

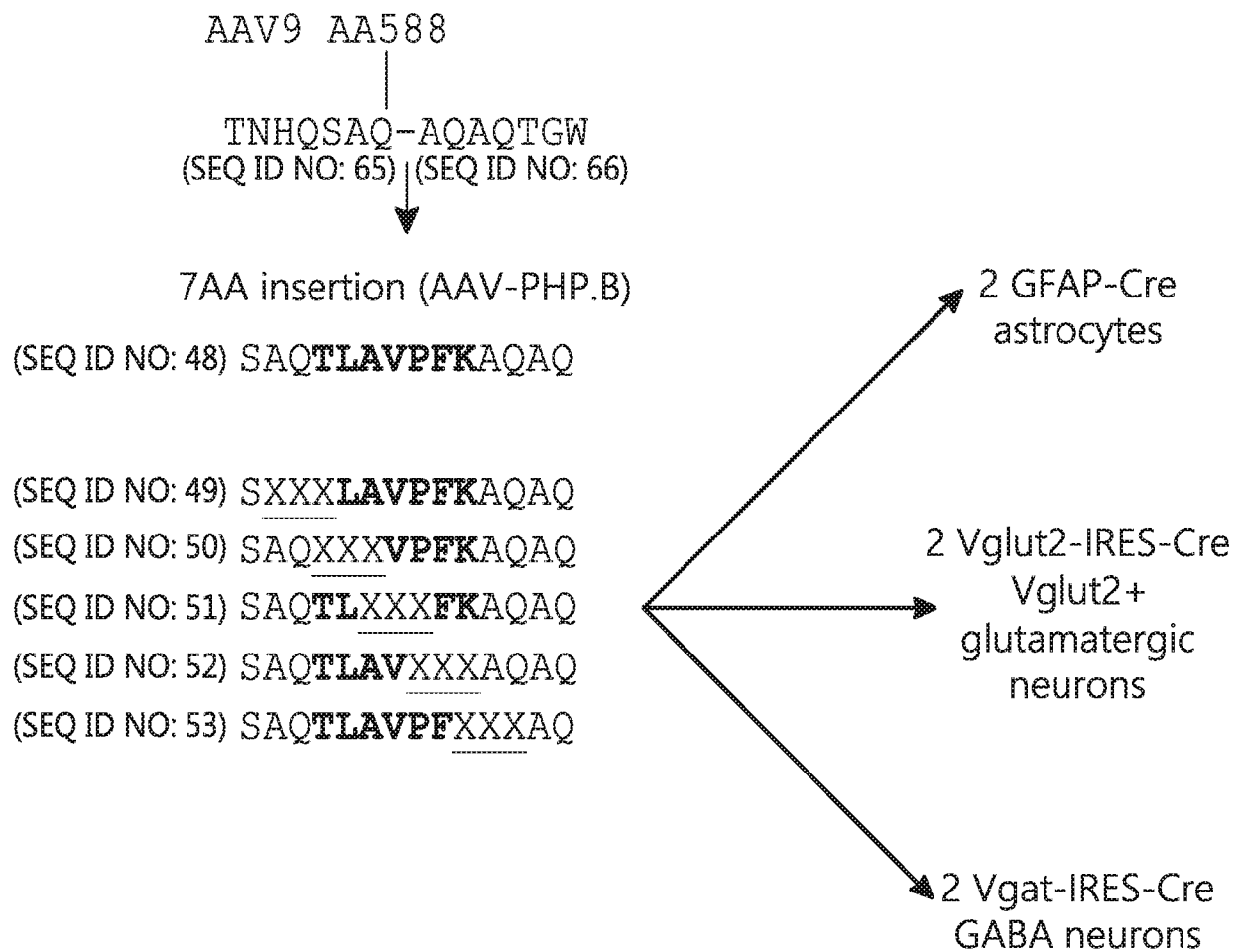


FIG. 1

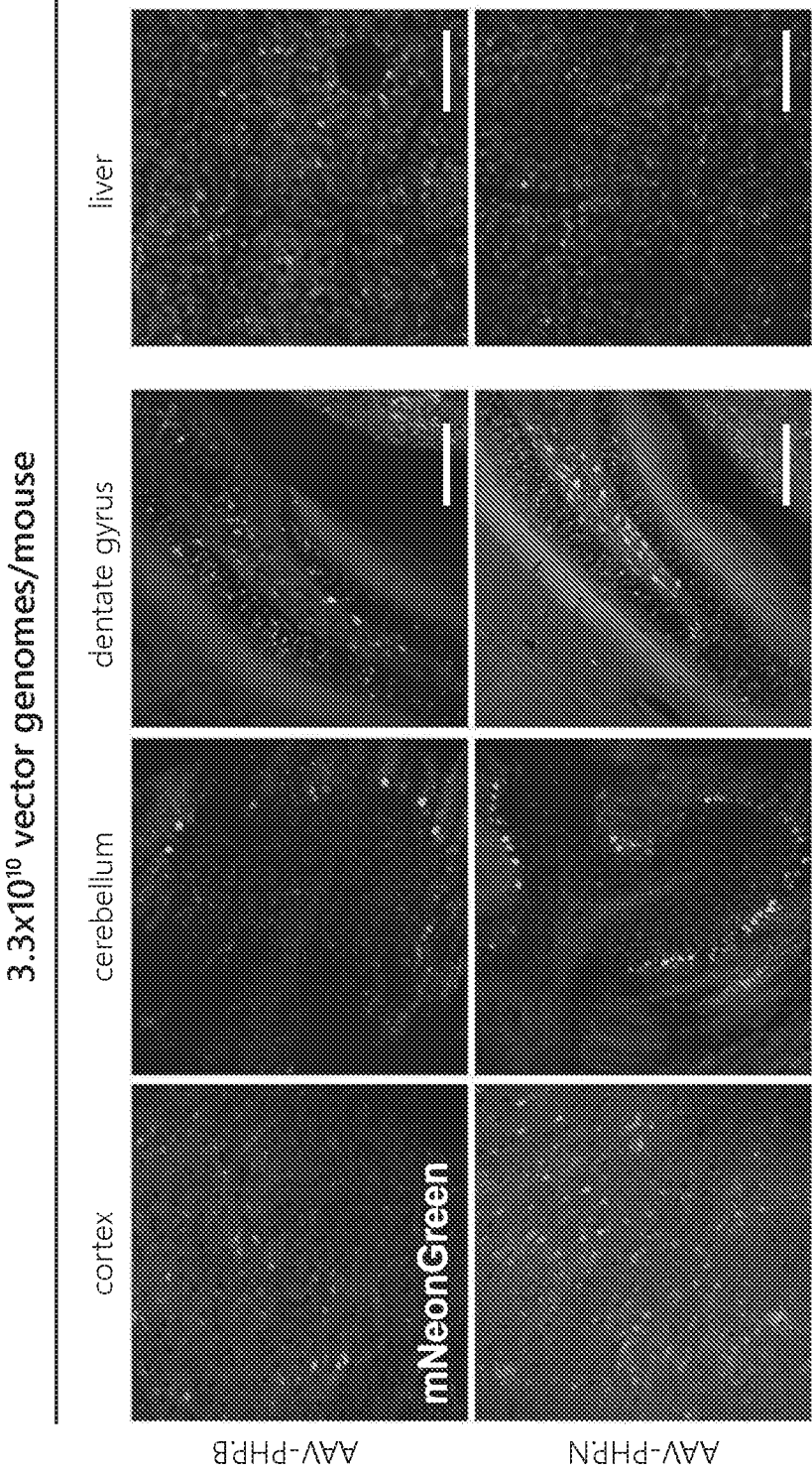


FIG. 2

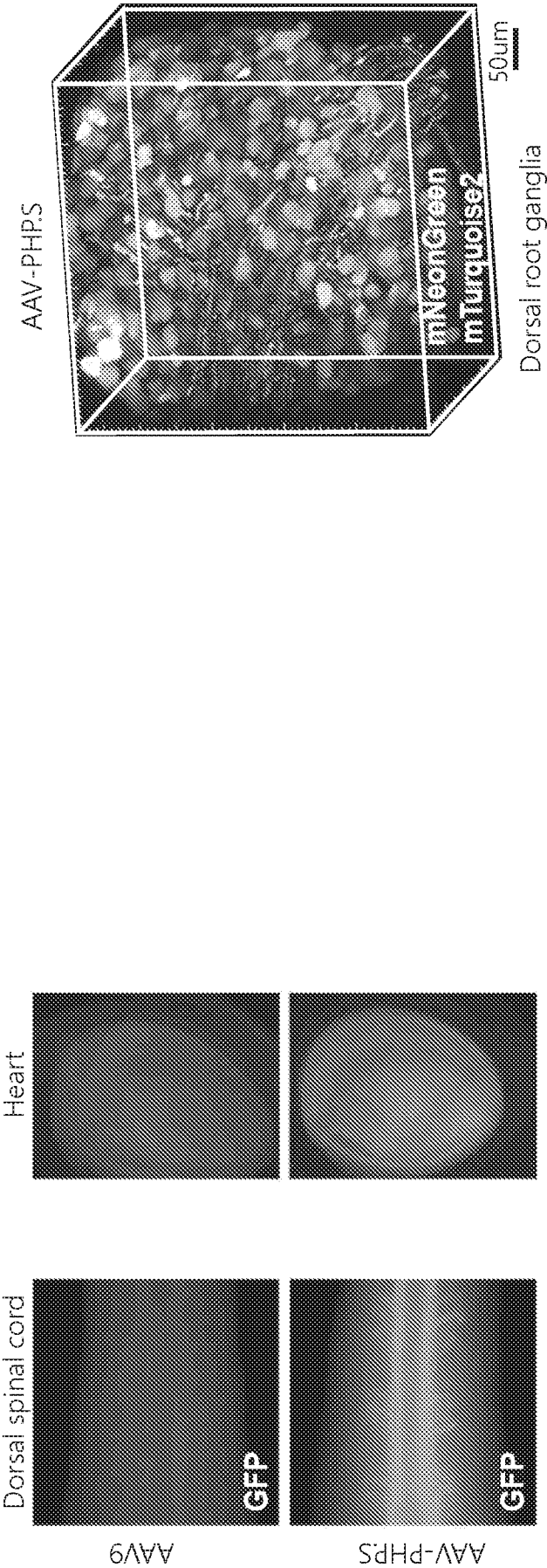


FIG. 3B

FIG. 3A

FIG. 3C

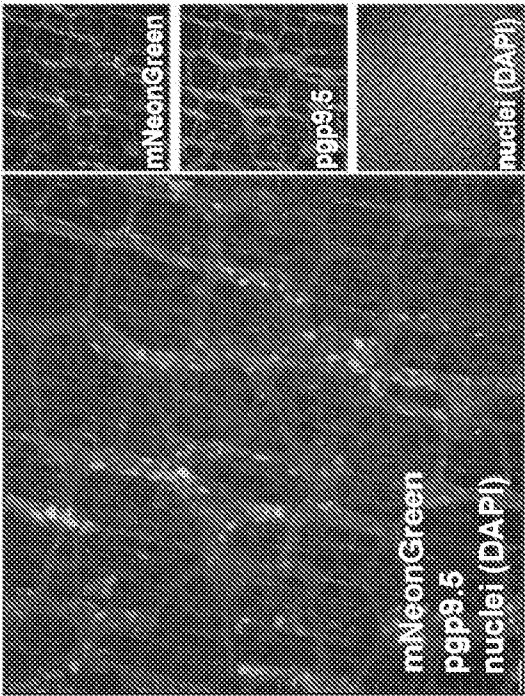


FIG. 3D

FIG. 4A

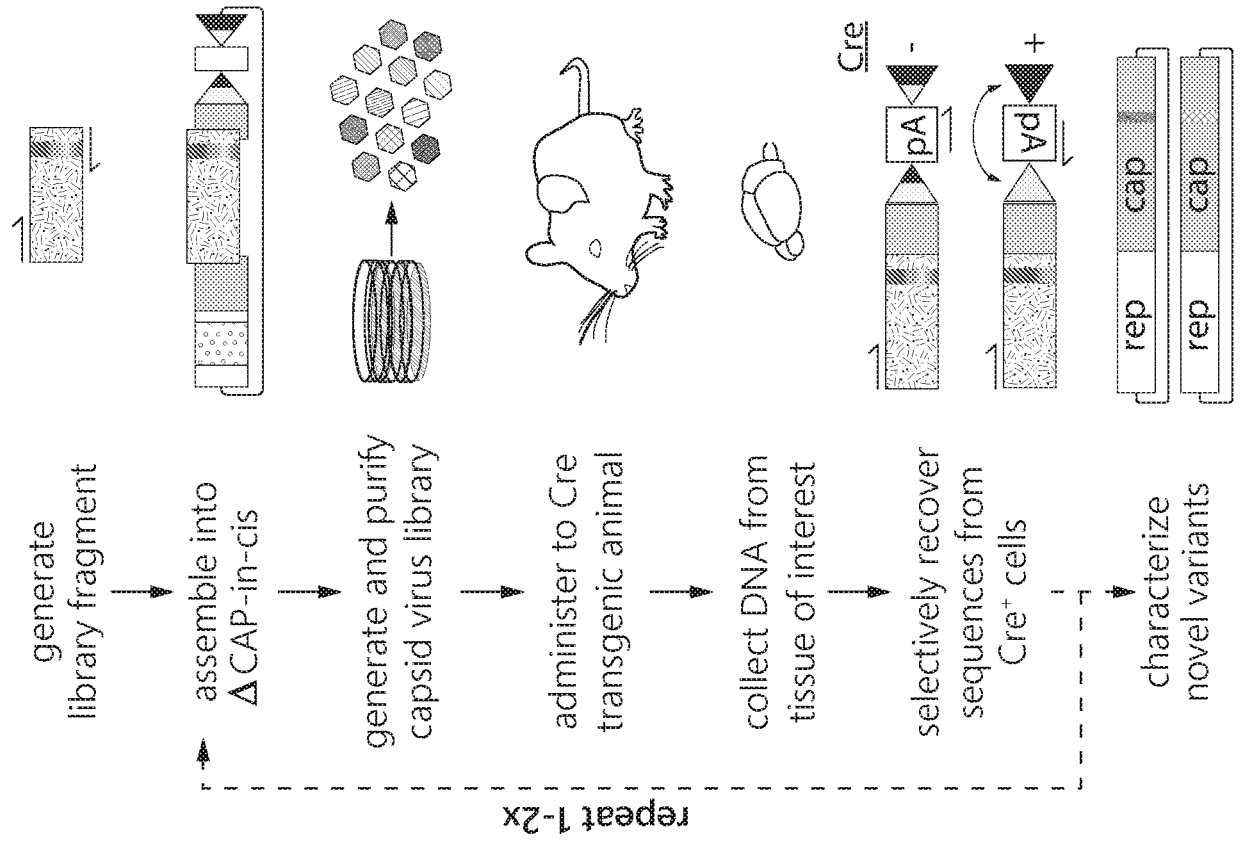
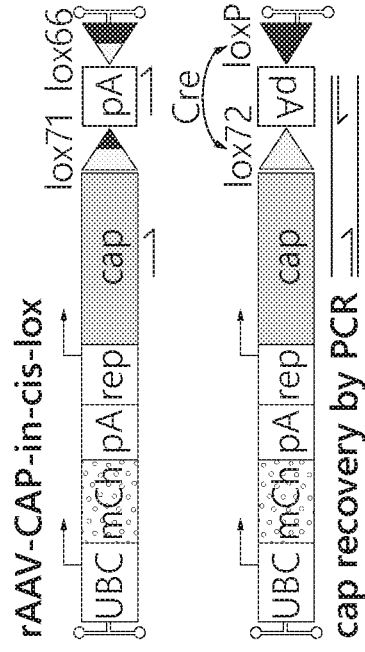


