

FIG 1A

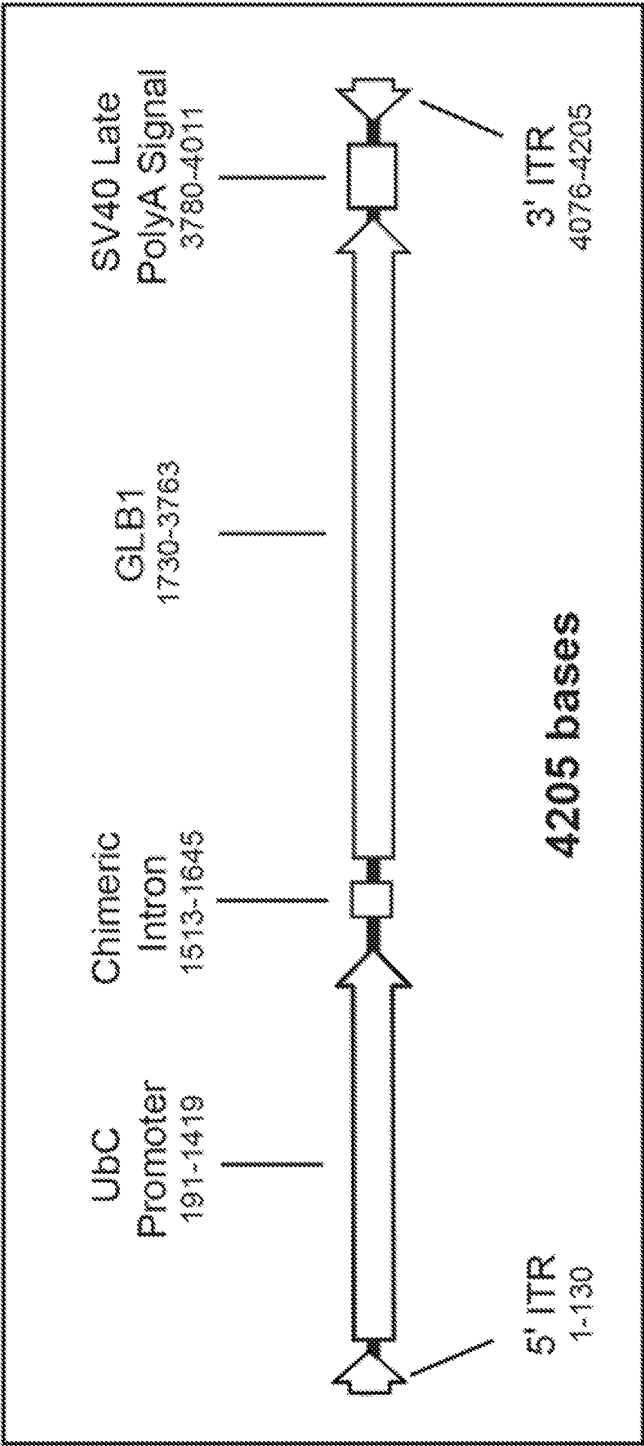


FIG 1B

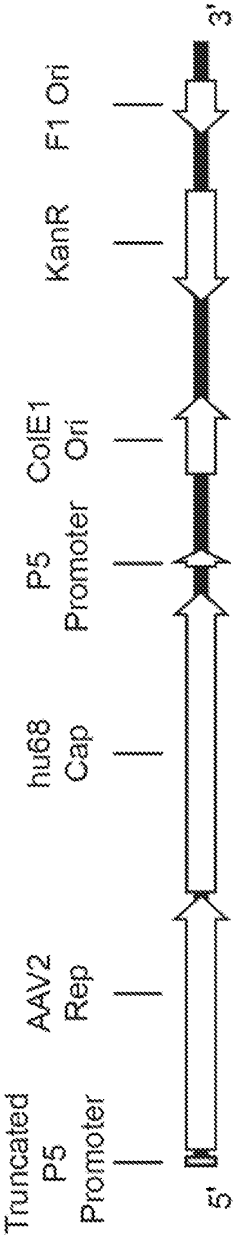
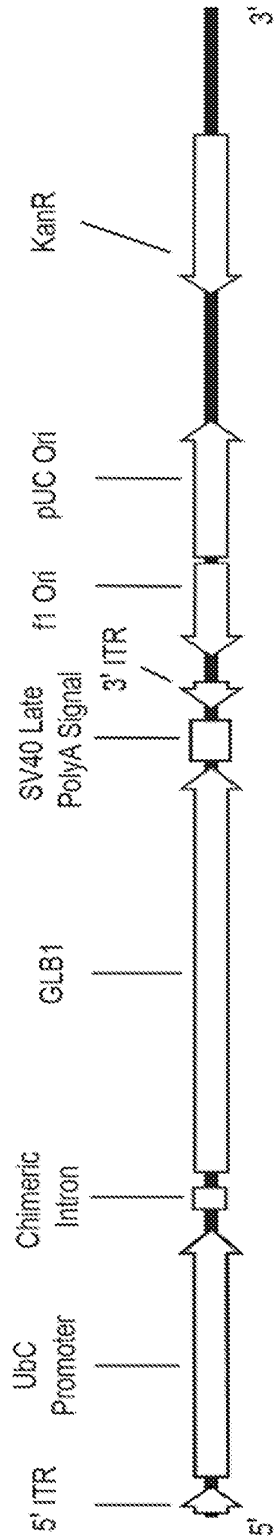


FIG 1C

FIG 2A

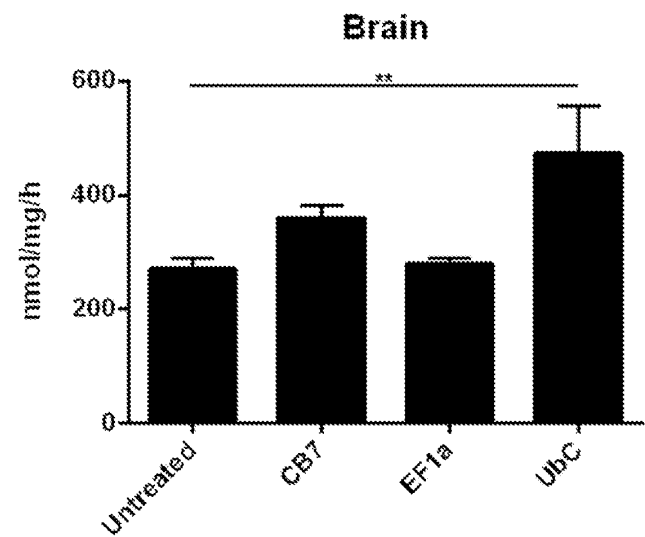
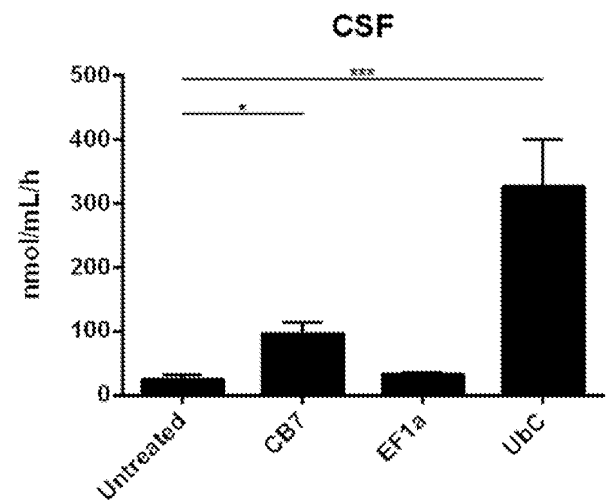


