

6<sup>th</sup> international  
congress  
of myology

AFMTELETHON   
CURE THROUGH INNOVATION

# myology

march 25-28 2019

Presidents:

**Odile Boespflug-Tanguy**

**Francesco Muntoni**

**BORDEAUX convention centre**  
**FRANCE**

© Généthon 2018



programme

6<sup>th</sup> international  
congress  
of myology

# myology

# 2019

march 25-28

**BORDEAUX convention centre**  
**FRANCE**

## Sponsors

Platinum sponsors



Gold sponsors



Silver sponsors



Autres



## Coordination AFM-Téléthon

Direction de la communication

Anne-Sophie GONON

Emmanuelle GUIRAUD

Mireille LAZNOWSKI

Laurence LESPINARD

Sabine MAILLET-CROZET



## Presidents

### **Odile BŒSPFLUG-TANGUY**

Robert Debré Hospital/Inserm - Paris - France  
President of AFM-Telethon Scientific Council

### **Francesco MUNTONI**

Director of The Dubowitz Neuromuscular Centre,  
Great Ormond Street Hospital - London - UK

## Scientific committee

### **Coordinator**

J. Andoni URTIZBEREA (Hendaye - France)

### **Members**

Valérie ALLAMAND (Paris - France)  
Enrico BERTINI (Roma - Italy)  
Serge BRAUN, (Evry-Courcouronnes - France)  
Bénédicte CHAZAUD (Lyon - France)  
Bertrand FONTAINE (Paris - France)  
Jean-Marie GILLIS (Brussels - Belgium)  
Nathalie GOEMANS (Leuven - Belgium)  
Cecilia JIMENEZ-MALLABRERA (Barcelona - Spain)  
Guilhem SOLÉ (Bordeaux - France)  
Shahragim TAJBAKSH (Paris - France)

# myology

## Éditos 2019



**Laurence TIENNOT-HERMENT,**  
President of AFM-Téléthon

Our association was founded 60 years ago in the corridors of Prof Debré's laboratory; he was one of the only people in France working on what were then called myopathies. Families were isolated, helpless in the face of the inadequacy of medicine and research. So nine of them decided to declare war on the disease and take the fate of their children into their own hands. The Association Française pour la Myopathie (French Muscular Dystrophy Association) was formed and, generation after generation, we have driven research, gathered skills, joined forces, built laboratories, invented a new partnership model between patients, researchers and doctors. We've fought unceasingly with a single objective: to conquer the disease!

Sixty years later, we have really changed the world! The medical treatment has taken a leap forward and, with it, so have patients' quality of life and life expectancy. **We can rely on a powerful international research movement that has brought myology to the forefront of therapeutic innovation.** The number of clinical trials is increasing and hundreds of patients are taking part across the world. And finally, the first innovative therapy drugs are arriving! They're not content to slow down development of the disease but

are reversing its progress! And it's children who are regaining movement they had lost, who can sit down by themselves without a corset, stand up and sometimes even...take their first steps! **We know, these very first victories turn a new page of our story, in which Hopefulness rhymes with Success.**

We are more determined than ever to continue the fight. Industrialisation of innovative biotherapies, treatment of the whole muscle mass, access for all patients to innovative treatments, drug prices... today's challenges are no less complex than yesterday's. But they confirm one thing: a major therapeutic revolution is in progress. In this new stage, we know that we can rely on support from the global myology community, which came together for these 4 days in Bordeaux!

**I express the wish that, during this edition of Myology 2019, together we can all share major new advances that will all lead to new Successes tomorrow.** A big Thank You for taking part in our big international Myology meeting.



**Odile BŒSPFLUG-TANGUY,**  
President of Myology 2019

#### Welcome to Myology 2019

As president of the scientific committee of the AFM-Téléthon it is with great pleasure that I welcome you to the Bordeaux convention centre for the 6<sup>th</sup> International Congress of Myology.

Bordeaux is not only a fantastic town in the middle of the most famous French vineyard, but also a very active University particularly in neurosciences with a growing interest in neuromuscular disorders.

Since the last edition of the congress in Lyon, **the results obtained by different clinical trials using innovative therapies has demonstrated the feasibility to treat "untreatable diseases"**.

The development of treatments must continue and accelerate by federating researchers, doctors and patients. I am convinced that Myology 2019 will participate to this fantastic effort by challenging the best specialists from the five continents in fundamental research as well as in clinical science and therapeutics.

