LISTE DES FINANCEMENTS ACCORDES PAR L'AFM-TELETHON EN 2023

Au titre de sa mission Guérir (hors financements accordés à Généthon, CECS-Istem, Institut de Myologie)

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'appel d'offres annuel

Commission thématique : Myologie fondamentale

Aides aux jeunes chercheurs post-doctorants

ANQUETIL	**Céline**	PARIS	FRANCE	CAchexia and Resident MAcrophages: fate and function study
SMITH	**Jacob Anderson Giffen**	BARCELONE	ESPAGNE	Influence of circadian regulation on motor neuron-muscle fiber crosstalk

Financements de projets Tremplins

TAYLOR	**Michael**	CARDIFF	ROYAUME-UNI	Mef2 dynamics in a model muscle injury/repair system
VOLK	**Talila**	REHOVOT	ISRAËL	The contribution of the LINC complex to epigenetic regulation of chromatin in mature muscle fibers

Financements de projets

BLAAUW	**Bert**	PADOUE	ITALIE	Identification of muscle-specific factors involved in NMJ maintenance and their regulation by mTORC1
CIENIEWSKI-BERNARD	**Caroline**	LOOS	FRANCE	Trojan-CryAB: an innovative therapeutic approach for skeletal muscle diseases resulting from proteotoxicity and protein aggregation
DUPREZ	**Delphine**	PARIS	FRANCE	Regionalisation of myogenesis
LEBRETON	**Gaëlle**	TOULOUSE	FRANCE	Muscle-hematopoietic organ crosstalk: alary somatic muscle as a secretory organ regulating hematopoiesis in Drosophila.
LEFKIMMIATIS	**Konstantinos**	PAVIE	ITALIE	The spatiotemporal map of nuclear signalling in the aging muscle
MAMMUCARI	**Cristina**	PADOUE	ITALIE	The role of mitochondrial calcium signalling in aging skeletal muscle.
MENZIES	**Keir**	OTTAWA	CANADA	Examining the role and therapeutic potential of Poly-ADP-Ribosylation (PARylation) in myopathies and skeletal muscle maintenance and function
PENDE	**Mario**	PARIS	FRANCE	Role of mitochondrial YAP and TAZ in muscle growth, regeneration and mechanotransduction
PIERCY	**Richard J.**	LONDRES	ROYAUME-UNI	Defining dystrophin's transcriptional domains in healthy and damaged muscle
POLGE	**Cécile**	SAINT GENÈS CHAMPANELLE	FRANCE	Towards the inhibition of skeletal muscle atrophy by selective inhibition of the ad hoc MuRF1- E2 couple
ROMANELLO	**Vanina**	PADOUE	ITALIE	The in vivo role of peroxisomes in the control of muscle function
SAURIN	**Andrew**	MARSEILLE	FRANCE	Hox control of Drosophila adult myogenesis
SHI	**De-Li**	PARIS	FRANCE	Rbm24-controlled cytoplasmic polyadenylation in the post-transcriptional regulation of muscle cell differentiation
SOTIROPOULOS	**Athanassia**	PARIS	FRANCE	Functional cell heterogeneity underlying muscle plasticity

Commission thématique : Bases Moléculaires et Physiopathologie des Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

APOLLONI	**Savina**	ROME	ITALIE	Targeting S100A4 to study the macrophages-muscle cells cross-talk in models of Duchenne Muscular Dystrophy
LÉGARÉ	**Cécilia**	CHICOUTIMI	CANADA	Identification of molecular signatures associated to strength training in myotonic dystrophy type 1

Financements de projets

AMTHOR	**Helge**	MONTIGNY-LE-BRETONNEUX	FRANCE	The syncytial organization of dystrophin mosaic in female mdx carrier mice
BOUTER	**Anthony**	PESSAC	FRANCE	Interplay of annexins, macrophages and fibroadipogenic progenitors in the development of muscular dystrophies
COPPEE	**Frédérique**	MONS	BELGIQUE	Characterization of DUX4 and DUX4c major protein partners to identify new therapeutic targets for faciocapulohumeral muscular dystrophy (FSHD)
CORDERO	**Gerardo Antonio**	LISBONNE	PORTUGAL	DYSTRO-NET2: Gene Regulatory Networks of LAMA2-CMD Based on Single-nucleus Sequencing
DI PIETRO	**Lorena**	ROME	ITALIE	DIssecting the pathogenetic RolE played by the different Cell types within the skeletal muscle sTem cell nIche Of FSHD patieNts
LANZUOLO	**Chiara**	MILAN	ITALIE	Exploring the genetic and epigenetic background underlying the phenotype's variability in Emery Dreifuss Muscular Dystrophy
MAZZONE	**Massimiliano**	TURIN	ITALIE	GLUD1 as a potential target in Muscular Dystrophy
PARROT	**Sandrine**	BRON	FRANCE	Alterations in brain glutamate and GABA neurotransmissions in DM1 disease: focus on neuronal subpopulations and related electrophysiological signals
VAN PUTTEN	**Maaike**	LEIDEN	PAYS-BAS	Characterization of four humanized mouse models for Duchenne muscular dystrophy
WANSINK	**Derick G.**	NIJMEGEN	PAYS-BAS	DM1 repeat length-dependent disease heterogeneity in muscles
ZAMMIT	**Peter**	LONDRES	ROYAUME-UNI	Investigating pathogenic mechanisms in FSHD myogenesis.

