LISTE DES PROJETS ET AIDES AUX JEUNES CHERCHEURS FINANCES PAR L'AFM-TELETHON EN 2017

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'AO

Commission : Myologie fondamentale

Aides aux jeunes chercheurs post-doctorants

GRAVEZ Basile, New York, Etats-Unis
Systematic whole genome analyses of the cardiopharyngeal gene regulatory network in a simple chordate
HUBERT Fabien, Marseille, France
Evaluation of FGF10 as a target for promoting adult cardiomyocyte proliferation after heart injury
SIEIRO MOSTI Julien, Boston, Etats-Unis
Use of a novel in vitro DMD model to study muscle fusion during health and disease

Financements de projets Tremplins

HNIA Karim, Toulouse, France
MTM1-UBQLN2 involvement in proteostasis networks controlling muscle fiber mass
MOHA OU MAATI Hamid, Montpellier, France
Mechanosensitive ionic channels in zebrafish heart regeneration
MORESI Viviana, Roma, Italie
Uncovering the function of HDAC4 in skeletal muscle

Financements de projets

CIENIEWSKI-BERNARD Caroline, Villeneuve d'Ascq, France
O-GlcNAcylation: an atypical post-translational modification crucial for skeletal muscle ultrastructure and function?
DAUBAS Philippe, Paris, France
Role of LRRFIP2 in the control of heart development and skeletal muscle regeneration
DE SANTA BARBARA Pascal, Montpellier, France
Function of LIX1 and its interacting partner during digestive smooth muscle plasticity
GILLET Germain, Villeubanne, France
Role of bcl-2 family proteins on calcium trafficking and cytoskeletal dynamics
GRAZIANI Maud, Milano, Italie
Acylated and Unacylated Ghrelin, inflammation, and muscle wasting: the unexpected role of novel and old ghrelin receptors
HELMBACHER Françoise, Marseille, France
FAT1 Signaling in skeletal muscle growth and repair: Muscle versus mesenchyme perspectives
MESSINA Graziella, Milano, Italie
Study of the multiple functions of Nfix in Muscular Dystrophies: a focus on macrophage biology
MOLINARI Suzanna, Modena, Italie
Pin1: a new potential target to induce slow fiber conversion in Duchenne Muscular Dystrophy
MUNOZ-CANOVES Pura, Barcelona, Espagne
Understanding muscle stem cell functional decline with aging
PEREZ RUIZ Ana, Pamplona, Espagne
Role of proteases in muscular homeostasis and aging
RAZ Vered, Leiden, Pays-Bas
RNA processing role in muscle degeneration opens therapeutical options for adult myopathies

TAILLANDIER Daniel, Saint Genès Champanelle, France
Role of E2 conjugating enzymes in the MuRF1-dependent targeting of telethonin in skeletal muscle
TAJBAKHSH Shahragim, Paris, France
Developmental origins and genetic regulation of esophagus skeletal muscles

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 signalling pathways in muscular dystrophies
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
OSSENI Alexis, Ottawa, Canada
Targeting of Histone Deacetylase 6 (HDAC6) in Neuromuscular Diseases

Financements de projets Tremplins

NOGALES-GADEA Gisela, Badalona, Espagne
Muscle single-cell analysis in patients with myotonic dystrophy type I
PERROTTA Cristiana, Milano, Italie
Therapeutic relevance of the sphingolipid-metabolizing enzyme acid sphingomyelinase as a new pathogenetic factor in Duchenne muscular dystrophy

