LISTE DES PROJETS ET AIDES AUX JEUNES CHERCHEURS FINANCES PAR L'AFM-TELETHON EN 2016

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'AO

Commission : Myologie fondamentale

Aides aux jeunes chercheurs post-doctorants

AJURIA ASTOBIZA Leiore, Lyon, France
Deciphering Lamin C regulatory networks during muscle development and autophagy
CHEVALIER Fabien, Ottawa, Canada
Elucidation of Wnt7a mechanism of action for muscle regeneration
ROBERT-PAGANIN Julien, Paris, France
Insights for novel treatment of HCM: structural studies of beta cardiac myosin to understand the impact of HCM mutations and the mechanism of modulators of force generation

Financements de projets Tremplins

GILSON Eric, Nice, France
Role of TRF2, a key telomere complex, in skeletal
RAFFAELLO Anna, Padoue, Italie
Regulation of skeletal muscle mass by a novel muscle-specific alternative splicing isoform of the mitochondrial calcium uniporter activator MICU1

Financements de projets

AMTHOR Helge, Montigny-Le Bretonneux, France
Bone morphogenetic protein-signaling regulates prenatal limb muscle development
BUSCHBECK Marcus, Barcelone, Espagne
How does the histone variant macroH2A regulate muscle metabolism in health and disease?
CHAZAUD Bénédicte, Villeurbanne, France
Macrophage-derived RNAseT2 stimulates myogenic cell fusion and myofiber formation
DE SANTA BARBARA Pascal, Montpellier, France
Function of double-strain RNA-Binding Protein and Collagen during digestive smooth muscle cell plasticity
FASANO Laurent, Marseille, France
Understanding the molecular underpinnings myogenic programs: skeletal versus smooth
FRIEDEN Maud, Genève, Suisse
Triggering human myoblast differentiation: from EGFR to myogenic transcription factors
GILLET Germain, Lyon, France
Role of bcl-2 family proteins on calcium trafficking and cytoskeletal dynamics
HOUDUSSE Anne, Paris, France
Hypertrophic Cardiomyopathy Caused by Myosin Mutations
MAMMUCARI Cristina, Padoue, Italie
Modulation of mitochondrial calcium signaling to combat skeletal muscle atrophy
MASSE Karine, Bordeaux, France
Roles of eya4 protein during somitogenesis
MESSINA Graziella, Milan, Italie
Study of the multiple functions of Nfix in Muscular Dystrophies: a focus on macrophage biology
MUNOZ-CANOVES Pura, Barcelone, Espagne
Understanding muscle stem cell functional decline with aging

PEREZ RUIZ Ana, Pampelune, Espagne
Role of proteases in muscular homeostasis and aging
TAJBAKHSH Shahragim, Paris, France
Developmental origins and genetic regulation of esophagus skeletal muscles
VANDEL Laurence, Toulouse, France
CREB Binding Protein (CBP) co-activator: a new player for mitochondrial (dys)function in myogenesis?

Commission : Bases Moléculaires et Physiopathologie des Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

HYZEWICZ Janek, Paris, France
Impact of protein modifications on their interactions and signaling pathways in muscular dystrophies
KEMALADEWI Dwi, Toronto, Canada
Elucidating the role of polyamine in laminin-deficient congenital muscular dystrophy
MOREAU Adrien, Lyon, France
Development and characterization of a cellular model of a laminopathy based on the cardiac differentiation of patient specific human induced pluripotent stem cells

Financements de projets

BALSE Elise, Paris, France
CASK, a new SODium channel partner belonging to the DYstrophin complex: role in heart and skeletal muscle excitability during dystrophinopathies
BODEGA Béatrice, Milan, Italie
Epigenetic role for DNA repeats and ncRNAs in FSHD manifestation
BOZZONI Irene, Rome, Italie
Role of long non coding RNAs in muscle differentiation and in Duchenne Muscular Dystrophy (DMD)
BRANCACCIO Andrea, Milan, Italie
Establishing new models for primary dystroglycanopathies
BRAND Thomas, Harefield, Royaume Uni
The genetic interaction of LMNA and POPDC1. Studies of mouse mutants and EDMD and LGMD-1b patients to evaluate the role of POPDC genes as genetic modifiers.
CHARLET-BERGUERAND Nicolas, Illkirch, France
Novel animal models of Myotonic Dystrophies
COSSEE Mireille, Montpellier, France
High throughput sequencing in patients with myopathy and muscular dystrophy: phenotype-genotype correlations studies, functional analyses of TTN variants, search for novel genes.
D'ANTONA Giuseppe, Pavie, Italie
Study of the role of muscle fatigue as predictor of muscle degeneration in facioscapulohumeral muscular dystrophy
DURBEEJ-HJALT Madeleine, Lund, Suède
Characterization of metabolic alterations in MDC1A and evaluation of potential therapies to improve muscle structure and function
JASMIN Bernard, Ottawa, Canada
Translational Regulation of Utrophin A as a Novel Therapeutic Strategy for DMD
LACAMPAGNE Alain, Montpellier, France
Cardiac dysfunction in Duchenne Muscle Dystrophy children: pathophysiological role of type 2 ryanodine receptor
LE RUMEUR Elisabeth, Rennes, France
Structure and interactions of dystrophin and their modifications in Becker muscular dystrophy
MARTELLI Fabio, San Donato Milanese, Italie
microRNA function and use as biomarkers in Myotonic Dystrophy type 1
MILLER Jeffrey, Boston, Etats-Unis
Pathogenesis of congenital muscular dystrophy Type 1A (laminin-alpha-2-deficiency)
MOREL Véronique, Lyon, France
Deciphering neuromuscular junction glutamate receptor density control by Msp300/Nesprin1 in drosophila

