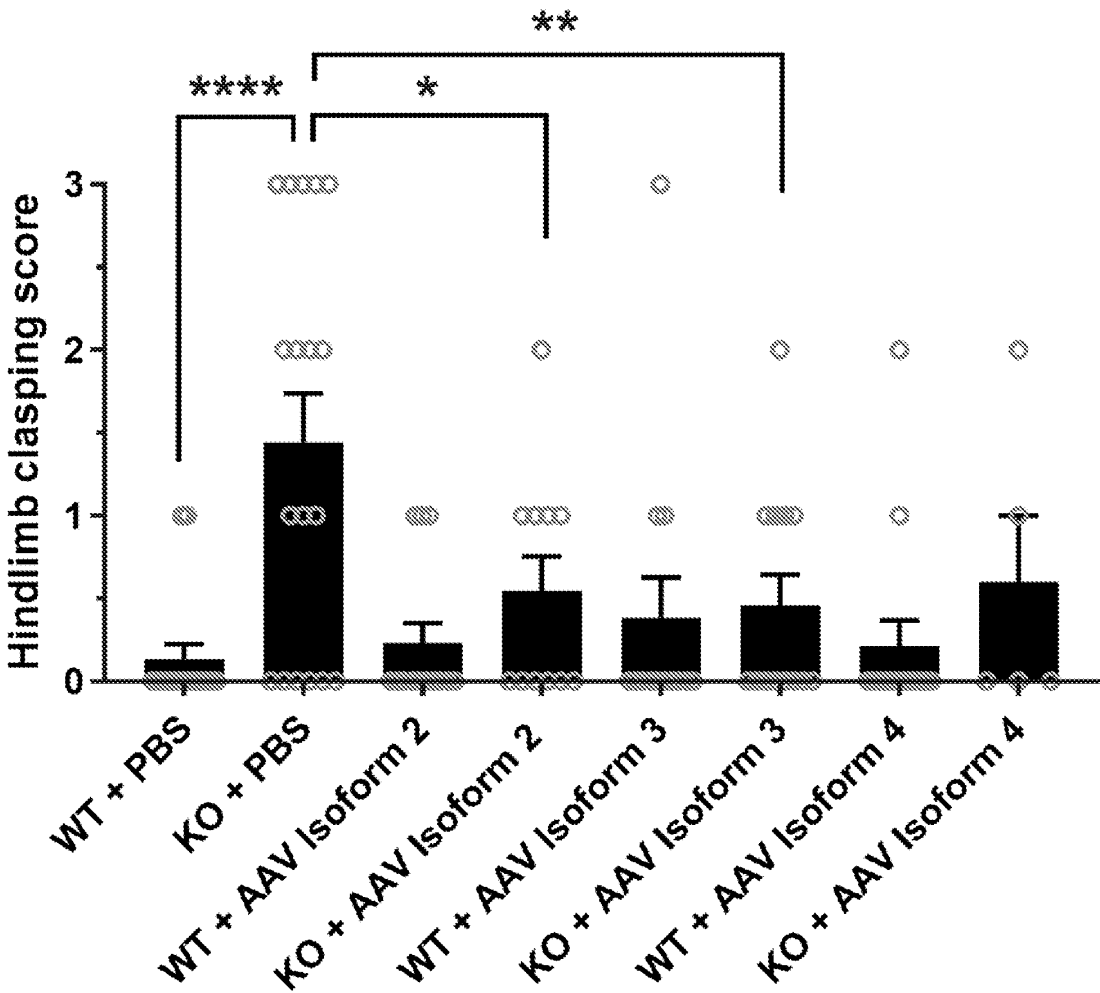


FIG 13A

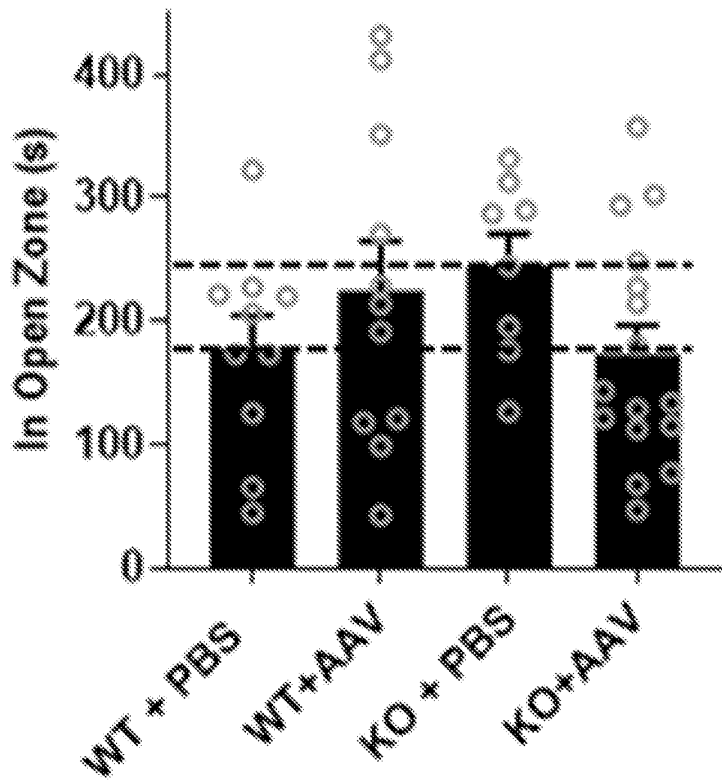


FIG 13B

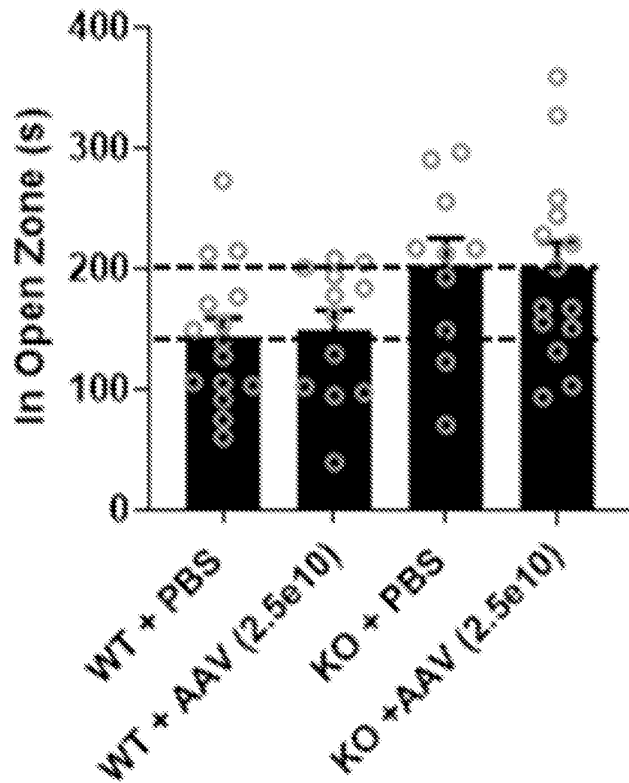


FIG 13C

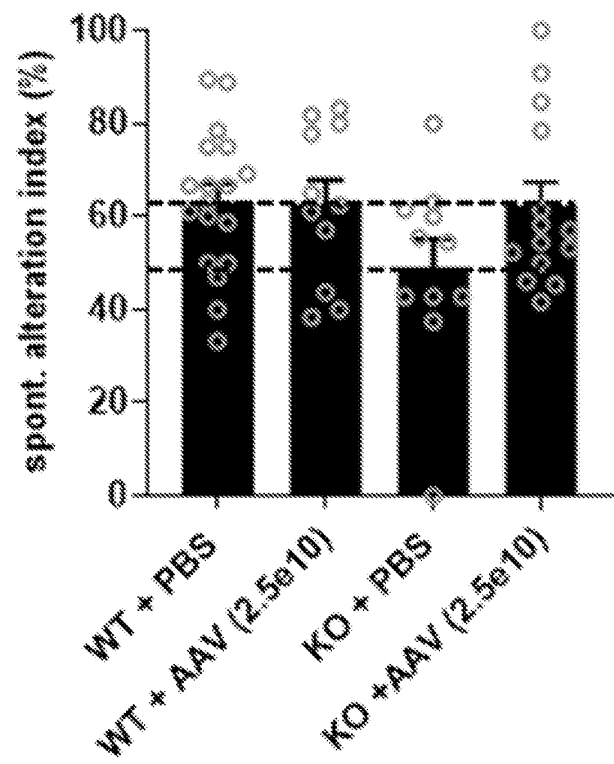


FIG 13D

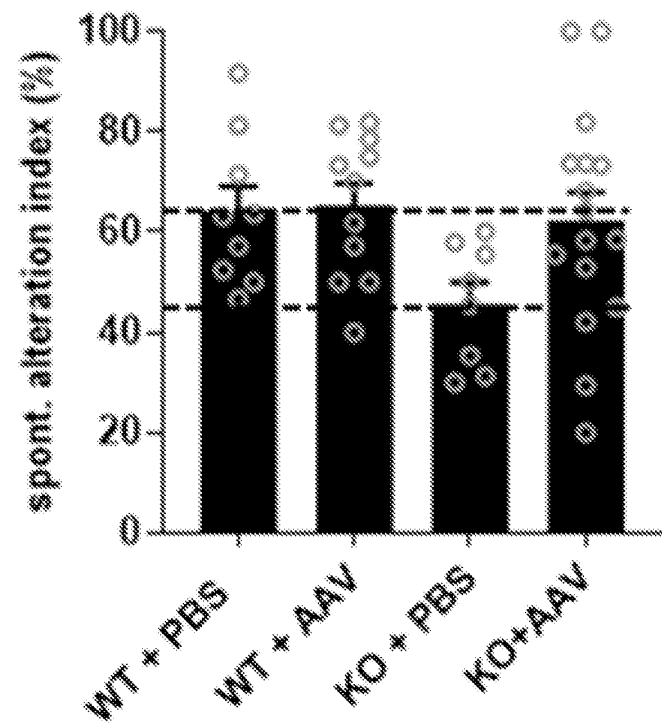


FIG 14A

Male Hindlimb clasping

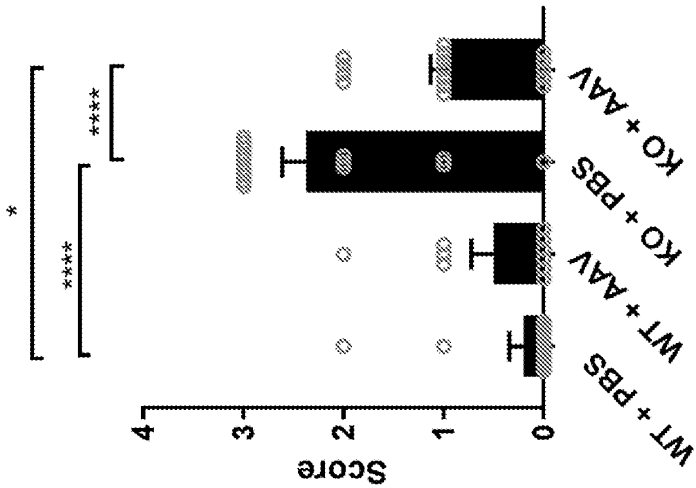


FIG 14B

Female Hindlimb clasping

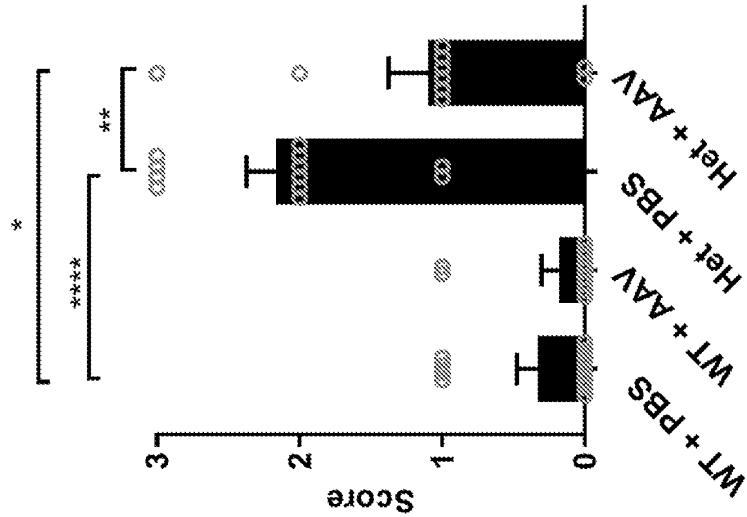


FIG 14C

