

LISTE DES FINANCEMENTS ACCORDES PAR L'AFM-TELETHON EN 2022

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'appel d'offres annuel

Commission thématique : Myologie fondamentale

Aides aux jeunes chercheurs post-doctorants

BIANCONI	Valeria	ROME	ITALIE	Deciphering the role of Prdm16-mediated H3K9 methylation in the control of Fibro-Adipogenic Progenitors identity and skeletal muscle repair
MAO	Qiyang	MARSEILLE	FRANCE	Muscle building: dissecting mechanisms of tension-controlled self-organisation in human iPSC-derived skeletal muscles

Financements de projets Tremplins

CARNESECCHI	Julie	LYON	FRANCE	Unravelling the function of TF-splicing network in muscle development
KIM	Minchul	ILLKIRCH	FRANCE	Dissecting developmental mechanisms of nuclear fates in skeletal muscle syncytium
NOGARA	Leonardo	PADOVA	ITALIE	Pharmacological regulation of the OFF state of myosin in skeletal muscle

Financements de projets

BLAAUW	Bert	PADOVA	ITALIE	Identification of muscle-specific factors involved in NMJ maintenance and their regulation by mTORC1
BORYCKI	Anne-Gaëlle	SHEFFIELD	ROYAUME UNI	Patched 1 function in skeletal muscle stem cells and its implication in muscle wasting
DUPREZ	Delphine	PARIS	FRANCE	Regionalisation of myogenesis
FAURE	Sandrine	MONTPELLIER	FRANCE	NEURONAL REGULATION OF INTESTINAL SMOOTH MUSCLE CELL DIFFERENTIATION AND FUNCTIONALITY
GOMES	Edgar	LISBOA	PORTUGAL	A role for the microenvironment on nuclear positioning in myofibers
HUBE	Florent	PARIS	FRANCE	SNORNAs in normal and pathological human muscle
KELLY	Robert G.	MARSEILLE	FRANCE	Defining the unique developmental program of the trapezius muscle at the head trunk interface
MAIRE	Pascal	PARIS	FRANCE	Fast myosin heavy chain locus regulation
MENZIES	Keir	OTTAWA	CANADA	Examining the role and therapeutic potential of Poly-ADP-Ribosylation (PARYlation) in myopathies and skeletal muscle maintenance and function
MIQUEROL	Lucile	MARSEILLE	FRANCE	Exploring ventricular conduction system structure and function in the regenerating heart and DMD mouse models
ROMANELLO	Vanina	PADOVA	ITALIE	The in vivo role of peroxisomes in the control of muscle function
SOTIROPOULOS	Athanassia	PARIS	FRANCE	Functional cell heterogeneity underlying muscle plasticity
STRICKER	Sigmar	BERLIN	ALLEMAGNE	Analysis of the NF1/RAS/MAPK pathway in muscle regeneration
VÉNIEN-BRYAN	Catherine	PARIS	FRANCE	Structural and cellular characterization of a potassium channel, Kir, involved in Andersen's syndrome
VOLK	Talila	REHOVOT	ISRAËL	The contribution of the LINC complex to epigenetic regulation of chromatin in mature muscle fibers

Commission thématique : Bases Moléculaires et Physiopathologie des Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

APOLLONI	Savina	ROMA	ITALIE	Targeting S100A4 to study the macrophages-muscle cells cross-talk in models of Duchenne Muscular Dystrophy
BERARDI	Andrea	MILANO	ITALIE	Molecular studies of DUX4/MATR3 complex: a therapy against FSHD
LÉGARÉ	Cécilia	CHICOUTIMI - QUEBEC	CANADA	Identification of molecular signatures associated to strength training in myotonic dystrophy type 1
RUPARELIA	Avnika	CLAYTON, VICTORIA	AUSTRALIE	Identification of therapies for Collagen V1-related congenital muscular dystrophy

Financements de projets Tremplins

DE PALMA	Clara	MILANO	ITALIE	Targeting Drp1 activation and mitochondrial Fission to Curtail muscle wasting during Duchenne Muscular Dystrophy progression
TUFFERY-GIRAUD	Sylvie	MONTPELLIER	FRANCE	Comprehensive epitranscriptomic profiling in Duchenne Muscular Dystrophy

