LISTE DES FINANCEMENTS ACCORDES PAR L'AFM-TELETHON EN 2021

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'appel d'offres

Commission : Myologie fondamentale

Aides aux jeunes chercheurs post-doctorants

BIANCONI Valeria, Roma, Italie
Deciphering the role of Prdm16-mediated H3K9 methylation in the control of Fibro-Adipogenic Progenitors identity and skeletal muscle repair

DE MARIO Agnese, Paris, France
Amorolfine, a positive modulator of the Mitochondrial Calcium Uniporter, as a tool to counteract atrophy and sarcopenia

HENRIQUE FERNANDES Carlos Alexandre, Paris, France
Structural characterization of a Kir potassium channel and its involvement in Andersen syndrome

Financements de projets Tremplins

SAURIN Andrew, Marseille, France
Transcriptional regulation of oxidative phosphorylation during Drosophila myogenesis

WALTZER Lucas, Clermont-Ferrand, France
Decrypting the function of the epigenetic enzyme TET in adult muscle progenitor maintenance and differentiation

Financements de projets

CARVAJAL Jaime, Seville, Espagne
Contribution of the Mrf4 gene towards muscle function during exercise-mediated hypertrophy, regeneration and ageing

DUPREZ Delphine, Paris, France
Molecular interactions between connective tissue fibroblasts and muscle cells in order to build a full musculoskeletal system

FAURE Sandrine, Montpellier, France
Neuronal regulation of intestinal smooth muscle cell differentiation and functionality

LEBRETON Gaëlle, Toulouse, France
Muscle-hematopoietic organ crosstalk: alary somatic muscle as a secretory organ regulating hematopoiesis in Drosophila

MAIRE Pascal, Paris, France
Fast myosin heavy chain locus regulation

MIQUEROL Lucile, Marseille, France
Exploring ventricular conduction system structure and function in the regenerating heart and DMD mouse models

MORO Cédric, Toulouse, France
Myolipotoxicity as a trigger of muscle weakness in metabolic diseases and aging?

MOZZETTA Chiara, Roma, Italie
Deciphering the role of Prdm16-mediated H3K9 methylation in the control of Fibro-Adipogenic Progenitors identity and skeletal muscle repair

MUNOZ-CANOVES Pura, Barcelona, Espagne
Role of circadian communication between skeletal muscle and brain to preserve muscle homeostasis and prevent age-associated muscle wasting
PENDE Mario, Paris, France
Role of mitochondrial YAP and TAZ in muscle growth, regeneration and mechanotransduction
POLGE Cécile, Saint-Genès-Champanelle, France
Toward the inhibition of ubiquitination of contractile proteins for preserving muscle mass
ROMANELLO Vanina, Padova, Italie
Deciphering a novel link between the ubiquitin proteasome system and mitochondrial function to control muscle mass
SANDRI Marco, Padova, Italie
Dissecting the role of an uncharacterized FoxO-dependent gene that controls autophagy and ageing
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
ZERVAS Christos, Athens, Grèce
Unravelling the mechanotransduction-dependent functions of Integrin-Linked Kinase (ILK) at myotendinous junctions

Commission : Bases Moléculaires et Physiopathologie des Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

REGGIO Alessio, Pozzuoli, Italie
Pharmacological reconstitution of the WNT5A/β-catenin axis to counteract fibroadipogenic degeneration in Duchenne Muscular Dystrophy
RENZINI Alessandra, Roma, Italie
Proteomic study of HDAC4 cytoplasmic signaling, partners and secretome in DMD skeletal muscle
RUPARELIA Avnika, Clayton, Australie
Identification of therapies for Collagen VI-related congenital muscular dystrophy