FIG. 4B

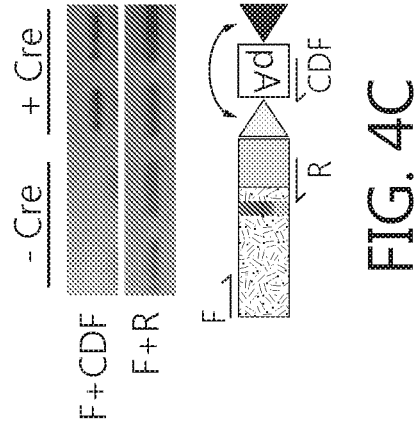


FIG. 4C

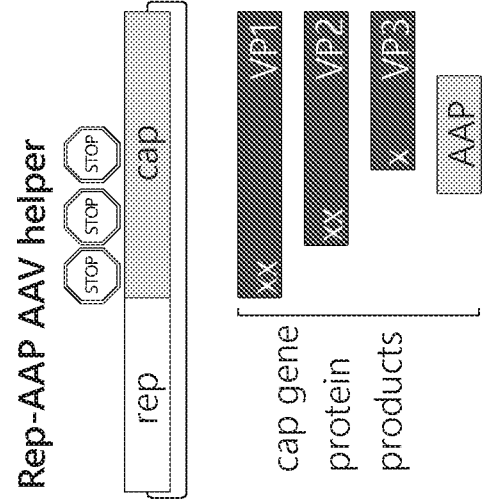


FIG. 4D

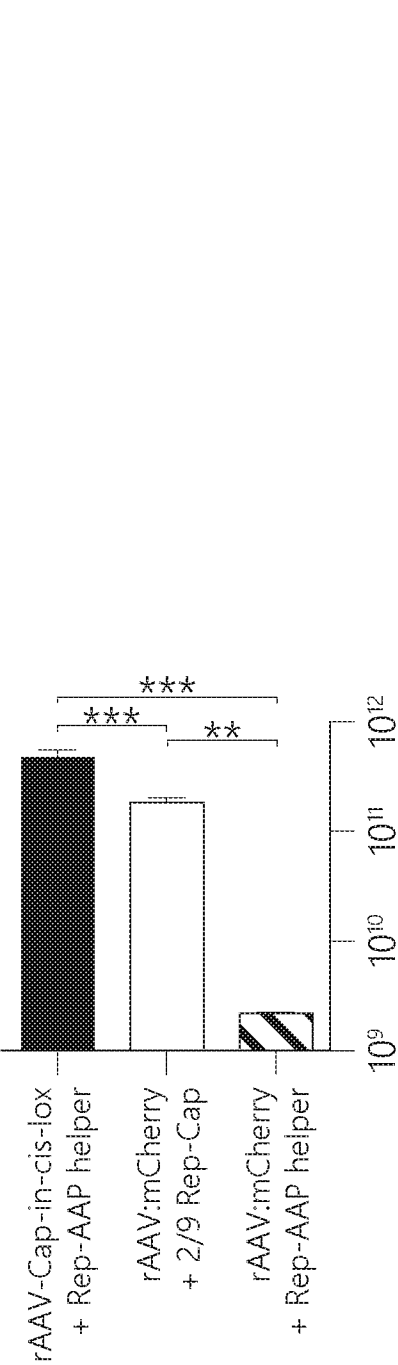


FIG. 4E

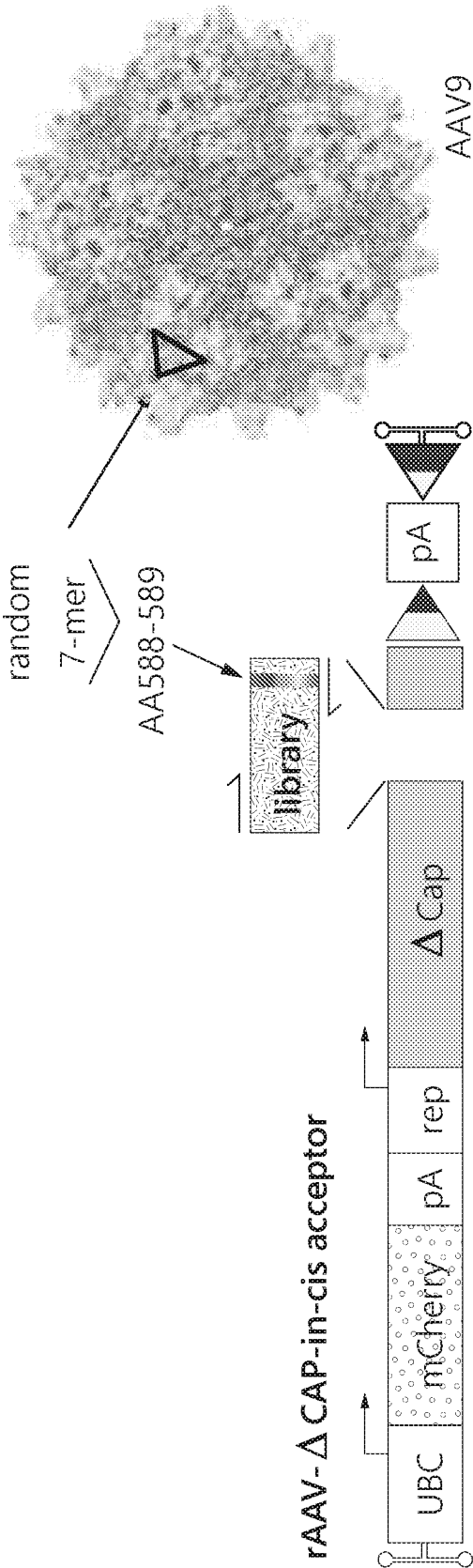


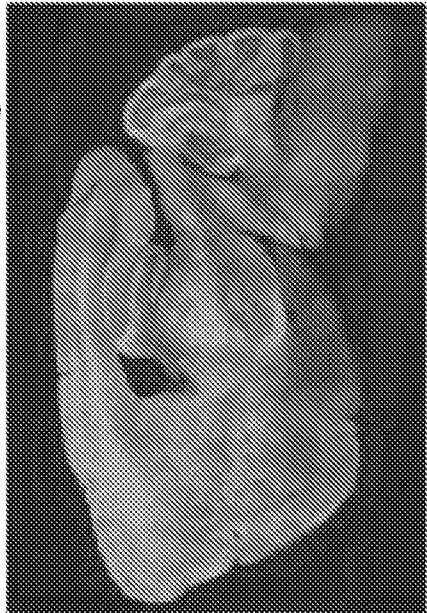
FIG. 4G

FIG. 4F

AAV9 (1x10¹² vg)



AAV-PHP.B (1x10¹² vg)

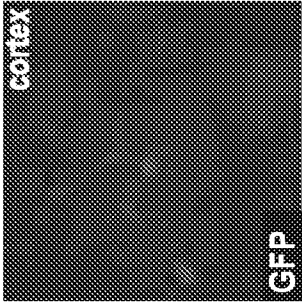


AAV-PHP.B (1x10¹¹ vg)