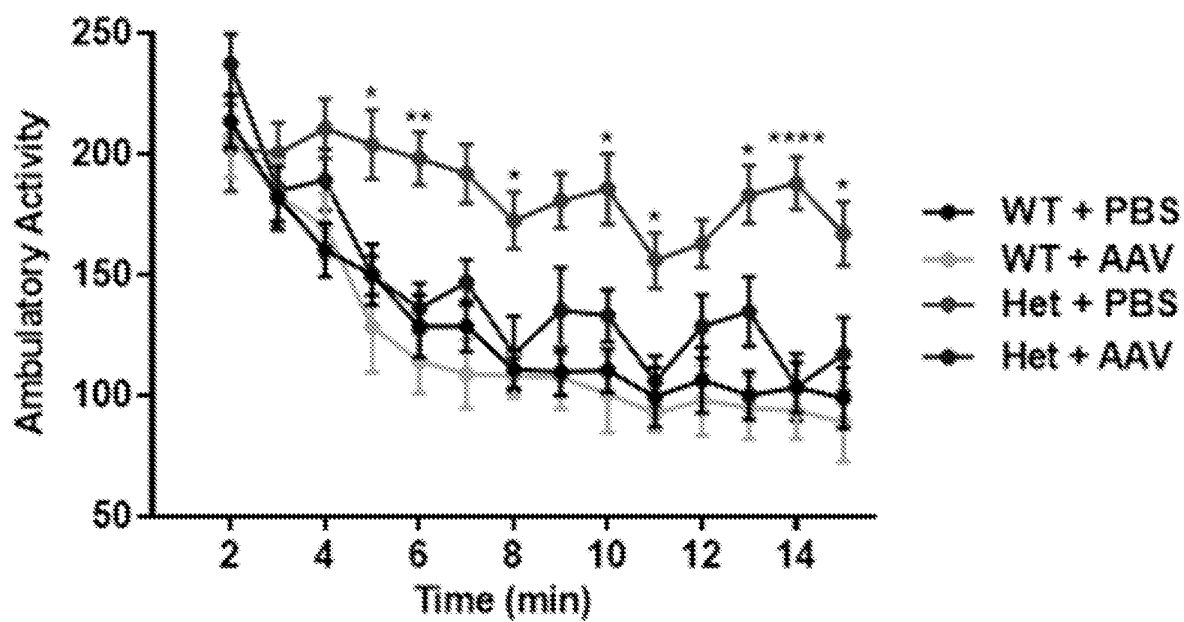


FIG 15A

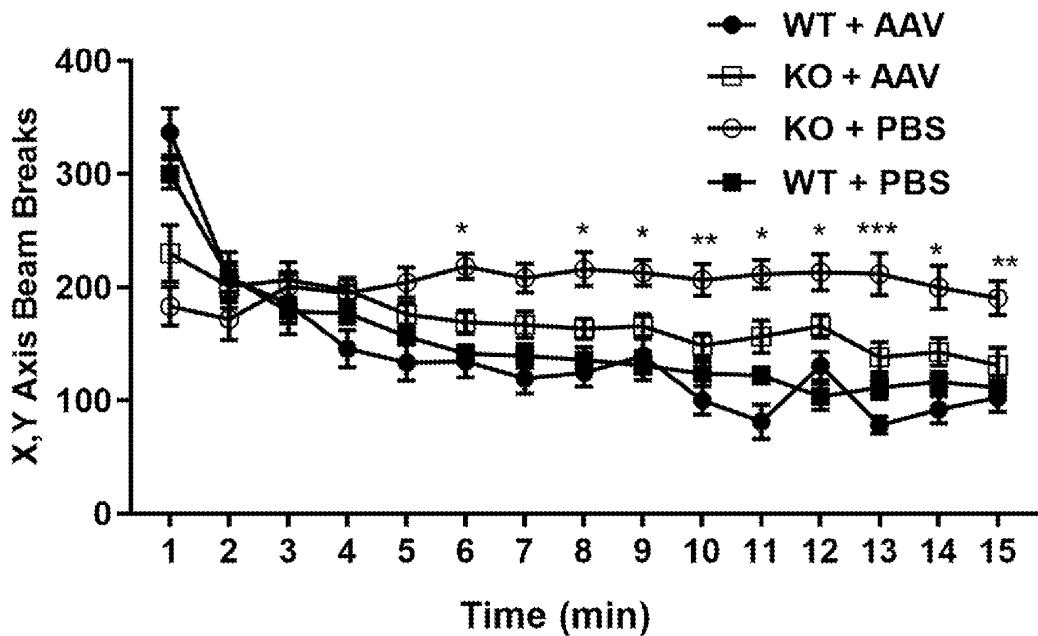


FIG 15B

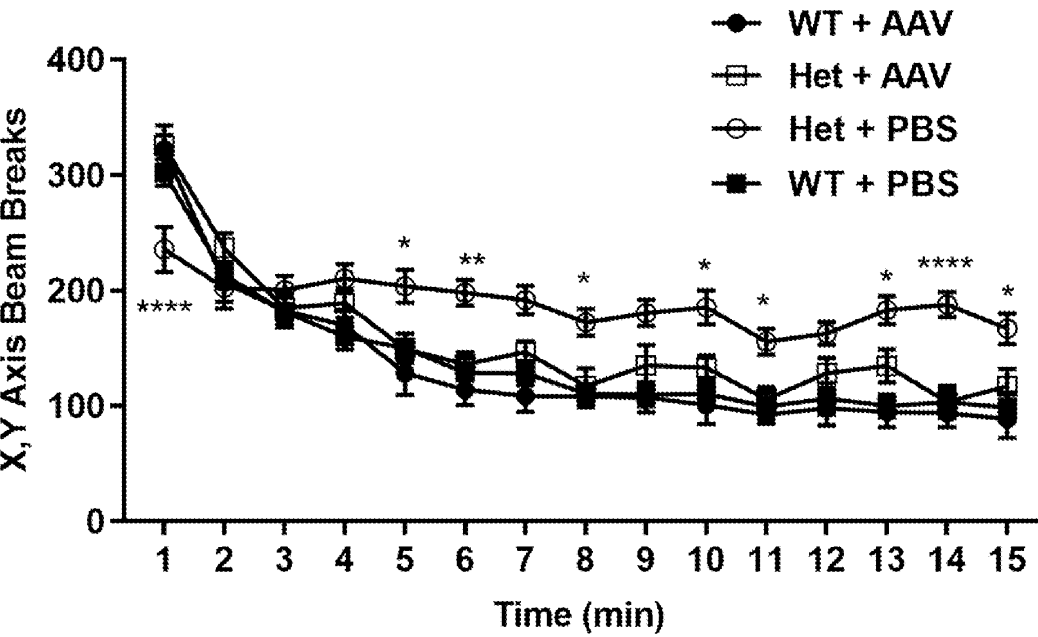


FIG 15C

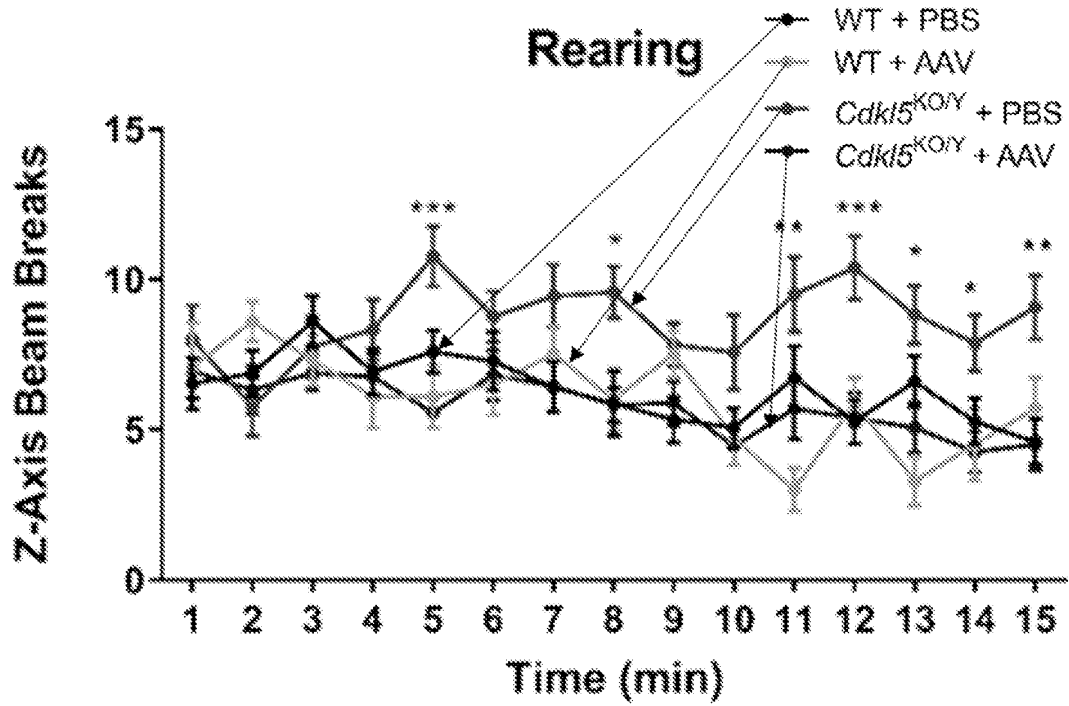


FIG 15D

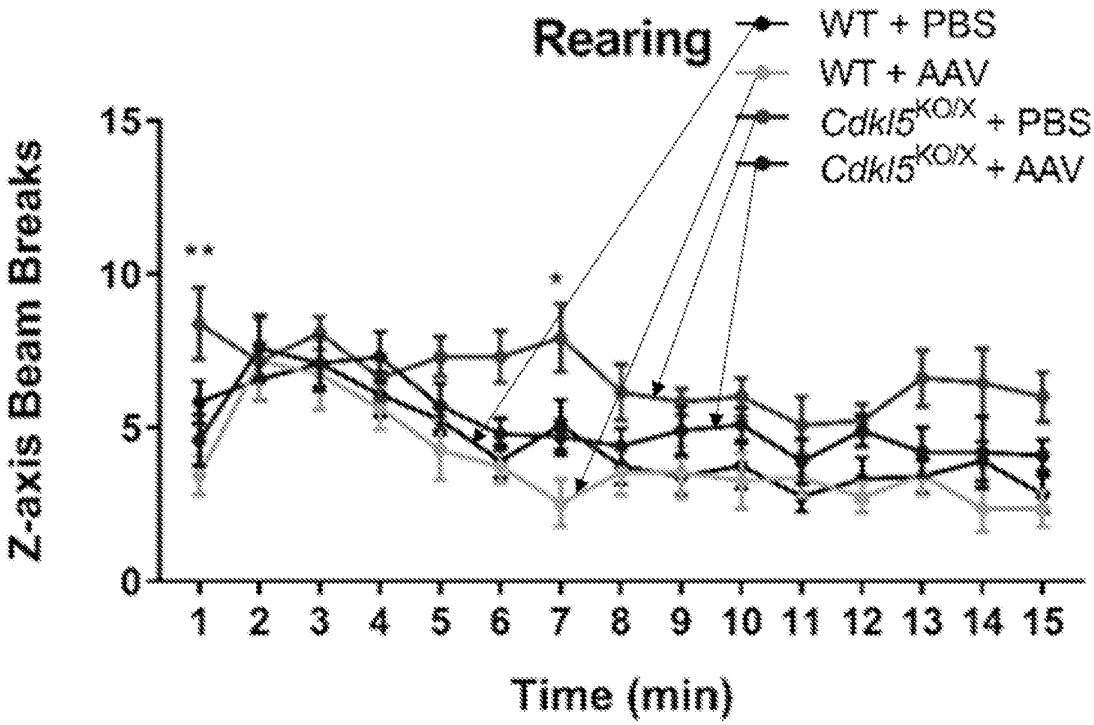


FIG 15E

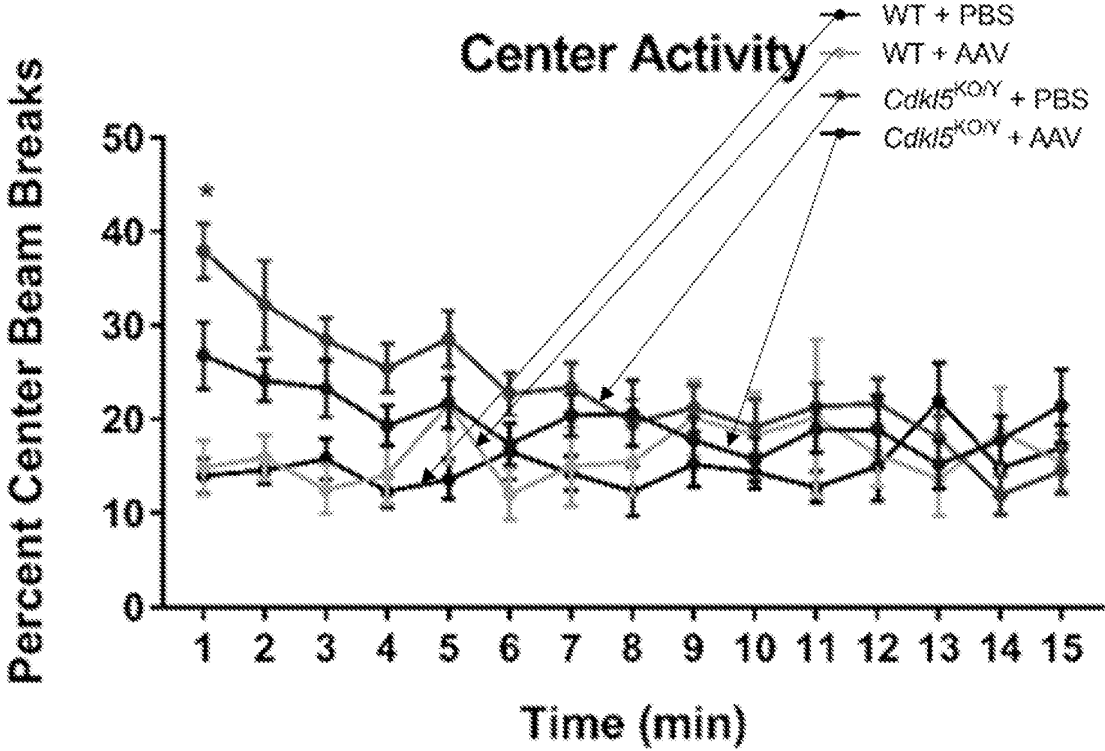
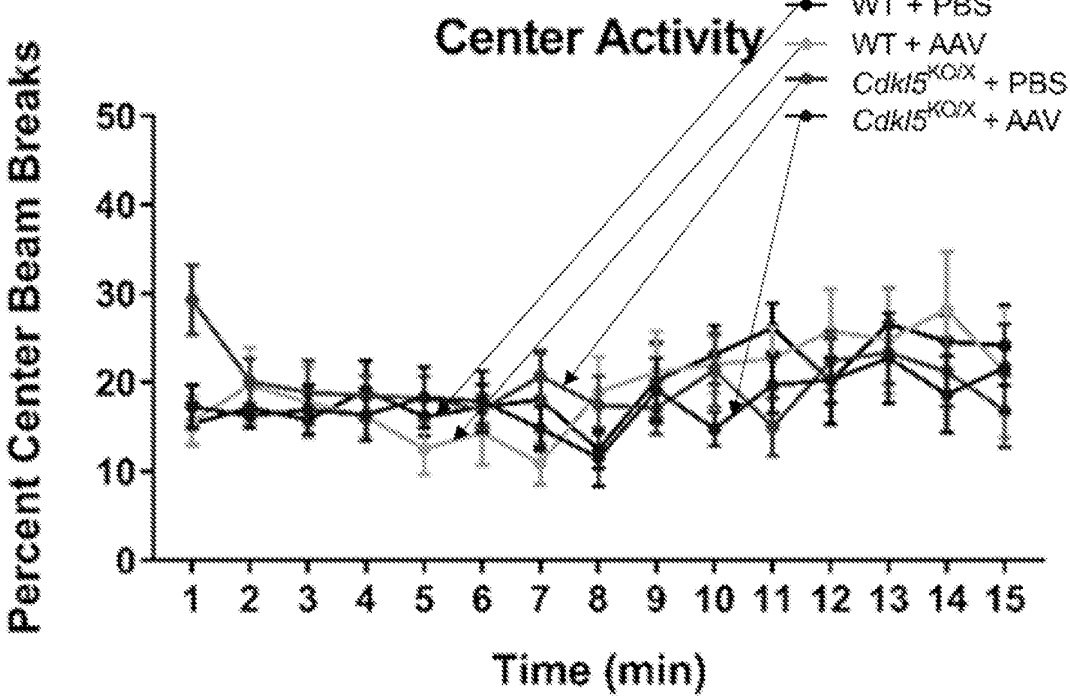


FIG 15F



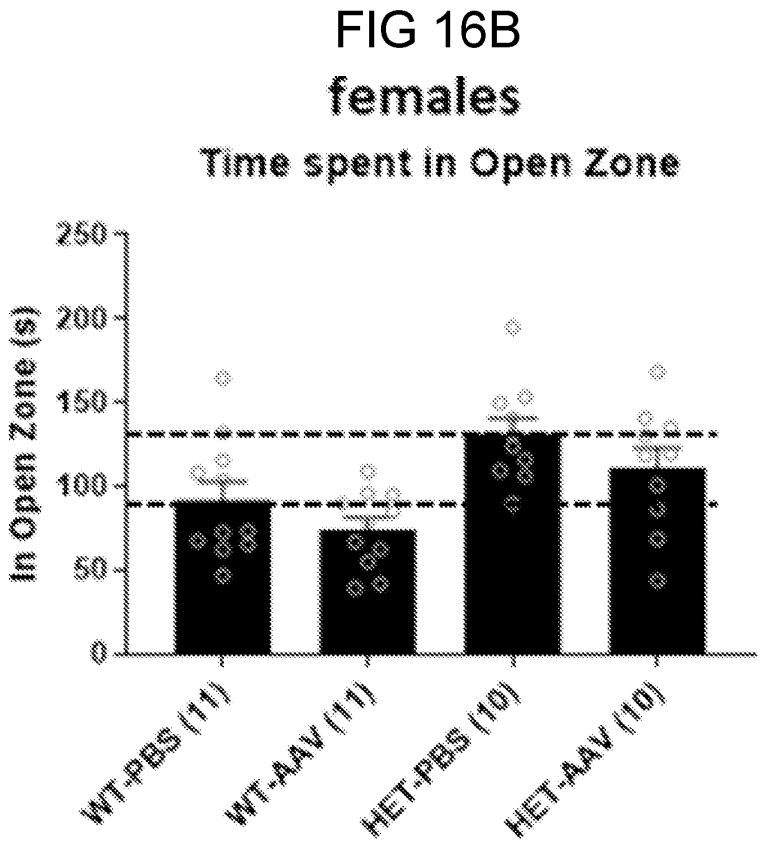
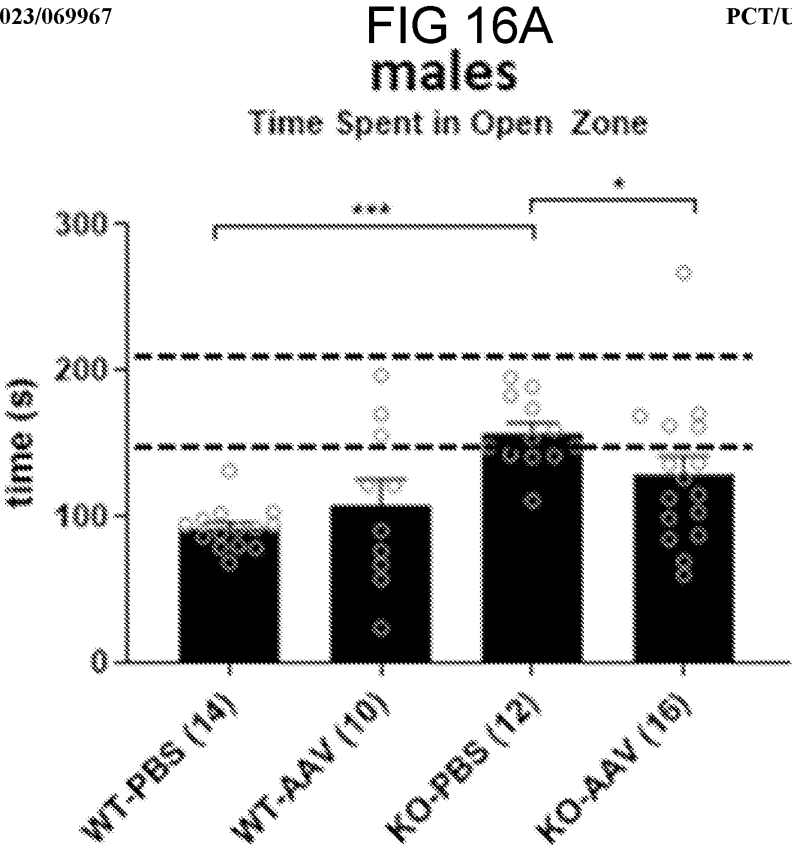


