LISTE DES FINANCEMENTS ACCORDES PAR L'AFM-TELETHON EN 2025

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

GUILCHER	**Marine**	MONTPELLIER	FRANCE	Elucidating the Hox transcription factor Ubx-RNA binding specificity at the nucleotide resolution during muscle morphogenesis
KUROSAWA	**Tamaki**	PARIS	FRANCE	Regionalization of fibroblasts of muscle attachments
PONTHEAUX	**Florian**	BANYULS-SUR-MER	FRANCE	Smooth muscles as mechano-chemical regulators of repair processes: nature of the actin-based topological defects during jellyfish regeneration
SACLIER	**Marielle**	PARIS	FRANCE	Impact of Senescent Cell-Macrophage Crosstalk in Muscle Repair

Financements de projets

BLAAUW	**Bert**	PADOVA	ITALIE	Identification of muscle-specific factors involved in NMJ maintenance and their regulation by mTORC1
CARNESECCHI	**Julie**	MONTPELLIER	FRANCE	Illuminating the isoform-regulatory function of the Hox transcription factor Ultrabithorax during muscle development
CHAHINE	**Mohamed**	QUÉBEC	CANADA	Unveiling the role of a key modulator in cardiac electrophysiology and ion channel trafficking: Insights from iPSC models and knock-in mice
DE ALMEIDA	**Sergio**	LISBOA	PORTUGAL	Unveiling the DNA Damage Response in Skeletal Muscle Resilience and Repair
DUPREZ	**Delphine**	PARIS	FRANCE	Regionalisation of myogenesis
ENRIQUEZ	**Jonathan**	LYON	FRANCE	Spatio temporal control of muscle diversity
KIM	**Minchul**	ILLKIRCH	FRANCE	Translational control of myotendinous junction by Tigd4/Staufen complex
LEFKIMMIATIS	**Konstantinos**	PAVIA	ITALIE	The spatiotemporal map of nuclear signalling in the aging muscle
MENZIES	**Keir**	OTTAWA	CANADA	Examining the role and therapeutic potential of Poly-ADP-Ribosylation (PARylation) in myopathies and skeletal muscle maintenance and function
NEVES	**Joana**	LISBOA	PORTUGAL	The contribution of immune aging to skeletal muscle regenerative failure
ROMAN	**William**	CLAYTON	AUSTRALIE	Monitoring the transcriptional repair program after local muscle damage
ROMANELLO	**Vanina**	PADOVA	ITALIE	The in vivo role of peroxisomes in the control of muscle function
ROMANELLO	**Vanina**	PADOVA	ITALIE	Study of peroxisomes as a metabolic link to muscle regeneration
SAURIN	**Andrew**	MARSEILLE	FRANCE	Hox control of Drosophila adult myogenesis
SINIGAGLIA	**Chiara**	BANYULS-SUR-MER	FRANCE	Jellyfish smooth muscles in development, tissue repair and regeneration

Commission thématique : Bases Moléculaires et Physiopathologie des Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

CALABRÒ	**Sonia**	PADOVA	ITALIE	Investigating the role of gut microbiota in the pathogenesis of Collagen VI-related myopathies
DASKALAKI	**Ioanna**	LAUSANNE	SUISSE	DeLIP-PAM: Targeting sphingolipids for the treatment of Protein Aggregate Myopathies
DEPREZ	**Alyson**	MONTRÉAL	CANADA	Trained immunity, macrophages and muscle stem cells in dystrophic respiratory muscle
FERNANDEZ SIMON	**Esther**	NEWCASTLE UPON TYNE	UK	Targeting C4BPA as a therapeutic approach in muscle dystrophies
TORCINARO	**Alessio**	MONTEROTONDO SCALO	ITALIE	Role of miR-200c and oxidative stress in FSHD

