LISTE DES FINANCEMENTS ACCORDES PAR L'AFM-TELETHON EN 2019

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'appel d'offres

Commission : Myologie fondamentale

Aides aux jeunes chercheurs post-doctorants

CHOQUET Caroline, Marseille, France
Deciphering the potency of ventricular trabeculae to repair the heart during cardiac regeneration in the mouse

STANTZOU Amalia, Montigny-Le-Bretonneux, France
Elucidate the cellular and molecular mechanisms involved in the generation of revertant dystrophin-positive fibers using the dystrophic DmdEGFP-mdx reporter mouse

VICENTE GARCIA Cristina, Séville, Espagne
Contribution of the Mrf4 gene towards muscle function during exercise-mediated hypertrophy, regeneration and aging

Financements de projets Tremplins

BIRMAN Serge, Paris, France
Are Rhesus glycoproteins and the control of ammonia homeostasis required for muscle integrity and neuromuscular function? A study in the Drosophila model

Financements de projets

AMTHOR Helge, Montigny-Le-Bretonneux, France
Elucidate the molecular mechanism leading to the generation of revertant dystrophin-positive fibers in muscle dystrophic DmdEGFP-mdx reporter mouse

AUBOEUF Didier, Lyon, France
Interplay between cell metabolism and alternative splicing

BLAAUW Bert, Padova, Italie
The role of Raptor/mTORC1 in adult skeletal muscle

BOUTER Anthony, Pessac, France
Annexins in sarcolemma repair of healthy and pathological human skeletal muscle

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

DANTZER Françoise, Illkirch, France
Role of PARP3 in the differentiation of muscle stem cells and impact in muscle pathologies

DAUBAS Philippe, Paris, France
Role of LRRFIP2 in the control of heart development and skeletal muscle regeneration

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

HOUDUSSE Anne, Paris, France
Structural studies of cardiac myosin for therapeutical approaches against HCM

MAIRE Pascal, Paris, France
Genetic and epigenetic control of adult muscle fiber phenotype

MIQUEROL Lucile, Marseille, France
Deciphering the potency of ventricular trabeculae to repair the heart during cardiac regeneration in the mouse
MOZZETTA Chiara, Roma, Italie
Deciphering the role of Prdm16-mediated H3K9 methylation in the control of Fibro-Adipogenic Progenitors identity and skeletal muscle repair
RAZ Vered, Leiden, Pays-Bas
RNA processing role in muscle degeneration opens therapeutical options for adult myopathies
RAZ Vered, Leiden, Pays-Bas
Identification of muscle-specific molecular networks
ROMANELLO Vanina, Padua, Italie
Deciphering a novel link between the ubiquitin proteasome system and mitochondrial function to control muscle mass
TAJBAKHSH Shahragim, Paris, France
Investigating heterogeneities and morphogenesis of cranial mesoderm derived skeletal muscles
VINCENT Alain, Toulouse, France
Combinatorial control of muscle morphology by conserved myogenic Transcription Factors
VOLK Talila, Rehovot, Israël
Mechanical signals transduced downstream of the LINC complex-mediated muscular dystrophies

Commission : Bases Moléculaires et Physiopathologie des Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

SCHIAVONE Marco, Padova, Italie
Role of Cyclophilins in Duchenne Muscular Dystrophy
TORCINARO Alessio, Roma, Italie
In vivo characterization of miR-200c in regeneration of dystrophic skeletal muscles in mdx mice

Financements de projets Tremplins

CENACCHI Giovanna, Bologna, Italie
Morphofunctional characterization of transportin 3 (TNPO3) in the pathogenesis of limb-girdle muscular dystrophy 1F
GABELLINI Davide, Milano, Italie
Characterization of a novel inhibitor of DUX4 expression
MAGENTA Alessandra, Roma, Italie
Role of miR-200c in dystrophic muscle regeneration of mdx mice and DMD patients

Financements de projets

CENCI Giovanni, Roma, Italie
Functional analysis of separase-dependent lamins' regulation in AD-EDMD
CHARLET-BERGUERAND Nicolas, Illkirch, France
Physiopathology of muscle atrophy in myotonic dystrophy
CLEMENTI Emilio, Milano, Italie
PGC1 alpha gene expression regulation and Mitochondrial Biogenesis impairment in Muscular Dystrophies: new molecular signatures for novel therapeutic strategy
HUBE Florent, Paris, France
Newly identified non-coding RNAs from alternatively spliced introns in normal and pathological muscle differentiation
LAMAZE Christophe, Paris, France
Caveolin-assisted sphingolipid transport to the plasma membrane in epidemiology of muscle dystrophies
LOPEZ PADRINO Jose Rafael, Miami, Etats-Unis
Whole body periodic acceleration a novel treatment for duchenne cardiomyopathy in mdx mice