PLAISIER Emmanuelle, Paris, France
Pathogenic role of basement membrane defects and endoplasmic reticulum stress in the myopathy related to COL4A1 HANAC mutations
RAVEL-CHAPUIS Aymeric, Ottawa, Canada
Role of Calcium Handling Proteins and Calcineurin Signaling in Myotonic Dystrophy type 1
RUCCI Nadia, L'Aquila, Italie
Bone phenotype in Duchenne muscular dystrophy: unveiling the role of LCN2 and implications for therapy
SANDRI Marco, Padoue, Italie
Dissecting the retrograde signaling that controls neuromuscular junction
THORSTEINSDOTTIR Solveig, Lisbonne, Portugal
Understanding the developmental onset of muscular dystrophy in a mouse model of MDC1A
TUFFERY-GIRAUD Sylvie, Montpellier, France
Defining the trans-acting factors that regulate normal DMD pre-messenger RNA splicing: a combination of RNA interference and targeted RNA-seq approaches.
VAILLEND Cyrille, Orsay, France
Neurobiology of intellectual diabilities in Duchenne muscular dystrophy: Characterization of the glial dysfunctions due to brain Dp71 loss in mice
ZAMMIT Peter, Londres, Royaume-Uni
Dynamic Mapping of Perturbed Signalling Underlying FSHD
ZAMMIT Peter, Londres, Royaume-Uni
The Ephrins as mediators of DUX4 toxicity in FSHD
ZINN-JUSTIN Sophie, Gif-Sur-Yvette, France
Emerin self-assembly and lamin/BAF binding: impact of emerin missense mutations causing Emery-Dreifuss muscular dystrophy versus isolated cardiac defects

Commission : Bases moléculaires et physiopathologie des autres maladies neuromusculaires

Aides aux jeunes chercheurs post-doctorants

SAVARESE Marco, Naples, Italie
Functional validation of rare TTN missense variants
VIAUD Julien, Toulouse, France
Role of PtdIns5P and MTM1/Tiam1 interaction in X-linked recessive centronuclear/myotubular myopathy
YALGIN Cagri, Tampere, Finlande
Mechanisms by which alternative oxidase ameliorates locomotor deficit due to cytochrome c oxidase deficiency in Drosophila

Financements de projets Tremplins

MOUILLET-RICHARD Sophie, Paris, France
Prion protein dysfunction in inherited metabolic myopathies
STEFFANN Julie, Paris, France
Does nuclear transfer alter mitochondrial-nuclear cross-talks in the human preimplantation embryo?

Financements de projets

AUTHIER François Jérôme, Créteil, France
Myofiber expression of HLA-DR in dysimmune myopathies
BENDAHHOU Said, Nice, France
Pathomechanisms in Andersen's syndrome: insights into excitable and non excitable tissues
BOYER Olivier, Rouen, France
Oxidative stress: the main pathogenic process in myositis?
DE LONLAY Pascale, Paris, France
Investigating the connections between inflammation, cell metabolism and calcium regulation in metabolic rhabdomyolysis to design new therapeutic approaches