Financements de projets Tremplins

GARCIA Susana, Helsinki, Finlande
Mechanisms of RNA toxicity in Myotonic Dystrophy – the role of mitochondrial dysfunction
GRASSI Francesca, Roma, Italie
Role of glia in neurological comorbidities of duchenne muscular dystrophy
SCHIAVONE Marco, Brescia, Italie
Identification of novel druggable targets for Duchenne muscular dystrophy
SPITALI Pietro, Leiden, Pays-Bas
Understanding histopathological alterations in neuromuscular disorders by spatial transcriptomics

Financements de projets

COWAN Kyle, Ottawa, Canada
Defining the Role and Therapeutic Potential of Pannexin 1 Channels in Duchenne Muscular Dystrophy using Mouse Models and Patients Cells
HUBE Florent, Paris, France
Newly identified non-coding RNAs from alternatively spliced introns in normal and pathological muscle differentiation
MAMMUCARI Cristina, Padova, Italie
Targeting the Mitochondrial Calcium Uniporter to counteract Duchenne Muscular Dystrophy

MERONI Germana, Trieste, Italie
Role of TRIM32, the Limb Girdle Muscular Dystrophy type R8 gene, in neuro-muscular differentiation and homeostasis
MORALES Fernando, San José, Costa Rica
Comparative expression profiling of multiple tissues in myotonic dystrophy
MORESI Viviana, Roma, Italie
Unveiling the cytoplasmic functions of HDAC4 in dystrophic skeletal muscle
MUNOZ-CANOVES Pura, Barcelona, Espagne
Novel strategies to ameliorate Duchenne Muscular Dystrophy
PERROTTA Cristiana, Milano, Italie
An integrated pharmacological/antioxidant approach for Duchenne Muscular Dystrophy: acid sphingomyelinase as new therapeutic target
RAVEL-CHAPUIS Aymeric, Ottawa, Canada
Role of the RNA-binding protein HuR in Myotonic Dystrophy type 1
SUELVES Monica, Badalona, Espagne
Exploring HDAC11 functions in Duchenne Muscular Dystrophy

Commission : Bases moléculaires et physiopathologie des autres maladies neuromusculaires

Aides aux jeunes chercheurs post-doctorants

CHAKOURI Nourdine, New York, Etats-Unis
Dissecting the role of stac3 in skeletal muscle excitation contraction coupling and congenital myopathies
LIONELLO Valentina Maria, London, Royaume-Uni
Complex modeling of myotubularin myopathy using human bioengineered skeletal muscles
PALMA Alessandro, Pozzuoli, Italie
Dissecting the role of ER-phagy in Pompe Disease
PIDOUX Ludivine, Valbonne, France
Investigation of the role of ASIC channels in spinal sensitization mechanisms associated to lipid-induced chronic muscle pain

Financements de projets Tremplins

SAARI Sina, Helsinki, Finlande
Role of mito-nuclear compatibility in the metabolic landscape of mitochondrial diseases

Financements de projets

BATONNET-PICHON Sabrina, Paris, France
Diving into the pathophysiological mechanisms of desminopathies: a comparative study of KI-mouse models and human isogenic cells
CHAHINE Mohamed, Québec, Canada
Omega currents cause cardiac arrhythmias and dilated cardiomyopathy
COLLE Marie-Anne, Nantes, France
Effects of enzyme replacement therapy on lysosome-autophagosome-mitochondria cross-talk and muscle repair in Pompe disease (LYSAUMI)
DEVAL Emmanuel, Valbonne, France
Acid-sensing ion channel 3 in the pathophysiology of chronic muscle pain
HNIA Karim, Toulouse, France
Molecular functions of MTM1-related phosphoinositides in XLCNM pathophysiology
MAZAT Jean-Pierre, Bordeaux, France
How to bypass mitochondrial ATP synthase deficiencies? The role of mitochondrial carriers MitoBAD (Mitochondrial Bypass ATPsynthase Deficiencies)
METODIEV Metodi, Paris, France
Deficient protein processing in mitochondrial diseases