MAMMUCARI Cristina, Padova, Italie
Targeting the Mitochondrial Calcium Uniporter to counteract Duchenne Muscular Dystrophy
MORALES Fernando, San José, Costa Rica
Comparitive expression profiling of multiple tissues in myotonic dystrophy
MUNOZ-CANOVES Pura, Barcelona, Espagne
Novel strategies to ameliorate Duchenne Muscular Dystrophy
RAVEL-CHAPUIS Aymeric, Ottawa, Canada
Novel AMPK activators as relevant therapeutics for the treatment of Myotonic Dystrophy type 1 (DM1)

Commission : Bases moléculaires et physiopathologie des autres maladies neuromusculaires

Aides aux jeunes chercheurs post-doctorants

HEISKE Margit, Toulouse, France
Virtual Cybrids: In silico approach to simulate the influence of haplogroups on the mitochondrial energy metabolism
HORAK Martin, Paris, France
Molecular mechanism of mitochondrial disease caused by impaired protein processing in mitochondria

Financements de projets Tremplins

BEGGS Alan, Boston, Etats-Unis
Modeling the heterogeneity of tropomyosin-related congenital myopathies
FIORILLO Chiara, Genoa, Italie
Defining molecular and functional consequences of titin mutations in human muscle progenitors from affected patients
SANCHEZ-DUFFHUES Gonzalo, Leiden, Pays-Bas
Normalization of the vasculature to prevent heterotopic ossification in Fibrodysplasia ossificans progressiva
SARPARANTA Jaakko, Helsinki, Finlande
Autophagy in C-terminal titinopathies

Financements de projets

COSSEE Mireille, Montpellier, France
Functional Analyses and Phenotype-Genotype Correlation Studies in Patients Suspected of Titinopathy
DEVAUX Jérôme, Marseille, France
IgG4 in inflammatory neuropathies: pathogenic effects and novel targets
FARGE Geraldine, Aubière, France
Molecular mechanisms of mtDNA maintenance in human health and disease
HEARD Edith, Heidelberg, Allemagne
Allele-specific epigenetic regulation of Bag3: physiopathological implications and new strategies for dilated cardiomyopathy
LAUNIKONIS Bradley, Brisbane, Australie
Junctional membrane calcium dynamics in skeletal muscle fibres with ryanodine receptor variants
MERCIER Sandra, Nantes, France
Unravelling POIKTMP pathophysiology for design of therapeutic approaches
METODIEV Metodi, Paris, France
Deficient protein processing in mitochondrial diseases
MEYER Alain, Strasbourg, France
Characterization of the mechanisms underlying perifascicular muscle fiber abnormalities in patients with dermatomyositis
SCORRANO Luca, Padova, Italie
Enhancing Opa1-dependent cristae structure to combat mitochondrial diseases

SWAN Laura, Liverpool, Royaume-Uni
INPP5K-mediated congenital muscular dystrophy models of motor neuron branching and function
TRIBOUILLARD-TANVIER Deborah, Bordeaux, France
Nuclear genetic suppressors in yeast models of mtDNA mutations associated to neuromuscular diseases

Commission : Système nerveux : Motoneurone et jonction neuromusculaire

Aides aux jeunes chercheurs post-doctorants

GONZÁLEZ David, Montpellier, France
Neurofilaments in Health and Charcot-Marie Tooth diseases
O'CONNOR Emily, Newcastle-Upon-Tyne, Royaume-Uni
A study of the NMJ in GFPT1-deficient zebrafish
ORTEGA CANO Juan Alberto, Chicago, Etats-Unis
Studying nucleocytoplasmic imbalances to design new therapeutic strategies for amyotrophic lateral sclerosis
TOUVIER Thierry, Milano, Italie
Role of the ER stress transcription factor XBP1 in Charcot-Marie-Tooth disease

Financements de projets Tremplins

GENDREL Marie, Paris, France
New actors for GABA transport
PEETERS Kristien, Antwerp, Belgique
Characterization of HINT1 knockout fly model for peripheral neuropathy
PIRAZZINI Marco, Padova, Italie
Signaling at the neuromuscular junction during aging