Financements de projets

AMTHOR	Helge	MONTIGNY-LE-BRETONNEUX	FRANCE	The syncytial organization of dystrophin mosaic in female mdx carrier mice
CENACCHI	Giovanna	BOLOGNA	ITALIE	Role of TNPO3 in the pathogenetic mechanism of LGMD D2: comparison between disease models
COWAN	Kyle	OTTAWA	CANADA	Defining the Role and Therapeutic Potential of Pannexin 1 Channels in Duchenne Muscular Dystrophy using Mouse Models and Patients Cells.
D'AMBROSI	Nadia	ROMA	ITALIE	Targeting S100A4 to improve inflammation and fibrosis in Duchenne Muscle Dystrophy
DE GREEF	Jessica	LEIDEN	PAYS-BAS	Muscle-on-chip: a novel approach for studying DUX4-induced events in FSHD
FARINI	Andrea	MILANO	ITALIE	Unsolved challenges in gut-muscle axis: the role of microbiota in mediating immune pathology in murine models of Duchenne muscular dystrophy
GABELLINI	Davide	MILANO	ITALIE	Structure-Function characterization of a novel DUX4 inhibitor to develop a drug-like treatment for FSHD muscular dystrophy
LANZUOLO	Chiara	MILANO	ITALIE	Exploring the genetic and epigenetic background underlying the phenotype's variability in Emery Dreifuss Muscular Dystrophy
MARTELLI	Fabio	MILAN	ITALIE	Circular RNA role in Myotonic Dystrophy type 1
MAZZONE	Massimiliano	TORINO	ITALIE	GLUD1 as a potential target in Muscular Dystrophy
MORESI	Viviana	ROME	ITALIE	Unveiling the cytoplasmic functions of HDAC4 in dystrophic skeletal muscle
PARROT	Sandrine	BRON CEDEX	FRANCE	Alterations in brain glutamate and GABA neurotransmissions in DM1 disease: focus on neuronal subpopulations and related electrophysiological signals
PREVITALI	Stefano Carlo	MILANO	ITALIE	Modulation of modifier genes to improve muscle regeneration and metabolism in LAMA2-RD animal models
RAVEL-CHAPUIS	Aymeric	OTTAWA	CANADA	Role of the RNA-binding protein HuR in Myotonic Dystrophy type 1
SACCONI	Valentina	ROMA	ITALIE	The RNA-Binding Protein hnRNP A2B1 is a key protein controlling microRNA sorting into Fibro-Adipogenic Progenitor derived Extracellular Vesicles, crucial for muscle regeneration in Duchenne Muscular Dystrophy
SUELVES	Monica	BADALONA	ESPAGNE	EXPLORING HDAC11 FUNCTIONS IN DUCHENNE MUSCULAR DYSTROPHY

Commission thématique : Bases moléculaires et physiopathologie des autres maladies neuromusculaires

Aides aux jeunes chercheurs post-doctorants

LIONELLO	Valentina Maria	LONDRES	ROYAUME UNI	Complex modeling of myotubularin myopathy using human bioengineered skeletal muscles
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Financements de projets Tremplins

LESCURE	Alain	STRASBOURG	FRANCE	Characterization of selenoprotein N catalytic activity, a key step forward a better understanding of SELENON-related myopathy physiopathology
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Financements de projets

BENARD	Giovanni	BORDEAUX	FRANCE	Studying the ribosomal-associated quality control in a mitochondrial syndrome
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 rhabdomyolysis
DEVAL	Emmanuel	VALBONNE	FRANCE	ACID-SENSING ION CHANNEL 3 IN THE PATHOPHYSIOLOGY OF CHRONIC MUSCLE PAIN
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
HEARD	Edith	HEIDELBERG	ALLEMAGNE	Allele-specific epigenetic regulation of Bag3: physiopathological implications and new strategies for dilated cardiomyopathy
MITRANI-ROSENBAUM	Stella	JERUSALEM	ISRAËL	Cellular Models to explore GNE functions in muscle
STEFFANN	Julie	PARIS	FRANCE	MITOCARE: Mitochondrial replacement therapy (MRT) against mitochondrial DNA (mtDNA) disorders: are we far from its clinical application?