Financements de projets Tremplins

CESCON	**Matilde**	PADOVA	ITALIE	Investigating the role of gut microbiota in the pathogenesis of Collagen VI-related myopathies
FERNANDEZ SIMON	**Esther**	NEWCASTLE UPON TYNE	UK	Targeting C4BPA as a therapeutic approach in muscle dystrophies
RUPARELIA	**Avnika**	VICTORIA	AUSTRALIE	Extracellular matrix remodelling in LAMA2-related muscular dystrophy: friend or foe?
SORGE	**Matteo**	TURIN	ITALIE	Melusin changes the fate of ERK1/2 in Emery-Dreifuss cardiomyopathy

Financements de projets

CHARLET-BERGUERAND	**Nicolas**	ILLKIRCH	FRANCE	Decipher the mechanisms underlying muscle weakness in Myotonic Dystrophy
COPPEE	**Frédérique**	MONS	BELGIQUE	Characterization of DUX4 and DUX4c major protein partners to identify new therapeutic targets for faciocapulohumeral muscular dystrophy (FSHD)
DE GREEF	**Jessica**	LEIDEN	PAYS-BAS	Muscle-on-chip: a novel approach for studying DUX4-induced events in FSHD
DÍAZ-MANERA	**Jordi**	NEWCASTLE	UK	MATRIX: Understanding the role of extracellular matrix in the process of muscle degeneration in muscular dystrophies
ESCANDE-BEILLARD	**Nathalie**	ISTANBUL	TURQUIE	Novel Muscular Dystrophy caused by SNUPN Mutations: From Innovative modeling to therapeutic insights
JASMIN	**Bernard**	OTTAWA	CANADA	Therapeutic potential of AMPK in Myotonic Dystrophy type 1 (DM1)
PEYRON	**Christelle**	BRON	FRANCE	Sleep alteration in a mouse model of DM1
RAVEL-CHAPUIS	**Aymeric**	OTTAWA	CANADA	Characterization of the DM1 myosecretome and its multisystemic impact
SACCONE	**Valentina**	ROME	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
TEDESCO	**Francesco Saverio**	LONDON	UK	Studying the impact of nuclear shape abnormalities in laminopathies using advanced in vitro models
THORSTEINSDOTTIR	**Solveig**	LISBON	PORTUGAL	Identifying and targeting molecular pathways involved in LAMA2-CMD onset
VAN PUTTEN	**Maaike**	LEIDEN	PAYS-BAS	Characterization of four humanized mouse models for Duchenne muscular dystrophy

Commission thématique : Bases Moléculaires & Physiopathologie des Maladies Neuromusculaires autres que les Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

SILVA-ROJAS	**Roberto**	MADRID	ESPAGNE	MetaTitin: The sleeping giant of skeletal muscle

Financements de projets

BRIS	**Céline**	ANGERS	FRANCE	Urine-derived cells for non-invasive diagnosis of mitochondrial diseases
CASTETS	**Perrine**	GENÈVE	SUISSE	Deciphering the multiple pathophysiological facets of the chaperone VMA21
COPIC	**Alenka**	MONTPELLIER	FRANCE	Establishment of molecular mechanisms leading to protein aggregation and amyloid formation in distal myopathy patients using multidisciplinary approaches including genetic screening, bioinformatics and biochemical and structural analysis
DUVEZIN-CAUBET	**Stéphane**	BORDEAUX	FRANCE	Characterization of the molecular role of TMEM70 in the biogenesis of mitochondrial F1Fo-ATP synthase
FERNANDEZ-VIZARRA	**Erika**	HUESCA	ESPAGNE	Tissue-specificity of complex III dysfunction in mitochondrial encephalomyopathies
GARAUDE	**Johan**	BORDEAUX	FRANCE	Revealing macrophages contribution to mitochondrial myopathy
KÜHL	**Inge**	GIF-SUR-YVETTE	FRANCE	Investigating the role of an uncharacterized mitochondrial RNA Polymerase-associated Factor: towards a finer understanding of mammalian OXPHOS biogenesis
METODIEV	**Metodi**	PARIS	FRANCE	Molecular basis of impaired OXPHOS biogenesis caused by deficient protein maturation in mitochondria
MITRANI-ROSENBAUM	**Stella**	JERUSALEM	ISRAËL	Novel insights in GNE Myopathy mechanism of action
STEFFANN	**Julie**	PARIS	FRANCE	MITOCARE: Mitochondrial replacement therapy (MRT) against mitochondrial DNA (mtDNA) disorders: are we far from its clinical application?
VANDROMME	**Marie**	TOULOUSE	FRANCE	Epigenetic contribution in the etiology of Myotubular Myopathy