Financements de projets

BERGHELLA Libera, Roma, Italie
Molecular mechanisms for neuromuscular junction (NMJ) disruption and reduced mitochondrial function in Duchenne Muscular Dystrophy
BOZZONI Irene, Roma, Italie
Role of long non coding RNAs in muscle differentiation and in Duchenne Muscular Dystrophy (DMD)
BRANCACCIO Andrea, Roma, Italie
Establishing new models for primary dystroglycanopathies
BRAND Thomas, London, 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
BRICHARD Sonia, Bruxelles, Belgique
The beneficial properties of AdipoRon in dystrophinopathies
CIAPPONI Laura, Roma, Italie
Analysis of the DM2 pathogenic mechanisms using Drosophila as model system
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
Deciphering new non-coding RNAs from introns: An atlas of new biomarkers/targets for DM1 patients
LACAMPAGNE Alain, Montpellier, France
Cardiac dysfunction in Duchenne Muscle Dystrophy children: pathophysiological role of type 2 ryanodine receptor
LANZUOLO Chiara, Roma, Italie
Deciphering the role of the heterochromatin conformation in Emery Dreifuss Muscular Dystrophy (EDMD)
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, Padova, Italie
Dissecting the retrograde signaling that controls neuromuscular junction
THORSTEINSDOTTIR Solveig, Lisboa, Portugal
Understanding the developmental onset of muscular dystrophy in a mouse model of MDC1A
VOLK Talila, Rehovot, Israël
Epigenetic analysis of myonuclei defective in nuclear envelope components in Drosophila muscles as a model for studying Emery-Dreifuss muscular dystrophy
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

FERNANDES CERQUEIRA Cátia Soraia, Stockholm, Suède
Histidyl tRNA synthetase – Connecting lungs to muscle inflammation
SAVARESE Marco, Helsinki, Finlande
Functional validation of rare TTN missense variants
SUAREZ CALVET Xavier, Barcelona, Espagne
The pathogenesis of dermatomyositis associated to anti-MDA5 autoantibodies
YALGIN Cagri, Tampere, Finlande
Mechanisms by which alternative oxidase ameliorates locomotor deficit due to cytochrome c oxidase deficiency in Drosophila

Financements de projets

BATONNET-PICHON Sabrina, Paris, France
A new mouse model for desminopathies: physiopathological mechanisms, muscle repair and therapy
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
FERREIRO Ana, Paris, France
ASC-1, a novel actor in the pathophysiology of congenital muscle disorders
MERCIER Sandra, Nantes, France
Unravelling POIKTMP pathophysiology for design of therapeutic approaches
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
Zebrafish as a model system for GNE Myopathy
NICOLE Sophie, Paris, France
Pathophysiology of congenital muscle weakness linked to abnormal neuromuscular excitability
PAQUIS-FLUCKLINGER Véronique, Nice, France
Mitochondrial myopathies with mtDNA instability: the role of cristae maintenance
ROVERE QUERINI Patrizia, Milano, Italie
Role of PTX3 in the pathogenesis of idiopathic inflammatory myopathies
SCHMIDT Jens, Göttingen, Allemagne
Epigenetic modulation of pro-inflammatory cell stress in inclusion body myositis
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

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

BONDURAND Nadège, Paris, France
Role of RNA editing during normal and abnormal Schwann cell development
BOWERMAN Melissa, Oxford, Royaume Uni
The glucocorticoid-KLF15-BCAA pathway as a novel therapeutic target for muscle and metabolic pathologies in SMA
GASCON Eduardo, Marseille, France
Generating novel tools to investigate the in vivo role of miR-124 in motor neurone physiology and ALS pathogenesis
GRANDIS Marina, Genova, Italie
Misglycosylation in Charcot-Marie-Tooth neuropathies associated to MPZ mutations

Financements de projets

BRITES Pedro, Porto, Portugal
Understanding how membrane ether-phospholipids regulate neuron development and function to mediate neuropathophysiology of RCDP
CHRAST Roman, Stockholm, Suède
The role of axonal metabolic changes in the pathophysiology of Charcot-Marie-Tooth disease
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
FORNE Thierry, Montpellier, France
Genome-wide profiling of nuclear-body associated regions: relation to cellular physiopathology of the spinal muscular atrophy disease
GREENSMITH Linda, London, Royaume-Uni
Targeting molecular pathways of disease in SBMA
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
LEGENDRE Pascal, Paris, France
Microglia instruct fate of embryonic neurons at the onset of developmental cell death in the embryonic spinal cord in vivo
LOCHMULLER Hanns, Newcastle-upon-Tyne, Royaume-Uni
Structural and molecular changes in the pre-synapse in disorders of the neuromuscular junction
MARTINEZ-MARTINEZ Pilar, Maastricht, Pays-Bas
Characterizing the autoantibody-producing B-cell in MuSK Myasthenia gravis and cloning the MuSK autoantibody to unravel pathogenic mechanisms of MuSK autoantibodies
MONANI Umrao, New York, Etats-Unis
Molecular mediators of the spinal muscular atrophy NMJ phenotype
PAREYSON Davide, Milano, Italie
Unravelling mechanisms of axonal loss in late-onset genetic neuropathies

