

**FINANCEMENTS DE PROJETS ET AIDES AUX JEUNES CHERCHEURS  
ATTRIBUES PAR L'AFM EN 2012**

**1/ APPELS D'OFFRES AFM (*SESSION 1 et SESSION 2*)**

**Groupe 1 : Myologie fondamentale**

**Aides aux jeunes chercheurs - Session 1**

**AGUETTAZ Elizabeth**, Dir Labo BECQ, POITIERS, France

*Projet* : Stretch-induced membrane damages and repair in cardiomyocytes from mdx mice with dilated cardiomyopathy

**ARADHYA Rajaguru**, Dir Labo VAURY, CLERMONT FERRAND, France

*Projet* : Dissecting developmental programs controlling the quiescent nature of Adult Muscle Precursors (AMPs) in Drosophila using cell-type specific translational profiling

**ARNOLD Ludovic**, Dir Labo MAZIER, PARIS, France

*Projet* : Inflammatory chemokines in skeletal muscle regeneration

**BOULBERDAA Mounia**, Dir Labo NEBIGIL-DESAUBRY, ILLKIRCH, France

*Projet* : Role of Epicardial-PKR1 signaling in angiogenesis and adipogenesis: implication of progenitor cells

**DYACHUK Vyacheslav**, Dir Labo HAENLIN, TOULOUSE, France

*Projet* : Identification of nesprin 1-interacting proteins and their role in muscle cell differentiation

**MARULLO Fabrizia**, Dir Labo PURI, ROMA, Italy

*Projet* : DNA damage signalling to MyoD and the control of the genomic stability of skeletal muscles

**NADRA Karim**, Dir Labo PENDE, PARIS, France

*Projet* : Role of Lipin 1 in skeletal muscle development and maintenance

**POLAY ESPINOZA Micaela**, Dir Labo PUISIEUX, LYON, France

*Projet* : Identification of the Muscle Specific Splicing Code and implication for neuromuscular diseases

**RYCKEBUSCH Lucile**, Dir Labo YELON, LA JOLLA, USA

*Projet* : Role of Tmem2, a novel transmembrane protein in ventricular maturation and muscle morphogenesis

**Aides aux jeunes chercheurs - Session 2**

**BEYER Sabrina**, Dir Labo LE BIVIC, MARSEILLE, France

Projet : Development of the ventricular conduction system: Impact on ventricular non-compactation

**BONNEAU Benjamin**, Dir Labo PUISIEUX, LYON, France

Projet : Implication of Bcl-2 family of proteins in actin-myosin contractility: Case of Nr2f1 in zebrafish development

**GOPALAKRISHNAN Swetha**, Dir Labo TAJBAKHSI, PARIS, France

Projet : Clonal lineage fate and temporal identity of skeletal muscle stem cell progeny

**JUNION Guillaume**, Dir Labo VAURY, CLERMONT- FERRAND, France

Projet : Molecular and functional analysis of cardiac diversification by new cell-specific genomic approaches

**LAURENT Anne-Coline**, Dir Labo PARINI, TOULOUSE, France

Projet : Role of autophagy and ER stress in B-adrenergic receptor and Epac induced pathological cardiac muscle growth

**MAYEUF Alicia**, Dir Labo BUCKINGHAM, PARIS, France

Projet : Regulation of skeletal muscle versus other cell fate decisions of multipotent progenitors in the somite

**RATTI Francesca**, Dir Labo SCHAEFFER, LYON, France

Projet : Role of Histone Deacetylase 6 in skeletal muscle atrophy

**SANTOLINI Marc**, Dir Labo PEREZ, PARIS, France

Projet : Genome-wide transcriptional synergy between Six and MyoD in myogenesis

#### Financements de projets Tremplins - Session 1

**DICKMEIS Thomas**, Dir Labo STRAHLE, KARLSRUHE, Germany

Projet : In vivo measurement of glucose dynamics in muscle cells by FRET based glucose nanosensors

**FAURE Sandrine**, Dir Labo MERCIER, MONTPELLIER, France

Projet : Neuromuscular development of the gastrointestinal tract and associated diseases

#### Financements de projets Tremplins - Session 2

**PALACIOS Daniela**, Dir Labo PURI, ROME, Italy

Projet : Mechanisms of p38alpha-mediated genome reorganization in muscle stem cells

#### Financements de projets - Session 1

**COMBADIERE Christophe**, Dir Labo MAZIER, PARIS, France

Projet : Inflammatory chemokines in skeletal muscle regeneration

**CREPALDI Tiziana**, Dir Labo PANZICA, TORINO, Italy

Projet : Cardiac cachexia and cardiac effects of muscular atrophy: a cross-talk of autophagic wasting

**CRESCENZI Marco**, Dir Labo GARACI, ROMA, Italy

Projet : Enhanced cell proliferation for muscle repair and regenerative medicine

**DANDOLO Luisa**, Dir Labo COURAUD, PARIS, France

Projet : Role of the imprinted H19 - Igf2 locus in myogenesis

**DE SANTA BARBARA Pascal**, Dir Labo MERCIER, MONTPELLIER, France  
*Projet* : Molecular control of the visceral Smooth Muscle Cell development

**DUPREZ Delphine**, Dir Labo JESUS, PARIS, France  
*Projet* : Bmp and Fgf signalling pathways, cell cycle and foetal myogenesis

**GAUTHIER-ROUVIERE Cécile**, Dir Labo DEBANT, MONTPELLIER, France  
*Projet* : Role of flotillins in cadherin-mediated signaling during myogenesis

**GHERARDI Romain**, Dir Labo GHERARDI, CRETEIL, France  
*Projet* : The microvasculature as a niche for muscle satellite cells: a role for pericytes

**JAGLA Krzysztof**, Dir Labo VAURY, CLERMONT FERRAND, France  
*Projet* : Identifying translational signatures underlying diversification of muscle subtypes and quiescent nature of muscle stem cells using targeted translational profiling approach in Drosophila model

**KALCHEIM Chaya**, Dir Labo KALCHEIM, JERUSALEM, Israel  
*Projet* : The molecular network underlying fate segregation of dermomyotome progenitors

**LALOUETTE Alexis**, Dir Labo BALDACCI, PARIS, France  
*Projet* : Interactions between Notch and Myocyte Enhancer Factor 2 during flight muscle differentiation in Drosophila

**LAMAZE Christophe**, Dir Labo GOUD, PARIS, France  
*Projet* : Role of caveolin-3 in the response of muscle cells to mechanical stress

**LE GRAND Fabien**, Dir Labo COURAUD, PARIS, France  
*Projet* : Role of Wnt signalling during Muscle Regeneration

**LEZOUALC'H Frank**, Dir Labo PARINI, TOULOUSE, France  
*Projet* : Role of autophagy and ER stress in Beta-adrenergic receptor induced pathological cardiac muscle growth

**MAIRE Pascal**, Dir Labo COURAUD, PARIS, France  
*Projet* : Six homeoproteins networks in adult muscle cells in different physiopathological conditions

**MAKARENKOVA Helen**, Dir Labo GALL, SAN DIEGO, USA  
*Projet* : Regulation of Wnt signaling during satellite cell activation and muscle regeneration

**MERDES Andreas**, Dir Labo HAENLIN, TOULOUSE, France  
*Projet* : The role of nesprins and centrosome proteins in muscle cell differentiation and disease

**MOOG-LUTZ Christel**, Dir Labo GIRARD, TOULOUSE, France  
*Projet* : Unravelling the roles of ASB2B and filamin b during myogenic differentiation

**MOUNIER Rémi**, Dir Labo COURAUD, PARIS, France  
*Projet* : Role of AMPK in the regulation of inflammation in skeletal muscle regeneration