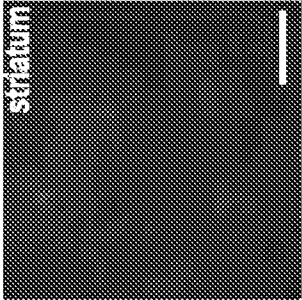


FIG. 5A

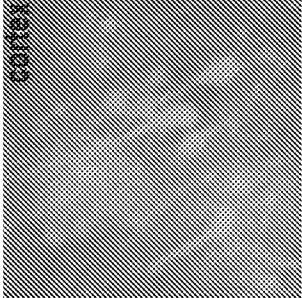
cortex



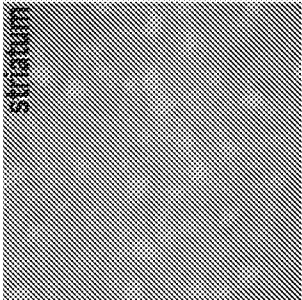
striatum



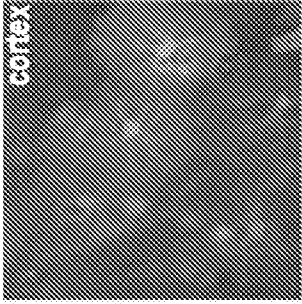
cortex



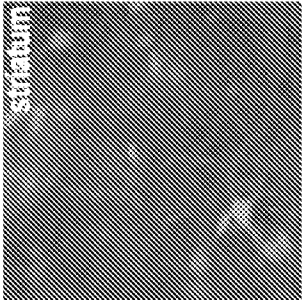
striatum



cortex



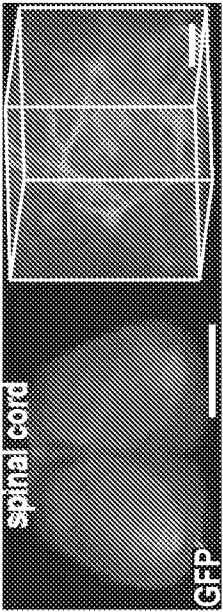
striatum



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FIG. 5B

spinal cord



retina

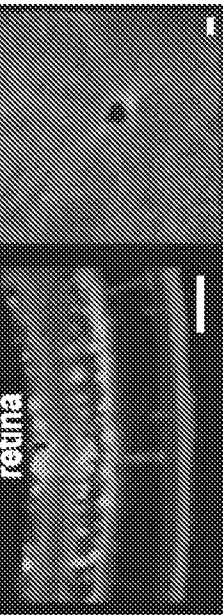


FIG. 5C

FIG. 5D

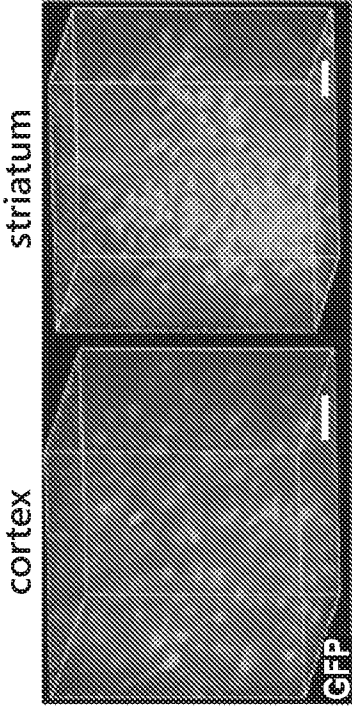


FIG. 5E

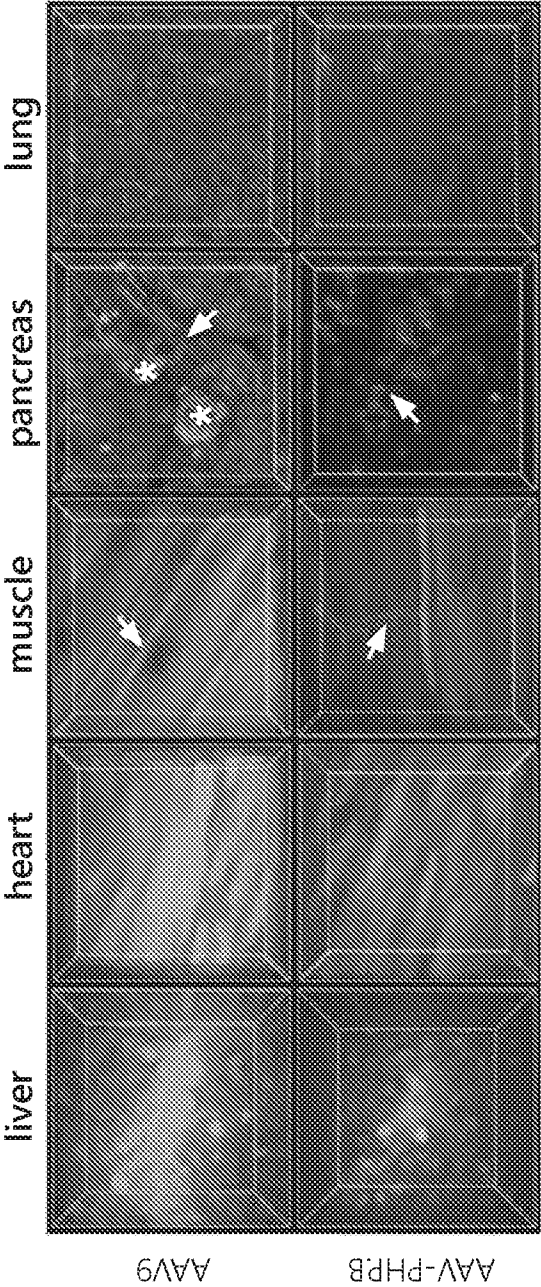


FIG. 5F

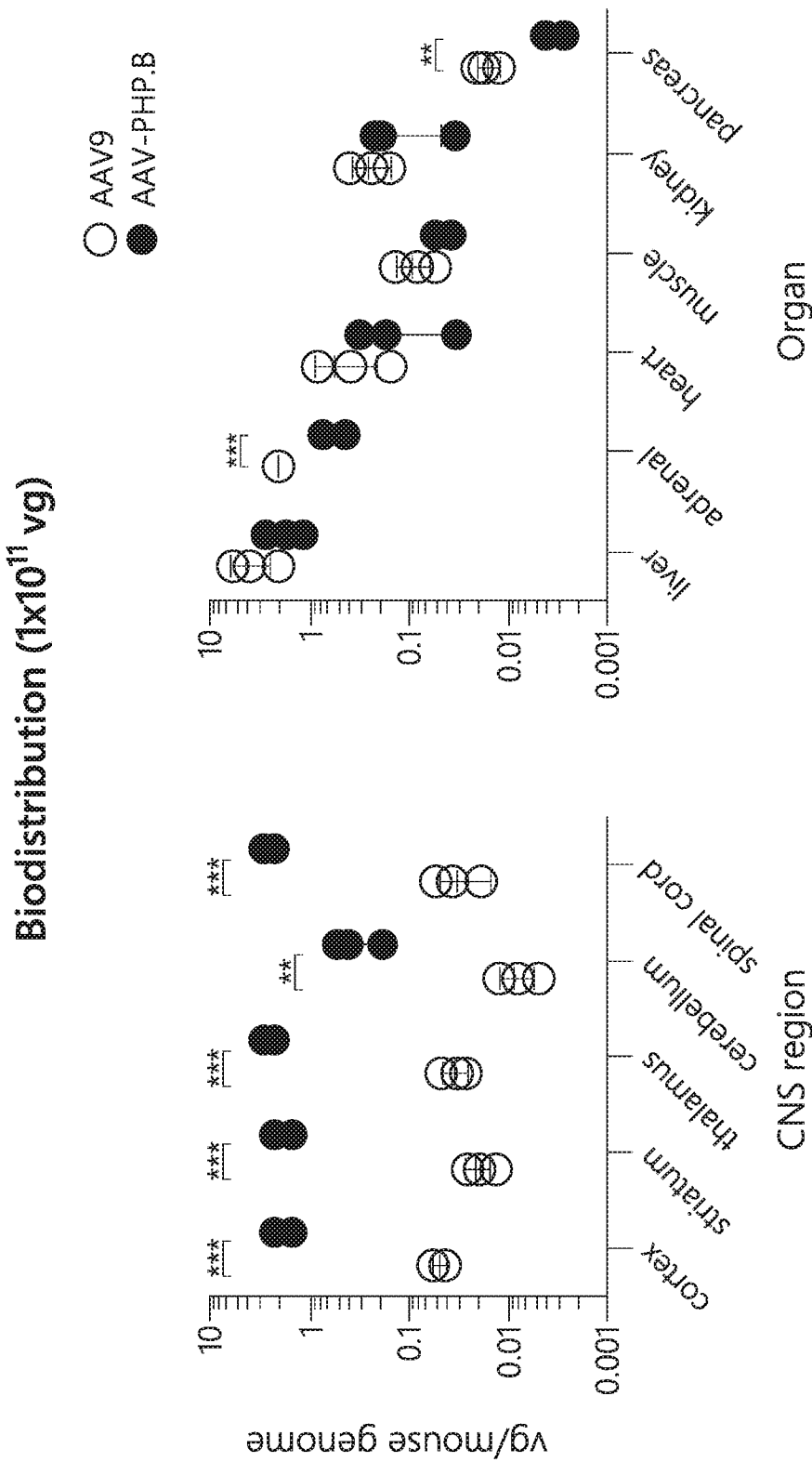


FIG. 5G

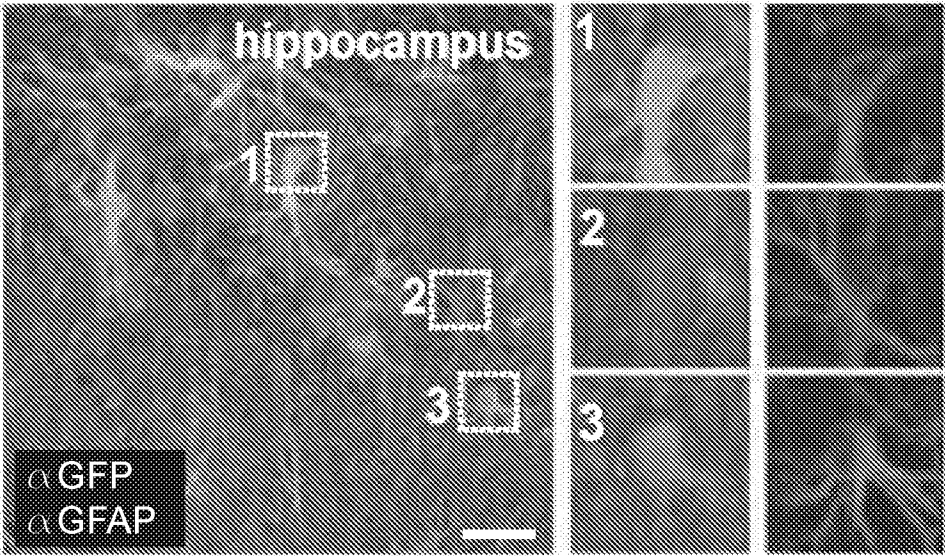


FIG. 6A

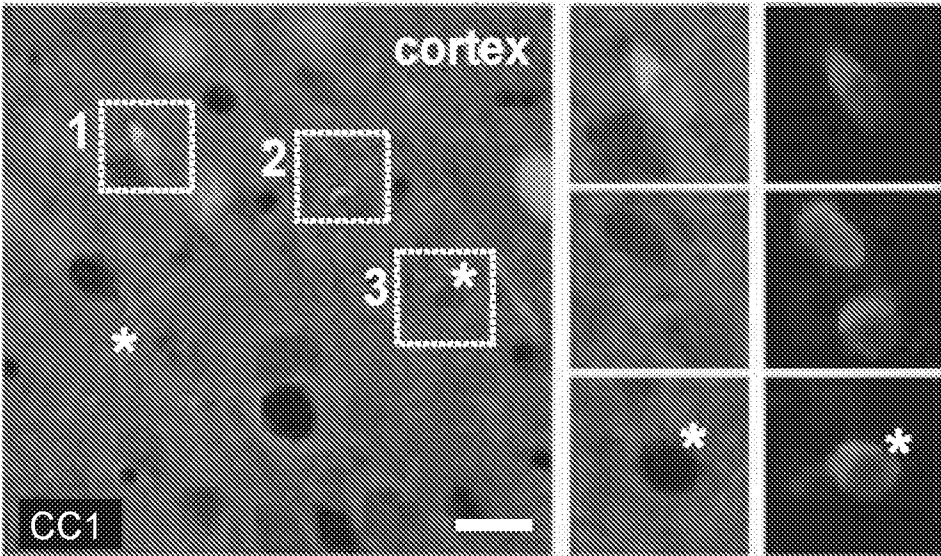


FIG. 6B

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AAV-PHP.B:CAG-GFP

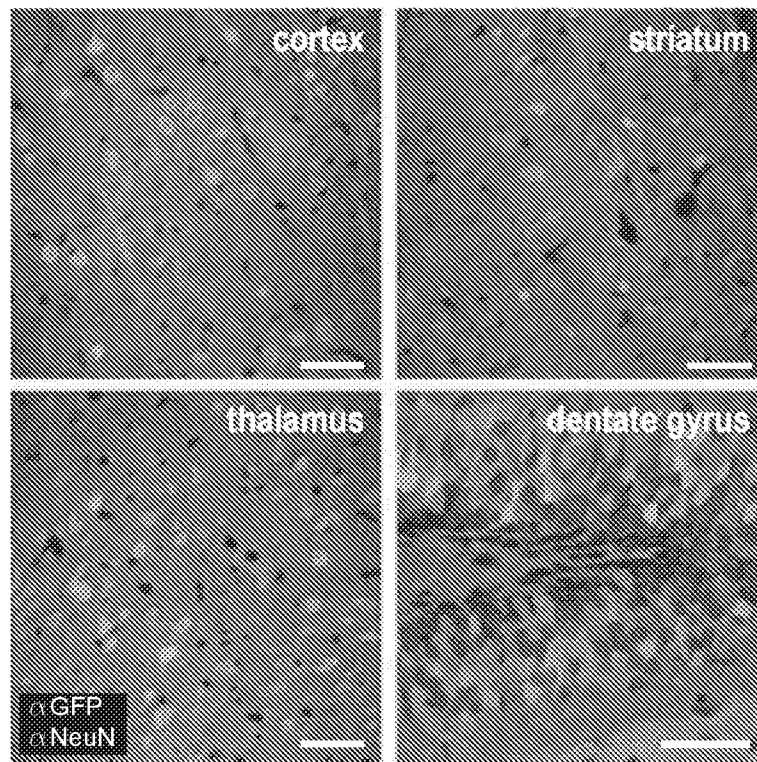


FIG. 6C

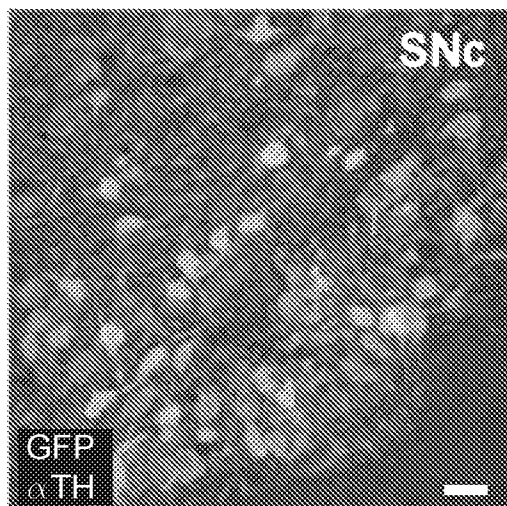


FIG. 6D

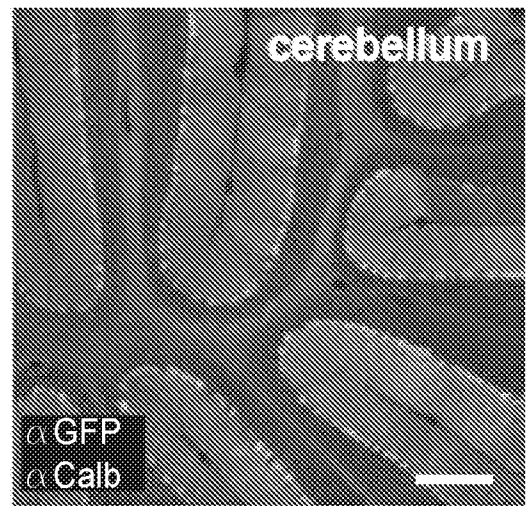


FIG. 6E

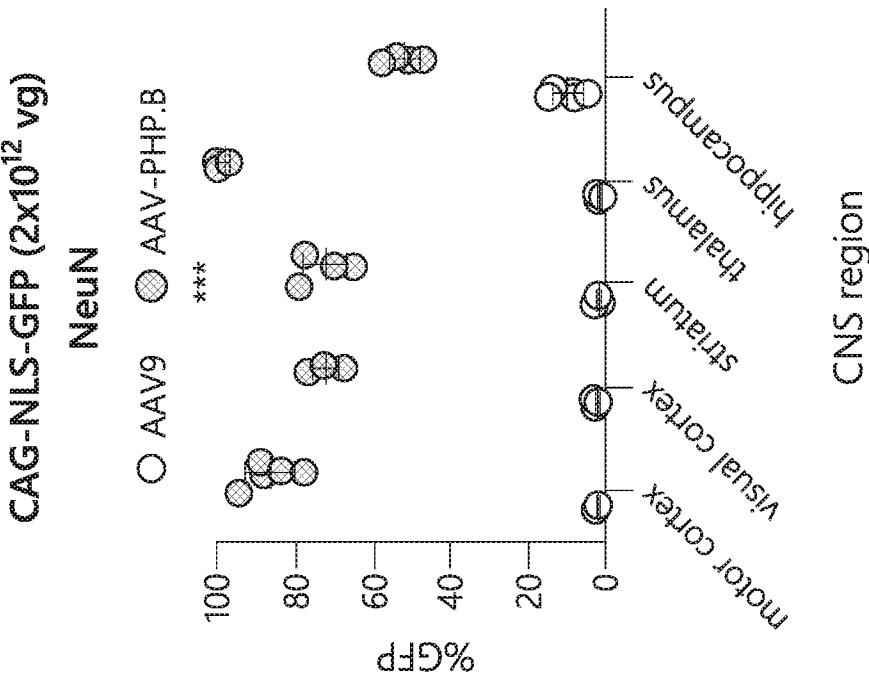


FIG. 6G

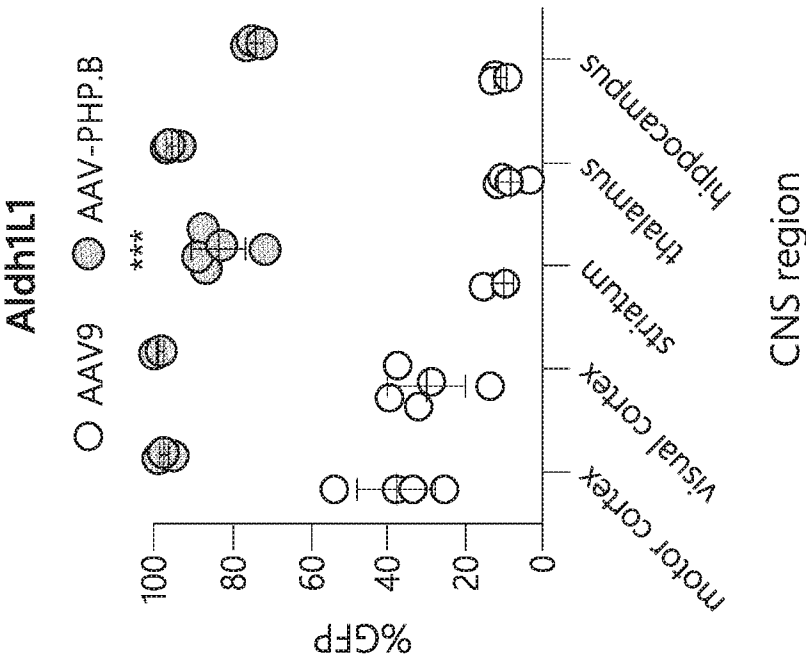


FIG. 6F

FIG. 6H

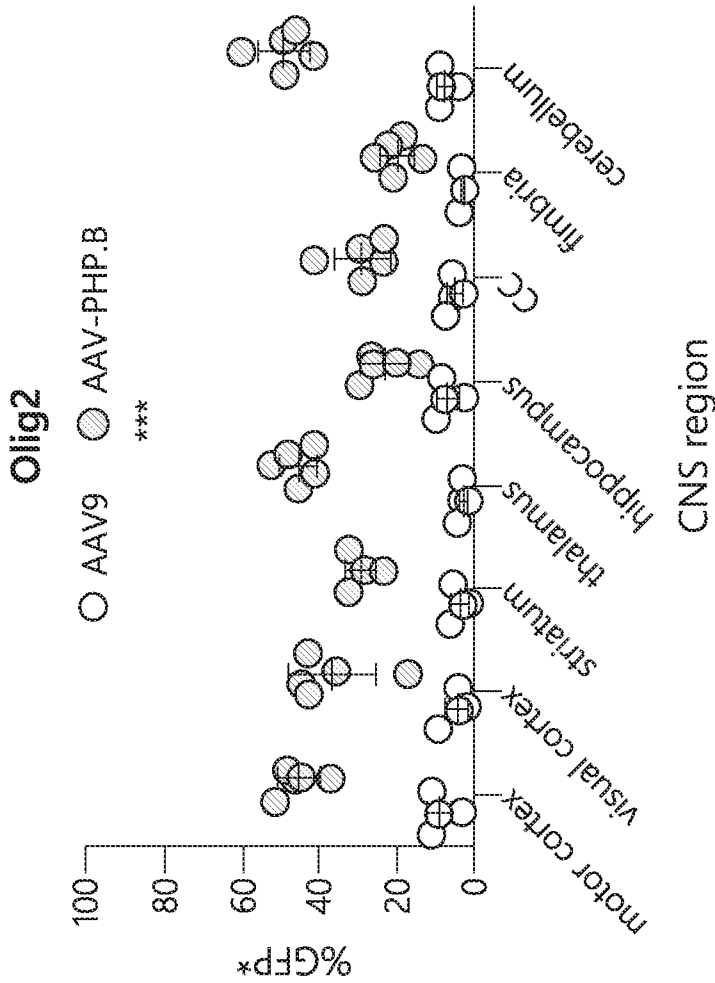
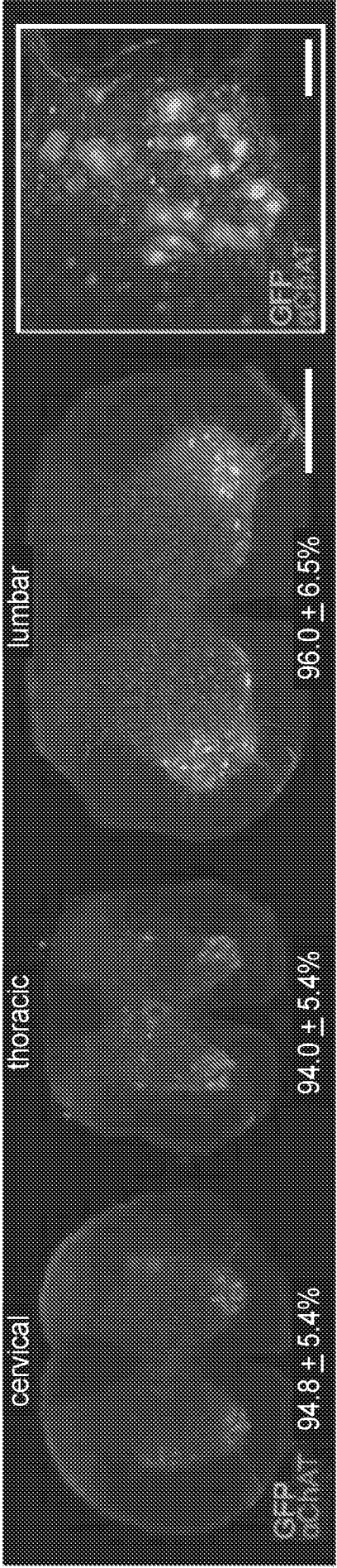


FIG. 6I

AAV-PHP.B:CAG-NLS-GFP (2×10^{12} vg)



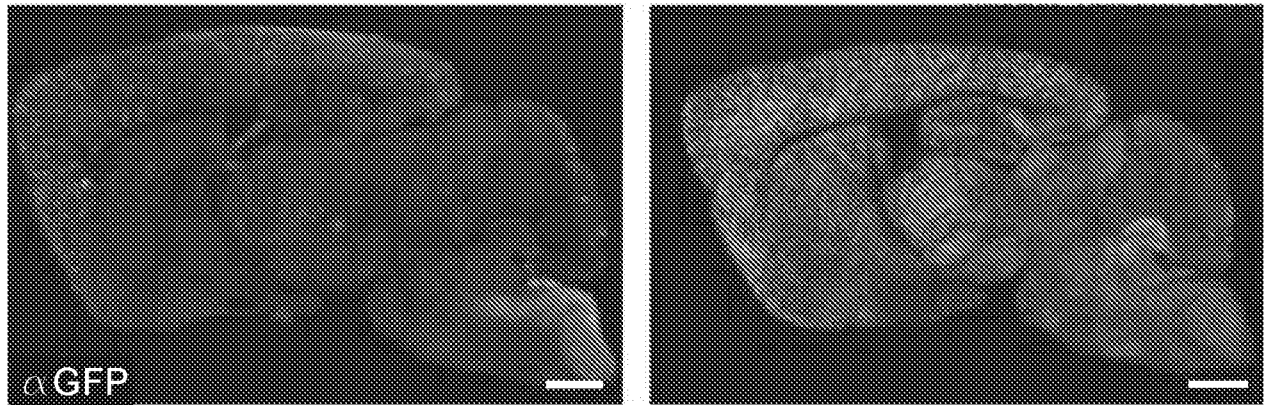
AAV9 (3×10^{11} vg)AAV-PHPA (3×10^{11} vg)