Commission thématique : Système Nerveux et Jonction Neuromusculaire

Aides aux jeunes chercheurs post-doctorants

BASTO	**Clara**	VILLEJUIF	FRANCE	GAA:TTC repeat expansions and neuromuscular degeneration
GUERRA	**Marika**	ROME	ITALIE	Investigating the functional role of SMN circRNAs in SMA motor neurons differentiation and viability
MILIOTO	**Carmelo**	PADOVA	ITALIE	Investigating spinal motor neurons vulnerability in C9orf72 DPR knock-in mouse models
MORRONI	**Jacopo**	ROME	ITALIE	Unveiling the phenotype of autoreactive B cells in MuSK-associated myasthenia gravis through combined single cell technologies
NABAVIZADEH	**Nasrinsadat**	ISTANBUL	TURQUIE	Deciphering the function of a novel gene in an ALS-like syndrome
RUSSO	**Loris**	PADOVA	ITALIE	Targeting Beclin 1-RegulatEd pathwayS to counTerAct peRipheral demyelinaTion

Financements de projets Tremplins

ESTEVES	**Filipa**	FARO	PORTUGAL	The role of RNA-binding proteins in Spinocerebellar ataxia type 3 (SCA3): from pathogenesis to disease-modifying therapy
MATOS RODRIGUES	**Gabriel**	VILLEJUIF	FRANCE	Pathogenic expanded GAA:TTC repeats and neuromuscular degeneration: mechanisms of gene silencing and repeat expansions
MORANT	**Laura**	ANTWERP	BELGIQUE	Exploring the role of YARS1 in peripheral neuropathy: reconstructing its interaction with actin
MORRONI	**Jacopo**	ROME	ITALIE	Unveiling the phenotype of autoreactive B cells in MuSK-associated myasthenia gravis through combined single cell technologies
ROMEO GUITART	**David**	PARIS	FRANCE	Bone-Primary Cilia Signaling in the progression of motoneuron disease

Financements de projets

BOWERMAN	**Melissa**	STAFFORDSHIRE	UK	Defining the role of brown adipose tissue in metabolic defects in SMA
CHEVROLLIER	**Arnaud**	ANGERS	FRANCE	Unexplored aspects of proteostasis in MFN2-related CMT2A2
LEE	**Young Il (Matt)**	GAINESVILLE	USA	Contribution of impaired and maladaptive motor innervation of muscle fibers to progressive pathology of muscular dystrophy
MONANI	**Umrao R.**	NEW YORK	USA	How does a novel spinal muscular atrophy modifier suppress motor neuron disease?
ORTEGA CANO	**Juan Alberto**	L'HOSPITALET DE LLOBREGAT	ESPAGNE	Defining the contribution of ALS-associated alterations in motor neuron microenvironment to disease pathogenesis
PENNUTO	**Maria**	PADOVA	ITALIE	Development of a therapeutic strategy to suppress LSD1 and PRMT6-mediated toxic gain of function in SBMA
POLETTI	**Angelo**	MILAN	ITALIE	Pathogenic mechanisms of HSPB8 mutations in neuromuscular diseases: the role of the ribosomal and protein quality control system and the integrated stress response
RINALDO	**Cinzia**	ROME	ITALIE	Spastin elevating approaches to counteract Hereditary Spastic Paraplegia
ROJO	**Manuel**	BORDEAUX	FRANCE	Cellular assays to establish the consequences and infer the pathogenicity of MFN2 variants
TARESTE	**David**	PARIS	FRANCE	Core Molecular Mechanisms and Lipid Determinants of Mitofusin-mediated Mitochondrial Fusion

