

FIG 1A

HUMAN_FRATAXIN	60	ATGTGGACTCTGGGGCGCGCGCAGTAGCGGCTCTCTGGCGTCACCCAGCCAGCCCGCAG
HUMAN_FXN_CODON-OPTIMIZED	60	ATGTGGACACTTGGCGCGCAGAGCTGTGTGGCTGTCTTCTCCATCTCCCGCTCAA
		***** ** ** ** **
HUMAN_FRATAXIN	120	GCCAGACCTCACCCGGGTCCGGCGCGGAGAGTTGGCCCACTCTGCGCGCGCGGT
HUMAN_FXN_CODON-OPTIMIZED	120	GCCAGACACTGACACAGAGTGCCTAGACCTGCTGACTGGCCCTCTGTGTGGCAGAAGA
		***** ** ** ** * * * * *
HUMAN_FRATAXIN	180	GCCTGGCAACCGACATCGATGGACCTGCACGCCCGCGCGCAAGTTCGAACCAACGT
HUMAN_FXN_CODON-OPTIMIZED	180	GCCTGAGAAACCGACATCGACGCCACATGCACACTAGAAAGGCCAGCAGCAATCAGCGG
		***** * ***** ** ** * * * * *
HUMAN_FRATAXIN	240	GGCTCAACACAGATTTGGAATCTCAAAAGCAGAGTCTCTATTTGATCAATTTGAGGAAA
HUMAN_FXN_CODON-OPTIMIZED	240	GGCTGAATCAGATCTGGAACGTGAAGAAACAGAGCTGTACCTGATGAACCTGAGAAG
		***** ** ***** ** ** * * * * *
HUMAN_FRATAXIN	300	TCGTGAACTTTGGGCCACCCAGGCTCTCTAGATGAGACCACTATCAAAAGACTAGCAGAG
HUMAN_FXN_CODON-OPTIMIZED	300	ACGGCACCTTGGGACACCCCTGGAAGCCTGGACGAGACAACTAGAGAGACTGGCGGAG
		** ** ***** ** ** * * * * *
HUMAN_FRATAXIN	360	GAAACGCTGGACTCTTTAGCAGAGTTTGTGAAGACCTTGCACACAAGCCATACACGTTT
HUMAN_FXN_CODON-OPTIMIZED	360	GAAACCTTGGATTCCTTGGCGGAGTTCTTGGAGGACCTGGCCGATTAAGCCCTACACCTTC
		***** ***** ** * * * * *
HUMAN_FRATAXIN	420	GAGCACTATGATGTCTCCTTTGGAGTGGTGTCTTAACCTGTCAAACTGGGTGGAGATCTA
HUMAN_FXN_CODON-OPTIMIZED	420	GAGGATTAGCAGGTGTCTTTGGACGGCGGTGTGACAGTGAAGTCTGGCGGAGATCTG
		***** ** ** ***** ** ** * * * * *
HUMAN_FRATAXIN	480	GGAACTATGTGATCAACAAGCAGACGGCCAAACAGCAAAATCTGSGCTATCTTCTCCATCC
HUMAN_FXN_CODON-OPTIMIZED	480	GGCACTTACGTGATCAACAAGCAGACCCCTAACAAACAGATCTGGCTAGCAGCCCTAGC
		** ***** ***** ** * * * * *

FIG 1B

HUMAN FRATAXIN	AGTGGACCTAAGCGTTATGACTGGACTGGGAAAAAACTGGGTGTACTCCACGACGGCGTG	540
HUMAN__FXN_CODON-OPTIMIZED	AGCGGCCCCCAAGAGATAACGATTGGACCGGGCAAGAACTGGGTGTACAGCCACGACGGCGTG	540
	** ** *	
HUMAN FRATAXIN	TCCCTCCATGAGCTGCTGGCCGCGAGAGCTCAGCTAAAGCCTTAAAAACCAAACTGGACTTG	600
HUMAN__FXN_CODON-OPTIMIZED	TCCCTGCACGAACTGCTGGCTGCCGAACTGACAAAGGCCCTGAAAAACAAGCTGGACCTG	600
	***** ** ** *	
HUMAN FRATAXIN	TCTTCCTTGGCCTATTCCGGAAAAGATGCT	630
HUMAN__FXN_CODON-OPTIMIZED	TCCAGCCTGGCCTACTCTGCAAGGATGCC	630
	** *	

