LISTE DES FINANCEMENTS ACCORDES PAR L'AFM-TELETHON EN 2018

POLITIQUE D'APPEL D'OFFRES

Projets soumis à l'appel d'offres

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
SARTORI Roberta, Padova, Italie
Modulating BMP axis to prevent muscle loss, dismantling of NMJ and denervation in cancer cachexia
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

Financements de projets Tremplins

CORREIA Jorge, Stockholm, Suède
Role of TRAIN in muscle regeneration and muscular disease
MORO Cédric, Toulouse, France
Intermuscular adipose tissue as a trigger of muscle wasting?
ROMAN William, Lisboa, Portugal
Discovering the mechanisms underlying intrinsic repair of muscle cells in wildtype and sarcopenia

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
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
GOMES Edgar, Lisboa, Portugal
A role for the microenvironment on nuclear positioning in myofibers
HELMBACHER Françoise, Marseille, France
FAT1 Signaling in skeletal muscle growth and repair: Muscle versus mesenchyme perspectives
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

MINCHIOTTI Gabriella, Napoli, Italie
Cripto-dependent modulation of macrophages inflammatory response in skeletal muscle regeneration and disease
MIQUEROL Lucille, Marseille, France
Deciphering the potency of ventricular trabeculae to repair the heart during cardiac regeneration in the mouse
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
Investigating heterogeneities and morphogenesis of cranial mesoderm derived skeletal muscles
TZAHOR Eldad, Rehovot, Israël
Deconstructing ERK/CaMKII signaling crosstalk during myogenesis
VINCENT Alain, Toulouse, France
Combinatorial control of muscle morphology by conserved myogenic Transcription Factors

Commission : Bases Moléculaires et Physiopathologie des Dystrophies Musculaires

Aides aux jeunes chercheurs post-doctorants

ANGEBAULT-PROUTEAU Claire, Montpellier, France
Involvement of endo/sarcoplasmic reticulum - mitochondria interaction in the Duchenne muscular dystrophy
CORDERO Gerardo, Lisboa, Portugal
DYSTRO-NET: A Network-Based Approach Towards Illuminating the Gene Regulatory Landscape of MDC1A
OSSENI Alexis, Ottawa, Canada
Targeting of Histone Deacetylase 6 (HDAC6) in Neuromuscular Diseases
SEBESTYÉN Endre, Milano, Italie
Dissecting the role of chromatin structure and alternative splicing in the development and pathology of Emery – Dreifuss Muscular Dystrophy

Financements de projets Tremplins

CLAEYS Kristl, Leuven, Belgique
Using gene expression signatures to gain novel pathophysiological insights in muscular dystrophies
NASCIMBENI Anna, Paris, France
Role of endoplasmic reticulum-plasma membrane contact sites (ER-PM CS) and autophagy in dysferlinopathy and caveolinopathy
VENEREAU Emilie, Milano, Italie
HMGB1 Redox Forms as New Targets in Duchenne Muscular Dystrophy

Financements de projets

BRICHARD Sonia, Bruxelles, Belgique
The beneficial properties of AdipoRon in dystrophinopathies
CANCELA José-Manuel, Orsay, France
Restoration of Ca2+-signalling in mdx mice by targeting the endo-lysosmal two-pore channel (TPC)
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

CIAPPONI Laura, Roma, Italie
Analysis of the DM2 pathogenic mechanisms using Drosophila as model system
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)
LOPEZ PADRINO Jose Rafael, Miami Beach, Etats-Unis
Whole body periodic acceleration a novel treatment for duchenne cardiomyopathy in mdx mice
MORALES Fernando, San-José, Costa Rica
Comparative expression profiling of multiple tissues in myotonic dystrophy
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

DUPONT Jean-Baptiste, Seattle, Etats-Unis
Molecular consequences of X-linked myotubular myopathy on the transcriptome and the epigenome of canine skeletal muscles and human myotubes derived from induced pluripotent stem cells
FERNANDES CERQUEIRA Cátia Soraia, Stockholm, Suède
Histidyl tRNA synthetase – Connecting lungs to muscle inflammation
HEISKE Margit, Toulouse, France
Virtual Cybrids: In silico approach to simulate the influence of haplogroups on the mitochondrial energy metabolism
PANJA Chiranjit, Strasbourg, France
Comparative study between MTM1 and MTMR2 myotubularins responsible for X-linked centronuclear myopathy or Charcot-Marie-Tooth neuropathy
SUAREZ CALVET Xavier, Barcelona, Espagne
The pathogenesis of dermatomyositis associated to anti-MDA5 autoantibodies

