LISTE DES FINANCEMENTS ACCORDES PAR L'AFM-TELETHON EN 2020

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'appel d'offres

Commission : Myologie fondamentale

Aides aux jeunes chercheurs post-doctorants

DE MARIO Agnese, Padova, Italie
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
KIM Minchul, Berlin, Allemagne
Transcriptional control of the myotendinous junction
SIDOR Clara, Marseille, France
Investigating the mechanism of sarcomere assembly during myogenesis
VICENTE GARCIA Cristina, Séville, Espagne
Contribution of the Mrf4 gene towards muscle function during exercise-mediated hypertrophy, regeneration and aging

Financements de projets Tremplins

COMAI Glenda, Paris, France
Cellular and genetic basis for robustness of cranial myogenic populations

Financements de projets

BORYCKI Anne-Gaëlle, Sheffield, Royaume Uni
Patched 1 function in skeletal muscle stem cells and its implication in muscle wasting
BOUTER Anthony, Pessac, France
Annexins in sarcolemma repair of healthy and pathological human skeletal muscle
DANTZER Françoise, Illkirch, France
Role of PARP3 in the differentiation of muscle stem cells and impact in muscle pathologies
DUPREZ Delphine, Paris, France
Molecular interactions between connective tissue fibroblasts and muscle cells in order to build a full musculoskeletal system
LECUIT Marc, Paris, France
Studying chikungunya virus infection of muscles to better understand the contribution of FHL1 to myogenesis
MAIRE Pascal, Paris, France
Fast myosin heavy chain locus regulation
PENDE Mario, Paris, France
Role of mitochondrial YAP and TAZ in muscle growth, regeneration and mechanotransduction
RAZ Vered, Leiden, Pays-Bas
Identification of muscle-specific molecular networks
SANDRI Marco, Padova, Italie
Dissecting the role of an uncharacterized FoxO-dependent gene that controls autophagy and ageing
VÉNIEN-BRYAN Catherine, Paris, France
Structural and cellular characterization of a potassium channel, Kir, involved in Andersen's syndrome

VOLK Talila, Rehovot, Israël
Mechanical signals transduced downstream of the LINC complex-mediated muscular dystrophies
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

TORCINARO Alessio, Roma, Italie
In vivo characterization of miR-200c in regeneration of dystrophic skeletal muscles in mdx mice

Financements de projets Tremplins

ANTHONY Karen, Northampton, Royaume Uni
RNA processing of the brain dystrophin isoform Dp71
DI PIETRO Lorena, Roma, Italie
Characterization of the FibroAdipogenic progenITors in FSHD patients and their role in the pathogenesis of the disease
VON WALDEN Ferdinand, Stockholm, Suède
Skeletal muscle ribosome specialization in DMD - friend or foe?

Financements de projets

CANCELA José-Manuel, Orsay, France
Restoration of Ca2+-signalling in mdx mice by targeting the endo-lysosmal two-pore channel (TPC)
CIAPPONI Laura, Roma, Italie
Analysis of the DM2 pathogenic mechanisms using Drosophila as model system
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
MARTELLI Fabio, Milano, Italie
Circular RNA role in Myotonic Dystrophy type 1
MAZZONE Massimiliano, Torino, Italie
GLUD1 as a potential target in Muscular Dystrophy
MERONI Germana, Trieste, Italie
Role of TRIM32, the Limb Girdle Muscular Dystrophy type R8 gene, in neuro-muscular differentiation and homeostasis
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
Novel AMPK activators as relevant therapeutics for the treatment of Myotonic Dystrophy type 1 (DM1)
TUFFERY-GIRAUD Sylvie, Montpellier, France
Towards a better understanding of the interplay between cis-acting elements and trans factors driving DMD pre-MRNA splicing

Commission : Bases moléculaires et physiopathologie des autres maladies neuromusculaires

Aides aux jeunes chercheurs post-doctorants

HORAK Martin, Paris, France
Molecular mechanism of mitochondrial disease caused by impaired protein processing in mitochondria

Financements de projets Tremplins

KÜHL Inge, Gif-sur-Yvette, France
Role of the N-terminal extension of POLRMT in the regulation of mammalian mitochondrial gene expression
SAVARESE Marco, Helsinki, Finlande
The expanding spectrum of ACTN2-related myopathies
SPINAZZI Marco, Angers, France
Unbiased study of the stress response mechanisms induced by mitochondrial dysfunction in Leigh syndrome

