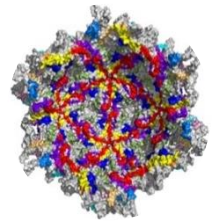


Canavan Gene Therapy

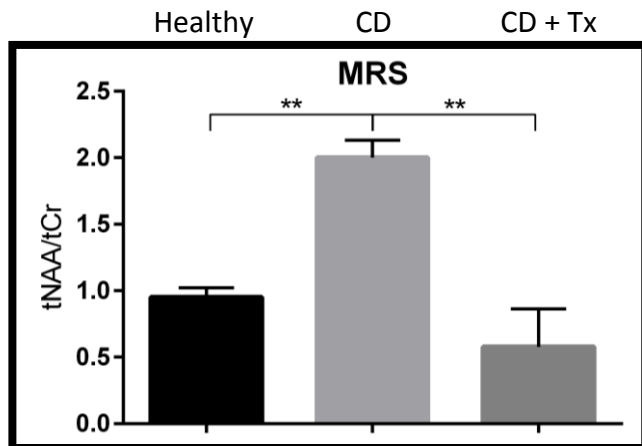


Tellez et al.
Adeno-associated
virus (AAV)

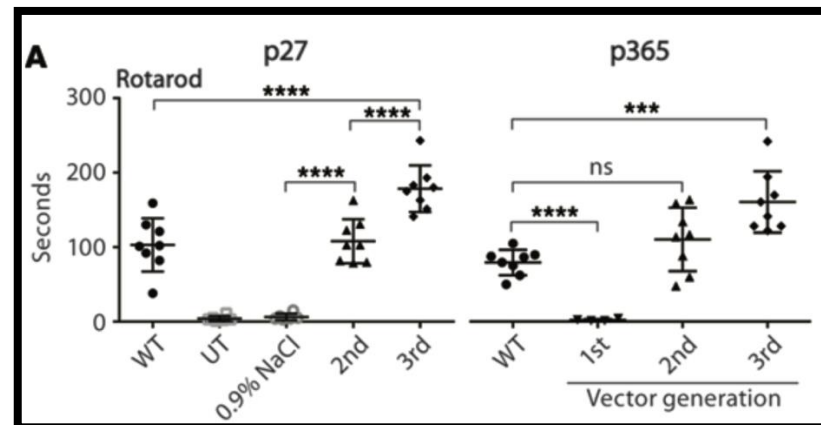
Strategy	Treatment age	Start	Status	Funding	FDA	Clinical trial
IV, 1 st gen	Newborn mice	2011	Completed	NTSAD/UMass/NIH	Pre-pre-IND	
ICV, 1 st gen	Newborn mice	2011	Completed	NTSAD/UMass/NIH	Pre-pre-IND	
IV, 3 rd gen	Newborn/adult mice	2013	Completed	NIH		In negotiation
ICV, 3 rd gen	Newborn/adult mice	2013	In progress	NIH		
IV	Nonhuman primate	2018	in preparation	NIH/UMass		

IV = intravenous; ICV = intracerebroventricular

NAA in the brain



Motor function



WT = healthy, UT = Canavan, 0.9% = control, 2nd and 3rd = Tx