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Article:
Scarrott, J.M., Herranz-Martín, S., Alrafiah, A.R. et al. (2 more authors) (2015) Current developments in gene therapy for amyotrophic lateral sclerosis. Expert Opinion on Biological Therapy.

https://doi.org/10.1517/14712598.2015.1044894

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DELIVERY METHOD	AIM OF THE STUDIES - THERAPEUTIC CARGO	MAIN FINDINGS	REFS.
ASOs	Therapeutic ASO targeting SOD1.	Increase survival in G93A mouse model.	70
	ASOs targeted towards different regions of C9orf72 mRNA.	Mitigate RNA toxicity in iPSC-differentiated neurons from C9orf72 ALS patients.	17
	ASOs to selectively reduce GGGGCC RNA foci in C9orf72 ALS patient fibroblasts.	Reduction in sense RNA foci without affecting the overall level of C9orf72 encoding RNA.	72
	ASO therapy against AChE mRNA in SOD1-G93A pre-symptomatic mice.	Slightly prolong in mice lifespan with an attenuation of motor neuron loss.	74
	ASOs to decrease the levels of miRNA-155 in SOD1-G93A mice.	Prolong mice survival.	78
AAV / LV-based vectors	AAV and LV vectors to deliver shRNA to knockdown SOD1.	Prolong survival in G93A mouse model.	67-69
	Intramuscular delivery of GDNF or IGF-1 by using AAV vectors [81, 84] or LV driving VEGF expression in SOD1-G93A mice [75].	Delay ALS onset, improve behavioral tasks and motor function and increase lifespan in G93A mice.	81,87, 90
	AAV delivering IGF-1 [82-83, 88-89], VEGF [83] or G-CSF [89] directly to different areas of the CNS in SOD1-G93A mice or rats models.	ICV [83], intraspinal [85, 88-89] or injections to the cerebellar nuclei [82] of these neurotrophic factors partially rescued the phenotype and increased the murine lifespan.	88-89, 91, 93-94
	LV driving the expression of antioxidant genes in cells *in vitro*.	Decrease oxidative stress levels and increase cell survival.	99
	Block misfolded SOD1 protein by using AAV to deliver a single chain fragment of the D3H5 antibody.	Levels of misfolded SOD1 protein in spinal cord were decreased, neuronal stress reduced and lifespan increased in SOD1-G93A mouse model.	101
	AAV expressing interleukin 10 in newborn SOD1-G93A mice.	Prolong expected lifespan	102
	AAV delivering hUPF1 in a TDP43 mouse model.	Rescue of forelimb paralysis.	103
	Normalize TDP43 expression in a mouse model for sALS (ADAR2 knockout): AAV delivering ADAR2.	Rescue of motor neurons phenotype and prevention of motor dysfunction.	106
LV vector systems in MSCs	Transplant of human MSCs transduced with LV encoding GDNF in SOD1-G93A rat model.	Neuroprotection in skeletal muscle, motor neurons and neuromuscular junctions. Increase in survival.	86
	Engineering hMSCs by using a LV-vector system to overexpress VEGF and GDNF of SOD1-G93A.	Delay the onset and prolong lifespan. VEGF and GDNF synergistic effect in the maintenance of ventral horn motor neurons and neuromuscular junctions.	84