FIG 2A

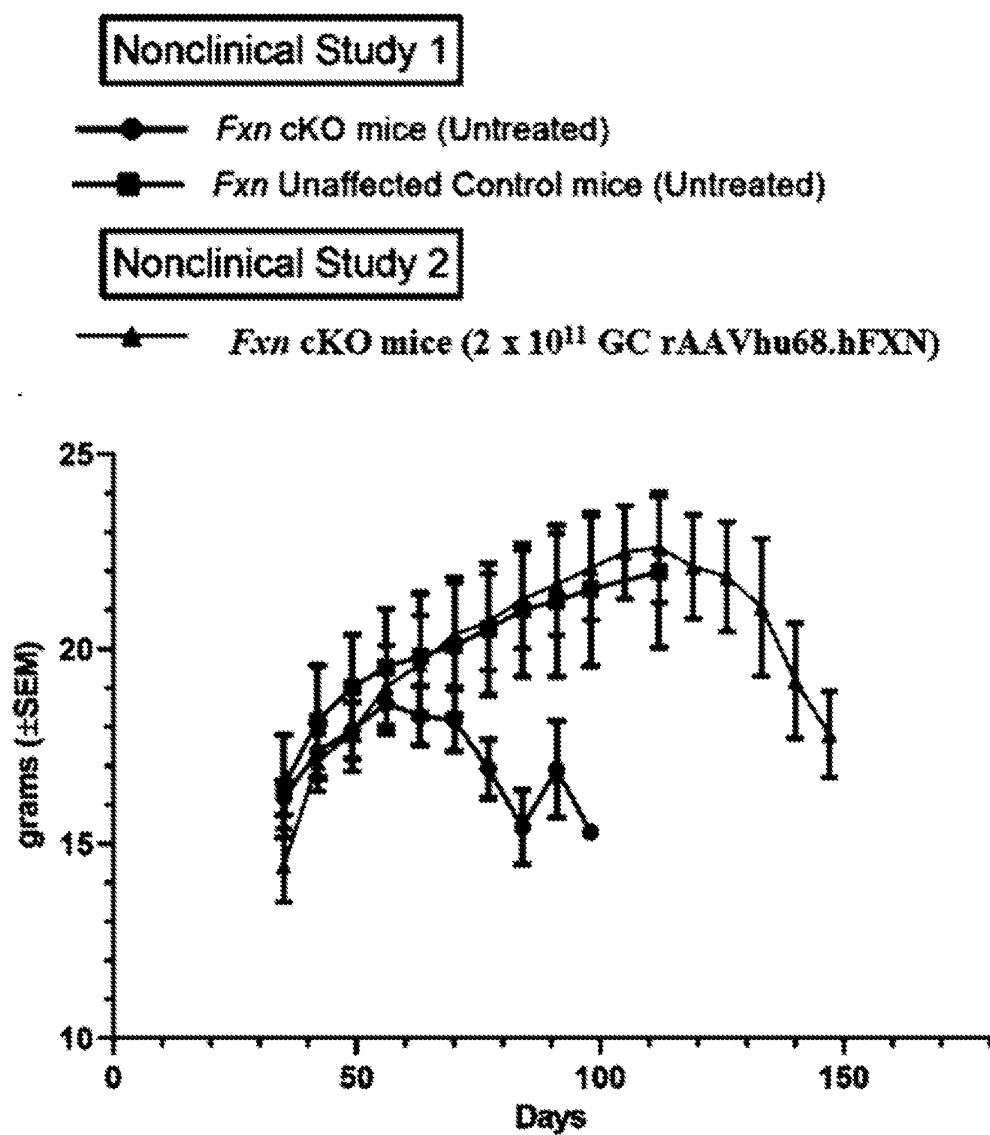


FIG 2B

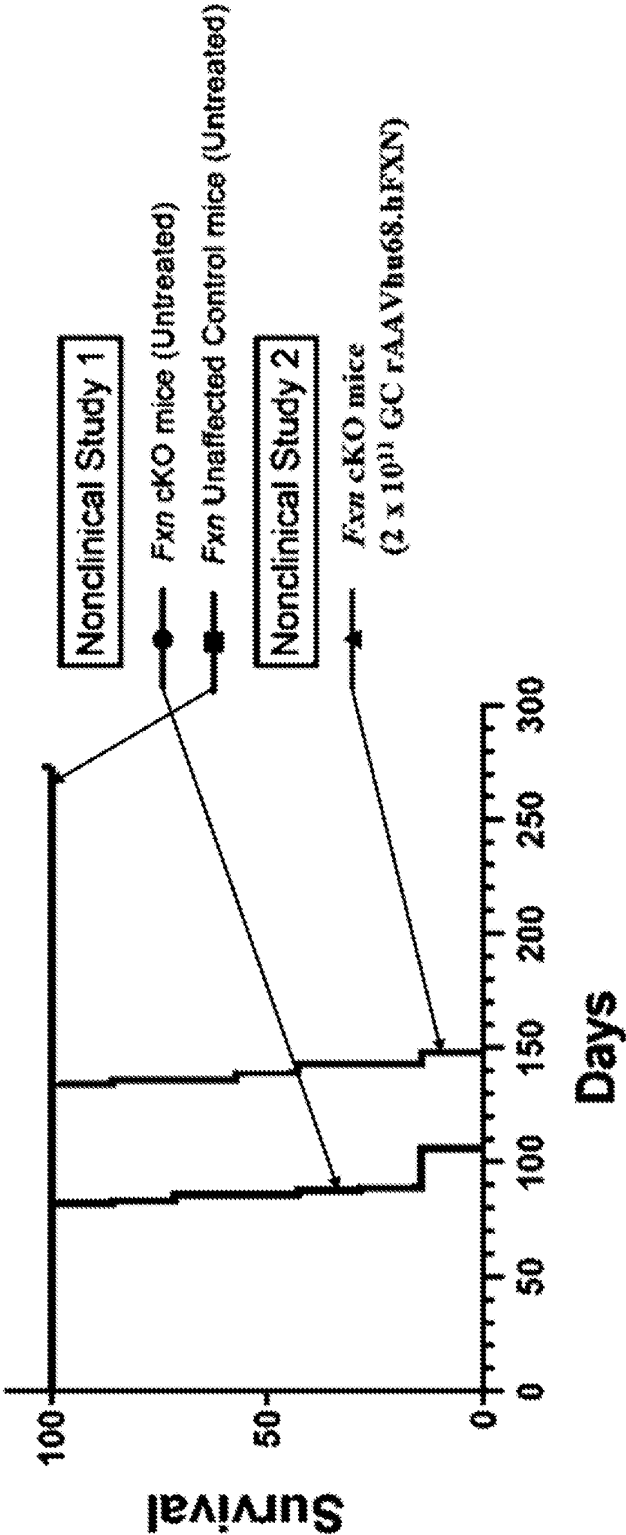