Financements de projets Tremplins

CASTETS Perrine, Basel, Suisse
Understanding Autophagic Vacuolar Myopathies: Development and characterization of mouse models for X-linked Myopathy with Excessive Autophagy
FLEMING Jennifer, Konstanz, Allemagne
Development of a novel computational biology pipeline for the efficient classification of titin SNPs for clinical use

Financements de projets

AGBULUT Onnik, Paris, France
Cardiac modeling of myofibrillar myopathy using human induced pluripotent stem cells to explore cardiac pathogenesis and drug testing
BATONNET-PICHON Sabrina, Paris, France
A new mouse model for desminopathies: physiopathological mechanisms, muscle repair and therapy
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 Géraldine, Clermont-Ferrand, France
Molecular mechanisms of mtDNA maintenance in human health and disease
FERREIRO Ana, Paris, France
ASC-1, a novel actor in the pathophysiology of congenital muscle disorders

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
LETELLIER Thierry, Toulouse, France
Influence of mtDNA background (haplogroups) on mitochondrial pathologies
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

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
SCEKIC-ZAHIROVIC Jelena, Strasbourg, France
Unravelling the role of upper motor neurons in amyotrophic lateral sclerosis onset and progression

Financements de projets Tremplins

CIURA Sorana, Paris, France
The effect of autophagy induction on the neuromuscular junction in ALS
ERFURTH Maria-Luise, Wilrijk, Belgique
Proteomic study of the YARS signaling complex in neuronal 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
Neurofilaments in Health and Charcot-Marie Tooth diseases
BORDONNE Rémy, Montpellier, France
Defining new functions for the SMN complex from yeast to human
BRITES Pedro, Porto, Portugal
Understanding how membrane ether-phospholipids regulate neuron development and function to mediate neuropathophysiology of RCDP
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
FORNE Thierry, Montpellier, France
Genome-wide profiling of nuclear-body associated regions: relation to cellular physiopathology of the spinal muscular atrophy disease
JASMIN Bernard, Ottawa, Canada
Inhibition of HDAC6 as Therapeutic Strategy in Neuromuscular Diseases
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
MuSK intracellular pathways in congenital myasthenic syndromes

MARTIN Franck, Strasbourg, France
Deciphering the mechanisms of repeat-associated non-AUG (RAN) translation in amyotrophic lateral sclerosis
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
MILLECAMPS Stéphanie, Paris, France
Genetics of Young Amyotrophic Lateral Sclerosis
PAREYSON Davide, Milano, Italie
Unravelling mechanisms of axonal loss in late-onset genetic neuropathies
ROOS Andreas, Dortmund, Allemagne
Proteogenomics to solve the unsolved exemplified by gene identification in congenital myasthenic syndromes
RUGGIERO Florence, Lyon, France
The extracellular matrix collagen XV as a new player of motor axon development and regeneration: a functional study using zebrafish
SCHENONE Angelo, Genova, Italie
Upper Limbs evaluation in hereditary NeuropAthies: the ULNA project
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

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
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

TEDESCO Francesco Saverio, London, Royaume Uni
Human iPS Cell-Derived Artificial Skeletal Muscles for Duchenne Muscular Dystrophy Therapy Development

Financements de projets

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
BORYCKI Anne-Gaëlle, Sheffield, Royaume-Uni
Control of satellite cell self-renewal by Sonic hedgehog signalling and primary cilia
PUYMIRAT Jacques, Québec, Canada
Human iPSC-derived cerebral organoids as a brain model of congenital myotonic dystrophy type 1
SACCO Alessandra, La Jolla, Etats-Unis
Role of Tenascin-C in muscle stem cell function
SACCONE Valentina, Roma, Italie
New therapeutic strategies based on FAPs-derived Exosomes in the treatment of Duchenne Muscular Dystrophy

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
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