Commission thématique : Cellules Souches

Aides aux jeunes chercheurs post-doctorants

GRISETI	**Elena**	TOULOUSE	FRANCE	Characterizing the features of mobilizable adipose stromal cells participating to muscle regeneration and their alterations during aging
LY	**Ha My**	OTTAWA	CANADA	The role of PINK1/PARKIN mediated the mitophagy in the regulation of muscle stem cell fate choices and muscle regeneration
POITOU MOUCAUD	**Blandine**	MONTRÉAL	CANADA	Optogenetics and muscle atrophy: delivery of optogenetic contractile properties to skeletal muscle by MuSCs

Financements de projets Tremplins

ORTS-DEL IMMAGINE	**Adeline**	PARIS	FRANCE	Warburg Micro syndrome : autophagy's role in the pathophysiological development of cortical neurons
VAN LENT	**Jonas**	BELLINZONA	SUISSE	Exploring human neuromuscular reflex arcs in vitro using assembloid models

Financements de projets

BIRCHMEIER	**Carmen**	BERLIN	ALLEMAGNE	Oscillatory gene expression in human iPSC-derived myogenic cells
BURELLE	**Yan**	OTTAWA	CANADA	Targeting mitochondrial quality control in stem cells to promote muscle repair
CARETTI	**Giuseppina**	MILAN	ITALIE	BET inhibitors rewire lipid metabolism in mdx satellite cells
FERRETTI	**Patrizia**	LONDON	UK	Metabolic defects as potential therapeutic targets for Duchenne Muscular Dystrophy neural pathology
FUKADA	**So-Ichiro**	OSAKA	JAPON	Identification of extrinsic factors involved in MuSC self-renewal in physiological and pathological environments
GUARDIOLA	**Ombretta**	NAPOLI	ITALIE	Exploring the complexity of chemokine/chemokine receptor dynamics in muscle stem cell population: insights into CXCR2 signalling and heterogeneity
KHACHO	**Mireille**	OTTAWA	CANADA	Targeting mitochondrial dynamics and metabolites in muscle stem cells as a therapy for Duchenne Muscular Dystrophy
ZAMMIT	**Peter**	LONDON	UK	Induced Pluripotent Stem Cell models to decipher pathomechanisms in FSHD

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

Financements de projets Tremplins

BARGIELA	**Ariadna**	BURJASSOT	ESPAGNE	Advanced Therapeutic Strategies Using Antisense Oligonucleotides Targeting MSI2 for the Treatment of Myotonic Dystrophies

Financements de projets

ARRIBAT	**Yoan**	MONTPELLIER	FRANCE	Development of the first system of organelle optimisation and transplantation to treat mitochondrial diseases
BELENGUER	**Pascale**	TOULOUSE	FRANCE	Proof of principle of an original gene therapy for a rare hereditary mitochondrial-related optic atrophy
BORTOLUSSI	**Giulia**	TRIESTE	ITALIE	Improving Targeted Genome Integration in the Adult Liver to Treat Rare Genetic Metabolic Diseases
BRUNETTI-PIERRI	**Nicola**	POZZUOLI	ITALIE	Gene therapy for Wolman disease
CHEMELLO	**Francesco**	BOLOGNA	ITALIE	Editing Detection In Transcriptome: Deciphering the Gene Editing Outcomes in DMD Using snRNA-seq and Spatial Transcriptomics
EL AMRAOUI	**Aziz**	PARIS	FRANCE	Humanized mice to expedite effective translation of reliable inner ear gene therapies
GRANDIS	**Marina**	GENOVA	ITALIE	Development of Allele-Specific Gene-Silencing siRNAs for MPZ-D61N in CMT1B Neuropathy
GRITTI	**Angela**	MILAN	ITALIE	Hematopoietic Stem Cell Gene Therapy (HSC GT) for GM2 Gangliosidosis: Advancing Therapeutic Efficacy and Translational Potential
HOVNANIAN	**Alain**	PARIS	FRANCE	Base editing-mediated correction of recurrent mutations in COL7A1 to treat RDEB
LATTANZI	**Wanda**	ROME	ITALIE	Nano-ink based Antisense oligonUcleoTIde deLivery for Ultra-personaIized treatment of Syndromic craniosynostoses
MINCZUK	**Michal**	CAMBRIDGE	UK	Mitochondrial Genome Base Editing for Advancing Therapies of Neuromuscular Diseases
MOINE	**Hervé**	ILLKIRCH	FRANCE	AAV-DGKk gene therapy for the treatment of Fragile X syndrome
MONANI	**Umrao R.**	NEW YORK	USA	Modulating SLC2A1 activity with a novel lncRNA to treat infantile-onset Glucose Transporter-1 deficiency syndrome
MORLANDO	**Mariangela**	ROME	ITALIE	Targeting IRF2BPL non-sense mutations with suppressor tRNAs
MURO	**Andrés F.**	TRIESTE	ITALIE	Combination of mRNA-LNP and gene targeting approaches as a potential cure for early onset severe Ornithine transcarbamylase deficiency
ROYBON	**Laurent**	GRAND RAPIDS	USA	Targeting STING to prevent astrocyte toxicity to motor neurons in ALS
SCHIFF	**Manuel**	PARIS	FRANCE	Optimization of AAV gene therapy in MSUD
SMEETS	**Hubert J. M.**	MAASTRICHT	PAYS-BAS	Assess efficacy of intra-arterial autologous myogenic stem cell therapy for m.3243A>G mutation carriers
SORRENTINO	**Vincenzo**	SIENA	ITALIE	Investigating the potential of miR-486 overexpression to enhance skeletal muscle function in mouse models of recessive RYR1-related myopathies
TASFAOUT	**Hichem**	SEATTLE	USA	Expression of large dystrophins using AAV and split inteins
TREPICCIONE	**Francesco**	NAPLES	ITALIE	Treating Fanconi-Bickel Syndrome with an AAV-KP1-pSglt2-Glut2 gene therapy approach