Commission thématique : Système nerveux - Motoneurone et jonction neuromusculaire

Aides aux jeunes chercheurs post-doctorants

CARPENTIER	Rémi	PARIS	FRANCE	Identifying key signaling events in motor circuit connectivity that rely on spastin activity: a starting point toward the development of novel therapeutic strategies in SPG4-linked spastic paraplegia
GAMBAROTTO	Lisa	PADOVA	ITALIE	Targeting Beclin1 interactome in demyelinating neuropathies
JHA	Narendra	NEW YORK	USA	Identification of modifier gene(s) of spinal muscular atrophy in model mice
LAURIA	Fabio	POVO (TRENTO)	ITALIE	Towards reconciling splicing and translational defects in Spinal Muscular Atrophy
REICHOVA	Alexandra	BORDEAUX	FRANCE	Oxytocin and 5-HT involvement in early dysregulation of the chloride homeostasis in spinal ALS motoneurons

Financements de projets Tremplins

RE CECCONI	Andrea David	MILANO	ITALIE	Dissecting the role of the myokine musclin against amyotrophic lateral sclerosis-induced muscle atrophy
ZUCCARO	Emanuela	PADOVA	ITALIE	Investigating motor neuron vulnerability in Spinal-Bulbar Muscular Atrophy (SBMA)

Financements de projets

BESSEREAU	Jean-Louis	LYON	FRANCE	Genetic control of acetylcholine receptor expression: from new mechanisms to functional genomics
BOMONT	Pascale	MONTPELLIER	FRANCE	Neurofilaments in Health and Charcot-Marie Tooth diseases
BONDURAND	Nadège	PARIS	FRANCE	Role of ADAR1 and RNA editing in Schwann cells development and myelin maintenance
BORTOLOZZI	Mario	PADOVA	ITALIE	Linking Cx32 hemichannel dysfunction to Charcot-Marie-Tooth disease pathogenesis.
CARRA	Serena	MODENA	ITALIE	Unraveling HSPB3 physiological functions to understand its implication in neuromuscular diseases
CASSEREAU	Julien	ANGERS	FRANCE	Metabolomic exploration of dysregulated lipid metabolism in MFN2-related CMT2A
D'ANTONIO	Maurizio	MILANO	ITALIE	Axonal degeneration in CMT2J/1 neuropathies: molecular mechanisms and therapeutic strategies
DÍAZ-MANERA	Jordi	NEWCASTLE	ROYAUME UNI	Transcript-MND: Transcribing the process of muscle degeneration in patients with motor neuron diseases
DUPUIS	Luc	STRASBOURG	FRANCE	Muscle contribution to FUS-associated ALS: genetic and mechanistic insights
FASSIER	Coralie	PARIS	FRANCE	Preventing motor neuron degeneration associated with spastin haploinsufficiency through TLL-mediated tubulin polyglutamylation
KREJCI	Eric	PARIS	FRANCE	Congenital myasthenic syndrome: acetylcholine and GABA
LEFEBVRE	Suzie	PARIS	FRANCE	Identification and regulation of neuromuscular pathways and its implication as biomarkers/targets in spinal muscular atrophy (Acronym: SMA-path-ID)
LIEVENS	Jean-Charles	MONTPELLIER	FRANCE	Deciphering the beneficial effects of Sigma-1 receptor in amyotrophic lateral sclerosis
MADARO	Luca	ROMA	ITALIE	Retinoic acid signalling in the regulation of muscle innervation
MANGIN	Jean-Marie	PARIS	FRANCE	Understanding how the electrical activity of midline radial glia participates to neuromuscular development and function
NEDELEC	Stéphane	PARIS	FRANCE	Axonal transport in motor neuron disease: Investigating the deregulation of vesicular transport and its consequences in SMA-LED using targeted differentiation of human iPSC cells into affected and preserved motor neuron subtypes
PENNUITO	Maria	PADOVA	ITALIE	Development of a therapeutic strategy to suppress LSD1 and PRMT6-mediated toxic gain of function in SBMA
ROJO	Manuel	BORDEAUX	FRANCE	Functional diagnosis of the pathogenicity of MFN2 variants identified in patients with CMTs
TARESTE	David	PARIS	FRANCE	Core Molecular Mechanisms and Lipid Determinants of Mitofusin-mediated Mitochondrial Fusion
TAWK	Marcel	LE KREMLIN BICÊTRE	FRANCE	Dissect the role of ADCY6 in arthrogryposis and peripheral myelination
VIERO	Gabriella	POVO (TRENTO)	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

ALESSANDRINI	Francesco	CHICAGO	USA	Develop an iPSC-based platform for interrogating sporadic ALS disease proteome compartmentalization
GALLAY	Laure	GENEVA	SUISSE	Pivotal role of muscle stem cells in idiopathic inflammatory myopathies pathogenesis.
SANDONÀ	Martina	ROME	ITALIE	Study of FAPs-derived Extracellular vesicles ability to restore dystrophic muscle integrity upon systemic administration: a new piece of the puzzle.