FIG 2B



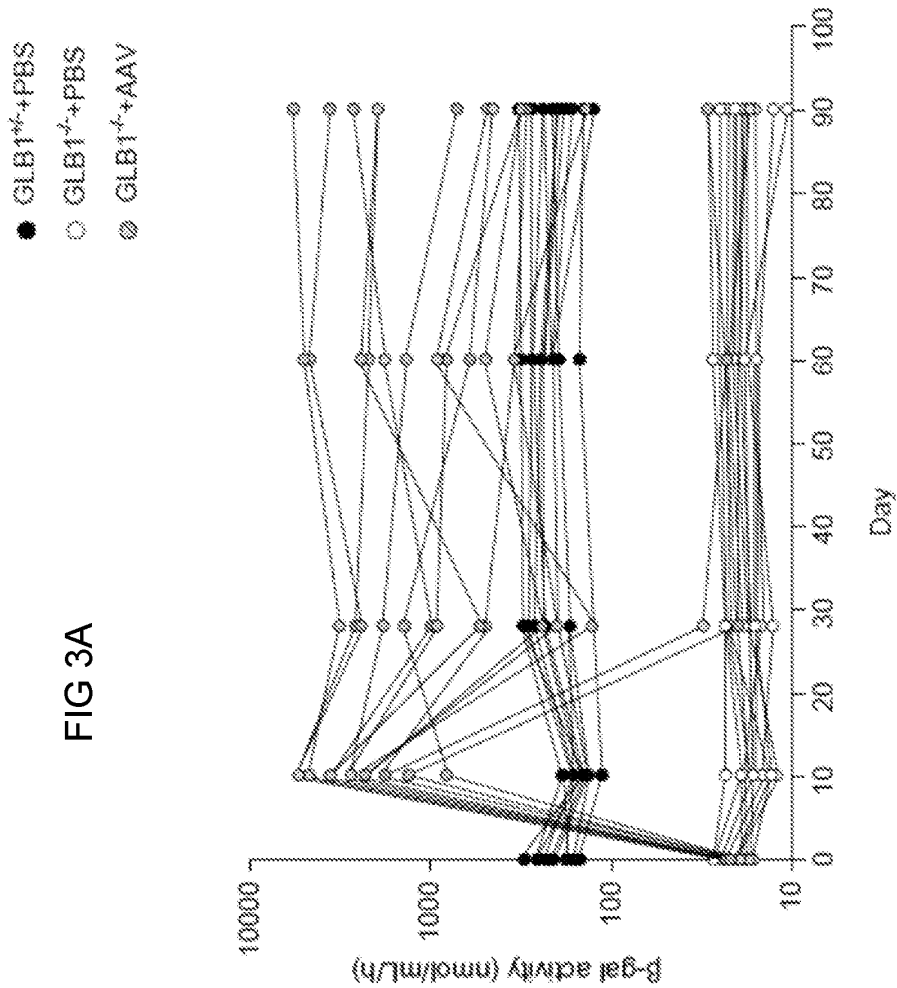


FIG 3B

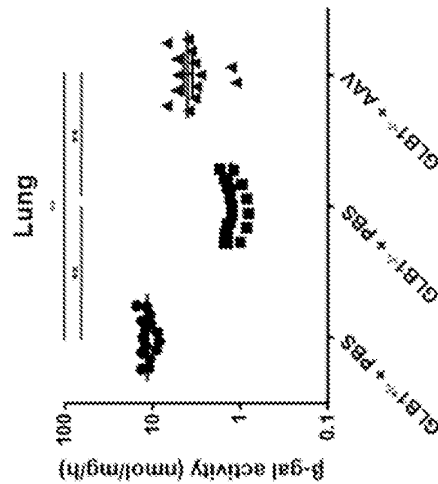


FIG 3C

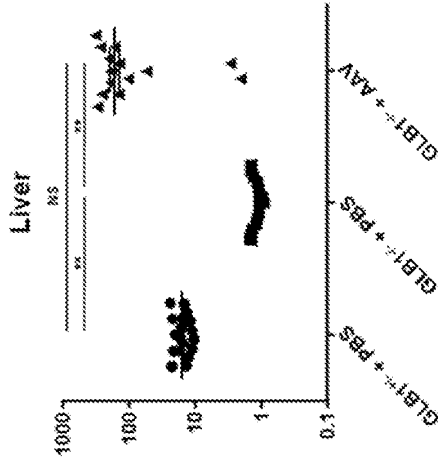


FIG 3D

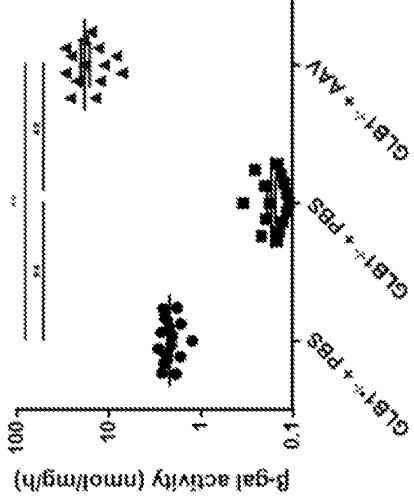


FIG 3E

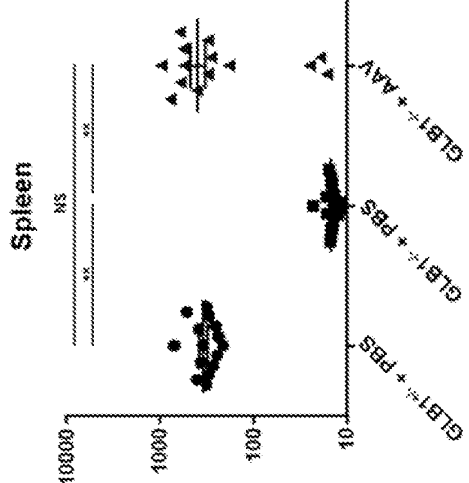


FIG 4A

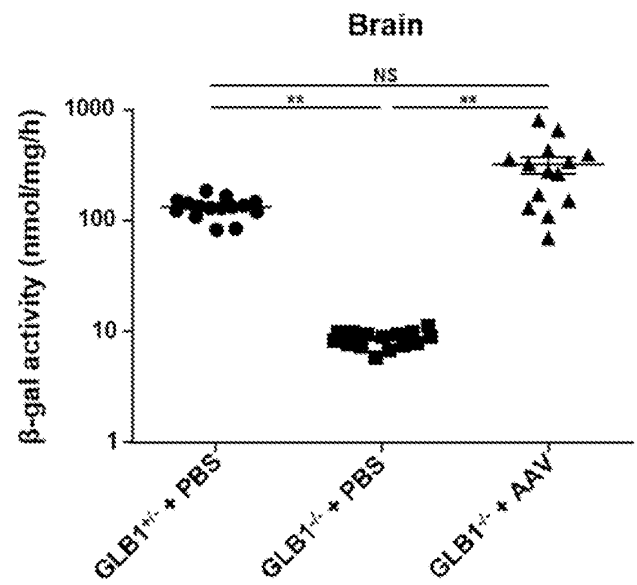


FIG 4B

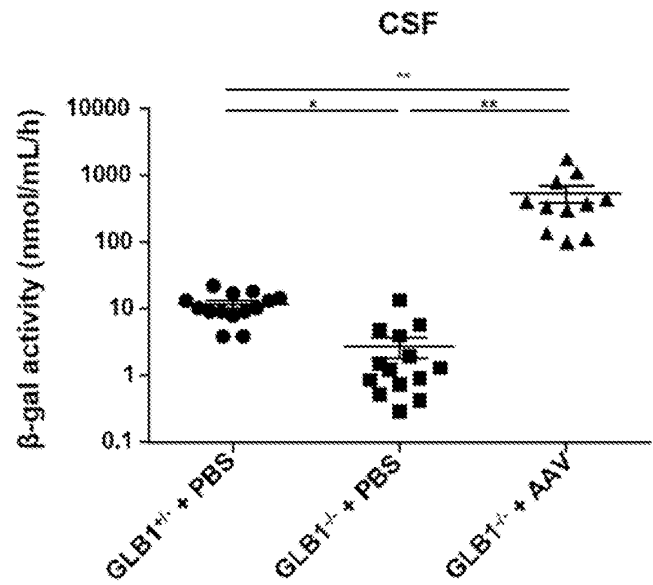


FIG 5

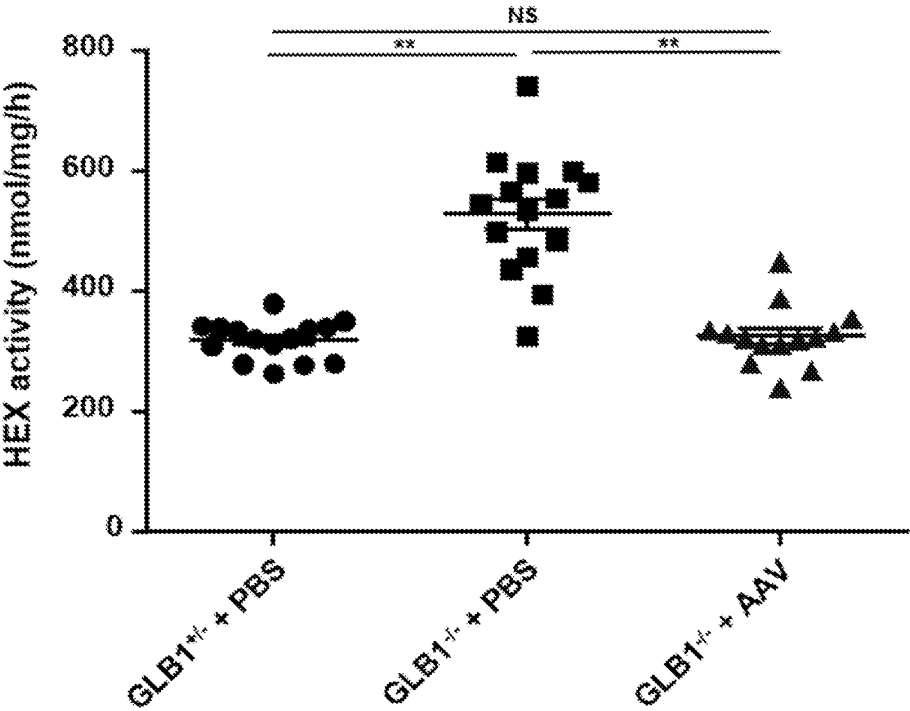
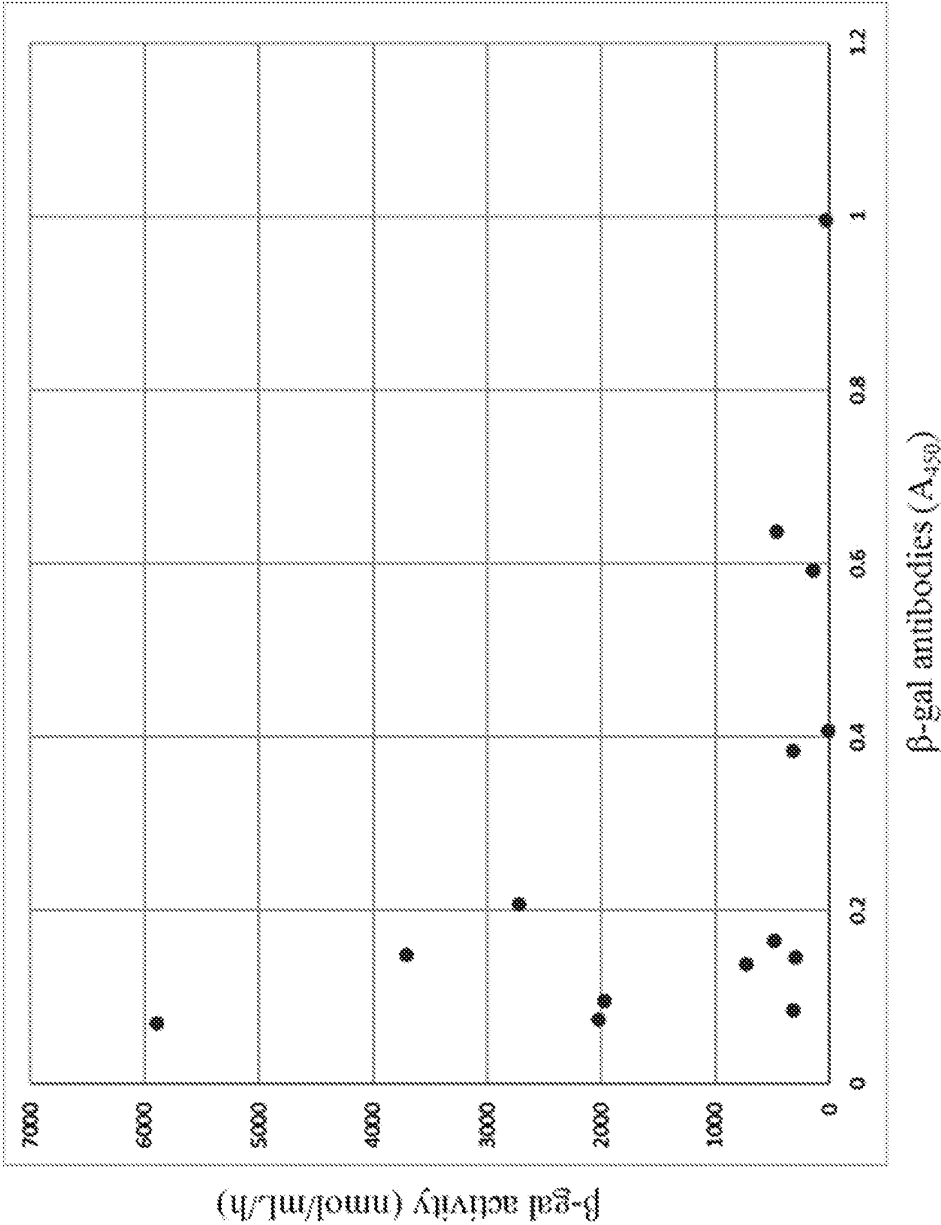


FIG 6



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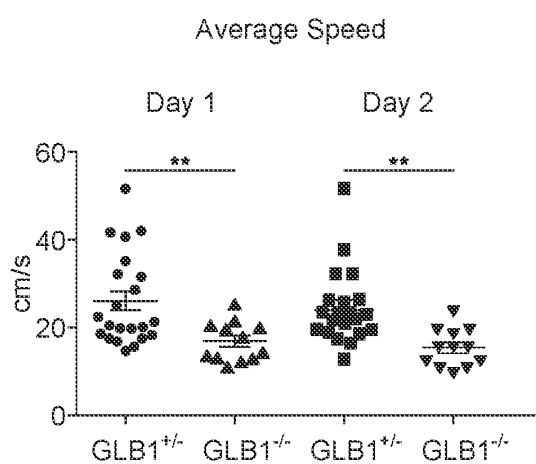