At Myology 2019 with 4 intense day program, more than 60 international speakers and more than 400 posters presentations, **excellence will be at the rendezvous for the patients!**



**Francesco MUNTONI,**  
President of Myology 2019

It is my great pleasure to invite you to the 6<sup>th</sup> edition of this international AFM Congress of Myology which will be held in Bordeaux, one the greatest city of France.

**This year programme celebrates the discoveries and new advances across a very wide fields of basic and clinical research**, providing education and stimulation to colleagues across multiple professional backgrounds. Advances in the identification of novel genes and improved understanding of pathophysiology of "old" disease genes using state of the art cellular and animal models will be presented alongside the very rapid developments in the field of biomarkers and of novel therapies, with the consolidation of the genetic therapies across a number of different disease fields. Dedicated sessions will also address comorbidities affecting brain, the eye and the heart, which represent important aspects of many neuromuscular diseases. Internationally renewed keynote speakers and junior investigators, selected for the novelty of their work by an international panel, will present the latest developments during the 4 days meeting, and this will be complemented by more than 400 posters. **This latest AFM congress of Myology 2019 will be a vibrant meeting that continue the excellent track records of the previous Myology editions.** I look forward meeting you in Bordeaux.



## innover pour guérir

L'AFM-Téléthon vient de célébrer ses 60 ans. Six décennies de combat sans relâche mené contre la maladie par des générations de malades et parents de malades mobilisés par un objectif : guérir les maladies neuromusculaires. Six décennies de conquêtes marquées aujourd'hui par la multiplication des essais à travers le monde et par l'arrivée des premiers médicaments de thérapie innovante.

**L'impossible devient possible !** Avec ce tournant majeur qui concrétise des années de recherche impulsées par l'AFM-Téléthon, c'est plus déterminé que jamais que nous abordons cette nouvelle ère et ses défis, en continuant à privilégier l'audace et l'innovation, l'efficacité thérapeutique et l'accès au médicament à un prix juste et maîtrisé.

Pour accélérer la mise au point des thérapies innovantes, l'AFM-Téléthon s'est dotée d'une force de frappe unique :

- ➔ **3 laboratoires à la pointe** des thérapies innovantes, regroupés au sein de l'Institut des Biothérapies des Maladies Rares :
  - l'**Institut de Myologie** dédié au muscle et à ses maladies, avec notamment I-Motion, sa plateforme d'essais cliniques pédiatriques
  - **Généthon** dédié à la thérapie génique des maladies rares
  - **I-STEM** dédié à la recherche sur les cellules souches et leurs applications thérapeutiques
- ➔ environ **250 programmes** de recherche et jeunes chercheurs financés chaque année

➔ un soutien à **36 essais cliniques** en cours ou en préparation pour des maladies du muscle, de la vision, du cerveau, du cœur, de la peau, du foie, du sang...

➔ un **fonds d'amorçage** « biothérapies innovantes et maladies rares » créé avec Bpifrance

➔ Yposkesi, **une plateforme industrielle** créée avec Bpifrance et dédiée au développement et à la production de médicaments de thérapie innovante

Dans le cadre de sa stratégie pour « **Guérir** », l'AFM-Téléthon s'appuie sur un conseil scientifique composé de **84 scientifiques, médecins et experts internationaux et un réseau mondial de 6000 experts**. Il est présidé par le Pr Odile Boespflug-Tanguy, généticienne, directrice de recherche Inserm et Chef du service de neuro-pédiatrie (hôpital Robert Debré, Paris).

Au-delà des appels à projets annuel et des appels d'offres spécifiques (comme par exemple l'appel d'offres « Technologies de rupture en bioproduction »), l'AFM-Téléthon et l'Institut des Biothérapies des Maladies Rares apportent leur soutien à de nombreux projets stratégiques labellisés. Depuis la preuve de concept jusqu'à la mise en place des protocoles cliniques, des spécialistes de la recherche fondamentale et préclinique, des experts du développement clinique et des affaires réglementaires peuvent vous aider tout au long de tels projets.

➔ **Vous avez un projet, l'AFM-Téléthon peut vous accompagner.**  
**Contact : [spcsafm@afm-telethon.fr](mailto:spcsafm@afm-telethon.fr)**

# cure through innovation

The AFM-Téléthon has just celebrated 60 years. Six decades of fighting continuously against the disease by generations of patients and their parents driven by one objective: to cure neuromuscular diseases. Six decades of victories, marked today by trials multiplying across the world and by the arrival of the first innovative therapy drugs.