Financements de projets Tremplins

SUAREZ CALVET	Xavier	BARCELONA	ESPAGNE	Spatial biology approach in dmd to dissect the macrophages-fap microniche
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Financements de projets

AMTHOR	Helge	MONTIGNY-LE-BRETONNEUX	FRANCE	The role of dystrophin in establishing the satellite cell niche
BIRESSI	Stefano	TRENTO	ITALIE	Novel satellite cell heterogeneity in healthy and pathological regeneration
BURELLE	Yan	OTTAWA	CANADA	Mitochondrial Quality Control in Muscle Stem Cells: A Determinant of Cell Fate Decision and Tissue Repair Capacity
DUMONT	Nicolas	MONTREAL	CANADA	Muscle stem cell defects in Myotonic Dystrophy Type 1: physiopathology and therapeutic avenues
DUTEIL	Delphine	ILLKIRCH	FRANCE	Role of androgens in muscle regeneration
FUKADA	So-Ichiro	SUITA	JAPON	Identification of factors inducing MuSC expansion from overloaded muscle
MAYEUF-LOUCHARTE	Alicia	LILLE	FRANCE	The circadian clock of muscle stem cells
SACCONI	Valentina	ROME	ITALIE	Therapeutic potential of Amniotic Mesenchymal Stromal Cells and their released extracellular vesicles in the treatment of Duchenne Muscular Dystrophy.
TIMMERMAN	Vincent	ANTWERPEN	BELGIQUE	Development of a neuromuscular system for testing therapeutic molecules in axonal Charcot-Marie-Tooth neuropathy

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

Aides aux jeunes chercheurs post-doctorants

GILLES	Melissa	MONTPELLIER	FRANCE	Treatment of autosomal dominant retinitis pigmentosa caused by G56R mutation in NR2E3 using CRISPR/Cas allele-specific knockout
POGGI	Lucie	PARIS	FRANCE	Gene editing of p110delta immunodeficiencies
TASFAOUT	Hichem	SEATTLE	USA	Expression of large dystrophins using Intein-mediated protein trans-splicing
ZANIN	Sofia	PARIS	FRANCE	Gene therapy cure for mitochondrial disease caused by mutations in the mitochondrial RNA stability factor LRPPRC

Financements de projets Tremplins

MALERBA	Alberto	EGHAM	ROYAUME UNI	Enhancing muscle strength in muscular dystrophies through RPL3L inhibition
SUMARA	Izabela	ILLKIRCH	FRANCE	Therapeutic dissolution of aberrant nucleoporin condensation in Fragile X syndrome using gene therapy approaches
GIOVARELLI	Matteo	MILANO	ITALIE	Metabolic activation FO for supporting micro-dystrophin gene therapy in DMD