FIG. 7A

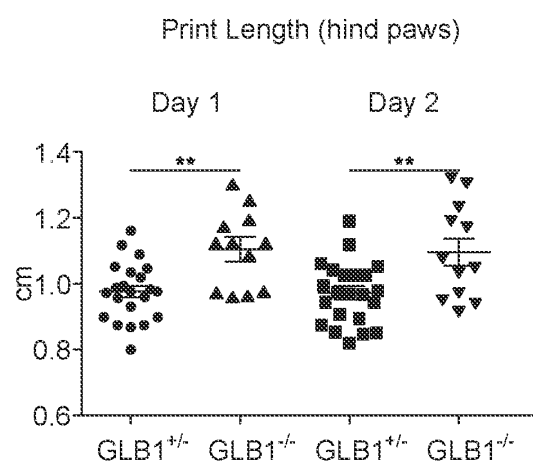


FIG. 7B

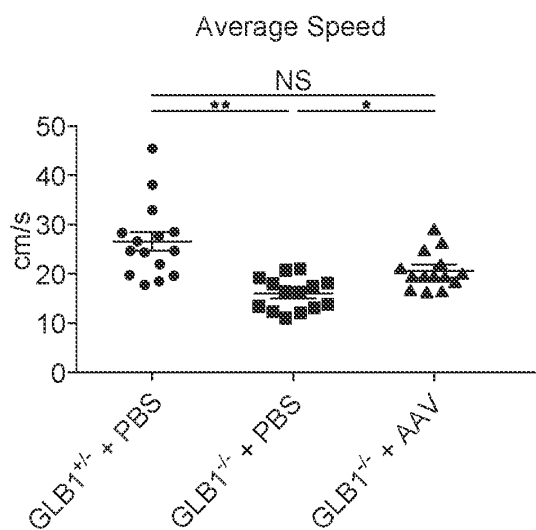


FIG. 7C

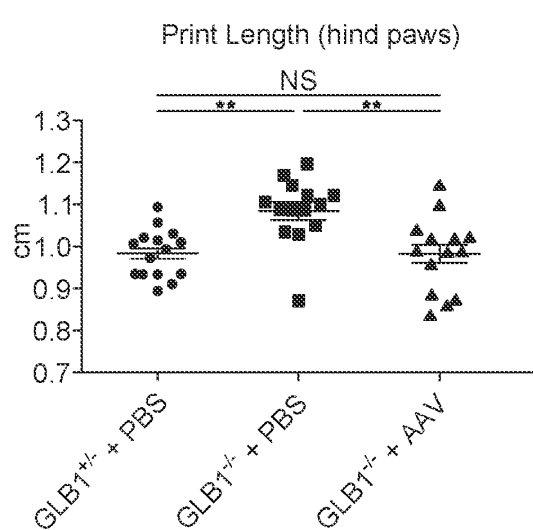


FIG. 7D

FIG 7E

GLB1^{+/+} + PBS

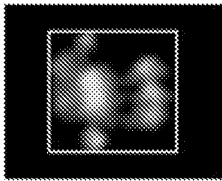


FIG 7F

GLB1^{+/+} + PBS

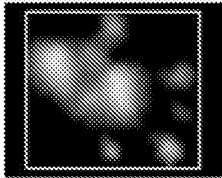


FIG 7G

GLB1^{+/+} + AAV

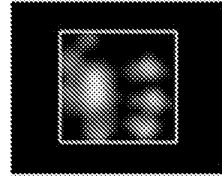


FIG 8A

Hind paw print length vs Speed

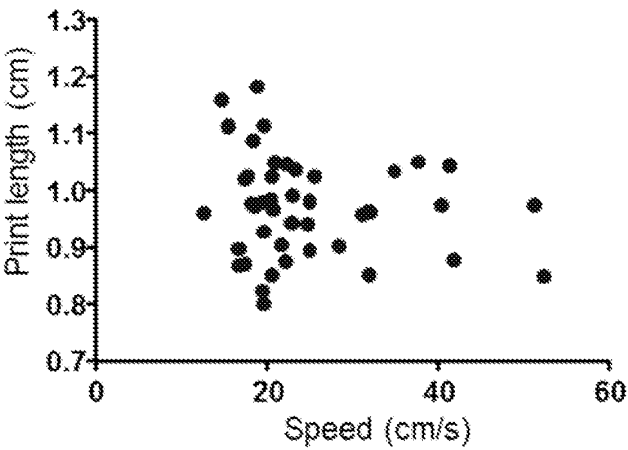


FIG 8B

Stride length vs Speed

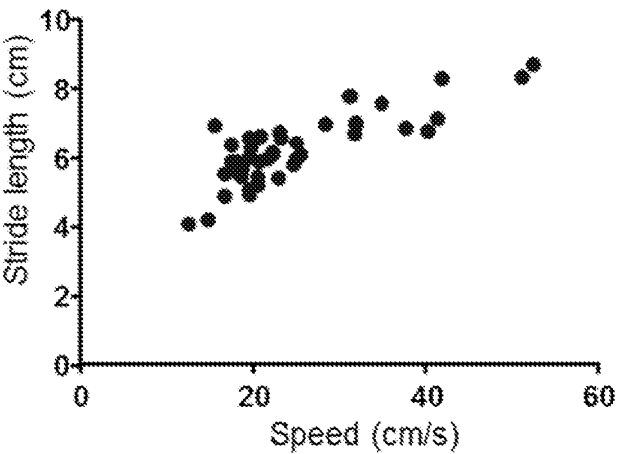


FIG 9A

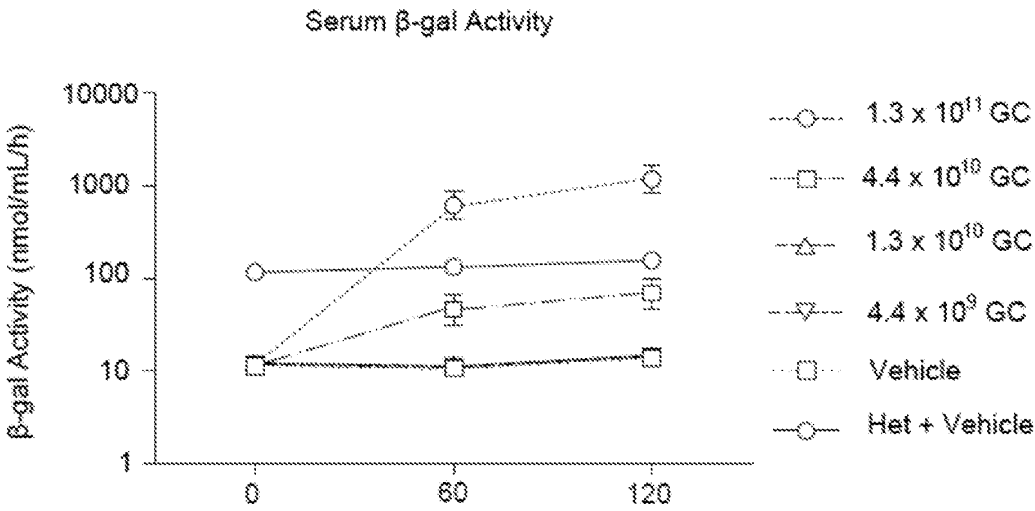


FIG 9B

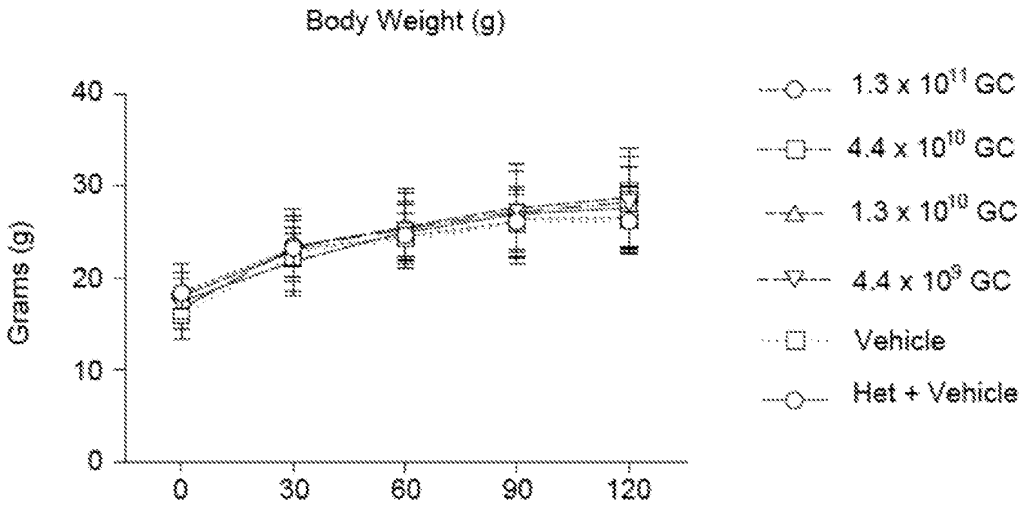


FIG 9C

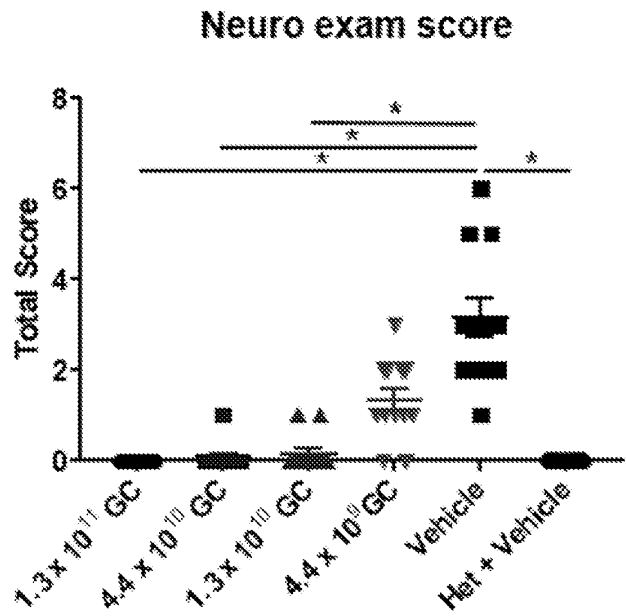


FIG 9D

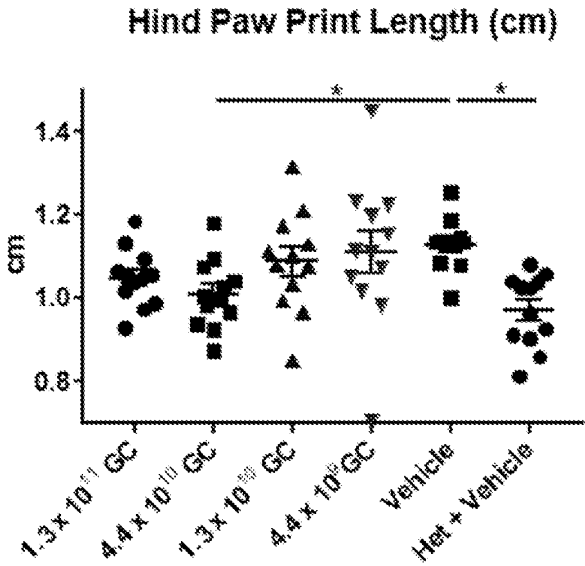


FIG 9E

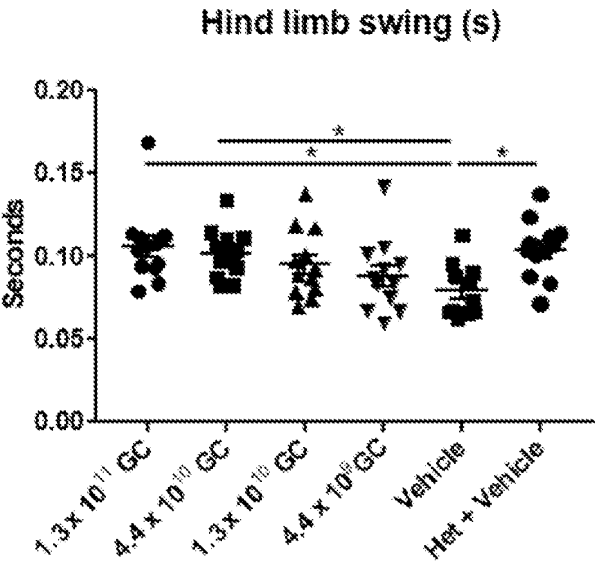
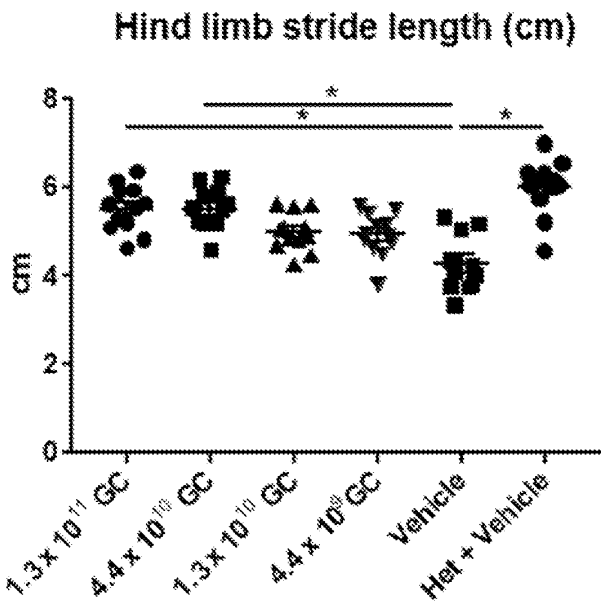
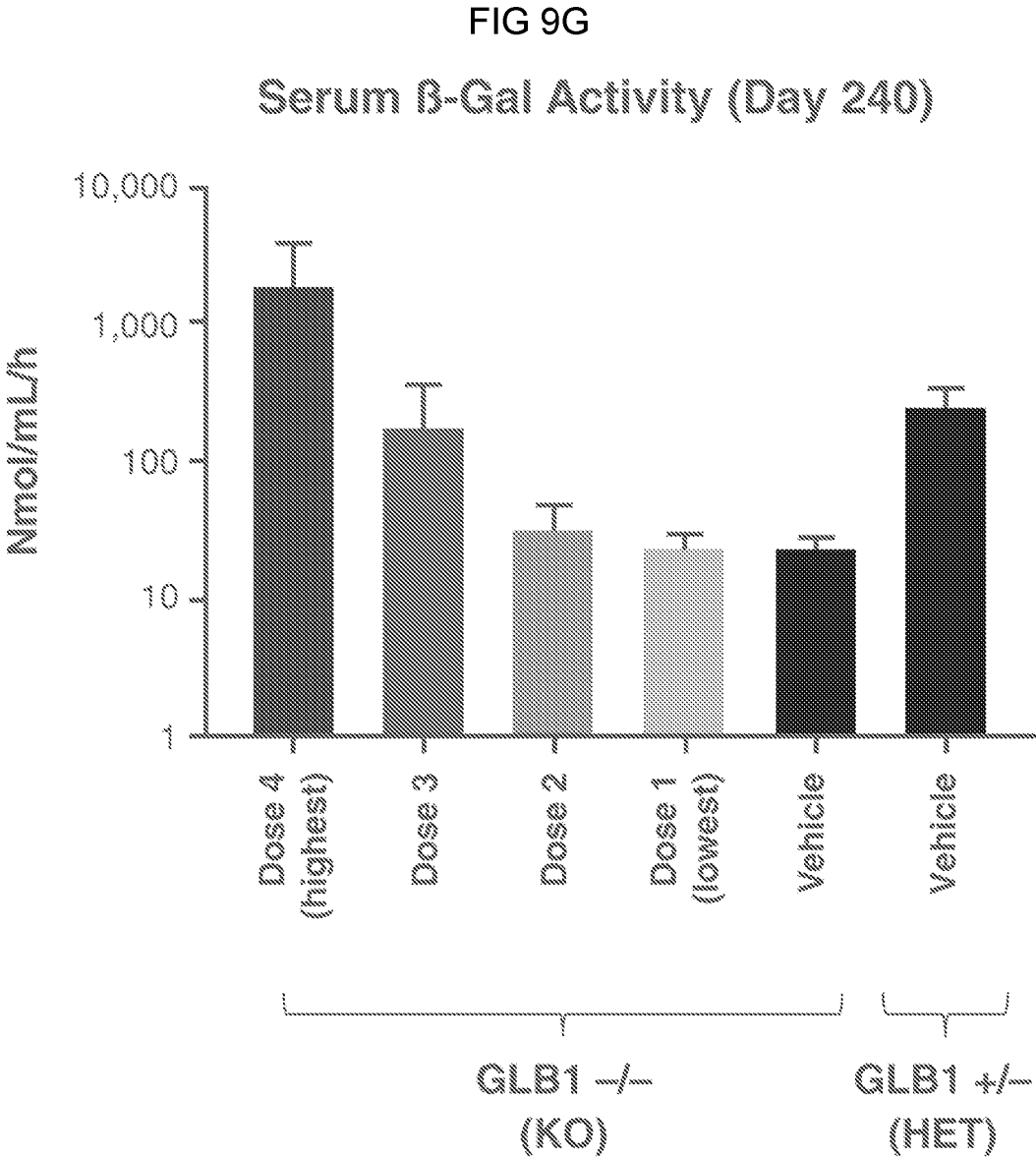