Commission thématique : Bases moléculaires et physiopathologie des autres maladies neuromusculaires

Aides aux jeunes chercheurs post-doctorants

GABILLARD	**Claudie**	ANGERS	FRANCE	Modulation of mitochondrial DYNAmics as a therapeutic approach to maintain mtDNA integrity and mitigate the accumulation of deficient myofibers in mitochondrial MYOpathies
JOHARI	**Mridul**	HELSINKI	FINLANDE	Improved diagnostics and gene discovery for unsolved myopathies
MORETTON	**Amandine**	AUBIÈRE	FRANCE	Identification and characterisation of new players in mtDNA maintenance in health and disease

Financements de projets

BARIS	**Olivier**	ANGERS	FRANCE	Modulation of mitochondrial DYNAmics as a therapeutic approach to maintain mtDNA integrity and mitigate the accumulation of deficient myofibers in mitochondrial MYOpathies
BENARD	**Giovanni**	BORDEAUX	FRANCE	Studying the ribosomal-associated quality control in a mitochondrial syndrome.
BRIS	**Céline**	ANGERS	FRANCE	Urine-derived cells for non-invasive diagnosis of mitochondrial diseases
CHEVROLLIER	**Arnaud**	ANGERS	FRANCE	Induced neurons resulting from direct conversion of fibroblasts from patients with mitochondrial diseases: a new model to explore ATP synthase reverse function and its role in mitochondrial fission.
COLLE	**Marie-Anne**	NANTES	FRANCE	Effects of enzyme replacement therapy on lysosome-autophagosome-mitochondria cross-talk and muscle repair in Pompe disease (LYSAUMI)
COSSEE	**Mireille**	MONTPELLIER	FRANCE	Transcriptomic and proteomic analyses to unravel pathophysiology and Phenotype-Genotype Correlations in Patients with skeletal Titinopathies
DE LONLAY	**Pascale**	PARIS	FRANCE	Autophagy dysregulation in acute rhadomyolysis
FARGE	**Geraldine**	CLERMONT-FERRAND	FRANCE	Exploring at the single molecule level the functional consequences of disease-causing mutations in human mitochondrial DNA maintenance and expression factors
FERNANDEZ-VIZARRA	**Erika**	PADOUE	ITALIE	Tissue-specificity of complex III dysfunction in mitochondrial encephalomyopathies
RAAPHORST	**Joost**	AMSTERDAM	PAYS-BAS	Auto-immunity and muscle strength: the effect of anti-SRP and anti-HMGCR antibodies on myofiber contractility in myositis
ROSSI	**Daniela**	SIENNE	ITALIE	CCDC78: from basic research to mechanisms of disease
STEFFANN	**Julie**	PARIS	FRANCE	MITOCARE: Mitochondrial replacement therapy (MRT) against mitochondrial DNA (mtDNA) disorders: are we far from its clinical application?
UDD	**Bjarne**	HELSINKI	FINLANDE	Titinopathies – disease models for translational therapeutics
VANDROMME	**Marie**	TOULOUSE	FRANCE	Epigenetic contribution in the etiology of Myotubular Myopathy

Commission thématique : Système nerveux - Motoneurone et jonction neuromusculaire

Aides aux jeunes chercheurs post-doctorants

KLEIJWEGT	**Constance**	MONTPELLIER	FRANCE	Chromatin proteomics of extended GAA/TTC trinucleotides repeats : Implication for Friedreich Ataxia
MAINO	**Eleonora**	BALE	SUISSE	Characterization of pathophysiological mechanisms involved in the selective vulnerability of muscles in Myasthenia Gravis
REICHOVA	**Alexandra**	BORDEAUX	FRANCE	Oxytocin and 5-HT involvement in early dysregulation of the chloride homeostasis in spinal ALS motoneurons
SARDINA	**Francesca**	ROME	ITALIE	Phenotypic evaluation of microtubules network as prognostic and predictive marker in response to spastin elevating approaches in Hereditary Spastic Paraplegia type 4