FIG 17

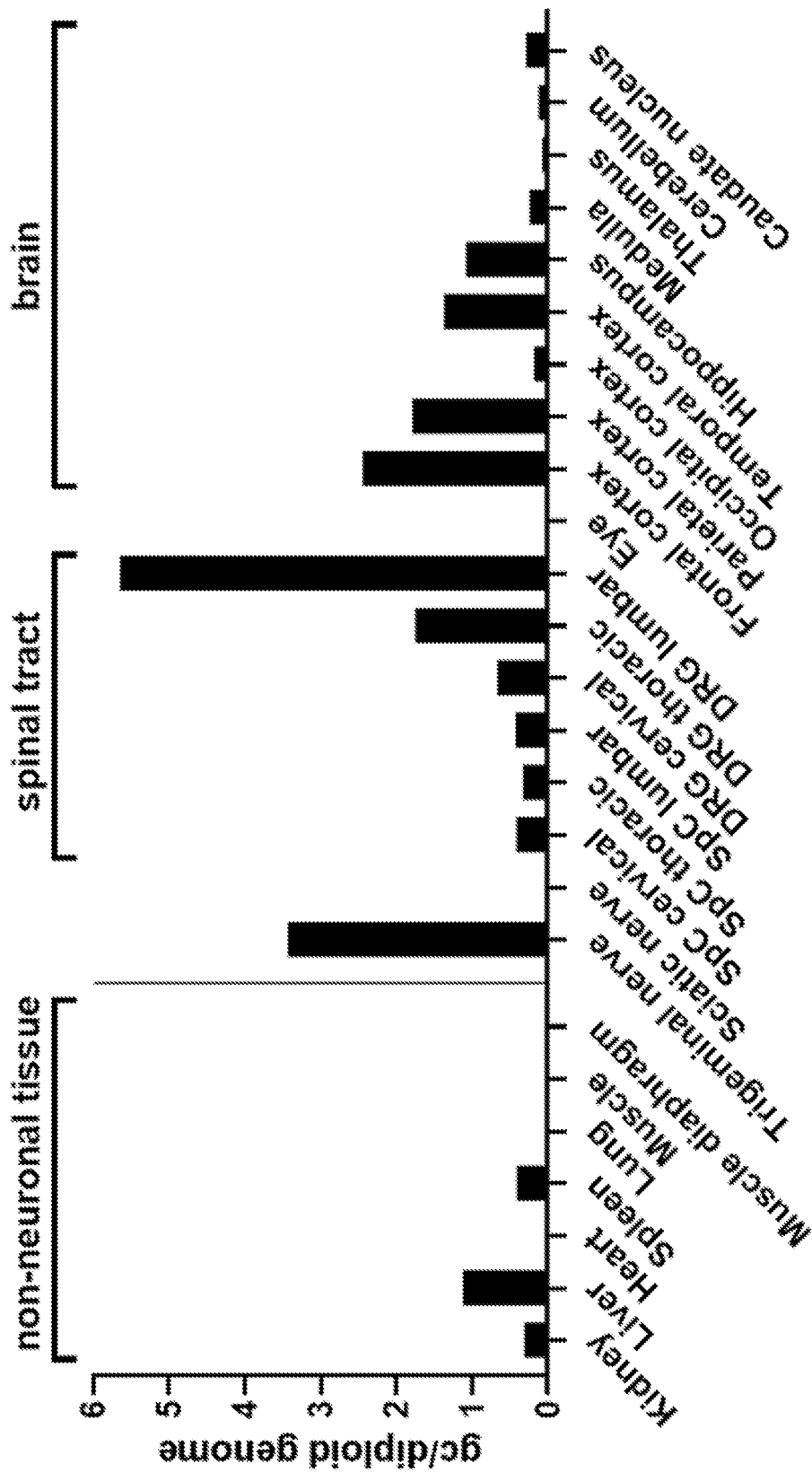


FIG 18

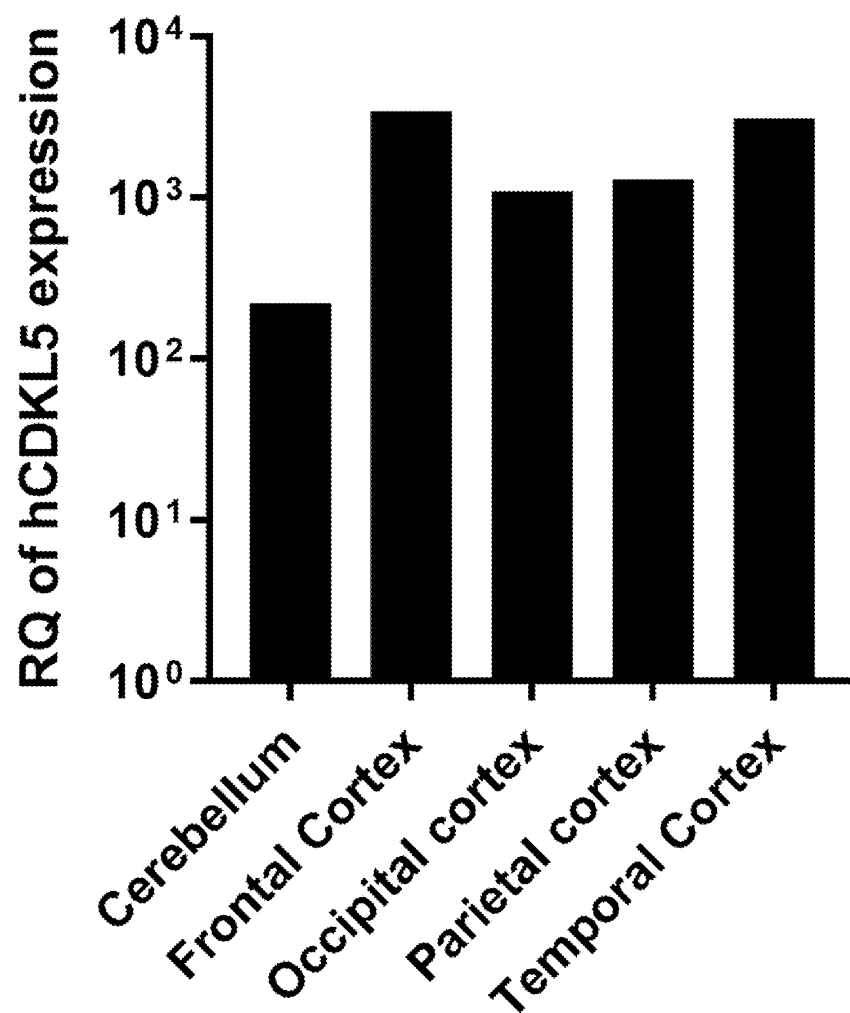


FIG 19A

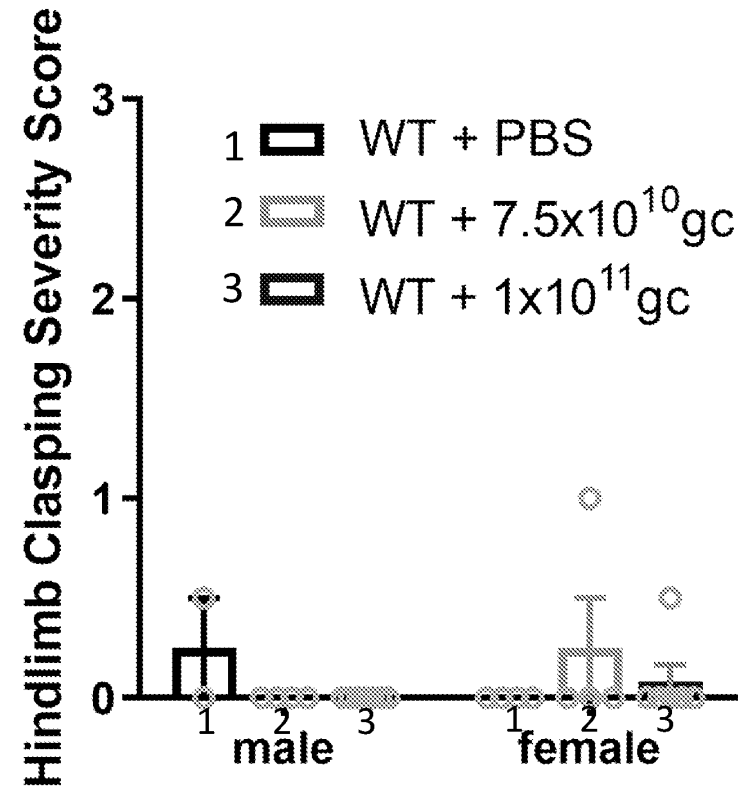


FIG 19B

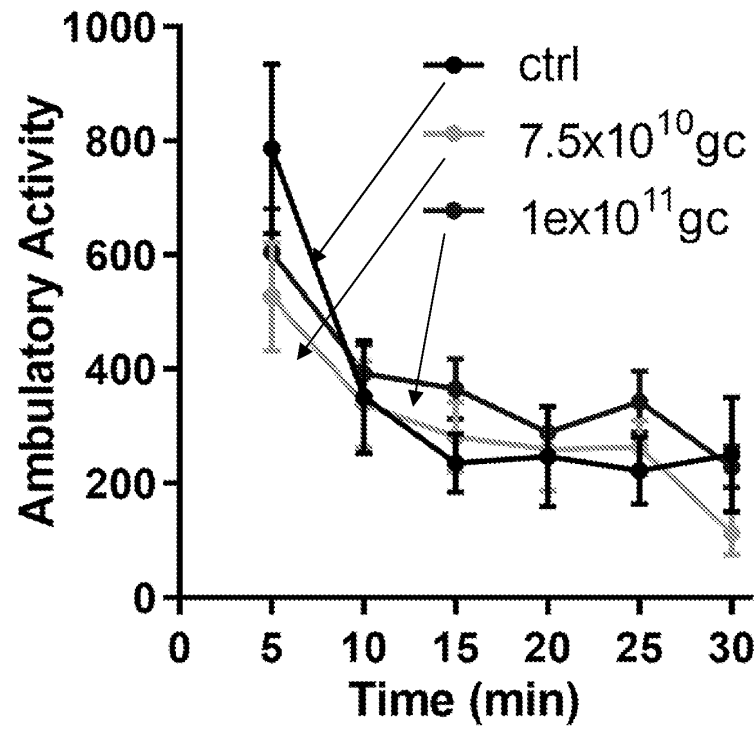


FIG 20

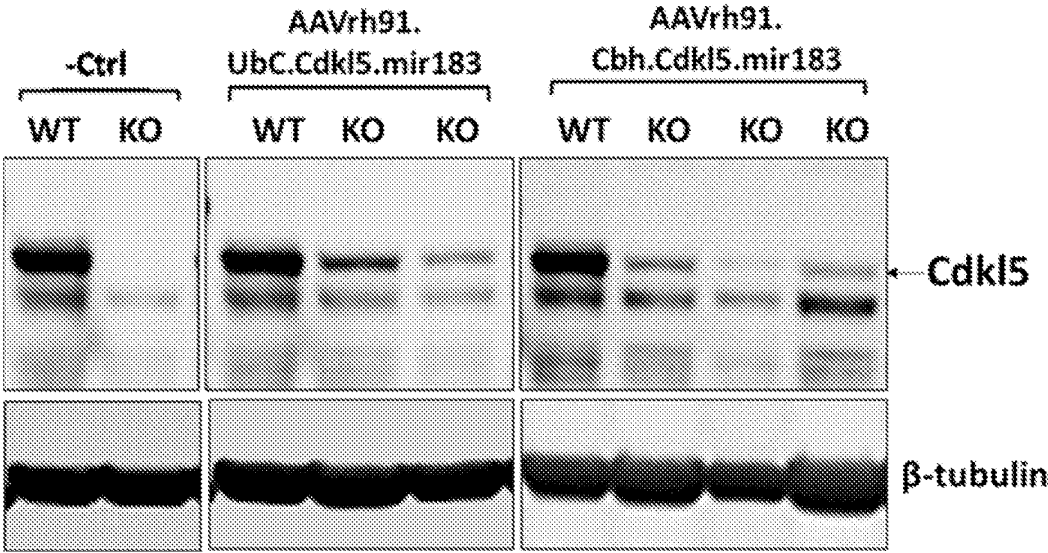


FIG 21A

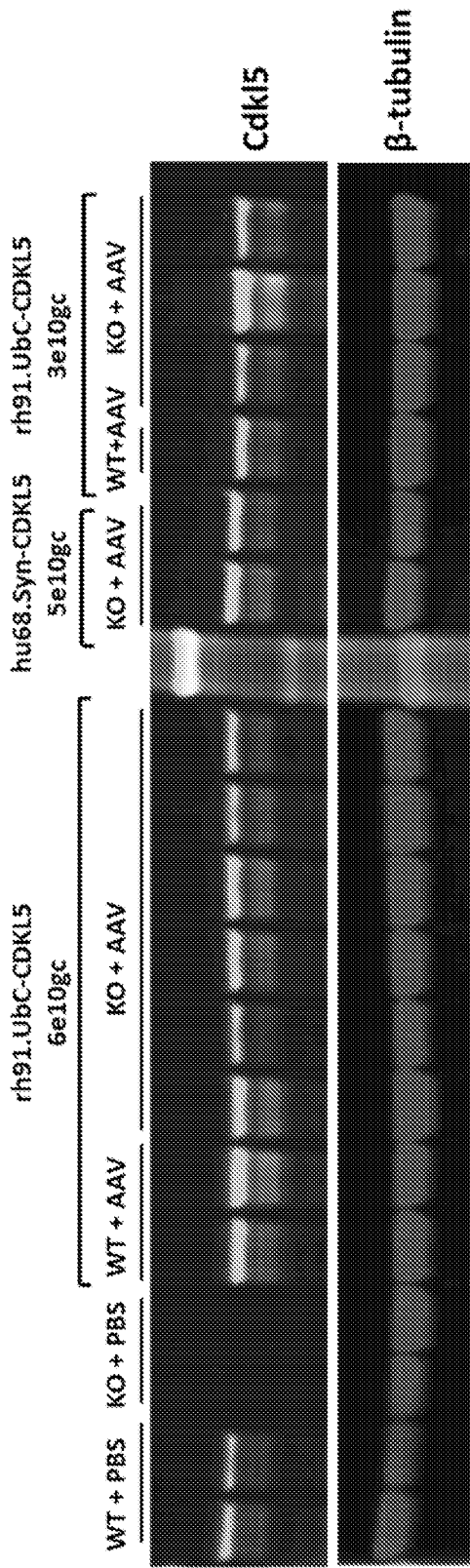


FIG 21B

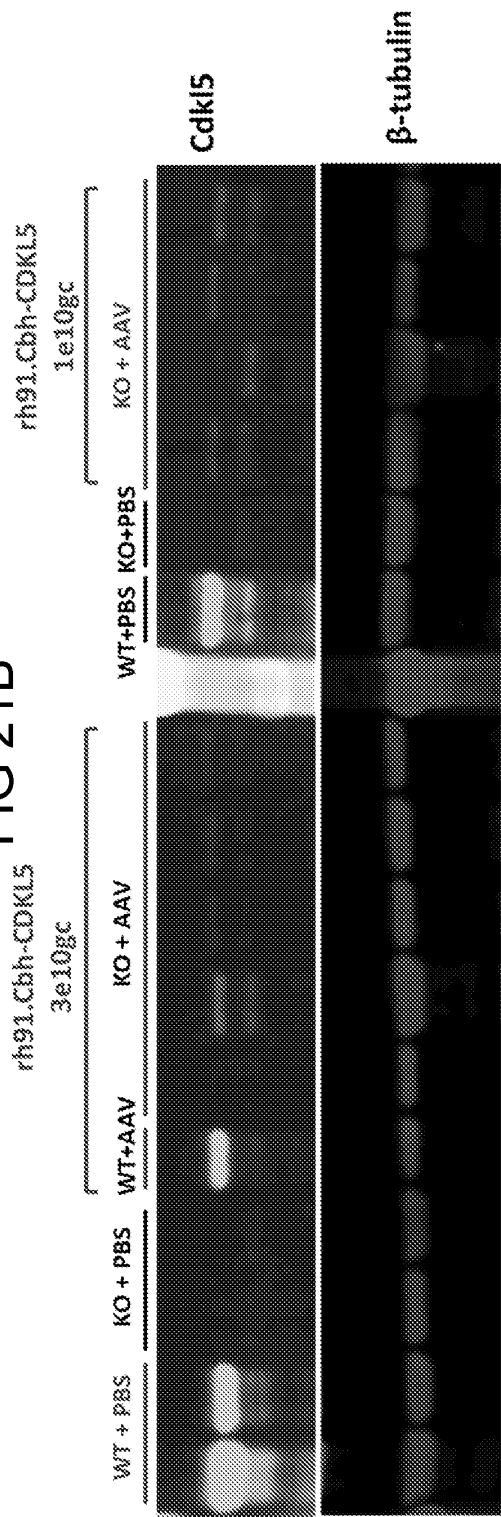


FIG 21C

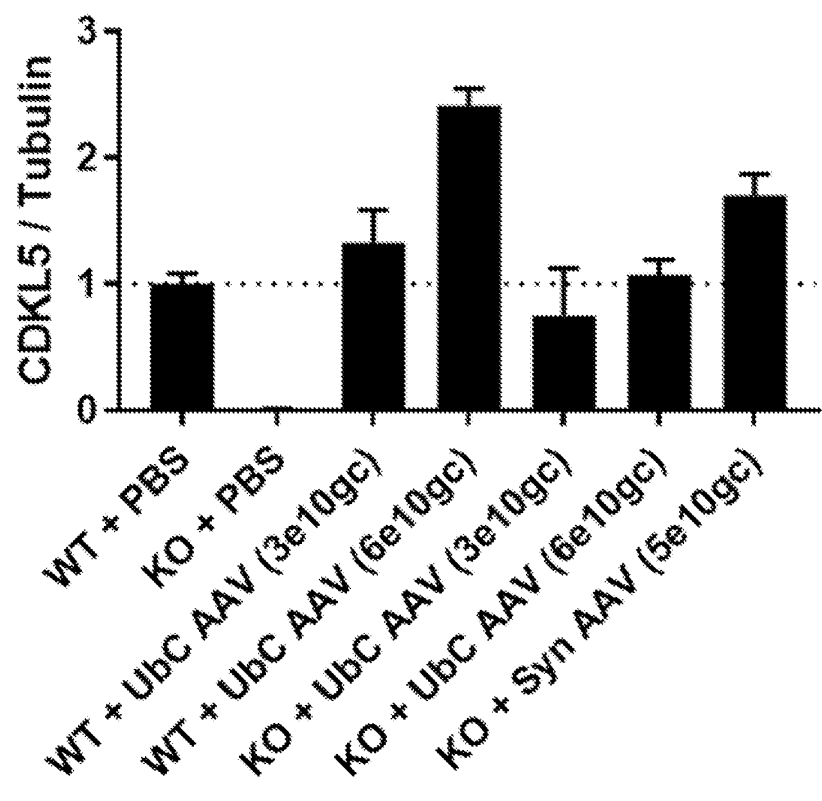


FIG 22A

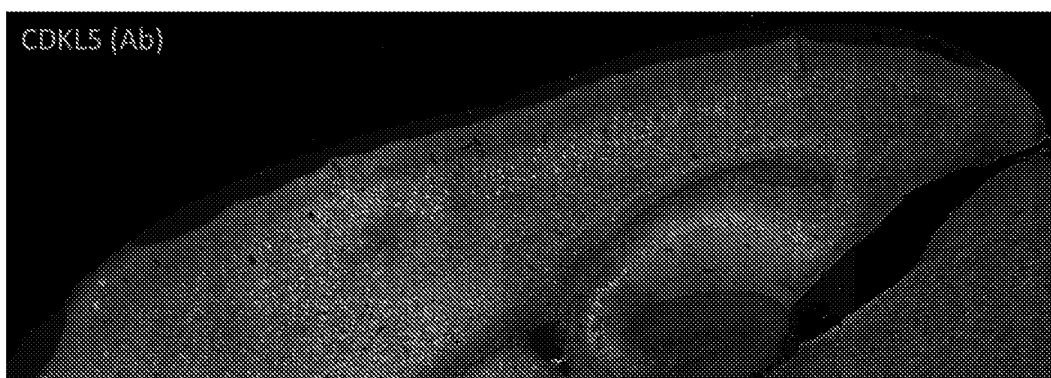


FIG 22B

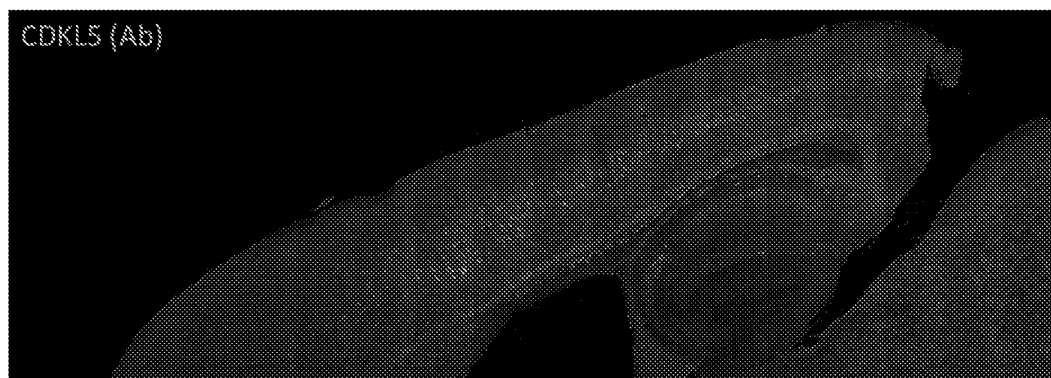
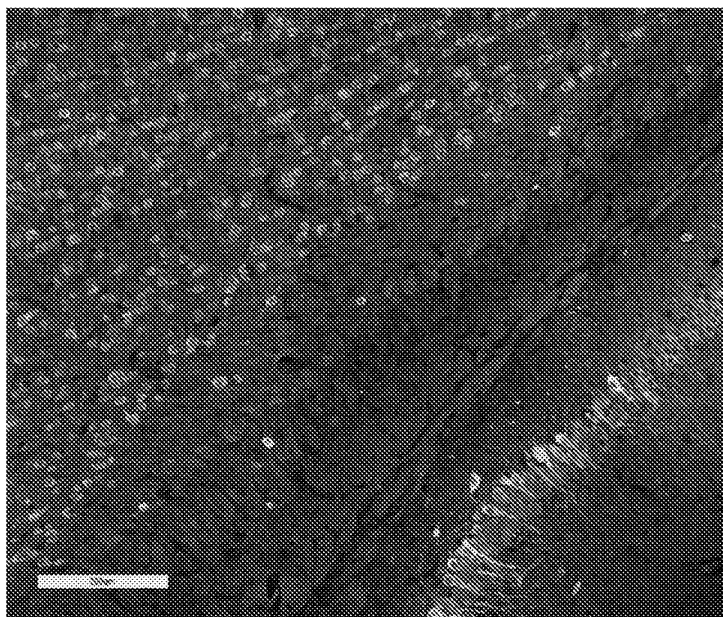


FIG 23A

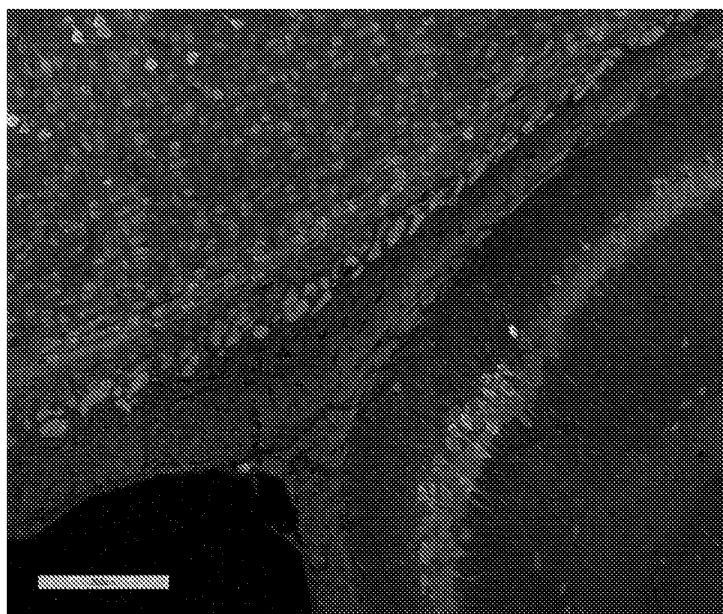
AAVrh91.UbC.CDKL5-1co.miR183



CDKL5 (Ab) NeuN (Ab)

FIG 23B

AAVrh91.CBh.CDKL5-1co.miR183



CDKL5 (Ab) NeuN (Ab)