FIG. 7A

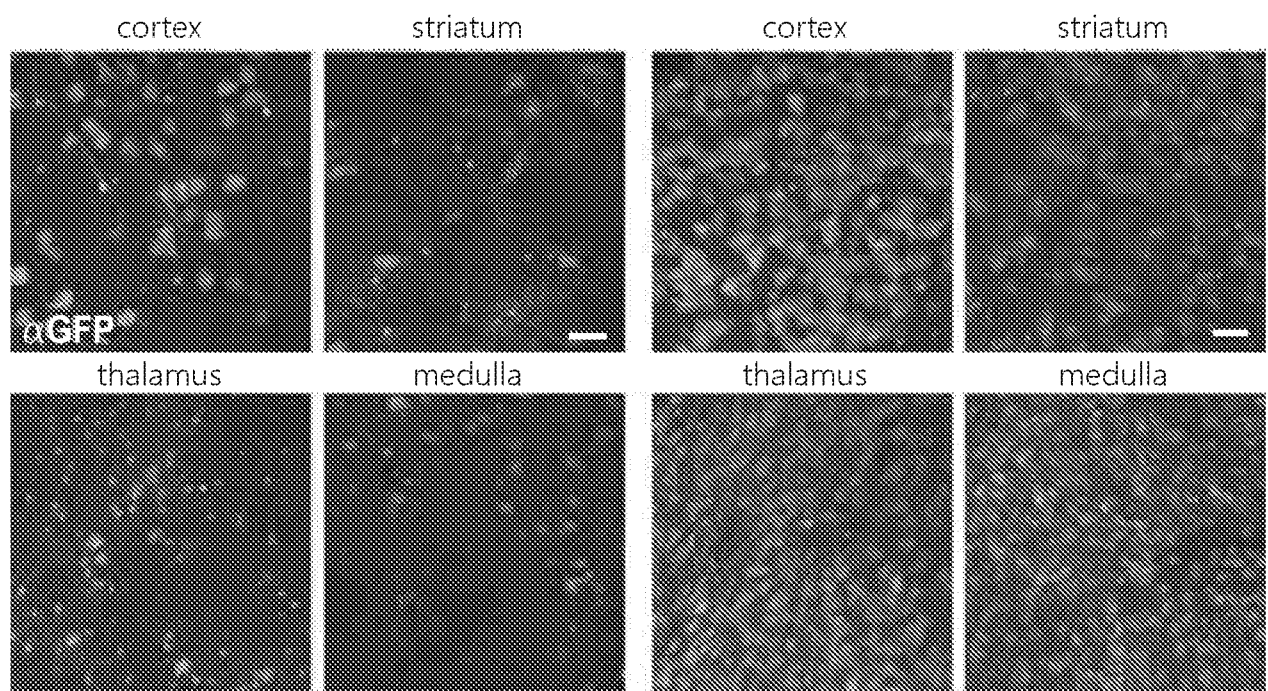


FIG. 7B

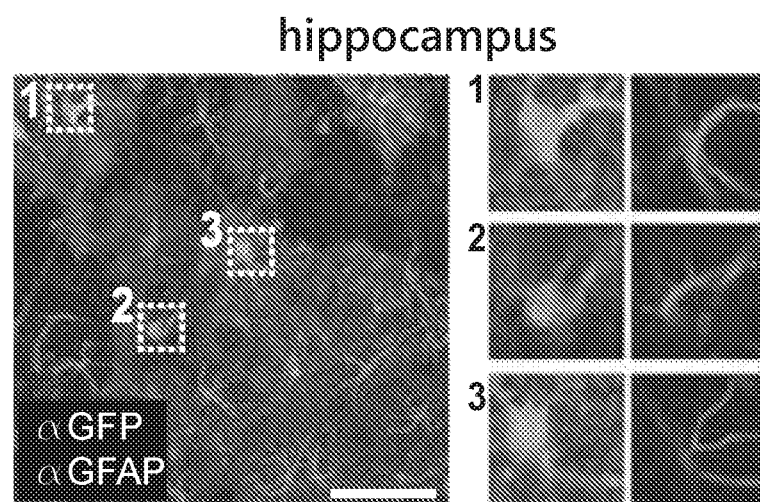


FIG. 7C

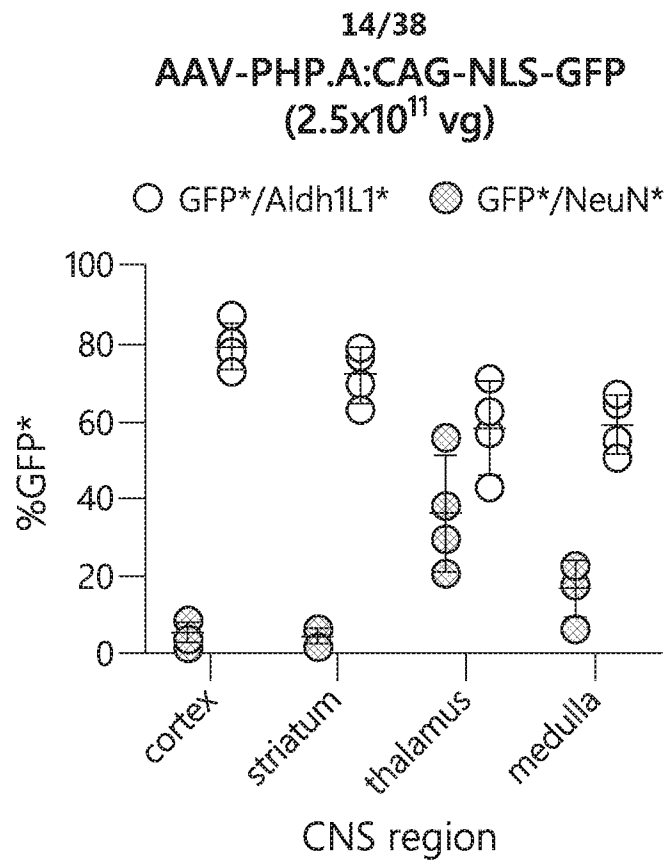


FIG. 7D

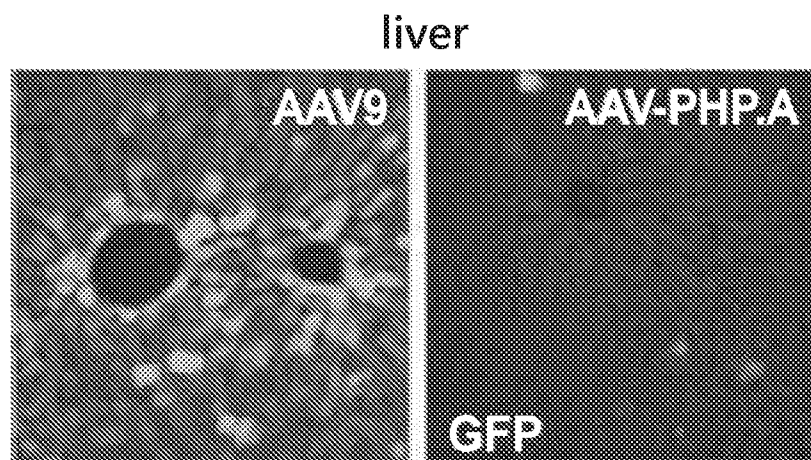


FIG. 7E

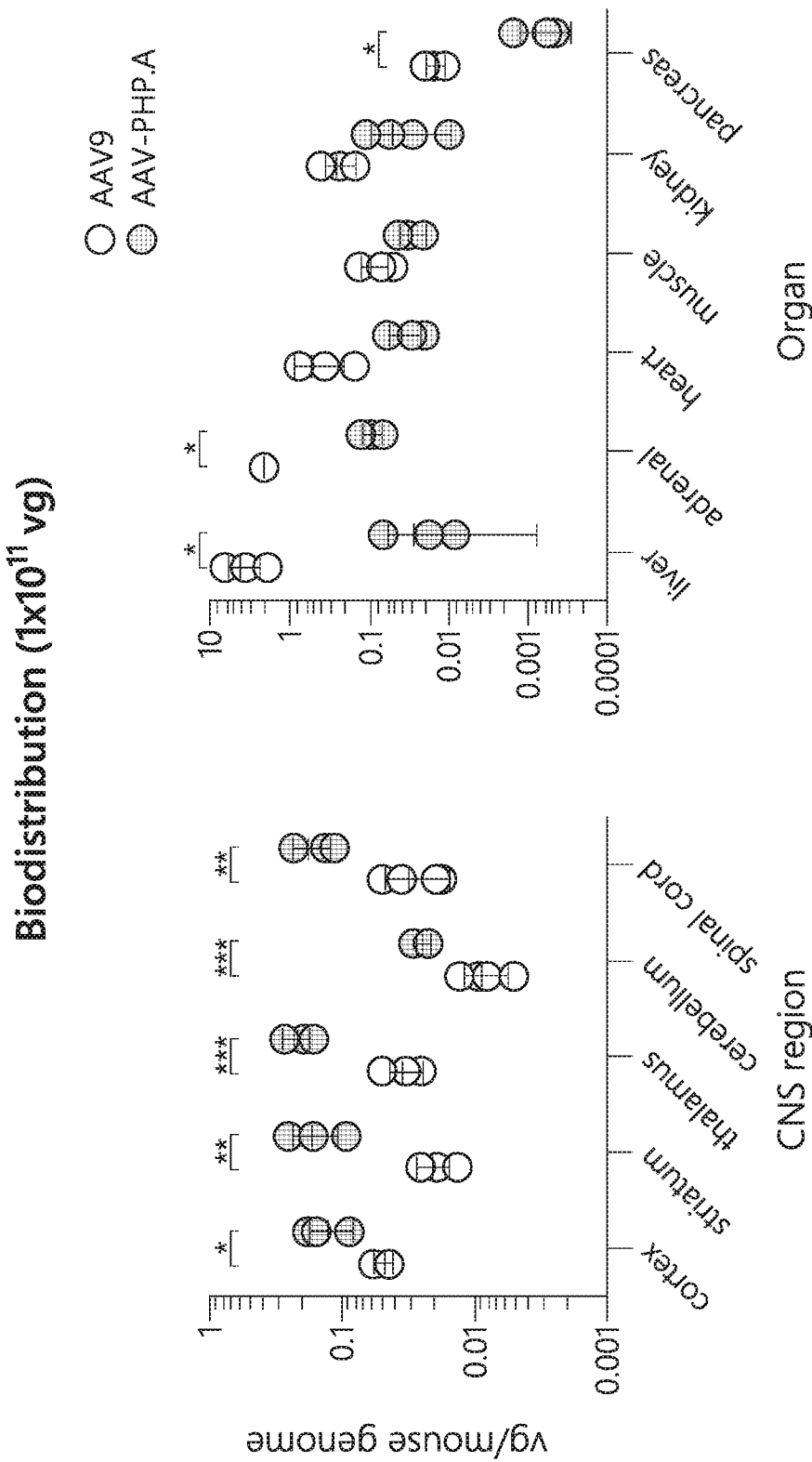


FIG. 7F

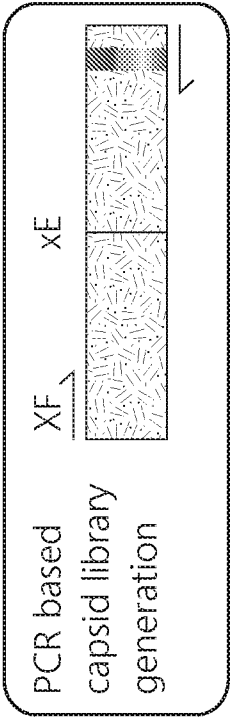


FIG. 8A

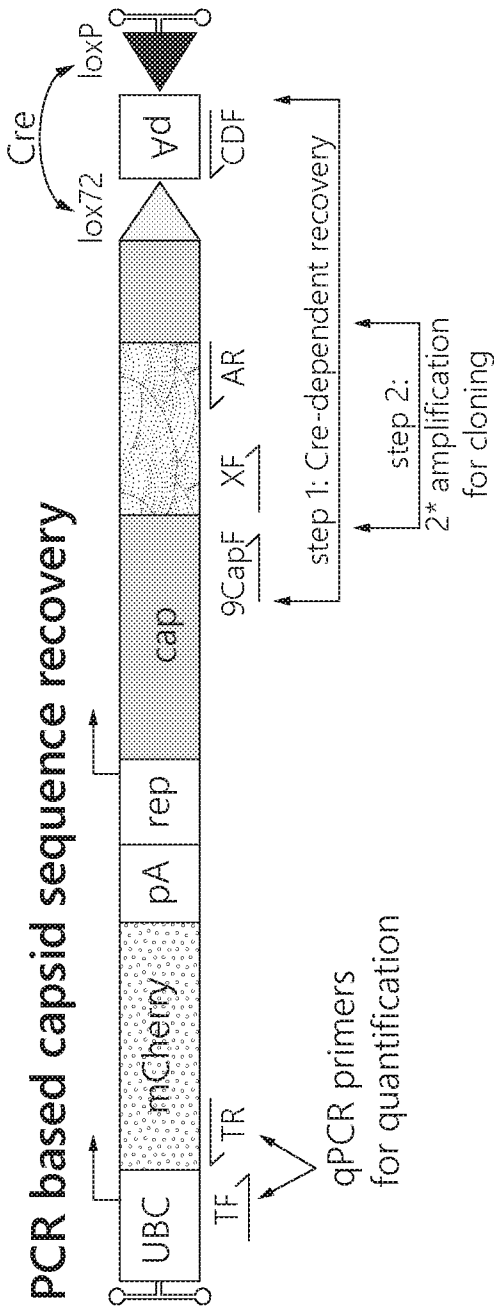


FIG. 8B

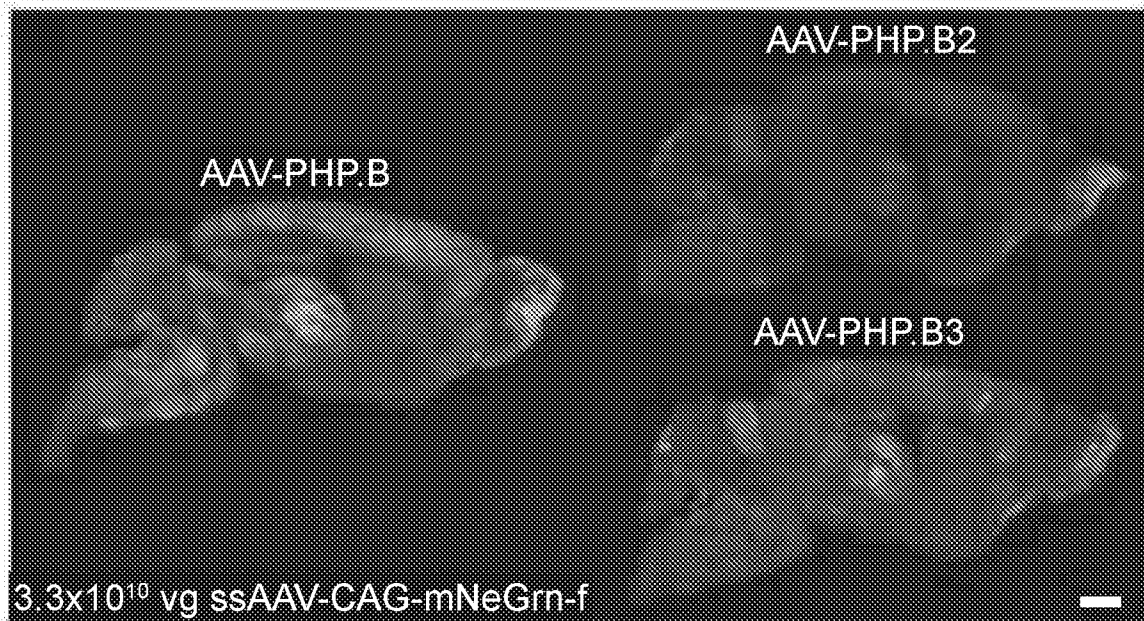


FIG. 9A

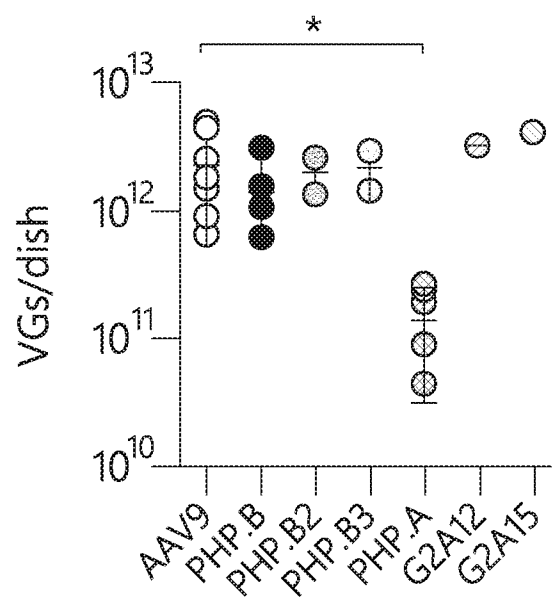


FIG. 9B

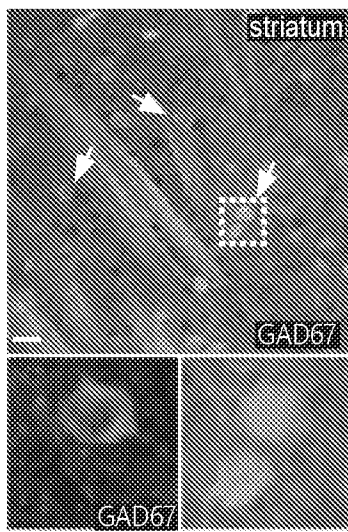


FIG. 10A

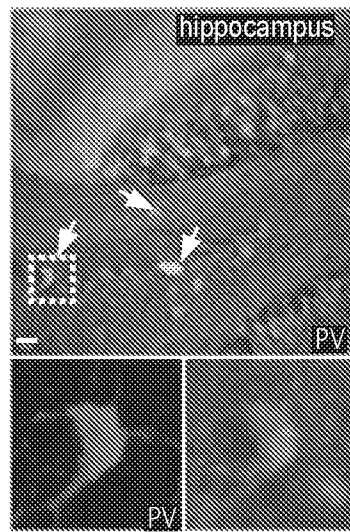


FIG. 10B

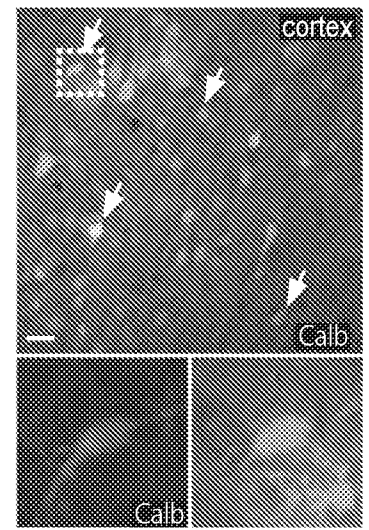


FIG. 10C

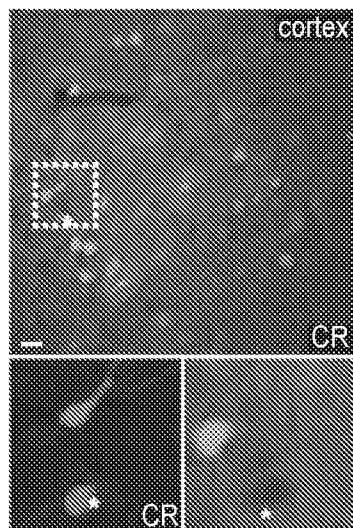


FIG. 10D

3.3×10^{10} vg ssAAV-CAG-mNeGrn-f

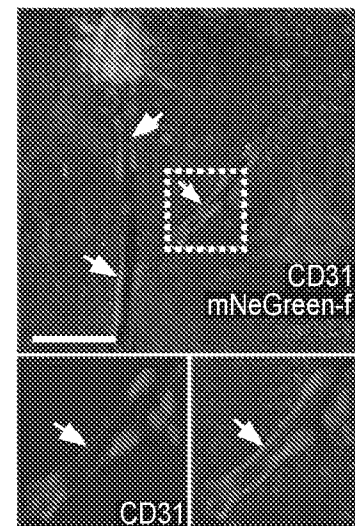


FIG. 10E

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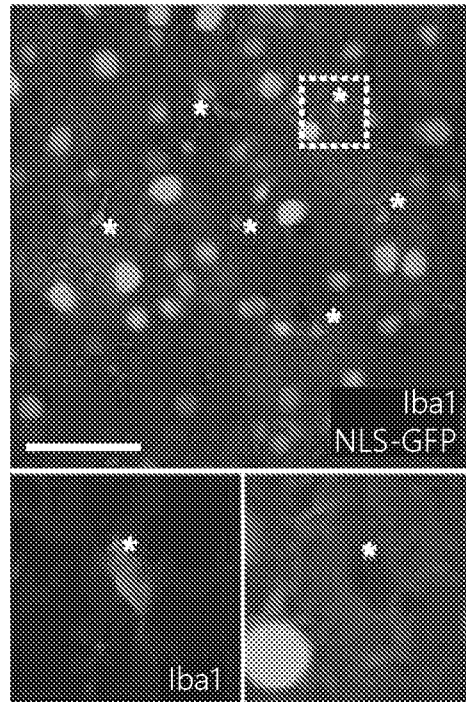


