

OMTM, Volume 5

Supplemental Information

**Efficacy of Gene Therapy Is Dependent
on Disease Progression in Dystrophic Mice
with Mutations in the FKRP Gene**

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SUPPLEMENTARY INFORMATION

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Figure S1. AAV9-*FKRP* vector construct. ITR, inverted terminal repeat of adenovirus; CK7, creatine kinase-based promoter; HuFKRP, full-length human *FKRP* coding sequence; SV40, Simian virus 40. Created with SnapGene®.

Age of Mice (Treatment Age)	52 Weeks (39 Weeks)	39 Weeks (n/a)	39 Weeks (26 Weeks)	26 Weeks (n/a)	26 Weeks (13 Weeks)	13 Weeks (n/a)	18 Weeks (5 Weeks)	5 Weeks (n/a)
AAV9- <i>FKRP</i>	+	-	+	-	+	-	+	-
Fibrotic Area (%)	45.7±0.7	44.2±1.7	35.5±1.0	32.0±0.7	20.5±1.3	19.9±0.9	9.5±0.9	11.1±1.5

Table S1. Quantification of the fibrotic area in the diaphragm of *FKRP*^{P448L} mutant mice injected with AAV9-*FKRP* (+) or untreated (-) for each cohort (n = 4).

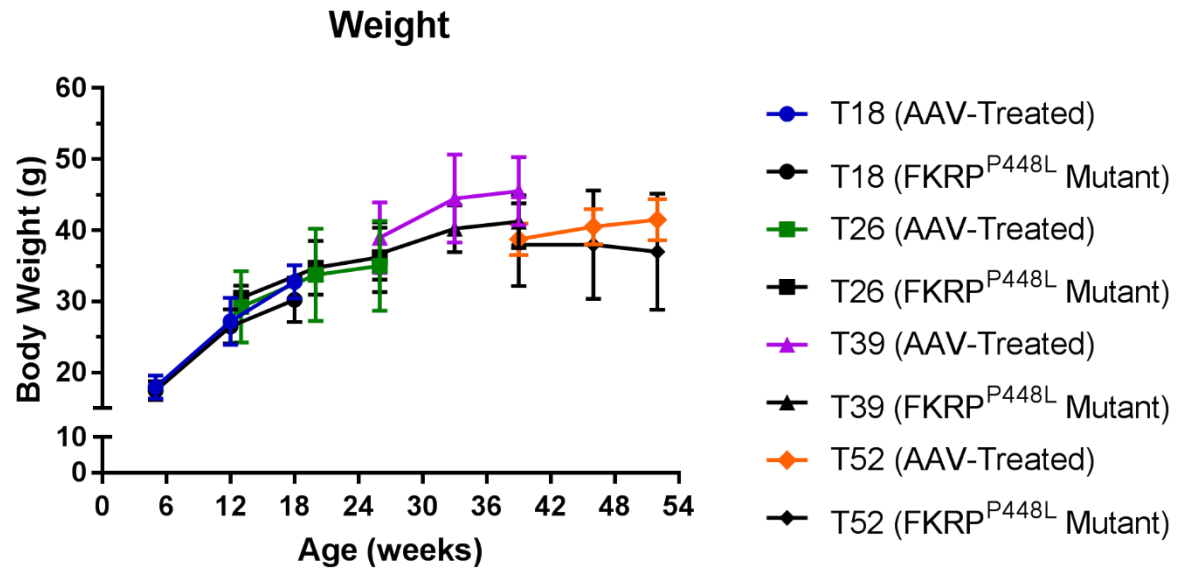


Figure S2. Weight profiles of the AAV-treated and FKRP^{P448L} mutant mice in each cohort.

Cardiac Parameters	Post-Injection Time Point	T18		T26		T39		T52	
		AAV Treated	FKRP ^{P448L} Mutant	AAV Treated	FKRP ^{P448L} Mutant	AAV Treated	FKRP ^{P448L} Mutant	AAV Treated	FKRP ^{P448L} Mutant
HR (beats/min)	0 Week	508±20	528±8	513±4	519±12	497±15	526±6	536±6	522±11
	12 Week	510±13	495±5	452±21	509±15	537±15	510±12	520±13	491±21
	% Change	0.4%	-6.3%	-11.8%	-1.8%	7.9%	-3.0%	-3.0%	-5.9%
LVEF (%)	0 Week	64.5±2.8	65.3±3.3	64.1±1.0	58.6±3.1	60.7±3.1	61.7±3.9	59.5±1.9	62.4±4.1
	12 Week	58.0±2.9	56.2±2.5	52.1±3.5	57.1±1.8	57.1±2.1	61.8±2.9	50.7±1.6	55.8±2.8
	% Change	-10.1%	-13.9%	-18.8%	-2.5%	-5.9%	0.1%	-14.8%	-10.6%
SV (mL)	0 Week	0.016±0.002	0.016±0.001	0.024±0.004	0.017±0.002	0.019±0.000	0.020±0.002	0.018±0.003	0.023±0.003
	12 Week	0.017±0.001	0.019±0.002	0.013±0.001	0.021±0.001	0.023±0.002	0.018±0.001	0.021±0.002	0.023±0.003
	% Change	8.8%	16.2%	-43.9%	23.2%	21.6%	-9.2%	16.5%	-0.6%
CO (mL/min)	0 Week	8.07±1.18	8.50±0.68	12.06±2.11	8.94±1.09	9.56±0.28	10.34±1.01	9.42±1.36	11.77±1.40
	12 Week	8.74±0.56	9.23±1.22	5.88±0.23	10.68±0.44	12.55±1.22	9.08±0.47	10.56±0.81	11.18±1.74
	% Change	8.3%	8.6%	-51.3%	19.5%	31.3%	-12.2%	12.0%	-5.0%

Table S2. Echocardiographic measurements. HR, heart rate; LVEF, left ventricle ejection fraction; SV, stroke volume; CO, cardiac output.