FIG 9F





AAV9 hu.68.VP1 hu.31 hu.32	(1)	1	MAADGYLPDWLEDMISEGIREMMALKPGAPQKAKNDQHQDNARGLVLPGYKYLPGNGLDKGEPMADAAALEHDKAYDQOLKAGDNPYLKYNHADAEF	100
		(1)	MAADGYLPDWLEDMISEGIREMMALKPGAPQKAKNDQHQDNARGLVLPGYKYLPGNGLDKGEPMADAAALEHDKAYDQOLKAGDNPYLKYNHADAEF	
		(1)	MAADGYLPDWLEDMISEGIREMMALKPGAPQKAKNDQHQDNARGLVLPGYKYLPGNGLDKGEPMADAAALEHDKAYDQOLKAGDNPYLKYNHADAEF	
		(1)	MAADGYLPDWLEDMISEGIREMMALKPGAPQKAKNDQHQDNARGLVLPGYKYLPGNGLDKGEPMADAAALEHDKAYDQOLKAGDNPYLKYNHADAEF	
AAV9 hu.68.VP1 hu.31 hu.32	(101)	101	QERLKEDTSFGGNLGRAVFOAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSAGGKSGAQPAKKRLNFGQTGDTESVDPQPIGEPPAAPSGVGS	200
		(101)	QERLKEDTSFGGNLGRAVFOAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSAGGKSGAQPAKKRLNFGQTGDTESVDPQPIGEPPAAPSGVGS	
		(101)	QERLKEDTSFGGNLGRAVFOAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSAGGKSGAQPAKKRLNFGQTGDTESVDPQPIGEPPAAPSGVGS	
		(101)	QERLKEDTSFGGNLGRAVFOAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSAGGKSGAQPAKKRLNFGQTGDTESVDPQPIGEPPAAPSGVGS	
AAV9 hu.68.VP1 hu.31 hu.32	(201)	201	LTMASGGGAPVADNNEGADGVGSSGNMHCDQMILGDRVITSTRTWTALPTYNHLYKOISNSTGGSSNDNAVFGYSTPWGYFDNFRHCHFSRDPWQR	300
		(201)	LTMASGGGAPVADNNEGADGVGSSGNMHCDQMILGDRVITSTRTWTALPTYNHLYKOISNSTGGSSNDNAVFGYSTPWGYFDNFRHCHFSRDPWQR	
		(201)	LTMASGGGAPVADNNEGADGVGSSGNMHCDQMILGDRVITSTRTWTALPTYNHLYKOISNSTGGSSNDNAVFGYSTPWGYFDNFRHCHFSRDPWQR	
		(201)	LTMASGGGAPVADNNEGADGVGSSGNMHCDQMILGDRVITSTRTWTALPTYNHLYKOISNSTGGSSNDNAVFGYSTPWGYFDNFRHCHFSRDPWQR	
AAV9 hu.68.VP1 hu.31 hu.32	(301)	301	LINNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLITSTVQVFTDSYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTINDGSDAVGRSSFYCLEYF	400
		(301)	LINNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLITSTVQVFTDSYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTINDGSDAVGRSSFYCLEYF	
		(301)	LINNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLITSTVQVFTDSYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTINDGSDAVGRSSFYCLEYF	
		(301)	LINNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLITSTVQVFTDSYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTINDGSDAVGRSSFYCLEYF	
AAV9 hu.68.VP1 hu.31 hu.32	(401)	401	PSQMLRTGNINFQFSYEFENVPFHSSYAHQSQSLDRLMNPLIDQYLYLSKTINGSGNQQTILKFSVAGPSNMVAQGRNYPGPSYRQQRVSTTVTQNNINSE	500
		(401)	PSQMLRTGNINFQFSYEFENVPFHSSYAHQSQSLDRLMNPLIDQYLYLSKTINGSGNQQTILKFSVAGPSNMVAQGRNYPGPSYRQQRVSTTVTQNNINSE	
		(401)	PSQMLRTGNINFQFSYEFENVPFHSSYAHQSQSLDRLMNPLIDQYLYLSKTINGSGNQQTILKFSVAGPSNMVAQGRNYPGPSYRQQRVSTTVTQNNINSE	
		(401)	PSQMLRTGNINFQFSYEFENVPFHSSYAHQSQSLDRLMNPLIDQYLYLSKTINGSGNQQTILKFSVAGPSNMVAQGRNYPGPSYRQQRVSTTVTQNNINSE	

FIG 10A

AAV9	(501)	FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLFGKQGTGRDNDVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQQTGWQNNQG
hu.68.VP1	(501)	FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLFGKQGTGRDNDVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQQTGWQNNQG
hu.31	(501)	FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLFGKQGTGRDNDVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQQTGWQNNQG
hu.32	(501)	FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLFGKQGTGRDNDVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQQTGWQNNQG
		601
AAV9	(601)	ILPGMWQDRDYYLQGPWWAKIPHTDGNFHPSPILMGGMKHPPPQILIKNTVPADPPTAFNKKLNLSFTQYSTGQVSVIEIWELOKENSKRWNPEIQ
hu.68.VP1	(601)	ILPGMWQDRDYYLQGPWWAKIPHTDGNFHPSPILMGGMKHPPPQILIKNTVPADPPTAFNKKLNLSFTQYSTGQVSVIEIWELOKENSKRWNPEIQ
hu.31	(601)	ILPGMWQDRDYYLQGPWWAKIPHTDGNFHPSPILMGGMKHPPPQILIKNTVPADPPTAFNKKLNLSFTQYSTGQVSVIEIWELOKENSKRWNPEIQ
hu.32	(601)	ILPGMWQDRDYYLQGPWWAKIPHTDGNFHPSPILMGGMKHPPPQILIKNTVPADPPTAFNKKLNLSFTQYSTGQVSVIEIWELOKENSKRWNPEIQ
		701
AAV9	(701)	YTSNYKSNVVEFAVNTTEGVYSEPRPIGTRYLTRNL
hu.68.VP1	(701)	YTSNYKSNVVEFAVNTTEGVYSEPRPIGTRYLTRNL
hu.31	(701)	YTSNYKSNVVEFAVNTTEGVYSEPRPIGTRYLTRNL
hu.32	(701)	YTSNYKSNVVEFAVNTTEGVYSEPRPIGTRYLTRNL

FIG 10B

AAV9	(501)	501	600
hu.68.VP1	(501)	TAAAGAGAGCTCAATTTGGTCAGACTGGCGACACAGAGTCAGTCCCGGACCCCTCAACCAATCGGAGAACCTCCCGCAGCCCTCAGGTGTGGGATCT	
hu.31	(501)	TAAAGAGAGCTCAATTTGGTCAGACTGGCGACACAGAGTCAGTCCCGGACCCCTCAACCAATCGGAGAACCTCCCGCAGCCCTCAGGTGTGGGATCT	
hu.32	(501)	TAAAGAGAGCTCAATTTGGTCAGACTGGCGACACAGAGTCAGTCCCGGACCCCTCAACCAATCGGAGAACCTCCCGCAGCCCTCAGGTGTGGGATCT	
AAV9	(601)	601	700
hu.68.VP1	(601)	CTTACAATGGCTTCAGGTGGTGGCGCACCCAGTGGCAGACAAATACGAAAGGTGCCGATGGAGTGGGTAGTTCTCGGGAAATTTGGCAITTCGGATCCCCAAT	
hu.31	(601)	CTTACAATGGCTTCAGGTGGTGGCGCACCCAGTGGCAGACAAATACGAAAGGTGCCGATGGAGTGGGTAGTTCTCGGGAAATTTGGCAITTCGGATCCCCAAT	
hu.32	(601)	CTTACAATGGCTTCAGGTGGTGGCGCACCCAGTGGCAGACAAATACGAAAGGTGCCGATGGAGTGGGTAGTTCTCGGGAAATTTGGCAITTCGGATCCCCAAT	
AAV9	(701)	701	800
hu.68.VP1	(701)	GGCTGGGGGACAGAGTCAATCAACACAGACACCCGACCTGGGGCTGCCACCTACAAATCAACCTCTACAAAGCAATCTCCAAACAGCACATCTGGAGG	
hu.31	(701)	GGCTGGGGGACAGAGTCAATCAACACAGACACCCGACCTGGGGCTGCCACCTACAAATCAACCTCTACAAAGCAATCTCCAAACAGCACATCTGGAGG	
hu.32	(701)	GGCTGGGGGACAGAGTCAATCAACACAGACACCCGACCTGGGGCTGCCACCTACAAATCAACCTCTACAAAGCAATCTCCAAACAGCACATCTGGAGG	
AAV9	(801)	801	900
hu.68.VP1	(801)	ATCTTCAAATGACAACGGCTACTTCGGCTACAGACACCCCTGGGGGTATTTTGACTTCAACAGATTCCACTGCCACTTCTACACAGTACTGGCAGCGA	
hu.31	(801)	ATCTTCAAATGACAACGGCTACTTCGGCTACAGACACCCCTGGGGGTATTTTGACTTCAACAGATTCCACTGCCACTTCTACACAGTACTGGCAGCGA	
hu.32	(801)	ATCTTCAAATGACAACGGCTACTTCGGCTACAGACACCCCTGGGGGTATTTTGACTTCAACAGATTCCACTGCCACTTCTACACAGTACTGGCAGCGA	
AAV9	(901)	901	1000
hu.68.VP1	(901)	CTCATCAACAACAACCTGGGGATTCGGGCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAGAGGTTACGGACAACAATGGAGTCAAGACCA	
hu.31	(901)	CTCATCAACAACAACCTGGGGATTCGGGCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAGAGGTTACGGACAACAATGGAGTCAAGACCA	
hu.32	(901)	CTCATCAACAACAACCTGGGGATTCGGGCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAGAGGTTACGGACAACAATGGAGTCAAGACCA	

FIG 11B

AAV9 hu.68.VP1 hu.31 hu.32	(901) (901) (901) (901)	901	CTCATCAACAACACTGGGATTCGGGCTAAGGACTCAACTCAAGCTCTTCAACATTCAGGTCACAGAGGTTACGGACAACAATGGAGTCAAGACCA	1000
			CTCATCAACAACACTGGGATTCGGGCTAAGGACTCAACTCAAGCTCTTCAACATTCAGGTCACAGAGGTTACGGACAACAATGGAGTCAAGACCA	
			CTCATCAACAACACTGGGATTCGGGCTAAGGACTCAACTCAAGCTCTTCAACATTCAGGTCACAGAGGTTACGGACAACAATGGAGTCAAGACCA	
			CTCATCAACAACACTGGGATTCGGGCTAAGGACTCAACTCAAGCTCTTCAACATTCAGGTCACAGAGGTTACGGACAACAATGGAGTCAAGACCA	
AAV9 hu.68.VP1 hu.31 hu.32	(1001) (1001) (1001) (1001)	1001	TGCGCAATAACCTTACCAGCACGGTCCAGGTCCTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCAGGAGGGCTGCCTCCCGCCGTT	1100
			TGCGCAATAACCTTACCAGCACGGTCCAGGTCCTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCAGGAGGGCTGCCTCCCGCCGTT	
			TGCGCAATAACCTTACCAGCACGGTCCAGGTCCTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCAGGAGGGCTGCCTCCCGCCGTT	
			TGCGCAATAACCTTACCAGCACGGTCCAGGTCCTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCAGGAGGGCTGCCTCCCGCCGTT	
AAV9 hu.68.VP1 hu.31 hu.32	(1101) (1101) (1101) (1101)	1101	CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTGACGGCTTAATGATGGAAGCAAGCGGTGGGTGCTGTCCTTTTACTGCCTGGAATATTTT	1200
			CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTGACGGCTTAATGATGGAAGCAAGCGGTGGGTGCTGTCCTTTTACTGCCTGGAATATTTT	
			CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTGACGGCTTAATGATGGAAGCAAGCGGTGGGTGCTGTCCTTTTACTGCCTGGAATATTTT	
			CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTGACGGCTTAATGATGGAAGCAAGCGGTGGGTGCTGTCCTTTTACTGCCTGGAATATTTT	
AAV9 hu.68.VP1 hu.31 hu.32	(1201) (1201) (1201) (1201)	1201	CCGTGCGCAATGCTAAGAACGGGTAAACAACCTCCAGTTCAGCTACGAGTTGAGAACGTACCTTCCATAGCAGCTACGCTCAGAGCCAAAGCCTGGACC	1300
			CCGTGCGCAATGCTAAGAACGGGTAAACAACCTCCAGTTCAGCTACGAGTTGAGAACGTACCTTCCATAGCAGCTACGCTCAGAGCCAAAGCCTGGACC	
			CCGTGCGCAATGCTAAGAACGGGTAAACAACCTCCAGTTCAGCTACGAGTTGAGAACGTACCTTCCATAGCAGCTACGCTCAGAGCCAAAGCCTGGACC	
			CCGTGCGCAATGCTAAGAACGGGTAAACAACCTCCAGTTCAGCTACGAGTTGAGAACGTACCTTCCATAGCAGCTACGCTCAGAGCCAAAGCCTGGACC	
AAV9 hu.68.VP1 hu.31 hu.32	(1301) (1301) (1301) (1301)	1301	GACTAATGAATCCACTCATCGACCAATACTTGACTATCTCTCAAGACTATTACGGTTCTGGACAGAAATCAACAACGCTAAAAATTCAGTGTGGCCGG	1400
			GACTAATGAATCCACTCATCGACCAATACTTGACTATCTCTCAAGACTATTACGGTTCTGGACAGAAATCAACAACGCTAAAAATTCAGTGTGGCCGG	
			GACTAATGAATCCACTCATCGACCAATACTTGACTATCTCTCAAGACTATTACGGTTCTGGACAGAAATCAACAACGCTAAAAATTCAGTGTGGCCGG	
			GACTAATGAATCCACTCATCGACCAATACTTGACTATCTCTCAAGACTATTACGGTTCTGGACAGAAATCAACAACGCTAAAAATTCAGTGTGGCCGG	

FIG 11C

[illegible]

FLIGHT

AAV9 hu.68.VP1 hu.31 hu.32	(1801)	1801	ATAC	1900
			TTCCGGG	
			TATGGTTGGCAGC	
			ACAGATGTGTACCTGCAAGGACCCCAATTTGGGCCAAATTCCTC	
AAV9 hu.68.VP1 hu.31 hu.32	(1901)	1901	ATAC	2000
			TTCCGGG	
			TATGGTTGGCAGC	
			ACAGATGTGTACCTGCAAGGACCCCAATTTGGGCCAAATTCCTC	
AAV9 hu.68.VP1 hu.31 hu.32	(2001)	2001	ATAC	2100
			TTCCGGG	
			TATGGTTGGCAGC	
			ACAGATGTGTACCTGCAAGGACCCCAATTTGGGCCAAATTCCTC	
AAV9 hu.68.VP1 hu.31 hu.32	(2101)	2101	ATAC	2200
			TTCCGGG	
			TATGGTTGGCAGC	
			ACAGATGTGTACCTGCAAGGACCCCAATTTGGGCCAAATTCCTC	
AAV9 hu.68.VP1 hu.31 hu.32	(2201)	2201	ATAC	2300
			TTCCGGG	
			TATGGTTGGCAGC	
			ACAGATGTGTACCTGCAAGGACCCCAATTTGGGCCAAATTCCTC	