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
RUGGIERO Florence, Lyon, France
The extracellular matrix collagen XV as a new player of motor axon development and regeneration: a functional study using zebrafish
SCAMPS Frédérique, Montpellier, France
Role of the Ca2+ activated Cl- channel, Anoctamin 6, in motoneuron pathophysiology
SCHENONE Angelo, Genova, Italie
Upper Limbs evaluation in hereditary NeuropAthies: the ULNA project
SORARU Gianni, Padova, Italie
Skeletal muscle: target tissue to cure Spinal and Bulbar Muscular Atrophy (SBMA)
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

COSTAMAGNA Domiziana, Leuven, Belgique
Smad9 perturbation studies to boost myogenic commitment of stem cells
SALA CANO David, La Jolla, Etats-Unis
STAT3 signaling network in MuSCs as therapeutic target for DMD

Financements de projets Tremplins

ROCHAIS Francesca, Marseille, France
Targets for heart regeneration: mechanisms controlling cardiomyocyte proliferation

Financements de projets

BOIS Patrick, Poitiers, France
Control and modulation of myogenic differentiation of stem cells by using optogenetic approaches
BORYCKI Anne-Gaëlle, Sheffield, Royaume-Uni
Control of satellite cell self-renewal by Sonic hedgehog signalling and primary cilia
CHAHINE Mohamed, Québec, Canada
Cardiac involvement in myotonic dystrophy type 1 disease: development of improved diagnoses and therapeutics
CHRETIEN Fabrice, Paris, France
Mesenchymal stem cells improve the muscle acquired myopathy after sepsis
HUGNOT Jean-Philippe, Montpellier, France
The niche of stem cells in the adult spinal cord: cellular diversity and molecular mechanisms underlying dormancy and activation
KOISTINAHO Jari, Kuopio, Finlande
Human Model of Neuromuscular Junctions in the Research of Amyotrophic Lateral Sclerosis
MUNOZ-CANOVES Pura, Barcelona, Espagne
Novel strategies to rescue stem cell regenerative impairment in DMD
SACCO Alessandra, La Jolla, Etats-Unis
Role of Tenascin-C in muscle stem cell function
SOTIROPOULOS Athanassia, Paris, France
Role of Srf transcription factor in adult muscle stem cells – Identification of downstream effectors
STUDER Michèle, Nice, France
In vivo and in vitro direct lineage reprogramming of neuronal and somatic cells to corticospinal motor neurons

Commission : Thérapie Génique et/ou Cellulaire des Maladies Rares

Financements de projets Tremplins

BASSO Manuela, Trento, Italie
Targeting PRMT6 to attenuate spinal and bulbar muscular atrophy: a silencing approach
BIGGER Brian, Manchester, Royaume Uni
Neonatal stem cell gene therapy for severe Mucopolysaccharidosis type II
MASTROYIANNOPOULOS Nikolaos, Nicosia, Chypre
LNA/2'OMe mixmers against toxic CUG expanded RNA
NOBRE Rui, Manchester, Royaume Uni
Exosomes as gene delivery vectors for the treatment of Machado-Joseph disease / Spinocerebellar Ataxia type 3