**MUSARO Antonio**, Dir Labo MUSARO, ROMA, Italy  
*Projet* : Characterize the role of IGF-1 isoforms on muscle regeneration

**PATEL Ketan**, Dir Labo PATEL, READING, United Kingdom  
*Projet* : Investigating the development of a newly discovered source of vertebrate skeletal muscle

**PRATS Anne-Catherine**, Dir Labo PRATS, TOULOUSE, France  
*Projet* : Coupled transcription-translation regulators for the design of new expression systems in muscle

**TAJBAKHSH Shahragim**, Dir Labo TAJBAKHSH, PARIS, France  
*Projet* : Generation and characterisation of skeletal muscle stem/progenitor and differentiated cells from human embryonic stem cells

**TZAHOR Eldad**, Dir Labo TZAHOR, REHOVOT, Israel

Projet : Many ways to myogenesis: unraveling distinct molecular paths in head muscle progenitors

**TZAHOR Eldad**, Dir Labo TZAHOR, REHOVOT, Israel

Projet : Cardiac and skeletal muscle development: from the embryo towards regenerative medicine

**VESCHAMBRE Philippe/THIEBAUD Pierre**, Dir Labo MOREAU, BORDEAUX, France

Projet : Calpain and Muscle Regeneration

**ZINN-JUSTIN Sophie**, Dir Labo GILQUIN, GIF-SUR-YVETTE, France

Projet : Understanding laminopathy mechanisms involving transcription deregulation at the inner nuclear membrane

## **Financements de projets - Session 2**

**ARANEGA Amelia**, Dir Labo ARANDA HARO, JAEN, Spain

Projet : Role of PITX2 regulating miRNAs expression during myogenesis

**BORYCKI Anne-Gaëlle**, Dir Labo HOLLEY, SHEFFIELD, United Kingdom

Projet : Characterization of Laminin alpha 1 function in adult skeletal muscles

**COLETTI Dario**, Dir Labo FRIGUET, PARIS, France

Projet : Mechanisms of satellite cell activation-fusion uncoupling induced by TNF: central role of Pax7 deregulation

**COMBARET Lydie**, Dir Labo CHARDIGNY, SAINT GENES CHAMPANELLE, France

Projet : Outside-in Signalling from ExtraCellular Matrix to Muscle Proteolysis during Atrophy and Recovery

**DUBOIS Laurence**, Dir Labo HAENLIN, TOULOUSE, France

Projet : Genome wide screens for muscle identity genes

**FRANCASTEL Claire**, Dir Labo WEITZMAN, PARIS, France

Projet : Regulation of the bifunctional SRA RNA by alternative splicing during normal and pathological muscle differentiation

**GIESELER Kathrin**, Dir Labo MOUCHIROUD, VILLEURBANNE, France

Projet : Comparative study of muscle degeneration in *C. elegans* models for muscular dystrophies

**MAIRE Pascal**, Dir Labo COURAUD, PARIS, France

Projet : Six homeoproteins in the commitment of Head and Epaxial Myogenesis

**MOLINARI Susanna**, Dir Labo MOLINARI, MODENA, Italy

Projet : Investigation of the role played by Pin1 in regulating SCs function and skeletal muscle regeneration

**MONTARRAS Didier**, Dir Labo MONTARRAS, PARIS, France

Projet : Role of Pitx2 and Pitx3 transcription factors in adult muscle stem cells

**PERDIGUERO Eusebio**, Dir Labo MUNOZ CANOVES, BARCELONA, Spain

Projet : Role p38gamma in muscle stem cell and macrophage homeostasis during muscle regeneration

**SCHIAFFINO Stefano**, Dir Labo PAGANO, PADUA, Italy

Projet : Role of Mrf4 in adult skeletal muscle: effect on muscle growth

**TIRET Laurent**, Dir Labo AUBIN-HOUZELSTEIN, MAISONS-ALFORT, France

Projet : Role of very long chain fatty acids in development and physiology of skeletal muscle and heart

**WEITZMAN Jonathan/MEDJKANE Souhila**, Dir Labo WEITZMAN, PARIS, France

Projet : The role of the Histone Methyltransferase SMYD3 in myogenic differentiation

## Groupe 2 : Bases moléculaires et physiopathologie des maladies neuro-musculaires

### Aides aux jeunes chercheurs - Session 1

- CONTE Talita Cristiane**, Dir Labo BRAIS, MONTREAL, Quebec, Canada  
*Projet* : Functional Characterization of ANO5 mutated in Limb-girdle muscle dystrophy type 2 L
- HARISSEH Rania**, Dir Labo BECQ, POITIERS, France  
*Projet* : TRPC dependent calcium influx and regulation by syntrophin: Involvement in DMD human muscle cells
- KHARRAZ Yacine**, Dir Labo MUNOZ-CANOVES, BARCELONA, Spain  
*Projet* : Analysis of the role of alternatively activated macrophages in inflammation-driven fibrosis: consequence of IL-13 and IL-4 signalling loss during muscular dystrophy progression
- OSSENI Alexis**, Dir Labo FEUERSTEIN, GRENOBLE, France  
*Projet* : Study of the link between triadin and microtubules
- RAYAVARAPU Sree Veera Vss**, Dir Labo NAGARAJU, WASHINGTON DC, USA  
*Projet* : Role of Endoplasmic Reticulum Stress in Idiopathic Inflammatory Muscle Diseases
- VACCA Ophélie**, Dir Labo SAHEL, PARIS, France  
*Projet* : Modulation of mouse angiogenesis by targeted expression fo dystrophin - Dp71

### Aides aux jeunes chercheurs - Session 2

- ATTALI Ruben**, Dir Labo MITRANI-ROSENBAUM, JERUSALEM, Israël  
*Projet* : Identification of genes responsible for rare human autosomal recessive neuromuscular diseases
- ESQUE Jérémy**, Dir Labo CECCHINI, STRASBOURG France  
*Projet* : Energy storage in Myosin: from molecular function to pathology
- FREYERMUTH Fernande**, Dir Labo POURQUIE, ILLKIRCH France  
*Projet* : Study of the molecular mechanisms involved in the heart arrhythmia of patients with Myotonic Dystrophy
- LE BIHAN Marie-Catherine**, Dir Labo KRATCHMAROVA, ODENSE M, Denmark  
*Projet* : Could modifications in the muscle secretome be involved in the physiopathology of Dysferlinopathies?
- LE DOUR Caroline**, Dir Labo WORMAN, NEW YORK - NY, USA  
*Projet* : Wnt signaling and dilated cardiomyopathy in Emery-Dreifuss muscular dystrophy
- MELI Albano**, Dir Labo MERCIER MONTPELLIER, France  
*Projet* : Reprogramming of DMD-patient cells into cardiomyocytes - insights into molecular pathology of dilated cardiomyopathy
- MORATAL Claudine**, Dir Labo DANI, NICE, France  
*Projet* : Regulation of intramuscular adipogenic lineage in healthy and dystrophic human muscles
- PERNET Lydia**, Dir Labo VOURC'H, GRENOBLE, France

Projet : Role of VCP in the regulation of the stress response Implication in IBMPFD myopathy

**SCHWENZER Hagen**, Dir Labo WESTHOF, STRASBOURG, France

Projet : Sub-mitochondrial organization of human aminoacyl-tRNA synthetases and connection to mitochondrial disorders

**SEGARD Bertrand-David**, Dir Labo DUPRET, PARIS, France

Projet : Myofibrillar Myopathies: study of new cell models

**SICOT Géraldine**, Dir Labo MUNNICH, PARIS, France

Projet : Investigation of calcium metabolism dysregulation and cerebellar dysfunction in DM1

**TONIN Yann**, Dir Labo POTIER, STRASBOURG, France

Projet : Modelling antigenomic strategy of mitochondrial diseases by exploitation of mitochondrial RNA pathway

#### Financements de projets Tremplins - Session 1

**RHODES Jeremy**, Dir Labo EDWARDS, NORWICH, United Kingdom

Projet : The potential role of the innate immune response in myotonic dystrophy

#### Financements de projets Tremplins - Session 2

**ANDERSSON Daniel**, Dir Labo WESTERBLAD, STOCKHOLM, Sweden

Projet : Leaky ryanodine receptors in Limb-girdle muscular dystrophy - a treatable case?