FIG 11E

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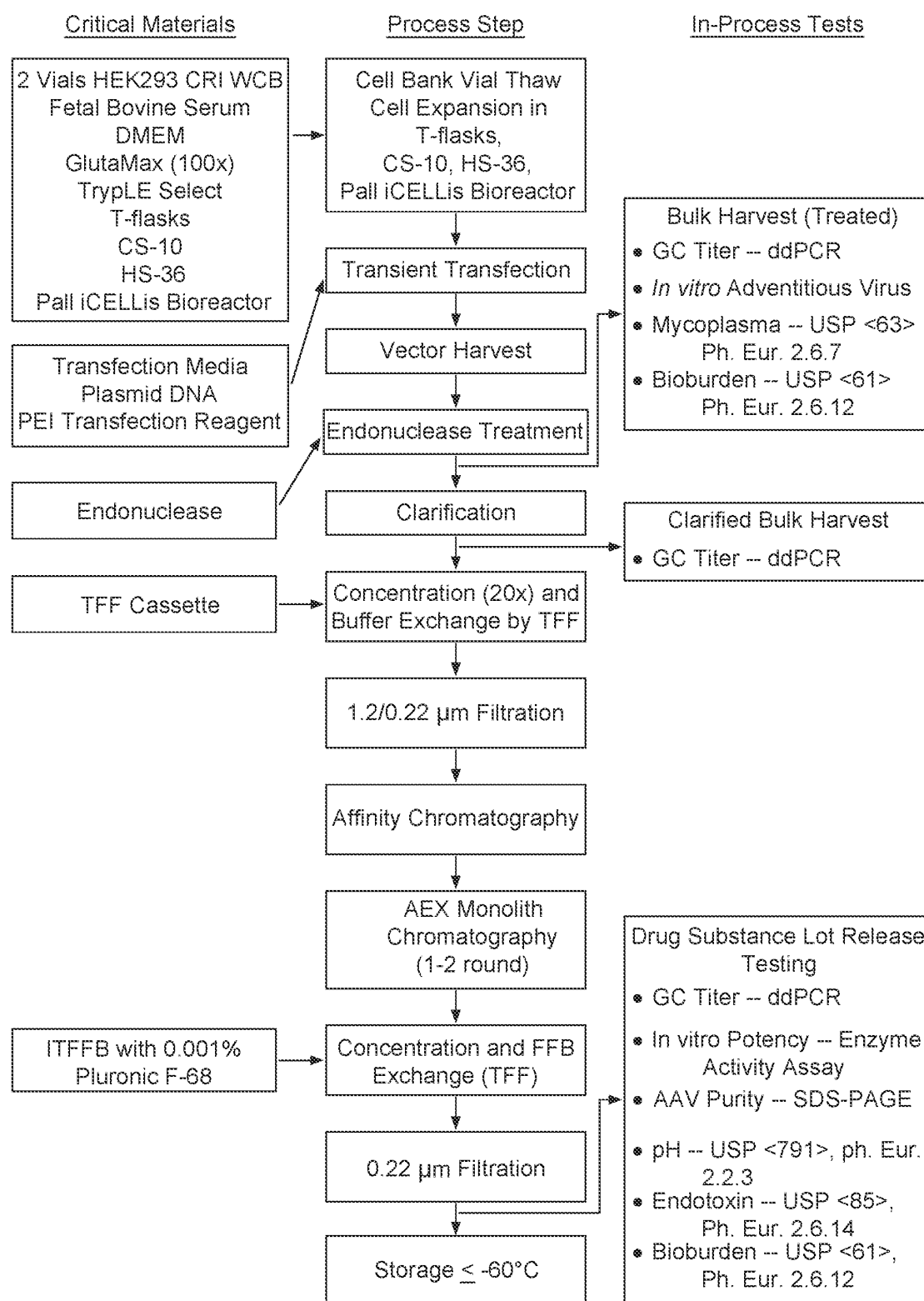


FIG. 12A

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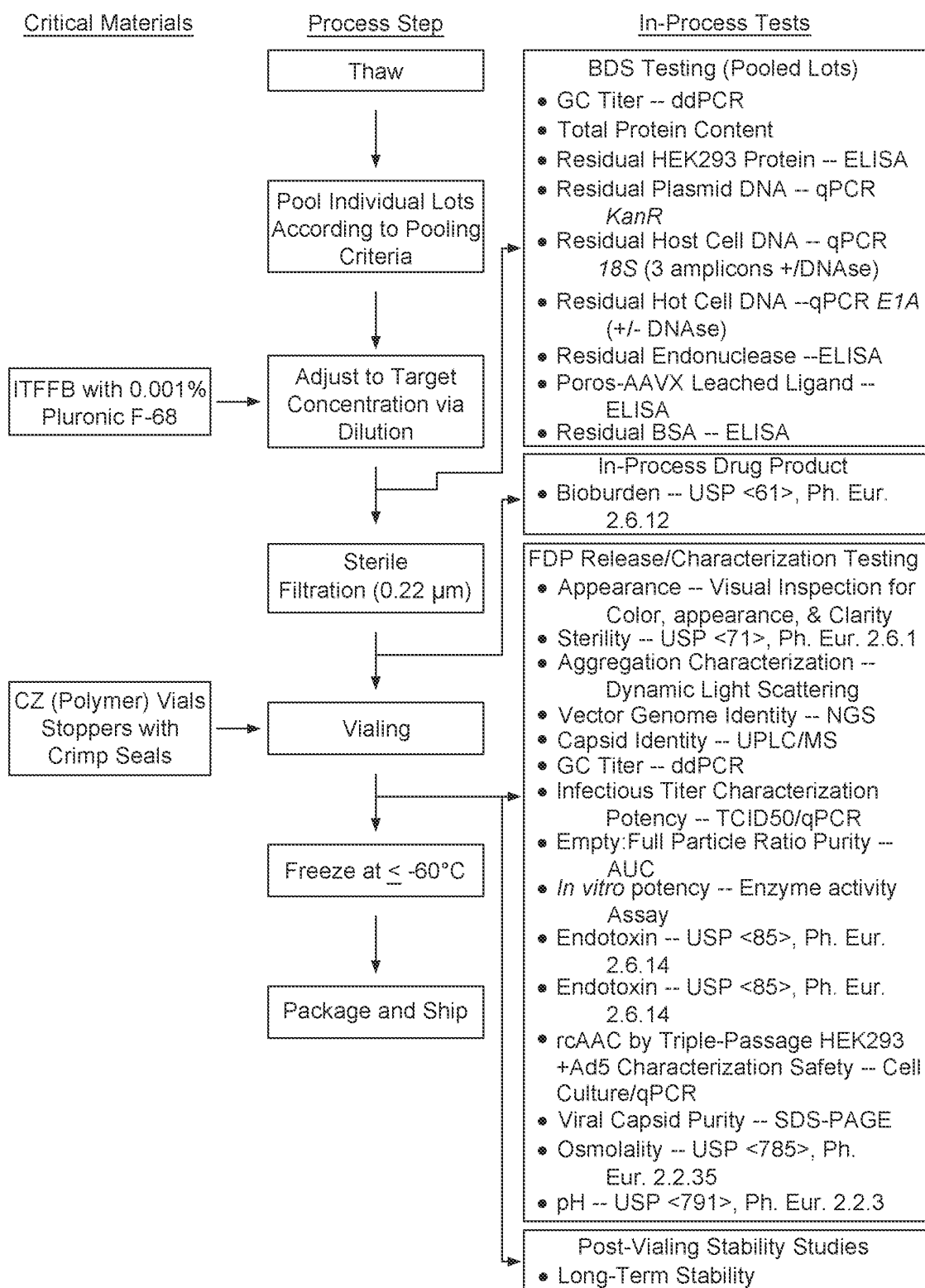


FIG. 12B

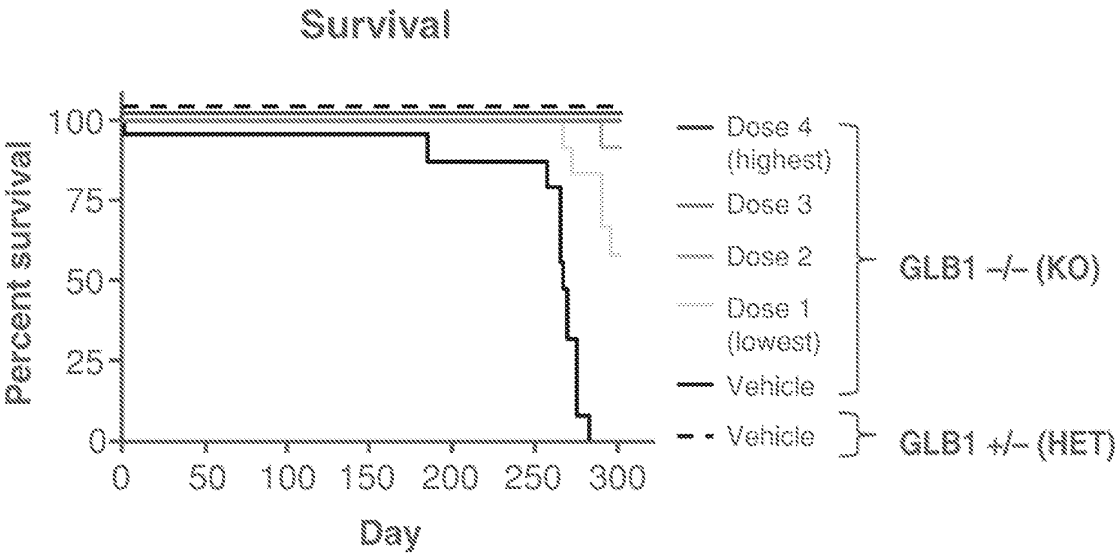


FIG 13

FIG 14A

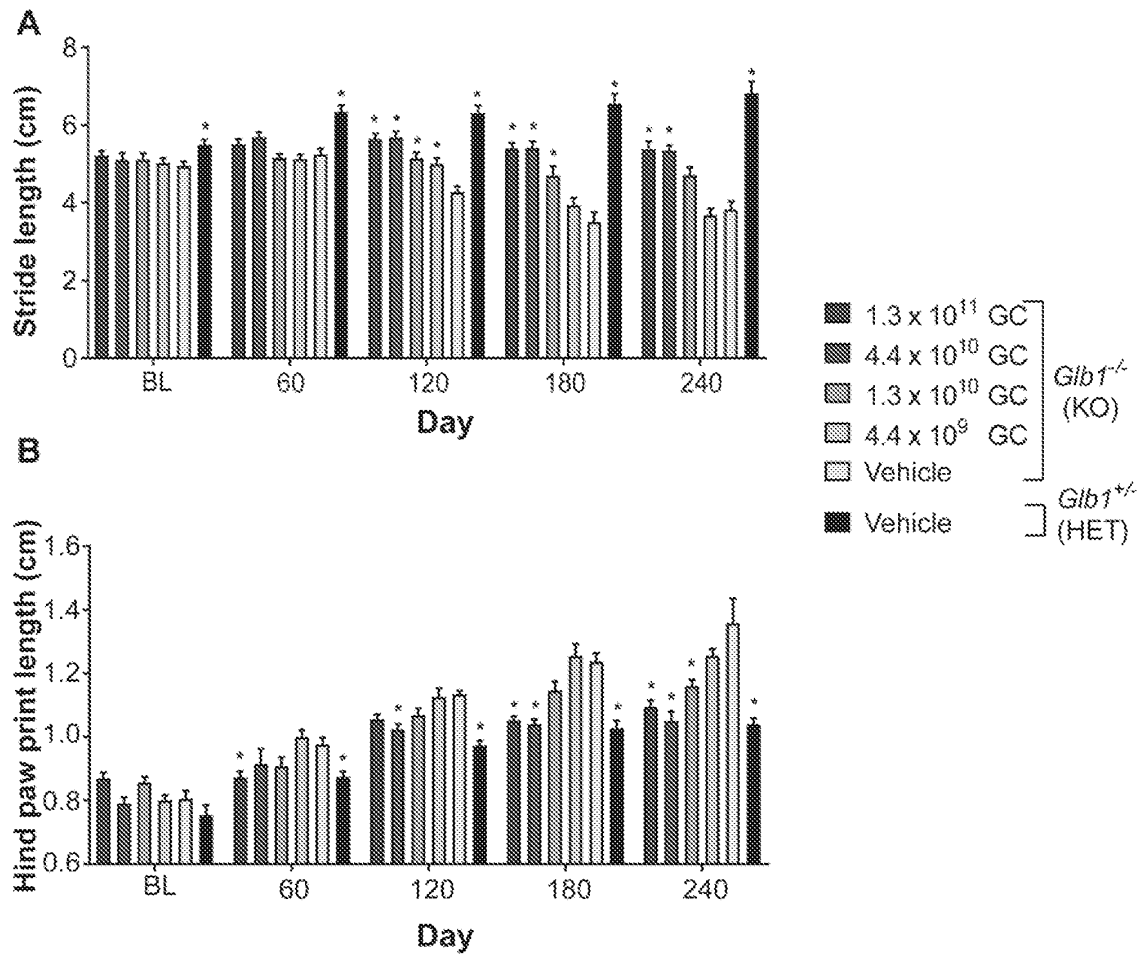


FIG 14B

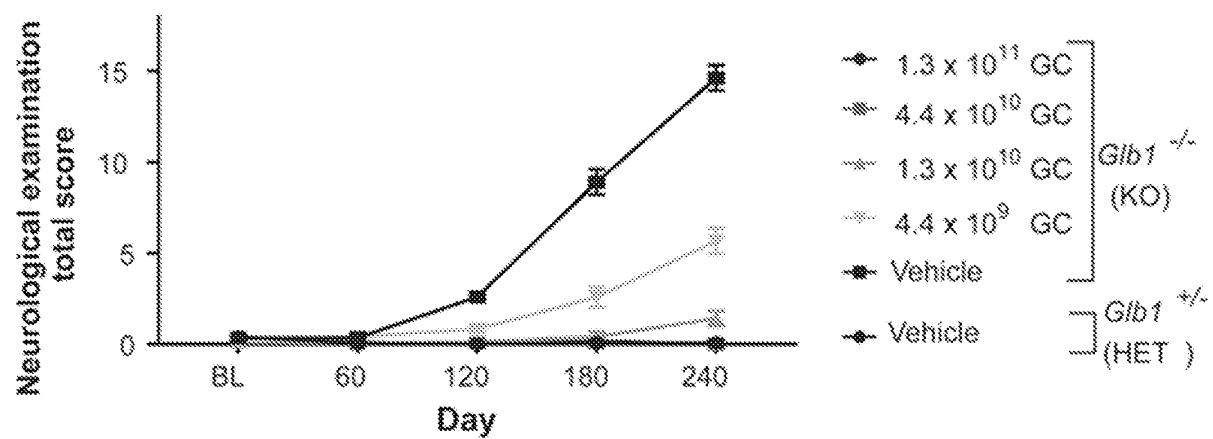


FIG 14C

FIG 15A

Day 1
(1 month old)