Financements de projets Tremplins

BALESTRA Dario, Ferrara, Italie
RNA-Based therapeutics for OTC deficiency
NOWAK Kristen, Perth, Australie
Evaluating an allele-specific genetic therapy for congenital myopathy caused by dominant mutations in the skeletal muscle actin (ACTA1) gene
WEIN Nicolas, Columbus, Etats-Unis
Functional characterization and therapeutic implications of a dystrophin isoform harboring a deletion in the dystrophin actin binding domain 1
ZHANG Nan, Houston, Etats-Unis
A CRISPR-C2c2 based system to target toxic RNA in myotonic dystrophy type 1

Financements de projets

BARON VAN EVERCOOREN Anne, Paris, France
Induced pluripotent stem cells to treat pelizaeus merzbacher disease
CHUAH Marinee, Bruxelles, Belgique
NEXTGEN-AAV: Development of next-generation AAV vectors for Duchenne muscular dystrophy
CHUAH Marinee, Bruxelles, Belgique
Development of next-generation AAV vectors as effective gene therapy platform for Limb Girdle Muscular Dystrophy Type 2E
CORRAL-DEBRINSKI Marisol, Paris, France
Development of a Gene Therapy with Neuroglobin aimed at treating Cerebellar Ataxias, independently of their genetic origin, by beneficiating of neuroglobin durable protective effect on mitochondrial function
DEGLON Nicole, Lausanne, Suisse
Toward gene repair for CNS genetic disorders
DI IORIO Enzo, Padova, Italie
Advanced therapy medicinal products for the treatment of ocular defects in Ectrodactyly-Ectodermal Dysplasia-Clefting (EEC) syndrome
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
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
MECHALI Marcel, Montpellier, France
Replication Origins containing Episomes for Gene Therapy
MOREAU-GAUDRY François, Bordeaux, France
Functional iPSC-based hematopoietic gene-therapy
MURO Andrés, Trieste, Italie
Metabolic diseases of the liver: promoterless gene targeting to cure citrullinemia type I
PAGANI Franco, Trieste, Italie
Molecular mechanism and therapeutic activity of modified U1 snRNAs in Spinal Muscular Athrophy
RICHARD Guy-Franck, Paris, France
Gene therapy in DM1 cells by induction of a TALE Nuclease

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, Bruxelles, Belgique
'Repeat-Repair': CRISPR/Cas9-mediated correction of triplet nucleotide repeats
VANDENDRIESSCHE Thierry, Bruxelles, Belgique
CRISPR/Cas-mediated in vivo gene editing for hemophilia A
ZIPPO Alessio, Povo, Italie
Exploiting epigenome editing in Kabuki syndrome: a new route towards gene therapy for rare genetic disorders

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
PAGANO Allan, Ottawa, Canada
Metformin and specific AMPK-allosteric activators: an AMPK-activating strategy for treatment of Duchenne Muscular Dystrophy
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 Tremplins

FIORENTINI Carla, Roma, Italie
The bacterial protein CNF1 as a novel strategy to counteract mitochondrial encephalomyopathies

Financements de projets

BURATI Emanuele, Trieste, Italie
Screening for splicing-modifying factors for late onset Pompe Disease
CHRISTADOSS Premkumar, League City, Etats-Unis
Acetylcholine receptor binding B cells as biomarker for myasthenia gravis
DE LONLAY Pascale, Paris, France
Acute rhabdomyolysis and muscle pain associated with mutations in the LPIN1 gene - Phase II study evaluating the safety and efficacy of Hydroxychloroquine Sulfate in patients with Lipin-1 deficiency
JASMIN Bernard, Ottawa, Canada
Repurposing drugs that target eEF1A2 to increase translation of utrophin in dystrophic muscle
ROSSI Daniela Maria, Pavia, Italie
Developing combinatorial therapies for the treatment of spinal muscular atrophy
TIMMERMAN Vincent, Antwerpen, Belgique
A preclinical study to treat neuromuscular diseases caused by mutations in the small heat shock protein HSPB8
TZARTOS Socrates, Athens, Grèce
Preclinical assessment of an antigen-specific therapeutic approach for MG
ZAMMIT Peter, London, Royaume Uni
Modelling FSHD as a tool for testing therapeutics

Commission : Médicale

Financements de projets Tremplins

RICCI Giulia, Pisa, Italie
Characterization of the phenotypic variability in FSHD families for assisting clinical research

