LISTE DES DOSSIERS FINANCES 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	**Qiyan**	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

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LÉGARÉ	**Cécilia**	CHICOUTIMI - QUEBE	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 Distrophy
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
SACCONE	**Valentina**	ROMA	ITALIE	The RNA-Binding Protein hnRNPA2B1 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

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

Financements de projets

BENARD	**Giovanni**	BORDEAUX	FRANCE	Studying the ribosomal-associated quality control in a mitochondrial syndrome

LISTE DES DOSSIERS FINANCES EN 2022

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
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/I 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 TTLL-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

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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 iPS cells into affected and preserved motor neuron subtypess
PENNUTO	**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

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-LOUCHART	**Alicia**	LILLE	FRANCE	The circadian clock of muscle stem cells □
SACCONE	**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 tans-splicing

LISTE DES DOSSIERS FINANCES EN 2022

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 FOr supporting micro-dySTrophin gene thERapy in DMD

Financements de projets

GOYENVALLE	**Aurélie**	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 inhibitiors efficacity 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 therapeutical avenue for ALS

LISTE DES DOSSIERS FINANCES EN 2022

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	Thèse rattachée au pôle stratégique
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

LISTE DES DOSSIERS FINANCES EN 2022

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	**Xaysongkhame Micky**	MONTIGNY-LE-BRETONNEUX	FRANCE	Evaluation of HDAC inhibitors to increase dystrophin rescue in DMD following exon skipping therapy
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
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

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

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

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

LISTE DES DOSSIERS FINANCES EN 2022

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

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 LesionsMicrovesicles, 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 (ld-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

LISTE DES DOSSIERS FINANCES EN 2022

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

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 Franscini, 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

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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
DESVIGNES	**Cyril**	MEZILLES	FRANCE	Financement CEDS
LAFORET	**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