

Trials on humans supported by the AFM

> Other diseases

GT : Gene therapy CT : Cell therapy P : Pharmacology

Diseases	Study	Type of therapy	Product	Phase
Adrenoleukodystrophy	<i>Ex vivo</i> gene therapy trial in patients affected with X-linked adrenoleukodystrophy	GT	Hematopoietic cells + ALD lentivirus	Phase I/II (ongoing)
Huntington disease	Multicentric study of intracerebral neuron transplant for the treatment of Huntington disease	CT	Neurons	Phase II (ongoing)
Myocardial infarction	- Evaluation of medullary progenitors and prevention of post-ischemic heart failure by endo-coronary injection of autologous medullary cells (BONAMI)	CT	Hematopoietic cells	Phase II (ongoing)
	- Cardiac cell therapy trial (MESAMI)	CT	Mesenchymatous cells	Phase I (planned)
Epidermolysis bullosa	- <i>Ex vivo</i> gene therapy trial in Junctional Epidermolysis Bullosa (JEB) patients	GT	Epidermis cells + laminin 5 retrovirus	Phase I/II (ongoing)
	- <i>Ex vivo</i> gene therapy trial in Junctional Epidermolysis Bullosa	GT	Epidermis cells + laminin 5	Phase I/II (planned)

	(JEB) patients		vector	
Immune deficiencies	- <i>Ex vivo</i> gene therapy trial in Dystrophic Junctional Epidermolysis Bullosa (DEB) patients	GT	Epidermis cells + collagen VII vector	Phase I/II (planned)
	- <i>Ex vivo</i> gene therapy trial in Wiskott Aldrich syndrome patients	GT	Hematopoietic cells + WASP lentivirus	Pre-clinical Development (ongoing - Génethon)
	- <i>Ex vivo</i> gene therapy trial in Adenosine Deaminase (ADA) patients	GT	Hematopoietic cells + ADA retrovirus	Phase I/II (ongoing)
β-hemoglobinopathies (drepanocytosis, thalassemia)	Gene therapy trial by <i>ex vivo</i> modified autologous CD34+ stem cell transplantation from affected patients	GT	Hemattopoietic cells + βA-T87Q-globin lentivirus	Phase I/II (ongoing)
Sanfilippo diseases	- AAV <i>in vivo</i> gene therapy trial in San Filippo disease (MPSIII B) patients	GT	AAV-NaGlu	Phase I/II (planned)
	- AAV <i>in vivo</i> gene therapy trial in San Filippo disease (MPSIII A)	GT	AAV-SGSH	Phase I/II (planned)
Retinal dystrophy	Gene therapy trial in patients affected by retinal dystrophy linked to an RPE65 gene defect (Leber Amaurosis)	GT	AAV-RPE65	Phase I/II (planned)
Congenital Erythropoietic Porphyria (CEP)	<i>Ex vivo</i> gene therapy trial in Congenital Erythropoietic Porphyria (CEP) patients	GT	Hematopoietic cells + UROS lentivirus	Phase I/II (planned)

Progeria	Combined drug treatment for progeria	P	Amino-biphospho- rate and statins	Phase I/II (planned)
Auto-immune diseases with secondary myelodysplasia complications	Allotransplants of genetically modified donor hematopoietic stem cells and T lymphocytes expressing the HSV-TK gene	CT	Hematopoeitic cells + T lymph	Phase I (planned)
Friedreich Ataxia	Effect of pioglitazone on Friedreich Ataxia patients	P	Pioglitazone	Phase III (planned)