

Figure 1A

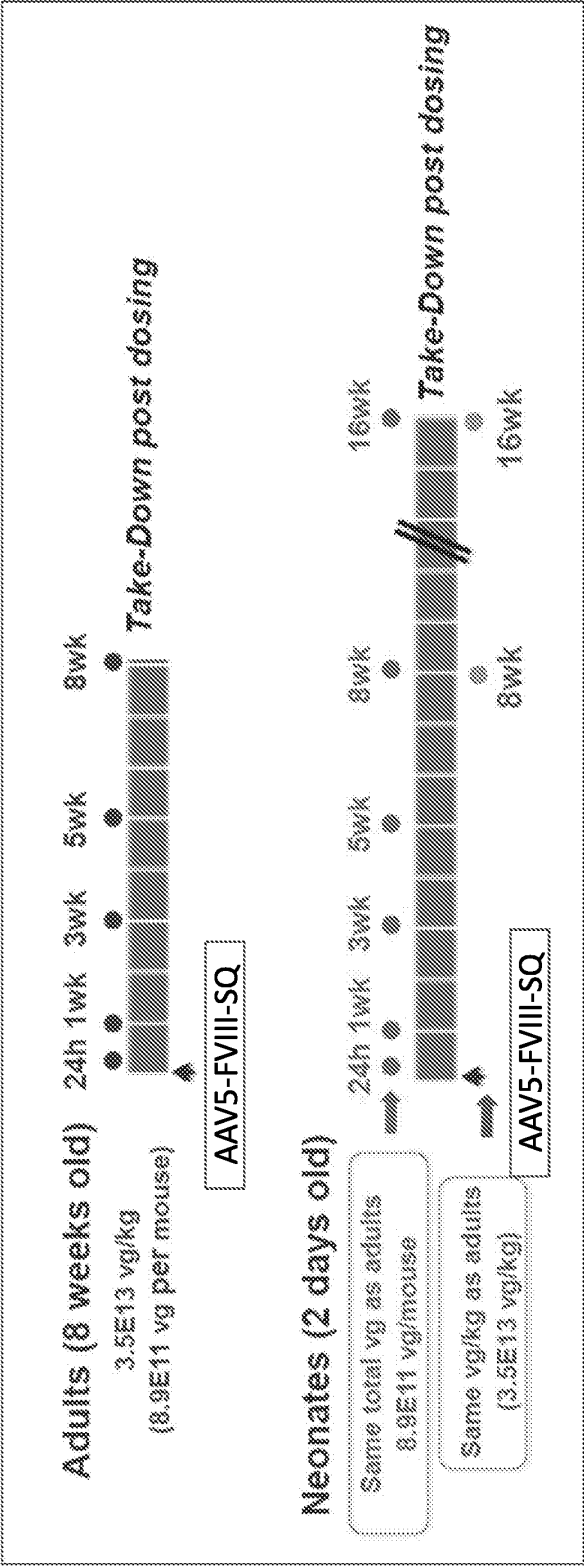


Figure 1B

Group	Mouse strain	Age at dosing time	Duration post dosing	Test article	Testing solution Ther (μg/ml)	Dosing Volume (μl)	N	Cohort	Dose (μg/kg)
1	FVIII/Rag2 DKO	8 week	24 hours	AAV5-FVIII-SQ	2.98E+13	30	10	Control	3.54E13
2	FVIII/Rag2 DKO	8 week	1 week	AAV5-FVIII-SQ	2.98E+13	30	10	Control	3.54E13
3	FVIII/Rag2 DKO	8 week	3 week	AAV5-FVIII-SQ	2.98E+13	30	10	Control	3.54E13
4	FVIII/Rag2 DKO	8 week	5 week	AAV5-FVIII-SQ	2.98E+13	30	10	Control	3.54E13
5	FVIII/Rag2 DKO	8 week	8 week	AAV5-FVIII-SQ	2.98E+13	30	10	Control	3.54E13
6	FVIII/Rag2 DKO	2 Day	24 hours	AAV5-FVIII-SQ	2.98E+13	30	10	2	4.47E14
7	FVIII/Rag2 DKO	2 Day	1 week	AAV5-FVIII-SQ	2.98E+13	30	10	2	4.47E14
8	FVIII/Rag2 DKO	2 Day	3 week	AAV5-FVIII-SQ	2.98E+13	30	10	2	4.47E14
9	FVIII/Rag2 DKO	2 Day	5 week	AAV5-FVIII-SQ	2.98E+13	30	10	2	4.47E14
10	FVIII/Rag2 DKO	2 Day	8 week	AAV5-FVIII-SQ	2.98E+13	30	10	2	4.47E14
11	FVIII/Rag2 DKO	2 Day	16 week	AAV5-FVIII-SQ	2.98E+13	30	10	2	4.47E14
12	FVIII/Rag2 DKO	2 Day	8 week	AAV5-FVIII-SQ	2.4E12	30	10	1	3.6E13
13	FVIII/Rag2 DKO	2 Day	16 week	AAV5-FVIII-SQ	2.4E12	30	10	1	3.6E13