Financements de projets Tremplins

CANDAYAN NIRON	**Ayse**	ANVERS	BELGIQUE	Investigation of NGS-intractable mutational classes in peripheral neurodegeneration
RODRIGUEZ CRUZ	**Pedro M.**	BARCELONA	ESPAGNE	A global analysis of the CMT1A locus: implications for the origin and susceptibility to Charcot-Marie-Tooth disease type 1A across populations

Financements de projets

ARRIBAT	**Yoan**	MONTPELLIER	FRANCE	Organelle coordination in hereditary spastic paraplegia: deciphering protein network and restoring lipid balance
BESSEREAU	**Jean-Louis**	LYON	FRANCE	Genetic control of acetylcholine receptor expression: from new mechanisms to functional genomics
BRANCHEREAU	**Pascal**	BORDEAUX	FRANCE	Chloride-related inhibition in spinal motoneurons during the ALS disease progression in mouse model
DÍAZ-MANERA	**Jordi**	NEWCASTLE	ROYAUME-UNI	Transcript-MND: Transcribing the process of muscle degeneration in patients with motor neuron diseases
FERRARO	**Elisabetta**	PISE	ITALIE	Metabolic reprogramming and activation of microglia in amyotrophic lateral sclerosis (ALS): a pivotal role for serotonin?
HAASE	**Georg**	MARSEILLE	FRANCE	Mini-Brains to Better Understand C9orf72-linked ALS
JORDANOVA	**Albena**	EDEGEM	BELGIQUE	Profiling the spatial RNA and protein signatures of peripheral neurons in YARS associated Charcot-Marie-Tooth neuropathies
LIEVENS	**Jean-Charles**	MONTPELLIER	FRANCE	Deciphering the beneficial effects of Sigma-1 receptor in amyotrophic lateral sclerosis
ORTEGA CANO	**Juan Alberto**	L'HOSPITALET DE LLOBREGAT	ESPAGNE	Defining the contribution of ALS-associated alterations in motor neuron microenvironment to disease pathogenesis.
OURY	**Franck**	PARIS	FRANCE	Decipher the role of the Ocn-induced autophagy in motoneurons and its anti-aging therapeutic potential
PENNUTO	**Maria**	PADOUE	ITALIE	Development of a therapeutic strategy to suppress LSD1 and PRMT6-mediated toxic gain of function in SBMA
POLETTI	**Angelo**	MILAN	ITALIE	The involvement of the small heat shock protein HSPB8 in amyotrophic lateral sclerosis
RINALDO	**Cinzia**	ROME	ITALIE	Spastin elevating approaches to counteract Hereditary Spastic Paraplegia
SELLIER	**Chantal**	STRASBOURG	FRANCE	Identify the role of NUP50 in motor neuron physiology and ALS pathology
VIERO	**Gabriella**	POVO (TRENTE)	ITALIE	Ribosome-based functions of the SMN protein: from fundamental biology to second-generation therapies for SMA

Commission thématique : Cellules souches

Aides aux jeunes chercheurs post-doctorants

TESTA	**Stefano**	MARSEILLE	FRANCE	3D bioprinting for human artificial skeletal muscle modeling

Financements de projets Tremplins

CANTONE	**Irene**	NAPLES	ITALIE	Understanding X chromosome reactivation in differentiated human muscle cells for a future therapy of X-linked neuromuscular diseases

Financements de projets

DUTEIL	**Delphine**	ILLKIRCH	FRANCE	Role of androgens in muscle regeneration
ELVASSORE	**Nicola**	PADOUE	ITALIE	Intravital 3D bioprinting approach for muscle stem cell delivery
FUKADA	**So-Ichiro**	SUITA	JAPON	Identification of factors inducing MuSC expansion from overloaded muscle
LI	**Zhenlin**	PARIS	FRANCE	Involvement of synemin in cardiomyopathies using human induced pluripotent stem cells (hiPSC) and cell type-specific knock-out murine models
MODJTAHEDI	**Nazanine**	VILLEJUIF	FRANCE	Regulation of the AIF/CHCHD4-dependent mitochondrial import pathway in human skeletal muscle stem cells
OLGUIN	**Hugo**	SANTIAGO	CHILI	Nedd4-1 promotes regeneration by regulating autophagy in differentiating muscle stem cells.
TIMMERMAN	**Vincent**	ANVERS	BELGIQUE	Development of a neuromuscular system for testing therapeutic molecules in axonal Charcot-Marie-Tooth neuropathy
ZAMMIT	**Peter**	LONDRES	ROYAUME-UNI	Induced Pluripotent Stem Cell models to decipher pathomechanisms in FSHD