MITRANI-ROSENBAUM Stella, Jerusalem, Israël
Cellular Models to explore GNE functions in muscle
SCHMIDT Jens, Göttingen, Allemagne
Epigenetic modulation of pro-inflammatory cell stress in inclusion body myositis
SCORRANO Luca, Padova, Italie
Enhancing Opa1-dependent cristae structure to combat mitochondrial diseases
STENZEL Werner, Berlin, Allemagne
Studies of protein quality control and proteolysis in the genesis of acute and chronic myositis – the example of Immune-Mediated Necrotizing Myopathy (IMNM) and Inclusion Body Myositis (sIBM)
SWAN Laura, Liverpool, Royaume-Uni
INPP5K-mediated congenital muscular dystrophy models of motor neuron branching and function
ZORZANO Antonio, Barcelona, Espagne
Mitochondrial dynamics as a key process to prevent muscle inflammation and search of novel therapies in inflammatory myopathies

Commission : Système nerveux : Motoneurone et jonction neuromusculaire

Aides aux jeunes chercheurs post-doctorants

JHA Narendra, New York, Etats-Unis
Identification of modifier gene(s) of spinal muscular atrophy in model mice
SARDINA Francesca, Roma, Italie
Phenotipic 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

BOEYNAEMS Steven, Stanford, Etats-Unis
sPAM: Developing peptide-based drugs to target ataxin-2 in neuromuscular disease

Financements de projets

BOMONT Pascale, Montpellier, France
Analysis of the first phenotypical mouse model for Giant Axonal Neuropathy
BONDURAND Nadège, Paris, France
Role of ADAR1 and RNA editing in Schwann cells development and myelin maintenance
BRANCHEREAU Pascal, Bordeaux, France
Chloride-related inhibition in spinal motoneurons during the ALS disease progression in mouse model
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
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
FERRARO Elisabetta, Pisa, Italie
Metabolic reprogramming and activation of microglia in amyotrophic lateral sclerosis (ALS): a pivotal role for serotonin?
JORDANOVA Albena, Antwerpen, Belgique
Profiling the spatial RNA and protein signatures of peripheral neurons in YARS associated Charcot-Marie-Tooth neuropathies

LEVY-LAHAD Ephrat, Jerusalem, Israël
The motor neuron disease gene VRK1: a conditional VRK1 knockout mouse as a novel model for neuromuscular disease
LIEVENS Jean-Charles, Montpellier, France
Deciphering the beneficial effects of Sigma-1 receptor in amyotrophic lateral sclerosis
MONANI Umrao, New York, Etats-Unis
Investigating the cellular and molecular basis of muscle defects in spinal muscular atrophy
PENNUTO Maria, Padova, Italie
Targeting AR co-regulators to attenuate spinal and bulbar muscular atrophy
RIVAL Thomas, Marseille, France
Characterizing Charcot-Marie-Tooth disease-associated alleles of mitofusin with enhanced mitochondrial fusion activity and interfering with their neurotoxicity in vivo
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
VETTORI Andrea, Verona, Italie
Involvement of BMP signaling pathway in Hereditary Spastic Paraplegia: identification of new therapies based on in-vivo drug screening
VIERO Gabriella, Povo, Italie
Ribosome-based functions of the SMN protein: from fundamental biology to second-generation therapies for SMA

Commission : Cellules souches

Aides aux jeunes chercheurs post-doctorants

ALESSANDRINI Francesco, Chicago, Etats-Unis
Develop an iPSC-based platform for interrogating sporadic ALS disease proteome compartmentalization
DI GIROLAMO Daniela, Paris, France
Metabolic regulation of muscle stem cell fates in physiological and pathological conditions
GALLAY Laure, Genève, Suisse
Pivotal role of muscle stem cells in idiopathic inflammatory myopathies pathogenesis
SANDONÀ Martina, Roma, 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

