

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

Comparative 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

Genuine 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 VPK1: a conditional VPK1 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 Duchenne-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 TGF β /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

SACCONI 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 benefiting 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
Thèse rattachée au pôle stratégique
- 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**, Aubièrre, 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 Cassandra**, 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 translosome 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