FIG 24

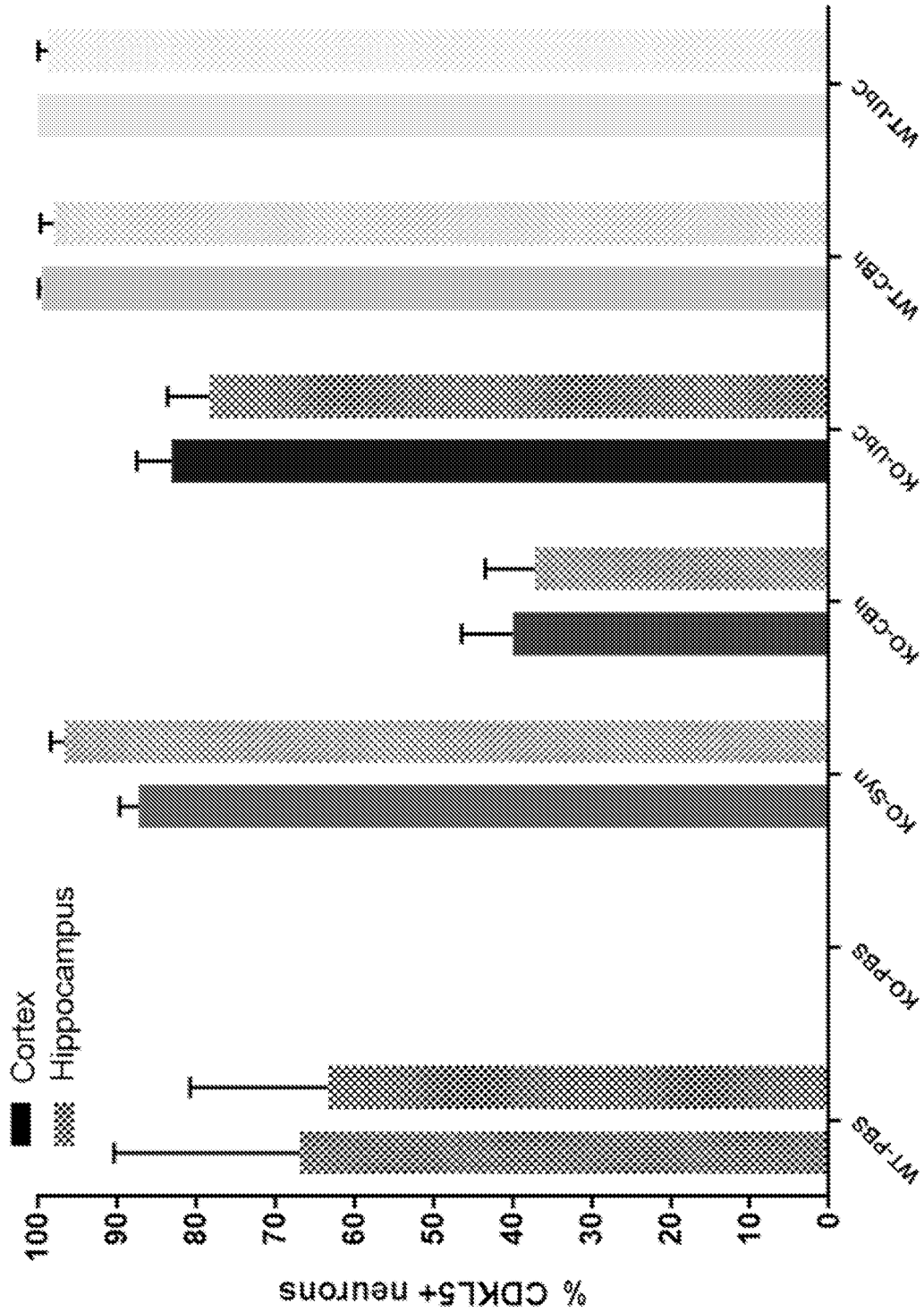


FIG 25A

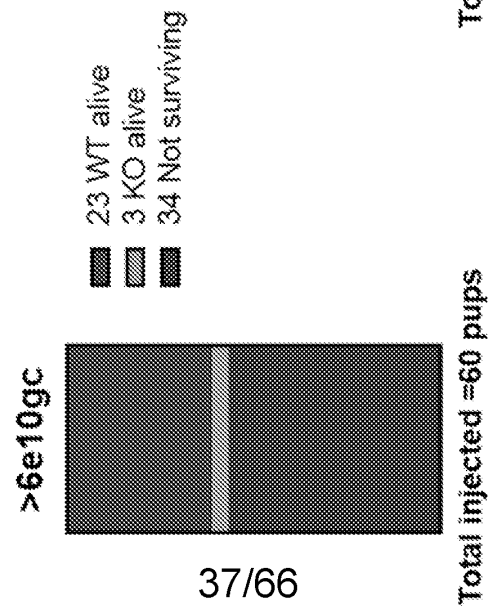


FIG 25B

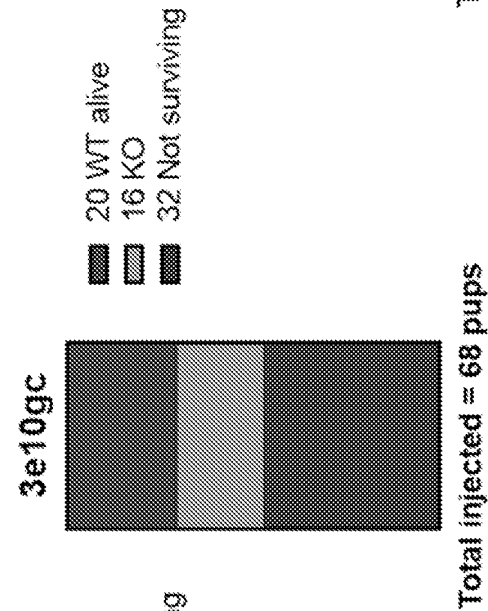


FIG 25C

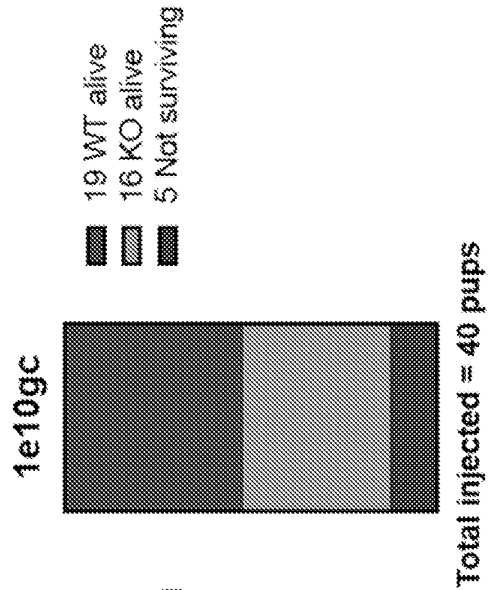


FIG 26A
DRG

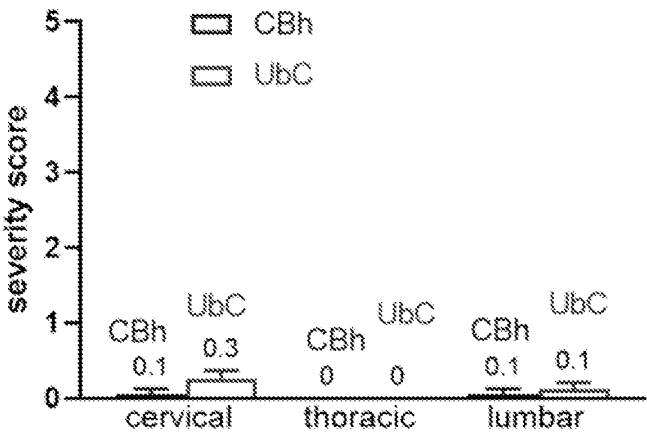


FIG 26B
Spinal Cord

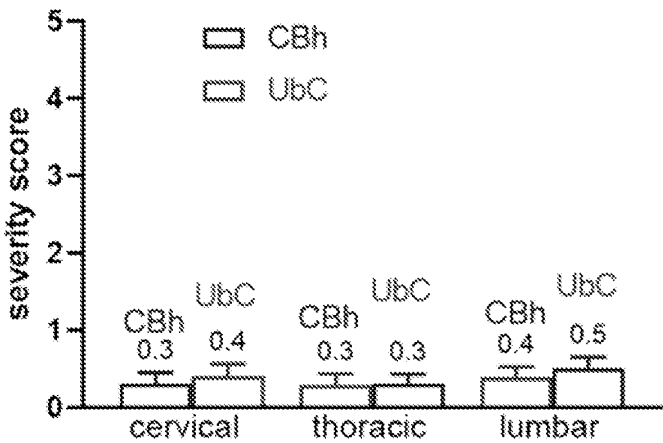


FIG 26C
Sural Nerve

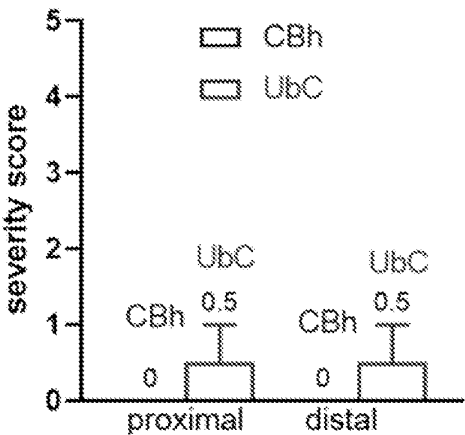


FIG 27

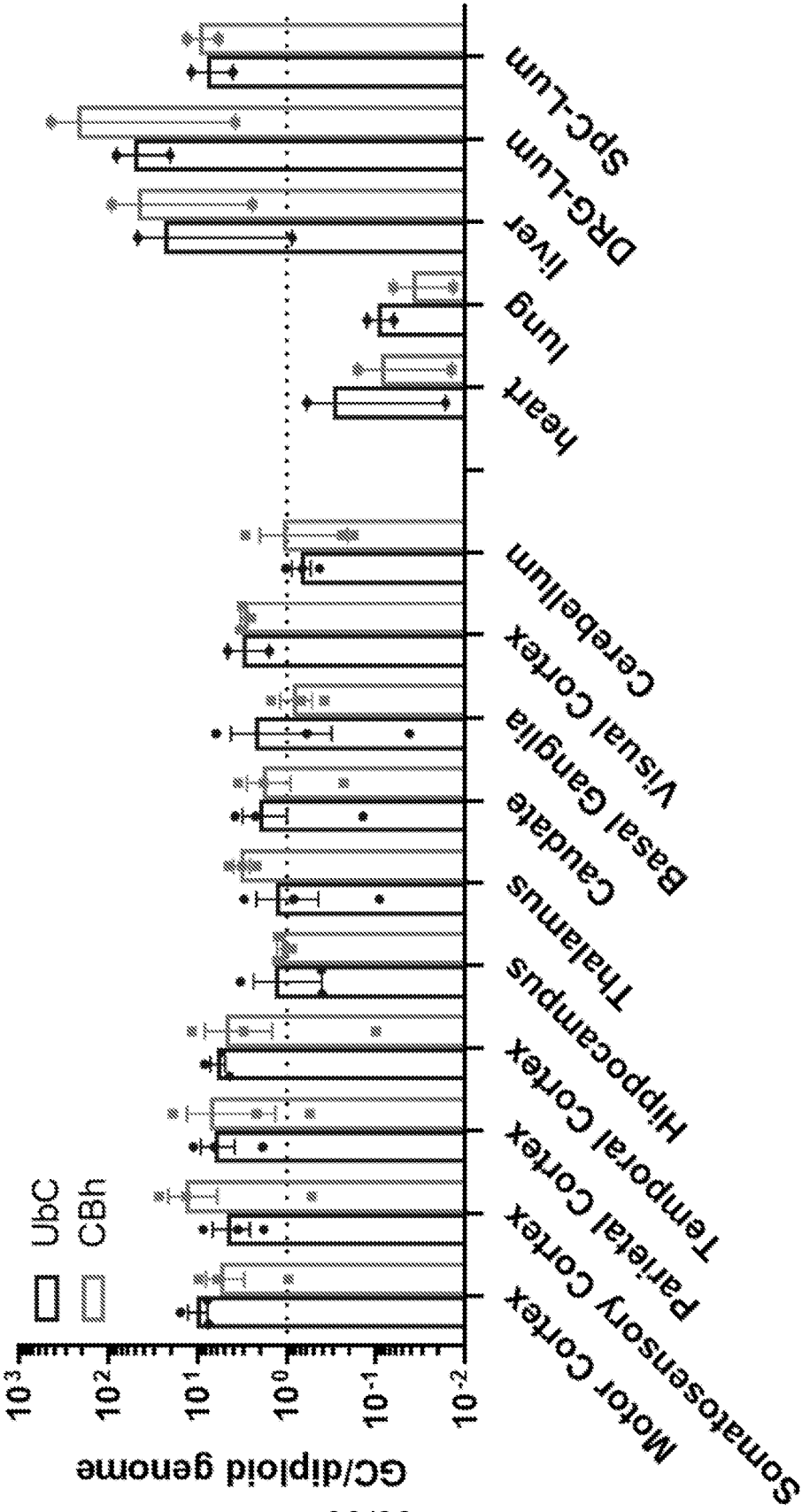


FIG 28

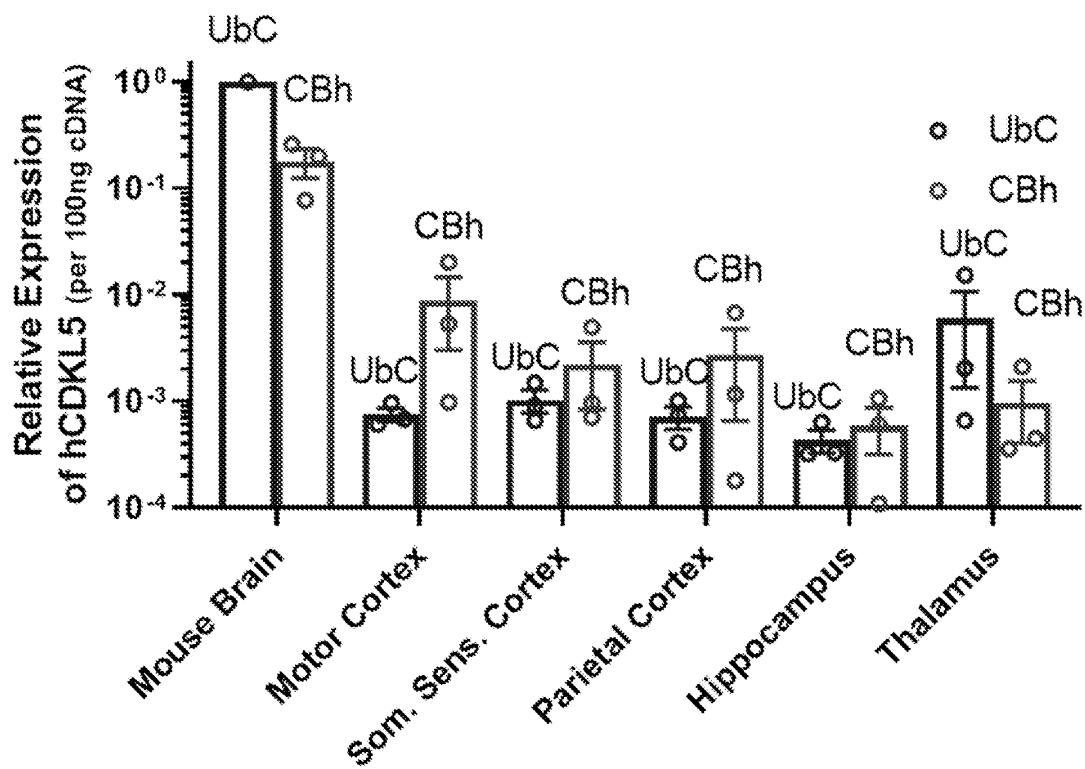


FIG 29A

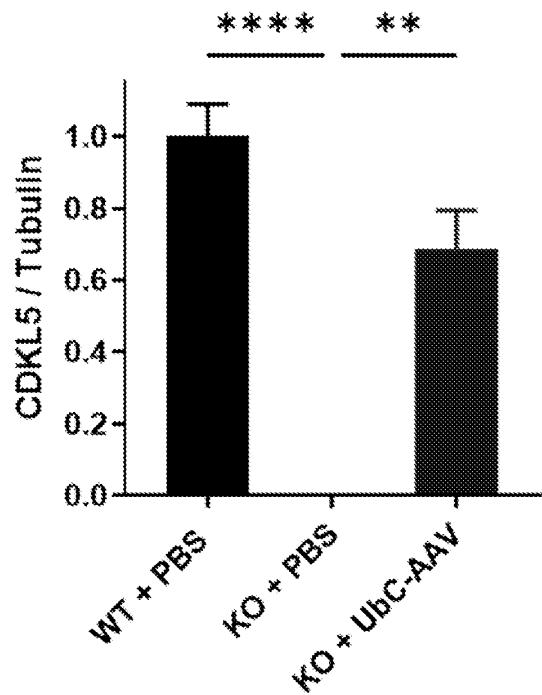


FIG 29B

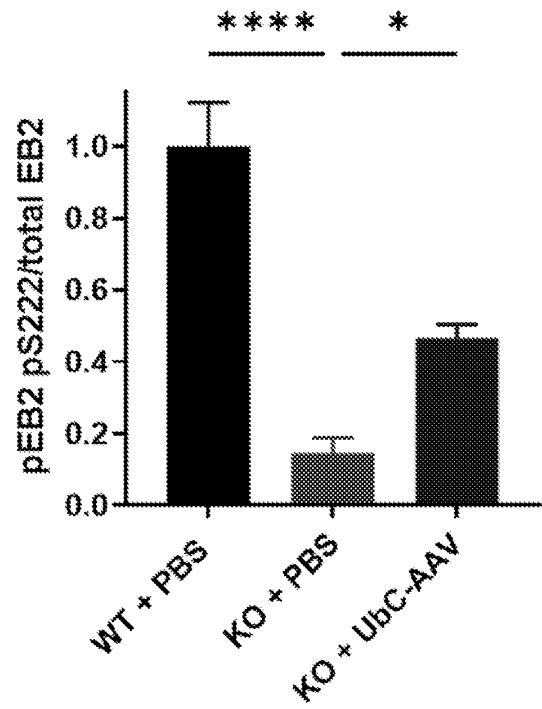


FIG 30

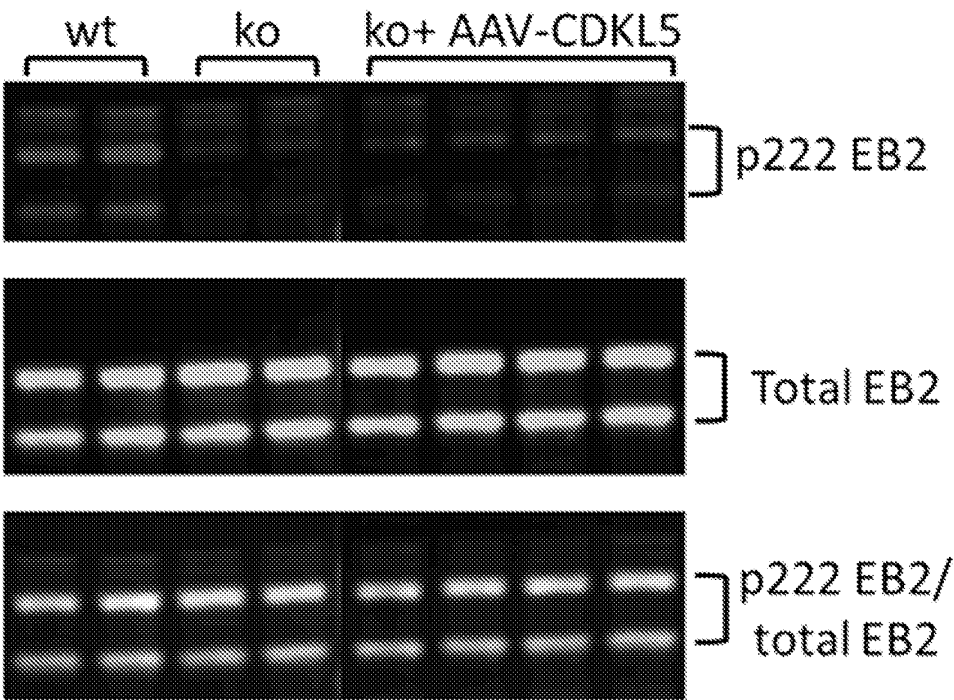