EVANO Brendan, Paris, France
Niche determinants of muscle stem cell in vivo dynamics
ORTEGA CANO Juan Alberto, Chicago, Etats-Unis
Defining the spinal cord matrisome to design more effective ALS models and treatments
PARFITT David, Leiden, Pays-Bas
Investigating cell-specific pathology in Huntington's disease and spinocerebellar ataxia iPSC-derived brain assembloids before and after treatment with targeted antisense oligonucleotides

Financements de projets

AIT-SI-ALI Slimane, Paris, France
Induced pluripotent stem cells-based strategies and disease modeling to unravel signaling-induced epigenetic networks in healthy and in Duchenne muscle dystrophic muscles: the TGFβ/Wnt regulated histone lysine methylome
AMTHOR Helge, Montigny-le-Bretonneux, France
The role of dystrophin in establishing the satellite cell niche

BIRCHMEIER Carmen, Berlin, Allemagne
Muscle stem cell maintenance and self-renewal: Prerequisites for a healthy muscle
BIRESSI Stefano, Povo, Italie
Novel satellite cell heterogeneity in healthy and pathological regeneration
ELVASSORE Nicola, Padova, Italie
Intravital 3D bioprinting approach for muscle stem cell delivery
FUKADA So-Ichiro, Osaka, Japon
Identification of factors inducing MuSC expansion from overloaded muscle
MAYEUF-LOUCHART Alicia, Lille, France
The circadian clock of muscle stem cells
MOREY Céline, Paris, France
Investigating the function of the FTX long non-coding RNA in the definition of spinal motoneuron identities
SEBILLE Stéphane, Poitiers, France
Functional characterization of muscle cells derived from healthy and DMD human induced Pluripotent Stem Cells
THORSTEINSDOTTIR Solveig, Lisbon, Portugal
Understanding the responses of fetal muscle stem cells and myoblasts to their niche in a mouse model for LAMA2-CMD

Commission : Thérapie Génique et/ou Cellulaire des Maladies Rares

Aides aux jeunes chercheurs post-doctorants

BOFFA Yolanda, Napoli, 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
RICHARD Elodie, Montpellier, France
Systemic gene therapy approach to treat Wolfram syndrome
TASFAOUT Hichem, Seattle, Etats-Unis
Expression of large dystrophins using Intein-mediated protein trans-splicing

Financements de projets Tremplins

INDRIERI Alessia, Pozzuoli, Italie
Therapeutic efficacy of miR-181a/b down regulation in inherited optic neuropathies
MALERBA Alberto, Egham, Royaume-Uni
Establishing a mouse model of disease to test combined antisense oligonucleotides and AAV systemic gene therapy as new treatment for Oculopharyngeal muscular dystrophy

Financements de projets

BETUING Sandrine, Paris, France
Efficient Gene and Sterol regulations by gene transfer for striatal protection in Huntington's Disease
BOYER Olivier, Rouen, France
CAAR-T cells: toward an antigen-specific immunotherapy for Immune Mediated Necrotizing Myopathies
DALKARA Deniz, Paris, France
Non viral gene editing for autosomal dominant retinitis pigmentosa
ERCEG Slaven, Valencia, Espagne
The cell therapeutic strategy for hereditary retinal dystrophies in small and large animals: MERTK associated Retinitis pigmentosa
HOVNANIAN Alain, Paris, France
Base editing-mediated correction of recurrent mutations in COL7A1 to treat RDEB
KEMALADEWI Dwi, Pittsburgh, Etats-Unis
Therapeutic genetics and disease modeling in LAMA2-CMD

LATTANZI Wanda, Roma, Italie
Personalized non-invasive nanotherapy of Crouzon syndrome through FGFR2 gene knock-down by recombinant human ferritin-based targeted siRNA delivery
ZIMMERMANN Valérie, Montpellier, France
Combinatorial treatment with gene and cell therapy for the treatment of SCID

Commission : Thérapie Pharmacologique des maladies neuromusculaires et Recherche Translationnelle