FIG. 10F

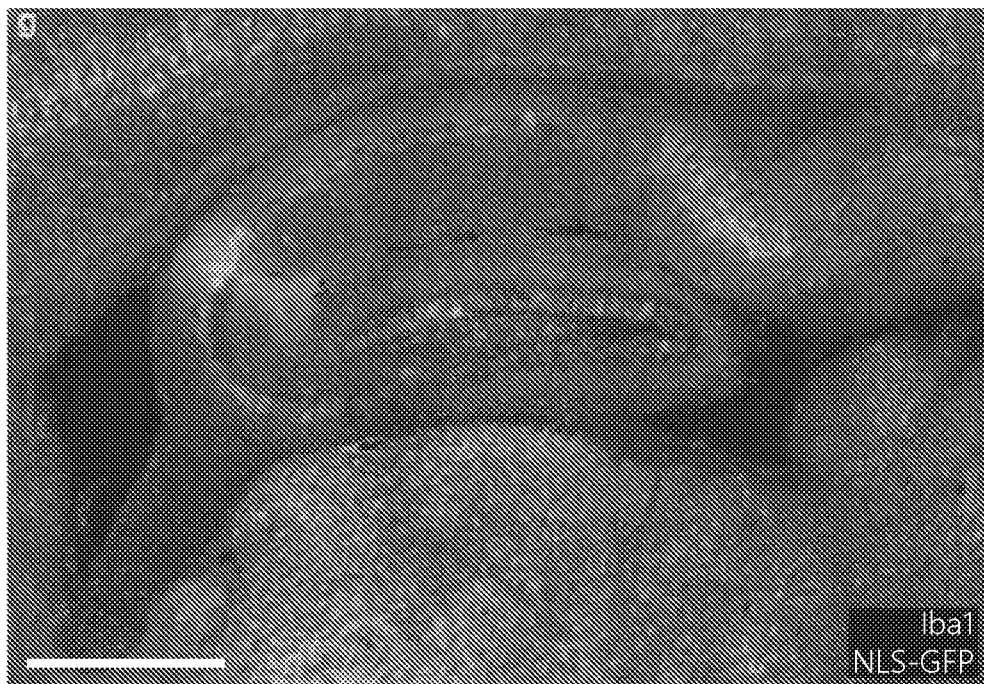


FIG. 10G

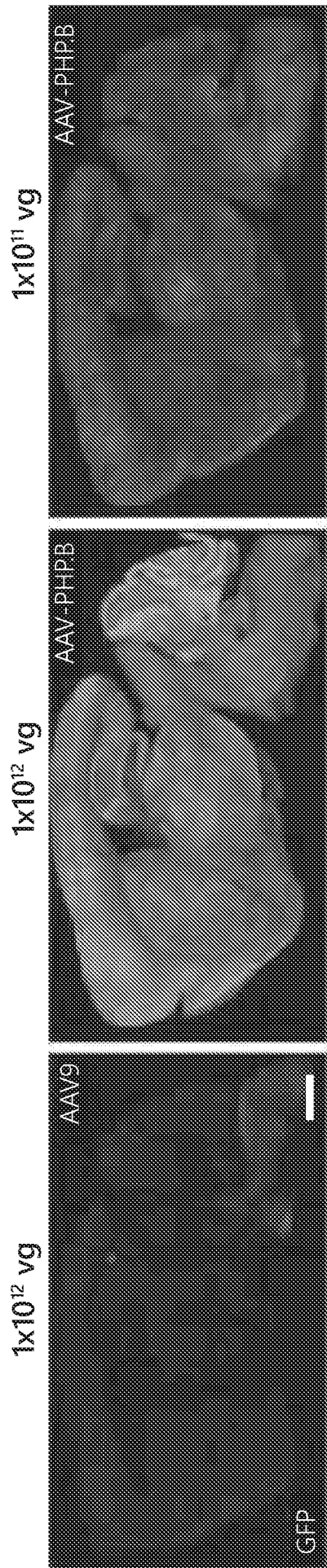


FIG. 11

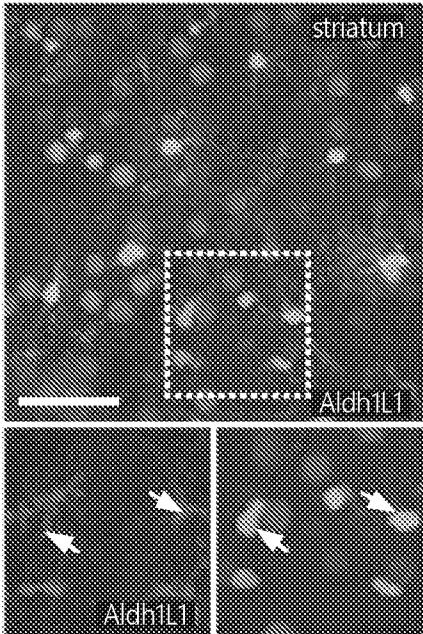


FIG. 12A

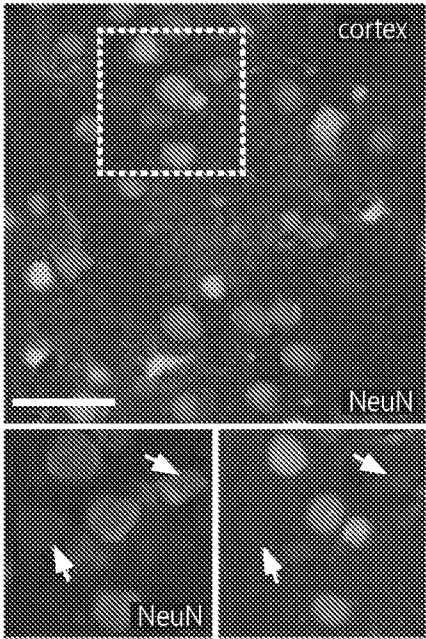


FIG. 12B

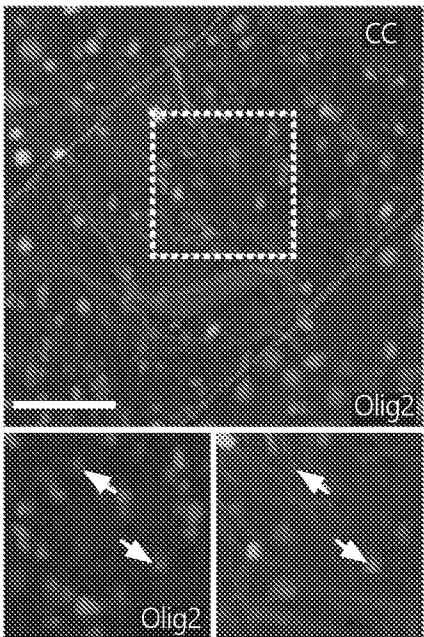


FIG. 12C

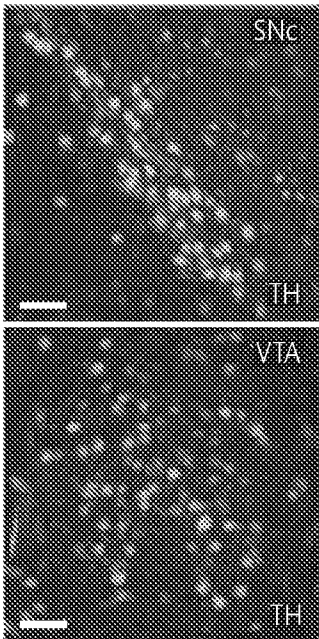


FIG. 12D

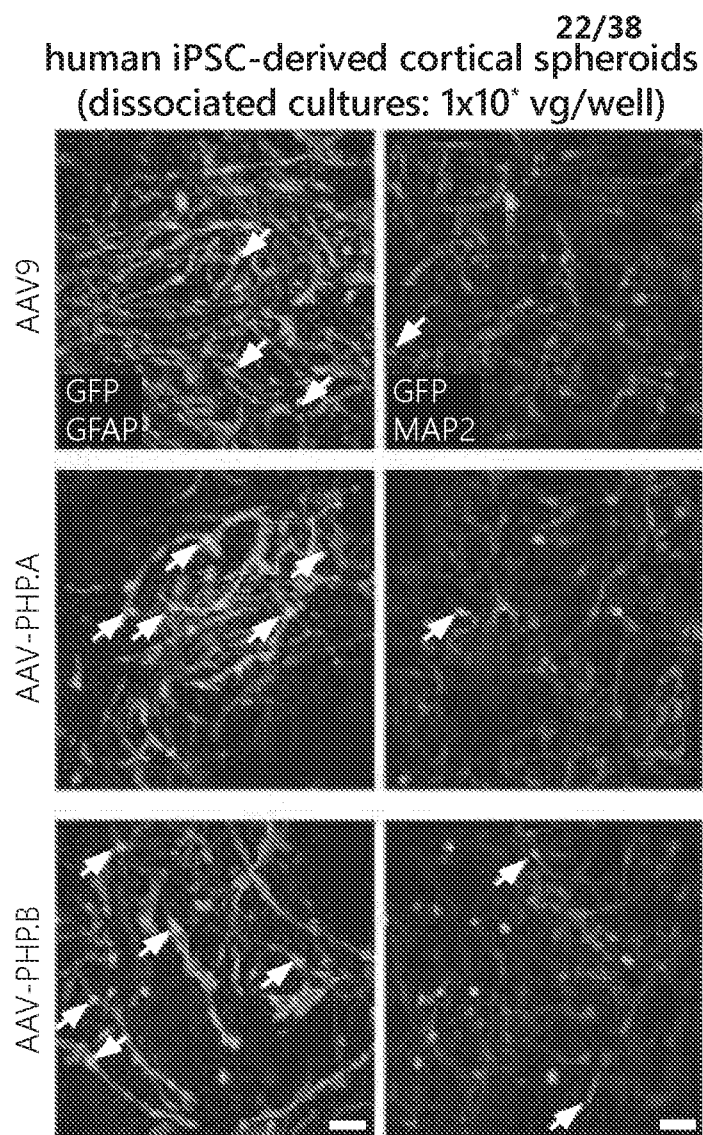


FIG. 13A

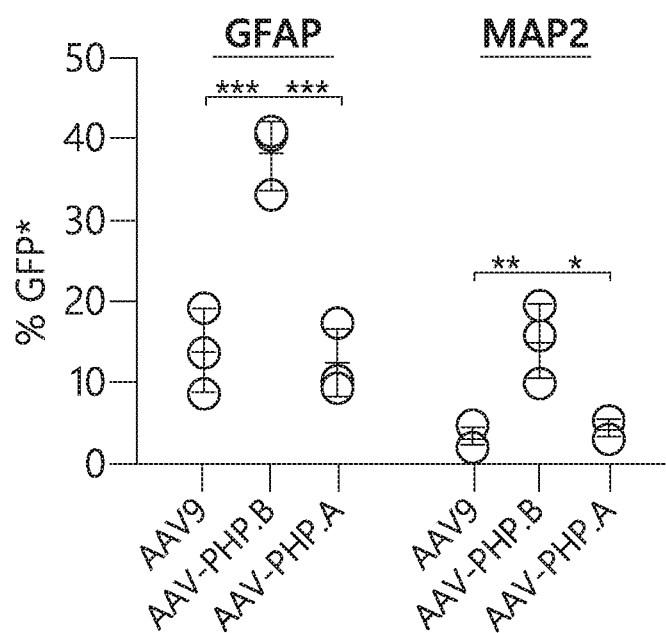


FIG. 13B

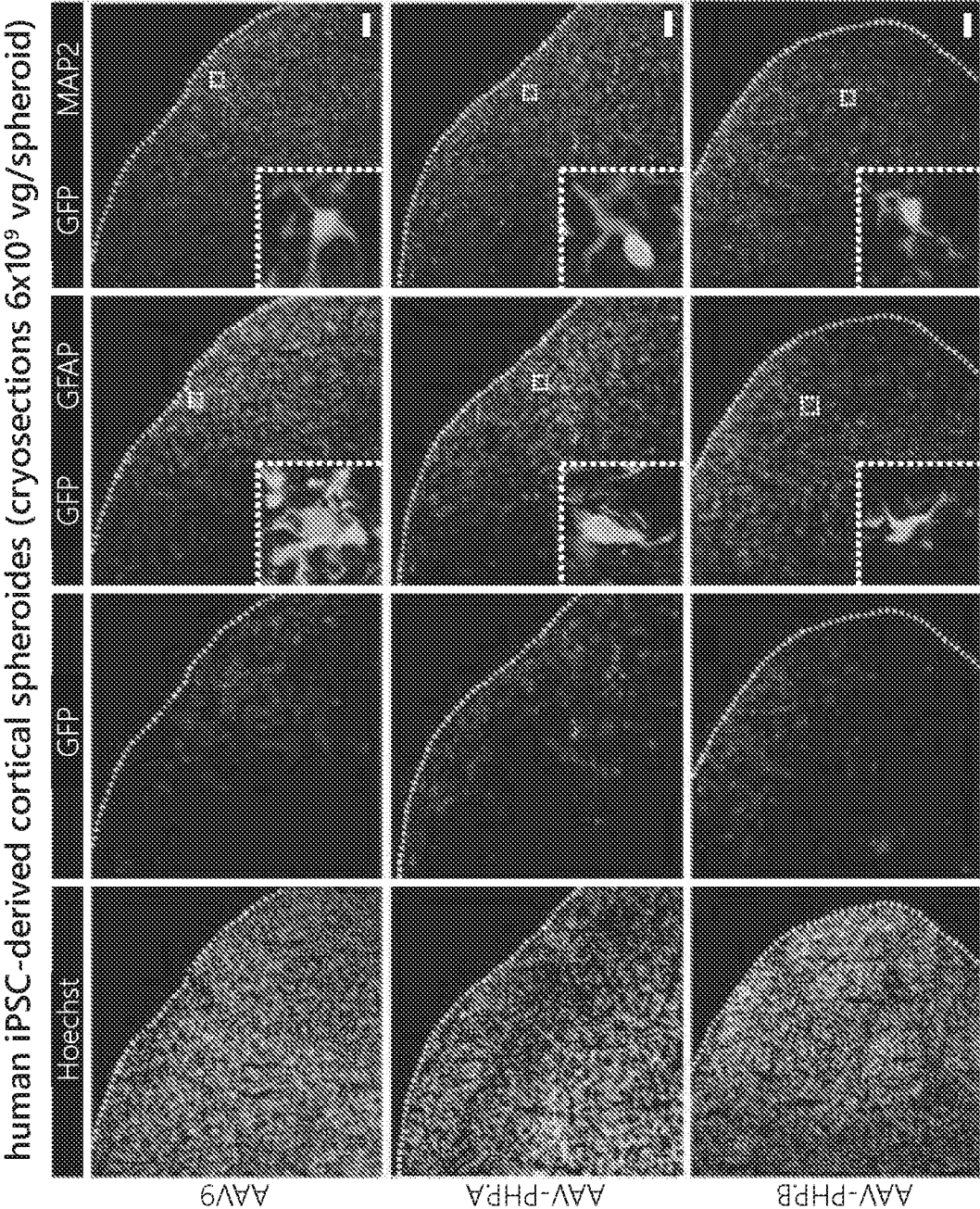


FIG. 13C

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1 hr capsid distribution (1×10^{11} vg)

