

Trials on humans supported by the AFM

> Neuromuscular diseases

GT : Gene therapy CT : Cell therapy P : Pharmacology

Diseases	Study	Type of therapy	Product	Phase
Duchenne and Becker muscular dystrophies	- Exon skipping by systemic injection of anti-sense oligonucleotides	GT	Oligonucleotide	Phase I/II (ongoing)
	- Gene therapy trial with with plasmid containing the whole coding sequence of the dystrophin gene. Intra-vascular/ locoregional injection.	GT	Naked plasmid integrating the dystrophin gene	Preclinical development (ongoing)
	-Myoblast transplant	CT	Myoblasts	Phase I (ongoing)
	-AAV-U7 exon skipping	GT	AAV-U7	Preclinical development (ongoing-Généthon)
Limb-girdle muscular dystrophy	- AAV2/1 gamma sarcoglycan gene transfer in type 2C limb-girdle muscular dystrophies	GT	AAV gamma-sarcoglycan	Phase I (ongoing-Généthon))
	- Alpha-sarcoglycan gene transfer	GT	AAV alpha-sarcoglycan	Preclinical development (planned - Généthon)

Facio-Scapulo-Humeral (FSH) dystrophy	- Facio-Scapulo-Humeral (FSH) dystrophy autologous cell therapy trial	CT	Myoblasts	Phase I/II (ongoing)
	- Evaluation of Salbutamol in FSH patients	P	Salbutamol	Phase III (ongoing)
Steinert myotony	Double-blind vs placebo randomised trial to evaluate the effectiveness of methylphenidate	P	Methylphenidate	Phase III (planned)
Spinal amyotrophies	- Double-blind vs placebo randomised trial to evaluate the effectiveness and tolerance of riluzole in types II and III spinal amyotrophy patients	P	Riluzole	Phase III (ongoing)
	- Single- and repeated-dose phase 1b study to evaluate the tolerance and pharmacokinetics of TRO19622 in Infantile Spinal Amyotrophy patients	P	TRO 19622	Phase IB (ongoing)
Inclusion body myositis	Regulator lymphocytes transduced with a tk lentivirus	T	Treg cells	Phase I (planned)
Oculo-pharyngeal muscular dystrophy (OPMD)	Oculopharyngeal muscular dystrophy dysphasia treatment by myoblast autotransplant	CT	Myoblasts	Phase II (ongoing)

Charcot-Marie-Tooth disease	Study into the effects of vitamin C on the muscle strength of Charcot-Marie-Tooth 1A patients	P	Vitamin C	Phase III (ongoing)
Periodic paralyses	Randomised multicentric therapeutic trial to test dichlorphenamid and acetazolamid vs placebo in periodic paralyses	P	Dichlorphenamid and acetazolamid	Phase III (planned)
Carnitin-Palmitoyl-Transferase 2 (CPT2) deficiency	Clinical trial into the effect of bezafibrate in the adult muscular form	P	Bezafibrate	Phase III (ongoing)
Dysferlinopathies	Trial to evaluate the therapeutic effect of Ig intravenous injection	P	Immuno-globins	Phase I (planned)