Aides aux jeunes chercheurs post-doctorants

DORT Junio, Montréal, Canada
Using specialized pro-resolving mediators to treat Duchenne muscular dystrophy

Financements de projets Tremplins

BEN-ELIEZER Noam, Tel Aviv, Israël
A quantitative MRI platform for simultaneous and automatic quantification of fat infiltration and T2 relaxation times in neuromuscular disorders
DUARTE Sonia, Coimbra, Portugal
Repurposing FDA-approved Drugs as microRNA-specific modifiers towards a new and promising therapeutic approach for Machado-Joseph disease/spinocerebellar ataxia type 3 (MJD/SCA3)
SACCHETTO Roberta, Legnaro, Italie
Use of small molecules to corREct defective SERCA Proteins cAusIng BRody disease
SNOWDEN Stuart, London, Royaume-Uni
Identifiying metabolic signatures of pathology in muscle and blood to identify biomarkers of oculopharyngeal muscular dystrophy

Financements de projets

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
CIRAK Sebahattin, Köln, Allemagne
Biomarker discovery and validation for LGMD2I/FKRP-related muscular dystrophy
DE LUCA Annamaria, Bari, Italie
Selected growth hormone secretagogues (GHS) with wide mechanism of actions as disease modifiers in Duchenne Muscular Dystrophy: a multidisciplinary proof-of-concept study in mdx mouse model
DEVAUX Jérôme, Montpellier, France
Pathogenic mechanisms of anti-neurofascin 155 IgG4: Role of bivalency and novel therapeutic approaches
DORCHIES Olivier, Genève, Suisse
Duchenne muscular dystrophy: Phenotyping and validation of better murine models for improving preclinical research and clinical translation
HORNSTEIN Eran, Rehovot, Israël
Rigorous, integrated miRNA-DNA plasma biomarkers for amyotrophic lateral sclerosis
LAZARIDIS Konstantinos, Athens, Grèce
Preclinical study of antigen-specific tolerance induction for the treatment of myasthenia gravis
MESSINA Graziella, Milano, 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
Development of serum miRNAs as biomarkers for the prognosis of Myotonic Dystrophy type I

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

Commission : Médicale

Financements de projets

DIAZ-MANEIRA Jordi, New Castle, Royaume-Uni
Implementation of an artificial intelligence module on the web-based digital platform MyoShare for guiding the diagnosis of muscle diseases

LEOTARD Antoine, Garches, France
Early identification of respiratory exacerbations using NIV device monitoring in slowly progressive neuromuscular disorders

LETELLIER Guy, Nantes, France
Efficacy and safety evaluation of the ExoMS exoskeleton: an innovative impressed 3D upper limb assistive device for children with neuromuscular diseases. A Single-Case Experimental Design trial using Goal Attainment Scaling

SACCONI Sabrina, Nice, France
New clinical outcome measures to evaluate non-ambulant FSHD patients

TASCA Giorgio, Roma, Italie
Natural history of distal and myofibrillar myopathies assessed by clinical and technological outcome measures: a new toolbox for clinical trials

VUILLEROT Carole, Bron, France
Development and validation study of a Motor Function Measure digitalized playful completion modules

Appel d'offres Doctorants

ALMYRE Claire, Bordeaux, France
Investigation in human cells and mice of FDA-approved chemicals showing beneficial effects in yeast models of distinct metabolic disorders with a nuclear or mitochondrial genetic origin

AMMAR Nourhene, Toulouse, France
Live-imaging of adult muscle stem cell activation in Drosophila

BEAUJARD Bettina, Paris, France
L'annonce diagnostique d'une maladie neuromusculaire à l'âge adulte. Retentissements psychologiques des processus communicationnels entre médecin et patient

BOUCHARD Laetitia, Marseille, France
Thèse rattachée au pôle stratégique

CHALUMEAU Anne, Paris, France
Development of a universal prime editing approach to β-hemoglobinopathies

CHEVREAU Robert, Montpellier, France
Role of Hippo/YAP pathway in maintenance and differentiation of adult spinal cord stem cells: a potential cellular source for spinal cord degenerative pathologies

CLAEYSSEN Charlotte, Loos, France
O-GlcNAcylation and its interplay with phosphorylation: which impact on the cytoarchitecture and the function of skeletal muscle?