**SCHESL Joachim**, Dir Labo SCHOSER, MÜNCHEN, Germany

Projet : Identification and characterization of novel and disease related binding and interaction partners of FHL1

#### Financements de projets - Session 1

**BARAKAT-WALTER Isabelle-Ibtissam**, Dir Labo FRACKOWIAK, LAUSANNE, Switzerland

Projet : Identification of the mechanisms underlying respiratory failure in dystrophy myotonic transgenic mice

**BENDAHHOU Said**, Dir Labo BARHANIN, NICE, France

Projet : Role of the inward potassium channels in muscle excitability

**BETTO Romeo**, Dir Labo POZZAN, PADOVA, Italy

Projet : Processing of disease-causing sarcoglycan mutants

**BONNEFOY Nathalie**, Dir Labo BOCCARD, GIF-SUR-YVETTE, France

Projet : The PPR proteins - a gateway to the identification of new factors involved in mitochondrial translation

**BORNEMANN Antje**, Dir Labo MEYERMANN, TÜBINGEN, Germany

Projet : Molecular regulation of the motility of telocytes, a new invasive cell type in idiopathic inflammatory myopathies

**CHARTRAND Pascal**, Dir Labo CHARTRAND, MONTREAL, Quebec, Canada

Projet : Characterization of chemical compounds which disrupt nuclear CUG-rich RNA foci in DM1

**COGNARD Christian**, Dir Labo BECQ, POITIERS, France



- Projet : Stretch-induced membrane damages and repair in cardiomyocytes from mdx mice with dilated cardiomyopathy  
**CONSTANTIN Bruno**, Dir Labo BECQ, POITIERS, France  
Projet : Deregulation of cation entries and TRP channels in DMD muscle: New therapeutic targets
- DE LONLAY Pascale**, Dir Labo MUNNICH, PARIS, France  
Projet : Rhabdomyolysis: pathogenic mechanisms of LPIN1-related disease and newly identified genes for therapeutic perspectives
- DURBEEJ Madeleine**, Dir Labo DURBEEJ, LUND, Sweden  
Projet : Fibrosis inhibition as treatment for congenital muscular dystrophy
- HELMBACHER Françoise**, Dir Labo ROUGON, MARSEILLE, France  
Projet : Tissue-specific silencing of FAT1 causes FSHD: motor neuron versus muscle drive
- JACQUEMOND Vincent**, Dir Labo MOUCHIROUD, VILLEURBANNE, France  
Projet : Role of phosphoinositides in calcium homeostasis in normal and diseased skeletal muscle
- JONES Peter**, Dir Labo JONES, WATERTOWN, Mass, USA  
Projet : DUX4, epigenetics, and pathogenesis of facioscapulohumeral muscular dystrophy
- KRAHE Ralf**, Dir Labo LOZANO, HOUSTON, Tx, USA  
Projet : The role of ZNF9 in the pathogenesis of myotonic dystrophy type 2 (DM2)
- LACAMPAGNE Alain**, Dir Labo MERCIER, MONTPELLIER, France  
Projet : Cardiac and respiratory muscles dysfunction in Duchenne muscular dystrophy: role of ryanodine receptors and caspases
- LAING Nigel/RAVENS-CROFT Gianina**, Dir Labo KLINKEN, NEDLANDS, Australia  
Projet : Foetal Akinesia: Genetic and Functional Studies
- MARTY Isabelle**, Dir Labo FEUERSTEIN, LA TRONCHE, France  
Projet : Pathologies of the calcium release complex
- MUNOZ-CANOVES Pura**, Dir Labo MUNOZ-CANOVES, BARCELONA, Spain  
Projet : Understanding and combating inflammation-driven fibrosis in muscular dystrophy
- PETIT Patrice X.**, Dir Labo BAROUKI, PARIS, France  
Projet : Barth syndrome and other diseases involving cardiolipin dismetabolism: mitochondrial metabolism and apoptotic dysfunctions
- PUYMIRAT Jack**, Dir Labo PUYMIRAT, QUEBEC, Canada  
Projet : Pathogenesis of myotonic dystrophy
- ROTIG Agnès**, Dir Labo MUNNICH, PARIS, France  
Projet : Identification of New Nuclear Genes of Mitochondrial Diseases
- TIRET Laurent**, Dir Labo AUBIN-HOUZELSTEIN, MAISONS-ALFORT, France  
Projet : Centronuclear myopathy: biochemical and functional characterization of PTPLA-deficient animal models
- WALLGREN-PETTERSSON Carina**, Dir Labo LEHESJOKI, HELSINKI, Finland  
Projet : Molecular mechanisms, diagnostic methods and working towards therapy for nemaline myopathy and related disorders
- ZAMMIT Peter**, Dir Labo ZAMMIT, LONDON, United Kingdom  
Projet : Role of RET in muscle stem cell function and FSHD pathology
- ZEVIANI Massimo**, Dir Labo ZEVIANI, MILANO, Italy  
Projet : Therapeutic strategies for the cure of Ethylmalonic Encephalopathy, a severe infantile mitochondrial disease

- ARNER Anders**, Dir Labo DAVIES, STOCKHOLM, Sweden  
Projet : Urinary bladder and intestinal smooth muscle function in Duchenne Muscular Dystrophy
- BRICHARD Sonia**, Dir Labo BRICHARD, BRUXELLES, Belgium  
Projet : Adiponectin and skeletal muscle: potential role in Duchenne muscular dystrophy
- BUSHBY Kate**, Dir Labo CHINNERY, NEWCASTLE - UPON - TYNE, United Kingdom  
Projet : A myogenic factor secreted by Myoblasts except in Dysferlinopathy
- CARRA Serena**, Dir Labo TOMASI, MODENA, Italy  
Projet : Identification of HSPB3 mutations in myopathic patients: understanding the mechanisms of disease
- CHAZAUD Bénédicte**, Dir Labo COURAUD, PARIS, France  
Projet : How inflammation regulates myogenic cell fate and fibrosis during degenerative myopathies
- CONTE CAMERINO Diana**, Dir Labo CONTE CAMERINO, BARI, Italy  
Projet : Pharmacotherapy of hereditary chloride and sodium channel myotonic disorders
- DECHESNE Claude**, Dir Labo DANI, NICE, France  
Projet : Impact of intramuscular adipogenic lineage on human muscle: origin, function and contribution to fibrosis
- ELVASSORE Nicola**, Dir Labo ELVASSORE, PADOVA, Italy  
Projet : Generation of 2D and 3D micro-structured substrates : implication of mechanical and biochemical properties in DMD pathology
- ESTEVEZ Raul**, Dir Labo ESTEVEZ, BARCELONA, Spain  
Projet : Development and characterization of a zebrafish model of myotonia congenita
- FARINA Cinthia**, Dir Labo FARINA, MILANO, Italy  
Projet : Role of neurotrophins and their receptors in human skeletal muscle
- FERNANDEZ-VIZARRA Erika**, Dir Labo MARIJUAN, ZARAGOZA, Spain  
Projet : Patho-physiology of mitochondrial respiratory chain complex III assembly
- GOILLOT Evelyne**, Dir Labo SCHAEFFER, LYON, France  
Projet : Regulation of protein aggregation through NBR1 and p62 phosphorylation: implication for protein aggregate myopathies
- GOMES-PEREIRA Mario**, Dir Labo MUNNICH, PARIS, France  
Projet : Synaptic and cytoskeleton dysfunction in DM1 transgenic mice
- GOURDON Geneviève**, Dir Labo MUNNICH, PARIS, France  
Projet : Unusual contractions in myotonic dystrophy type 1 families: identification of the CTG repeat contraction mechanisms
- JASMIN Bernard**, Dir Labo JASMIN, OTTAWA, Ontario, Canada  
Projet : Translation Regulation of Utrophin A in Skeletal Muscle: Therapeutic Implications for DMD
- KAN Hermien**, Dir Labo BILARS, LEIDEN, The Netherlands  
Projet : Muscle MRI in Becker muscular dystrophy - relation to dystrophin expression
- LI Zhenlin**, Dir Labo FRIGUET, PARIS, France  
Projet : Muscle integrin binding protein: a link between integrin and NAD signaling
- MORGAN Jennifer**, Dir Labo MUNTONI, LONDON, United Kingdom  
Projet : The novel concept of necroptosis: its implication in DMD pathogenesis and myoblast transfer therapy
- PAQUIS-FLUCKLINGER Véronique**, Dir Labo GILSON, NICE, France  
Projet : Identification of new genes and molecular mechanisms involved in mitochondrial diseases with mtDNA instability
- RAVEL-CHAPUIS Aymeric**, Dir Labo JASMIN, OTTAWA, Ontario, Canada



