



Composition of AFM-Telethon's 10th Scientific Council

Permanent Committee

The Permanent Committee of the Scientific Council addresses major transversal and strategic issues. It is the advisory body of the AFM-Telethon Board of Directors in the implementation of the scientific policy and the Association's strategy.

Melki Judith, MD, PhD

- **President.**
- Professor of Medical Genetics, Medicine Faculty, Inserm, University of Paris Saclay, Centre Hospitalier Sud-Francilien, Kremlin-Bicetre Hospital (France).

Fardeau Michel, MD, PhD

- **Honorary President.**
- University Professor, Professor of Neurology, La Pitié-Salpêtrière Hospital, Paris (France).

Gillis Jean-Marie, MD, PhD

- **Honorary President.**
- Professor Emeritus, Catholic University of Leuven (Belgium).

Fischbeck Kenneth H., MD, PhD

- **Vice-President.**
- Professor of Neurology, Chief of the Neurogenetics Branch, National Institute of Health, Bethesda (USA).

Lehn Pierre, MD, PhD

- **Vice-President.**
- Professor Emeritus, Faculty of Medicine, "Université de Bretagne Occidentale", Brest (France).

Tajbakhsh Shahragim, PhD

- **Vice-President.**
- CNRS Research Director, "Stem Cells and Development" Labex coordinator, Institut Pasteur, Paris (France).

Taylor Naomi, MD, PhD

- **Vice-President.**
- Inserm Research Director, Head of the Hematopoiesis and Immunotherapy Research Group, Institute of Molecular Genetics of Montpellier (IGMM) (France).
- Head of the Section "Basic to Translational Oncology", National Cancer Institute, Bethesda (USA).

Butler-Browne Gillian, PhD

- **General Secretary.**
- Inserm Emeritus Research Director, Center of Research in Myology, Myology Institute, Paris (France).

Attarian Shahram, MD, PhD

- **Advisor.**
- University Professor, Professor of Neurology, Head of the Reference Center for Neuromuscular Diseases and ALS, Timone Hospital, Marseille (France).

Vilquin Jean-Thomas, PharmD, PhD

- **Advisor.**
- CNRS Research Director, Stem Cells and Cell Therapy Group, Institute of Myology, Paris (France).

Other members of the Permanent Committee:

- ***The Presidents of the Call for Proposals Commissions.***
- ***The Presidents of the Strategic and Therapeutic Development Committees (COSETs).***

Call for Proposals Commissions

The Scientific Council is organized into 8 thematic commissions. The projects submitted to the AFM-Telethon in the framework of the Call for Proposals are selected by the corresponding commissions, based on their scientific quality and their relevance to our strategy.

"Molecular & Physiopathological Basis of Muscular Dystrophies" Commission

Aartsma-Rus Annemieke, PhD

- ***President of the Commission.***
- Professor of Translational Genetics, Department of Human Genetics, Leiden University (The Netherlands).

Bendahan David, PhD

- CNRS Research Director, Head of the Team "Musculoskeletal System", Center for Magnetic Resonance in Biology and Medicine (CRMBM), Marseille (France).

Claeys Kristl, MD, PhD

- Head of the laboratory for Muscle Diseases and Neuropathies, Neurologist, Department of Neurosciences, KU Leuven (Belgium).

De Greef Jessica, PhD

- Senior Researcher, Department of Human Genetics, Leiden University Medical Center (The Netherlands).

Flanigan Kevin, MD, PhD

- Director of the Center for Gene Therapy, Nationwide Children's Hospital, Columbus (USA).

Patel Ketan, PhD

- Professor of Developmental Biology and Physiology, Head of Molecular and Cellular Medicine, University of Reading (United Kingdom).

Schara-Schmidt Ulrike, MD, PhD

- Professor for Neuropaediatrics and Neuromuscular Diseases, Head of the Department of Neuropaediatrics, Head of the Centre for Neuromuscular Diseases in Children and Adolescents, University Hospital Essen (Germany).

