Disease	AAV Serotype	Transgene	Clinical Phase	Route of Administration	Clinical Trial Identifier	Successes	Limitations	References
Skeletal Muscle								
LPL Deficiency	AAV1	LPL	Phase I/II/III	Intramuscular	NCT01109498	decrease of median tryglyceride levels seen in all patients	drop in triglyceride levels was transient, anti-AAV capsid-specific T cells were detected in half of subjects	1, 2, 3
					NCT00891306	significant reduction in mean total plasma triglyceride levels; improved postprandial chylomicron metabolism	1/5 patients did not have decreased triglyceride levels; plasma glucose and insulin levels did not change	
Alpha 1 Anti-trypsin Deficiency	AAV2	α1 antrypsin	Phase I/II	Intramuscular	NCT00377416	Phase I: vector DNA sequences detected in the blood of most patients receiving mid to high doses; one patient exhibited low-level expression of AAT; Phase II: AAT expression oin serum was dose dependent, peaked on day 30, and persisted for at least 90 days	Phase I: transgene expression was below therapeutic levels in most patients, anti-AAV2 capsid antibodies were present and rose after vector injection; Phase II: transgene expression was below therapeutic levels	4, 5
	AAV1				NCT00430768			
Duchenne Muscular Dystrophy	AAV1/AAV2 chimera	Microdystrophin	Phase I	Intramuscular	NCT00428935	first demonstration of safety of an engineered AAV vector; no cellular immune response was mounted against capsid	weak or undetectable transgene levels in biopsied muscle tissue 1.5-3 months post-administration	6
Limb-Girdle Muscular Dystrophy	AAV1	α-sarcoglycan	Phase I	Intramuscular	NCT00494195	persistent α-sarcoglycan gene expression for six months in most subjects, increase in muscle fiber size, and restoration of the full sarcoglycan complex	one patient had early rise in neutralizing antibody titers and AAV capsid-specific T cells;	7, 8
Cardiac Muscle								
Severe Heart Failure	AAV1	SERCA2a	Phase I/II	Coronary Artery Infusion	NCT00454818	Phase I: decrease in symptoms, functional status, biomarker presence, and left ventricular function; Phase II: significant increases in the time to clinical events, decreased frequency of cardiovascular events, and decreased mean duration of cardiovascular hospitalizations over 12 months post-administration	Phase I: 2/9 patients showed no improvement (although pre-exisiting anti-AAV antibodies were detected); individual patients did not show improvements across all parameters; Phase II: improvements in all primary end point success criteria was seen only in highest dose cohort	19, 20

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