AFM-TELETHON PIPELINE
of clinical trials

Clinical trials (ongoing or in preparation)

	DISEASES		TYPE OF THERAPY	PRODUCT	PHASE
NEUROMUSCULAR DISORDERS	Spinal muscular atrophy with progressive myoclonic epilepsy (SMA-PME) and Farber disease		GT	AAV-ASAH1	Preclinical
	Duchenne muscular dystrophy		GT	AAV-microdystrophin	Phase I/II/III
			P	Sarconeos (BIO101)	Phase I/II (in preparation)
			P	Tamoxifen	Phase III*
	LGMD R1 (calpain)		GT	AAV-CAPN3	Preclinical
	LGMD R3 (α-sarcoglycan)		P	Givinostat	Preclinical
	LGMD R5 (γ-sarcoglycan)		GT	AAV-SGCG	Preclinical
			P	Givinostat	Preclinical
	LGMD R9 (FKRP)		GT	AAV-FKRP	Phase I/II
	Myotonic dystrophy type 1 (Steinert)		GT	AAV-MBNLΔ	Preclinical
	Type 3 glycogenosis (Cori-Forbes disease)		TG	AAV-GDE	Preclinical
	Charcot-Marie-Tooth disease		P	PXT3003	Phase III*
			P	IFB-088	Phase I completed
	Pompe disease		GT	AAV-GAA	Phase I/II
	Myotubular myopathy		GT	AAV-MTM	Phase I/II*
	Inclusion body myositis		P	Rapamycin	Phase III*
	Sarcopenia		P	recGDF5	Preclinical
	Amyotrophic lateral sclerosis		GT	AAV-SOD1	Preclinical
			GT	AAV-C9	Preclinical
			P	Anti-CD38	Preclinical
			P	IFB-088	Phase II
			P	Interleukin 2	Phase IIb
OTHER DISEASES	Fanconi anemia		GT	Hematopoietic stem cells + LV-FANCA	Phase I/II*
	Immune deficiencies	Artemis deficiency	GT	Hematopoietic stem cells + LV-Artemis	Phase I/II
		X-linked severe combined immunodeficiency (X-SCID)	GT	Hematopoietic stem cells + LV-XSCID	Phase I/II*
		Chronic granulomatosis	GT	Hematopoietic stem cells + LV-CGD	Follow-up study (15 years) Phase I/II USA
		Wiskott-Aldrich syndrome	GT	Hematopoietic stem cells + LV-WAS	Follow-up study (15 years)
	Dystrophic epidermolysis bullosa		GT	Genetically modified autologous skin cells	Phase I/II*
	Junctional epidermolysis bullosa		GT	Genetically modified autologous skin cells	Pilot study*
	Glycogen storage disease type 1a (Von Gierke disease)		GT	AAV-G6PC	Preclinical
	Disseminated Lupus Erythematosus		CT	Mesenchymal stem cells	Phase I/II
	Crigler-Najjar disease		GT	AAV-UGT1A1	Phase III (confirmatory study)
	Retinitis pigmentosa		CT	Embryonic stem cells	Phase I/II
			GT	AAV-RdCVF	Preclinical
	Multiple sclerosis		CT	Cytotoxic T cells	Phase I
	Phelan-McDermid syndrome (genetic form of autism)		P	Lithium	Phase III
	Wolfram syndrome		P	Valproic acid	Phase II
	Sickle cell skin ulcers		CT	Embryonic stem cells	Preclinical

Approved drugs

Cuprior® P
Wilson disease

Firdapse® P
Lambert-Eaton syndrome

Namuscla® P
Myotonic syndromes

Skysona® TG
Adrenoleucodystrophy

Strimvelis® TG
X-SCID

Zynteglo® TG
β-thalassemia

Zolgensma® TG
Spinal muscular atrophy

Off-label drugs

Lumevoq® TG
Leber optic neuropathy

Metformin P
Steinert myotonic dystrophy

Patient databases

- 13 patients databases
- 16,000 neuromuscular disease patients

GT Gene therapy | CT Cell therapy | P Pharmacology

* Preclinical or previous clinical phases funded by AFM-Telethon

AFMTÉLÉTHON INNOVER POUR GUÉRIR

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