

Essais en cours dans les maladies musculaires de l'adulte

Acronyme	Essai	Promoteur	Type d'étude	Pathologie	Traitement
HIBM-Phase III	A Phase 3 Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of Sialic Acid Extended-Release Tablets in Patients with GNE Myopathy (GNEM) or Hereditary Inclusion Body Myopathy (HIBM)	ULTRAGENYX	Multicentrique Internationale	HIBM (Nonaka)	SA-ER Placebo
RAPAMI	Rapamycine vs Placebo for the Treatment of Inclusion Body Myositis	INSERM U974	Monocentrique	Myosite à inclusions	Rapamycine Placebo
ATYR-C4	An Open-Label, Inpatient Dose Escalation Study to Evaluate the Safety, Tolerability, Immunogenicity, and Biological Activity of ATYR1940 in Patients with Limb Girdle and Facioscapulohumeral Muscular Dystrophies	aTyr Pharma	Multicentrique Internatio	FSHD LGMD	ATYR1940 Placebo
CBYM338B2203E1	Extension of the CBYM338B2203 phase IIb/III study to evaluate the long-term efficacy, safety and tolerability of intravenous BYM338 in patients with sporadic inclusion body myositis	NOVARTIS	Multicentrique Internatio	Myosite à inclusions	BYM 338
MGEX	Effect of training on quality of life in Myasthenia Gravis	APHP	Monocentrique	Myasthenia Gravis	Physical training
NeoGAA	An open-label, multicenter, multinational, ascending dose study of the safety, tolerability, pharmacokinetics, pharmacodynamics, and exploratory efficacy of repeated biweekly infusion of neoGAA in naïve and alglucosidase alfa treated late-inset Pompe disease patients.	Sanofi	Multicentrique Internationale	Maladie de Pompe	NeoGAA
MyoMet	A randomized, double blind, placebo-controlled phase II study of Metformin in Myotonic Dystrophy type I patients	Genethon	Monocentrique	Maladie de Steinert DM1	Metformin
COMET	A phase 3 randomized, multicenter, multinational, double blinded study comparing the efficacy and safety of repeated biweekly infusions of neo GAA (GZ402666) and alglucosidase alfa in treatment-naïve patients with late onset Pompe disease	Sanofi	Multicentrique Internationale	Maladie de Pompe	neoGAA
	The Effect of Triheptanoin in Adults with McArdle Disease (Glycogen Storage Disease Type V)	INSERM	Monocentrique X 2	Maladie de McArdle	Triheptanoïne