Sweeney Lee H., PhD

- Professor in the Department of Pharmacology and Therapeutics, Director of the Myology Institute, University of Florida (USA).

Topaloglu Haluk, MD, PhD

- Professor of Pediatrics and Neurology, Hacettepe University School of Medicine, Ankara (Turkey).

Tuffery-Giraud Sylvie, PhD

- Research Engineer, Laboratory for Genetics of Rare Diseases, University Institute of Clinical Research, Montpellier (France).

Wahbi Karim, MD, PhD

- Associated Professor of Cardiology, Cardiology Department, Cochin Hospital, Paris (France).

"Molecular & Physiopathological Basis of Other Neuromuscular Diseases" Commission

Procaccio Vincent, MD, PhD

- ***President of the Commission.***
- University Professor, Professor of Medical Genetics, Leader of the Team "Mitochondrial Genetics and Therapeutics", Director of the Clinical Molecular Diagnostic laboratory, Angers (France).

Allard Bruno, PhD

- University Professor, NeuroMyoGène Institute (INMG), Claude Bernard Lyon 1 University, Villeurbanne (France).

Allenbach Yves, MD, PhD

- Associate Professor, Department of Internal Medicine and Clinical Immunology, Myology Research Center, Pitié-Salpêtrière University Hospital, Paris (France).

Bendahhou Saïd, PhD

- CNRS Research Director, Molecular PhysioMedicine Laboratory, Nice Sophia Antipolis University, Nice (France).

Böhm Johann, PhD

- Associate Professor, Inserm Research Officer, Department of Translational Medicine and Neurogenetics, Institute of Genetics and Molecular and Cellular Biology (IGBMC), Illkirch (France).

Gitiaux Cyril, PhD

- Pediatric neurologist, Pediatric clinical neurophysiology, Reference Center for Neuromuscular Diseases, Necker-Enfants Malades University Hospital, Paris (France).

Marty Isabelle, PhD

- Inserm Leader of the Team "Cellular Myology and Pathologies", Grenoble-Institute Neurosciences, Grenoble (France).

Prigione Alessandro, MD, PhD

- Professor of Pediatric Metabolic Medicine, Department of General Pediatrics, Neonatology and Pediatric Cardiology, University Clinic Düsseldorf (Germany).

Viscomi Carlo, PhD

- Associate Professor, Department of Biomedical Sciences, University of Padova (Italy).

"Stem Cells" Commission

Morgan Jennifer E., PhD

- **President of the Commission.**
- Professor of Cell Biology, Dubowitz Neuromuscular Centre, Institute of Child Health, University College of London (United Kingdom).

Agbulut Onnik, PhD

- Group leader of the research Team "Stem Cells, Cardiovascular Pathophysiology and Biotherapies", Department of Biological Adaptation and Ageing, Institute of Biology Paris-Seine, Paris (France).

Chapel Alain, PhD

- Research Officer, Institute for Radiological Protection and Nuclear Safety (IRSN), Fontenay-aux-Roses (France).

Chazaud Bénédicte, PhD

- Inserm Research Director, Head of the Team "Stem Cell Environment and Skeletal Muscle Homeostasis, Stem Cells and Glioma", NeuroMyoGène Institute (INMG), Lyon (France).

Jaconi Marisa, PhD

- Principal Investigator with Faculty position, Faculty of Medicine, Department of Basic Neurosciences, University of Geneva (Switzerland).

Malgrange Brigitte, PharmD, PhD

- Research Director, GIGA-Neurosciences, Developmental Neurobiology Unit, University of Liège (Belgium).

Martinat Cécile, PhD

- Inserm Research Director, Institute for Stem Cell Therapy and Exploration of Monogenic Diseases (I-Stem), Corbeil-Essonnes (France).

Peault Bruno, PhD

- Group leader of the Team "Tissue Development, Regeneration and Repair research", University of Edinburgh (United Kingdom).
- Professor at UCLA, Los Angeles (USA).

Sampaolesi Maurilio, PhD

- Professor of Medicine, Head of the Translational Cardiomyology Laboratory, Stem Cell Research Institute, Catholic University of Leuven (Belgium).