- Projet : The RNA-binding protein Staufen 1 as a novel therapeutic target for Myotonic Dystrophy (DM1)
- RICCHETTI Miria**, Dir Labo DUJON, PARIS, France  
Projet : A novel single-cell analysis of mitochondrial DNA dynamics for investigation and diagnosis of myopathies
- SIMILI Marcella**, Dir Labo GAMBALE, PISA, Italy  
Projet : Role of altered MEK-ERK and p53 pathways in muscle pathology of DM1 patients: can miRNAs modulation reverse these aberrant mechanisms
- SINNREICH Michael**, Dir Labo SINNREICH, BASEL, Switzerland  
Projet : The Dysferlin Interactome - Implications for Therapeutic Strategies
- TRONCHERE Hélène**, Dir Labo PARINI, TOULOUSE, France  
Projet : The myotubularin product PI5P, as a new regulator of vesicle trafficking
- VERGANI Lodovica**, Dir Labo POZZAN, PADOVA, Italy  
Projet : Focus on mutant mtDNA segregation: influence of the mitochondrial dynamic, nuclear background and autophagy
- ZAMMIT Peter**, Dir Labo ZAMMIT, LONDON, United Kingdom  
Projet : The Ephrins as mediators of DUX4 toxicity in FSHD

### Groupe 3 : Systeme nerveux : Motoneurone et jonction neuro-musculaire

#### Aides aux jeunes chercheurs - Session 1

- BELLANCE Nadège**, Dir Labo LACOMBE, BORDEAUX, France  
Projet : Mitochondrial pathophysiology of hereditary spastic paraplegia caused by mutations in spg31 and spg15
- BELLOUZE Sarah**, Dir Labo MASSON, MARSEILLE, France  
Projet : Golgi fragmentation: causes and consequences
- DESCLAUX Mathieu**, Dir Labo HENDERSON, NEW YORK, NY, USA  
Projet : Human ES cell models of spinal muscular atrophy (SMA): cellular mechanisms and molecular targets
- FASSIER Coralie**, Dir Labo GIROS, PARIS, France  
Projet : Microtubule-associated proteins in spinal motor axon guidance
- PINAN-LUCARRE Bérangère**, Dir Labo BESSEREAU, PARIS, France  
Projet : An ADAMTS-like protein is required for clustering acetylcholine receptors at the *C. elegans* neuromuscular junction
- SALVI Julie**, Dir Labo PIN, MONTPELLIER, France  
Projet : Pathogenic Mechanisms of Cav2.1 calcium channel associated Ataxia and potential therapy approaches
- STUM Morgane**, Dir Labo W. BURGESS, BAR HARBOR, ME, USA  
Projet : Testing cell autonomy in glycyl tRNA synthetase using in vivo and in vitro models of CMT2D
- TREMBLIER Boris**, Dir Labo PUEL, Montpellier, France  
Projet : Neuroinflammation and regulation of chloride homeostasis of sensory-motor neurons

## Aides aux jeunes chercheurs - Session 2

**DURAND Christelle**, Dir Labo LACOMBE, BORDEAUX, France

Projet : Genetic and functional studies of hereditary spastic paraplegia: impact on axonal outgrowth and dendritic arborisation

**FRANCOIS Amaury**, Dir Labo PIN, MONTPELLIER, France

Projet : A new genetic approach to decipher the role of T-type calcium channels in the pathophysiology of muscle pain

**MILAN Léa**, Dir Labo CAZALET, BORDEAUX, France

Projet : The cholinergic system in SOD1 spinal locomotor network

**NAPOLETANO Francesco**, Dir Labo SCHAEFFER, LYON, France

Projet : A Drosophila model to study neurodegeneration and caspase-independent cell death in health and disease

**PIGUET Françoise**, Dir Labo BOLINO, MILANO, Italy

Projet : Modulating Neuregulin-1 signals to treat hereditary demyelinating neuropathies

**RIGATO Chiara**, Dir Labo GIROS, PARIS, France

Projet : Implication of microglial cell invasion in the early synaptogenesis on motoneurons in the embryonic spinal cord

**SCHMITT Florent**, Dir Labo LOEFFLER, STRASBOURG, France

Projet : Role of Lipid Metabolism in ALS and motor neuron survival

## Financements de projets Tremplins - Session 1

**KABASHI Edor**, Dir Labo ZALC, PARIS, France

Projet : Characterization of transgenic models for FUS and TDP-43 in zebrafish

## Financements de projets Tremplins - Session 2

**BOGDANIK Laurent**, Dir Labo BURGESS, BAR HARBOR, USA

Projet : Protection of neuromuscular junctions in neuromuscular disorders

**LIEBERAM Ivo**, Dir Labo LIEBERAM, LONDON, United Kingdom

Projet : Development of a neural tissue culture model of ALS by TALEN-mediated gene targeting in ES cells

## Financements de projets - Session 1

**BOURINET Emmanuel**, Dir Labo PIN, MONTPELLIER, France

Projet : A new genetic approach to decipher the role of T-type calcium channels in the pathophysiology of muscle pain

**CHARBONNIER Frédéric**, Dir Labo VIDAL, PARIS, France

Projet : Mechanisms of NMDA-induced benefits for SMA motor neurons in spinal muscular atrophy

**CHARNAY Patrick**, Dir Labo TRILLER, PARIS, France

Projet : Molecular Genetics of peripheral glia

**DAGA Andrea**, Dir Labo DAGA, PADOVA, Italy

Projet : Modelling the role of Atlastin in physiology and HSP motor neuron pathology