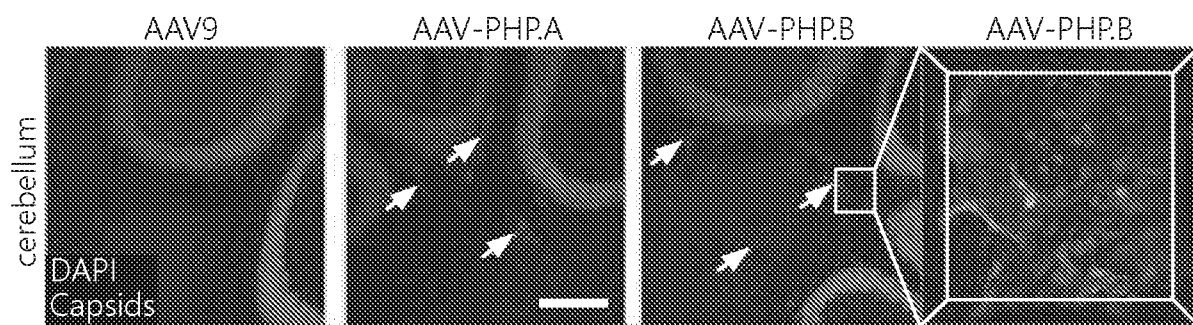


FIG. 14A

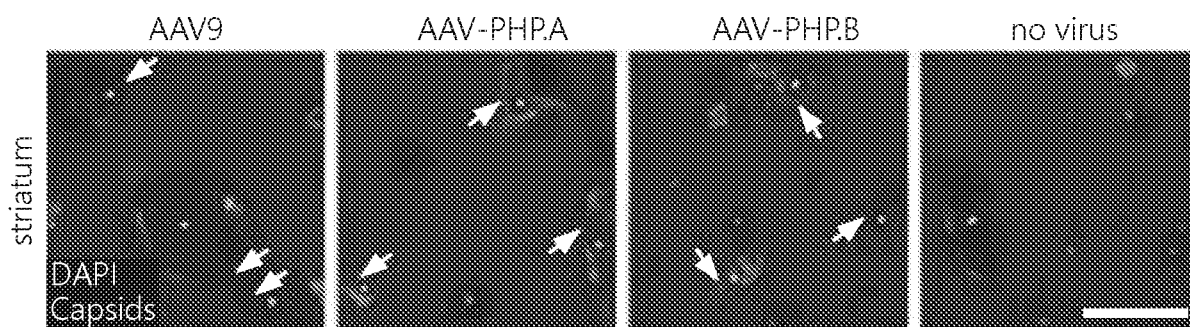


FIG. 14B

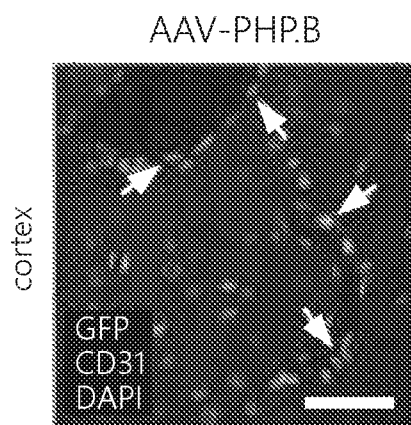
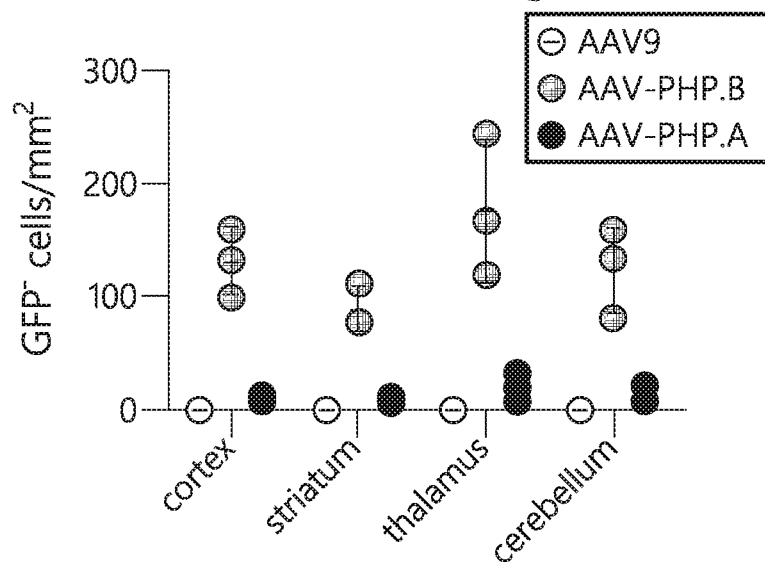


FIG. 14C

FIG. 14D

24 hr (1×10^{11} vg)