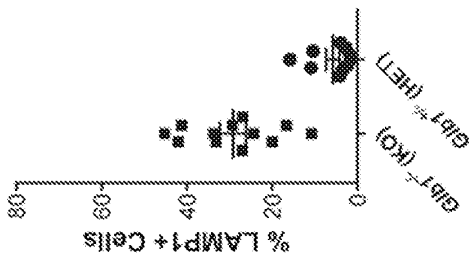


FIG 15B

Day 150
(6 months old)

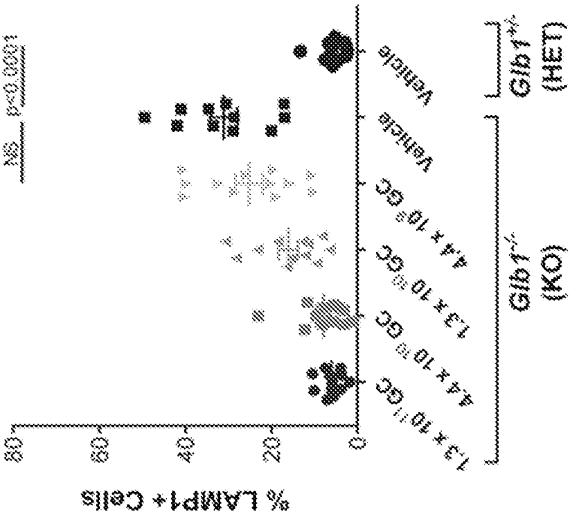


FIG 15C

Day 300
(11 months old)

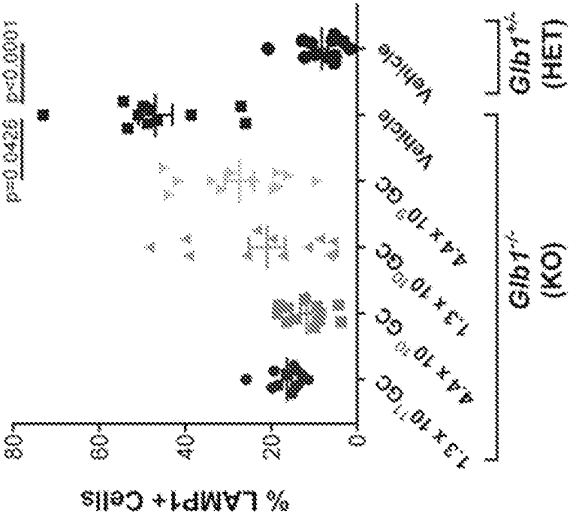


FIG 16A

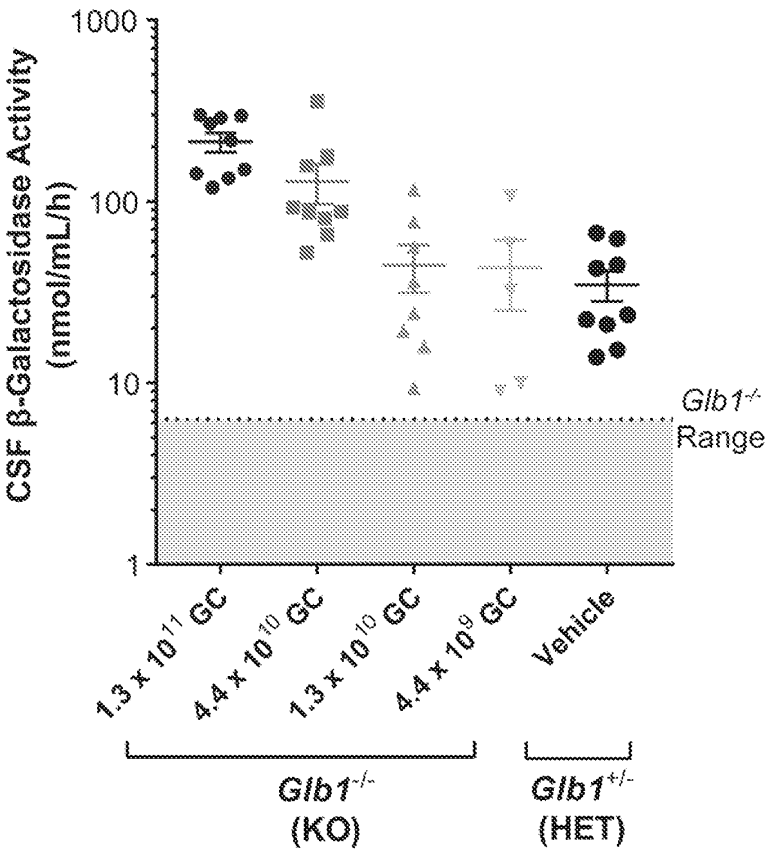
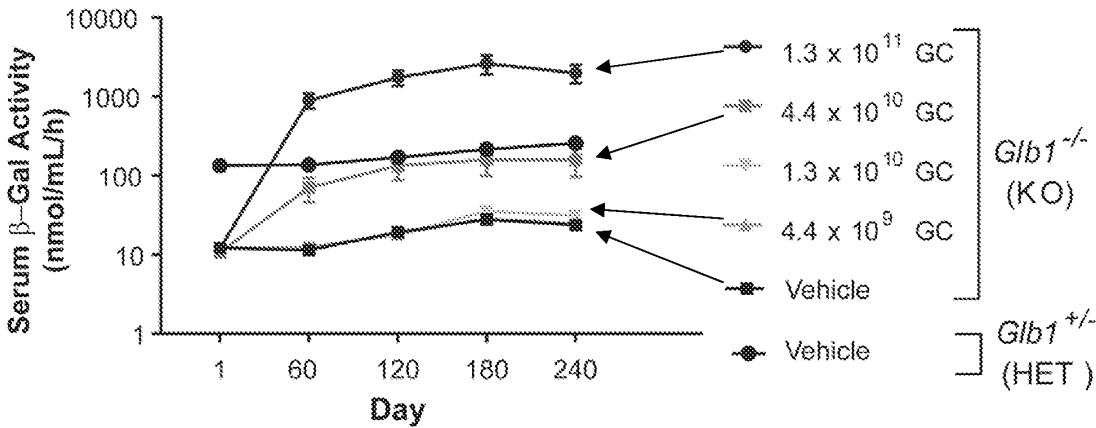


FIG 16B

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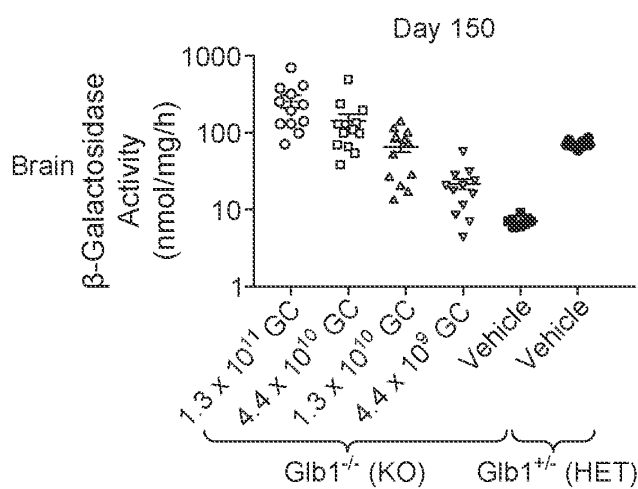


FIG. 17A

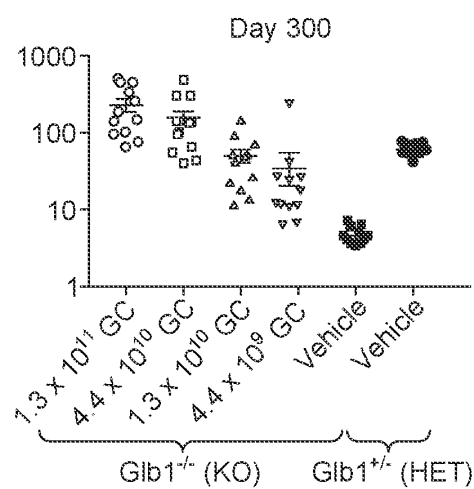


FIG. 17B

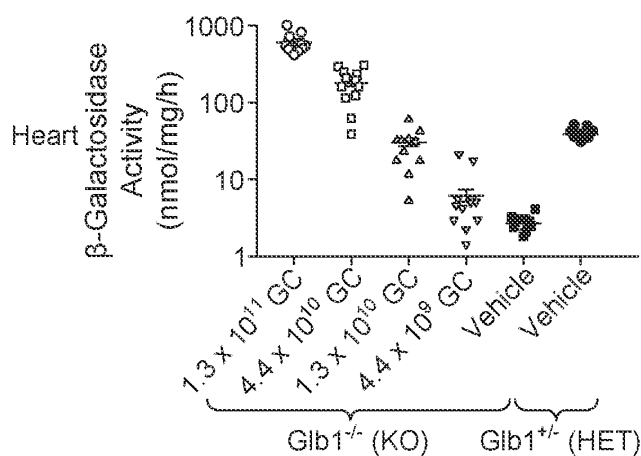


FIG. 17C

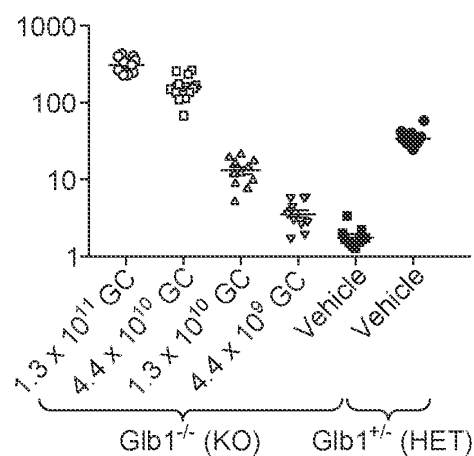


FIG. 17D

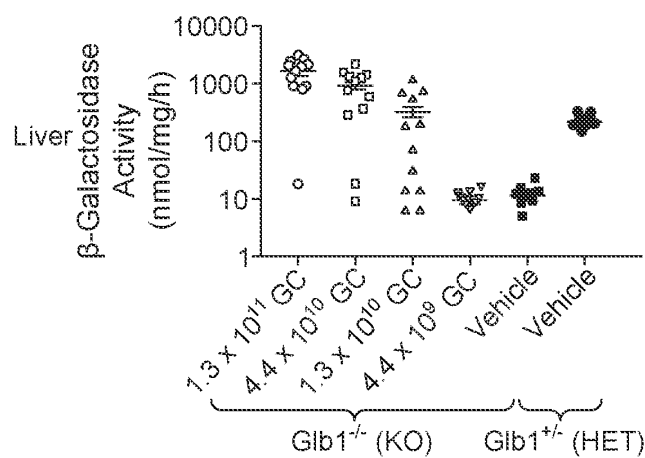


FIG. 17E

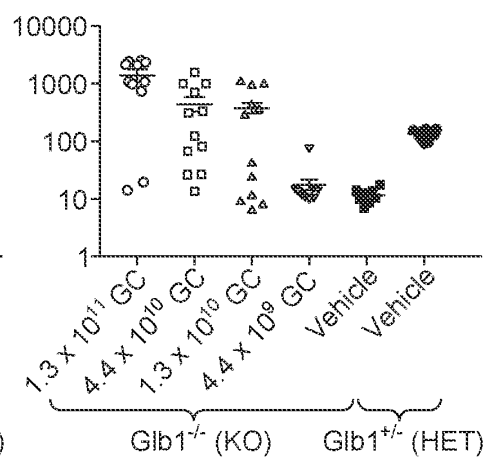


FIG. 17F

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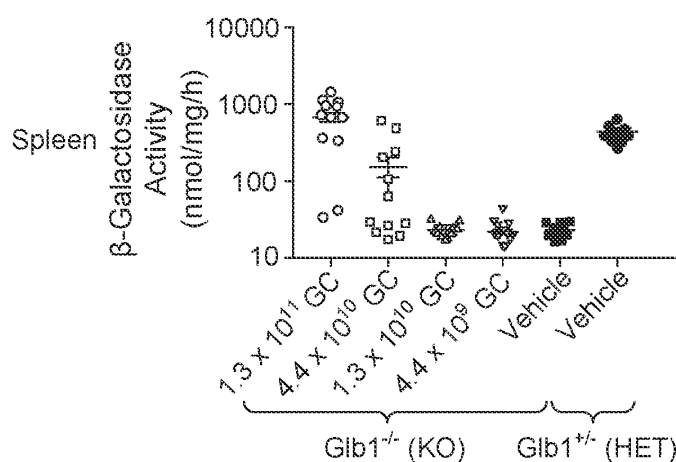