Commission thématique : Thérapie Génique et/ou Cellulaire des Maladies Rares

Aides aux jeunes chercheurs post-doctorants

AKINYELE	**Oluwaseun**	PITTSBURGH	USA	Development of a Gene Therapy Approach in an Inborn Error of Polyamine Metabolism
ALMEIDA	**Camila**	COLUMBUS	USA	Evaluation of AAV9.U7snRNAs in Myotonic Dystrophy type 1 preclinical mouse models
BOFFA	**Iolanda**	NAPLES	ITALIE	New gene therapy strategies for the treatment of gyrate atrophy of the choroid and retina (GACR), a progressive retinopathy due to the deficiency of the enzyme ornithine amino-transferase
GILLES	**Melissa**	MONTPELLIER	FRANCE	Treatment of autosomal dominant retinitis pigmentosa caused by G56R mutation in NR2E3 using CRISPR/Cas allele-specific knockout
LÉGER	**Elise**	PARIS	FRANCE	Optogenetic engineering of stem cell-derived photoreceptors to improve visual restoration
ZANIN	**Sofia**	PARIS	FRANCE	Gene therapy cure for mitochondrial disease caused by mutations in the mitochondrial RNA stability factor LRPPRC

Financements de projets Tremplins

CARRELLA	**Sabrina**	NAPLES	ITALIE	OxymiRs modulation as gene-independent therapeutic approach in rare mitochondrial diseases
DARBEY	**Annalucia**	LONDRES	ROYAUME-UNI	A Muscle Targeted Gene Therapy for Kennedy's Disease
PERDIGÃO	**Pedro**	COIMBRA	PORTUGAL	Engineering RNA-Targeting CRISPR/Cas13 for the Treatment of Spinocerebellar Ataxia Type 3
RICHARD	**Guy-Franck**	PARIS	FRANCE	Single-chain antibodies against pathogenic CTG/CUG repeats: diagnosis and therapeutic applications for myotonic dystrophy

Financements de projets

BIONDI	**Olivier**	EVRY	FRANCE	Role of precision exercise in enhancing gene therapy efficacy in Limb Girdle Muscular Dystrophies
BRUNETTI-PIERRI	**Nicola**	POUZZOLES	ITALIE	Gene therapy for Wolman disease
GOYENVALLE	**Aurélie**	MONTIGNY-LE-BRETONNEUX	FRANCE	Evaluation of HDAC inhibitors to increase dystrophin rescue in DMD following exon skipping therapy
LOLLO	**Giovanna**	VILLEURBANNE	FRANCE	Lipid nanoparticles delivery of antisense oligonucleotides to correct the pathological cause of muscular dystrophies
MOREAU-GAUDRY	**François**	PESSAC	FRANCE	Development of novel Cas9-nickases for gene therapy of rare genetic diseases: application to the congenital erythropoietic porphyria
MURO	**Andrés F.**	TRIESTE	ITALIE	Combination of mRNA-LNP and gene targeting approaches as a potential cure for early onset severe Ornithine transcarbamylase deficiency
PIGUET	**Françoise**	PARIS	FRANCE	Combined gene therapy approach for Pelizaeus Merzbacher disease
ROSSI	**Andrea**	DÜSSELDORF	ALLEMAGNE	Small molecules that enhance Prime Editing in therapeutic applications
TASFAOUT	**Hichem**	SEATTLE	USA	Expression of large dystrophins using AAV and split inteins
WEIN	**Nicolas**	COLUMBUS	USA	Preclinical evaluation of AAV.U7snRNAs in Myotonic Dystrophy type 1 mouse models

Commission thématique : Thérapie Pharmacologique des maladies neuromusculaires et Recherche Translationnelle

Financements de projets Tremplins

BERLAND	**Chloé**	PARIS	FRANCE	Novel animal behavior tracking solutions to predict neuromuscular disorders
DOBROWOLNY	**Gabriella**	ROME	ITALIE	Deconvoluting human ALS muscle environment by Spatial Transcriptomics
VILLA	**Chiara**	MILAN	ITALIE	Investigating molecular mechanisms underlying dose effects of gold nanoclusters in Friedreich Ataxia