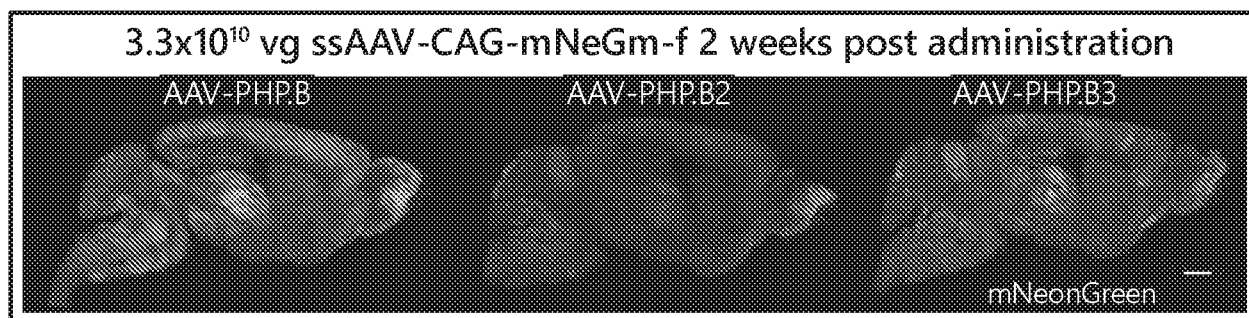


FIG. 15A

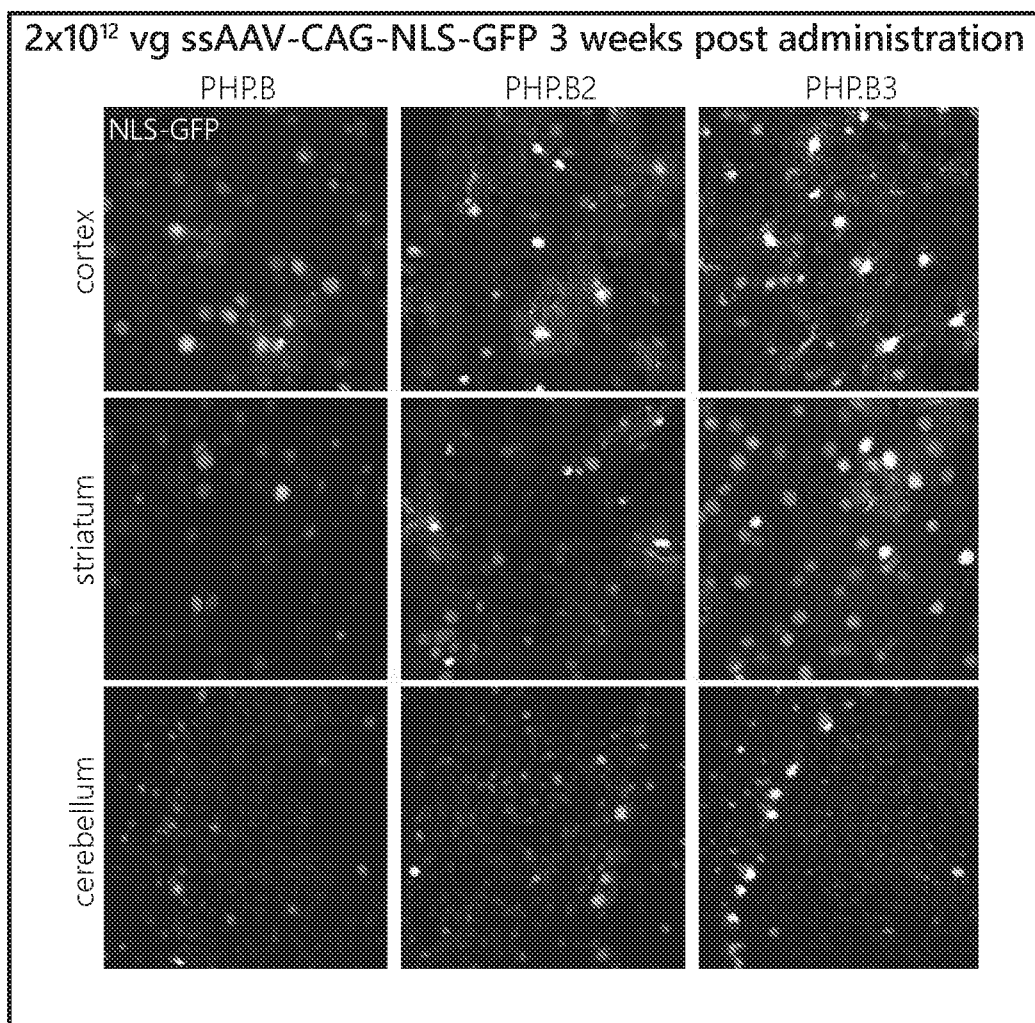


FIG. 15B

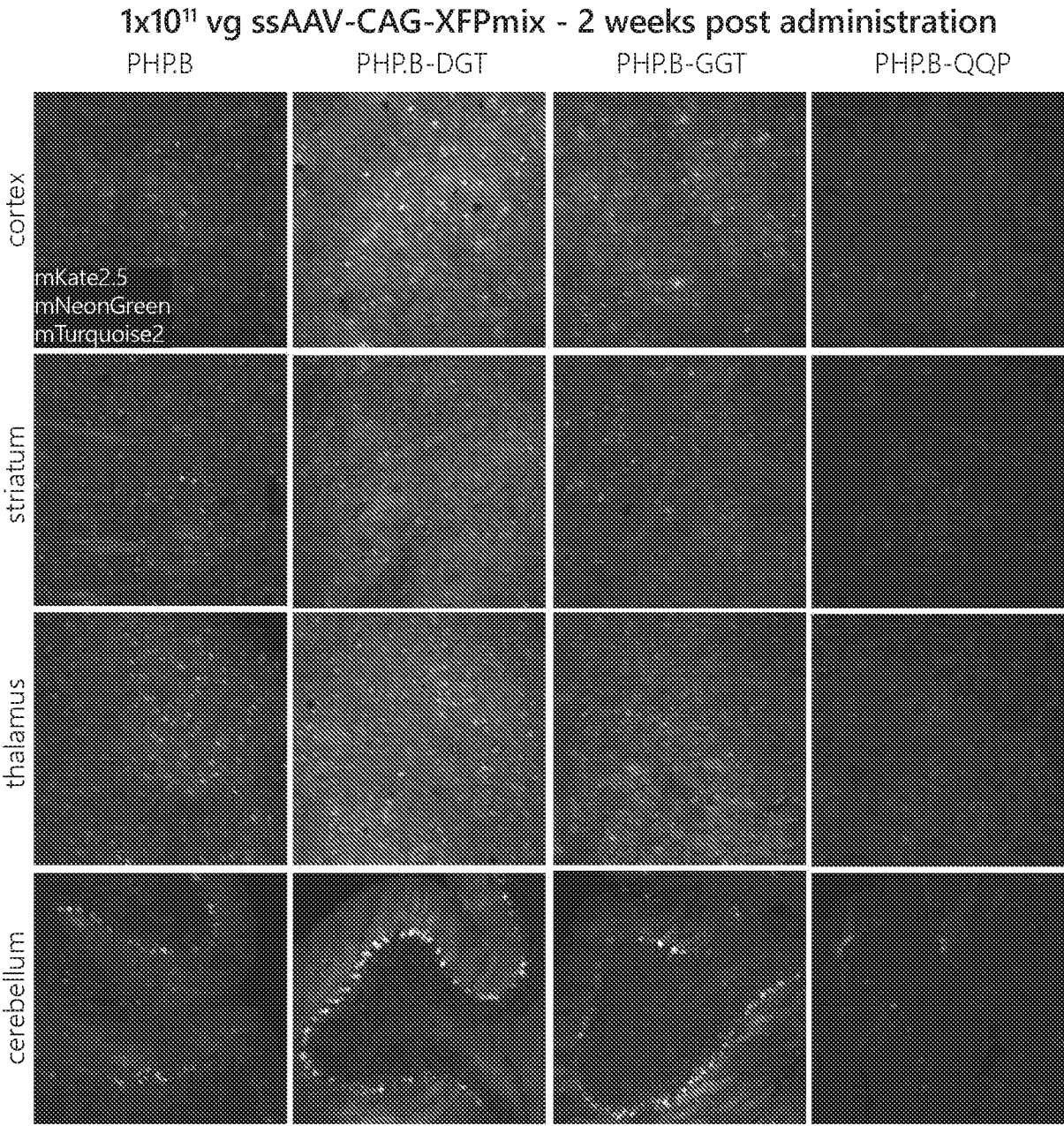


FIG. 16

3x10¹¹ vg ssAAV-CAG-XFPmix - 3 weeks post administration

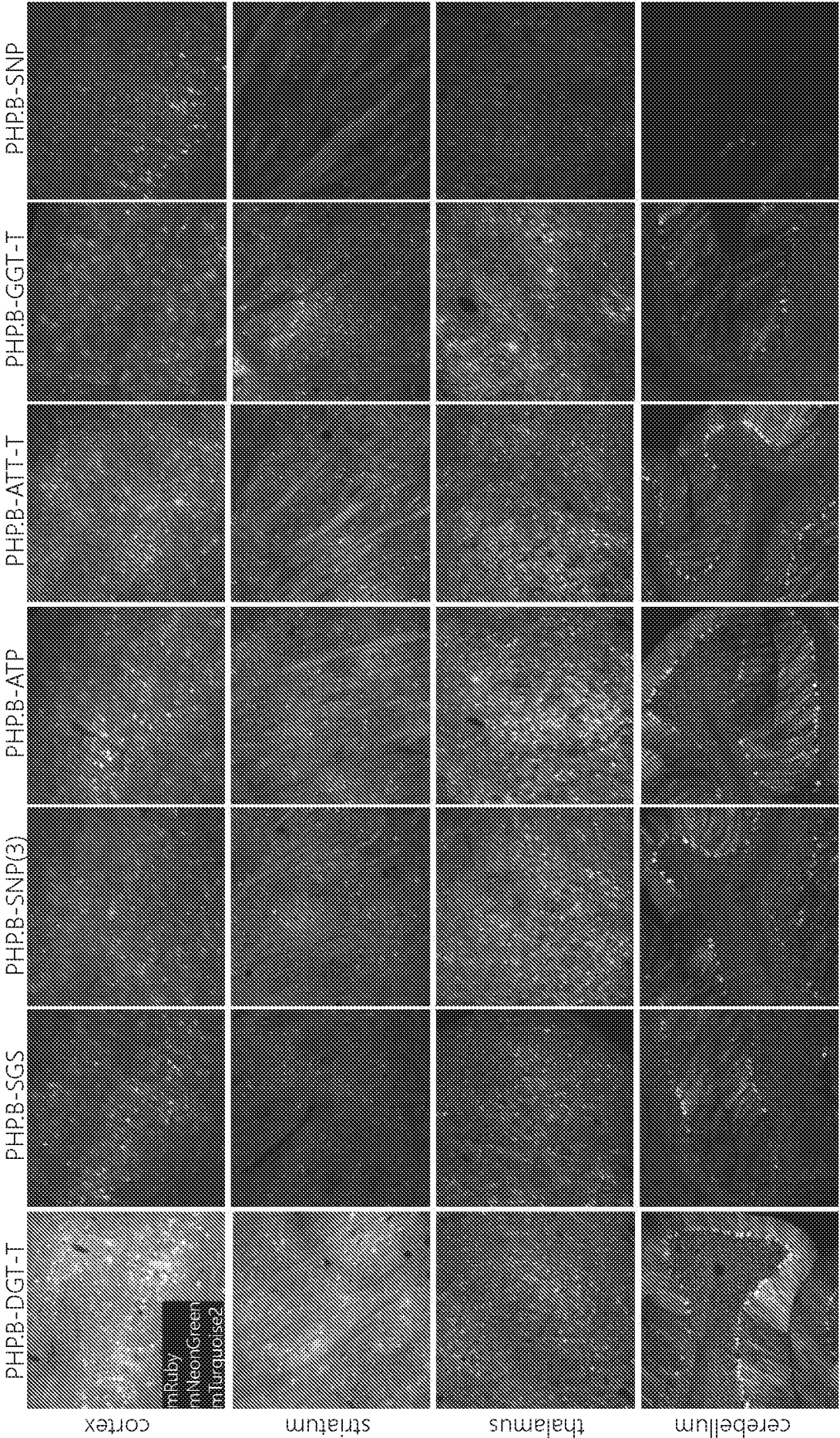


FIG. 17

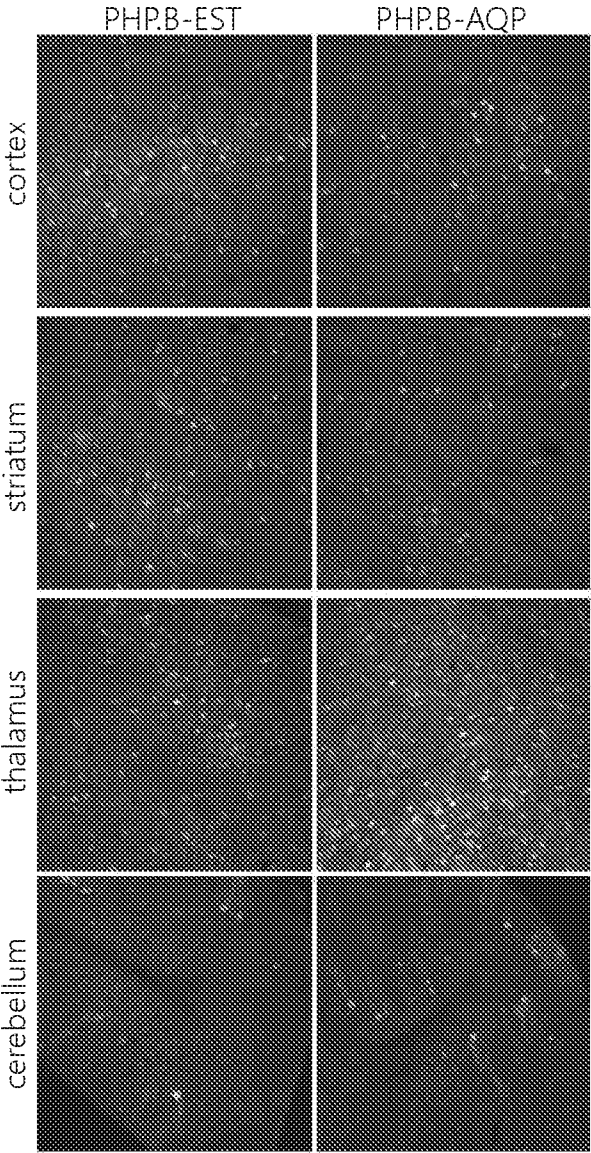


FIG. 18

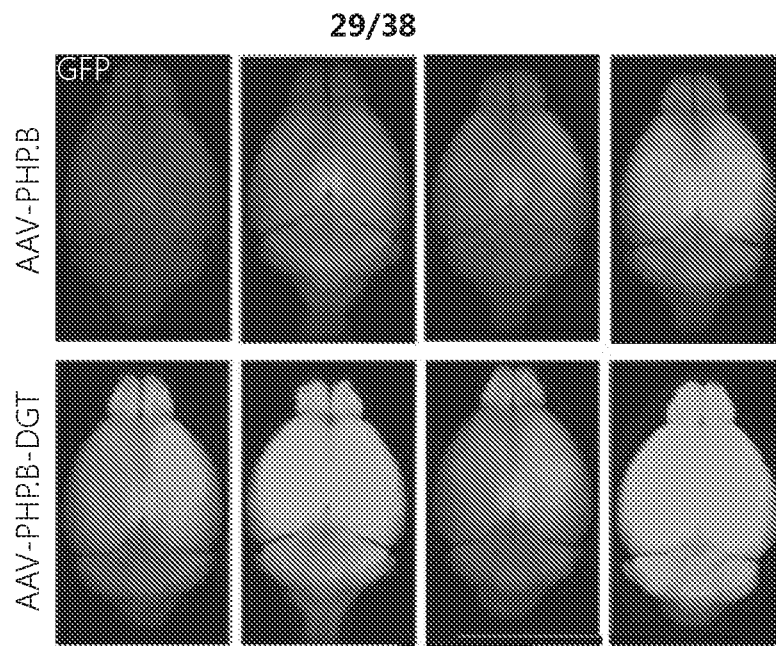
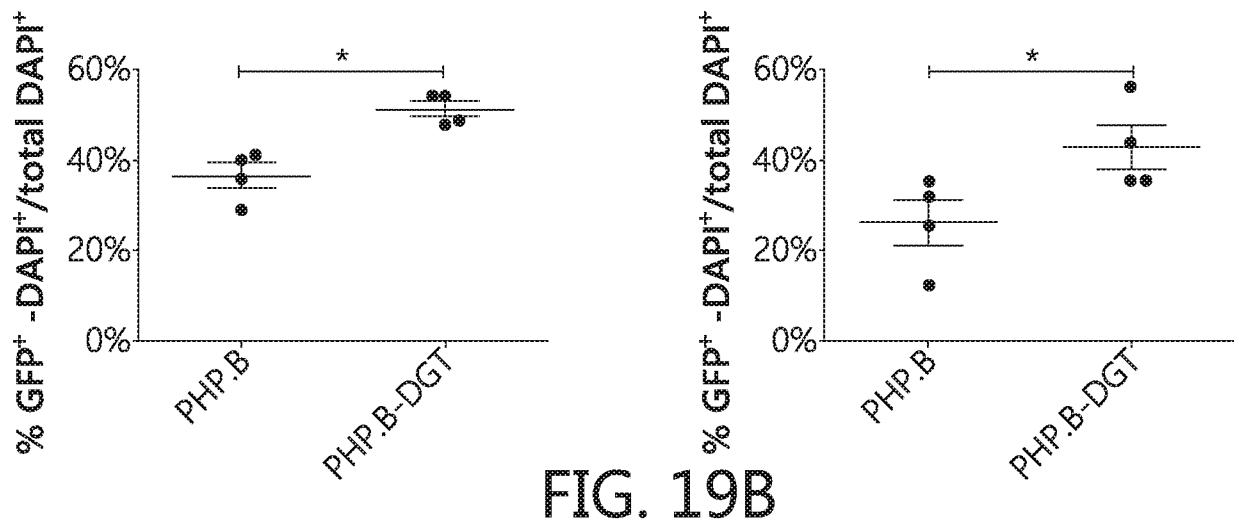
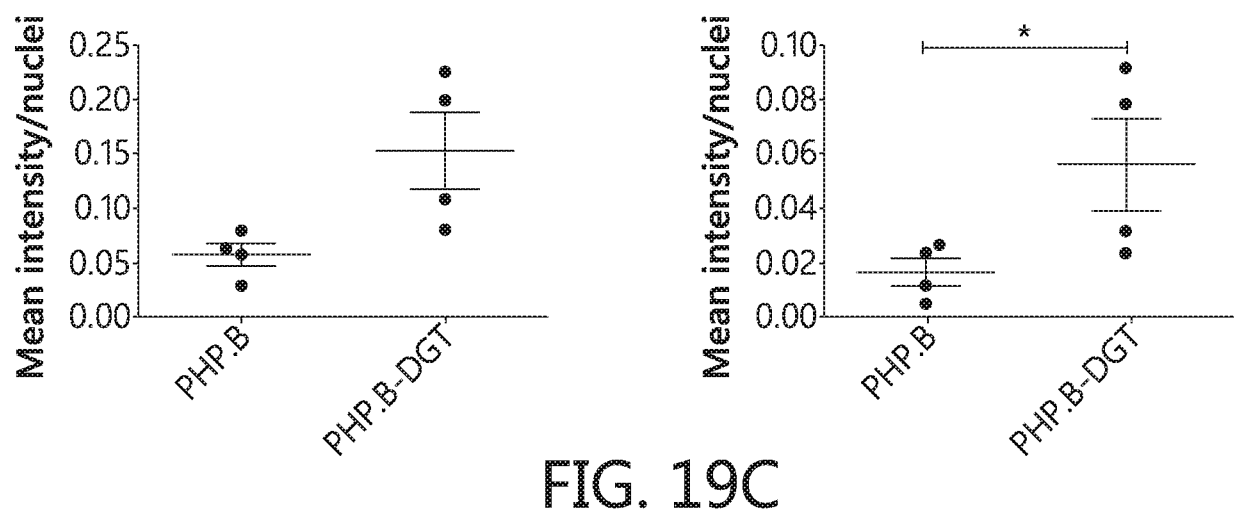
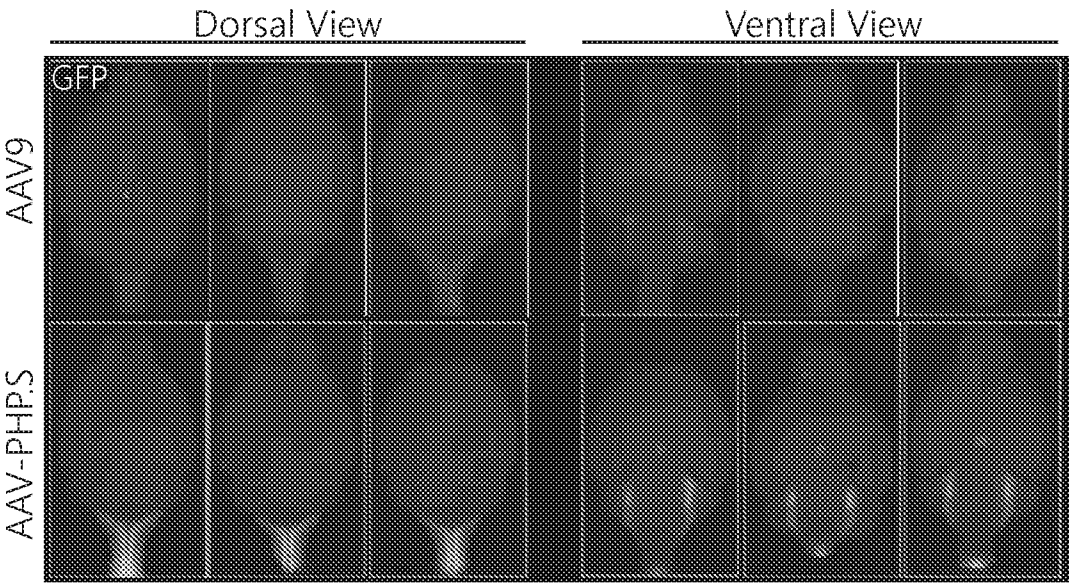


FIG. 19A



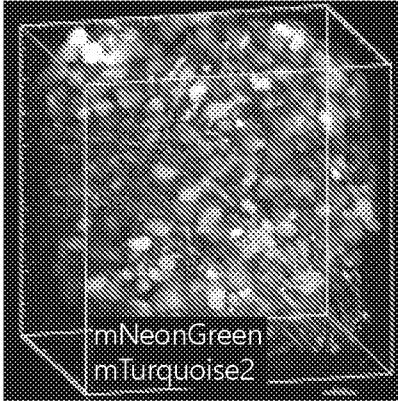
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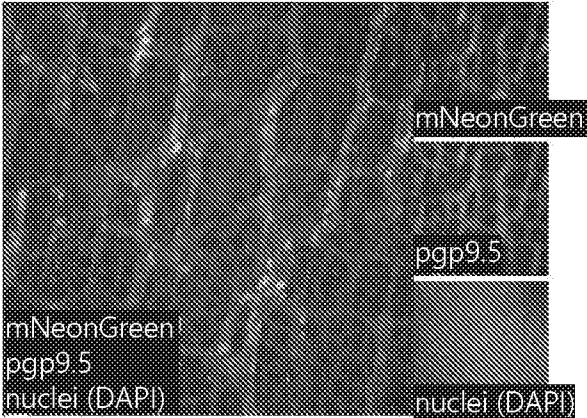
GFP fluorescence - whole brain images
FIG. 20A



Spinal Cord
FIG. 20B



Dorsal root ganglia
FIG. 20C



Intestine
FIG. 20D

PHPB 7-mer - inserted between AA 588-589	XXXX1	<u>SAQXXX</u> <u>TLAVPF</u> <u>FKAQAQ</u> (SEQ ID NO: 49)
	XXXX2	<u>SAQXXX</u> <u>VPF</u> <u>FKAQAQ</u> (SEQ ID NO: 50)
	XXXX3	<u>SAQ</u> <u>TL</u> <u>XXXX</u> <u>FKAQAQ</u> (SEQ ID NO: 51)
	XXXX4	<u>SAQ</u> <u>TL</u> <u>AV</u> <u>XXXX</u> <u>AQAQ</u> (SEQ ID NO: 52)
	XXXX5	<u>SAQ</u> <u>TL</u> <u>AVPF</u> <u>XXXX</u> <u>AQ</u> (SEQ ID NO: 53)

FIG. 21

FIG. 22A

AAV-PHP.N VP1 Capsid Sequence
SEQ ID NO: 46

MAADGYLPDWLEDNLSEGIREWWALKPGAPQPKANQQHQDNARGLVLPGYKYLPGNGLDKGEF
VNAADAAALEHDKAYDQQLKAGDNPYLKYNHADAEEFQERLKEDTSFGNIGRAVFOAKRLLLEP
LGLVEEAAKTAPGKKRPVEQSPQEPDSSAGIGKSGAQPAKKRLNFGQTGDTESVDPQPIGEPP
AAPSGVGSLTMASGGGAPVADNNEGADGVGSSSGNWHCDSQWLGDRTVITSTRTWTALPTYNNHL
YKQISNSTSGSSNDNAYFGYSTPWGYFDENRFCHFSRDPDQRLINNNWGFPRKRLNFKLFNI
QVKEVTDNNGVKTIANNLTSVQVFTDSYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTIND
GSQAVGRSSFYCYCLEYFPSQMLRTGNNFQFSYEFENVPFHSSYAHSQSLDRLMNPLIDQYLYYLS
RTINGSGQNQQTLLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSEFAWPGASSWALN
GRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGRDNVDADKVMITNEEEIKTTNPVATES
YGQVATNHQSAQAVRTSLAQQTGWVQNQGILPGMVWQDRDVYLQGPWAKIPHDTGDNFHPSP
LMGGFGMKHPPPPQILIKNTVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENSKRWNP
EIQYTSNYYKSNNVEFAVNTGVYSEPRPIGTRYLTRNL

FIG. 22B

AAV-PHP.S VP1 Capsid Sequence
SEQ ID NO: 47

MAADGYLPDWLEDNLSEGIREWWALKPGAPQPKANQQHQDNARGLVLPGYKYLPGNGLDKGEF
VNAADAAALEHDKAYDQQLKAGDNPYLKYNHADAEEFQERLKEDTSFGNIGRAVFOAKRLLLEP
LGLVEEAAKTAPGKKRPVEQSPQEPDSSAGIGKSGAQPAKKRLNFGQTGDTESVDPQPIGEPP
AAPSGVGSLTMASGGGAPVADNNEGADGVGSSSGNWHCDSQWLGDRTVITSTRTWTALPTYNNHL
YKQISNSTSGSSNDNAYFGYSTPWGYFDENRFCHFSRDPDQRLINNNWGFPRKRLNFKLFNI
QVKEVTDNNGVKTIANNLTSVQVFTDSYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTIND
GSQAVGRSSFYCYCLEYFPSQMLRTGNNFQFSYEFENVPFHSSYAHSQSLDRLMNPLIDQYLYYLS
RTINGSGQNQQTLLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSEFAWPGASSWALN
GRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGRDNVDADKVMITNEEEIKTTNPVATES
YGQVATNHQSAQAVRTSLAQQTGWVQNQGILPGMVWQDRDVYLQGPWAKIPHDTGDNFHPSP
LMGGFGMKHPPPPQILIKNTVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENSKRWNP
EIQYTSNYYKSNNVEFAVNTGVYSEPRPIGTRYLTRNL