FIG. 17G

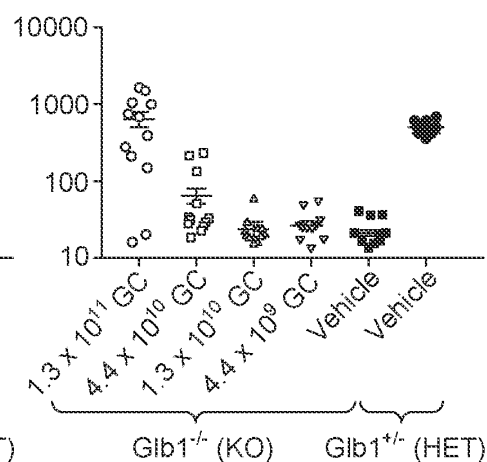


FIG. 17H

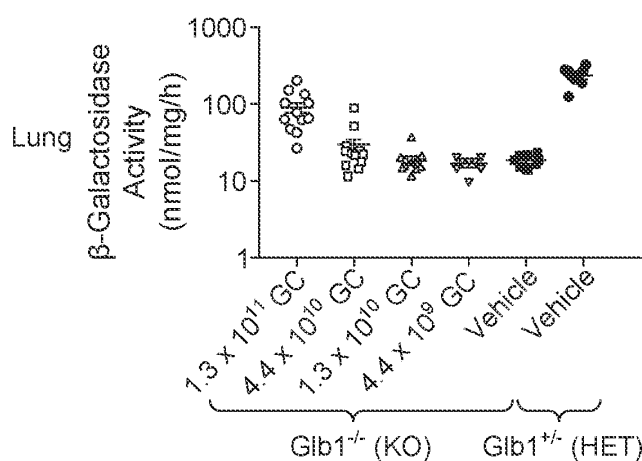


FIG. 17I

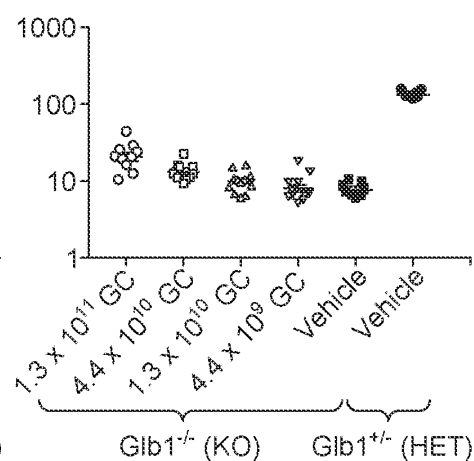


FIG. 17J

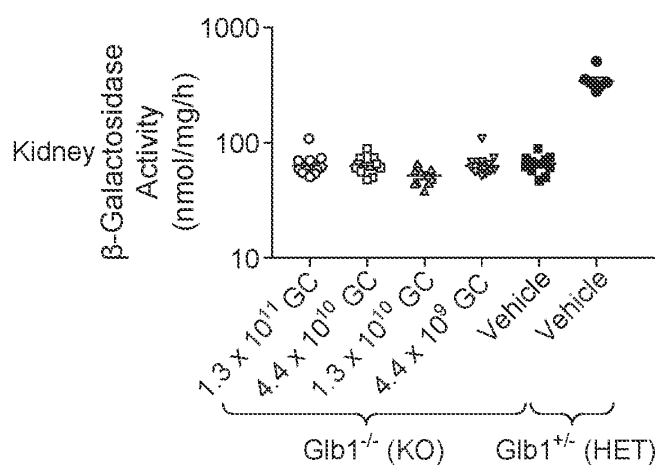


FIG. 17K

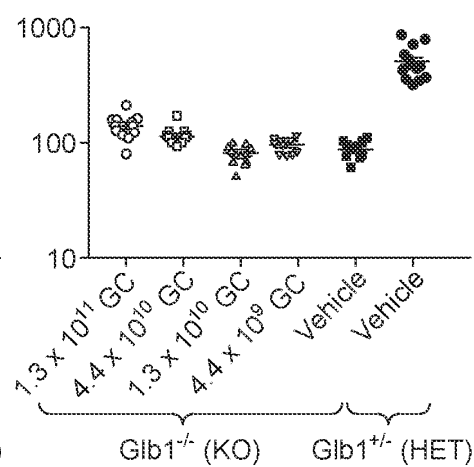


FIG. 17L

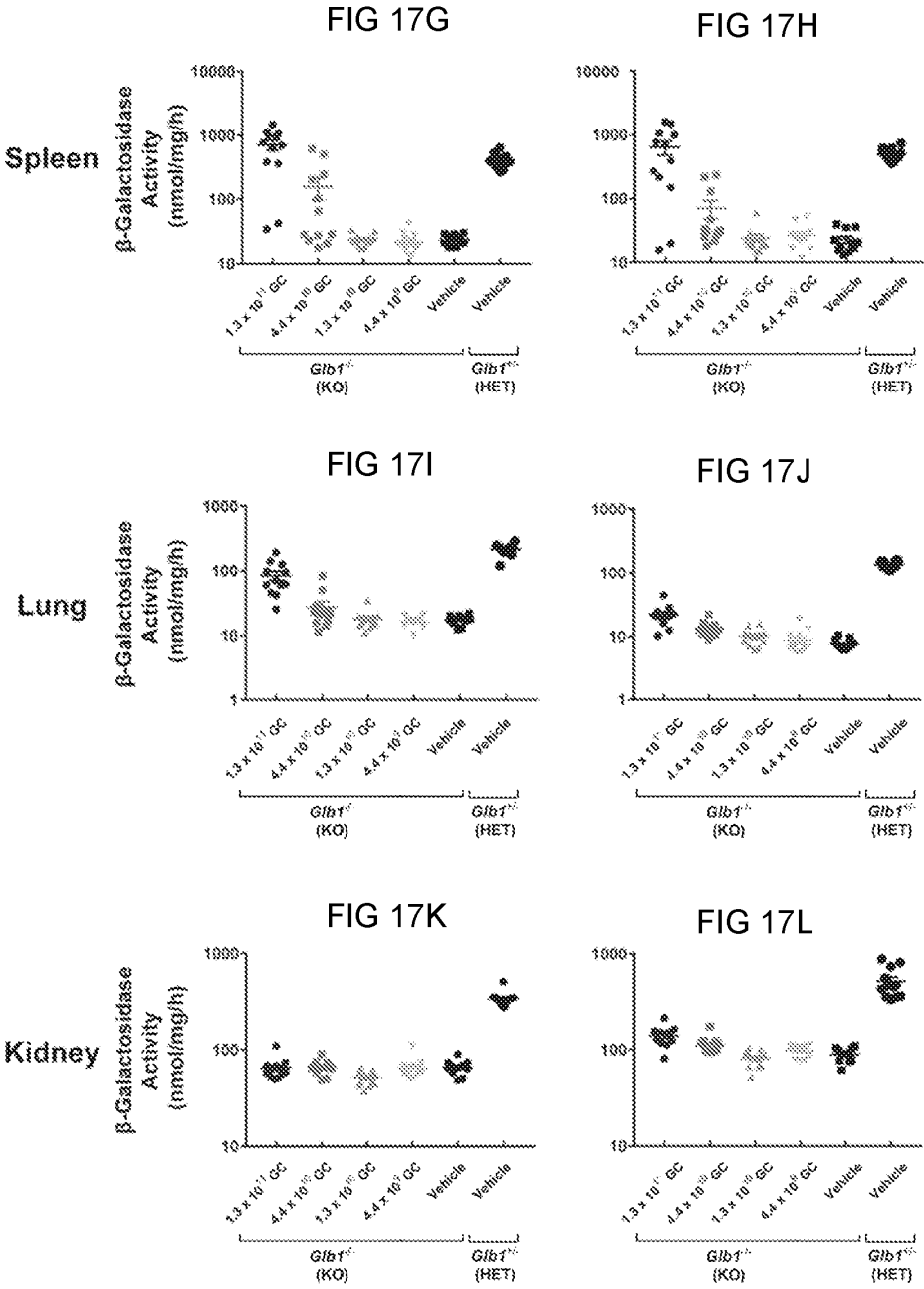


FIG 18A

ORG degeneration D120

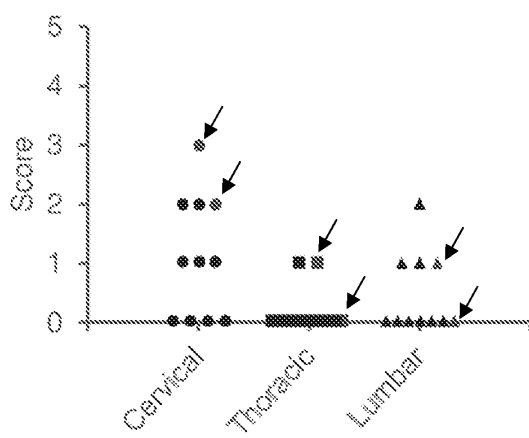


FIG 18B

SC axonopathy 0120

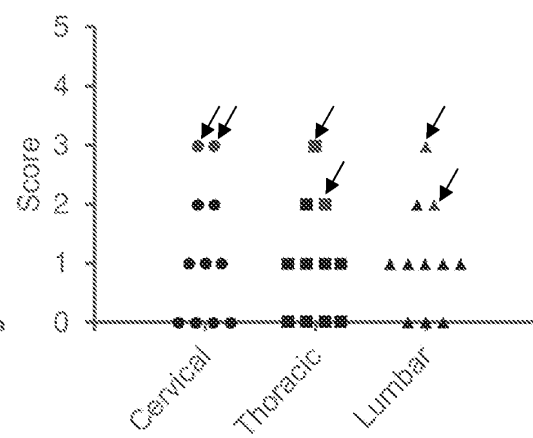


FIG 19A

Median nerve axonopathy D120

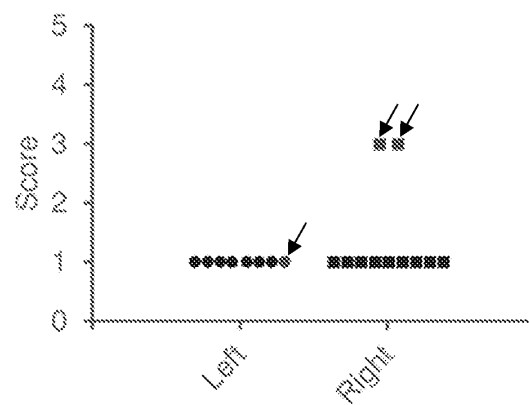
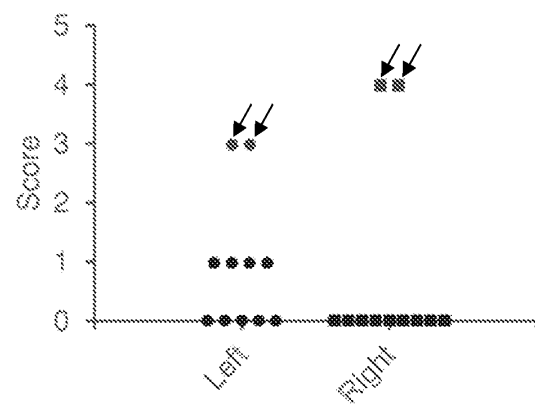


FIG 19B

Median nerve periaxonal fibrosis D120



Median Sensory Nerve Conduction Studies

Vehicle Dose 1 (lowest) Dose 2 Dose 3 (highest)