Financements de projets

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
BETUING Sandrine, Paris, France
Neuroprotective role of CYP46A1 in Huntington's disease using gene transfer therapy in brain
CHUAH Marinee, Bruxelles, Belgique
NEXTGEN-AAV: Development of next-generation AAV vectors for Duchenne muscular dystrophy
CONCHON Sophie, Nantes, France
Liver Gene Transfer-Induced allogeneic Tolerance for organ transplantation and cell therapy
DAVOUST Jean, Paris, France
Gene transfer tolerance in combined liver and muscle rAAV gene therapy
DELETTRE-CRIBAILLET Cécile, Montpellier, France
Gene therapy for Wolfram Syndrome
DI IORIO Enzo, Padova, Italie
Advanced therapy medicinal products for the treatment of ocular defects in Ectrodactyly-Ectodermal Dysplasia-Clefting (EEC) syndrome
ERCEG Slaven, Valencia, Espagne
CORRET: Cell therapy with genetically corrected retinal pigment epithelium in hereditary retinal dystrophies
GRAY Steven, Chapel Hill, Etats-Unis
Aspartylglucosaminuria gene therapy using AAV vectors to target the CNS
HARBOTTLE Richard, Heidelberg, Allemagne
Using novel non-viral DNA vectors for the genetic correction of defective dioxygenase in alkaptonuric mice
KALATZIS Vasiliki, Montpellier, France
Developing and studying the effects of innovative therapies for rare inherited retinal disease using human retinal models derived from patient iPS cells
KLEOPA Kleopas, Nicosia, Chypre
A gene therapy approach for treating CMT4C
LEGRAND Alexandre, Mons, Belgique
Evaluation of DUX4 silencing tools in mice
MOREAU-GAUDRY François, Bordeaux, France
Functional iPSC-based hematopoietic gene-therapy
NAVARRO Xavier, Barcelona, Espagne
Gene therapy targeting neuregulins for the treatment of amyotrophic lateral sclerosis
VANDENDRIESSCHE Thierry, Bruxelles, Belgique
'Repeat-Repair': CRISPR/Cas9-mediated correction of triplet nucleotide repeats

Commission : Thérapie Pharmacologique des maladies neuromusculaires et Recherche Translationnelle

Aides aux jeunes chercheurs post-doctorants

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

TERRILL Jessica, Perth, Australie
Functional readouts of neutrophil mediated oxidative stress as biomarkers in plasma and urine from the Golden Retriever Muscular Dystrophy dog model for Duchenne Muscular Dystrophy

YUSEIN-MYASHKOVA Shazie, Anvers, Belgique
A chemical genetic screen for candidate drugs rescuing CMT-associated phenotypes in Drosophila

Financements de projets Tremplins

STRAUB Volker, Newcastle-upon-Tyne, Royaume-Uni
Assessing muscle fibrosis by magnetic resonance imaging using a novel contrast agent

TERRILL Jessica, Perth, Australie
Functional readouts of neutrophil mediated oxidative stress as biomarkers in plasma and urine from the Golden Retriever Muscular Dystrophy dog model for Duchenne Muscular Dystrophy

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

BARTOCCIONI Emanuela, Roma, Italie
Effect of Rituximab therapy on T-cell repertoire in MuSK positive Myasthenia Gravis

CHRISTADOSS Premkumar, League City, Etats-Unis
Acetylcholine receptor binding B cells as biomarker for myasthenia gravis

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

DORCHIES Olivier, Genève, Suisse
Enhancing estrogenic signalling to fight devastating muscular dystrophies: Mechanisms of action and repurposing estrogenic drugs approved for Human use

DORCHIES Olivier, Genève, 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

JASMIN Bernard, Ottawa, Canada
Repurposing drugs that target eEF1A2 to increase translation of utrophin in dystrophic muscle

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

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

PARENTI Giancarlo, Napoli, Italie
Strategies to enhance the efficacy of enzyme replacement and gene therapy in Pompe disease

SANDONÀ Dorianna, Padova, Italie
Small molecule-based therapy for sarcoglycanopathies. Assessment of efficacy and tolerability in novel animal models

SPITALI Stefano Carlo, Leiden, Pays-Bas
Identification of blood derived transcriptomic biomarkers for Duchenne muscular dystrophy

TORRENTE Yvan, Milano, Italie
Immune system involvement in DMD pathology: the role of the immunoproteasome and its therapeutic potential
TZARTOS Socrates, Athens, Grèce
Preclinical assessment of an antigen-specific therapeutic approach for MG

Commission : Médicale

Financements de projets

PEPIN Jean-Louis, Grenoble, France
Impact of the type of interface in neuromuscular patients treated with nocturnal Noninvasive Ventilation: a randomized crossover trial