Figure 2

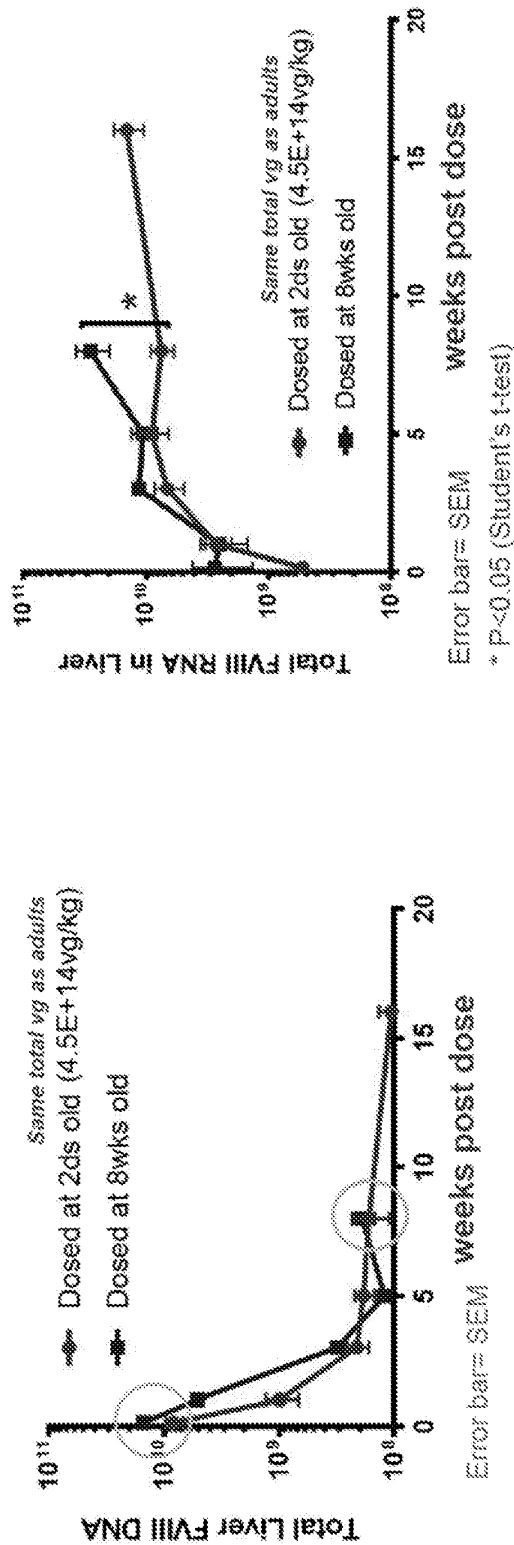


Figure 3

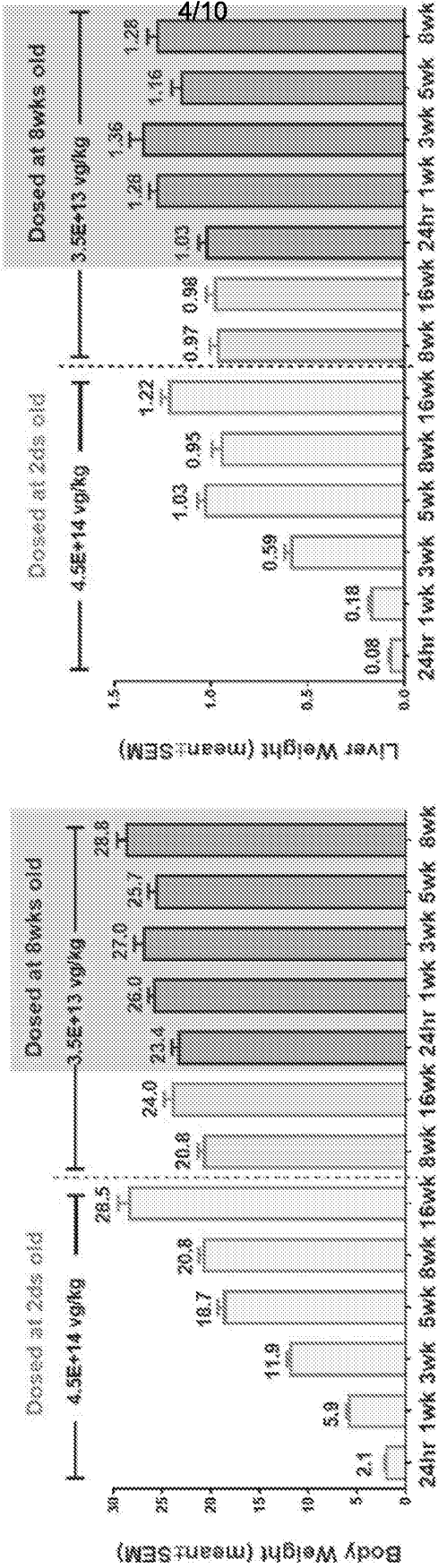


Figure 4

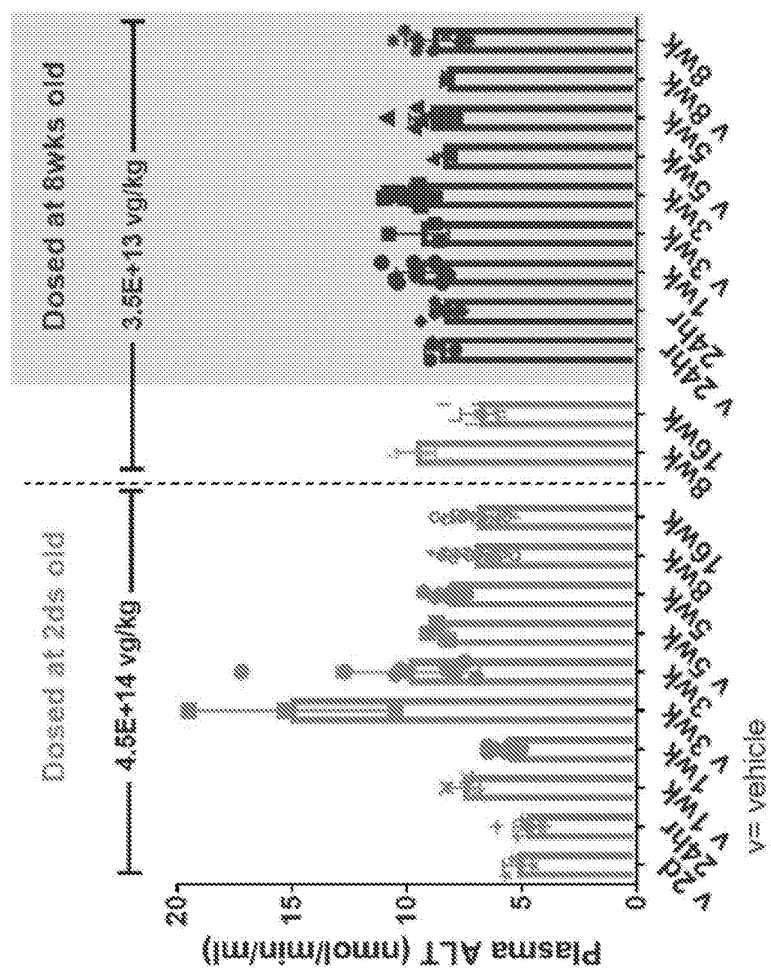


Figure 5

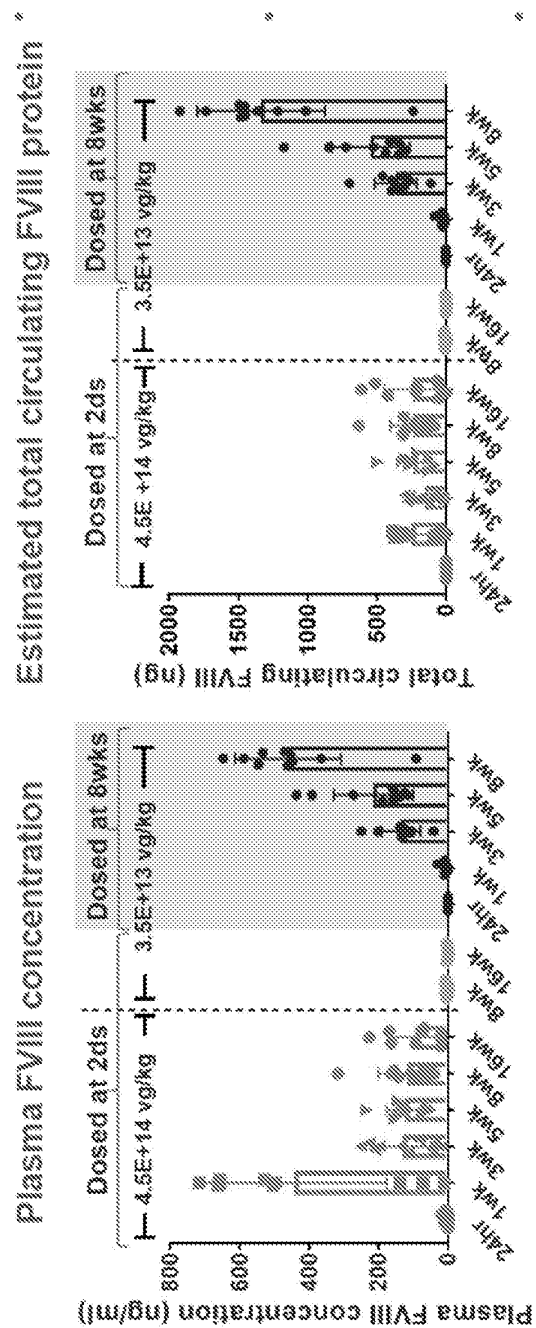


Figure 6

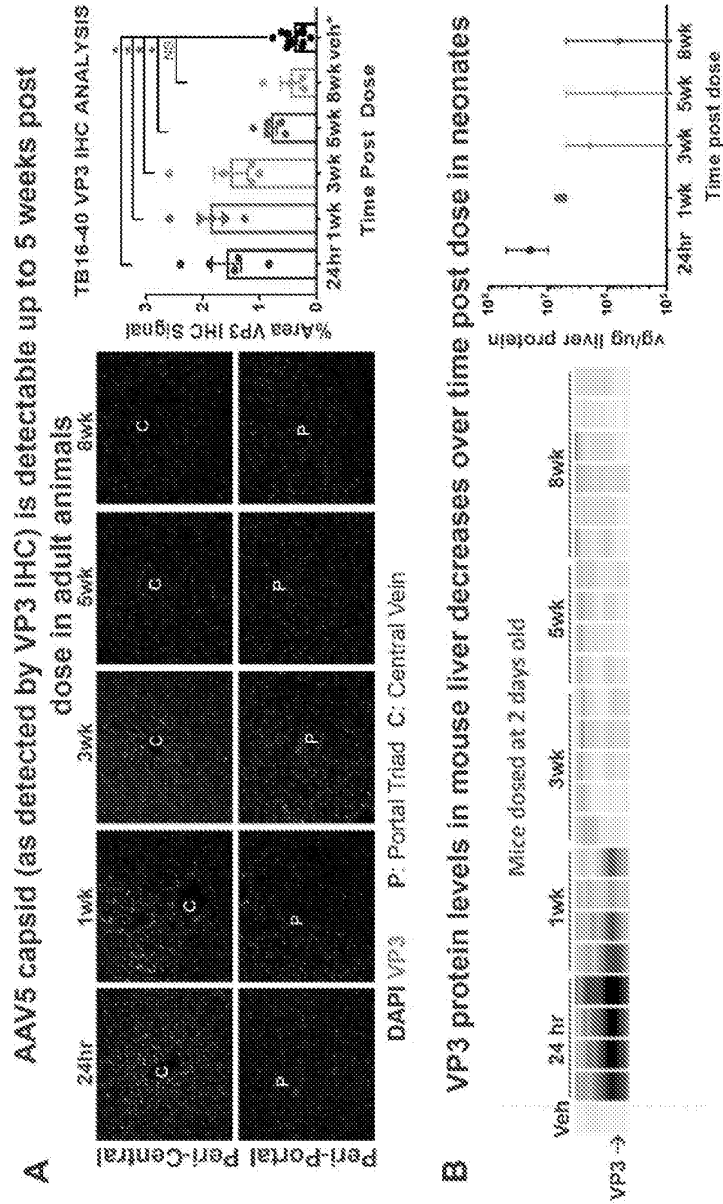


Figure 7