Vilquin Jean-Thomas, PharmD, PhD

- CNRS Research Director, Stem Cells and Cell Therapy Group, Institute of Myology, Paris (France).

"Fundamental Myology" Commission

Munoz-Canoves Pura, PhD

- **President of the Commission.**
- Professor of Cell Biology, Altos Labs, Inc., Institute of Science of San Diego (USA).

Amthor Helge, MD, PhD

- University Professor, Professor of Pediatrics, Raymond Poincaré Hospital (Garches) & Versailles-Saint-Quentin-en-Yvelines University (France).

Borycki Anne-Gaëlle, PhD

- Senior Lecturer, Department of Biomedical Science, University of Sheffield (United Kingdom).

Gomes Edgar, PhD

- Associate Professor, Unit Director, Institute of Molecular Medicine (IMM), Lisbon (Portugal).

Jagla Krzysztof, PhD

- Inserm Research Director, Team Leader, Laboratory of Genetics, Reproduction and Development (GReD), University of Clermont Auvergne, Clermont-Ferrand (France).

Kelly Robert G., PhD

- Inserm Research Director, Head of Team "Genetic Control of Heart Development", Developmental Biology Institute of Marseille (IBDM), Marseille (France).

Krüger Marcus, PhD

- Full Professor, CECAD Research Center, Leader of Research Platform, Institute for Genetics, University of Cologne (Germany).

Maire Pascal, PhD

- Inserm Research Director, Department of Neuromuscular Development, Genetics and Physiopathology, Cochin Institute, Paris (France).

Philippou Mourikis, PhD

- CNRS Research Director, Group leader of the research Team "Muscle Stem Cells-Niche interactions", Mondor Institute of Biomedical Research (IMRB), Créteil (France).

Schiaffino Stefano, MD, PhD

- Professor Emeritus in General Pathology, Vice-President of the Venetian Institute of Molecular Medicine, University of Padua (Italy).

"Nervous System (Motoneuron & Neuromuscular Junction)" Commission

Kalb Robert G., MD, PhD

- ***President of the Commission.***
- Professor of Neurology, Director of Les Turner ALS Center, Division Chief of Neuromuscular Diseases, Department of Neurology, Northwestern University, Chicago (USA).

Bolino Alessandra, PhD

- Head of the Human Inherited Neuropathies Unit, Division of Neuroscience, San Raffaele Scientific Institute, INSPE, Milan (Italy).

Fallon Justin, PhD

- Professor of Neuroscience, Co-Director of the Center for the Neurobiology of Cells and Synapses, Department of Neuroscience, Brown University, Providence (USA).

Funalot Benoît, MD, PhD

- Professor of Genetics, Head of the Department of Genetics, Henri Mondor Hospital, "Assistance Publique-Hôpitaux de Paris" (APHP), Créteil (France).

Herbst Ruth, PhD

- Associate Professor, Medical University of Vienna, Institute of Specific Prophylaxis & Tropical Medicine, Center for Pathophysiology, Infectiology and Immunology, Vienna (Austria).

Julien Jean-Pierre, PhD

- Professor, Department of Psychiatry and Neuroscience, CERVO Brain Research Centre, University of Laval, Quebec (Canada).

Lefebvre Suzie, PhD

- Inserm Research Officer, T3S Unit (Environmental Toxicity, Therapeutic Targets, Cellular Signaling and Biomarkers), Degeneration and Plasticity of the Locomotor System Team, Centre Universitaire des Saints-Pères, Paris (France).

Monani Umrao R., PhD

- Associate Professor of Pathology and Cell Biology (in Neurology), Columbia University, New York (USA).

Pareyson Davide, MD, PhD

- Professor of Neurology, Head of the Functional Department on Rare Neurological Diseases, Chief of the Unit of Clinic of Central and Peripheral Degenerative Neuropathies, Foundation of the Carlo Besta Neurological Institute, IRCCS, Milan (Italy).

Perlson Eran, MD, PhD

- Department of Physiology and Pharmacology, Sackler Faculty of Medicine, Tel Aviv University (Israel).