Appel d'Offres Doctorants

ASFOUR Hasan, Montigny-le-Bretonneux, France
Bone morphogenetic proteins regulate patterning of limb muscles
BABSKI Hélène, Montpellier, France
Getting wired up for locomotion: the premotor interneuron/motoneuron connection in Drosophila
BELAL Sophie, Angers, France
Understanding the pathophysiology of MELAS Syndrome
BEURIOT Adeline, Paris, France
Characterization of CASK protein function in the regulation of ion channels in cardiomyocytes and its role in cardiac electrophysiology in vivo
BOGARD Baptiste, Paris, France
Small non-coding RNAs of intron origin in Myotonic Dystrophy type 1: innovative biomarkers and candidate drivers of splicing defects
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
DANIEL Malo, Nantes, France
Characterization of Liver-induced allo-specific CD8 regulatory T lymphocytes
DE ZÉLICOURT Antoine, Orsay, France
Characterization of a new model of mdx mice deficient in the enzyme CD38: towards the protective role on the Ca2+ homeostasis deregulation
DOS SANTOS Matthieu, Paris, France
Genetic control of adult muscle fiber type
DUPATY Léa, Rouen, France
Successive targeting of CTLA-4/B7 and PD-L1/PD-1 immunoregulatory pathways to improve transgene persistence and tolerance in the context of rAAV-mediated muscle gene therapy
FEFEU Mylène, Paris, France
Dysfunction of muscle stem cells in sepsis and regenerative therapy
GAZALAH Hussein, Montpellier, France
Enigmatic perineuronal cells in the mouse and human spinal cord: properties, isolation and function
GRIMALDI Alexandre, Paris, France
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

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

POLYDOROU Ioanna, Versailles, France
Investigation of the crosstalk between the BMP and NOTCH signalling pathways in satellite cells during adult skeletal muscle regeneration

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

Partenariats institutionnels

ENRIQUEZ Jonathan, Paris, France,
ATIP-Avenir 2017 : Making motoneurons unique
GACHE Vincent, Lyon, France
ATIP-Avenir 2016 : Interplay between cytoskeleton network regulation during muscle development and muscle function
NEDELEC Stéphane, Paris, France
ATIP-Avenir 2015 : Stem cell approaches of human motor neuron diversity in development and diseases

Partenariats associatifs

SMA EUROPE
ARTERO Ruben, Burjassot, Espagne,
A Spinal Muscular Atrophy Drosophila model for in vivo drug discovery,
GILLINGWATER Thomas, Edinburgh, Royaume-Uni,
Defining the role of the motor axon translatome in SMA pathogenesis,
Fondation pour l'Aide à la Recherche sur la Sclérose en Plaques (ARSEP),
CALZA Laura, Bologna, Italie
Role of RXRγ in T3-mediated oligodendrocytes differentiation and remyelination
ZALC Bernard, Paris, France
Investigating a microglial receptor tyrosine kinase as a therapeutic target for myelin repair,
Fonds de dotation pour la recherche sur l'Hyperplasie Congénitale des Surrénales (IFCAH),
SCHEDL Andreas, Nice, France,
Adrenal stem cells: identification, generation and culture for genetic modification,
Institut pour la Recherche sur la Moelle Epinière (IRME),
GUEROUT Nicolas, Rouen, France,
Recrutement des cellules souches endogènes médullaires comme biothérapie non invasive innovante médiée par la stimulation magnétique répétitive dans le cadre des lésions de la moelle épinière,
BROCARD Frédéric, Marseille, France,
Thérapie génétique de la spasticité après une lésion de la moelle épinière
GAILLARD Afsaneh, Poitiers, France,
Greffe de neurones corticaux dérivés de cellules souches pluripotentes induites humaines pour le traitement de lésions corticales,
LIU Song, Paris, France,
Reestablishment of primary sensory connections between lower intercostal nerves and lumbar spinal nerves via genetically modified predegenerated nerve grafts in adult rats after hemi-paraplegic,
SOARES Sylvia, Paris, France,
Approche combinatoire pour le trauma de la moelle épinière chez le rat : biomatériaux – Cellules souches,
Association Gêniris
ABERDAM Daniel, Paris, France,
Modeling of human aniridia and gene therapy approaches,
RETINA France,
ORIEUX Gael, Paris, France
Cell therapy for optic neuropathies: analysis of the potential of human iPS cells derived retinal ganglion cells in a rodent model of optic nerve lesion,
ZEITZ Christina, Paris, France,
Towards improvement of gene augmentation therapy for congenital stationary night blindness caused by mutations in GRM6 and LRIT3,
Vaincre la Mucoviscidose (VLM), Paris, France
CADARS Magalie, Montpellier, France,
Détermination des éléments cis- et trans-régulateurs dans le contrôle de l'expression du gène CFTR en contexte physiologique et pathologique
DEVOS John, Montpellier, France,
Modélisation in vitro d'un épithélium bronchique mucoviscidosique par la technologie des cellules souches pluripotentes induites