Financements de projets

GAGNON Cynthia, Québec, Canada
Development of a questionnaire to assess the severity of dysphagia in oculopharyngeal muscular dystrophy
LAFORCE JR Robert, Québec, Canada
PET Imaging of Tau Pathology in Myotonic Dystrophy type 1: A Pilot Study

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
BRAZ Sandra, Paris, France
Oligodendrocyte dysfunction and myelin abnormalities in a transgenic mouse model of myotonic dystrophy type 1
CHATZOVOULOU Kalliopi, Paris, France
Mitochondrial gene expression in normal, mutant and 3-parent preimplantation embryos
CLAEYSSEN Charlotte, Lille, France
O-GlcNAcylation and its interplay with phosphorylation: which impact on the cytoarchitecture and the function of skeletal muscle?
CROISSANT Coralie, Pessac, France
Role of annexins in membrane repair of human 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
DAHER Marie-Thérèse, Paris, France
Bcl11b/CTIP2, a transcriptional repressor: its role in cardiac homeostasis and remodeling
DANDELOT Elodie, Paris, France
Unusual CTG repeat in DM1 families: analyze of the interruptions role in trinucleotides instability mechanisms
DANIEL Malo, Nantes, France
Characterization of Liver-induced allo-specific CD8 regulatory T lymphocytes
DOS SANTOS Matthieu, Paris, France
Genetic control of adult muscle fiber type
FEFEU Mylène, Paris, France
Dysfunction of muscle stem cells in sepsis and regenerative therapy
GAZALAH Hussein, Montpellier, France
Regeneration of the spinal cord in mammals: characterization, purification and properties of motoneuron perineuronal cells and Msx1+ roof stem cells
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

KOBON Kassandra, Paris, France
Control of myoblast cell-cell fusion - Crucial role of actin-based structures
MACHADO Alice, Montpellier, France
Nutrient transporters: a new tool for characterization of hematopoietic progenitors with optimal transplantation potential
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
PICOT Mélanie, Toulouse, France
Phosphoinositide-dependent regulation of mTORC1 in skeletal muscle: molecular insights and involvement in myotubular myopathy
QUETIN Marie, Créteil, France
Influence of hypoxia on myo-angiogenesis coupling during muscle repair
SIMON Isabelle, Marseille, France
Deciphering the potency of venricular trabeculae to repair the heart during cardiac regeneration in the mouse
SLEIMAN Yvonne, Montpellier, France
Cardiac dysfunction in Duchenne Muscle Dystrophy children: pathophysiological role of type 2 ryanodine receptor through hiPSC-derived cardiomyocytes
SOULARD Claire, Montpellier, France
Role of the calcium-activated chloride channel TMEM16F in amyotrophic lateral sclerosis
TERRONE Sophie, Lyon, France
Interplay between chromatin topological organization and alternative splicing regulation by RNA helicases DDX5 and DDX17
VARUK Olena, Marseille, France
Deciphering the dominant active mechanism of mitofusin alleles associated with Charcot-Marie-Tooth type 2A disease
ZARROUKI Faouzi, Montigny-le-Bretonneux, France
Dystrophin and neuronal plasticity: towards a possible treatment of the cognitive defects in DMD using novel antisense oligonucleotides

Appel d'offres ARN médicament et cible

D'AMATI Giulia, Roma, Italie
Stabilization of tRNAs as a therapeutic strategy for diseases due to mutations in mt-tRNAs
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 Technologies de rupture en bioproduction

ROSSI Axel, Hannover, Allemagne
Enveloped AAV: a novel « All in one » gene transfer system
SCHNEIDER Bernard, Lausanne, Suisse
Production of AAV vectors by plasmid-based transient transfection of S2 Drosophila cells

Partenariats institutionnels

NEDELEC Stéphane, Paris, France
Stem cell approaches of human motor neuron diversity in development and diseases