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
COSSEE Mireille, Montpellier, France
Functional Analyses and Phenotype-Genotype Correlation Studies in Patients Suspected of Titinopathy
FRIANT Sylvie, Strasbourg, France
Study of two neuromuscular diseases due to mutations in myotubularins MTM1 or MTMR2 by using yeast, cell lines and mice models
HNIA Karim, Toulouse, France
Molecular functions of MTM1-related phosphoinositides in XLCNM pathophysiology
LAUNIKONIS Bradley, Brisbane, Australie
Junctional membrane calcium dynamics in skeletal muscle fibres with ryanodine receptor variants
MAZAT Jean-Pierre, Bordeaux, France
How to bypass mitochondrial ATP synthase deficiencies? The role of mitochondrial carriers MitoBAD (Mitochondrial Bypass ATPsynthase Deficiencies)
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
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
TRIBOUILLARD-TANVIER Deborah, Bordeaux, France
Nuclear genetic suppressors in yeast models of mtDNA mutations associated to neuromuscular diseases
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

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

Financements de projets Tremplins

GRUMATI Paolo, Pozzuoli, Italie
Role of ER-PHAGY in motor neuron degeneration

HAASE Georg, Marseille, France
G4C2 Repeat Sizing by Bionano Optical Mapping

MADARO Luca, Roma, Italie
Cellular network driving neuromuscular junction stability

TOSATTO Laura, Trento, Italie
Investigating the effect of poly-Glutamine expanded androgen receptor phosphorylation in muscle cells

Financements de projets

BOLINO Alessandra, Milano, Italie
Identification of phospholipid effectors and potential biomarkers in Charcot-Marie-Tooth type 4B neuropathies

BOMONT Pascale, Montpellier, France
Analysis of the first phenotypical mouse model for Giant Axonal Neuropathy

BORTOLOZZI Mario, Padova, Italie
Linking Cx32 hemichannel dysfunction to Charcot-Marie-Tooth disease pathogenesis

BRANCHEREAU Pascal, Bordeaux, France
Chloride-related inhibition in spinal motoneurons during the ALS disease progression in mouse model

CASSEREAU Julien, Angers, France
Metabolomic exploration of dysregulated lipid metabolism in MFN2-related CMT2A

KREJCI Eric, Paris, France
Congenital myasthenic syndrome: acetylcholine and GABA

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

POLETTI Angelo, Milano, Italie
The involvement of the small heat shock protein HSPB8 in amyotrophic lateral sclerosis

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

RUZZENE Maria, Padova, Italie
Evaluation of protein kinase CK2 as a novel target for the treatment of Friedreich Ataxia

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

Commission : Cellules souches

Aides aux jeunes chercheurs post-doctorants

BOUKHATMI Hadi, Toulouse, France
Molecular mechanisms governing Drosophila Satellite Cells stemness and reactivation
PROLA Alexandre, Genève, Suisse
Metabolic profiling of adult muscle stem cells in normal and pathological conditions

Financements de projets Tremplins

BOUKHATMI Hadi, Toulouse, France
Decoding the molecular and cellular mechanisms governing Drosophila muscle stem cells maintenance and activation
GIROUSSE Amandine, Toulouse, France
Adipose-derived fibro-adipogenic progenitors subsets: promising partners to potentiate muscle regeneration?

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
ELVASSORE Nicola, Padova, Italie
Intravital 3D bioprinting approach for muscle stem cell delivery
SACCONE Valentina, Roma, Italie
Therapeutic potential of Amniotic Mesenchymal Stromal Cells and their released extracellular vesicles in the treatment of Duchenne Muscular Dystrophy
SALVATORE Domenico, Napoli, Italie
The intracellular control of Thyroid hormone signaling in the biological activity of muscle stem cells
SEBILLE Stéphane, Poitiers, France
Functional characterization of muscle cells derived from healthy and DMD human induced Pluripotent Stem Cells
SOTIROPOULOS Athanassia, Paris, France
How myoblast cell-cell fusion is controlled? Crucial role of actin-based structures and of Srf
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