- DEVAUX Jérôme**, Dir Labo ENJALBERT, MARSEILLE, France  
*Projet* : The role of antibodies against proteins of the node of Ranvier in the pathogenesis of Guillain-Barré syndrome: finding novel immune targets
- IBRAHIM El Cherif**, Dir Labo ENJALBERT, MARSEILLE, France  
*Projet* : Understanding splicing defects underlying familial dysautonomia
- LEGAY Claire**, Dir Labo VIDAL, PARIS, France  
*Projet* : Structure and activity of the ColQ/MuSK complex, consequences for congenital myasthenic syndroms
- MARTINS Alexandra**, Dir Labo FREBOURG, ROUEN, France  
*Projet* : Evaluation of the contribution of SMN1 and SMN2 sequence variants to the clinical severity of SMA
- MASSAAD Charbel**, Dir Labo VIDAL, PARIS, France  
*Projet* : Role of the transcription coregulator RIP140 in myelination: relevance in CMT1A pathology
- PEREIRA DE ALMEIDA Luis**, Dir Labo PEREIRA DE ALMEIDA, COIMBRA, Portugal  
*Projet* : Role of the autophagy-related protein Beclin-1 in Machado-Joseph disease
- RAOUL Cédric**, Dir Labo PUEL, MONTPELLIER, France  
*Projet* : The Contribution of TWEAK in Amyotrophic Lateral Sclerosis
- ROBBERECHT Wim**, Dir Labo ROBBERECHT, LEUVEN, Belgium  
*Projet* : Tubulin acetylation in motor neuron degeneration a translational approach
- SCAMPS Frédérique**, Dir Labo PUEL, MONTPELLIER, France  
*Projet* : Peripheral nerve regeneration: regulation and role of cation-chloride co-transporters
- VIOLLET Louis**, Dir Labo SWOBODA, SALT LAKE CITY, USA  
*Projet* : Genetic study of autosomal recessive chronic distal spinal muscular atrophy

### **Financements de projets - Session 2**

- BERTRAND Sandrine**, Dir Labo CAZALET, BORDEAUX, France  
*Projet* : The spinal muscarinic system in early stages of amyotrophic lateral sclerosis in SOD1 mice
- BOLINO Alessandra**, Dir Labo BOLINO, MILANO, Italy  
*Projet* : Modulating Neuregulin-1 signals to treat hereditary demyelinating neuropathies
- CARROLL Patrick**, Dir Labo PUEL, MONTPELLIER, France  
*Projet* : Roles of Maf family proteins in sensorimotor system
- DEMENEIX Barbara**, Dir Labo DEMENEIX, PARIS, France  
*Projet* : Gene networks underlying thyroid hormone control of neural Stem Cell proliferation
- GOIZET Cyril**, Dir Labo LACOMBE, BORDEAUX, France  
*Projet* : Genetic and functional analyses of hereditary spastic paraplegia caused by lipid gene mutations
- HAZAN Jamilé**, Dir Labo GIROS, PARIS, France  
*Projet* : Microtubule-associated proteins in spinal motor axon guidance
- JORDANOVA Albena**, Dir Labo VAN BROECKHOVEN, ANTWERPEN, Belgium  
*Projet* : Identification of disease mechanisms and therapeutic targets of CMT neuropathies in drosophila
- KARIYA/ MONANI Shingo/Umrao**, Dir Labo MONANI, NEW YORK, USA  
*Projet* : The Protective Effects of SMN in Motor Neuron Disease

**LAMOTTE D'INCAMPS Boris**, Dir Labo MEUNIER, PARIS, France

*Projet* : Corelease of acetylcholine and glutamate by motoneurons

**LEGENBRE Pascal**, Dir Labo GIROS, PARIS, France

*Projet* : Implication of microglial cell invasion in the early synaptogenesis on motoneurons at the onset of the development of the embryonic spinal cord networks

**MAGNAGHI Valerio**, Dir Labo RACAGNI, MILAN, Italy

*Projet* : Unrevealing mechanisms of gabaergic control of neuro-schwann cell interaction in peripheral neuropathies

**MEJAT Alexandre**, Dir Labo SCHAEFFER, LYON, France

*Projet* : AChR gene coregulation by long distance interactions

**SAOUDI Abdelhadi**, Dir Labo LIBLAU, TOULOUSE, France

*Projet* : Investigation of Vav1 signalosome and analysis of its implication in myasthenia gravis

**SORARU Gianni**, Dir Labo PEGORARO, PADOVA, Italy

*Projet* : Skeletal muscle involvement in spinal and bulbar muscular atrophy (SBMA)

#### **Groupe 4 : Thérapie cellulaire et/ou génique des Maladies Rares**

##### **Aides aux jeunes chercheurs - Session 1**

**ALVES Sandro**, Dir Labo ZALC, PARIS, France

*Projet* : RNA antisense strategy for treatment of Spinocerebellar ataxia type 7 in a knock-in mouse model

**BUCHER Thomas**, Dir Labo MOULLIER, NANTES, France

*Projet* : AAV9-mediated gene transfer in the spinal cord of a feline SMA model

**COCHONNEAU DE BARROS Stéphanie**, Dir Labo PIECHACZYK, MONTPELLIER, France

*Projet* : T cell reconstitution by intrathymic delivery of hematopoietic stem cells

**GERARD Xavier**, Dir Labo KAPLAN, PARIS, France

*Projet* : Development of an exon skipping approach for the treatment of Leber congenital amaurosis

**JIMENEZ Gina**, Dir Labo BRICCA, LYON, France

*Projet* : Mouse in vivo gene therapy for Primary Ciliary Dyskinesia

**LIU Xiaoxuan**, Dir Labo HENRY, MARSEILLE, France

*Projet* : Dendrimersomes as nanocarriers for RNA therapeutics

**MITUTSOVA Violeta**, Dir Labo CAVALLI, MONTPELLIER, France

*Projet* : Skeletal muscle stem cell cardiogenic and neurogenic differentiation: an in vitro and in vivo analysis

**MOUMNE Lara**, Dir Labo GIROS, PARIS, France

*Projet* : Restoration of MSK-1 expression: a new brain gene therapy approach in Huntington's Disease

**ROUANET Sophie**, Dir Labo MAGNALDO, NICE, France

*Projet* : Genetic correction of Xeroderma Pigmentosum skin cells

**TOUIL Soumia**, Dir Labo KLATZMANN, PARIS, France

*Projet* : microRNA contribution to the stabilization of protein and mRNA signature of regulatory T cells

## **Aides aux jeunes chercheurs - Session 2**

**BAI Qiang**, Dir Labo KLEIN, MONTPELLIER, France

*Projet* : Effect of culture conditions on the genetic stability of pluripotent stem cells

**CARPENTIER Maxime**, Dir Labo VAN ENDERT, PARIS, France

*Projet* : Control of immune responses with miRNA-regulated AAV vectors: potency and limits for muscle transduction

**FRITZ Vanessa**, Dir Labo PIECHACZYK, MONTPELLIER, France

*Projet* : Targeted delivery of hematopoietic stem cells for T cell reconstitution in genetic immunodeficiencies

**NEGRONI Elisa**, Dir Labo UZAN, VILLEJUIF, France

*Projet* : Combination of Myoblasts, endothelial progenitors and ECM elements to improve regeneration of dystrophic muscles

**NIEMIR Natalia**, Dir Labo FRIEDLANDER, PARIS, France

*Projet* : Gene transfer in the Sandhoff mouse model using a specific recombinant AAV9 vector

**RENAUD Edith**, Dir Labo PRATS, TOULOUSE, France

*Projet* : Translational regulation of gene expression during heart ischemia: applications to cardiac gene therapy

**STEICHEN Clara**, Dir Labo UZAN, LE KREMLIN-BICETRE, France

*Projet* : Simian model of stem cell-derived hepatocyte transplantation

## **Financements de projets - Session 1**

**BOUCHE Marina**, Dir Labo ARBARELLO, ROMA, Italy

*Projet* : Targeting PKC $\theta$  to modulate innate and adaptive immune response in DMD