Financements de projets

GOYENVALLE	Aurélié	MONTIGNY-LE-BRETONNEUX	FRANCE	Evaluation of HDAC inhibitors to increase dystrophin rescue in DMD following exon skipping therapy
RUZZENENTE	Benedetta	PARIS	FRANCE	Gene therapy cure for mitochondrial disease caused by mutations in the mitochondrial RNA stability factor LRPPRC
ABERDAM	Daniel	PARIS	FRANCE	NON-INTEGRATIVE TRANSDIFFERENTIATION FOR CORNEAL THERAPIES
MOREAU-GAUDRY	François	BORDEAUX	FRANCE	Development of novel Cas9-nickases for gene therapy of rare genetic diseases: application to the congenital erythropoietic porphyria
PARENTI	Giancarlo	NAPOLI	ITALIE	Targeting secondary dysregulation of cellular pathways and functions to improve gene therapy for Pompe Disease
CONCORDET	Jean-Paul	PARIS	FRANCE	Novel prime editing strategies for gene therapy and their testing in beta-hemoglobinopathies
MINCZUK	Michal	CAMBRIDGE	ROYAUME UNI	In vivo correction of mitochondrial genome by base editing: towards therapies for neuromuscular diseases caused by mitochondrial DNA dysfunction
BRUNETTI-PIERRI	Nicola	POZZUOLI	ITALIE	Gene therapy for Wolman disease
BIONDI	Olivier	EVRY	FRANCE	Role of precision exercise in enhancing gene therapy efficacy in Limb Girdle Muscular Dystrophies
BETUING	Sandrine	PARIS	FRANCE	Efficient Gene and Sterol regulations by gene transfer for striatal protection in Huntington's Disease
KALATZIS	Vasiliki	MONTPELLIER	FRANCE	CRISPR/Cas genome-editing to treat autosomal dominant retinitis pigmentosa caused by the G56R mutation in NR2E3: pre-clinical proof-of-concept in human retinal organoids
LATTANZI	Wanda	ROMA	ITALIE	Personalized non-invasive nanotherapy of Crouzon syndrome through FGFR2 gene knock-down by recombinant human ferritin-based targeted siRNA delivery

Commission thématique : Thérapie Pharmacologique des maladies neuromusculaires et Recherche Translationnelle

Aides aux jeunes chercheurs post-doctorants

BRAZ	Sandra	COIMBRA	PORTUGAL	Evaluating the potential of iPSC-derived neuroepithelial stem cells depleted for mutant ataxin-3 for Machado-Joseph disease treatment
DEBRUT	Léa	ILLKIRCH	FRANCE	Evaluation of proteasome inhibitors efficacy in preclinical models of dermatomyositis

Financements de projets Tremplins

BOWERMAN	Melissa	STAFFORDSHIRE	ROYAUME UNI	Muscle extracellular vesicles as biomarkers for spinal muscular atrophy pathology and therapies
PALLAFACCHINA	Giorgia	PADOVA	ITALIE	Small-molecule-based therapeutic approach for distal hereditary motor neuropathy caused by Sigma-1R mutations

Financements de projets

BRICHARD	Sonia	BRUXELLES	BELGIQUE	Adiponectin and its mimics for the treatment of muscular dystrophies
BRYSON-RICHARDSON	Robert	MELBOURNE	AUSTRALIE	Pre-clinical drug screen for LAMA2 congenital muscular dystrophy
BURATTI	Emanuele	TRIESTE	ITALIE	Novel therapeutic targets derived from modulation of RNA metabolism in late onset Pompe disease
CORDERO-ERAUSQUIN	Matilde	STRASBOURG	FRANCE	Counteracting cortical hyperexcitability as a therapeutic avenue for ALS
DEVAUX	Jérôme	MONTPELLIER	FRANCE	Pathogenic mechanisms of anti-neurofascin 155 IgG4: Role of bivalency and novel therapeutic approaches.
DI SCHIAVI	Elia	NAPOLI	ITALIE	A combinatorial pharmacotherapeutic approach to counteract Spinal Muscular Atrophy
KAKHLON	Or	JERUSALEM	ISRAËL	Testing new glycogen reducing small molecules for the treatment of glycogen storage disorder type 3
LATELLA	Lucia	ROMA	ITALIE	Serpine1 inhibitor as therapeutic tool in the treatment of DMD
LEVY-LAHAD	Ephrat	JERUSALEM	ISRAËL	VRK1-related motor neuron disease: biological models for drug repurposing
MESSINA	Graziella	MILAN	ITALIE	Drug repurposing of ERK inhibitors to target the transcription factor Nfix in dystrophic muscles: development of a new proof-of-concept study to hinder Muscular Dystrophies
PHYLACTOU	Leonidas	NICOSIA	CHYPRE	Identifying unique characteristics of Extracellular Vesicles circulating in Myotonic Dystrophy type 1 patients and their use as blood-based biomarkers
RYBALKA	Emma	MELBOURNE	AUSTRALIE	Toward the Clinic: Evaluating the long-term efficacy of re-purposed dimethyl fumarate for the treatment of Duchenne Muscular Dystrophy
SANDONÀ	Dorianna	PADOVA	ITALIE	CFTR correctors to treat sarcoglycanopathy, a repurposing story.
VAN DEN BOSCH	Ludo	LEUVEN	BELGIQUE	Identification of therapies targeting lipid metabolism & myelination for Charcot-Marie-Tooth disease type 1A using patient derived Schwann cells
VENEREAU	Emilie	MILAN	ITALIE	Preclinical Evaluation of a Designer HMGB1 as a Drug Candidate for Duchenne Muscular Dystrophy