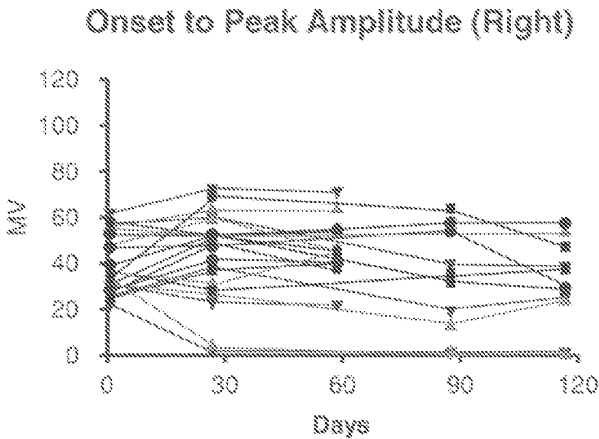


FIG 20A

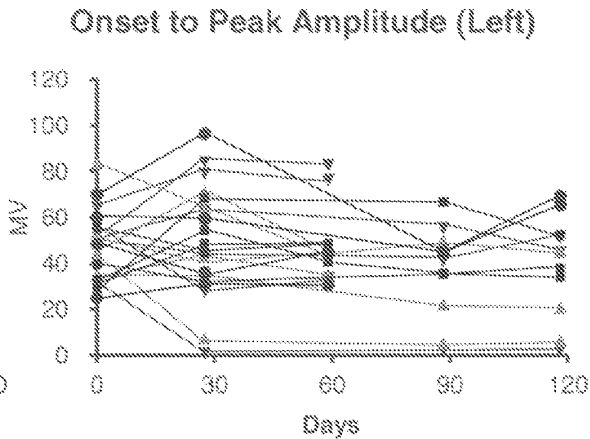


FIG 20B

FIG 21A

SNAP Amplitude

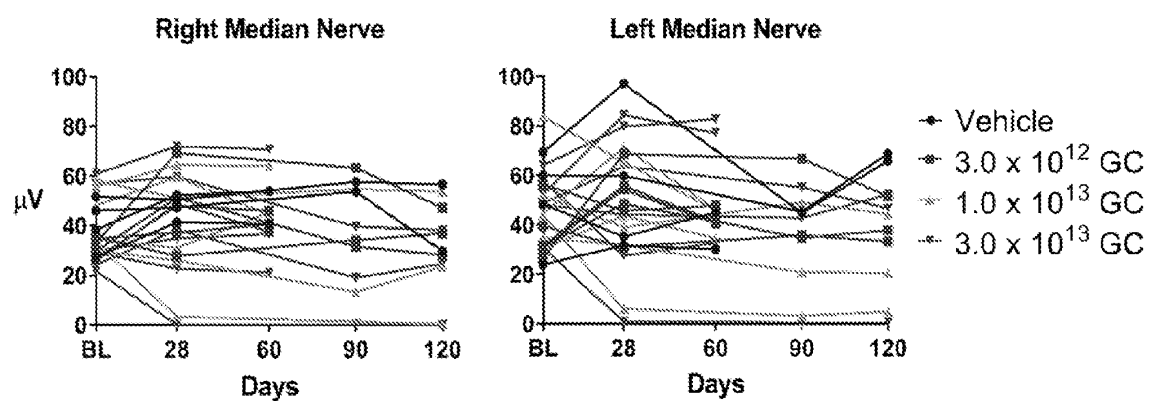


FIG 21B

Nerve Conduction Velocity

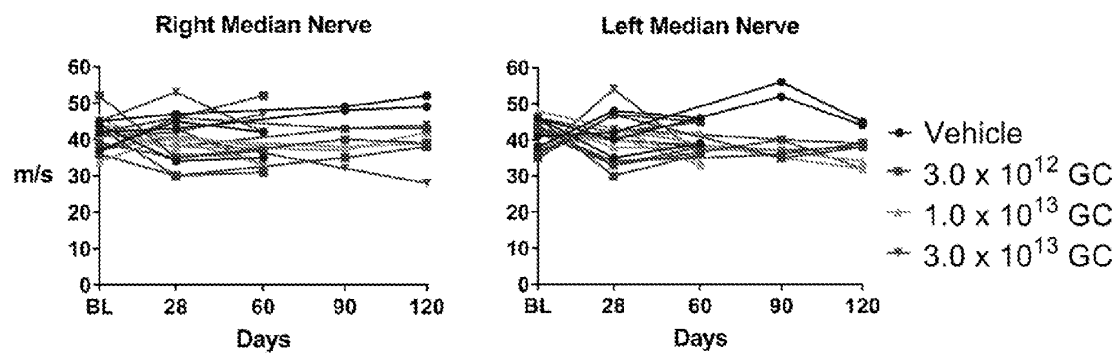


FIG 22A

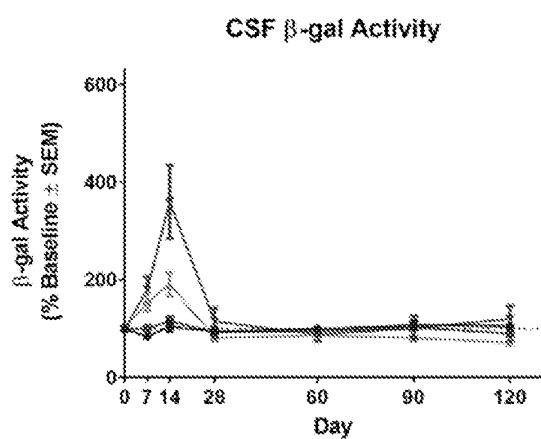


FIG 22B

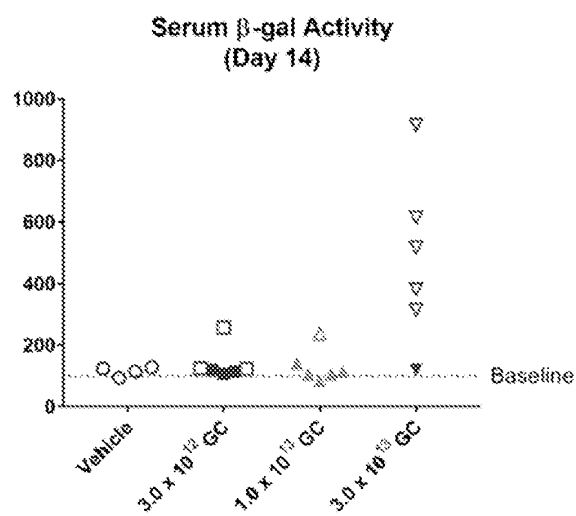
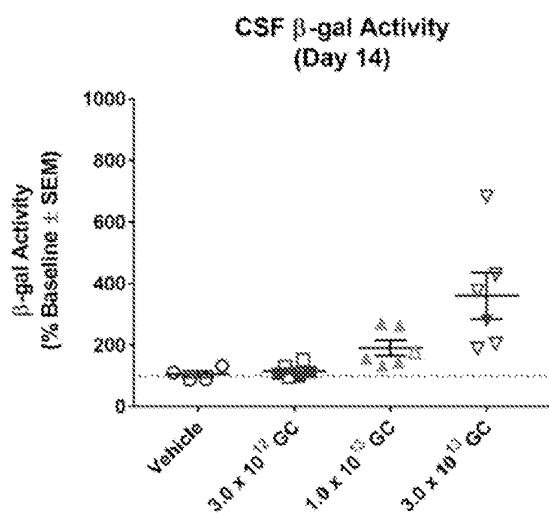
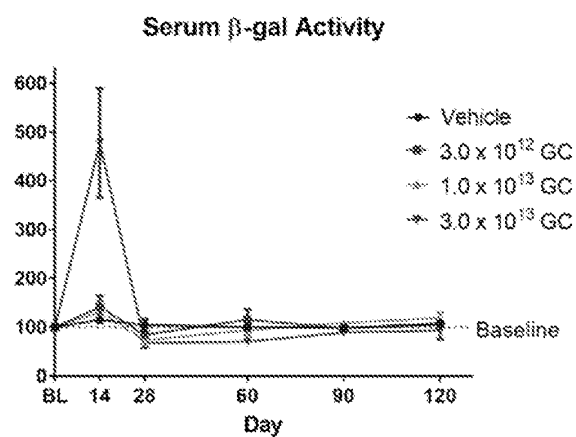


FIG 22C

FIG 22D

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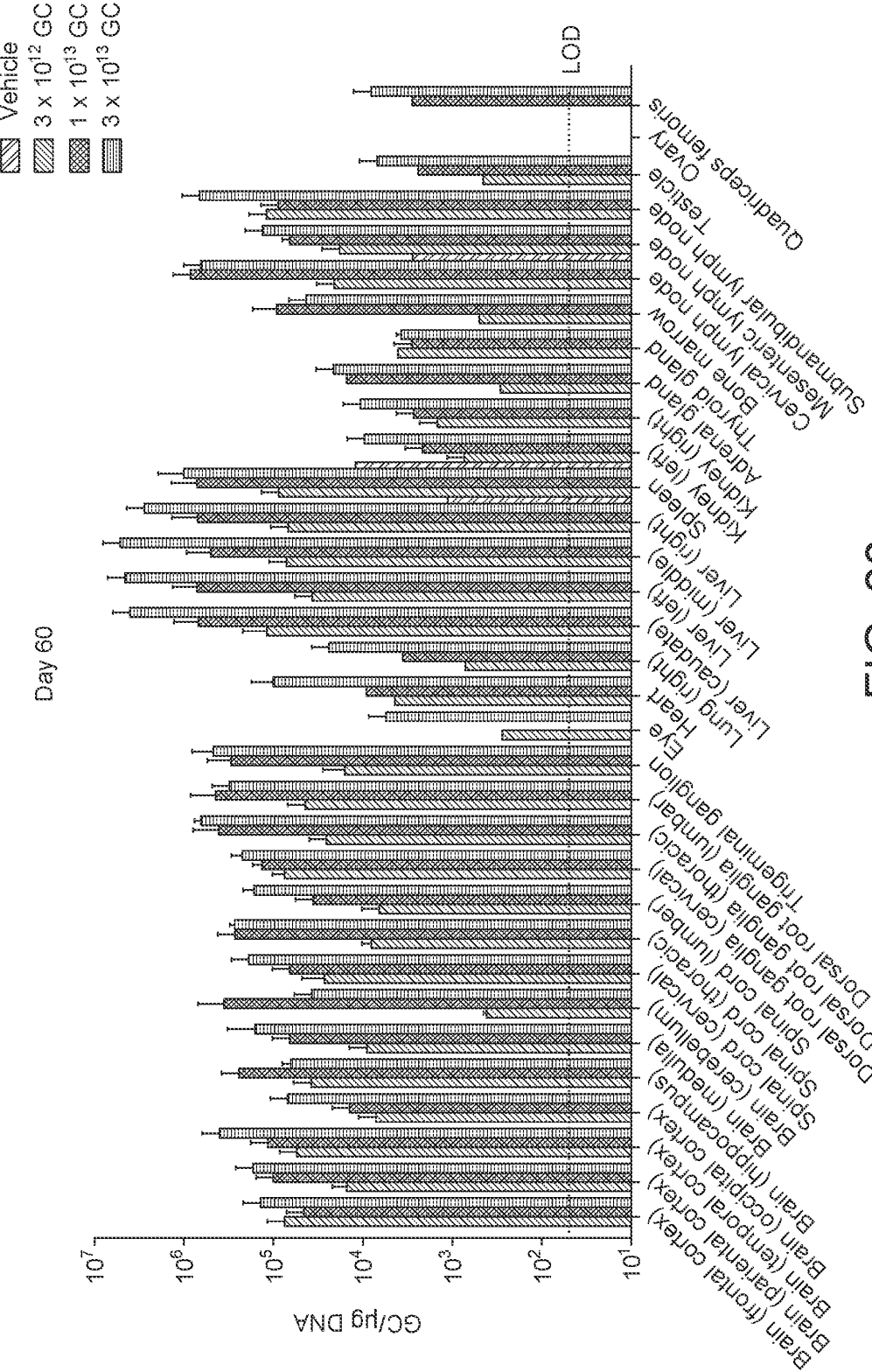


FIG. 23

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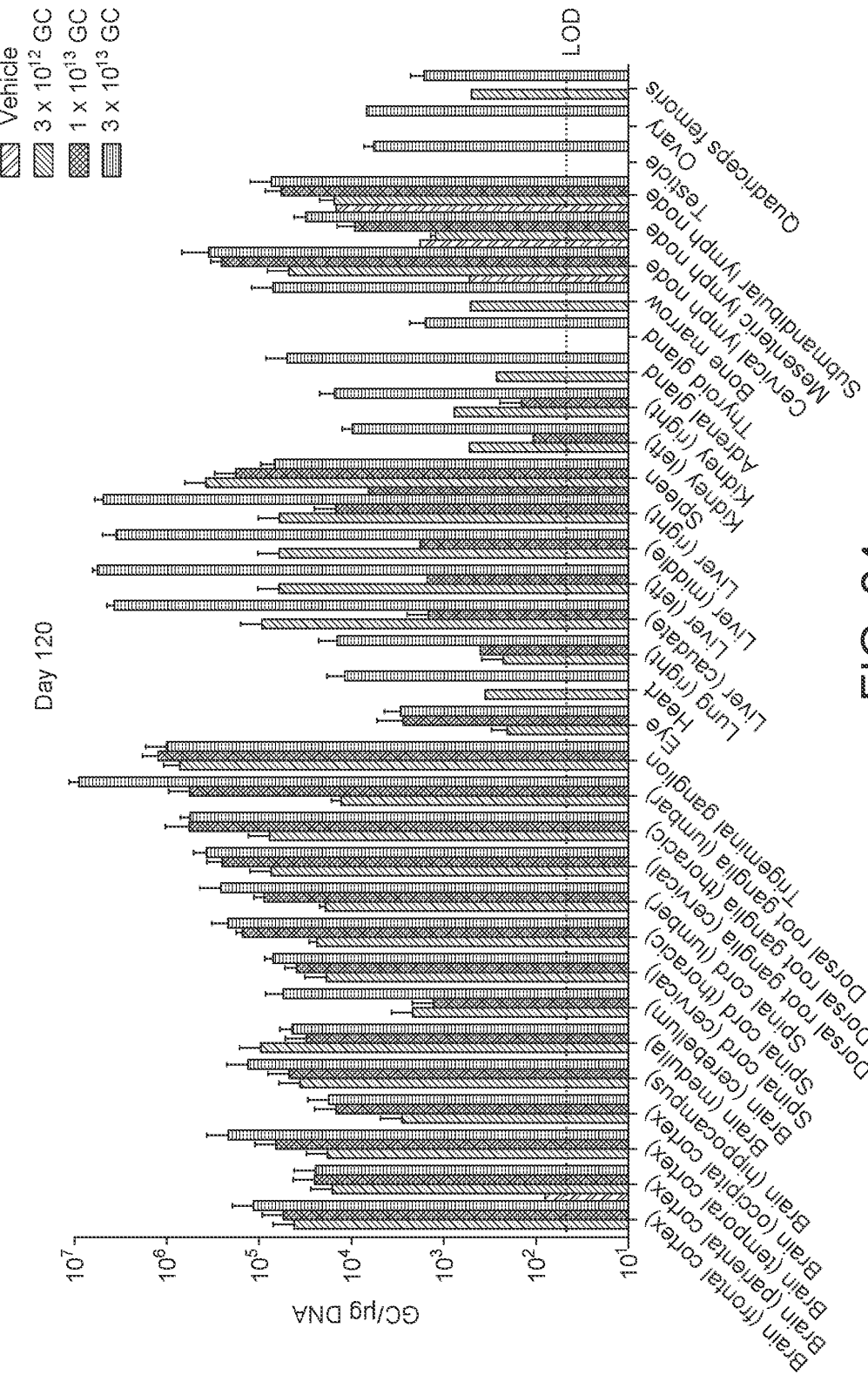


FIG. 24