Financements de projets

BADRISING	**Umesh**	LEIDEN	PAYS-BAS	Optimism for Inclusion Body Myositis: a double-blind randomized placebo-controlled clinical trial with Sirolimus to halt disease progression
CHARTRAND	**Pascal**	MONTREAL	CANADA	Pre-clinical testing of novel pharmacological inhibitors of toxic DMPK mRNA in a DM1 mouse model
CORDERO-ERAUSQUIN	**Matilde**	STRASBOURG	FRANCE	Counteracting cortical hyperexcitability as a therapeutical avenue for ALS
D'ANTONIO	**Maurizio**	MILAN	ITALIE	Targeting the P-eIF2alpha/PPP1R15A branch of the UPR with IFB-088 as a therapeutic strategy in adult CMT1A/B mice
DEVAUX	**Jérôme**	MONTPELLIER	FRANCE	Pathogenic mechanisms of anti-neurofascin 155 IgG4: Role of bivalency and novel therapeutic approaches.
DORCHIES	**Olivier**	GENEVE	SUISSE	Duchenne muscular dystrophy: Phenotyping and validation of better murine models for improving preclinical research and clinical translation – a step further
HORNSTEIN	**Eran**	REHOVOT	ISRAËL	microRNA biomarkers for ALS progression
LAZARIDIS	**Konstantinos**	ATHENES	GRÈCE	Preclinical study of antigen-specific tolerance induction for the treatment of myasthenia gravis
NARDO	**Giovanni**	MILAN	ITALIE	Allosteric agonism of purinergic P2X7 receptor as a pharmacological approach to enhance skeletal muscle regeneration in Spinal Bulbar Muscular Atrophy
NOGALES-GADEA	**Gisela**	BADALONE	ESPAGNE	DM1-HEART-EXTEND: Extending the search for biomarkers of heart damage in Myotonic Dystrophy 1
PHYLACTOU	**Leonidas**	NICOSIE	CHYPRE	Identifying unique characteristics of Extracellular Vesicles circulating in Myotonic Dystrophy type 1 patients and their use as blood-based biomarkers
PISTOCCHI	**Anna**	SEGRATE	ITALIE	Improving the effects of HDAC8 inhibition by combining the activation of SIRT1 in a zebrafish model of Duchenne muscular dystrophy
RYBALKA	**Emma**	MELBOURNE	AUSTRALIE	Toward the Clinic: Evaluating the long-term efficacy of re-purposed dimethyl fumarate for the treatment of Duchenne Muscular Dystrophy
SAENZ PENA	**Amets**	SAINT-SEBASTIEN	ESPAGNE	Wnt signaling pathway activation in muscular dystrophy models: a search of therapeutic use in LGMDR1 muscular dystrophy
SPINAZZI	**Marco**	ANGERS	FRANCE	THERAPEUTICAL TRIAL EFFICACY OF IMMUNE MODULATION BY LEUKOCYTE DEPLETION IN A MOUSE MODEL OF LEIGH SYNDROME

Commission thématique : Recherche Médicale

Financements de projets

BONNYAUD	**Céline**	GARCHES	FRANCE	New endpoints on neuromuscular impairments, locomotor and postural control (motor) functions in patients with Late-onset Pompe disease
SACCONI	**Sabrina**	NICE	FRANCE	New clinical outcome measures to evaluate non-ambulant FSHD patients
SERGEANT	**Nicolas**	LILLE	FRANCE	Assessment of central nervous system neurodegeneration and impact of type II diabetis in Myotonic Dystrophy type I
TASCA	**Giorgio**	ROME	ITALIE	Natural history of distal and myofibrillar myopathies assessed by clinical and technological outcome measures: a new toolbox for clinical trials
VOET	**Nicole**	NIJMEGEN	PAYS-BAS	Testing a tailored Home Exercise program to Gain insight into performance fatigability and Reduce fatigue In Patients with FSHD: THE GRIP on FSHD study
VUILLEROT	**Carole**	LYON	FRANCE	Development and validation study of a Motor Function Measure digitalized playful completion modules

Appel d'offres Doctorants

AMMAR	**Nourhene**	RENNES	FRANCE	Live-imaging of adult muscle stem cell activation in Drosophila
BATAILLARD	**Méghane**	CASTELNAU-LE-LEZ	FRANCE	Systemic gene therapy using NCS1 for treating Wolfram syndrome
BOURGETON	**Tiffany**	NANTES	FRANCE	Effect of Enzyme replacement therapy (ERT) on skeletal muscle pathophysiology in Pompe disease : specific focus on satellite cells and autophagy
D'AGATA	**Léna**	PESSAC	FRANCE	Annexins as genetic modifiers of human muscular dystrophies
DAY	**Lucie**	PARIS	FRANCE	Role of ADAR1 and RNA editing in peripheral myelin maintenance
DELAFENETRE	**Arnaud**	POITIERS	FRANCE	Functional characterization of muscle cells derived from healthy and DMD human induced Pluripotent Stem Cells: focus on calcium release channels
DELEPINE	**Georges**	OTTIGNIES-LOUVAIN-LA-NEUVE	BELGIQUE	Role of Hox proteins in the development of adult tubular muscle in Drosophila melanogaster
DELIVRY	**Léa**	PARIS	FRANCE	Analysis of the transcriptional networks and cellular communications during skeletal muscle hypertrophy
GIL HERNANDEZ	**Esther Aurora**	BURES-SUR-YVETTE	FRANCE	Functional interplay between the mitochondrial fusion and the lipid transport machineries at ER-mitochondria contact sites
GISBERT	**Vincent**	MONTPELLIER	FRANCE	Organelle alterations converge on neurodegeneration and lack of regeneration in hereditary spastic paraplegia
HAUTBOIS	**Marie**	PARIS	FRANCE	Base editing-mediated correction of recurrent mutations in COL7A1 to treat RDEB
HOUQUES	**Chloe**	MONTPELLIER	FRANCE	Combinatorial treatment with gene and cell therapy for the treatment of SCID
MAIELLANO	**Greta**	LYON	FRANCE	Role of the TMED proteins in acetylcholine receptor biosynthesis and implication in neuromuscular diseases
PHONGSAVANH	**Xaysongkhame Micky**	MONTIGNY-LE-BRETONNEUX	FRANCE	Evaluation of HDAC inhibitors to increase dystrophin rescue in DMD following exon skipping therapy
ROUÉ	**Clémence**	MONTPELLIER	FRANCE	Autoimmune Nodopathy : DEtermine the RoleS Of ADAM10/17 in Neuropathy
ROUSSET	**Célia**	MARSEILLE	FRANCE	Defining the unique developmental program of the trapezius muscle
RUBENS	**Paula**	PARIS	FRANCE	DNA Methylation Landscape of Normal, Mitochondrial Mutated, and Three-Parent Preimplantation Embryos
THIBAULT	**Chloé**	BORDEAUX	FRANCE	Development of novel Cas9-nickases for gene therapy of rare genetic diseases: application to the congenital erythropoietic porphyria
VAHDAT	**Juliette**	MARSEILLE	FRANCE	Exploring ventricular conduction system structure and function in DMD mouse models
VAUCOURT	**Mathilde**	TOULOUSE	FRANCE	Selective autophagy in X-linked centronuclear myopathy: molecular mechanisms and pathophysiological relevance