DUFOUR Eric, Tampere, Finlande
Altering complex I response to OXPHOS dysfunctions; a new tool to combat mitochondrial diseases
DURIEUX Anne-Cécile, Saint Priest En Jarez, France
Deepen the pathophysiological mechanisms of the Dynamin 2-associated centronuclear myopathy and combat muscle dysfunction by targeting myostatin
FERREIRO Ana, Paris, France
SEPN1-related myopathy: mitochondrial dysfunction as a novel pathophysiological mechanism and therapeutic target
JACQUEMOND Vincent, Villeurbanne, France
Phosphoinositides and Ca2+ signaling in normal and diseased skeletal muscle
LETELLIER Thierry, Toulouse, France
Influence of mtDNA background (haplogroups) on mitochondrial pathologies.
MERICSKAY Mathias, Châtenay Malabry, France
Role of Actin-SRF-MRTFA Axis in the development of dilated cardiomyopathy
METODIEV Metodi, Paris, France
Abnormal mitoribosomal biogenesis and protein maturation in human mitochondrial diseases.
MITRANI-ROSENBAUM Stella, Jerusalem, Israël
Characterization of the microRNA profile of GNE Myopathy
NICOLE Sophie, Paris, France
Pathophysiology of congenital muscle weakness linked to abnormal neuromuscular excitability
PRUIJN Ger, Nijmegen, Pays-Bas
Viral infections and the autoimmune response in inclusion body myositis
RAVENSCROFT Gianina, Nedlands, Australie
Neuromuscular disease presenting in utero - gene discovery and pathobiology
STENZEL Werner, Berlin, Allemagne
Roles of hypoxia and innate immunity in juvenile and adult dermatomyositis
TARESTE David, Paris, France
Unraveling the mode of action of Mitofusins in healthy and pathogenic mitochondrial fusion
WALLGREN-PETTERSSON Carina, Helsinki, Finlande
Nemaline myopathy and related disorders: diagnostic methods, disease-gene identification, pathogenesis and genotype-phenotype correlations

Commission : Système nerveux : Motoneurone et jonction neuro-musculaire

Aides aux jeunes chercheurs post-doctorants

CANO Raquel, Séville, Espagne
Monitoring synaptic function in the neuromuscular junction of a SMA mouse model which express SypHy-pHluorin
CIURA Sorana, Paris, France
Physiological analysis of C9orf72 depletion, a major genetic cause in ALS using zebrafish models
D'ALESSANDRO Manuela, Villeurbanne, France
Regulation of muscle acetylcholine receptor expression by novel genes identified in C. elegans genetic screens
DELESTREE Nicolas, New York, Etats-Unis
Alteration of the neuromodulatory pathways and their relationship with motor neuron hyperexcitability in Spinal Muscular Atrophy

Financements de projets Tremplins

BOMONT Pascale, Montpellier, France
Neurofilaments in Health and Charcot-Marie-Tooth diseases

MONTEIL Arnaud, Montpellier, France
Modeling infantile neuroaxonal dystrophy, a nalcn channel-related disorder, in zebrafish
MOURIER Arnaud, Bordeaux, France
Metabolic defects caused by Mitofusin 2 loss of function open up new therapeutic strategies for the Charcot-Marie-Tooth type 2A disease

ZAGLIA Tania, Padoue, Italie
Functional assessment of skeletal muscle beta2-adrenoceptor modulation by the catecholaminergic component of motor nerves, explored using in vivo optogenetics and multiphoton imaging

Financements de projets

BESSEREAU Jean-Louis, Villeurbanne, France
Genetic control of acetylcholine receptor expression: from C. elegans to mammals
CHARLET-BERGUERAND Nicolas, Illkirch, France
Role of C9ORF72 in amyotrophic lateral sclerosis & frontotemporal dementia
CHRAST Roman, Stockholm, Suède
The role of axonal metabolic changes in the pathophysiology of Charcot-Marie-Tooth disease
CHRISTADOSS Premkumar, League City, Etats-Unis
MuSK specific IgG4 B cells as biomarker for MuSK myasthenia gravis
DEVAL Emmanuel, Valbonne, France
Molecular mechanisms of chronic muscle pain - Focus on Acid-Sensing Ion Channels (ASICs)
DUPUIS Luc, Strasbourg, France
Motor neuron loss triggered by defective nuclear import of RNA-binding proteins: mechanistic studies using FUS as a prototypical example
GALINDO Máximo Ibo, Valence, Espagne
Metabolic and functional characterization of clinically relevant Charcot-Marie-Tooth genotypes in a Drosophila model
GREENSMITH Linda, Londres, Royaume-Uni
Targeting molecular pathways of disease in SBMA
HECKMANN Jeannine, Cape Town, Afrique Du Sud
Investigating the Molecular Genetic Basis of treatment-resistant ophthalmoplegia in a subset of Myasthenia Gravis Patients of African-genetic Ancestry
KABASHI Edor, Paris, France
Development of zebrafish models for C9orf72, the major genetic cause in Amyotrophic Lateral Sclerosis and related neurological diseases
LEGAY Claire, Paris, France
Post-transcriptional control of Acetylcholine receptor mRNA; implications for neuromuscular diseases
LEGENDRE Pascal, Paris, France
Microglia instruct fate of embryonic neurons at the onset of developmental cell death in the embryonic spinal cord in vivo
MANUEL Marin, Paris, France
Is hyperexcitability protective or harmful in ALS?
MILLECAMPS Stéphanie, Paris, France
Genetics of Young Amyotrophic Lateral Sclerosis
MONANI Umrao, New York, Etats-Unis
Molecular mediators of the spinal muscular atrophy NMJ phenotype
NOBREGA Clevio, Coimbra, Portugal
Ataxin-2 as a new molecular target in Machado-Joseph disease: from translation regulation to disease alleviation
PEREIRA DE ALMEIDA Luis, Coimbra, Portugal
Activating autophagy to block Machado-Joseph disease progression
RIVAL Thomas, Marseille, France
Using drosophila as a model system to dissect the mechanisms by which mitochondrial fusion defect triggers neuronal dysfunctions in Charcot-Marie-Tooth type 2A disease
SCAMPS Frédérique, Montpellier, France
Role of the Ca2+ activated Cl- channel, Anoctamin 6, in motoneuron pathophysiology
SORARU Gianni, Padoue, Italie
Skeletal muscle: target tissue to cure Spinal and Bulbar Muscular Atrophy (SBMA)
SORET Johann, Montpellier, France
Genome-wide identification of SMN-interacting mRNAs showing axonal localization defects in SMA
STROCHLIC Laure, Paris, France
MuSK frizzled-like domain at the neuromuscular junction: novel insight into pathophysiology and therapeutics for myasthenia