Commission thématique : Thérapie Pharmacologique et Recherche Translationnelle sur les Maladies NeuroMusculaires

Aides aux jeunes chercheurs post-doctorants

BARQUISSAU	**Valentin**	TOULOUSE	FRANCE	Role of phosphoinositides in the pathophysiology of X-linked centronuclear myopathy (XLCNM)
BOULINGUIEZ	**Alexis**	EGHAM	UK	Pharmacological approaches to improve folding of microdystrophin in Duchenne muscular dystrophy
FABBRIZIO	**Paola**	MILAN	ITALIE	Modulating the P2X7 axis in Spinal and Bulbar Muscular Atrophy muscle cell populations as an innovative approach to promote myogenesis
VAN DE WAL	**Melissa**	DÜSSELDORF	ALLEMAGNE	iPSC-driven repositioning of Sildenafil to treat Leigh syndrome

Financements de projets Tremplins

BELTRÀ BACH	**Marc**	BARCELONA	ESPAGNE	Validation of a new Charcot-Marie-Tooth 2A model amenable for therapy search
MANTUANO	**Paola**	BARI	ITALIE	Investigating the role of mitochondrial translocator protein (TSPO) in Duchenne muscular dystrophy: pathophysiological and therapeutic implications

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
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
DE PALMA	**Clara**	MILAN	ITALIE	Evaluation of SRT2104 potential in skeletal and cardiac muscle recovery in a severe model of DMD
DEVAUX	**Jérôme**	MONTPELLIER	FRANCE	Role of ADAM MEtalloproteases in autoimmune Nodopathy
DUPUIS	**Luc**	STRASBOURG	FRANCE	Preclinical evaluation of dual orexin receptor antagonist in amyotrophic lateral sclerosis
HNIA	**Karim**	TOULOUSE	FRANCE	Development and validation of PI3KC2b inhibitors for therapeutic application in XLCNM
MALERBA	**Alberto**	EGHAM	UK	Improving protein folding to enhance microdystrophin expression in Duchenne muscular dystrophy
NARDO	**Giovanni**	MILAN	ITALIE	Allosteric agonism of purinergic P2X7 receptor as a pharmacological approach to enhance skeletal muscle regeneration in Spinal Bulbar Muscular Atrophy
NICOLAOU	**Paschalis**	NICOSIA	CHYPRE	Charcot-Marie-Tooth microRNA biomarkers discovery
SANDONÀ	**Dorianna**	PADOVA	ITALIE	Target validation approach to unveil the mechanism of action of C17 in sarcoglycanopathies