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

Vaincre les Maladies Lysosomales (VML), Massy, France

CAILLAUD Catherine, Paris, France
Preclinical steps for a gene transfer approach in Sandhoff disease

ORTOLANO Saïda, Vigo, Espagne
Functional evaluation of an AAV9 based vector expressing alpha-Galactosidase A for potential gene therapy of Fabry disease

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

BECANE Henri-Marc, Paris, France
Study of the effect of preventive treatment with Nebivolol on the development and progression of cardiac dysfunction in children with Duchenne

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

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

UDD Bjarne, Helsinki, Finlande
LGMD2D – natural history in R77C mutated patients

VASSETSKY 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

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

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

SIMONELIG Martine, Montpellier, France
Development of IFB-088 for the treatment of OPMD

Plateformes stratégiques

I-MOTION : Création d'un centre de recherche clinique neuromusculaire pédiatrique Parisien

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

GIPTIS - Genetics Institute for Patients, Therapies, Innovation & Science, Marseille, France

GENATLANTIC, Nantes, France

GENOPOLE, Evry, France

Outils stratégiques

BASSEZ Guillaume, Créteil, France
DM-Scope International French-Quebec Myotonic Dystrophy Registry
SACCONI Sabrina, Nice, France
French National Registry for FacioScapuloHumeral muscular Dystrophy (FSHD)

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

BATONNET-PICHON Sabrina, Paris, France
European Meeting of Intermediate Filaments June 14-17, 2017 in Saint Malo
BIRCHMEIER Carmen, Berlin, Allemagne
Muscle Development, Regeneration and Disease 2018
BONNE Gisèle, Paris, France
22nd International Congress of The World Muscle Society
CHAZAUD Bénédicte, Villeurbanne, France
2017 Gordon Research Conference and Gordon Research Seminar Myogenesis: Advanced Mechanisms of Growth and Repair
COLLOQUE JEUNES CHERCHEURS, Paris, France
Colloque Jeunes Chercheurs 2017 - JDF Parc Floral de Paris - 23 juin 2017
DE CHASTONAY Sabine, Torrance, Etats-Unis
2017 CMD Scientific and Family Conference
ECHANIZ-LAGUNA Yon Andoni, Strasbourg, France
Organization of the "15èmes Journées de la Société Française de Myologie"
GABELLINI Davide, Milano, Italie
IIM-Myology Meeting: Pathogenesis and Therapies of Rare Diseases
GISCLARD Valérie, Fourques, France
3ème Congrès International Syndromes d'Ehlers-Danlos
JAMAR Gaëlle, Paris, France
Annual congress of the European Society for Gene and Cell Therapy, Public Engagement Day
LATTANZI Giovanna, Bologna, Italie
International Meeting on Laminopathies
LEBLEU Bernard, Montpellier, France
SF Nano Summer school: Nucleic acids-based strategies to control gene expression: Principles, applications in biology, clinical translation and delivery issues
LOMBES Anne, Paris, France
Annual meeting of MeetOchondrie Network

MARTY Isabelle, La Tronche, France

2017 Gordon Research Conference (GRC) and Gordon Research Seminar (GRS): Muscle - Excitation-contraction coupling- June 3 and 4 (GRS) and June 4-9 (GRC), 2017 - Les Diablerets, Switzerland

PARKS Robin, Ottawa, Canada

4th Ottawa International Conference on Neuromuscular Disease and Biology

STRAUB Volker, Newcastle-Upon-Tyne, Royaume-Uni

Imaging in Neuromuscular Disease 2017 - First International Conference on Imaging in Neuromuscular Disease 2017

TAILLANDIER Daniel, Saint-Genès-Champanelle, France

8th Proteasome & Autophagy Congress

Plateforme non stratégique

Financement CEDS