D'AGATA Léna, Paris, France
Annexins as genetic modifiers of human muscular dystrophies

DA CUNHA Dylan, Montpellier, France
Unravelling molecular mechanisms of Exon Junction Complex (EJC) in splicing regulation of the DMD gene

DEBAR Louis, Aubière, France
Disease-causing mutations in human mitochondrial DNA replication factors: A single-molecule study

DELAFENETRE Arnaud, Poitiers, France
Functional characterization of muscle cells derived from healthy and DMD human induced Pluripotent Stem Cells: focus on calcium release channels

HOUQUES Chloé, Montpellier, France
Combinatorial treatment with gene and cell therapy for the treatment of SCID

KOBON Kassandra, Paris, France
Control of myoblast cell-cell fusion - Crucial role of actin-based structures

KOTAICH Farah, Montpellier, France
Neurofilaments in health and Charcot-Marie-Tooth diseases

LAPENDRY Audrey, Lyon, France
Amino acid metabolism and alternative splicing

MATHIEU Maxime, Toulouse, France
Characterization and functional role of adipose-derived fibro-adipogenic progenitors (AD-FAPs) in skeletal muscle regeneration

MATOUK Meriem, Montigny-Le-Bretonneux, France
Elucidate the role of dystrophin in the muscle stem cell niche

MOUNIER Coline, Paris, France
Cell-Type-Specific Gene and Sterol Profiling following CYP46A1 gene therapy in Huntington's disease

NAIT-SAIDI Rima, Montpellier, France
Role of oxidative stress and endoplasmic reticulum stress in OPMD: molecular mechanisms and pharmacological treatments

VAHDAT Juliette, Marseille, France
Exploring ventricular conduction system structure and function in DMD mouse models

VARUK Olena, Marseille, France
Analysis of an allelic series of mitofusin MARF mimicking mutations associated to Charcot-Marie-Tooth type 2A disease in the Drosophila motor neurons

YILDIRIM Zuleyha, Illkirch, France
Role of PARP3 in the differentiation of muscle stem cells in mice

ZERAD Lisa, Paris, France
Role of ADAR1 and RNA editing in Schwann cells development and myelin maintenance

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
D'AMATI Giulia, Roma, Italie
Stabilization of tRNAs as a therapeutic strategy for diseases due to mutations in mt-tRNAs

Appel d'offres thématique Recherche Dystrophie Myotonique

FURLING Denis, Paris, France
Tricyclo-DNA antisense oligonucleotide treatment for Myotonic Dystrophy

Partenariats institutionnels

KIM Minchul, Montpellier, France
The biology of syncytial cells: Dissecting the mechanisms and functions of nuclear differentiation inside skeletal muscle syncytium
LAUGEL Vincent, Strasbourg, France
Cohorte de suivi longitudinal SMA-DNN: Dépistage néonatal de l'Amyotrophie Spinale - Projet pilote SMA-DNN France

Partenariats associatifs

Soutien à l'association Cure CMD
ZITO Ester, Torrance, Etats-Unis
Ablation of the maladaptive ER stress response improves altered calcium handling and restores diaphragm function in SELENON knock-out mice
Soutien au Fond de Dotation IFCAH
BABOT Ruiz, Dresden, Allemagne
Generation of human steroidproducing organoids: a new approach towards a treatment for CAH
Soutien à l'association Retina France
ROGER Jérôme, Saclay, France
Expression de CRX par thérapie génique visant des modèles de dystrophies rétiniennes indépendantes de mutations dans CRX
Soutien à l'association Vaincre la Mucoviscidose (VLM)
CADARS Magali, Montpellier, France
ONB-CFTR : Stratégie OligoNucléotide Bloqueur : un outil spécifique pour stabiliser ou corriger les transcrits CFTR
LEJEUNE Fabrice, Lille, France
Optimisation de la restauration de la fonction CFTR in vivo à partir d'un gène porteur d'une mutation non-sens par inhibition du NMD et activation de la translecture
Soutien à l'IRME, Paris, France
HUGNOT Jean-Philippe, Montpellier, France
Aging and Controlling the Fate of Human Spinal Cord stem cells