FRATI Giacomo, Paris, France
CRISPR/Cas9 mediated induction of fetal hemoglobin synthesis for the treatment of β-hemoglobinopathies
RUZZENENTE Benedetta, Paris, France
Design of an AAV-based gene therapy for mitochondrial disease caused by mutations in the RNA stability factor LRPPRC
SAVCHENKO Ekaterina, Lund, Suède
AAV-mediated delivery of FGF as a therapeutic strategy for Amyotrophic Lateral Sclerosis

Financements de projets Tremplins

CARRELLA Sabrina, Napoli, Italie
CRISPR/Cas9 microRNAs Editing as gene-independent therapeutic approach in Inherited Retinal Dystrophies (IRDs)
TRAPANI Ivana, Pozzuoli, Italie
A mutation-independent genome editing approach for ABCA4 gene correction in photoreceptors

Financements de projets

ABERDAM Daniel, Paris, France
TRansdifferentiation for Autologous Corneal Cell ThERapy (TRACER)
BETUING Sandrine, Paris, France
Efficient Gene and Sterol regulations by gene transfer for striatal protection in Huntington's Disease
CHUAH Marinee, Brussel, Belgigue
NEXTGEN-AAV: Development of next-generation AAV vectors for Duchenne muscular dystrophy
DALKARA Deniz, Paris, France
Non viral gene editing for autosomal dominant retinitis pigmentosa
DITADI Andrea, Milano, Italie
Adoptive transfer of gene edited lymphoid progenitors from patient specific human induced pluripotent stem cells as a treatment of X-linked severe combined immunodeficiency
ERCEG Slaven, Valencia, Espagne
The cell therapeutic strategy for hereditary retinal dystrophies in small and large animals: MERTK associated Retinitis pigmentosa
KEMALADEWI Dwi, Pittsburgh, Etats-Unis
Therapeutic genetics and disease modeling in LAMA2-CMD
MICCIO Annarita, Paris, France
Development of Innovative Therapeutic Strategies for Beta-Hemoglobinopathies
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
ROYBON Laurent, Lund, Suède
AAV-mediated delivery of FGF as a therapeutic strategy for amyotrophic lateral sclerosis
TREMBLAY Jacques, Québec, Canada
Removal of the GAA repeat with the CRISPR/Cas9 system in Friedreich patient cells and in the YG8sR mouse model
VANDENDRIESSCHE Thierry, Brussel, Belgique
'Repeat-Repair': CRISPR/Cas9-mediated correction of triplet nucleotide repeats
VANDENDRIESSCHE Thierry, Brussel, Belgique
CRISPR/Cas-mediated in vivo gene editing for hemophilia A
YLA-HERTTUALA Seppo, Kuopio, Finlande
Gene therapy of familial hypercholesterolemia
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

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

Financements de projets

ARTERO Ruben, Burjassot, Espagne
Development of combinatorial therapies for SMA

DORCHIES Olivier, Genève, Suisse
Pre-clinical evaluation of tamoxifen in mouse models of X-linked centronuclear myopathy and other severe muscular diseases

DORCHIES Olivier, Genève, Suisse
Duchenne muscular dystrophy: Phenotyping and validation of better murine models for improving preclinical research and clinical translation

KAKHLON Or, Jerusalem, Israël
Testing new glycogen reducing small molecules for the treatment of glycogen storage disorder type 3

LOEFFLER Jean-Philippe, Strasbourg, France
Modulation of metabolic flexibility in Amyotrophic Lateral Sclerosis as a new therapeutic approach

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 miRNA-based biomarkers for monitoring the progress in Myotonic Dystrophy type I

PHYLACTOU Leonidas, Nicosia, Chypre
Development of serum miRNAs as biomarkers for the prognosis of Myotonic Dystrophy type I

RONJAT Michel, Nantes, France
New modulators of the skeletal and cardiac ryanodine receptors

ROSSI Daniela Maria, Pavia, Italie
Developing combinatorial therapies for the treatment of spinal muscular atrophy

SANDONÀ Dorianna, Padova, Italie
CFTR correctors to treat sarcoglycanopathy, a repurposing story

VAN DEN BOSCH Ludo, Leuven, Belgique
Identification of therapies targeting lipid metabolism & myelination for Charcot-Marie-Tooth disease type 1A using patient derived Schwann cells