Commission thématique : Recherche Médicale

Aides aux jeunes chercheurs post-doctorants

ANDRADE	**Ricardo**	NANTES	FRANCE	Innovative Multiparametric UtraSound biomarkers for muscular dystrophies: towards a comprehensive monitoring of musCLE degeneration and responses to therapy
FORTIN	**Julie**	SAGUENAY	CANADA	Evaluating the Effectiveness of Light Therapy Interventions to Enhance Sleep Quality and Social Participation in Myotonic Dystrophy Type 1

Financements de projets

ANGEARD	**Nathalie**	BOULOGNE-BILLANCOURT	FRANCE	Towards a better understanding of the neurocognitive profile of adults with the childhood phenotype of myotonic dystrophy type 1: an interdisciplinary approach
CLAEYS	**Kristl**	LEUVEN	BELGIQUE	Multimodal approach to study bone and muscle involvement in Facio-Scapulo-Humeral muscular dystrophy (FSHD)
CORTESE	**Andrea**	LONDON	UK	Long-read Sequencing In Undiagnosed Neuromuscular Diseases
GAGNON	**Cynthia**	JONQUIERE	CANADA	Selecting and validating a patient-reported outcome measure for the assessment of dysphagia in DM1
SERGEANT	**Nicolas**	LILLE	FRANCE	Assessment of central nervous system neurodegeneration and impact of type II diabetis in Myotonic Dystrophy type I

Appel d'offres Doctorants

BOURGETON	**Tiffany**	NANTES	FRANCE	Effect of Enzyme replacement therapy (ERT) on skeletal muscle pathophysiology in Pompe disease : specific focus on satellite cells and autophagy
DAY	**Lucie**	PARIS	FRANCE	Role of ADAR1 and RNA editing in peripheral myelin maintenance
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
JAULIAC	**Edgar**	PARIS	FRANCE	Role of Innervation in the Coordination and Activation of the Fast Glycolytic Program in Adult Muscle Fiber: Study of the c-MAF Transcription Factor
LECLERCQ	**Rémi**	PARIS	FRANCE	Unequal mitotic mitochondrial segregation as an asymmetric fate determinant in neural stem cells
LEROUX	**Emma**	RENNES	FRANCE	The transcriptional dynamics of muscle stem cells response to Notch signaling
MACAUX	**Gaspard**	PARIS	FRANCE	The crosstalk between motoneurons and myofibers
ROLLAND	**Fanny**	MONTPELLIER	FRANCE	Characterization of the first organelle factory for therapeutic transplantation
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
SMITH	**Coalesco**	STRASBOURG	FRANCE	Myotendinous junction development driven by transient embryonic gene expression program
VAUCOURT	**Mathilde**	TOULOUSE	FRANCE	Selective autophagy in X-linked centronuclear myopathy: molecular mechanisms and pathophysiological relevance
YTIER	**Charline**	MARSEILLE	FRANCE	Modeling, modulating, and monitoring intramuscular fibro-fatty infiltrates

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

MARTI	**Ramon**	BARCELONA	ESPAGNE	Deoxynucleosides as a potential therapy for mitochondrial DNA maintenance disorders
PROCACCIO	**Vincent**	ANGERS	FRANCE	Therapeutic strategies targeting mitochondrial dysfunction and inflammation in mitochondrial diseases

Partenariats institutionnels

AUTHIER	**François-Jérôme**	CRETEIL	FRANCE	BIGTIM: Blocking Interferon-gamma by Ruxolitinib for Treating Inclusion body Myositis - a phase IIb trial"
LAUGEL	**Vincent**	STRASBOURG	FRANCE	Cohorte de suivi longitudinal SMA-DNN: Dépistage néonatal de l'Amyotrophie Spinale - Projet pilote SMA-DNN France