FIG 31A

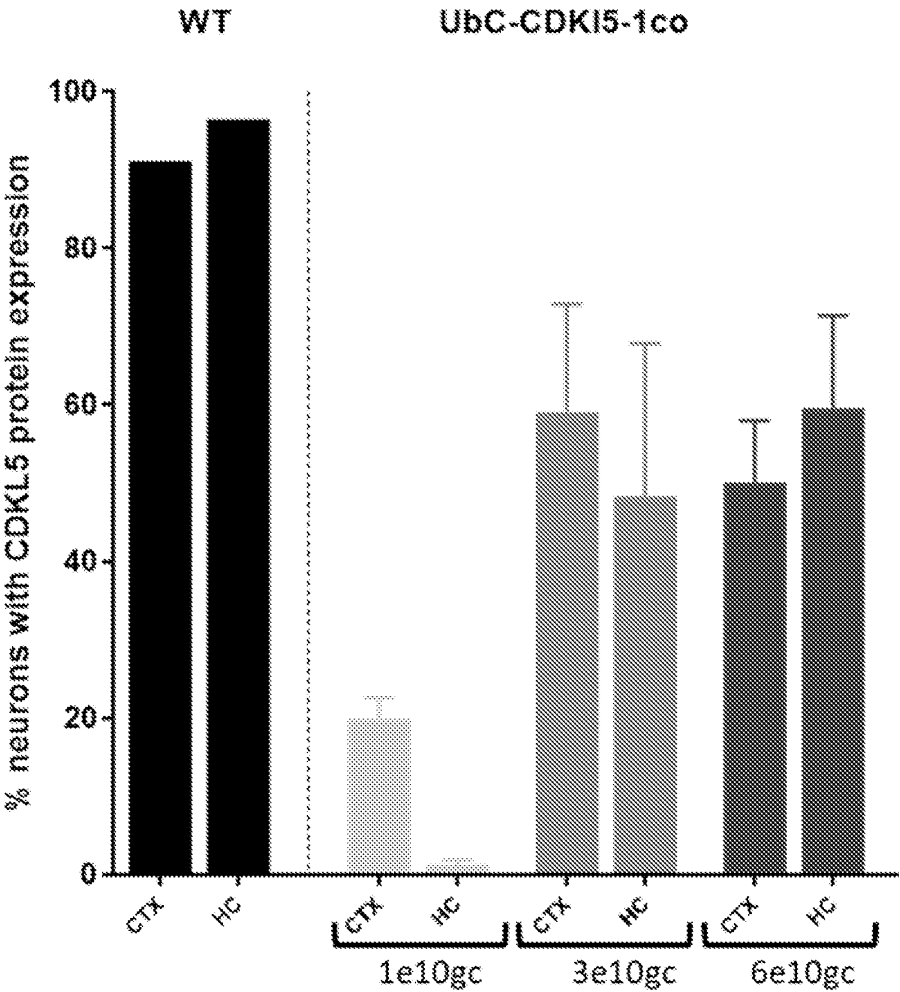
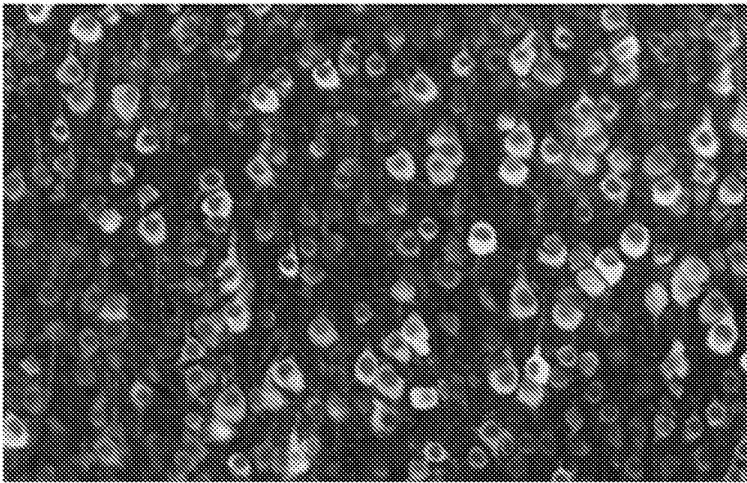


FIG 31B



DAPI CDKL5 NeuN

FIG 32

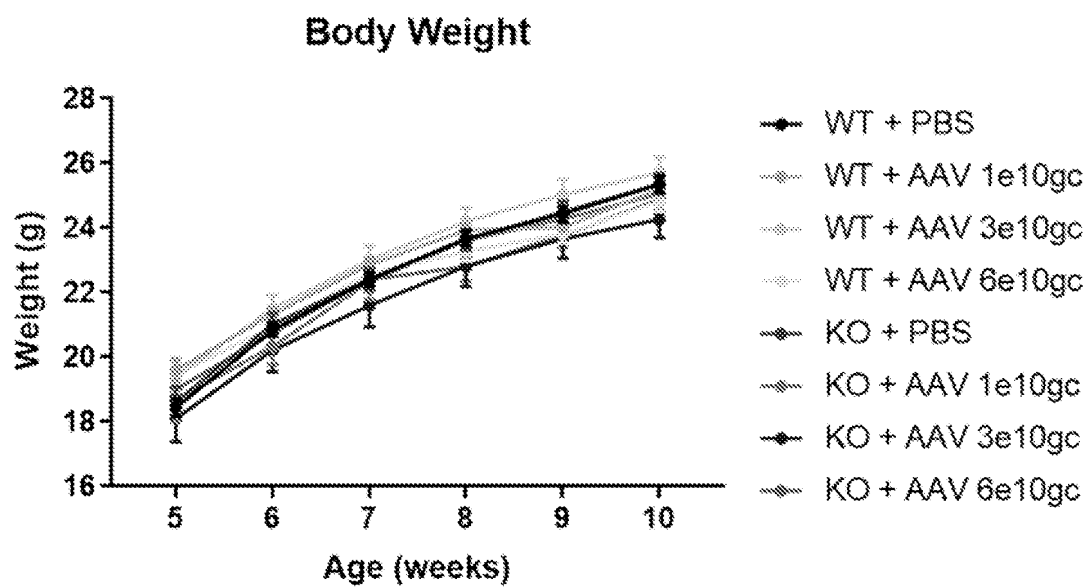


FIG 33A

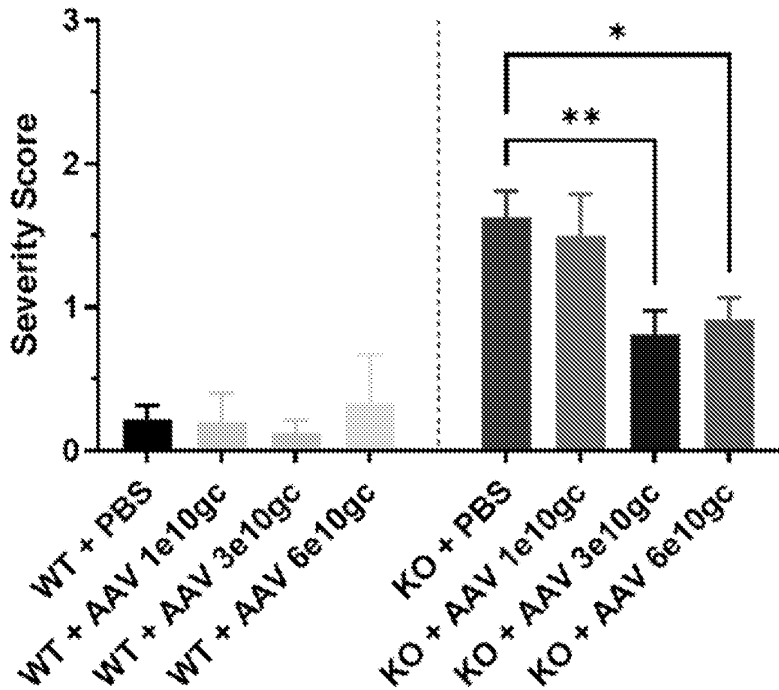


FIG 33B

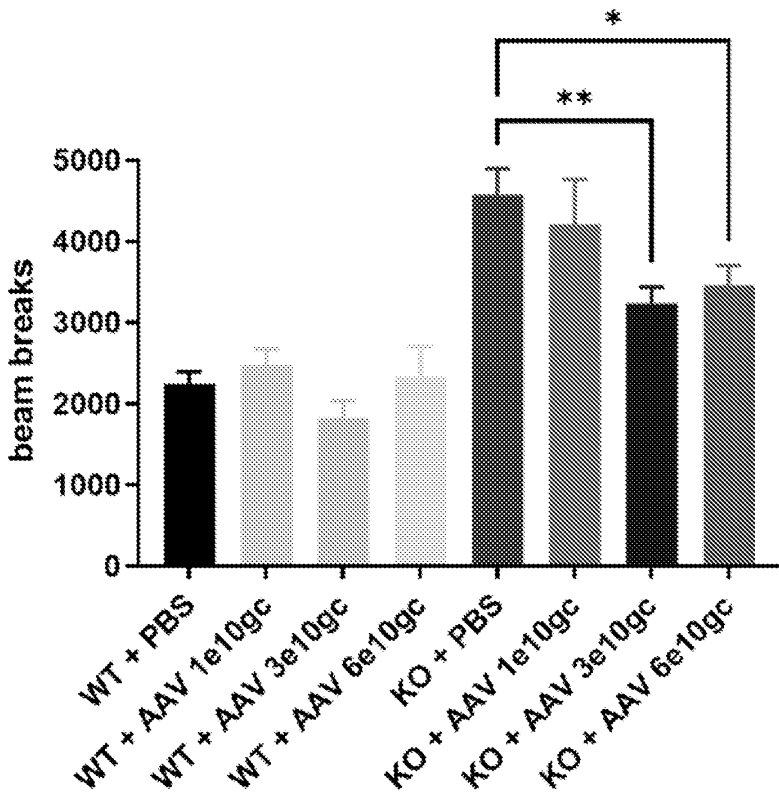


FIG 34A

Low dose

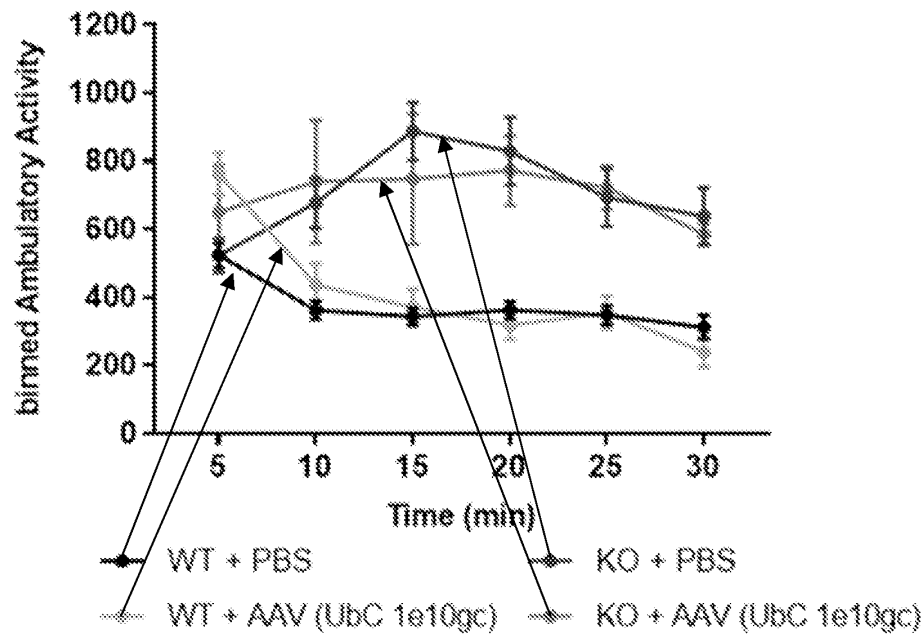


FIG 34B

Medium dose

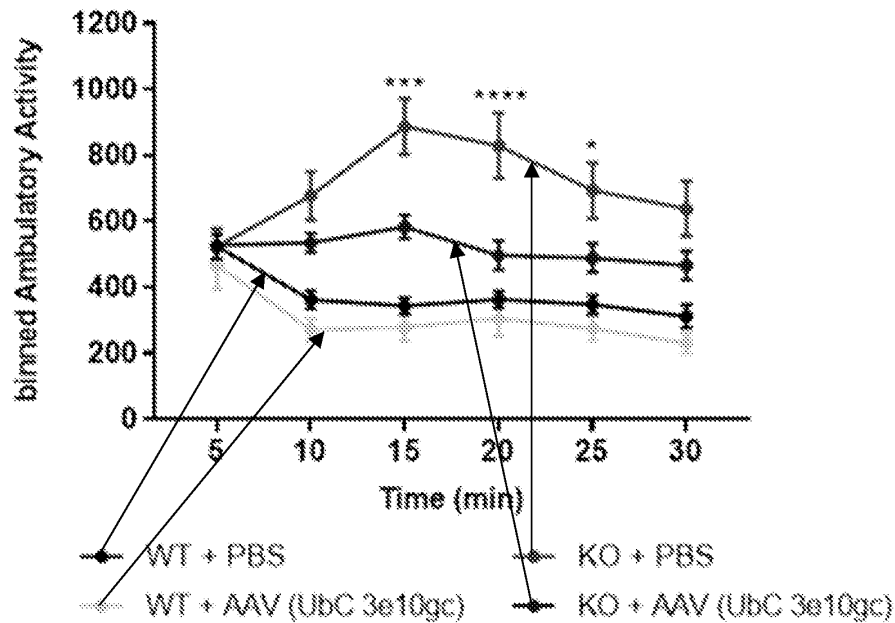


FIG 34C

High Dose

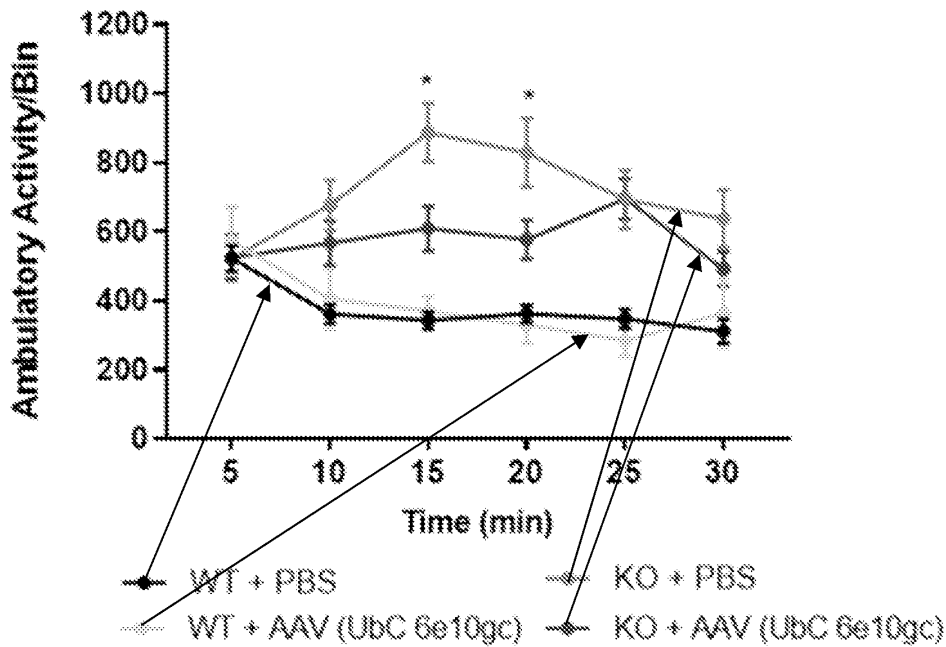


FIG 35

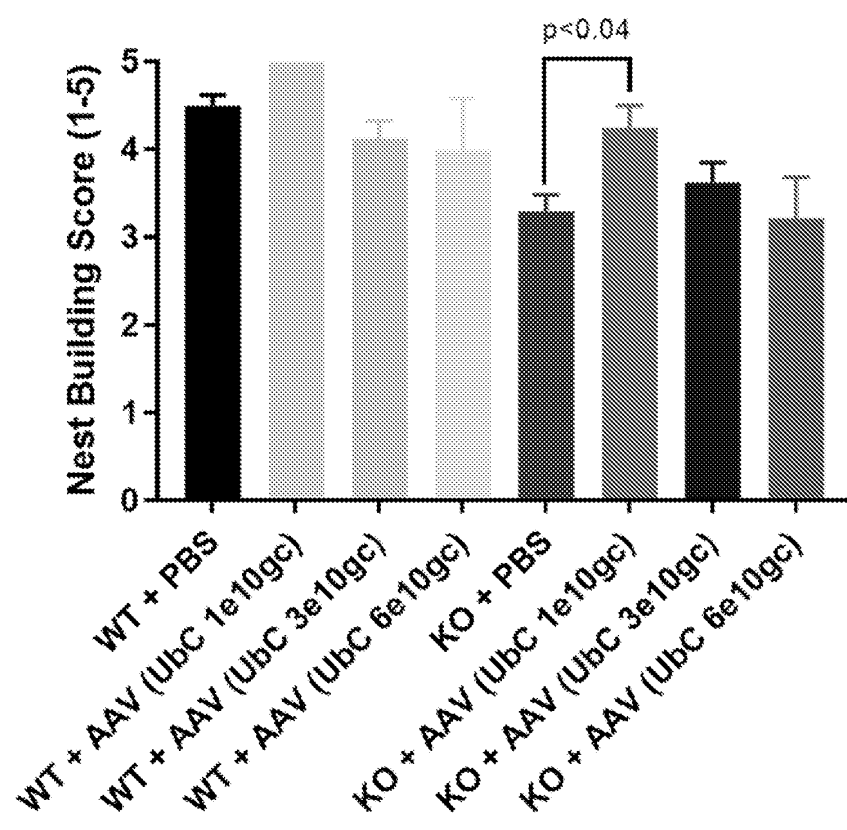


FIG 36A

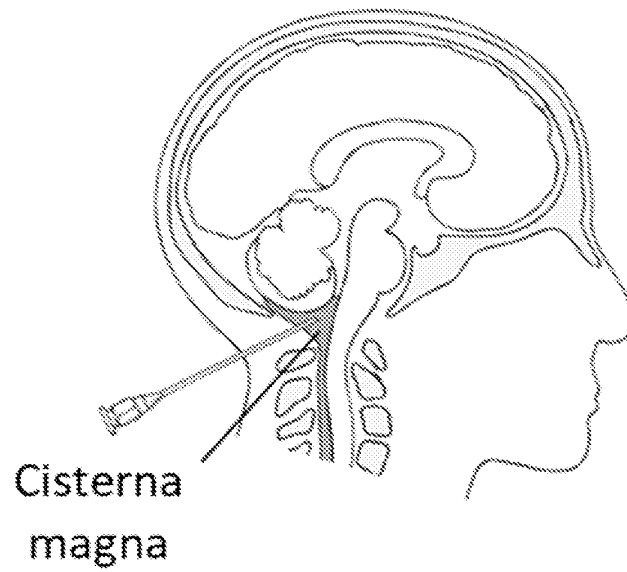
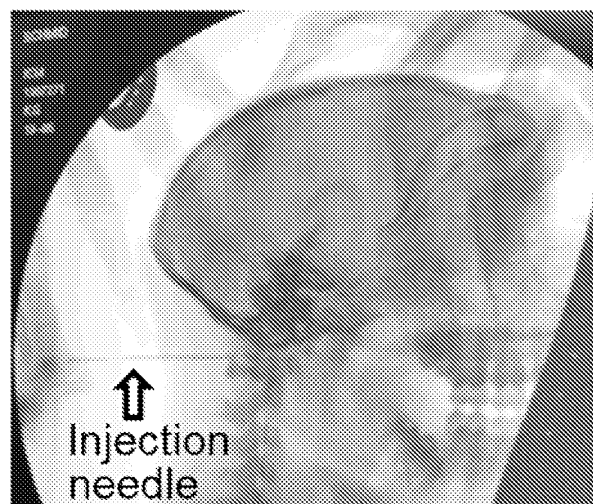