Commission : Cellules souches

Aides aux jeunes chercheurs post-doctorants

FRANCOIS Stéphanie, Milan, Italie
Unravelling differences between Satellite Cells and Mesoangioblasts: modulation of the Rho/ROCK pathway in the activation of Satellite Cells migration.
GIROUSSE Amandine, Toulouse, France
White adipose tissue: a stem/stromal cell provider involved in muscle regeneration?
STUELSATZ Pascal, Seattle, Etats-Unis
EOM satellite cells: high performance myo-engines for muscular dystrophy therapy

Financements de projets Tremplins

MORIN Xavier, Paris, France
Dynamic distribution of Mib1 during mitosis of motor neuron progenitors derived from mouse ES cells
NOTARNICOLA Cécile, Montpellier, France
Human reserve cells: a dynamic tool to discover genes involved in muscle stem cell quiescence
PLUN-FAVREAU Hélène, Londres, Royaume-Uni
Understanding the mechanism underpinning segregation of mitochondrial DNA mutation during somatic cell reprogramming
THORREZ Lieven, Kortrijk, Belgique
Tissue engineering of human muscle: proof of principle for using iPSC-derived myogenic precursor cells and a prevascularization strategy

Financements de projets

BIRCHMEIER Carmen, Berlin, Allemagne
Hes1 and quiescence in satellite cells
BOHL Delphine, Paris, France
Characterization of pathological defects in motor neurons derived from patients with Amyotrophic Lateral Sclerosis
BOIS Patrick, Poitiers, France
Control and modulation of myogenic differentiation of stem cells by using optogenetic approaches
CHAHINE Mohamed, Québec, Canada
Cardiac involvement in myotonic dystrophy type 1 disease: development of improved diagnoses and therapeutics
COMI Giacomo Pietro, Milan, Italie
Optimized transplantation of hiPSC derived LEX+CXCR4+VLA4+ neural stem cells as a therapy for SMARD1
FERNANDEZ Anne, Montpellier, France
Skeletal Muscle as a reservoir of multipotent adult stem cells.
HUGNOT Jean-Philippe, Montpellier, France
The niche of stem cells in the adult spinal cord: cellular diversity and molecular mechanisms underlying dormancy and activation
IMBRIANO Carol, Modène, Italie
NF-YA as a molecular switch with therapeutic potential in muscle regeneration
JAGLA Krzysztof, Clermont-Ferrand, France
A systems level cell-type specific approach to assess diversification of cell fates
KELLY Robert, Marseille, France
Investigation of the emergence of craniofacial muscle progenitor cells in pharyngeal mesoderm
KOISTINAHO Jari, Kuopio, Finlande
Human Model of Neuromuscular Junctions in the Research of Amyotrophic Lateral Sclerosis
MAIRE Pascal, Paris, France
Myogenic fate of satellite cells
MOUNIER Rémi, Villeurbanne, France
AMPKa1 as a regulator of adult muscle stem cell fate choice
PARLAKIAN Ara, Paris, France
Bcl11b/CTIP2, a newly identified transcriptional repressor: its role in cardiac hypertrophy and commitment of cardiac stem cells.