Group	Species	Strain	Animal #	Agent or Therapeutic	Dose (ex. ug or mg/kg)	Volume (ex. uL, mL)	Route (ex. IV, IP, SC, ICV)	Dose Regimen (frequency, rate)	Dosing Age
1	Mouse	male P10 ^{Cre} mouse	10	pFB-ApoE-hAAT-hPAH(vector #1)	2e14 vg/kg	4ul/gram of body weight	Femoral Vein	Single bolus	2 days
2	Mouse	male P10 ^{Cre} mouse	10	pFB-ApoE-hAAT-hPAH(vector #1)	2e14 vg/kg	4ul/gram of body weight	Femoral Vein	Single bolus	1 week
3	Mouse	male P10 ^{Cre} mouse	10	pFB-ApoE-hAAT-hPAH(vector #1)	2e14 vg/kg	4ul/gram of body weight	Femoral Vein	Single bolus	2 week
4	Mouse	male P10 ^{Cre} mouse	10	pFB-ApoE-hAAT-hPAH(vector #1)	2e14 vg/kg	4ul/gram of body weight	Femoral Vein	Single bolus	3 week
5	Mouse	male P10 ^{Cre} mouse	10	pFB-ApoE-hAAT-hPAH(vector #1)	2e14 vg/kg	4ul/gram of body weight	Femoral Vein	Single bolus	5 week
6	Mouse	male P10 ^{Cre} mouse	10	pFB-ApoE-hAAT-hPAH(vector #1)	2e14 vg/kg	4ul/gram of body weight	Femoral Vein	Single bolus	8 week