FIG 36B



Fluoroscopy-guided
procedure

FIG 37A

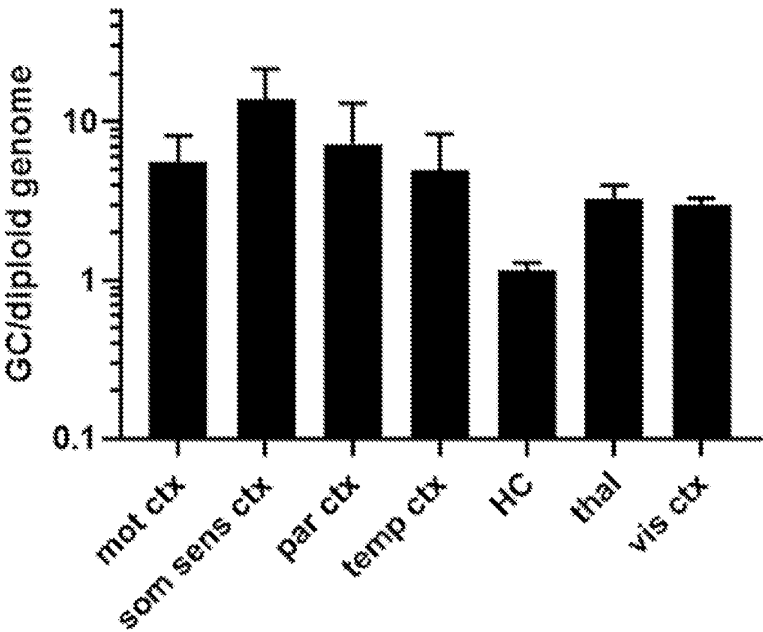
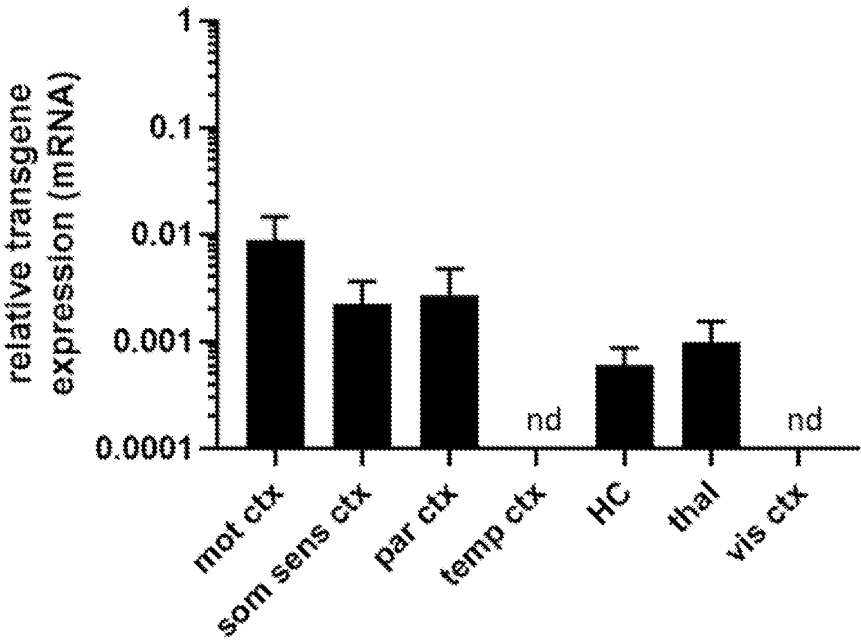


FIG 37B



nd = not determined
Expression relative to mouse brain (3e10gc dose)

FIG 38A

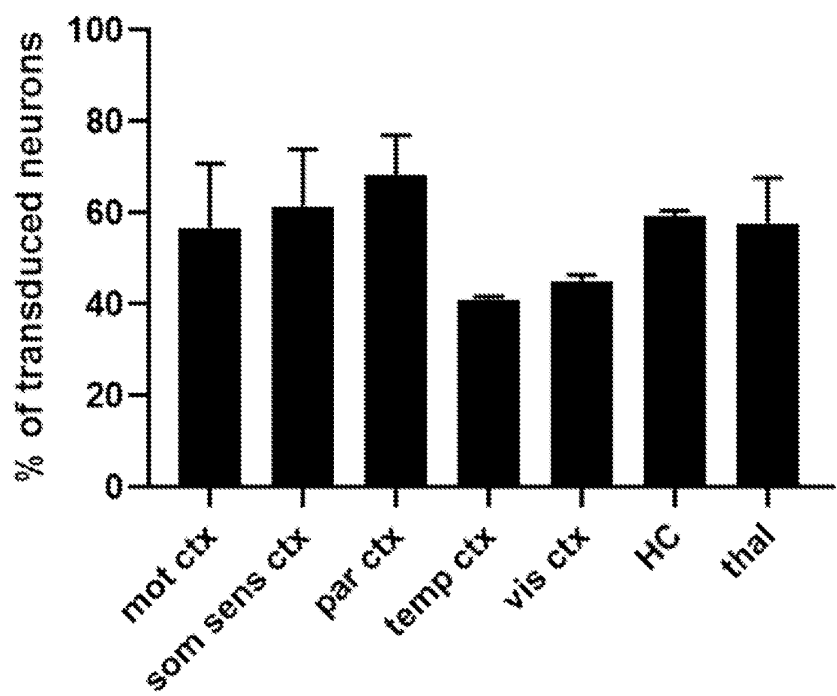
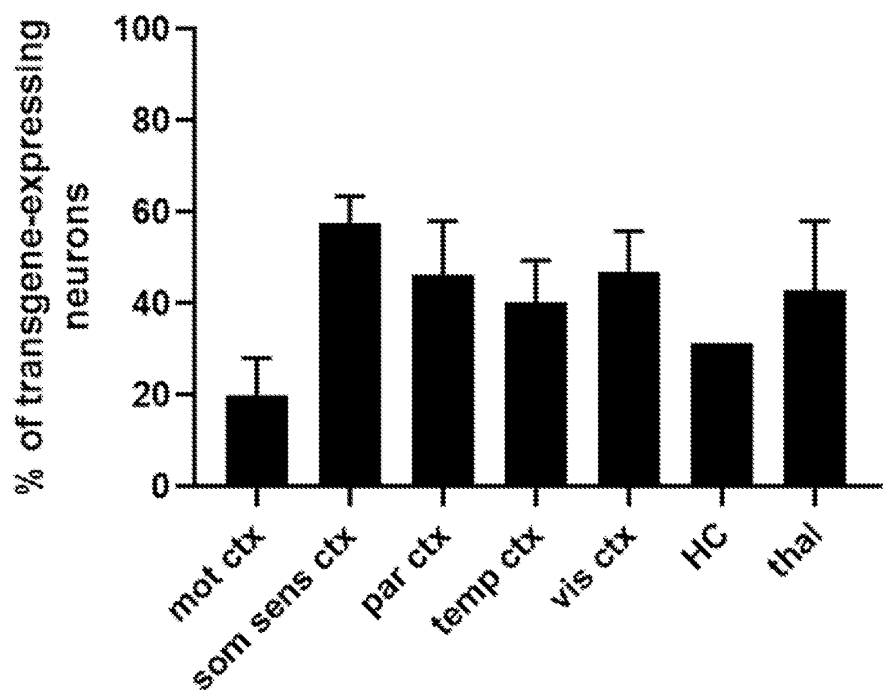


FIG 38B



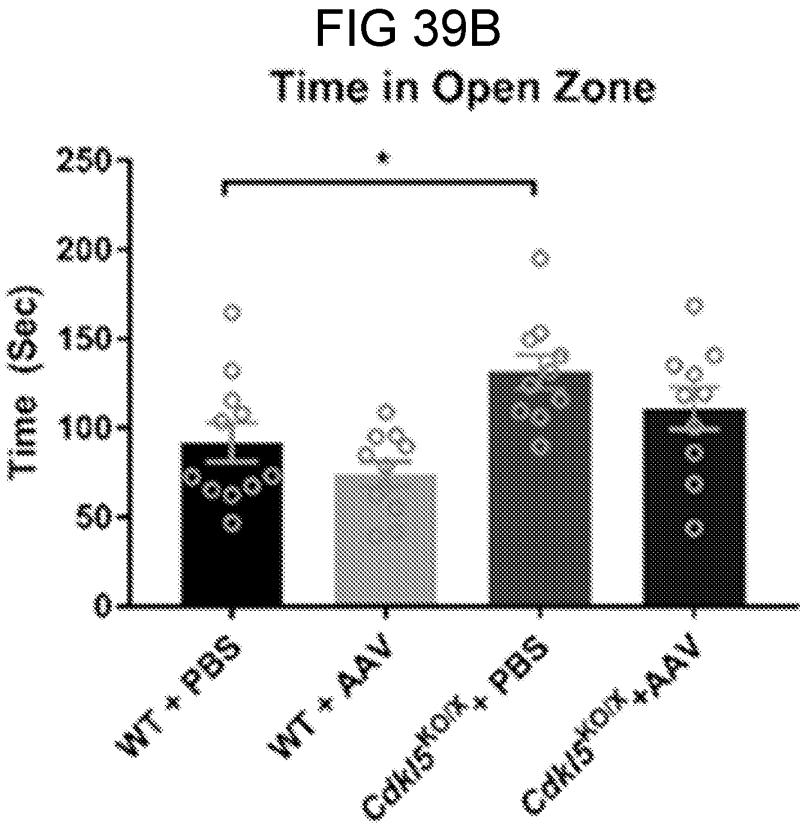
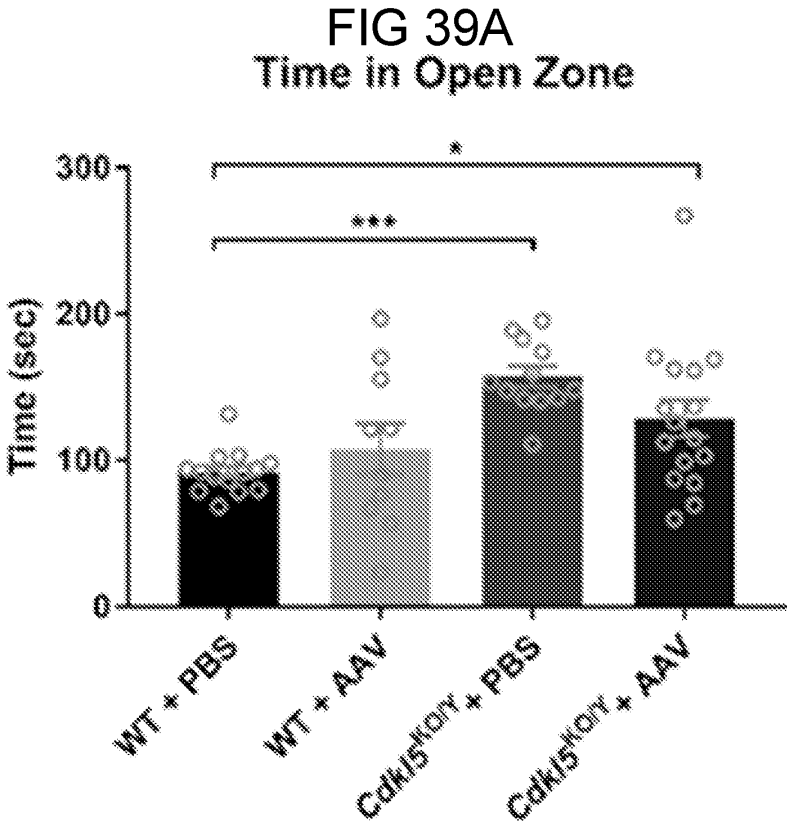