FIG 3

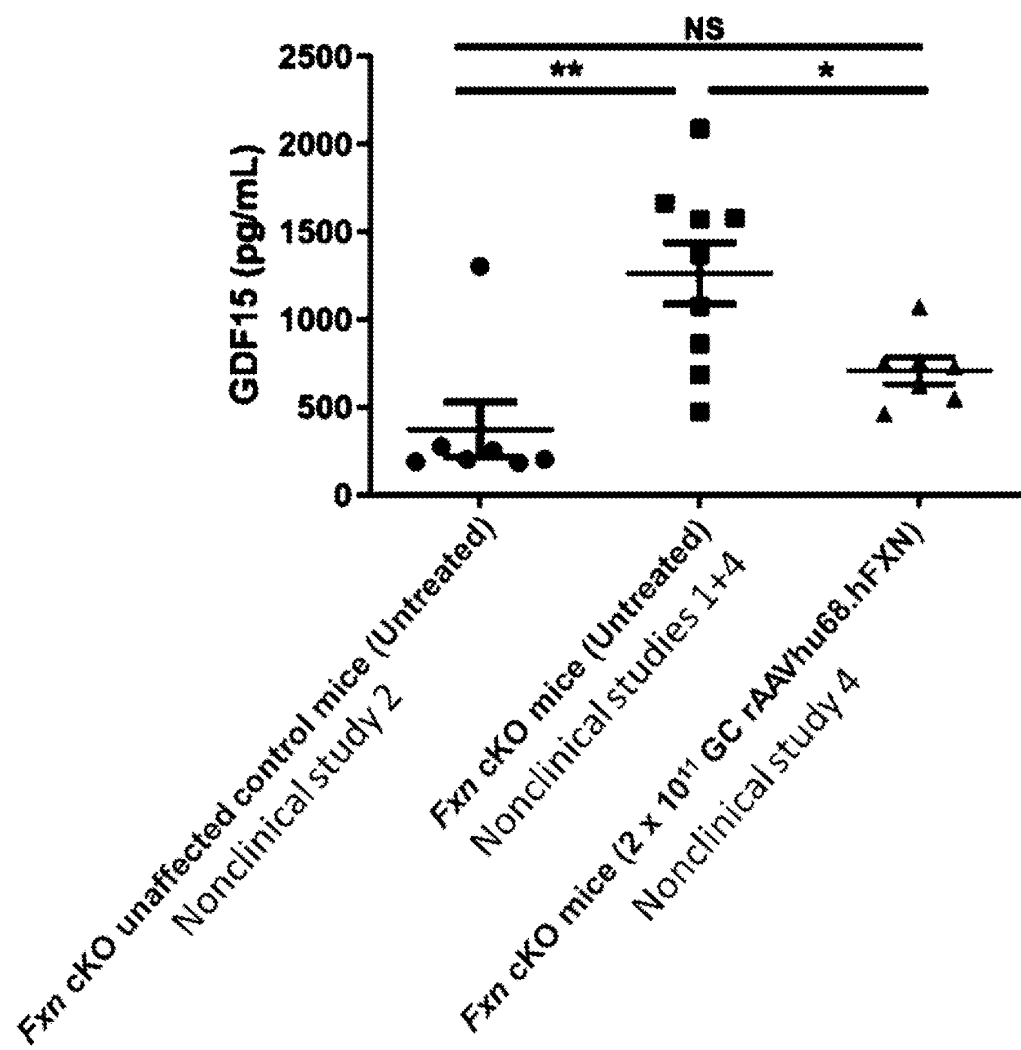
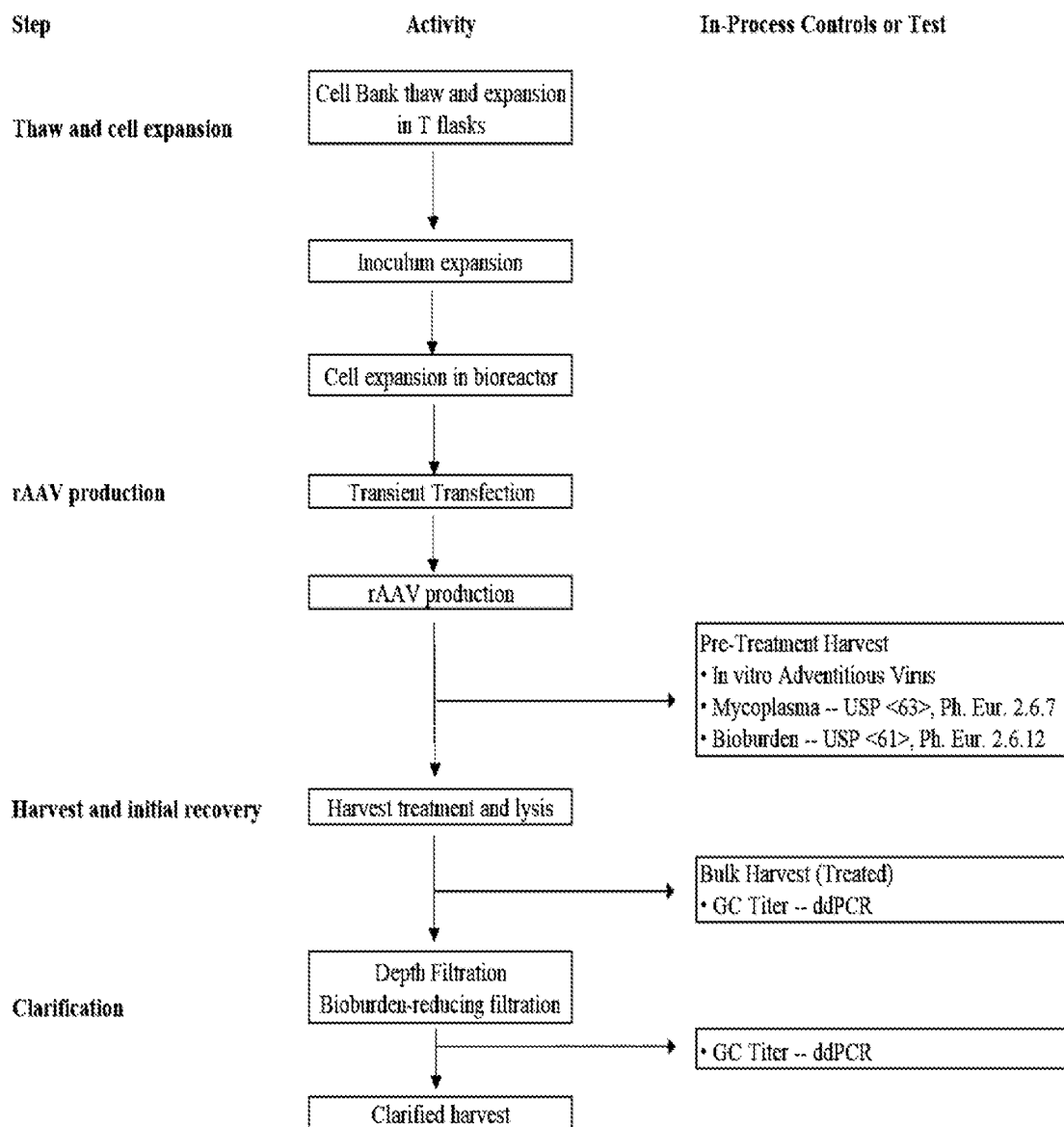
