

Table S1. Peripheral disease symptoms in post-symptomatic AAV-treated SD cats and untreated controls

	Incidence of symptom (number of cats (percent)) and age of symptom onset (mean, s.d.)			
	EPS cohort (n = 4)	LPS cohort (n = 3)	SD no tx (n = 14)	Normal (n = 7)
Heart abnormality ^A	3/4 (75) 11 ± 6.5	-	2/14 (14) 3.1 ± 1.5	-
GI dysfunction ^B	4/4 (100) 12 ± 5.6	-	1/14 (7.1) 1.8	-
Bladder dysfunction ^C	2/4 (50) 13 ± 2.0	-	-	-
Oral mass	1/4 (25) 6.0	-	-	-
Recurrent diarrhea ^D	-	1/3 (33) 3.0	-	-
Respiratory infection ^D	2/4 (50) 9.4 ± 9.8	-	1/14 (7.1) 1.8	-
Recurrent ocular/nasal discharge ^D	1/4 (25) 2.5	-	6/14 (43) 1.3 ± 0.67	-

^A Symptoms include heart enlargement diagnosed from radiographs or postmortem analysis; heart murmur upon auscultation; or abnormality associated with papillary muscle in the left ventricle.

^B Refers to distended small intestines, distended abdomen, or esophageal dysmotility.

^C Refers to distended bladder upon palpation.

^D Common conditions that also occur in normal controls so may be unrelated to SD or treatment

Abbreviations: LPS = late post-symptomatic; EPS = early postsymptomatic; GI = gastrointestinal; tx = treatment

Table S2. Vector copy number in brain, spinal cord, skeletal muscle, liver, and heart of post-symptomatic AAV-treated SD cats

Region	Block	Vector copy number per μg genomic DNA (mean, s.d.)	
		Post-symptomatic cohort	
		Early	Late
Cerebrum	A	19,000 (16,000)	20,000 (6,000)
	B	5,300 (3,000)	6,400 (330)
	C	12,000 (11,000)	6,900 (2,300)
	D	30,000 (22,000)	13,000 (11,000)
	E	12,000 (5,000)	18,000 (16,000)
Cerebellum	F	38,000 (44,000)	6,100 (5,800)
	G	57,000 (73,000)	4,100 (2,800)
	H	32,000 (44,000)	9,000 (6,200)
Spinal cord	I	3,200 (2,400)	1,700 (810)
	J	4,500 (2,800)	2,900 (850)
	K	2,100 (150)	1,900 (760)
	L	5,000 (3,200)	2,400 (5,600)
	M	5,000 (900)	1,900 (1,400) [▼]
	N	5,800 (2,300)	2,000 (680) [▼]
	O	4,400 (1,100)	1,600 (1,100) [▼]
Sciatic nerve	-	0 (0)	2,200 (3,400)
Liver	-	20,000 (20,000)	37,000 (26,000)
Skeletal muscle	-	0 (0)	2,200 (3,900)
Heart	-	1,800 (2,800)	180 (260)

[▼], significantly lower than the early post-symptomatic cohort, $P = 0.026$.

Block lettering corresponds to **Figure 4a**.

Abbreviations: CNS = central nervous system

Samples were analyzed in triplicate and the average value is reported