**BOYER Olivier**, Dir Labo BOYER, ROUEN, France

*Projet* : Oral tolerization for improved immunological tolerance following AAV-mediated muscle gene transfer

**CAILLAUD Catherine**, Dir Labo FRIEDLANDER, PARIS, France

*Projet* : Gene transfer in the Sandhoff mouse model using specific recombinant AAV9 vectors

**CARTIER Nathalie**, Dir Labo AUBOURG, PARIS, France

*Projet* : X-ALD paradigm: identification of microglia dysfunction and microglia precursor for brain gene therapy

**CHUAH Marinee**, Dir Labo VANDENDRIESSCHE, BRUSSEL, Belgium

*Projet* : Designer transcription activator -like effector nucleases (dTALENs) for gene correction in myotonic dystrophy

**COHEN-HAGUENAUER Odile**, Dir Labo BUCKLE, CACHAN, France

*Projet* : Evaluation of safety improved integrating gene transfer vectors

**DI IORIO Enzo**, Dir Labo PONZIN, ZELARINO VENEZIA, Italy

*Projet* : Towards an innovative therapy of Ectrodactyly-Ectodermal dysplasia-Clefting syndrome using allele-specific RNA silencing

**DONATO Rosario**, Dir Labo BECCHETTI, PERUGIA, Italy

*Projet* : Use of Sertoli Cells as a Novel Therapeutic Approach to Duchenne Muscular Dystrophy and Polymyositis

**EPSTEIN Alberto**, Dir Labo MOUCHIROUD, VILLEURBANNE, France



- Projet : Site-specific Excision-Dependent (SED) vectors A novel concept in HSV-1-based gene transfer vectors  
**LAGRESLE-PEYROU Chantal**, Dir Labo FISCHER, PARIS, France  
Projet : Preclinical development of gene therapy of hereditary immune disorders
- LENAERS Guy**, Dir Labo PUEL, MONTPELLIER, France  
Projet : Gene Therapy for Dominant Optic Neuropathies
- TETI Anna Maria**, Dir Labo TETI, L'AQUILA, Italy  
Projet : Experimental bio-implant strategy to cure osteoclast-poor osteopetrosis due to RANKL ablation
- TREMBLAY Jacques**, Dir Labo TREMBLAY, QUEBEC, Canada  
Projet : Transplantation of myoblasts derived from hiPSCs genetically corrected with a human artificial chromosome

## Financements de projets - Session 2

- BELAYEW Alexandra**, Dir Labo BELAYEW, MONS, Belgium  
Projet : Validation of antisense oligonucleotides against the DUX4 mRNA as a therapeutic approach for FSHD
- BRICE Alexis**, Dir Labo ZALC, PARIS, France  
Projet : RNA antisense strategy for treatment of spinocerebellar ataxia type 7 in a knock-in mouse model
- BUNING Hildegard**, Dir Labo HALLEK, COLOGNE, Germany  
Projet : Host interaction with capsid modified rAAV vectors
- CABOCHE Jocelyne**, Dir Labo GIROS, PARIS, France  
Projet : Restoration of MSK-1 expression: a new brain gene therapy approach in Huntington's Disease
- CARMELIET Peter**, Dir Labo CARMELIET, LEUVEN, Belgium  
Projet : Evaluation of the safety profile of viral VEGF-B gene transfer for ALS
- CHUAH Marinee**, Dir Labo VANDENDRIESSCHE, BRUSSEL, Belgium  
Projet : Generation and functional correction of dystrophic patient-specific induced pluripotent stem cells
- CHUAH Marinee**, Dir Labo VANDENDRIESSCHE, BRUSSEL, Belgium  
Projet : De novo design and validation of tissue-specific regulatory elements for heart and muscle-directed gene therapy
- FORLINO Antonella**, Dir Labo FORLINO, PAVIA, Italy  
Projet : Combined gene-cellular therapeutic approach for the treatment of Classical Osteogenesis Imperfecta
- GROSS David**, Dir Labo VAN ENDERT, PARIS, France  
Projet : Control of immune responses with miRNA-regulated AAV vectors: Potency and limits for induction of immune tolerance
- NGUYEN Tuan Huy**, Dir Labo ANEGON, NANTES, France  
Projet : In vivo targeted gene therapy of liver diseases using artificial nucleases: application to Crigler-Najjar
- PAGANI Franco**, Dir Labo PAGANI, TRIESTE, Italy  
Projet : SMN splicing correction mediated by Exon Specific U1 snRNA as therapy for spinal muscular atrophy
- SAHEL José-Alain**, Dir Labo SAHEL, PARIS, France



Projet : Trans-splicing of rhodopsin mRNA: modeling and therapeutic strategy for retinitis pigmentosa

**TAYLOR Naomi/KLATZMANN David**, Dir Labo PIECHACZYK, MONTPELLIER, France

Projet : Intrathymic vector targeting for gene therapy of ZAP-70 deficiency

**UZAN Georges**, Dir Labo UZAN, VILLEJUIF, France

Projet : Combined cell-matrix therapy for muscular dystrophies: role on muscle regeneration and fibrosis regression

**ZIMMERMANN Valérie/TAYLOR Naomi**, Dir Labo PIECHACZYK, MONTPELLIER, France

Projet : Intrathymic delivery of hematopoietic stem cells for T cell reconstitution

## **Groupe 5 : Recherche Translationnelle**

### **Aides aux jeunes chercheurs - Session 1**

**BARAIBAR Martin**, Dir Labo FRIGUET, PARIS, France

Projet : Oxidative proteome alterations in muscular dystrophies

**BARBIER Mathieu**, Dir Labo AUBOURG, PARIS, France

Projet : Identification of genetic markers to predict candidates for gene therapy in ALD

### **Aides aux jeunes chercheurs - Session 2**

**GINESTE Charlotte**, Dir Labo BERNARD, MARSEILLE, France

Projet : Anatomic, Functional and Metabolic characterization of murine models of severe and mild forms of NEmaline MYOPathy

**MOLZA Anne-Elisabeth**, Dir Labo PRIGENT, RENNES, France

Projet : In silico interactions of actin and nNOS with dystrophin rod domain in relation with the BMD hot spot deletion region

**VUILLEROT Carole**, Dir Labo BONNEMANN, BETHESDA - MD, USA

Projet : Advanced quality assessment of the Motor Function Measure (MFM) in congenital muscle disease

### **Financements de projets - Session 1**

**GIRARD Christian**, Dir Labo SCHERMAN, PARIS, France

Projet : Study of nonsense-mediated mRNA decay (NMD) inhibition using small molecules as molecular probes

**SEREDA Michael**, Dir Labo NAVE, GOTTINGEN, Germany

Projet : Validation of prognostic and disease biomarkers for Charcot-Marie-Tooth disease 1A (CMT1A)

**TZARTOS Socrates**, Dir Labo TZARTOS, ATHENS, Greece

Projet : Diagnosis, prevalence and characterization of a novel myasthenia gravis subtype, LRP4-MG

## Financements de projets - Session 2

**KARADUMAN Ayse**, Dir Labo. YAVUZ, ANKARA, Turkey

Projet : Creation of databases for Duchenne Muscular Dystrophy and Spinal Muscular Atrophy in Turkey

**LOCHMULLER Hanns**, Dir Labo CHINNERY, NEWCASTLE UPON TYNE, United Kingdom

Projet : Coordination of the global patient registries for neuromuscular disorder

**POLLET Nicolas**, Dir Labo FAULON, EVRY, France

Projet : Knockout frogs using engineered nucleases as phenotypic screening tools for motoneurons diseases