FIG 40A
Open Zone Entries

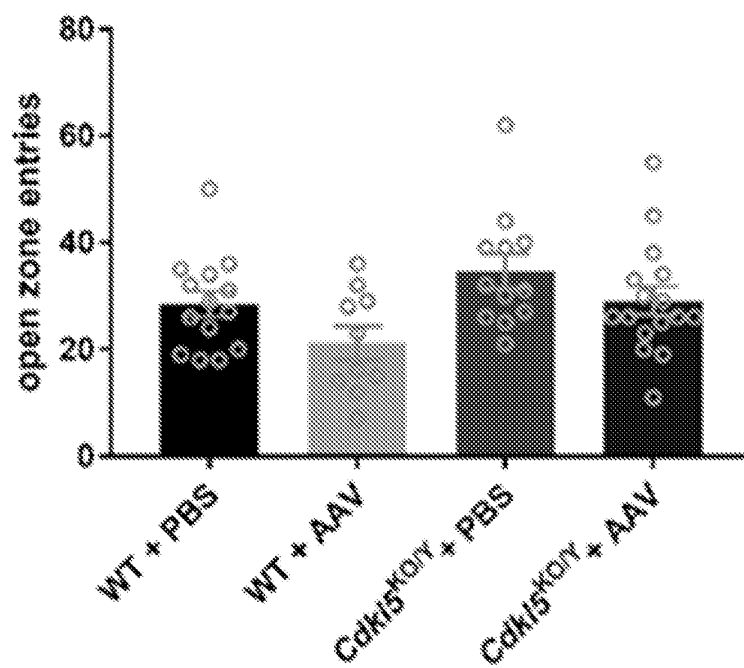


FIG 40B

Open Zone Entries

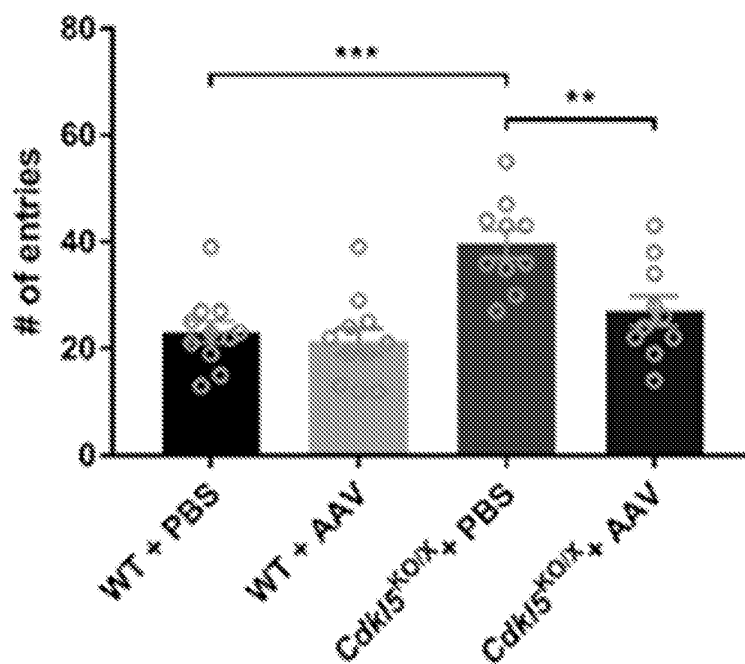


FIG 41A
Total Distance Moved

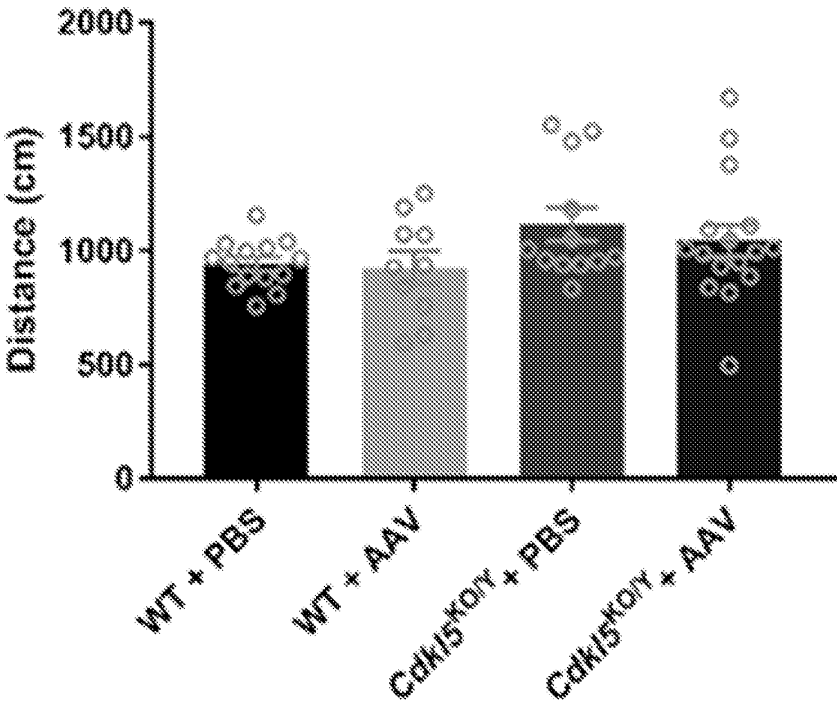


FIG 41B
Total Distance Moved

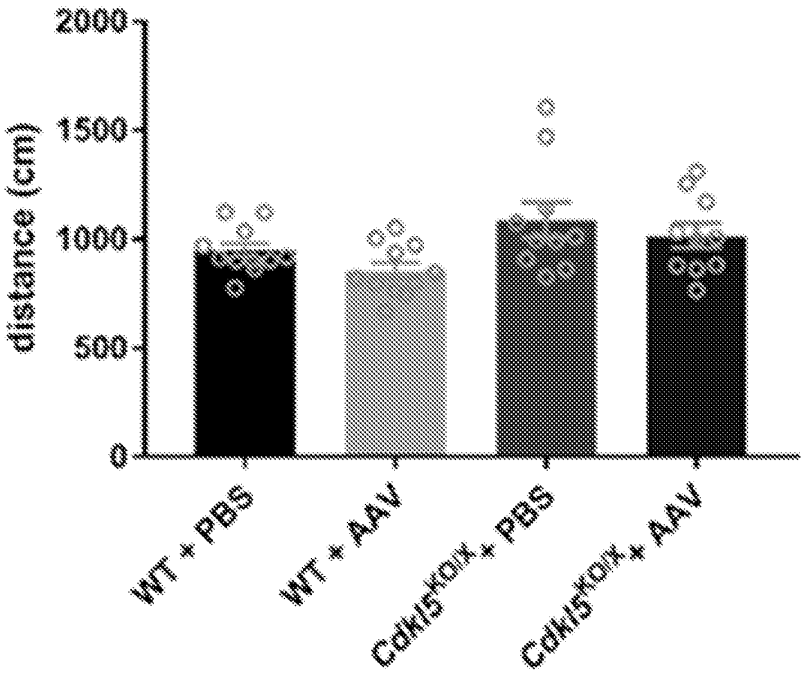


FIG 42A
Males

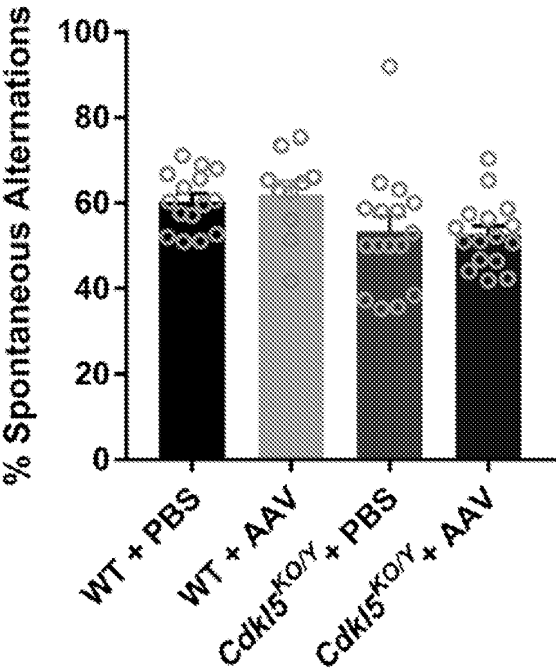


FIG 42B
Females

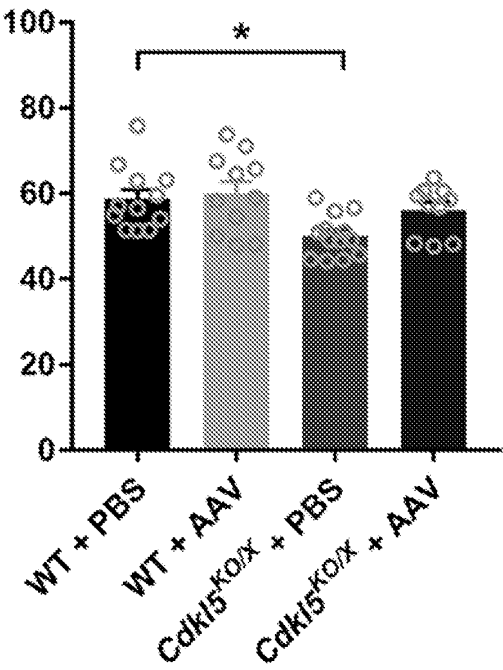


FIG 43A
Males

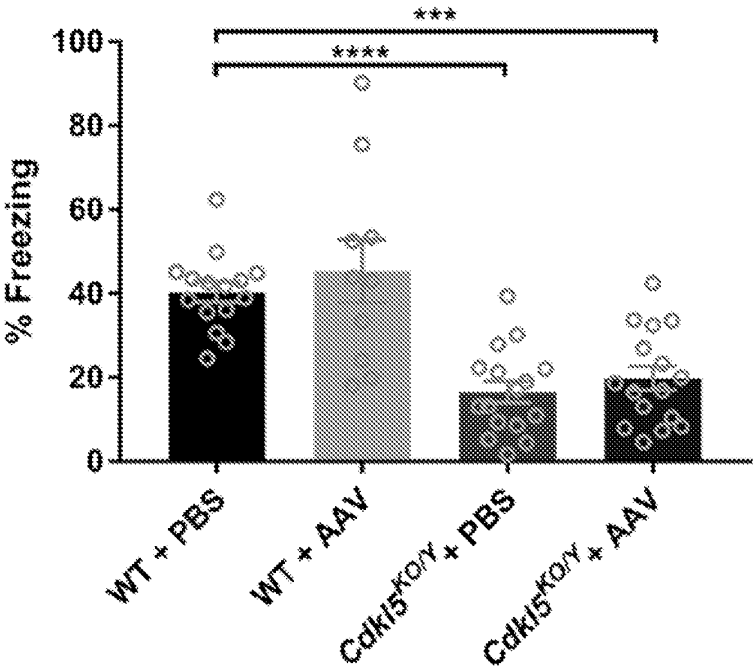


FIG 43B
Females

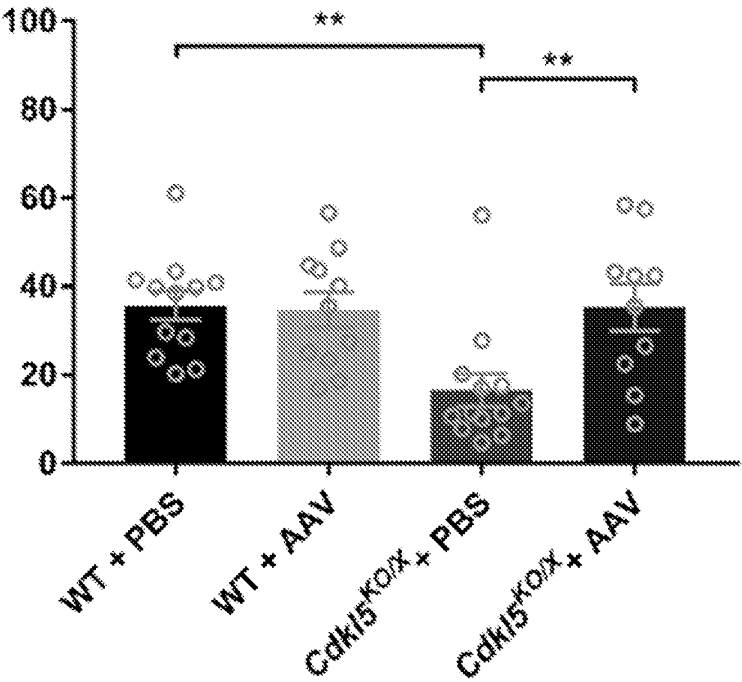


FIG 44A
Western Blot
CDKL5

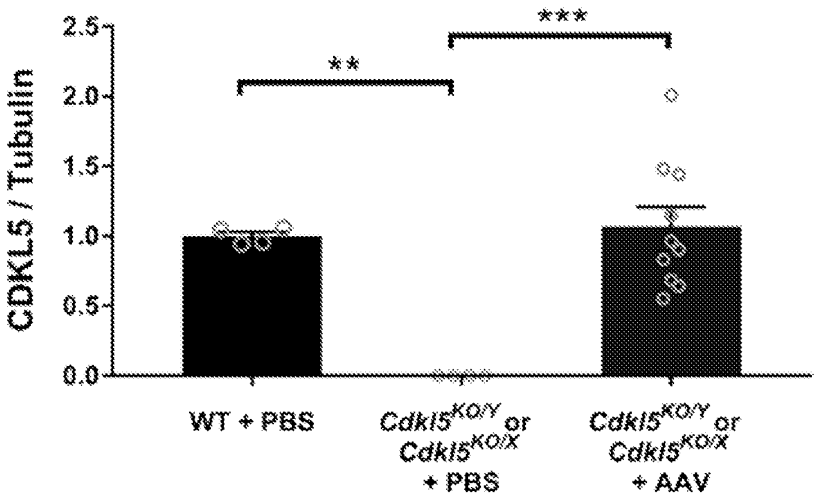


FIG 44B
Phospho-EB2

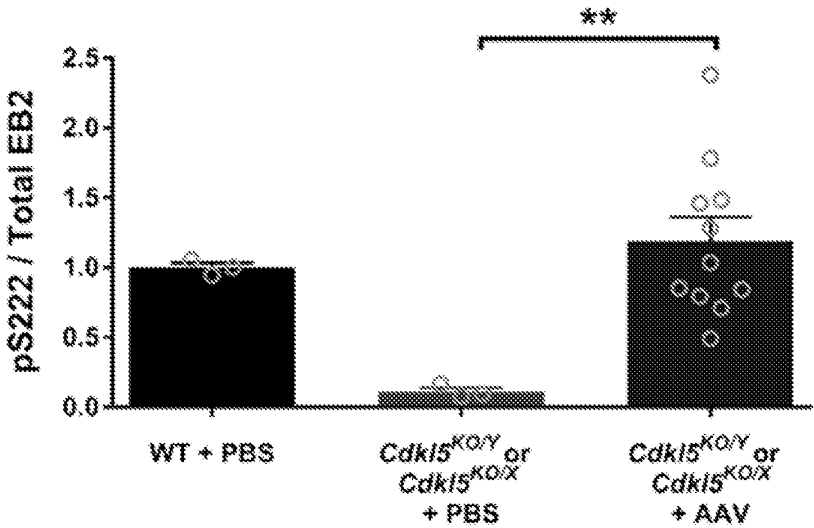


FIG 45A

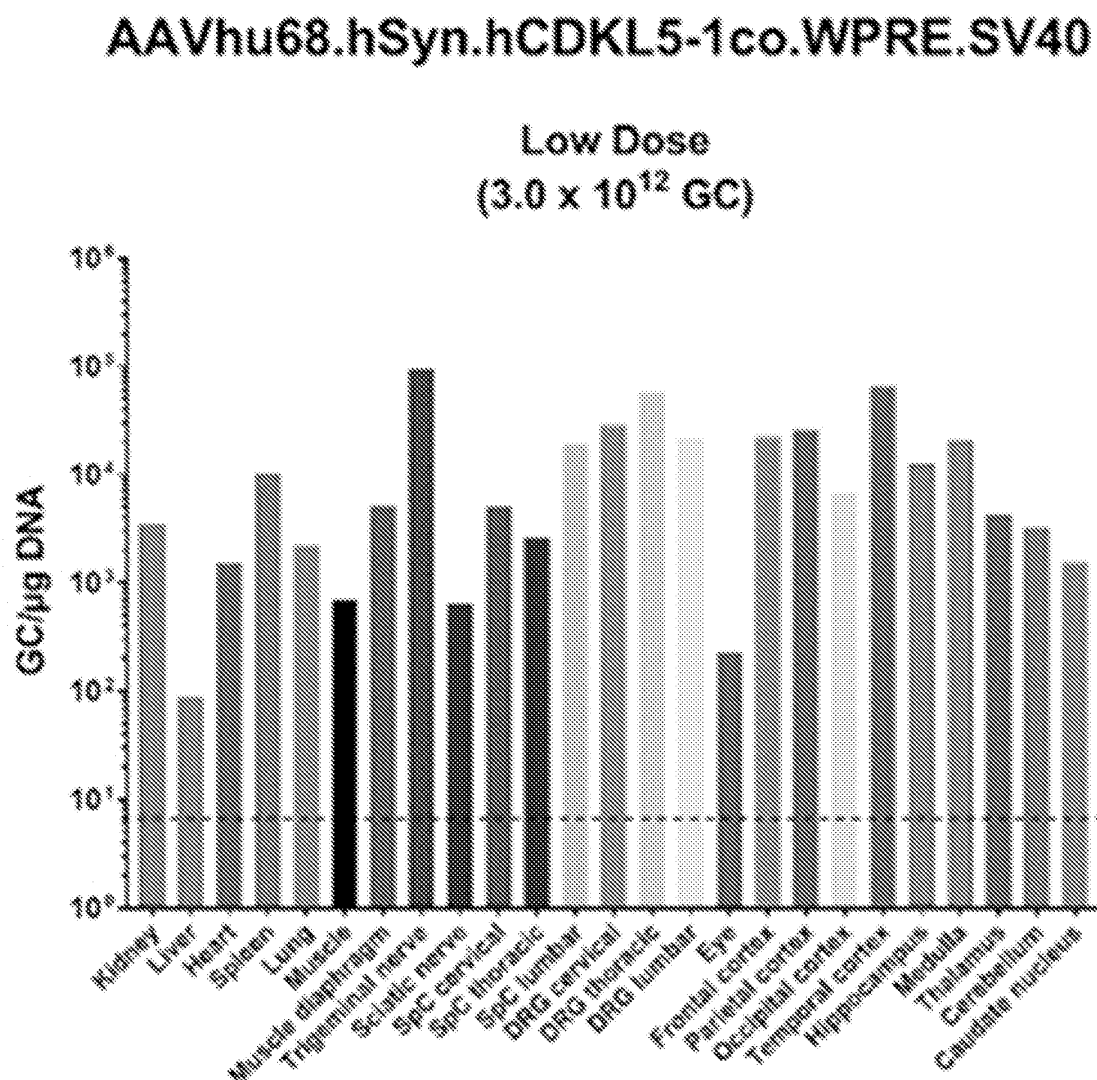


FIG 45B

AAVhu68.UbC.hCDKL5-1co.SV40

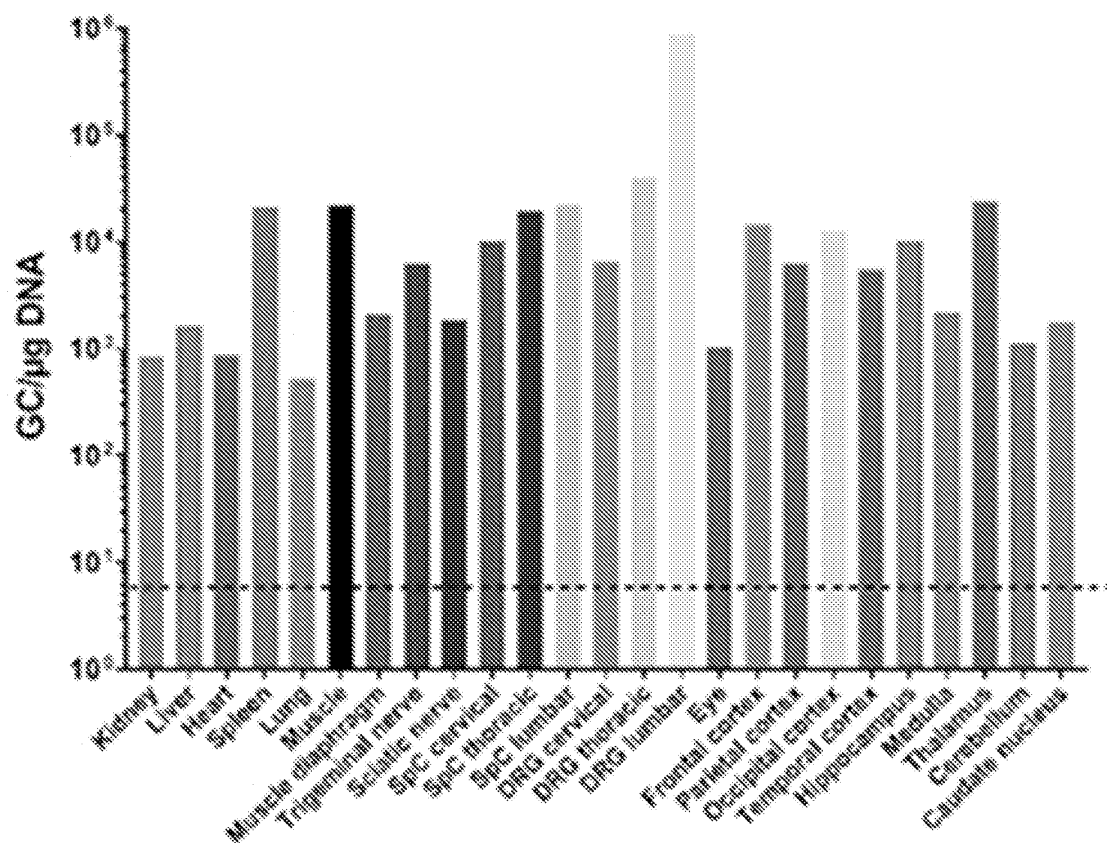
Low Dose
(3.0×10^{12} GC)