Figure 8

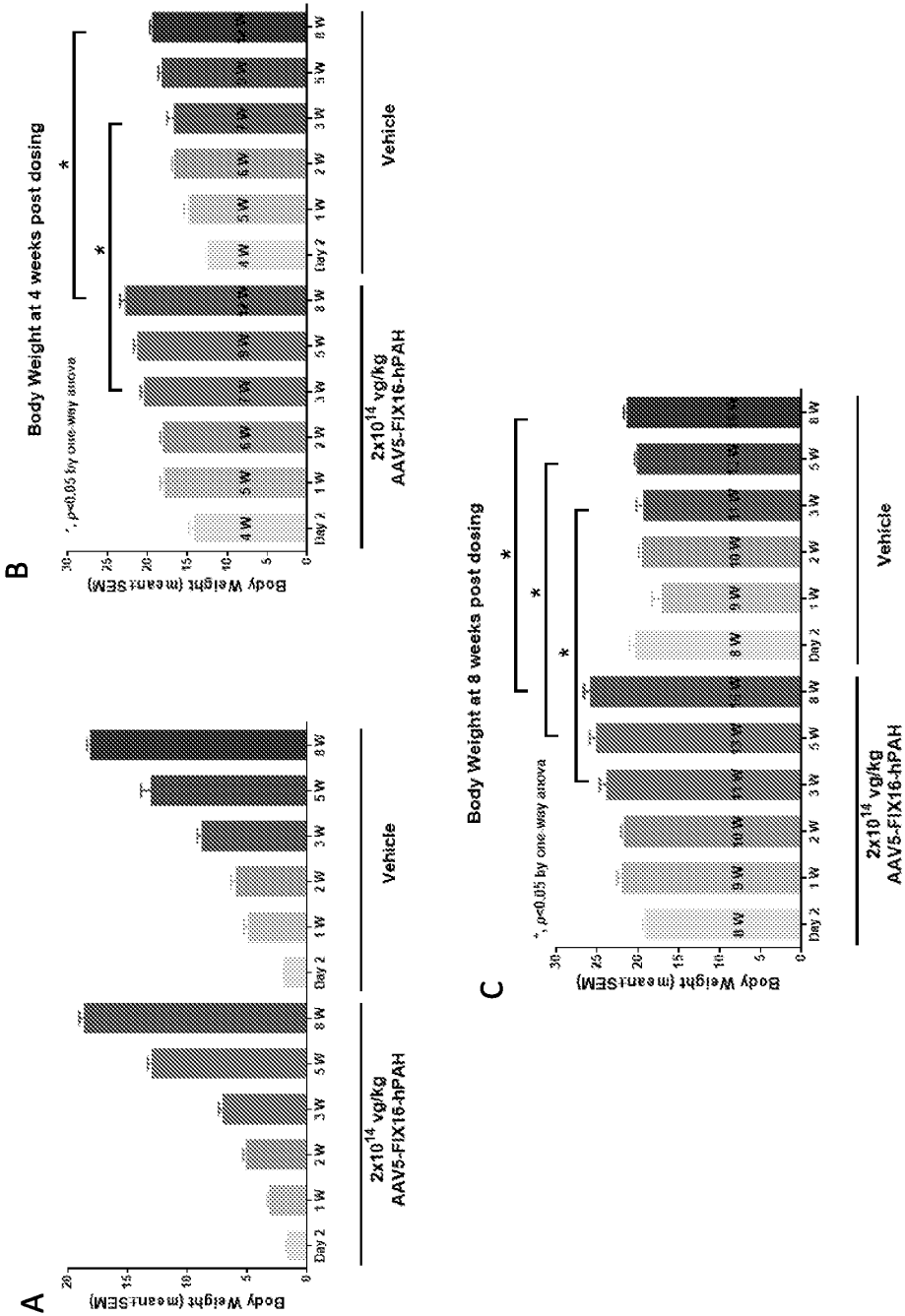


Figure 9