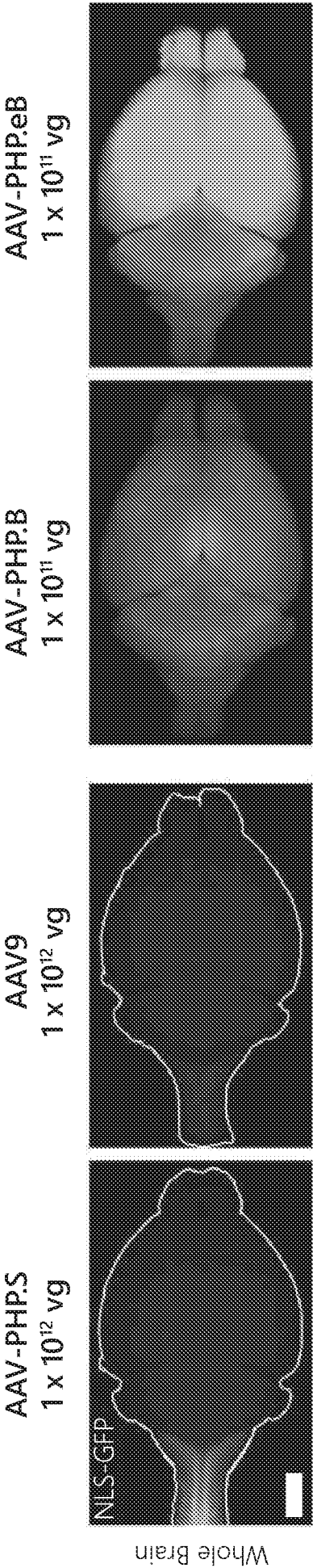


FIG. 23A

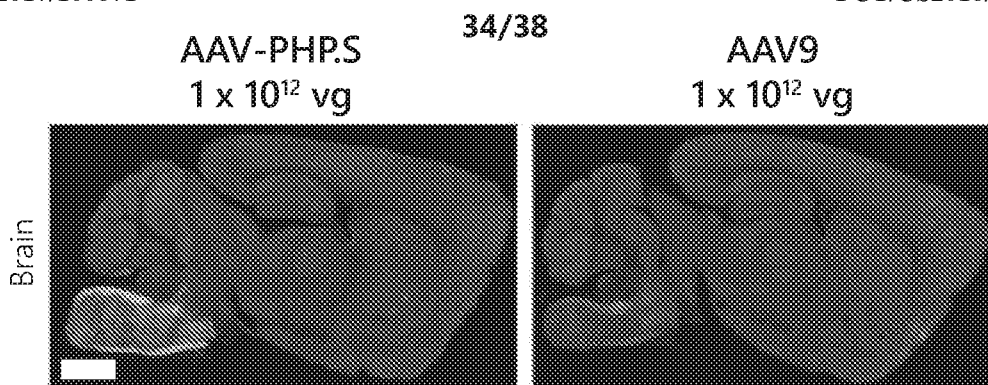


FIG. 23B

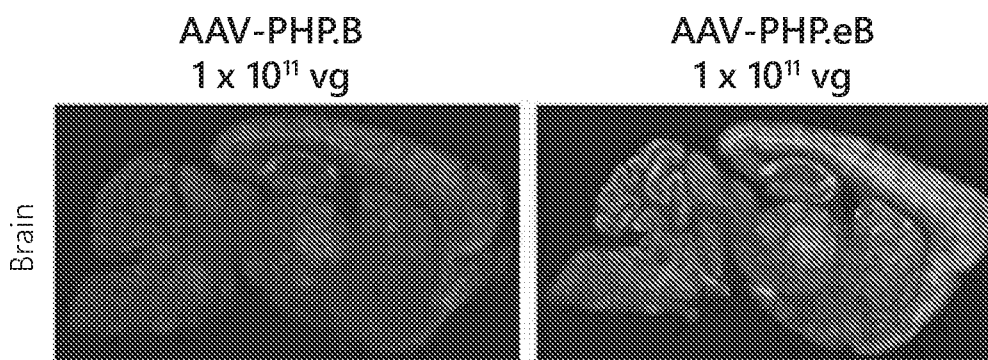


FIG. 23C

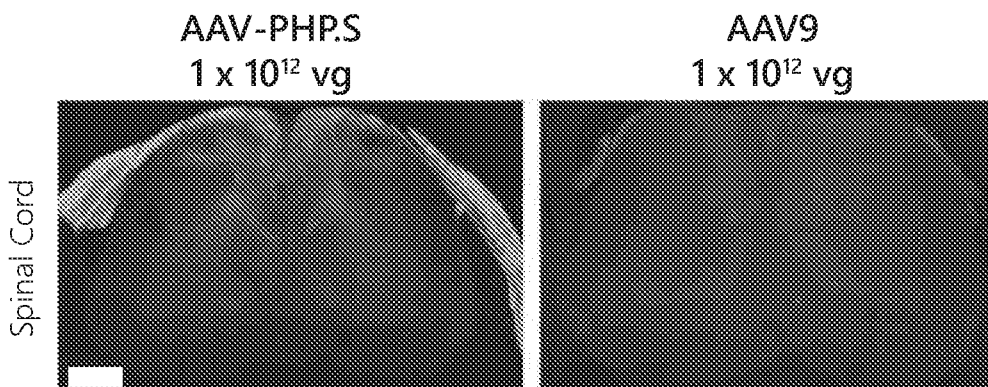


FIG. 23D

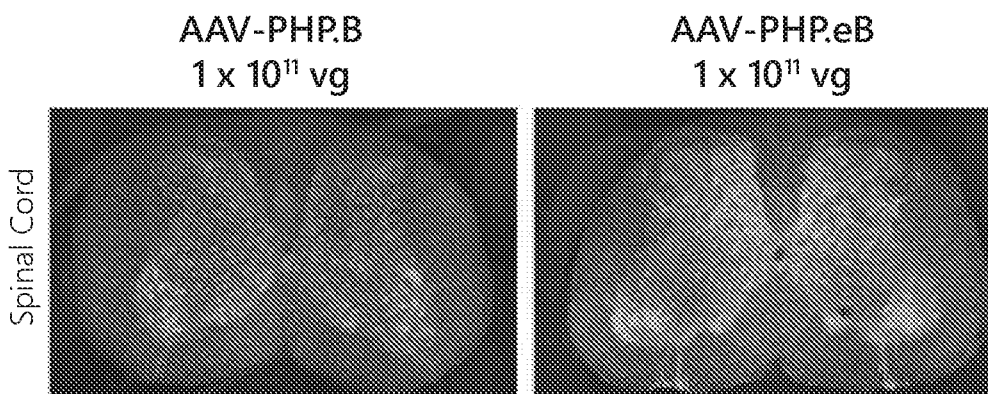


FIG. 23E

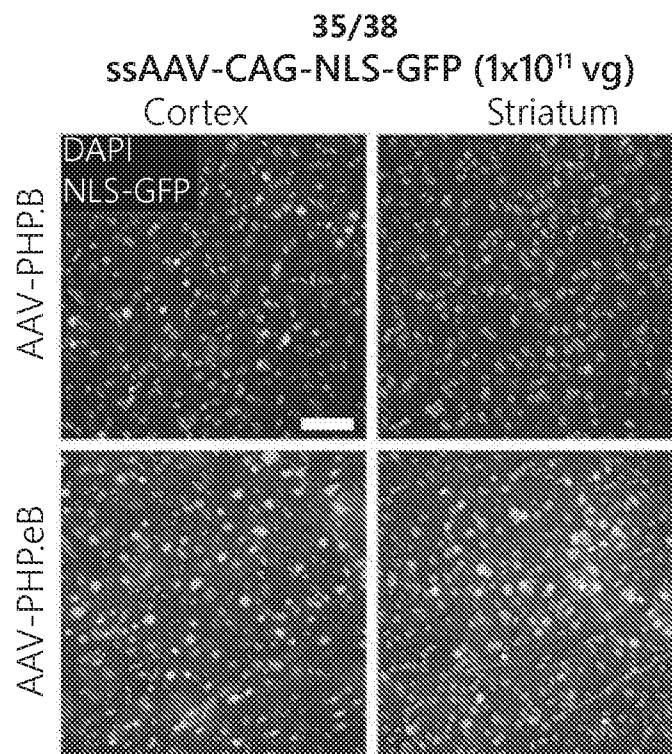


FIG. 24A

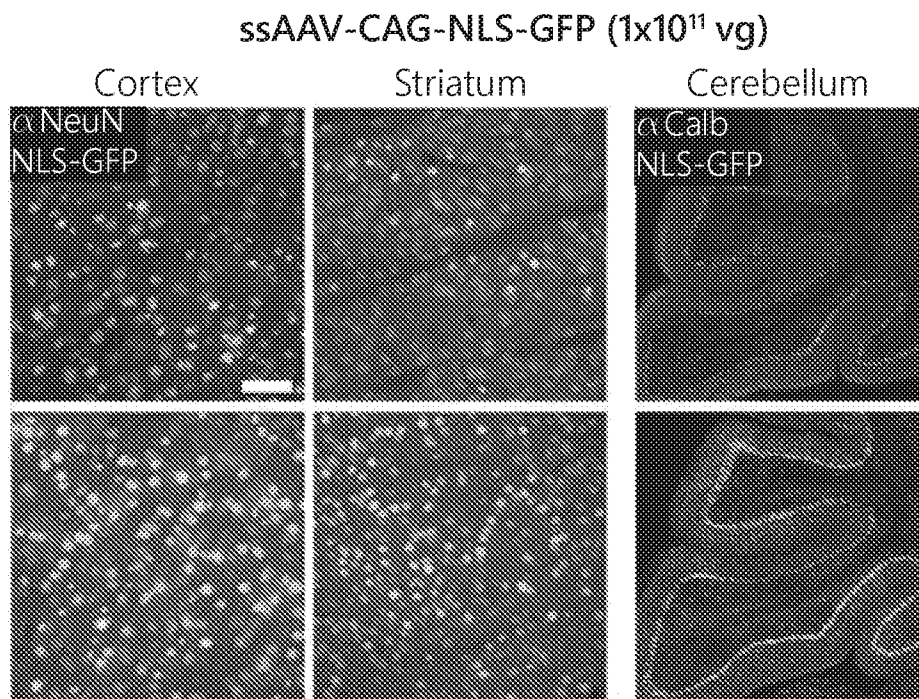


FIG. 24B

FIG. 24C

○PHP.B ⊗PHP.eB

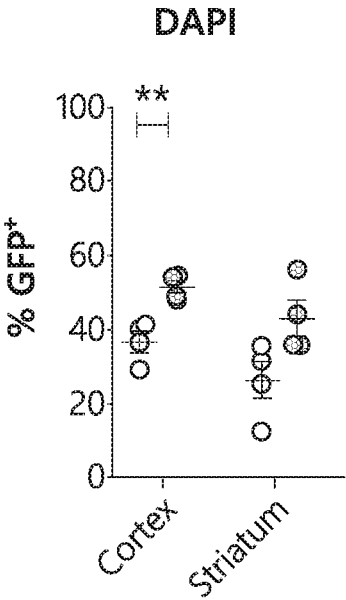


FIG. 24D

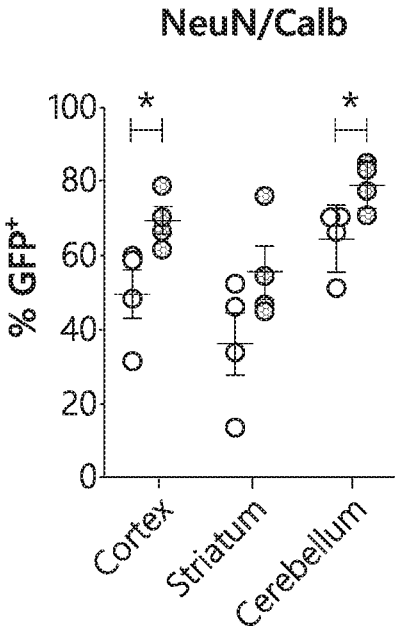


FIG. 24E

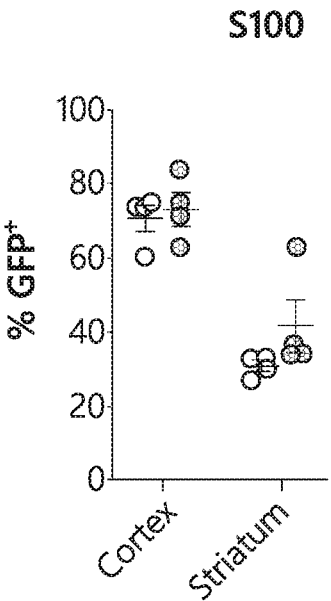


FIG. 24F

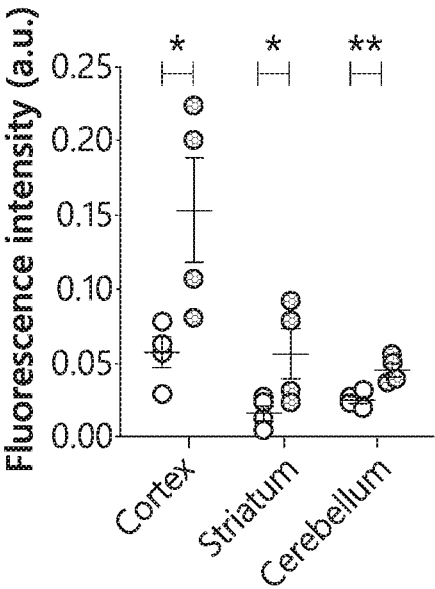


FIG. 24G

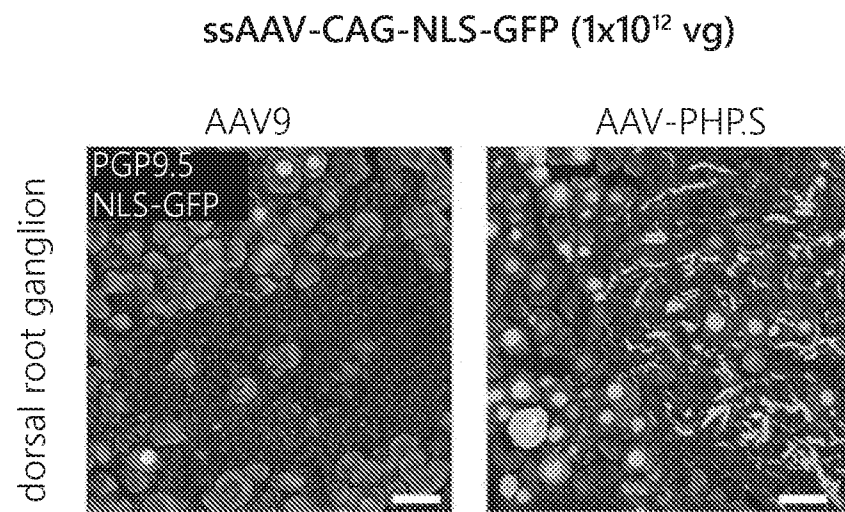


FIG. 25A

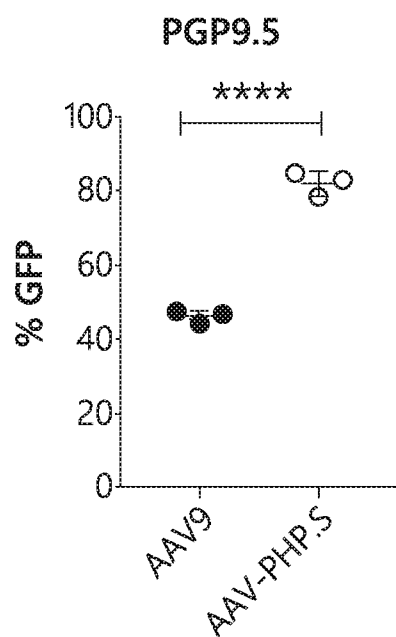


FIG. 25B

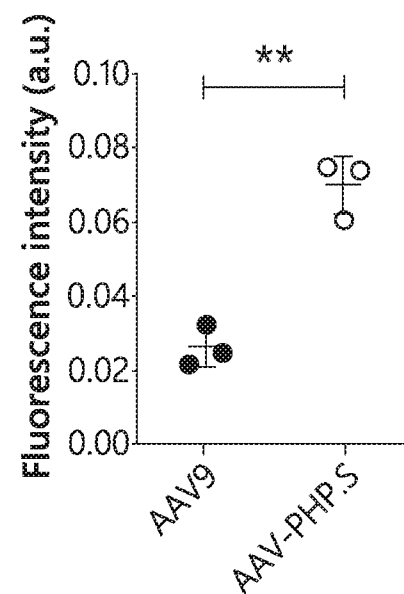


FIG. 25C

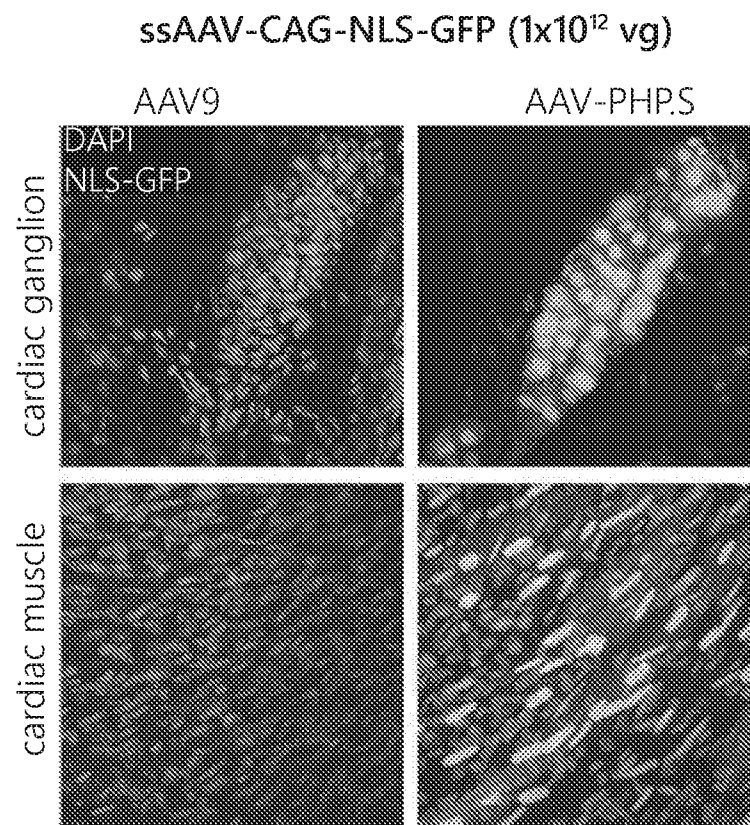


FIG. 25D