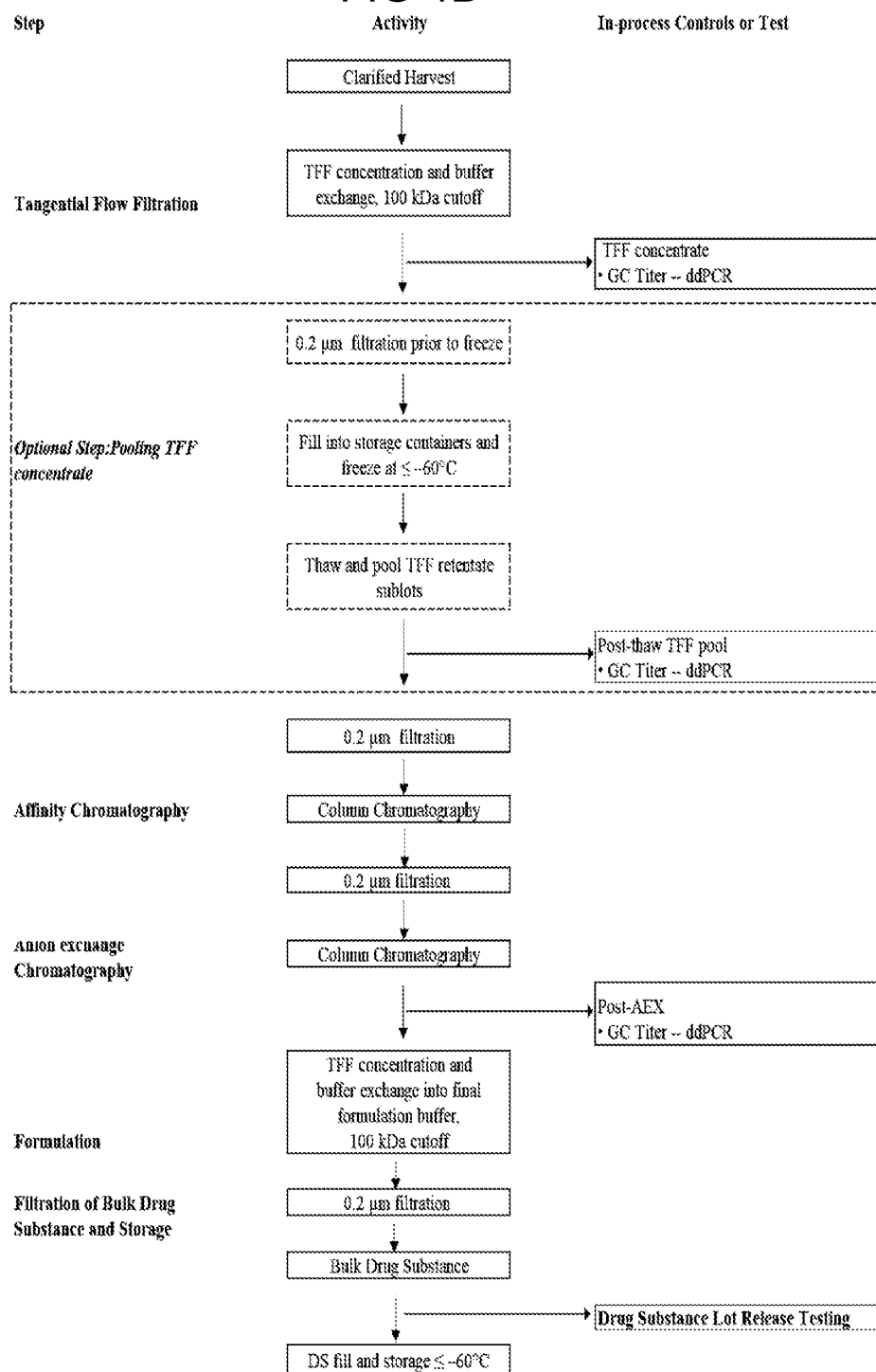


FIG 4A



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

FIG 4B



AEX, anion exchange; ddPCR, droplet digital polymerase chain reaction