**PURI Pier Lorenzo**, Dir Labo PURI, ROME, Italy

Projet : HDAC/miR-regulated SWI/SNF sub-unit exchange & stage-specific response to HDAC inhibitors in dystrophic muscles

**ROSE Michael**, Dir Labo CHAUDHURI, LONDON, United Kingdom

Projet : Agreeing international Best Practice Guideline (BPG) for Inclusion Body Myositis (IBM)

**RUEGG Markus**, Dir Labo RUEGG, BASEL , Switzerland

Projet : Congenital Muscle Disease Preclinical Research Network

## Groupe 6 : Pharmacologie des Maladies Neuromusculaires

### Aides aux jeunes chercheurs - Session 1

**EL CHEIKH Khaled**, Dir Labo MARTINEZ, MONTPELLIER, France

Projet : Neoglyco-GAA for Pompe disease treatment

### Aides aux jeunes chercheurs - Session 2

**RINALDI Carlo**, Dir Labo FISCHBECK, BETHESDA, USA

Projet : Targeting AR degradation in SBMA: ASC-J9 and ASC-JM17 as novel therapeutic strategies

## Financements de projets Tremplins - Session 2

**LOSSOS Alexander**, Dir Labo BEN-HUR, JERUSALEM, Israel

Projet : Testing small molecules, glycogen autophagy and microtubule transport as treatments of glycogenoses

**PEREZ FERNANDEZ Daniel**, Dir Labo PEREZ-PEREZ, MADRID, Spain

Projet : Casein Kinase 1 inhibitors as an innovative treatment for Amyotrophic Lateral Sclerosis

**ROSSI Daniela Maria**, Dir Labo ROSSI, PAVIA, Italy

*Projet* : Novel therapeutic strategies for SMA: testing the efficacy of cell-permeable peptides in preclinical models

### **Financements de projets - Session 1**

**GARCIA Marcel**, Dir Labo MARTINEZ, MONTPELLIER, France

*Projet* : Neoglycoenzymes as a new enzyme recombinant therapy

**LYNCH Gordon**, Dir Labo S. LYNCH, VICTORIA, Australia

*Projet* : Optimizing the therapeutic potential of formoterol for muscular dystrophy and related muscle wasting diseases

**MARTINEZ-MARTINEZ Pilar**, Dir Labo STEINBUSCH, MAASTRICHT, The Netherlands

*Projet* : A novel immunotherapy for myasthenia gravis - Effect of bortezomib on autoantibody production

### **Financements de projets - Session 2**

**DI RAGO Jean-Paul**, Dir Labo DAIGNAN-FORNIER, BORDEAUX, France

*Projet* : Yeast models of MPV17-based diseases: mechanisms and drug screening

**HUSSON Isabelle /RUSTIN Pierre**, Dir Labo GRESSENS, PARIS, France

*Projet* : Effect of pioglitazone administrated to patients with friedreich's ataxia : proof of concept

**KICHLER Antoine**, Dir Labo SCHERMAN, PARIS, France

*Projet* : Evaluation of the anti-myostatin activity of peptides deriving from the Small Leucine Rich Proteoglycan family

**LOSSOS Alexander**, Dir Labo BEN-HUR, JERUSALEM, Israël

*Projet* : Testing small molecules, glycogen autophagy and microtubule transport as treatments of glycogenoses

### **Groupe 7 : Commission Clinique**

### **Aides aux jeunes chercheurs - Session 2**

**BANKOLE Senakpon Landry Cyrille**, Dir Labo BELLI, SAINT ETIENNE, France

*Projet* : Prospective study for 24-months of physical training introduced in lifestyle of patients with facioscapulohumeral Dystrophy - Tolerance, sustainability and efficiency of unsupervised training program

### **Financements de projets - Session 1**

**PETITJEAN Michel**, Dir Labo LOFASO, AMIENS, France

Projet : Effect of mechanical insufflation-exsufflation on respiratory parameters in two neuromuscular populations

### Financements de projets - Session 2

**FEASSON Leonard**, Dir Labo BELLI, SAINT-ETIENNE, France

Projet : Prospective study for 24-months of physical training introduced in lifestyle of patients with FacioScapuloHumeral Dystrophy: Tolerance, sustainability and efficiency of unsupervised training program

**SICILIANO Gabriele**, Dir Labo SICILIANO, PISA, Italy

Projet : Brain involvement in myotonic dystrophy type I: from functional neuroimaging to the impact on quality of life

**VANDENBERGHE Nadia**, Dir Labo VIAL, LYON-BRON, France

Projet : Evaluation of efficacy of botulinum toxin A in the treatment for sialorrhea of amyotrophic lateral sclerosis patient

## 2/ AUTRES TYPES DE FINANCEMENTS

### Aides aux jeunes chercheurs

**KARMOUCH Jennifer**, Dir Labo VIDAL, PARIS, France

Projet : ColQ and the post transcriptional regulation of AchR mRNA

**MURAUER Eva**, Dir Labo CLAUSTRES, MONTPELLIER France

Projet : Towards a better understanding of splicing regulation in the DMD gene: a combined RNAi and RNA-seq approach

### A/ Pôles stratégiques

**LEVY Nicolas**, Dir Labo LEVY, MARSEILLE, France

Projet : Genomics, pathophysiology and therapeutic approaches in a panel of rare genetics diseases mainly affecting muscle, heart & nervous system

**MOULLIER Philippe/BOISBONNE/ONIRIS**, Dir Labo MOULLIER, NANTES, France

Projet : Institut Nantais - Progress reports

### B/ Projets stratégiques

**DICKSON George J**, Dir Labo DICKSON, SURREY, UK

Projet : Gene surgery for Duchenne Muscular Dystrophy (DMD) using site-specific Endonucleases

**HEARD Jean-Michel**, Dir Labo HEARD, PARIS, France

Projet : Projet pré-clinique de thérapie génique des mucopolysaccharidoses

**POURQUIE Olivier**, Dir Labo POURQUIE, ILLKIRCH, France

*Projet* : Differentiating ES Cells or Induced Pluripotent Cells into Skeletal Muscle as Therapy for Muscular Dystrophies

**PUYMIRAT Jack/WOOD Matthew**, Dir Labo PUYMIRAT, QUEBEC, Canada

*Projet* : Evaluation of peptide antisense oligonucleotides as gene therapy for myotonic dystrophy

**ROLLING Fabienne**, Dir Labo MOULLIER, NANTES, France

*Projet* : Phase 1/2 clinical gene therapy protocol for the treatment of retinal dystrophy caused by defects in RPE65

**PROSENSA/DOCHEZ Luc**, LEIDEN, The Netherlands

*Projet* : Accelerated development of three clinical candidates into Phase I/II studies for the treatment of selected patient populations in Duchenne Muscular Dystrophy

**SIMONELIG Martine**, Dir Labo BUCHETON, MONTPELLIER, France

*Projet* : Pathophysiology and therapeutic approaches in Oculopharyngeal Muscular Dystrophy (OPMD)

**TROPHOS**, MARSEILLE, France

*Projet* : Clinical development of the olesoxime in SMA

**WOOD Matthew/MUNTONI Francesco**, Dir Labo DAVIES, OXFORD, UK

*Projet* : Advances in oligonucleotide-mediated exon skipping for DMD and related disorders

### **C/ Plateformes stratégiques**

**BLOT Stephane/ENVA**, Coordinateur BLOT, MAISONS-ALFORT, France

*Projet* : Budget 2012

**CIC GARCHES/ANNANE Djillali**, Coordinateur ANNANE, GARCHES, France

*Projet* : Renouvellement de la contribution financière au CIC Innovation Technologique de Garches - 1ère année