Commission thématique : Recherche Médicale

Financements de projets

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
VOORN	Eric	AMSTERDAM	PAYS-BAS	B-FIT! A guide to individualized exercise in slowly progressive neuromuscular diseases

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
BEAUJARD	Bettina	PARIS	FRANCE	L'annonce diagnostique d'une maladie neuromusculaire à l'âge adulte. Retentissements psychologiques des processus communicationnels entre médecin et patient
BONNOT	Chloé	LYON	FRANCE	Fusion of blood cells to skeletal muscle as a therapeutic strategy for inherited muscle diseases
BOUCHARD	Laetitia	MARSEILLE	FRANCE	Evaluation of FGF10 as a target for promoting heart regeneration in Lmna-related dilated cardiomyopathy
BRUNET	Marine	LYON	FRANCE	Etude de la fonction des gènes à domaine ALMS dans la biogenèse du centriole et du cil chez Drosophila melanogaster
CHALUMEAU	Anne	PARIS	FRANCE	Development of a universal prime editing approach to β -hemoglobinopathies.
CHEVREAU	Robert	MONTPELLIER	FRANCE	Role of the Hippo/YAP pathway in the maintenance, proliferation and differentiation of the human and mouse spinal cord stem cells
D'AGATA	Léna	PESSAC	FRANCE	Annexins as genetic modifiers of human muscular dystrophies
DELAFENETRE	Arnaud	POITIERS	FRANCE	Functional characterization of muscle cells derived from healthy and DMD human induced Pluripotent Stem Cells: focus on calcium release channels
DUMAIRE	Nicolas	LYON	FRANCE	Rôle des protéines TMED dans la synthèse des récepteurs de l'acétylcholine et implications dans les pathologies de la jonction neuromusculaire
DUMAS	Camille	MARSEILLE	FRANCE	Regulatory networks governing craniofacial muscles diversity
HOUQUES	Chloe	MONTPELLIER	FRANCE	Combinatorial treatment with gene and cell therapy for the treatment of SCID
JAULIAC	Edgar	PARIS	FRANCE	Regulation of the fast Myh locus
JOHARI	Mridul	PERTH	AUSTRALIA	Improved diagnostics and gene discovery for unsolved myopathies
KOTAICH	Farah	MONTPELLIER	FRANCE	Neurofilaments in health and Charcot-Marie-Tooth diseases
LAFONT	Agathe	PARIS	FRANCE	Role of midline radial glia excitability in the motoneuronal activity at fetal stages
LAPENDRY	Audrey	LYON	FRANCE	Amino acid metabolism and alternative splicing
MAIELLANO	Greta	LYON	FRANCE	Role of the TMED proteins in acetylcholine receptor biosynthesis and implication in neuromuscular diseases
MATHIEU	Maxime	TOULOUSE	FRANCE	Characterization and functional role of adipose-derived fibro-adipogenic progenitors (AD-FAPs) in skeletal muscle regeneration
PENC	Axelle	NANTES	FRANCE	Effect of Enzyme replacement therapy (ERT) on skeletal muscle pathophysiology in Pompe disease : specific focus on satellite cells and autophagy
PHONGSAVANH	Xaysongkham Micky	MONTIGNY-LE-BRETONNEUX	FRANCE	Evaluation of HDAC inhibitors to increase dystrophin rescue in DMD following exon skipping therapy
THIBAUT	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
YILDIRIM	Zuleyha	ILLKIRCH	FRANCE	PARP3 promotes myogenic differentiation and skeletal muscle function in cooperation with the histone methyltransferase EZH2

Projets 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
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Projets soumis à l'appel d'offres thématique Recherche Dystrophie Myotonique

FURLING	Denis	PARIS	FRANCE	Tricyclo-DNA antisense oligonucleotide treatment for Myotonic Dystrophy
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Partenariats institutionnels