Commission : Thérapie Génique et/ou Cellulaire des Maladies Rares

Aides aux jeunes chercheurs post-doctorants

CHAPPERT Pascal, Paris, France
Cross-tolerance approaches for AAV-mediated muscle gene transfer
PETIT Lolita, Worcester, Etats-Unis
Prolonging vision in retinitis pigmentosa by AAV-mediated genetic modulation of the insulin/AKT/mTOR pathway
SANCHEZ-DUFFHUES Gonzalo, Leiden, Pays-Bas
Putting the brakes on fop: development of novel strategies to block heterotopic ossification
VACCA Ophelie, Orsay, France
AAV-Mediated Dystrophin-Dp71 Gene Therapy In The Central Nervous System

Financements de projets Tremplins

EVERS Melvin, Leiden, Pays-Bas
Targeting ataxin-1 in spinocerebellar ataxia type 1 (SCA1) patient-specific induced pluripotent stem cell-derived neurons
MENDONÇA Liliana, Coimbra, Portugal
Does the transplantation of mutant ataxin-3-depleted patient-derived NSC alleviates Machado Joseph disease (MJD)?

Financements de projets

ADRIOUCH Sahil, Rouen, France
Induction of immunological tolerance following AAV-mediated muscle gene transfer using bi-functional fusion proteins combining extracellular domains of CTLA-4 and PD-L1
BARON VAN EVERCOOREN Anne, Paris, France
Induced pluripotent stem cells to treat pelizaeus merzbacher disease
BELENGUER Pascale, Toulouse, France
Proof of principle of an original therapeutic strategy for a rare disease, dominant optic atrophy
BOSCH Fatima, Bellaterra, Espagne
AAV-mediated gene therapy for the treatment of MPSIIID (Sanfilippo D)
DAVOUST Jean, Paris, France
Gene transfer tolerance in combined liver and muscle rAAV gene therapy
DEGLON Nicole, Lausanne, Suisse
In vitro and in vivo gene editing using viral delivered CRISPR system for Huntington's disease
GRAY Steven, Chapel Hill, Etats-Unis
Aspartylglucosaminuria gene therapy using AAV vectors to target the CNS
KLEOPA Kleopas, Nicosia, Chypre
A gene therapy approach for treating CMT4C
LOPEZ Bernard, Villejuif, France
Stimulating homologous recombination for gene correction of monogenic diseases
NAVARRO Xavier, Barcelone, Espagne
Gene therapy targeting neuregulins for the treatment of amyotrophic lateral sclerosis
NOWAK Kristen, Perth, Australie
ACTA1 congenital myopathies: evaluating viral and gene therapy
PAGANI Franco, Trieste, Italie
Molecular mechanism and therapeutic activity of modified U1 snRNAs in Spinal Muscular Athrophy
TAYLOR Naomi, Montpellier, France
Intrathymic hematopoietic stem cell transplantation for the correction of severe combined immunodeficiency

Commission : Thérapie Pharmacologique des maladies neuromusculaires et Recherche Translationnelle

Aides aux jeunes chercheurs post-doctorants

DURAND Sebastien, Lyon, France
Characterization of INT6/EIF3E functions during Nonsense Mediated Decay (NMD) and development of new NMD inhibitors

SZTAL Tamar, Melbourne, Australie
Investigation of Therapies for Nemaline Myopathy

Financements de projets Tremplins

CAMARA Yolanda, Barcelone, Espagne
Novel therapeutic perspectives for mitochondrial DNA depletion and deletion syndrome due to defective dNTP homeostasis: The specific case of TK2 deficiency

GINESTE Charlotte, Marseille, France
In vivo investigation of skeletal muscle function after chronic administration of CsA in a mouse model of MItochondrial MYopathy: a combined anatomical, metabolic and functional study using MRI and 31P-MRS

SPICHTY Martin, Lyon, France
Selective inhibition of heat-shock binding protein 1: A proof of concept towards a novel strategy to enhance stress response in pathological motor neurons.

SZELECHOWSKI Marion, Bordeaux, France
Evaluation of the therapeutic potential of the Bornavirus X protein and X-derived peptides in amyotrophic lateral sclerosis (ALS)

Financements de projets

AARTSMA-RUS Annemieke, Leiden, Pays-Bas
Cross-sectional study to assess detailed natural disease history of limb girdle muscular dystrophy mouse models

BOITARD Christian, Paris, France
A new model of myositis in Icos-/- and Icosl-/- NOD mice: from biomarkers to pathogenesis

D'ANTONIO Maurizio, Milan, Italie
Targeting Schwann cell proteostasis as a therapeutic strategy in Charcot-Marie-Tooth disease

DESAPHY Jean-François, Bari, Italie
Riluzole, lubeluzole and benzothiazolamine derivatives as new potent antimyotonic drugs

GIRARD Christian, Paris, France
New nonsense-mediated mRNA decay (NMD) inhibitor molecules

MATECKI Stefan, Montpellier, France
Dystrophin-deficient diaphragm dysfunction induced by mechanical ventilation in mdx mice.