Zeviani Massimo, MD, PhD

- Scientific Director, Professor of Neurology, Institute for Maternal and Child Health, IRCCS "Burlo Garofolo", Trieste (Italy).

"Gene and/or Cell Therapy of Rare Disorders" Commission

Büning Hildegard, PhD

- ***President of the Commission.***
- Head of the Laboratory for Infection Biology & Gene Transfer, Institute of Experimental Hematology, Hannover Medical School (Germany).

Cathomen Toni, PhD

- Professor of Cell and Gene Therapy, Director of the Institute for Transfusion Medicine and Gene Therapy, University of Freiburg (Germany).

Ertl Hildegund C.J., MD

- Professor, Chair of the Immunology Department, Director of the Vaccine & Immunotherapy Center, The Wistar Institute, Philadelphia (USA).

Malerba Alberto, PhD

- Lecturer in Gene Therapy, Department of Biological Sciences, Royal Holloway, University of London (United Kingdom).

Dalkara Deniz, PhD

- Group Leader, Gene Therapies and Animal Models for Neurodegenerative Diseases (ERC-Inserm), Department of Therapeutics, The Vision Institute, Paris (France).

Mingozzi Federico, PhD

- Chief Scientific Officer at Spark Therapeutics Inc., Philadelphia (USA).

Montier Tristan, MD, PhD

- Professor of Cell Biology, Inserm Research Teacher, Head of the Team "Gene Transfer and Gene Therapy", "Université de Bretagne Occidentale", Brest (France).

Mouly Vincent, PhD

- Inserm Research Director, Head of the Team "Regeneration, Physiopathology and Therapeutic Approaches: Cellular Models Research", Institute of Myology, Paris (France).

Raoul Cédric, PhD

- Inserm Research Director, Head of the Research Team "Motoneuron Disease: Neuroinflammation and Therapy", Saint-Eloi Hospital, Institute for Neurosciences of Montpellier (INM) (France).

Zimmermann Valérie, PhD

- CNRS Research Officer, Hematopoiesis and Immunotherapy Research Group, Institute of Molecular Genetics of Montpellier (IGMM) (France).

"Pharmacological Therapy & Translational Research of Neuromuscular Diseases" Commission

Callizot Noelle, PharmD, PhD

- **Co-President of the Commission.**
- Neuro-Pharmacologist, Neuro-SYS, Aix-en-Provence (France).

Haiech Jacques, MD, PhD

- **Co-President of the Commission.**
- University Professor, Member of the Chemistry – Integrative Biology Group, Strasbourg (France).

Bowser Robert, PhD

- Professor of Neurobiology, Barrow Neurological Institute, Founder and President of Iron Horse Diagnostics, Inc., Phoenix (USA).

Cougoule Céline, PhD

- Research Associate, team "Immune Detection and Elimination of Pathogens", Institute of Pharmacology and Structural Biology (IPBS), CNRS, Toulouse (France).

Cox Gregory A., PhD

- Associate Professor, The Jackson Laboratory, Bar Harbor (USA).

Galzi Jean-Luc, PhD

- CNRS Research Director, Biotechnology and Cellular Signalling, Strasbourg School of Biotechnology (ESBS), University of Strasbourg, Illkirch (France).

Howson Patrick, PhD

- Chief Executive Officer of Junaxo Inc., Head of Medicinal Chemistry at Atuka Inc., Scientific Associate, University Health Network, Toronto (Canada).

Levy Nicolas, MD, PhD

- Professor of Medical Genetics, Scientific Head for Rare Diseases, Servier, Laboratory, Suresnes (France).

Niks Erik H., MD, PhD

- Consultant in Neurology and Pediatric Neurology, Myologist, Department of Neurology, University of Leiden (The Netherlands).

Scherman Daniel, PhD

- CNRS Research Director, Director of the Chemical and Biological Technologies for Health Unit (UTCBS), Director of the "Centre de Recherche Pharmaceutique de Paris" (France).