Projet soumis à l'appel d'offres thématique ARN médicament et cible

NAMY	**Olivier**	ORSAY	FRANCE	Translectin: A new termination codon readthrough inducer to expand therapeutics choices for nonsense genetic diseases

Projets soumis à l'appel d'offres thématique Médecine Mitochondriale

DELEIDI	**Michela**	PARIS	FRANCE	NAD supplementation for mitochondrial disease therapy
DELETTRE-CRIBAILLET	**Cécile**	MONTPELLIER	FRANCE	Development of novel molecule derived from polyamines to treat mitochondrial optic neuropathies
LEPELLEY	**Alice**	PARIS	FRANCE	Anti-interferon therapeutic strategies for primary mitochondrial disorders
MARTI	**Ramon**	BARCELONE	ESPAGNE	Deoxynucleosides as a potential therapy for mitochondrial DNA maintenance disorders
PRIGIONE	**Alessandro**	DÜSSELDORF	ALLEMAGNE	iPSC-driven repositioning of Sildenafil for treating Mitochondrial Disorders
PROCACCIO	**Vincent**	ANGERS	FRANCE	Therapeutic strategies targeting mitochondrial dysfunction and inflammation in mitochondrial diseases
TRIFUNOVIC	**Aleksandra**	COLOGNE	ALLEMAGNE	REgulation of CLPP protease as a treatment for MItochondrial DIseases

Partenariats institutionnels

BOUKHATMI	**Hadi**	PARIS	FRANCE	Programme ATIP-Avenir
DUPONT	**Jean-Baptiste**	PARIS	FRANCE	Programme ATIP-Avenir
KIM	**Minchul**	PARIS	FRANCE	Programme ATIP-Avenir
LAUGEL	**Vincent**	STRASBOURG	FRANCE	Cohorte de suivi longitudinal SMA-DNN: Dépistage néonatal de l'Amyotrophie Spinale - Projet pilote SMA-DNN France

Partenariats associatifs

MUSCULAR DYSTROPHY UK		LONDRES	ROYAUME-UNI	Third year of the Collagen VI Alliance Call : - Trial readiness for Collagen VI Myopathies - STRAUB Volker
SMA Europe		CHIPPING CAMPDEN	ROYAUME-UNI	11th international Call for SMA Research Projects
Cure CMD		TORRANCE	USA	Targeting Primary Cilium-Related Molecular Pathways to Correct Tendon Cell Defects in Collagen VI Congenital Muscular Dystrophies - CENNI Vittoria
IRME		PARIS	FRANCE	Aging and Controlling the Fate of Human Spinal Cord stem cells - HUGNOT Jean-Philippe
RETINA France		COLOMIERS	FRANCE	- Approches de thérapie oligonucléotidique antisens et de thérapie génique pour le traitement des maladies dégénératives rétiniennes en ciblant la voie glycogène synthase kinase 3 - ROGER Jérôme '- Régénération de la rétine : interaction entre les cellules de Müller et la microglie - PERRON Muriel
Vaincre La Mucoviscidose (VLM)		PARIS	FRANCE	Appel à projets scientifiques 2023 : - Evaluation d'une stratégie thérapeutique à base d'oligonucléotides pour des patients CF non éligibles aux modulateurs CFTR - CADARS Magali - AV6.2FF : nouvelle opportunité de thérapie génique à tropisme pulmonaire pour traiter tous les patients atteints de mucoviscidose - TABARY Olivier
Vaincre les Maladies Lysosomales (VML)		MASSY	FRANCE	Appel à projets de recherche 2023 : Enzyme replacement therapy by cell encapsulation for metachromatic leukodystrophy - PIGUET Françoise