NAMY Olivier, Orsay, France
Translational suppression of nonsense mutations found in DMD

PHYLACTOU Leonidas, Nicosia, Chypre
Developing miRNA serum-based biomarkers for Myotonic Dystrophy type 1

PREVITALI Stefano Carlo, Milan, Italie
Modulation of Jab1/p27 levels to rescue peripheral neuropathy and muscular dystrophy in Congenital Muscular Dystrophy type 1A.

ROSSI Daniela Maria, Pavia Italie
Assessing the in vivo efficacy of peptide therapeutics towards the progression of spinal muscular atrophy

T HOEN Peter, Leiden, Pays-Bas
Validation of serum biomarkers to monitor disease progression and response to therapy in DMD and other Muscular Dystrophies

ZAMMIT Peter, Londres, Royaume-Uni
Modelling FSHD as a tool for testing therapeutics

Commission : Médicale

Aides aux jeunes chercheurs post-doctorants

GARGUILO Marine, Garches, France
Impact of pre-phonation inspiratory volume on speech quality of neuromuscular patients requiring non invasive ventilation

Financements de projets

COSTEDOAT-CHALUMEAU Nathalie, Paris, France
Muscular diseases and pregnancy
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
WAHBI Karim, Paris, France
Venous thromboembolism in myotonic dystrophy type 1

Appel d'Offres Doctorants

ANGELIM Monara, Paris, France
Microglia regulate fate of embryonic sensory neurons at the onset of dorsal root ganglia formation in the embryonic spinal cord in vivo
BABSKI Hélène, Montpellier, France
Getting wired up for locomotion: the premotor interneuron/motoneuron connection in Drosophila
BERTIN Benjamin, Clermont Ferrand, France
Molecular and functional analyses of muscle diversification processes by cell type specific genomic approaches in Drosophila
CROISSANT Coralie, Pessac, France
Role of annexins in membrane repair of human skeletal muscle
DAHER Marie-Thérèse, Paris, France
Bcl11b/CTIP2, a newly identified transcriptional repressor: its role in cardiac hypertrophy and commitment of cardiac stem cells.
DE BONO Christopher, Marseille, France
Dissecting the mechanisms by which distinct muscle cell types arise in posterior cardiopharyngeal mesoderm.
DEWULF Melissa, Paris, France
Functional analysis of Cav3 mutations in muscular dystrophy diseases
DINCA Diana Mihaela, Paris, France
Pathophysiological impact of the CTG repeat expansion on neuronal and astroglial biology in DM1 brains
DIOUF Sarah, Toulouse, France
Decryption of the roles of CBP methylation in human primary myoblast differentiation: cross-talk between nuclear and mitochondrial genomes.
DOS SANTOS Matthieu, Paris, France
Genetic control of adult muscle fiber type
EL FISSI Najla, Marseille, France
Using drosophila as a model system to investigate how altered mitochondrial fusion triggers mitochondrial damages and neuromuscular disorders
GAZALAH Hussein, Fanar, Liban
Enigmatic perineuronal cells in the mouse and human spinal cord: properties, isolation and function
GRIMALDI Alexandre, Los Angeles, Etats-Unis
Regulation and morphogenesis of cranial muscle derivatives

GUERIN Amandine, Montpellier, France
Function of double-strain RNA-Binding Protein LIX1 and its interacting protein partner during digestive smooth muscle cell plasticity.

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

KHABOU Hanen, Paris, France
Retinitis Pigmentosa: gene therapies to prevent and restore vision loss

KUTCHUKIAN Candice, Villeurbanne, France
Phosphoinositides and Ca2+ signaling in normal and diseased skeletal muscle

LAVERGNE Guillaume, Clermont-Ferrand, France
Studying homing behaviour of Drosophila Adult Muscle Precursor (AMP) cells using genome wide cell specific approaches

MARSAC Roxane, Bordeaux, France
Adenylosuccinate Lyase deficiency - from metabolic deficiency to muscle disease using C. elegans as model organism

MATONTI Julia, Nice, France
Investigating novel functions of the Kir2.1 potassium channels in muscle and during bone morphogenesis.

MIAS-LUCQUIN Dominique, Rennes, France
Dynamics and mechanics of the myopathy-related protein dystrophin in macromolecular complexes with filamentous partners

MOUSSAOUI Nadir, Saint-Priest En Jarez, France
Targeting mitochondria to improve muscle function in a mouse model of autosomal dominant centronuclear myopathy.