Financements de projets

BOMONT Pascale, Montpellier, France
Neurofilaments in Health and Charcot-Marie Tooth diseases
BOMONT Pascale, Montpellier, France
Analysis of the first phenotypical mouse model for Giant Axonal Neuropathy
BORDONNE Rémy, Montpellier, France
Defining new functions for the SMN complex from yeast to human
EL BEHI Mohamed, Paris, France
Pathophysiology of chronic inflammatory demyelinating polyneuropathies (CIDP): from patients' deep immunophenotyping to preclinical testing. Toward the identification of new therapeutic targets
ESPINOS Carmen, Valencia, Espagne
An integrative approach to develop cellular models and characterize disease mechanisms implicated in CMT2Z, a newly described axonal form of neuropathy
JASMIN Bernard, Ottawa, Canada
Inhibition of HDAC6 as Therapeutic Strategy in Neuromuscular Diseases
KREJCI Eric, Paris, France
Guenuine mouse models to analyze congenital myasthenic syndrome with acetylcholinesterase deficit
LAMOTTE D'INCAMPS Boris, Paris, France
Synaptic Inputs from and to Vulnerable and Resistant Motoneurons in Amyotrophic Lateral Sclerosis
LEGAY Claire, Paris, France
MuSK intracellular pathways in congenital myasthenic syndromes
LEVY-LAHAD Ephrat, Jerusalem, Israël
The motor neuron disease gene VRK1: a conditional VRK1 knockout mouse as a novel model for neuromuscular disease

MARTIN Franck, Strasbourg, France
Deciphering the mechanisms of repeat-associated non-AUG (RAN) translation in amyotrophic lateral sclerosis

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 subtypes

PENNUTO Maria, Padova, Italie
Targeting AR co-regulators to attenuate spinal and bulbar muscular atrophy

RINALDO Cinzia, Roma, Italie
The HIPK2 kinase/spastin axis in Hereditary Spastic Paraplegia (HSP): functional roles and potential therapeutic applications

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

ROOS Andreas, Dortmund, Allemagne
Proteogenomics to solve the unsolved exemplified by gene identification in congenital myasthenic syndromes

VIERO Gabriella, Povo (Trento), Italie
In vivo translational alterations in spinal muscular atrophy at single nucleotide resolution

Commission : Cellules souches

Aides aux jeunes chercheurs post-doctorants

BOUKHATMI Hadi, Toulouse, France
Molecular mechanisms governing Drosophila Satellite Cells stemness and reactivation.

BRUN Caroline, Ottawa, Canada
Stimulating cilia-mediated Hedgehog signaling to restore dystrophin-deficient satellite cell function

CONSALVI Silvia, Roma, Italie
Exosome-mediated HDACi/miR-143/STAT3 network in the regulation of satellite cells expansion and muscle regeneration

THERET Marine, Vancouver, Canada
Role of Tak1 activity in muscle-resident fibro/adipogenic progenitors. A key modulator of the inflammatory environment and a therapeutic target in chronic disease.

Financements de projets Tremplins

CONSALVI Silvia, Roma, Italie
Identification of the chromatin profile predicting the responsiveness of dystrophic patients to epigenetic therapy

CRISAN Mihaela, Edinburgh, Royaume-Uni
Mesenchymal stem cell behaviour in pericyte-deficient mice in vivo: are cardiac and skeletal muscle affected?

LI Han, Paris, France
The impact of cellular senescence and in vivo reprogramming in Duchen-Muscular Dystrophy

MAYEUF-LOUCHART Alicia, Lille, France
Role of the biological clock in muscle stem cells

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

BIRCHMEIER Carmen, Berlin, Allemagne
Oscillatory expression controlling the fate of muscle stem cells
BOHL Delphine, Paris, France
Deciphering respective contributions of macrophages and microglia to motor neuron degeneration in Spinal Muscular Atrophy and Amyotrophic Lateral Sclerosis with microfluidic devices
MOREY Céline, Paris, France
Investigating the function of the FTX long non-coding RNA in the definition of spinal motoneuron identities
PUYMIRAT Jack, Québec, Canada
Human iPSC-derived cerebral organoids as a brain model of congenital myotonic dystrophy type 1
SACCONE Valentina, Roma, Italie
New therapeutic strategies based on FAPs-derived Exosomes in the treatment of Duchenne Muscular Dystrophy
SOTIROPOULOS Athanassia, Paris, France
How myoblast cell-cell fusion is controlled? Crucial role of actin-based structures and of Srf
STUDER Michèle, Nice, France
In vivo and in vitro direct lineage reprogramming of neuronal and somatic cells to corticospinal motor neurons
TAJBAKHSH Shahragim, Paris, France
DNA methylation and transcriptional profiling of muscle stem cells in distinct states