BOUKHATMI	Hadi	PARIS	FRANCE	Programme ATIP-Avenir
DUPONT	Jean-Baptiste	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

CHRISTIE-BROWN	Vanessa	CHIPPING CAMPDEN	ROYAUME UNI	11th international Call for SMA Research Projects.
IFCAH	Fonds de Dotation	PARIS	FRANCE	Pathophysiology and therapeutics challenges of congenital adrenal hyperplasia
RETINA FRANCE/MOSER	Eric	COLOMIERS	FRANCE	APPEL D'OFFRES 2021 ASSOCIATION RETINA FRANCE
VLM/DE CARLI	Paola	PARIS	FRANCE	APPEL A PROJETS SCIENTIFIQUES 2022
VML ASSOCIATION/ANTOLIN	Stéphane	MASSY	FRANCE	Call for grant 2022 - Lysosomal Diseases

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

SOLE	Guilhem	BORDEAUX	FRANCE	Evaluation of the effectiveness of COVID-19 vaccination by seeking neutralizing antibodies in patients with neuromuscular disease with severe amyotrophy
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Projets Ignition

BARTHELEMY	Inès	MAISONS-ALFORT	FRANCE	Adoptive Cell Therapy In Fibrosis : application to Duchenne Muscular Dystrophy
BENCHOUA	Alexandra	CORBEIL-ESSONNES	FRANCE	Paediatric application of dopamine synthesizing lentiviral vectors in genetic dopamine deficiency disorders: Lesch-Nyhan Disease as proof of concept
DELPRAT	Benjamin	MONTPELLIER	FRANCE	Original systemic gene therapy approach to treat Wolfram syndrome

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
ALLAMAND	Valérie	PARIS	FRANCE	Physiopathology and therapeutic strategies for LAMA2-CMD
BOMONT	Pascale	LYON	FRANCE	Therapy for Giant Axonal Neuropathy
BRESSAC	Laurence	SAINT-MAUR-DES-FOSSES	FRANCE	Preclinical development of an anti-CD38 antibody against ALS
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
GABISON	Eric	PARIS	FRANCE	Microvesicles, Cell and Gene Therapies Alternative For Blinding Eye Surface Lesions
GUIVARCH	Yann	EVRY	FRANCE	Collaborative program for a systematic, mecanistic and pharmacologic approach of rare neuromuscular diseases
LAPORTE	Jocelyn	ILLKIRCH	FRANCE	Pathophysiology and therapeutic proof-of-concepts for congenital myopathies
MAUREL	Jean-Claude	BAILLARGUES	FRANCE	Development and validation of GMP Chemistry, Manufacturing and Control of siRNA in the drug delivery technology, Aonys for a first application in targeting Single Nucleotide Polymorphism in Huntington's Disease.
MENASCHE	Philippe	PARIS	FRANCE	Treatment of Anthacycline-Induced Cardiomyopathy by Intravenous Administration of Cardiovascular Progenitor Cell-Derived Extracellular Vesicles
POURQUIE	Olivier	BOSTON	USA	Toward cell therapy for Duchenne Muscular Dystrophy: characterization of regenerative potential of hIPS-derived Pax7+ cells
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
RICHARD	Isabelle	EVRY-COURCOURONNES	FRANCE	IN VIVO VALIDATION OF RESCUE OF MISSENSE MUTATIONS IN SARCOGLYCANOPATHIES
SACCONI	Sabrina	NICE	FRANCE	A 12 months prospective natural history study to gain insight FSHD2 pathophysiology and disease progression
SAKER	Safaa	EVRY	FRANCE	Efficacy, activity and safety of low-dose IL-2 (Id-IL-2) as a Treg enhancer for anti-neuroinflammatory therapy in newly diagnosed Amyotrophic Lateral Sclerosis (ALS) patients
SEFERIAN	Andreea	PARIS	FRANCE	A prospective, longitudinal, interventional natural history study of children with LAMA2 congenital muscular dystrophy
VILQUIN	Jean-Thomas	PARIS	FRANCE	Comparative study of iPSC-derived myogenic precursors proposed for cell therapy in a model of Duchenne muscular Dystrophy
WOOD	Matthew J.A.	OXFORD	ROYAUME UNI	PRE-CLINICAL DEVELOPMENT OF PEPTIDE-OLIGONUCLEOTIDES FOR DM1