Partenariats associatifs

IFCAH		PARIS	FRANCE	Financement du projet : - Adrenocortical cells and organoids derived from human induced-pluripotent stem cells - Yasmine NEIRIJINCK
RETINA France		COLOMIERS	FRANCE	Financement du renouvellement des projets : - Une thérapie génique innovante pour l'amaurose congénitale de Leber liée à la RDH12 - Deniz DALKARA - Approches de thérapie oligonucléotidique antisens et de thérapie génique pour le traitement des maladies dégénératives rétiniennes en ciblant la voie glycogène synthase kinase 3 - Jérôme ROGER - Stratégie de restauration visuelle par approche combinée de la transplantation cellulaire et de l'optogénétique - Gaël ORIEUX
SMA EUROPE		CHIPPING CAMDEN	UK	12ème appel à projets international pour la recherche sur la SMA - Co-financement des projets : - Leveraging SMN role in translation to develop the next-gen of biomarkers for SMA - VIERO Gabriella - Skeletal Muscle Stem Cells as untapped therapeutic targets for SMA long-term treatment - DIDIER Nathalie - Deciphering the Molecular Landscape of Neuromuscular Development in Spinal Muscular Atrophy - GAZZOLA Morgan - Investigating Calcium-induced mitochondrial dysfunction in zebrafish and iPSC models of SMA - CIURA Sorana - Are microvascular defects relevant in Spinal Muscular Atrophy ?: Characterization of the mouse model - PARSON Simon
VAINCRE LA MUCOVISCIDOSE (VLM)		PARIS	FRANCE	Financement des projets : - Ciblage des ARNt pour une suppression traductionnelle spécifique de mutations non-sens dans le gène CFTR - Olivier NAMY - Ciblage de l'ARNm TTP comme régulateur de la réponse inflammatoire - Magali CADARS

Projets Ignition

EVANO	**Brendan**	PARIS	FRANCE	Targeted Genetic Manipulation of Muscle Stem Cells in vivo Towards New Treatment Strategies Against Muscular Dystrophies
NICOLE	**Sophie**	MONTPELLIER	FRANCE	Nav1.4 activators for a correction of SCN4A-related muscle weaknesses

ACTIONS STRATEGIQUES

Projets stratégiques

AGBULUT	**Onnik**	PARIS	FRANCE	DESmin-related CARdiomyopathy ThErapeuticS Development - DESCARTES
ALLAMAND	**Valérie**	PARIS	FRANCE	Physiopathology and therapeutic strategies for LAMA2-CMD
AUSSEIL	**Jérôme**	TOULOUSE	FRANCE	Gene therapy for Sanfilippo B syndrome
BOMONT	**Pascale**	LYON	FRANCE	Therapy for Giant Axonal Neuropathy
DOWLING	**James**	PHILADELPHIA	USA	Development of a non-viral delivery based genetic medicine platform for neuromuscular disorders
DUMONCEAUX	**Julie**	LONDON	UK	Myostatin: a biomarker for Facioscapulohumeral dystrophy
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	UPHolD - Ultrarare PYROXD1, ACTN2, and FHL1 congenital-myopathies Pharmacological treatment through HiPSC-based Drug repurposing
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	Immuno-evasion of human satellite cells generated in vitro
PROCACCIO	**Vincent**	ANGERS	FRANCE	Identifying candidate drugs in mitochondrial cardiomyopathies:From Mouse to Human
PROCACCIO	**Vincent**	ANGERS	FRANCE	From mechanisms to therapies: Mitochondrial drug combinations to tackle multiple mitochondrial disorders
REYNGOUDT	**Harmen**	PARIS	FRANCE	MyoBrain-BMD: A multi-modal study including quantitative brain and muscle MRI, (neuro)psychological tests and muscle functional tests to investigate differences between Dp140+ and Dp140- Becker muscular dystrophy patients
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
SEREDA	**Michael**	GÖTTINGEN	ALLEMAGNE	A multi-omic approach to the identification of novel biomarkers in early Charcot-Marie-Tooth 1A disease (CMT1A)
VAILLEND	**Cyrille**	ORSAY	FRANCE	DECODING CENTRAL DEFECTS IN DYSTROPHINOPATHIES : FROM DIAGNOSTIC TO REMEDIATION