Commission : Thérapie Génique et/ou Cellulaire des Maladies Rares

Aides aux jeunes chercheurs post-doctorants

FRATI Giacomo, Paris, France
CRISPR/Cas9 mediated induction of fetal hemoglobin synthesis for the treatment of β-hemoglobinopathies
SAVCHENKO Ekaterina, Lund, Suède
AAV-mediated delivery of FGF as a therapeutic strategy for Amyotrophic Lateral Sclerosis

Financements de projets Tremplins

RUZZENENTE Benedetta, Paris, France
Design of an AAV-based gene therapy for mitochondrial disease caused by mutations in the RNA stability factor LRPPRC

Financements de projets

CORRAL-DEBRINSKI Marisol, Paris, France
Development of a Gene Therapy with Neuroglobin aimed at treating Cerebellar Ataxias, independently of their genetic origin, by beneficiating of neuroglobin durable protective effect on mitochondrial function
DALKARA Deniz, Paris, France
Non viral gene editing for autosomal dominant retinitis pigmentosa
DEGLON Nicole, Lausanne, Suisse
Toward gene repair for CNS genetic disorders
MECHALI Marcel, Montpellier, France
Replication Origins containing Episomes for Gene Therapy
MICCIO Annarita, Paris, France
Development of Innovative Therapeutic Strategies for Beta-Hemoglobinopathies
MOREAU-GAUDRY François, Bordeaux, France
Safety of CRISPR-Cas9 nuclease use
MUSCATELLI Françoise, Marseille, France
Necdin gene therapy to restore normal breathing in a mouse model of Prader-Willi syndrome

NOBREGA Clevio, Faro, Portugal
Overexpression of the RNA-binding protein G3BP1 as a therapeutic strategy to Machado-Joseph disease and other Polyglutamine diseases
RICHARD Guy-Franck, Paris, France
Gene therapy in DM1 cells by induction of a TALE Nuclease
ROYBON Laurent, Lund, Suède
AAV-mediated delivery of FGF as a therapeutic strategy for amyotrophic lateral sclerosis
ZIMMERMANN Valérie, Montpellier, France
Intrathymic AAV gene transfer: a novel strategy for the correction of genetic immunodeficiencies

Commission : Thérapie Pharmacologique des maladies neuromusculaires et Recherche Translationnelle

Aides aux jeunes chercheurs post-doctorants

LAMBERT Matthias, Boston, Etats-Unis
Discovery of small-molecules modulating new modifier genes in Duchenne Muscular Dystrophy: a novel and promising therapy to escape the dystrophic phenotype

Financements de projets Tremplins

CESCON Matilde, Padova, Italie
The neuromuscular junction as a novel therapeutic target for Collagen VI myopathies
DÍAZ-MANERA Jordi, Barcelona, Espagne
Effect of Nintedanib in muscle fibrosis in a mouse model of sarcoglycanopathy and in the function of human and mouse FAP cells

Financements de projets

ARTERO Ruben, Burjassot, Espagne
Development of combinatorial therapies for SMA
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
DORCHIES Olivier, Geneva, Suisse
Pre-clinical evaluation of tamoxifen in mouse models of X-linked centronuclear myopathy and other severe muscular diseases
FERREIRO Ana, Paris, France
Selnac : towards a first therapeutic trial for SEPN1-related myopathy
HORNSTEIN Eran, Rehovot, Israël
Rigorous, integrated miRNA-DNA plasma biomarkers for amyotrophic lateral sclerosis
LOEFFLER Jean-Philippe, Strasbourg, France
Modulation of metabolic flexibility in Amyotrophic Lateral Sclerosis as a new therapeutic approach
PHYLACTOU Leonidas, Nicosia, Chypre
Development of miRNA-based biomarkers for monitoring the progress in Myotonic Dystrophy type I
RONJAT Michel, Nantes, France
New modulators of the skeletal and cardiac ryanodine receptors

Commission : Médicale

Financements de projets Tremplins

GALLAIS Benjamin, Jonquière, Canada
Abilities of adults with the childhood phenotype of myotonic dystrophy type 1 to live independently: an integrated occupational therapy and neuropsychological study