"Medical Research" Commission

Klein Andrea, MD, PhD

- **President of the Commission.**
- Senior Neuropediatrics, Department of Neuropediatrics, University Hospital of Bern (Switzerland).

Attarian Shahram, MD, PhD

- University Professor, Professor of Neurology, Head of the Reference Center for Neuromuscular Diseases and ALS, Timone Hospital, Marseille (France).

Bernert Günther, MD

- Head of Department of Child and Adolescent Medicine, Preyer Children's Hospital, Vienna (Austria).

Claeys Kristl, MD, PhD

- Head of the laboratory for Muscle Diseases and Neuropathies, Neurologist, Department of Neurosciences, KU Leuven (Belgium).

De Visser Marianne, MD

- Professor of Neuromuscular Diseases, Amsterdam University Medical Centre, Amsterdam (The Netherlands).

Laugel Vincent, MD, PhD

- University Professor, Professor of Pediatrics and Neurology, Department of Pediatrics, Strasbourg Hospital (France).

Solé Guilhem, MD

- Neurologist, Head of the Reference Center for Neuromuscular Diseases, Bordeaux University Hospital (France).

Strategic and Therapeutic Development Committees (COSETs)

The Strategic and Therapeutic Development Committees (COSETs) monitor projects that extend over several years and represent important investments. A specific COSET follows each strategic project, providing advice and coaching to it. The presidents of these COSETs are members of the Permanent Committee of the Scientific Council.

Aartsma-Rus Annemieke, PhD

- ***President of the COSET "Tricyclo-DNA antisense oligonucleotide treatment for Myotonic Dystrophy".***
- Professor of Translational Genetics, Department of Human Genetics, Leiden University (The Netherlands).

Büning Hildegard, PhD

- ***President of the COSET "Therapy for Giant Axonal Neuropathy".***
- ***President of the COSET "Overexpression of the neuroglobin gene for the treatment of various neurological diseases"***
- Head of the Laboratory for Infection Biology & Gene Transfer, Institute of Experimental Hematology, Hannover Medical School (Germany).

Butler-Browne Gillian, PhD

- ***President of the COSET "Pathophysiology and therapeutic proof-of-concepts for congenital myopathies".***
- Inserm Emeritus Research Director, Center of Research in Myology, Myology Institute, Paris (France).

Desnuelle Claude, MD, PhD

- ***President of the COSET "DUX4 in FSHD: pathophysiology and therapeutic approaches".***
- Professor of Neurology, Hospital Doctor, Nice Hospital (France).

Funalot Benoît, MD, PhD

- ***President of the COSET "Identifying candidate drugs in mitochondrial cardiomyopathies From Mouse to Human".***
- Professor of Genetics, Head of the Department of Genetics, Henri Mondor Hospital, "Assistance Publique-Hôpitaux de Paris" (APHP), Créteil (France).

Maire Pascal, PhD

- ***President of the COSET "Toward cell therapy for Duchenne Muscular Dystrophy: characterization of regenerative potential of hIPS-derived Pax7+ cells".***
- Inserm Research Director, Department of Neuromuscular Development, Genetics and Physiopathology, Cochin Institute, Paris (France).

Montier Tristan, MD, PhD

- ***President of the COSET "DUX4 in FSHD: pathophysiology and therapeutic approaches".***
- Professor of Cell Biology, Inserm Research Teacher, Head of the Team "Gene Transfer and Gene Therapy", "Université de Bretagne Occidentale", Brest (France).

Sampaolesi Maurilio, PhD

- ***President of the COSET "Innovative bio-inspired cell and tissue models of genetic dilated cardiomyopathy for disease modelling and drug discovery".***
- Professor of Medicine, Head of the Translational Cardiomyology Laboratory, Stem Cell Research Institute, Catholic University of Leuven (Belgium).

Tuffery-Giraud Sylvie, PhD

- ***President of the COSET "Pre-clinical development of peptide-oligonucleotides for DM1".***
- Research Engineer, Laboratory for Genetics of Rare Diseases, University Institute of Clinical Research, Montpellier (France).