Projets soumis en dehors du calendrier AO ou sortis de l'AO pour des raisons de PI

BRUNETEAU	**Gaelle**	PARIS	FRANCE	Cell signaling, Reinnervation and Metabolism in Amyotrophic Lateral Sclerosis (CERMALS)

Projets Ignition

BUNING	**Hildegard**	HANNOVRE	ALLEMAGNE	Rational-designed enveloped AAV: a novel all-in-one gene transfer system
DECRESSAC	**Michael**	LA TRONCHE	FRANCE	Towards gene replacement tHErapy for seveRE mitochondrial diSEase
DUMONT	**Nicolas**	MONTREAL	CANADA	Muscle stem cell defects in Myotonic Dystrophy Type 1: physiopathology and therapeutic avenues
FERNANDEZ-COSTA	**Juan M.**	BARCELONE	ESPAGNE	Monitoring of fibrotic processes in 3D skeletal muscle co-cultures for Muscular Dystrophies using plasmonic biosensors
MUSARO	**Antonio**	ROME	ITALIE	Understanding the contribution of molecular mediators of inflammation to DMD progression and implications for therapy: studying the impact of IL-6 transignaling on dystrophic muscle stability
PALACIOS	**Daniela**	ROME	ITALIE	Functionalized nanoparticles for targeted genome editing in Duchenne Muscular Dystrophy
SUMARA	**Izabela**	ILLKIRCH	FRANCE	Therapeutic dissolution of aberrant nucleoporin condensation in Fragile X syndrome using gene therapy approaches
VISCOMI	**Carlo**	PADOUE	ITALIE	Harnessing mitophagy to treat mitochondrial myopathies

ACTIONS STRATEGIQUES

Projets stratégiques

AGBULUT	**Onnik**	PARIS	FRANCE	Innovative bio-inspired cell and tissue models of genetic dilated cardiomyopathy for disease modelling and drug discovery
BOMONT	**Pascale**	LYON	FRANCE	Therapy for Giant Axonal Neuropathy
CORRAL-DEBRINSKI	**Marisol**	PARIS	FRANCE	Neuroglobin gene overexpression: a promising tool for treating a large spectrum of neurological disorders thanks to its ability to safely and sustainably preserve mitochondrial integrity
DUMONCEAUX	**Julie**	LONDRES	ROYAUME-UNI	DUX4 in FSHD: pathophysiology and therapeutic approaches
GALY	**Anne**	CORBEIL-ESSONNES	FRANCE	Sickle cell disease gene therapy program
HUDA	**Ruksana**	GALVESTON	USA	Developing mAb-siRNA conjugate for therapy of myasthenia gravis
LAPORTE	**Jocelyn**	ILLKIRCH	FRANCE	Pathophysiology and therapeutic proof-of-concepts for congenital myopathies
MALFATTI	**Edoardo**	CRÉTEIL	FRANCE	UPHoID - Ultrarare PYROXD1, ACTN2, and FHL1 congenital-myopathies Pharmacological treatment through HiPSC-based Drug repurposing
OLIVIER	**Sophie**	EVRY	FRANCE	Preclinical and clinical development of gene therapy candidates for the treatment of LGMD-R9, LGMD-R5 and LGMD-R1
OLIVIER-FAIVRE	**Laurence**	DIJON	FRANCE	PERIGENOMED-CLINICS 1: a pre-pilot to assess the feasibility and acceptability of newborn screening using panel-based genome sequencing in France
POURQUIE	**Olivier**	BOSTON	USA	Toward cell therapy for Duchenne Muscular Dystrophy: characterization of regenerative potential of hIPS derived Pax7+ cells
PROCACCIO	**Vincent**	ANGERS	FRANCE	Identifying candidate drugs in mitochondrial cardiomyopathies:From Mouse to Human
STURTZ	**Franck**	LIMOGES	FRANCE	NeuroCur-2 project - Pursuing the development of a curcumin-based nanoparticle treatment for patients with peripheral neuropathies
VAILLEND	**Cyrille**	ORSAY	FRANCE	DECODING CENTRAL DEFECTS IN DYSTROPHINOPATHIES : FROM DIAGNOSTIC TO REMEDIATION
VILQUIN	**Jean-Thomas**	PARIS	FRANCE	Comparative study of iPSC-derived myogenic precursors proposed for cell therapy in a model of Duchenne muscular Dystrophy