Commission : Médicale

Financements de projets

LEOTARD Antoine, Garches, France
Patient-ventilator asynchrony in neuromuscular disease: real life evaluation using devices software's

LOFASO Frédéric, Garches, France
Validation of non-invasive non-volitional methods for monitoring respiratory muscle function in Duchenne muscular dystrophy

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

SARIS Christiaan, Nijmegen, Pays-Bas
Evaluation of dysphagia in inclusion body myositis and oculopharyngeal muscle dystrophy by combining novel ultrasound and real-time MRI

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

WAHBI Karim, Paris, France
Venous Thromboembolism in Myotonic Dystrophy Type 1: Unravelment of Molecular Mechanisms and Biomarker Development

Appel d'offres Doctorants

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

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

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

FEFEU Mylène, Paris, France
Dysfunction of muscle stem cells in sepsis and regenerative therapy

IMARRAINE Soumaiya, Paris, France
New actors involved in GABA transport

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

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

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

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

MARTINUCCI Pierre, Paris, France
Development of a universal pRIme editinG Approach To beta-hemOglobiNopathIes

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

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

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

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

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

Participation de l'AFM-Téléthon au financement du 10ème appel d'offres international SMA-Europe

Partenariats institutionnels

ENRIQUEZ Jonathan, Paris, France
Making motoneurons unique
BOUKHATMI Hadi, Paris, France
Decoding the molecular and cellular mechanisms governing Drosophila Satellite Cells maintenance and activation
DUPONT Jean-Baptiste, Paris, France
Next generation modelling of human myogenesis and skeletal muscle diseases with pluripotent stem cells

Partenariats associatifs

Soutien à l'association Debra France
HOVNANIAN Alain, Toulouse, France
CRISPR/CAS9–based editing to treat recessive dystrophic epidermolysis bullosa
FUENTES Ignacia, Santiago, Chili
Topical and systemic MSC-based therapies for wound healing in epidermolysis bullosa
Soutien au Fond de Dotation IFCAH
BABOT Ruiz, Dresden, Allemagne
Generation of human steroidproducing organoids: a new approach towards a treatment for CAH
SCHEDL Andreas, Nice, France
Differentiation of stem cells into adrenal organoids
Soutien à l'association Retina France
DELETTRE Cécile, Montpellier, France
Emerging treatments for optic neuropathies
ZEITZ Christina, Paris, France
How to circumvent the challenge to efficiently target the outer plexiform layer of the retina to efficiently rescue the congenital stationary night blindness phenotype
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
Correction de mutation non-sens in vivo dans le gène CFTR par des composés récemment identifiés

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
STROCHLIC Laure, Paris, France
MuSK frizzled-like domain at the NMJ: novel insight into pathophysiology and therapeutics for myasthenia
STURTZ Franck, Limoges, France
Development of a nanoparticle curcumin-based treatment for patients with peripheral neuropathies
VASSETZKY Yegor, Villejuif, France
CTCF-dCas9 fusion protein targeting the 4q35 insulator for re-establishment of the epigenetic state and FSHD treatment

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
BOMONT Pascale, Lyon, France
Therapy for Giant Axonal Neuropathy
CORRAL-DEBRINSKI Marisol, Paris, France
Neuroglobin gene overexpression: a promising tool for treating a large spectrum of neurological disorders thanks to its ability to safely and sustainably preserve mitochondrial integrity
LAPORTE Jocelyn, Illkirch, France
Pathophysiology and therapeutic proof-of-concepts for congenital myopathies
VAILLEND Cyrille, Orsay, France
Decoding central defects in dystrophinopaties: from diagnostic to remedation

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

Structures stratégiques

FONDATION MALADIES RARES, Paris, France
Subvention 2020
GENOPOLE, Evry, France
Participation financière de l'AFM au budget 2020 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)
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

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

BARIS Olivier, Angers, France
11ème colloque du Réseau MeetOchondrie
BRACK Andrew, San Francisco, Etats-Unis
Skeletal Muscle Satellite Cells and Regeneration
JAMAR Gaëlle, Paris, France
Annual Congress of the SFTCG
KINOSHITA June, Lexington, Etats-Unis
FSHD Society 27th Annual International Research Congress

Plateforme non stratégique

CARRE Monique, Mézilles, France
Financement CEDS