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

BÜNING Hildegard, Hannover, 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
MUSARO Antonio, Roma, 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
NICOLE Sophie, Montpellier, France
Nav1.4 activators for a correction of SCN4A-related muscle weaknesses
PALACIOS Daniela, Roma, Italie
Functionalized nanoparticles for targeted genome editing in Duchenne Muscular Dystrophy
VISCOMI Carlo, Padova, 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
DUMONCEAUX Julie, London, Royaume-Uni
DUX4 in FSHD: pathophysiology and therapeutic approaches
GUEDAT Philippe, Nantes, France
A double-blind, randomized, placebo-controlled, parallel-group phase 2 study to evaluate the safety, efficacy, and pharmacokinetics of IFB-088 in patients with a Charcot-Marie-Tooth disease caused by either a duplication of PMP22 gene (CMT1A), a point mutation in the PMP22 gene (CMT1E) or a mutation in MPZ gene (CMT1B)
LAPORTE Jocelyn, Illkirch, France
Pathophysiology and therapeutic proof-of-concepts for congenital myopathies
MUNTONI Francesco, London, Royaume-Uni
Advances in oligonucleotide-mediated exonskipping for DMD and related disorders - WP3 - Natural history extension
MYOPHARM Program, Evry, France
Collaborative program for a systematic, mecanistic and pharmacologic approach of rare neuromuscular diseases
POURQUIE Olivier, Boston, Etats-Unis
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

Pôles stratégiques

LEVY Nicolas, Marseille, France
Translational Research in Marseille: towards Therapeutic Development for Rare Diseases
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, Paris, France
Appel à projet auprès des filières de santé maladies rares
FONDATION MALADIES RARES, Paris, France
Subvention 2021
GENOPOLE, Evry, France
Participation financière de l'AFM au budget 2021 du GIP GENOPOLE

Outils stratégiques

BASSEZ Guillaume, Créteil, France
iDM-scope: the international French-Quebec myotonic dystrophy registry
DESGUERRE Isabelle, Paris, France
A national clinical (and genetic) database for dystrophinopathies (DYS Registry)
LAFORET Pascal, Garches, France
Sarcoglycanopathy French Registry
MALFATTI Edoardo, Créteil, France
French national registry for calpainopathies
SACCONI Sabrina, Nice, France
French National Registry for FacioScapuloHumeral muscular Dystrophy (FSHD)
SACCONI Sabrina, Nice, France
Resolve FSHD: clinical trial readiness to solve barriers to drug development in FSHD
WAHBI Karim, Paris, France
Cardiomyopathy database

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

BERRIH-AKNIN Sonia, Paris, France
ISNI Congress 2021
BRYANT Kathryn, Columbus, Etats-Unis
International Limb Girdle Muscular Dystrophy Conference
GOMEZ Ana Maria, Châtenay-Malabry, France
Calcium Signaling and Excitation-Contraction Coupling
JAMAR Gaëlle, Paris, France
ESGCT and national societies of Europe Virtual congress
KINOSHITA June, Lexington, Etats-Unis
28th Annual FSHD Society International Research Congress
TEDESCO Francesco Saverio, London, Royaume-Uni
Society for Muscle Biology (SMB) 2021 Frontiers in Myogenesis Conference "Skeletal muscle: development, regeneration and disease”

Plateforme non stratégique

CARRE Monique, Mézilles, France
Financement CEDS