Partenariats associatifs

Association Gêniris
ABERDAM Daniel, Paris, France
Modeling of human aniridia and gene therapy approaches
Collagen VI Alliance
STRAUB Volker, Newcastle, Royaume Uni
Trial readiness for Collagen VI Myopathies
Cure Congenital Muscular Dystrophy (Cure CMD)
ZITO Ester, Milano, Italie
Ablation of the maladaptive ER stress response improves altered calcium handling and restores diaphragm function in SELENON knockout mice
Fondation du Rein
FARGE Dominique, Paris, France
Use of allogenic mesenchymal stem cells in severe systemic lupus erythematosus
Fonds de dotation pour la recherche sur l'Hyperplasie Congénitale des Surrénales (IFCAH)
RUIZ-BABOT Gérard, Boston, Etats-Unis
Pathophysiology and therapeutics challenges of congenital adrenal hyperplasia
Retina France
DELETTRE-CRIBAILLET Cécile, Paris, France
Gene therapy to treat optic atrophy in Wolfram Syndrome
ZEITZ Christina, Paris, France
Follow up on gene therapy for CSNB
BELENGUER Pascale, Toulouse, France
Proof of principle of an original therapeutic strategy for dominant optic atrophy
Vaincre la Mucoviscidose (VLM)
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
MONTIER Tristan, Brest, France
Développement et optimisation de formulations lipidiques multi-modulaires pour la transfection par aérosolisation
Vaincre les Maladies Lysosomales (VML)
GRITTI Angela, Roma, Italie
Combined gene/cell therapy approaches to provide full rescue of the Sandhoff pathological phenotype

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
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
RICHARD Isabelle, Evry, France
Consolidating data for rescue of missense mutations from degradation
STROCHLIC Laure, Paris, France
MuSK frizzled-like domain at the NMJ: novel insight into pathophysiology and therapeutics for myasthenia
TOME Stéphanie, Paris, France
How to contract CAG.CTG repeats in myotonic dystrophy type 1

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
Pathophysiology and therapeutic proof-of-concepts for congenital myopathies
MENASCHE Philippe, Paris, France
Treatment of Anthacycline-Induced Cardiomyopathy by Intravenous Administration of Cardiovascular Progenitor Cell-Derived Extracellular Vesicles
MUNTONI Francesco, London, Royaume Uni
Advances in oligonucleotide-mediated exonskipping for DMD and related disorders - WP3 - Natural history extension
MYOPHARM Program, Evry, France
Collaborative program for a systematic, mecanistic and pharmacologic approach of rare neuromuscular diseases
ROTIG Agnès, Paris, France
An integrated approach for MITOchondrial disorder THERApeutics from yeasts and worms to humans

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

Structures stratégiques

FONDATION MALADIES RARES, Paris, France
Subvention 2018
GENATLANTIC, Nantes, France
Financement 2018
GENOPOLE, Evry, France
Participation financière de l'AFM au budget 2018 du GIP GENOPOLE

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)
SACCONI Sabrina, Nice, France
Resolve FSHD: clinical trial readiness to solve barriers to drug development in FSHD

AUTRES ACTIONS

Manifestations scientifiques (congrès, colloques)

BENVENISTE Olivier, Paris, France
3RD GLOBAL CONFERENCE ON MYOSITIS (GCOM), Berlin 27-30 March 2019
BOUILLAUD Frédéric, Paris, France
Annual meeting of MeetOchondrie Network
CHAZAUD Bénédicte, Villeurbanne, France
2019 Gordon Research Conference and Gordon Research Seminar Myogenesis: Building, Maintaining and Regenerating Skeletal Muscle
GABELLINI Davide, Milano, Italie
IIM-Myology Meeting: Pathogenesis and Therapies of Rare Diseases
JAMAR Gaëlle, Paris, France
Annual congress of the European Society for Gene and Cell Therapy, Public Engagement Day
KATEB Babak, West Hollywood, Etats-Unis
15th Annual World Brain Mapping of SBMT in Los Angeles
KINOSHITA June, Lexington, Etats-Unis
FSH Society 2018 International Research Conference
KINOSHITA June, Lexington, Etats-Unis
2018 FSHD Connect Conference
KRAUSS Robert, New York, Etats-Unis
Skeletal Muscle Satellite Cells and Regeneration
LEGAY Claire, Paris, France
Let's meet at the neuromuscular junction
ZAGNOLI Fabien, Brest, France
Organization of the "16èmes Journées de la Société Française de Myologie"

Plateforme non stratégique

CARRE Monique, Mézilles, France
Financement CEDS