Pôles stratégiques

LACAMPAGNE	**Alain**	MONTPELLIER	FRANCE	Liste des pathologies principalement étudiées : Dystrophies musculaires : DMD / BMD / FHSD ; Titinopathies ; Syndrome de pseudo-obstruction intestinale chronique ; Dyskinésie ciliaire primitive ; Mucoviscidose ; Sarcopénie
MAGDINIER	**Frédérique**	MARSEILLE	FRANCE	Liste des pathologies principalement étudiées : Pathologies liées au gène STIM1 ; Dysferlinopathies ; CMT ; FSHD ; Cardiomyopathies génétiques (LMNA ; Rasopathies…) ; Encephalopathie épileptique KCQN2 ; Syndrome de Rett ; Pathologies du vieillissement (Progeria, progeria like, MADaM syndrome, Hallermann-Streiff syndrome…) ; David syndrome
RELAIX	**Frédéric**	CRETEIL	FRANCE	Liste des pathologies principalement étudiées : Dystrophies musculaires : DMD / FSHD ; SMA ; Myopathies congénitales ; Myopathies inflammatoires
SCHAEFFER	**Laurent**	LYON	FRANCE	Liste des pathologies principalement étudiées : Dystrophies musculaires : DMD / DM1 ; Myopathies inflammatoires ; Myopathie centronucléaire ; Myopathies congénitales / Myopathie congénitale à « central cores » ; Hyperthermie maligne ; Ataxies, dont Ataxie de Friedreich ; Maladies du motoneurone (SLA / SMA / CMT) ; Myasthénie ; Neuropathies sensorielles

Structures stratégiques

FONDATION MALADIES RARES	**Fmr**	PARIS	FRANCE	Appel à projets auprès des Filières de Santé Maladies Rares : '- Orai1 Ca2+ channel: a new therapeutic target in PAH - ANTIGNY Fabrice '- Promoting regenerative inflammation to improve muscle homeostasis in Duchenne Muscular Dystrophy - CHAZAUD Bénédicte '- Consolidation of an innovative strategy for the treatment of calcitriol-mediated hypercalcemia - LAVERNY Gilles '- Inducing albumin-mediated recycling of endogenous proteins: a novel strategy to treat quantitative deficiencies of plasma proteins - LENTING Peter '- HDAC6 inhibitors to treat Duchenne Muscular Dystrophy - SCHAEFFER Laurent
FONDATION MALADIES RARES	**Fmr**	PARIS	FRANCE	Subvention 2023
GENOPOLE	**Genopole**	EVRY	FRANCE	Participation financière de l'AFM au budget 2023 du GIP GENOPOLE

Outils stratégiques

BASSEZ	**Guillaume**	PARIS	FRANCE	International Myotonic Dystrophy Registry - iDM-Scope
SACCONI	**Sabrina**	NICE	FRANCE	French national registry for FacioScapuloHumeral muscular Dystrophy (FSHD)

Plateformes stratégiques

SOLE	**Guilhem**	BORDEAUX	FRANCE	Plateformes d'essais thérapeutiques Pédiatriques et Adultes Neuromusculaires - BORDEAUX

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

BOUTHEMY	**Mathieu**	PARIS	FRANCE	SLA & maladies du motoneurone : quelles pistes pour demain ?
CHARRETON	**Amandine**	RENNES	FRANCE	Séminaire de Génétique de l'Ouest - "Thérapies et enjeux sociétaux"- 14&15 septembre 2023 (Le Croisic)
CHAZAUD	**Bénédicte**	LYON	FRANCE	Final Meeting of the European Network RENOIR "From tissue repair to Bio-engineering in skeletal and cardiac muscles"
GOMEZ	**Ana Maria**	CHÂTENAY-MALABRY	FRANCE	Gordon Research Conference : "Muscle: excitation-contraction coupling"
KAN	**Hermien**	LEIDEN	PAYS-BAS	Fourth International Imaging in Neuromuscular Disease Conference 2023
KINOSHITA	**June**	LEXINGTON	USA	30th Annual International Congress on FSHD
LAFUSTE	**Peggy**	CRETEIL	FRANCE	From basic discovery to preclinical modeling: at the forefront of muscle disease therapies
MOURIKIS	**Philippos**	CRETEIL	FRANCE	Frontiers in Myogenesis Conference Advances in Skeletal Muscle Growth, Repair and Disease
PEREZ DE CASTRO	**Ignacio**	MADRID	ESPAGNE	4th International Meeting on Laminopathies
SORCI	**Guglielmo**	PEROUSE	ITALIE	20th IIM international meeting
VAN ENGELEN	**Baziel**	NIJMEGEN	PAYS-BAS	IDMC-14 International myotonic dystrophy symposium number 14

Plateformes

DESVIGNES	**Cyril**	MEZILLES	FRANCE	Financement CEDS