Pôles stratégiques

LACAMPAGNE	**Alain**	MONTPELLIER	FRANCE	Pôle Stratégique AFM MYOccitanie : Principales maladies étudiées: Dystrophies musculaires (DMD/BMD/FHSD), titinopathies, syndrome de pseudo-obstruction intestinale chronique (POIC), dyskinésie ciliaire primitive (DCP), mucoviscidose, sarcopénie
MAGDINIER	**Frédérique**	MARSEILLE	FRANCE	Modelisation and Therapeutic Approaches for Rare Diseases: Principales maladies 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**	CRÉTEIL	FRANCE	An integrated translational program for neuromuscular disorders: Principales maladies étudiées: Dystrophies musculaires : DMD ; BMD ; FSHD ; SMA ; Myopathies congénitales ; Myopathies inflammatoires
SCHAEFFER	**Laurent**	LYON	FRANCE	MyoNeurALP2, The Research Network dedicated to Neuromuscular Disorders in Rhone Alpes Auvergne: Principales maladies 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

Association AIDY		PARIS	FRANCE	Aide financière 2026
FONDATION MALADIES RARES		PARIS	FRANCE	Aide financière 2025
GENOPOLE		EVRY-COURCOURONNES	FRANCE	Participation financière de l'AFM-Téléthon au budget 2025 du GIP GENOPOLE

Outils stratégiques

BASSEZ	**Guillaume**	PARIS	FRANCE	International Myotonic Dystrophy Registry - iDM-Scope
LAFORET	**Pascal**	GARCHES	FRANCE	French observatory of patients with type III Glycogenosis
SACCONI	**Sabrina**	NICE	FRANCE	French national registry for FacioScapuloHumeral muscular Dystrophy (FSHD)

Plateformes stratégiques

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

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

BARIS	**Olivier**	ANGERS	FRANCE	Euromit 2026: International Meeting on Mitochondrial Pathology
BOMONT	**Pascale**	LYON	FRANCE	European Intermediate Filament meeting 2025
BOYER	**Olivier**	ROUEN	FRANCE	FOCIS European Advanced Course and Conference on Immunology and Immunopathology, Normandie-France
COLLOQUE JEUNES CHERCHEURS		PARIS	FRANCE	Colloque Jeunes Chercheurs 2025 - Journées Des Familles 2025
CRIST	**Colin**	MONTREAL	CANADA	Society for Muscle Biology 2025 Frontiers in Myogenesis Conference
DE HAAS	**Ria**	DEN HAAG	PAYS-BAS	FSHD Connect Europe Meeting
DZIEWCZAPOLSKI	**Gustavo**	LAKEWOOD	USA	2025 Congenital Muscle Disease Scientific & Family Conference
GARCIA	**Pauline**	MONTREAL	CANADA	2025 Myogenesis Gordon Research Seminar-Gordon Research Conference "Mechanisms of myogenesis in development, health, and disease"
GOLDBERG	**Michael**	PITTSBURGH	USA	2025 RYR-1-Related Diseases Patient-Led International Research Workshop: Novel Perspectives, Treatments, & Interventions
HESKAMP	**Linda**	UTRECHT	PAYS-BAS	Fifth International Imaging in Neuromuscular Disease Conference 2023
HOYAU	**Alexandre**	SAINT-ALBAN	FRANCE	2nd European CMT Specialist Conference
KINOSHITA	**June**	RANDOLPH	USA	32nd Annual International Research Congress on FSHD
MICCIO	**Annarita**	PARIS	FRANCE	CRISPR-Cas: From biology to therapeutic applications
MUCHIR	**Antoine**	PARIS	FRANCE	5th International Meeting on Laminopathies
RUEGG	**Markus**	BASEL	SUISSE	International Conference on Muscle Wasting= Molecular Mechanisms of muscle wasting during aging and disease
SMEETS	**Hubert J. M.**	MAASTRICHT	PAYS-BAS	LAMA2-Muscular Dystrophy: Paving the road to therapy
SORCI	**Guglielmo**	PERUGIA	ITALIE	22nd IIM International Meeting