NASSARI Sonya, Paris, France
Involvement of CXCL12 and CXCL14 chemokines in connective tissue-mediated limb muscle morphogenesis.

RAESS Matthieu, Strasbourg, France
Deciphering the functional and molecular differences between MTM1 and MTMR2 to understand two neuromuscular diseases.

SAMSON Camille, Gif-Sur-Yvette, France
Structural analysis of the emerin-lamin complex mutated in Emery-Dreifuss muscular dystrophy

SCIONTI Isabella, Lyon, France
Epigenetics in muscle lipid metabolism

SLEIMAN Yvonne, Montpellier, France
Cardiac dysfunction in Duchenne Muscle Dystrophy children: pathophysiological role of type 2 ryanodine receptor through hiPSC-derived cardiomyocytes

SUTCU Haser, Paris, France
Implication of DNA Damage and Repair in Viability and Differentiation of Muscle Stem Cells

TERRONE Sophie, Lyon, France
Interplay between epigenetic marks and alternative splicing during myogenesis

TEYSSIER Perrine, La Tronche, France
Mechanisms of triad formation

TEYSSOU Elisa, Paris, France
In vitro and in vivo functional analysis of 2 genes identified in familial amyotrophic lateral sclerosis

WATTIN Marion, Villeurbanne, France
Comparative study of proteostasis during muscle degeneration in models for muscular dystrophies

WURMSER Maud, Paris, France
SIX homeoproteins and muscle stem cells properties

Appel d'Offres « Projets de recherche translationnelle sur l'ARN en tant que médicament et cible thérapeutique »

D'AMATI Giulia, Rome, Italie
Stabilization of tRNAs as a therapeutic strategy for diseases due to mutations in mt-tRNAs.
FRANCO Brunella, Pozzuoli (Naples), 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
NAMY Olivier, Orsay, France
Translectin: A new termination codon readthrough inducer to expand therapeutics choices for nonsense genetic diseases
VAN ROON-MOM Willeke, Leiden, Pays-Bas
Final proof of concept for the advancement of antisense oligonucleotide treatment for SCA3 towards the clinic

Partenariats institutionnels

GACHE Vincent, Lyon, France
Soutien du projet de recherche du candidat ATIP-Avenir 2016: Interplay between cytoskeleton network regulation during muscle development and muscle function

Partenariats associatifs

SMA EUROPE
ALLAIN Frédéric, Zurich, Suisse
Seeking small molecules that stabilize protein-RNA interactions to cure Spinal Muscular Atrophy
GARCIA-LOPEZ Amparo, Post Doctorant, Genève, Suisse
Using RNA secondary structure as a therapeutic target for Spinal Muscular Atrophy: a new drug discovery approach
Fondation pour l'Aide à la Recherche sur la Sclérose en Plaques (ARSEP), Call for proposal 2016 "Myelin: from lesion to reparation"
STANKOFF Bruno, Paris, France
Remyelination at the early stage of Multiple Sclerosis: a PET-MR longitudinal study investigating individual profiles of myelin repair and the contribution of neuroinflammation
CLEMENTE LOPEZ Diego, Toledo, Espagne
Myeloid-derived suppressor cells and disease aggressiveness: a novel cell therapy to accelerate myelin repair in multiple sclerosis
IFCAH, Paris, France
GUASTI Leonardo, Londres, Angleterre
Study of CAH adrenocortical cells reprogrammed from urine: a step towards a novel cell therapy
Vaincre les Maladies Lysosommales (VML), Massy, France
ROUVIERE Laura, Post Doctorant, Paris, France
Gene transfer in the murine model of Sandhoff disease using a specific AAV9 vector
Vaincre la Mucoviscidose (VLM),
CADARS-TAULAN Magali, Montpellier, France
Détermination des éléments cis- et trans-régulateurs dans le contrôle de l'expression du gène CFTR en contexte physiologique et pathologique
MIDOUX Patrick, Orléans, France
Transfert de gènes par des vecteurs synthétiques : Construction d'un plasmide CFTR optimisé pour son transport dans le noyau des cellules épithéliales pulmonaires CF
MONTIER Tristan, Brest, France
Développement et optimisation de formulations lipidiques originales pour la transfection pulmonaire par aérosolisation sur modèles murins cf-/-
HONG BOULANGER Saw See, Lyon, France
Vésicules extracellulaires (microvésicules et exosomes) comme vecteurs du CFTR: mode de délivrance, biodistribution et ciblage tissulaire in vivo