**CIC NANTES**, Coordinateur GUSTIN, NANTES, France

*Projet* : Financement de la 3ème année (2012) du CIC de Biothérapies (CIC-BT) de Nantes

**CIC NECKER**, Coordinateur CAVAZZANA-CALVO, PARIS, France

*Projet* : Financement de la 3ème année du CIC Biothérapies de Necker suite au rapport d'activité annuel

**CIC SAINT-LOUIS**, Coordinateur LARGHERO, PARIS, France

*Projet* : Financement de la 4ème année du CIC Biothérapies de Saint-Louis

### **D/ Structures stratégiques**

**FCS-FONDATION MALADIES RARES**, Président LEVY, PARIS, France

*Projet* : Soutien AFM 2012

**MOULLIER Philippe**, Dir Lab MOULLIER, NANTES, France

*Projet* : Atlantic Bio GMP - Engagement 2012

### **E/ Outils stratégiques**

**SACCONI Sabrina**, Dir Labo DESNUELLE, NICE, France

*Projet* : French National FSHD patient registry for clinical trial planning and translational research

## **F/ Partenariats institutionnels**

**TRICAUD Nicolas/AVENIR**, Dir Labo HAMEL, MONTPELLIER, France

*Projet* : Molecular mechanisms of myelination/demyelination and gene therapy in the peripheral nervous system

## **G/ Partenariats associatifs**

### **Appel d'offres SMA Europe**

## **Financements de projets**

**MONANI Umrao**, Dir Labo MONANI, New York, USA

*Projet* : Investigating novel genetic determinants of the SMA phenotype

## **Autres partenariats**

**ASS. pour la RECHERCHE sur la SCLEROSE EN PLAQUES (ARSEP)**, IVRY-SUR-SEINE, France

*Projet* : Financement de projets sélectionnés par l'Association

**ASS. BELGE des MALADIES MUSCULAIRES/HUET Jean-Marie**, MONS, Belgium

*Projet* : Financement de projets sélectionnés par l'Association

**ASS. RETINA France**, COLOMIERS, France

*Projet* : Financement de projets sélectionnés par l'Association

**INSTITUT DE RECHERCHE SUR LA MOELLE EPINIERE ET L'ENCEPHALE (IRME)**, PARIS, France

*Projet* : Financement de projets sélectionnés par l'Association

**LANTZ Brigitte/FONDATION DU REIN**, PARIS, France

*Projet* : Financement de projets sélectionnés par l'Association

**VLM/VAINCRE LA MUCOVISCIDOSE**, PARIS, France

*Projet* : Financement de projets sélectionnés par l'Association

**VML/ VAINCRE LES MALADIES LYSOSOMALES**, MASSY, France

*Projet* : Financement de projets sélectionnés par l'Association

## **H/ Projets soumis en dehors de l'appel d'offres ou sortis de l'appel d'offres**

**COHEN-HAGUENAUER Odile**, Dir Labo BUCKLE, CACHAN, France

*Projet* : Title of the project: Lentivirus vector platform intended for service and development of emerging technologies

**HOVNANIAN Alain**, Dir Labo MUNNICH, PARIS, France



Projet : Meganuclease-based therapy for dystrophic epidermolysis bullosa  
**POURQUIE Olivier**, Dir Labo POURQUIE, ILLKIRCH, France

Projet : AFM-IGBMC Partnership - Projets additionnels PUCCIO - TROTTIER  
**RUEGG Urs**, Dir Labo RUEGG, GENEVE, Switzerland

Projet : Essai pré-clinique sur le traitement de la myopathie de Duchenne par le tamoxifène

## **I/ Manifestations scientifiques**

**BRAS Hélène**, MARSEILLE, France

Titre : Inauguration de l'Institut des Neurosciences de la Timone, les 20 - 21 septembre 2012 à Marseille

**BURBAN Aude**, PARIS, France

Titre : Les Transversales Santé 2012

**CHARBONNIER Jean-Baptiste**, GIF-SUR-YVETTE, France

Titre : 10ème Colloque des 3R (Réplication, Recombinaison, Réparation de l'ADN), Presqu'île de Giens, 21 - 24 mai 2013

**CHAZAUD Bénédicte**, PARIS, France

Titre : Journée Jean-Claude DREYFUS", 7 septembre 2012 : « Skeletal muscle homeostasis: cell interactions, molecular mechanisms and metabolic regulations »

**FSH SOCIETY/PEREZ Daniel Paul**, BEDFORD - MA, USA

Titre : FSH Society's 2012 biennial Fascioscapulohumeral Muscular Dystrophy (FSHD) International Patient Network (IPRN) Meeting, Atlanta, Georgia, USA, 30th June 2012 - 1st July 2012

**FSH SOCIETY/PEREZ Daniel Paul**, BEDFORD - MA, USA

Titre : Annual International Research Consortium and Research Planning workshop for FSHD, November 6th, 2012, San Francisco, California, USA

**JAFFREDO Thierry**, PARIS, France

Titre : 3ème USCI (UPMC Stem cell initiative) Meeting 'the Biology of stem cells", 21-22 Décembre 2012, Paris

**LAING Nigel**, NEDLANDS - Australia

Titre : 17th International Congress of the World Muscle Society, Perth, Western Australia, 9th - 13th October 2012

**MANDEL Jean-Louis/MERIENNE Karine**, ILLKIRCH, France

Titre : 7ème Conférence Internationale sur les Microsatellites Instables associés aux Maladies Humaines, Mont Saint-Odile, 9 - 14 juin 2012

**MARTY Isabelle/MAIRE Pascal**, LA TRONCHE, France

Titre : Xèmes journées annuelles de la Société Française de Myologie et 15ème Colloque Myogénèse, Grenoble, 14 - 16 novembre 2012

**PAVLATH Grace K.**, ATLANTA - GE, USA

Titre : FASEB Research Conferences - Skeletal muscle satellite cells and stem cells, in Lucca, Italy, 12th - 18th August 2012

**PRIP-BUUS Carina**, PARIS, France

Titre : Meet-ochondrie, Soustons, Septembre 2012

**RAJAS Fabienne**, LYON, France

Titre : Symposium International sur les Glycogénoses, Lyon, 4 - 6 avril 2012

**RENAULT Daniel**, PARIS, France

Titre : 7ème Conférence Internationale sur la Cystinose, Paris, 29 juin - 1er juillet 2012

**SWEENEY Lee H.**, PHILADELPHIA-PE, USA

Titre : The New directions in Biology and Disease of skeletal Muscle", 17th - 20th June 2012, New Orleans, USA

**TADIE Marc**, PARIS, France

Titre : Congrès Scientifique IRME "Avancées dans les traumatismes de la moelle épinière du laboratoire aux applications cliniques", 1er février 2013, Institut de Myologie, Paris

**TAILLANDIER Daniel**, ST GENES CHAMPANELLE, France

Titre : 6ème Colloque Protéolyse Cellulaire de la SFBBM, Clermont-Ferrand, 28 - 30 novembre 2012

**VANDENDRIESSCHE Thierry**, BRUSSELS, Belgium

Titre : FASEB Summer Research Conferences - Genome Engineering - Research & Applications, Lucca, Italy, 2 - 7 septembre 2012

**YANIV Moshe**, PARIS, France

Titre : Colloque "Allosteric Interactions and Biological Regulations", Institut Pasteur, Paris, 14 - 17 mai 2013

### **J/Plateformes non stratégiques**

**CEDS/GRMD**, MEZILLES, France

Projet : Financement 2012 CEDS