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Development of AAV-based regenerative medicine expressing HGF gene (AAV9-HGF) for the treatment of ALS disease

Helixmith

HELIXMITH

NEUROSCIENCE	Candidate

Product Type	Gene Therapy
Indication	Amyotrophic Lateral Sclerosis (ALS, Lou Gehrig's disease)
Target	HGF/c-Met signaling pathway
MoA(Mechanism of Action)	Supply HGF protein to the injured or degenerated area of spinal cord activating c-Met receptor to promote regeneration process
Competitiveness	• There are high unmet medical needs for ALS disease where the development of regenerative medicine to reverse the death of motor neuron is highly demanded. • By supplying neurotrophic growth factor for relatively long period of time (>16 weeks), AAV9-HGF will show fundamental regenerative therapeutic effect by inducing the regeneration process of degenerating motor neuron and the surrounding environment.
Development Stage	Candidate
Route of Administration	Intrathecal administration

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