Pôles stratégiques

MAGDINIER	Frédérique	MARSEILLE	FRANCE	Modelisation and Therapeutic Approaches for Rare Diseases
RELAIX	Frédéric	CRÉTEIL	FRANCE	An integrated translational program for neuromuscular disorders
SCHAEFFER	Laurent	LYON	FRANCE	MyoNeurALP2, The Research Network dedicated to Neuromuscular Disorders in Rhone Alpes Auvergne

Structures stratégiques

FONDATION MALADIES RARES	Fmr	PARIS	FRANCE	Appel à projet auprès des filières de santé maladies rares.
FONDATION MALADIES RARES	Fmr	PARIS	FRANCE	Subvention 2022
GENOPOLE	Genopole	EVRY-COURCOURONNES	FRANCE	Participation financière de l'AFM au budget 2022 du GIP GENOPOLE

Outils stratégiques

BASSEZ	Guillaume	PARIS	FRANCE	iDM-scope: the international French-Quebec myotonic dystrophy registry
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AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

BARIS	Olivier	ANGERS	FRANCE	11ème Colloque du réseau MeetOchondrie 2022
BONNE	Gisèle	PARIS	FRANCE	12th Japanese-French Workshop - "New insights in personalized medicine for neuromuscular diseases: From Basic to Applied Myology" 9-10 September 2022, Giverny, France
COLLOQUE JEUNES CHERCHEURS		PARIS	FRANCE	Colloques jeunes Chercheurs 2022 - Myology 2022 & Young investigator Poster MitoNice 2022 - Nice 2022
DILWORTH	Jeffrey F.	OTTAWA	CANADA	2023 Gordon Research Conference on Myogenesis - Intrinsic and Extrinsic Control of Myogenesis Under Physiological and Pathological Conditions
DZIEWCZAPOLSKI	Gustavo	TORRANCE	USA	2022 CMD Scientific and Family Conference
GABELLINI	Davide	MILANO	ITALIE	IIM-Myology Meeting: Pathogenesis and Therapies of Rare Diseases
GOURDON	Geneviève	PARIS	FRANCE	IDMC13 Hybrid meeting: "Myotonic Dystrophy – current progress and insights from other muscular disease"
KINOSHITA	June	ORLANDO	USA	29th International Research Congress on FSHD
MILLAY	Douglas P.	CINCINNATI	USA	Skeletal muscle stem cells and regeneration
MONTAZEL	Constance	PARIS	FRANCE	"ALS & motor neuron diseases: what avenues for the future?"
MOURIKIS	Philippos	CRETEIL	FRANCE	EMBO Workshop: Muscle formation, maintenance, regeneration and pathology.
MOURIKIS	Philippos	CRETEIL	FRANCE	1st Symposium on "Muscle stem cells in Growth and Disease".
MUNOZ-CANOVES	Pura	BARCELONA	ESPAGNE	5th International Conference on Muscle Wasting: "Molecular mechanisms of muscle wasting during aging and disease" - Congressi Stefano Francini, Monte Verità, Ascona, Switzerland September 11, 2022 –September 16, 2022
RELAIX	Frédéric	CRETEIL	FRANCE	Workshop on muscle tissue repair and disorders.
TANESSE	Daniel	BRUXELLES	BELGIQUE	First European CMT Specialists Conference
TURCQ	Béatrice	TALENCE	FRANCE	CRISPR and Translational Medicine
VENCOVSKY	Jiri	PRAHA	RÉPUBLIQUE TCHÈQUE	GCOM2022 GLOBAL CONFERENCE ON MYOSITIS 2022

Plateformes

ATTARIAN	Shahram	MARSEILLE	FRANCE	Plateformes d'essais thérapeutiques Pédiatriques et Adultes Neuromusculaires - MARSEILLE
DESIGNES	Cyril	MEZILLES	FRANCE	Financement CEDS
LAFRET	Pascal	GARCHES	FRANCE	Plateformes d'essais thérapeutiques Pédiatriques et Adultes Neuromusculaires - GARCHES
SOLE	Guilhem	BORDEAUX	FRANCE	Plateformes d'essais thérapeutiques Pédiatriques et Adultes Neuromusculaires - BORDEAUX