Financements de projets

GAGNON Cynthia, Sherbrooke, Canada
Development of a questionnaire to assess the severity of dysphagia in oculopharyngeal muscular dystrophy

NECTOUX Juliette, Paris, France
Clinical Implementation of Noninvasive Prenatal Testing for Duchenne Muscular Dystrophies

Appel d'offres Doctorants

ASFOUR Hasan, Montigny-le-Bretonneux, France
Bone morphogenetic proteins regulate patterning of limb muscles

BARBEAU Susie, Paris, France
MuSK intracellular pathways in congenital myasthenic syndromes

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

BOGARD Baptiste, Paris, France
Small non-coding RNAs of intron origin in Myotonic Dystrophy type 1: innovative biomarkers and candidate drivers of splicing defects

BOUCHARD Laetitia, Marseille, France
Evaluation of FGF10 as a target for promoting heart regeneration in Lmna-related dilated cardiomyopathy

CHATZOVOULOU Kalliopi, Paris, France
Mitochondrial gene expression in normal, mutant and 3-parent preimplantation embryos

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

DA CUNHA Dylan, Montpellier, France
Unravelling molecular mechanisms of DMD gene splicing regulation and their roles as disease modifiers in Duchenne muscular Dystrophy

DANIEL Malo, Nantes, France
Characterization of Liver-induced allo-specific CD8 regulatory T lymphocytes

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

HOVHANNISYAN Yeranuhi, Paris, France
Cardiac modelling of myofibrillar myopathy using human pluripotent stem cells to explore cardiac pathogenesis and drug testing

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

LAURENT Adrien, Montigny-Le-Bretonneux, France
Induction of immunological tolerance by dual muscle and liver gene transfer for Duchenne muscular dystrophy

MALKO-BAVEREL Vassilissa, Paris, France
Can a gene therapy with Neuroglobin (NGB) prevent or delay the ataxic phenotype in Harlequin mice (Hq) by preserving mitochondrial function in their cerebellum?

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

NOVIELLO Chiara, Paris, France
Study of the role of RhoA in skeletal muscle plasticity

PICOT Mélanie, Toulouse, France
Phosphoinositide-dependent regulation of mTORC1 in skeletal muscle: molecular insights and involvement in myotubular myopathy

POGGI Lucie, Paris, France
Gene therapy in DM1 cells by induction of a TALE Nuclease

VARUK Olena, Marseille, France
Deciphering the dominant active mechanism of mitofusin alleles associated with Charcot-Marie-Tooth type 2A disease

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

Appel d'offres « ARN médicament et cible thérapeutique »

D'AMATI Giulia, Roma, Italie
Stabilization of tRNAs as a therapeutic strategy for diseases due to mutations in mt-tRNAs
FRANCO Brunella, Pozzuoli, Italie
Modulation of miR181a/b as a new therapeutic approach for Leber hereditary optic neuropathy (LHON)
HUDA Ruksana, Galveston, Etats-Unis
Target specific antibody-siRNA conjugate therapy for experimental myasthenia
VAN ROON-MOM Willeke, Leiden, Pays-Bas
Final proof of concept for the advancement of antisense oligonucleotide treatment for SCA3 towards the clinic

Appel d'offres « Recherche Translationnelle sur la Dystrophie Myotonique »

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

Financement de projet de l'Appel d'offres « SMA Europe »

ALLAIN Frédéric, Zurich, Suisse
Seeking small molecules that stabilize protein-RNA interactions to cure Spinal Muscular Atrophy
GILLINGWATER Thomas, Edinburgh, Royaume-Uni
Defining the role of the motor axon translatome in SMA pathogenesis

Partenariats institutionnels

ENRIQUEZ Jonathan, Paris, France
Making motoneurons unique

Partenariats associatifs

Soutien à l'association Retina France
BELENGUER Pascale, Toulouse, France
Proof of principle of an original therapeutic strategy for dominant optic atrophy
ZEITZ Christina, Paris, France
Validation of a promising gene therapy for CSNB
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
HINZPETER Alexandre, Paris, France
Influence de la partie non codante 3'UTR sur le niveau de dégradation par le NMD de transcrits contenant des codons stop prématurés.