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

GOMES-PEREIRA Mario, Paris, France
DM1 disease mechanisms in the central nervous system: from brain cell-specific pathogenesis to misregulated glutamate homeostasis
GOURDON Geneviève, Paris, France
Mechanisms of CTG repeat contractions in unusual DM1 families
GOURDON Geneviève, Paris, France
CDM features and mechanisms in the DMSXL mouse model for DM1
MARCELLE Christophe, Clayton, Australie
Muscle fusion as a delivery mechanism to repair ailing muscles from heritable muscle diseases
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
SERGEANT Nicolas, Paris, France
Modified MBNL constructs for a gene therapy targeting nuclear expanded-CUG repeats to counteract toxic RNA gain-of-function in Myotonic Dystrophy
ZEITZ Christina, Paris, France
Développement d'une thérapie génique pour restaurer la fonction de GRM6/mGluR6 dans la cécité nocturne congénitale stationnaire

ACTIONS STRATEGIQUES

Projets stratégiques

GUEDAT Philippe, Nantes, France
Pre-clinical and clinical development of IFB-088, a new chemical entity to treat Charcot-Marie-Tooth disease
LAPORTE Jocelyn, Illkirch, France
Genetic bases, pathomechanisms and preclinical developments in congenital myopathies
MUNTONI Francesco , Londres, Royaume Uni
Advances in oligonucleotide-mediated exonskipping for DMD and related disorders - WP3 - Natural history extension
PORTE-THOME Florence, Plan les Ouates, Suisse
Translational studies to evaluate the efficacy of Rimeporide, a NHE-1 inhibitor, in patients with Duchenne Muscular Dystrophy
ROTIG Agnès, Paris, France
An integrated approach for MITOchondrial disorder THERApeutics from yeasts and worms to humans
SIMONELIG Martine, Montpellier, France
Towards a clinical trial for OPMD

Plateformes stratégiques

GOTTRAND Frédéric, Lille, France
Financement CIC Lille

Pôles stratégiques

LEVY Nicolas, Marseille, France
Translational Research in Marseille: towards Therapeutic Development for Rare Diseases
RELAIX Frédéric, Créteil, France
TRANSLAMUSCLE: An integrated translational program from basic research to biotherapies in stem cells and molecular medicine of the neuromuscular system
SCHAEFFER Laurent, Lyon, France
Alliance MyoNeurALP - a research network dedicated to neuromuscular disorders

Structures stratégiques

FONDATION MALADIES RARES, Paris, France
Subvention 2016
GENOPOLE, Evry, France
Participation financière de l'AFM au budget 2016 du GIP GENOPOLE

Outils stratégiques

BASSEZ Guillaume, Créteil, France
DM-Scope International French-Quebec Myotonic Dystrophy Registry
DESGUERRE Isabelle, Paris, France
A national clinical (and genetic) database for dystrophinopathies
SACCONI Sabrina, Nice, France
French National Registry for FacioScapuloHumeral muscular Dystrophy (FSHD) (projet 1)
SACCONI Sabrina, Nice, France
French National FSHD patient registry for clinical trial planning and translational research (projet 2)

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

COLLOQUE JEUNES CHERCHEURS, Lyon, France
Colloque Jeunes Chercheurs 2016 - 17 mars 2016 - Congrès Myologie 2016
CORNELISON Dawn D., Columbia – MO, Etats-Unis
FASEB Science Research Conference on Skeletal Muscle Satellite Cells and Regeneration
DUSSAULE Jean-Claude, Paris, France
"Physiology and inherited rare diseases: When physiology meets the genes" and "PHOX2B and Ondine's Curse, a story from gene to disease"
GOIZET Cyril, Bordeaux, France
Organization of the "14èmes Journées de la Société Française de Myologie"
JAMAR Gaëlle, Paris, France
Congrès Annuel de la Société Française de Thérapie Cellulaire et Génique
JAMAR Gaëlle, Paris, France
Annual congress of the European Society for Gene and Cell Therapy, Public Engagement Day
KALATSIS Vasiliki, Montpellier, France
Vision beyond the genome (a conference of the European Union Marie-Curie Initial Training Network, EyeTN)
KINOSHITA June, Lexington, Etats-Unis
FSHD Connect International Research Conference and Patient Conference
LEGAY Claire, Paris, France
XVth International Symposium on Cholinergic Mechanisms
MATECKI Stefan, Montpellier, France
Organisation of the 45th congress of the European Musculat Conference
MUSARO Antonio, Rome, Italie
IIM-Myology meeting: focus on pathogenesis of rare diseases and therapies
TAILLANDIER Daniel, St Genès Campanelle, France
7th Proteasomes & Autophagy Workshop

Plateforme non stratégique

CARRE Monique, Mézilles, France