FIG 45C

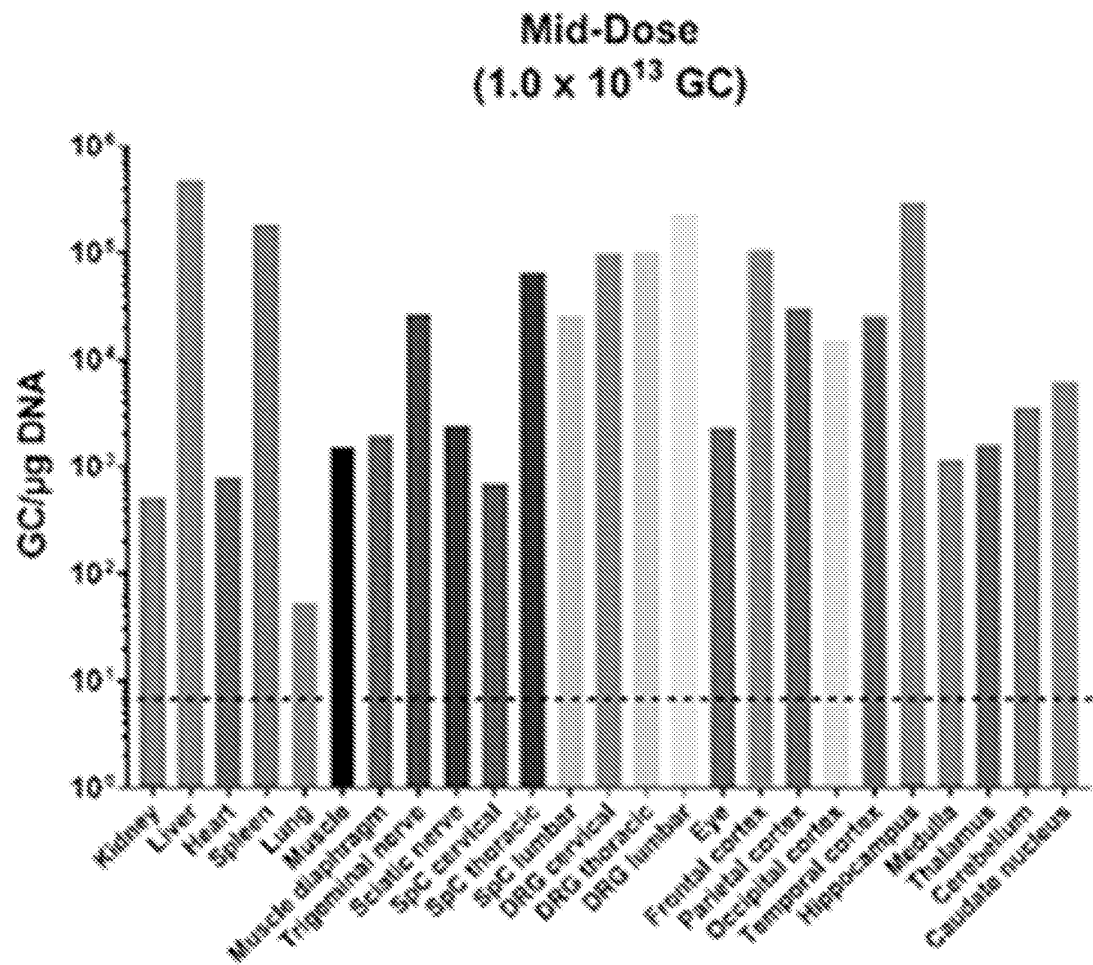


FIG 45D

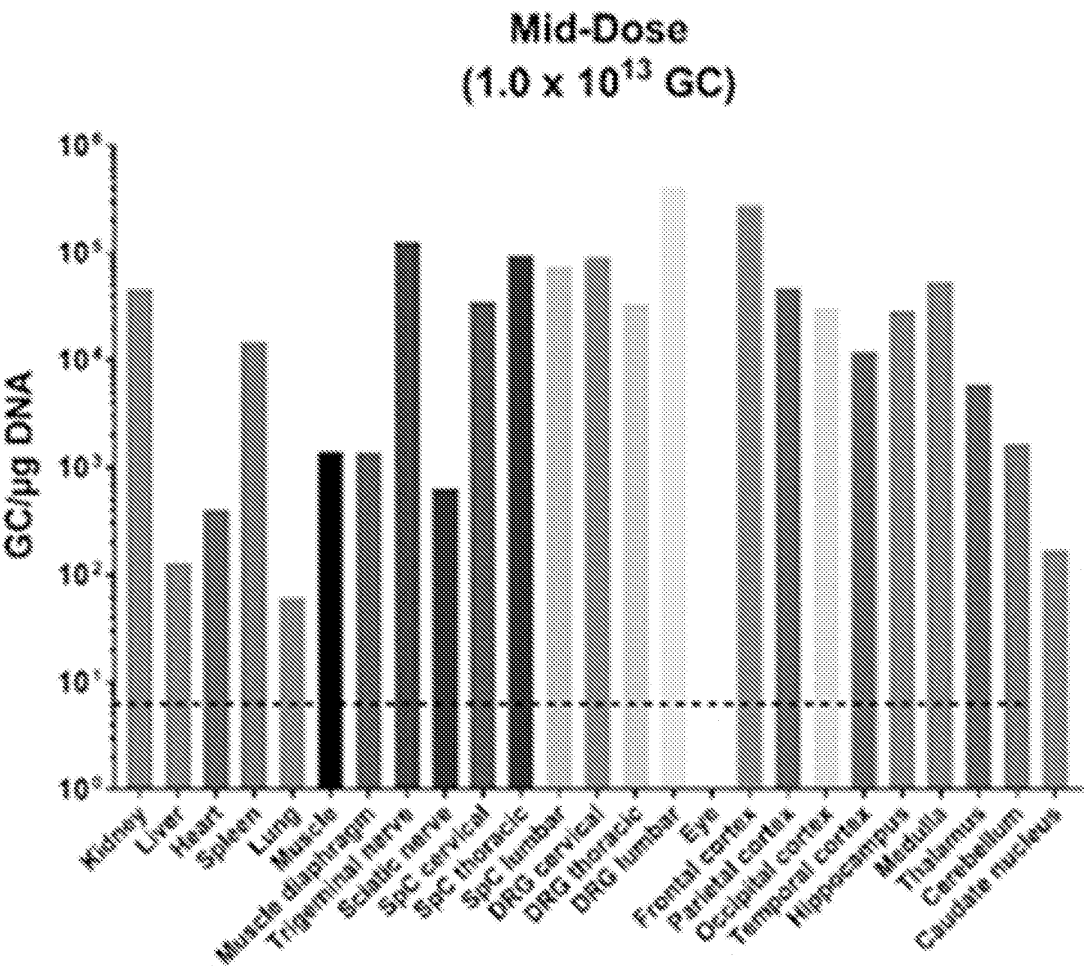


FIG 45E

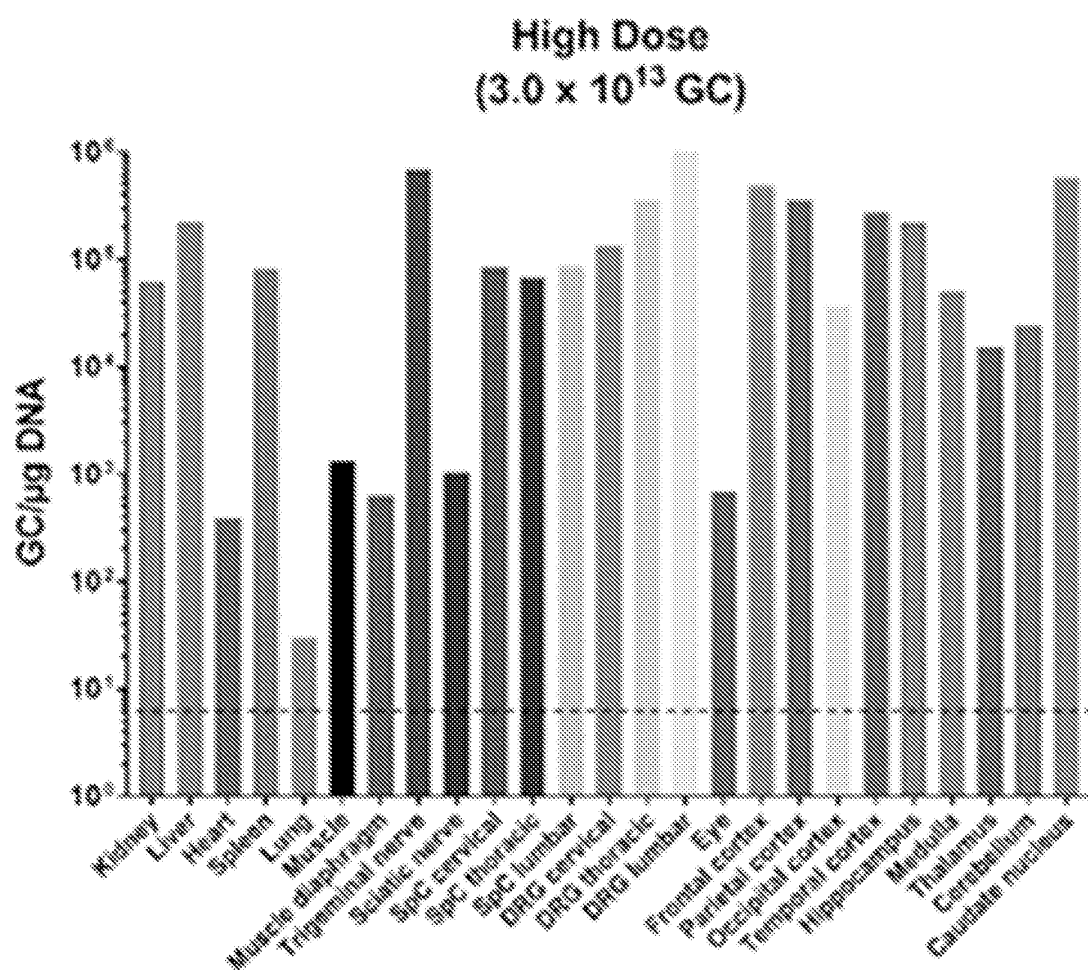


FIG 45F

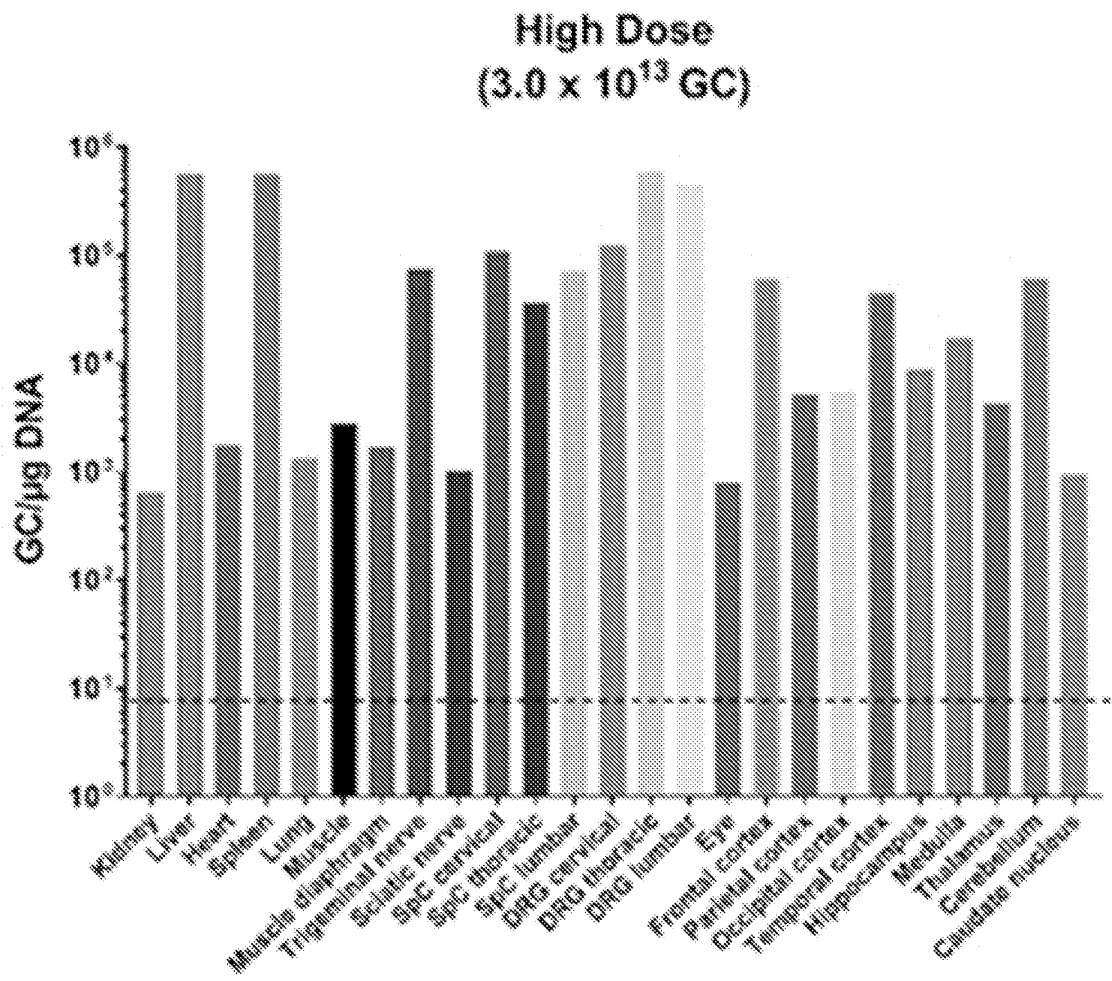


FIG 46

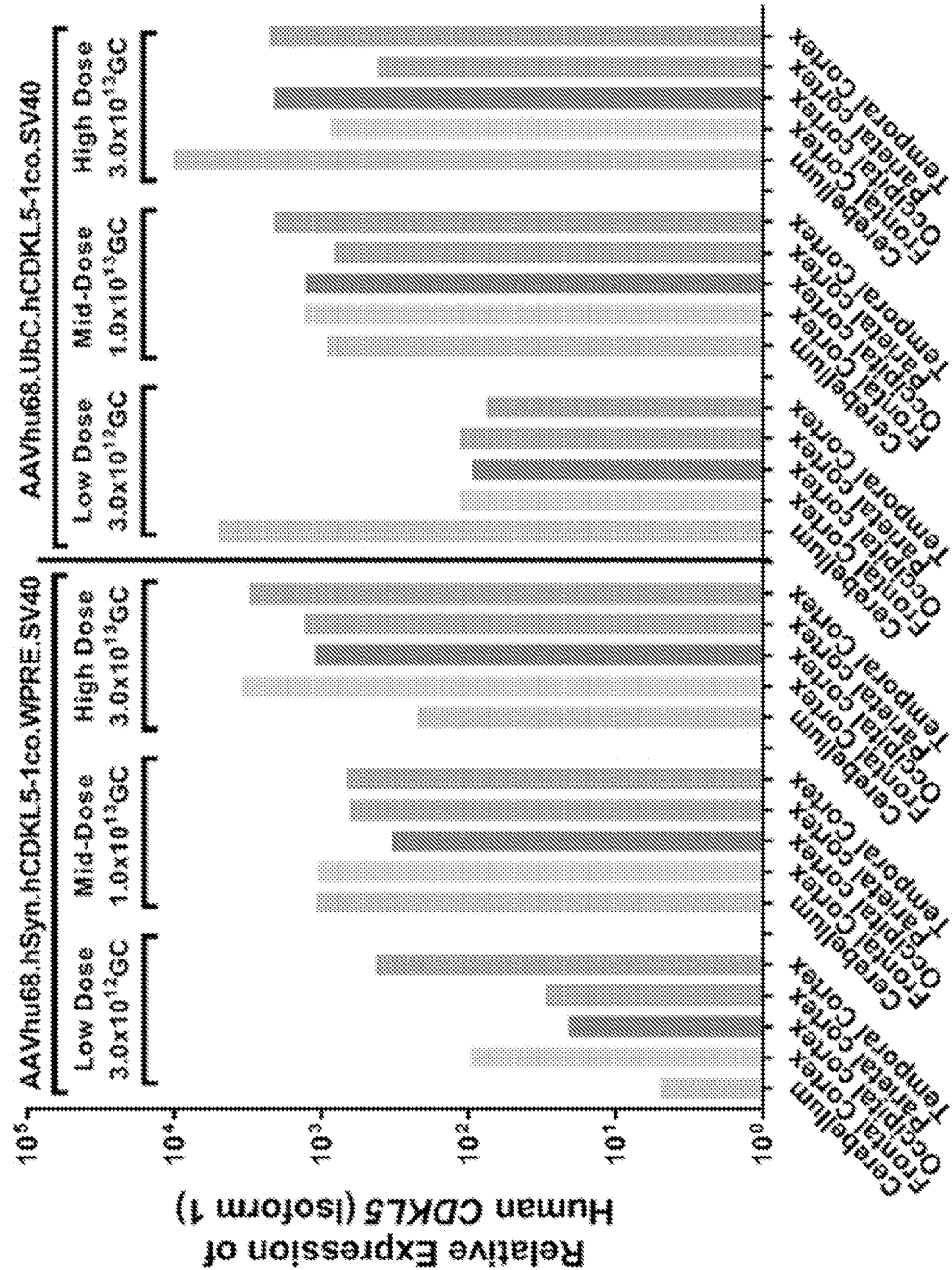
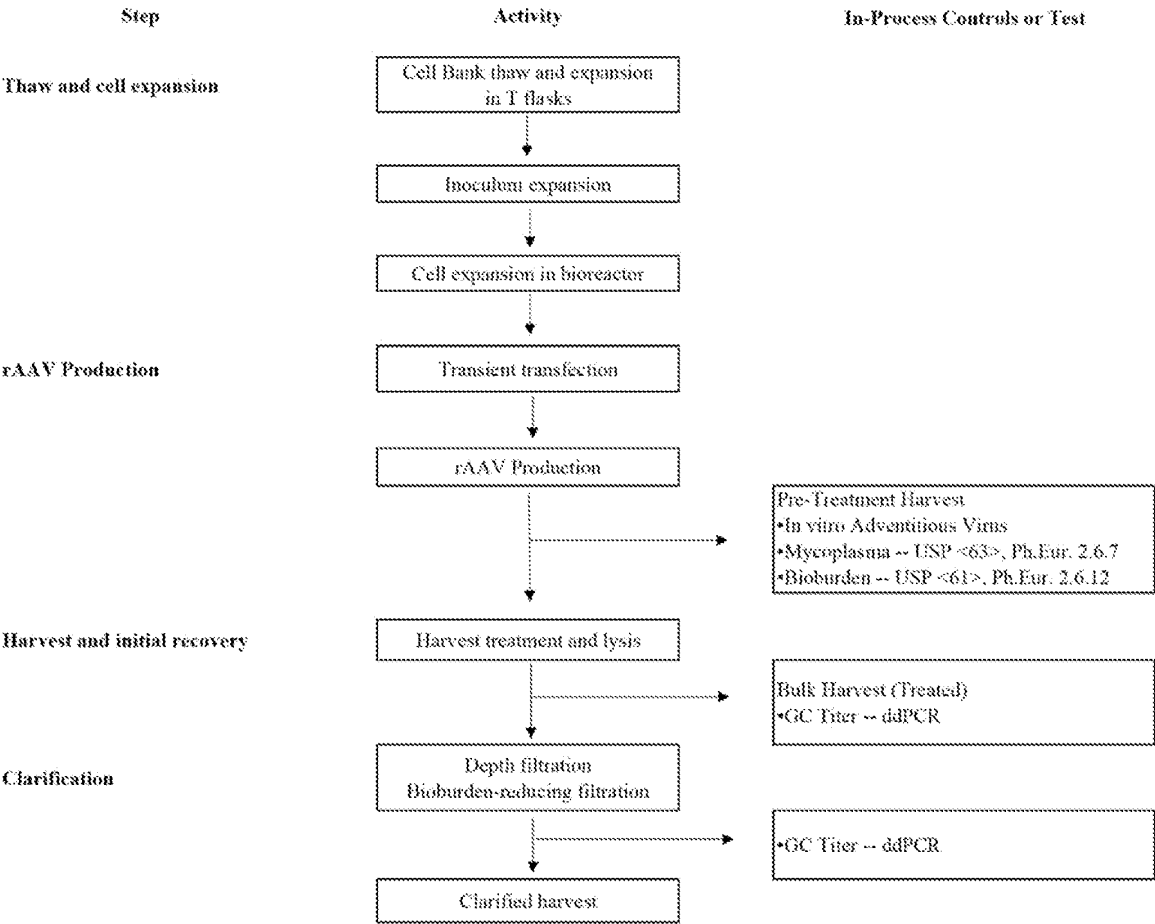
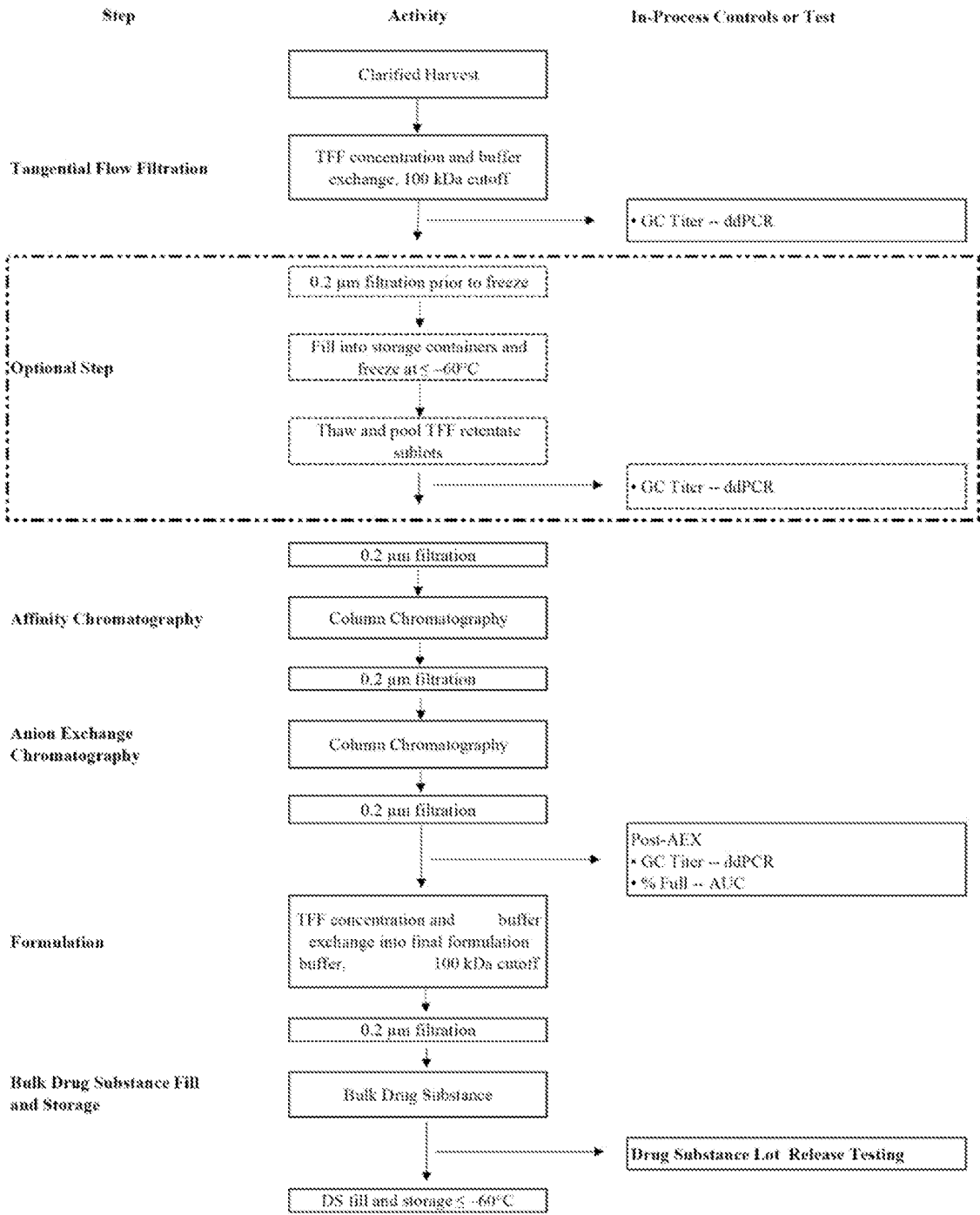


FIG 47A



Abbreviations: ddPCR, droplet digital polymerase chain reaction; GC, genome copies; Ph. Eur., European Pharmacopoeia; rAAV, recombinant adeno-associated virus; USP, United States Pharmacopoeia.

FIG 47B



Abbreviations: AEX, anion exchange; ddPCR, droplet digital polymerase chain reaction; DS, drug substance; GC, genome copies; TFF, tangential flow filtration.