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

MELKI Judith, Le Kremlin-Bicêtre, France
New Genomics Approaches in Neuromuscular Disorders: Applications in the Identification of New Disease Causing Genes and Mechanisms, of Revertant Somatic Mosaicism and in Undiagnosed Patients
MIDOUX Patrick, Orléans, France
DMD non-viral gene therapy
STROCHLIC Laure, Paris, France
MuSK frizzled-like domain at the NMJ: novel insight into pathophysiology and therapeutics for myasthenia

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

BIOPHYTIS, Paris, France
A 3-part, Randomized, Multicentric, Double Blind, Seamless Phase 1-3 Study to Evaluate the Safety, Efficacy, Pharmacokinetics, and Pharmacodynamics of BIO101 in Pediatric Patients with a Genetically Confirmed Diagnosis of Duchenne Muscular Dystrophy (International study)

DUMONCEAUX Julie, London, Royaume-Uni
DUX4 in FSHD: pathophysiology and therapeutic approaches

LAPORTE Jocelyn, Illkirch, France
Pathophysiology and therapeutic proof-of-concepts for congenital myopathies

MYOPHARM Program, Evry, France
Collaborative program for a systematic, mecanistic and pharmacologic approach of rare neuromuscular diseases

PROCACCIO Vincent, Angers, France
Identifying candidate drugs in mitochondrial cardiomyopathies: From Mouse to Human

ROTIG Agnès, Paris, France
An integrated approach for MITOchondrial disorder THERApeutics from yeasts and worms to humans

VASSETZKY Yegor, Villejuif, France
CTCF-dCas9 fusion protein targeting the 4q35 insulator for re-establishment of the epigenetic state and FSHD treatment

WOOD Matthew J.A., Oxford, Royaume-Uni
Pre-clinical development of peptide-oligonucleotides for DM1

Pôles stratégiques

LEVY Nicolas, Marseille, France
Translational Research in Marseille: towards Therapeutic Development for Rare Diseases

RELAIX Frédéric, Créteil, France
An integrated translational program for neuromuscular disorders

SCHAEFFER Laurent, Villeurbanne, France
Alliance MyoNeurALP - a research network dedicated to Neuromuscular disorders

Structures stratégiques

FONDATION MALADIES RARES, Paris, France
Subvention 2019

GENOPOLE, Evry, France
Participation financière de l'AFM au budget 2019 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

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

AFM-TELETHON SPCS, Paris, France
Colloque Jeunes Chercheurs 2019 - Myologie 2019 Bordeaux
CARLIER Pierre, Paris, France
MYO-MRI MRI and MRS IN NEUROMUSCULAR DISEASE
DZIEWCZAPOLSKI Gustavo, Torrance, Etats-Unis
Stronger Together: Nonprofit Groups Propelling Patient-Driven Research of Rare Neuromuscular Diseases
GABELLINI Davide, Milano, Italie
XVI International IIM-Myology Meeting: Pathogenesis and Therapies of Neuromuscular Diseases
GOMEZ Ana Maria, Châtenay-Malabry, France
Gordon research conferences on Muscle: Excitation contraction coupling
GOURDON Geneviève, Paris, France
12ième congrès du consortium international sur les dystrophies myotoniques IDMC12: "Challenges in Myotonic Dystrophy – from basic science to therapy"
JAMAR Gaëlle, Paris, France
Thematic days of the SFTCG
KATEB Babak, West Hollywood, Etats-Unis
16th Annual World Congress of Society for Brain Mapping and Therapeutics (SBMT)
LEGAY Claire, Paris, France
XVIth International Symposium on Cholinergic Mechanisms
MAGDINIER Frédérique, Marseille, France
FSH Society's FSHD International Research Congress
RELAIX Frédéric, Créteil, France
EMBO Workshop: Muscle formation, maintenance, regeneration and pathology
SPENCER Melissa, Los Angeles, Etats-Unis
FASEB Summer Research Conference The Biology of Calpains in Health and Disease
TAILLANDIER Daniel, St Genès Champanelle, France
9th Proteasome & Autophagy Congress
VINCENT Michel, Lyon, France
First International Workshop on therapeutic strategy for Farber and SMA-PME diseases
ZAMMIT Peter, London, Royaume-Uni
Frontiers in Myogenesis Skeletal Muscle: Development, Regeneration and Disease
ZHANG Qiuping, London, Royaume-Uni
9th Nuclear Envelope Disease and Chromatin Organization meeting and 3rd International Meeting on Laminopathies joint conference

Plateforme non stratégique

CARRE Monique, Mézilles, France
Financement CEDS