**The impossible comes possible!** With this major turning-point that represents years of research driven by the AFM-Téléthon, it is more determined than ever that we tackle this new era and its challenges, by continuing to favour daring and innovation, therapeutic efficacy and access to the drug at a fair and controlled price.

To accelerate the development of innovative therapies, the AFM-Téléthon has acquired a unique strike force:

⇒ **3 laboratories at the forefront** of innovative therapies, combined within the Rare Diseases Biotherapies Institute:

- the **Institute of Myology** dedicated to muscles and their diseases, in particular with I-Motion, its paediatric clinical trials platform

- **Genethon** dedicated to gene therapy for rare diseases

- **I-Stem**, dedicated to research on stem cells and their therapeutic applications

⇒ **250 research programmes**

and young researchers financed every year

⇒ support to **36 current and upcoming clinical trials** for diseases of muscles, vision, brain, heart, skin, liver, blood, etc.

⇒ an 'innovative biotherapies and rare diseases' **fund** created with Bpifrance




⇒ Yposkesi, **an industrial platform** created with Bpifrance and dedicated to developing and producing innovative therapy drugs

As part of its strategy for 'Cure', AFM-Téléthon relies on its Scientific Council, comprising **84 international scientists, doctors and experts and a global network of 6,000 experts**. It is chaired by Prof Odile Boespflug-Tanguy, geneticist, Inserm research director and Head of the Neuropaediatrics department (Robert Debré Hospital, Paris).

Beyond annual calls for projects and specific calls for tender (such as the call for tender 'Breakthrough technologies in bioproduction'), the AFM-Téléthon and the Institut des Biothérapies des Maladies Rares offer their support to numerous accredited strategic projects. From proof of concept to implementing clinical protocols, specialists in basic and preclinical research, experts in clinical development and regulatory affairs can support you throughout such projects.

⇒ **If you have a project, the AFM-Téléthon can support you.**  
**Contact: [spcsafm@afm-telethon.fr](mailto:spcsafm@afm-telethon.fr)**

# programme at a glance

	<i>Morning</i>	<i>Noon</i>	<i>Afternoon</i>	<i>Evening</i>
<b>Monday</b> March 25th			17:30-18:45 • Welcome ceremony • Opening lecture	 19:00-20:30 Welcome cocktail
<b>Tuesday</b> March 26th	 9:00-10:30 Plenary session 11:00-12:30 Parallel symposia	12:30-14:00 • Lunch & exhibition • AveXis sponsored symposium	14:00-15:00 Young investigator symposium  15:00-16:00 Plenary session 16:30-18:00 Parallel symposia	18:00-20:00 Sarepta sponsored symposium
<b>Wednesday</b> March 27th	 9:00-10:30 Plenary session 11:00-12:30 Parallel symposia	12:30-14:30 • Poster lunch 12:45-14:30 • Biogen sponsored symposium	 14:30-16:00 Plenary session 16:30-18:00 Parallel symposia	18:00-19:30 Akcea sponsored symposium  20:30 Gala dinner
<b>Thursday</b> March 28th	 9:00-10:30 Plenary session 11:00-12:30 Parallel symposia	12:30-14:00 • Lunch & exhibition • Roche sponsored symposium	14:00-15:30 Parallel symposia 16:00-17:00 Late breaking news 17:00-17:30 Closing ceremony	



# programme

Monday, March 25th · afternoon ·

14:00-17:30

*Registration & setting up of posters*

17:30-18:00

*Welcome ceremony*

AMPHI A

18:00-18:45

*Opening lecture*

AMPHI A

From soil to brain, journey in a glass of wine

Axel MARCHAL

Lecturer in Oenology, Institute of Vine and Wine Sciences, University of Bordeaux

19:00-20:30

*Welcome cocktail*



PLURIEL ROOM

# Tuesday, March 26th - morning -

9:00-10:30



*Plenary session*

AMPHI A

## • Transational Myology

Chairpersons: Odile BOESPFLUG-TANGUY (Paris - France), Pascal MAIRE (Paris - France)

Molecular mechanisms regulating muscle satellite cell function

Frédéric RELAX (Creteil - France)

Approaches to delay the progression of Muscular Dystrophy

Graziella MESSINA (Milano - Italy)

Expression and functional analyses of Dlk1 in muscle stem cells and mesenchymal progenitors during regeneration

So-Ichiro FUKADA (Osaka - Japan)

10:30-11:00

Coffee break and exhibition



PLURIEL ROOM

11:00-12:30

*Parallel symposia*

## • Congenital Myopathies

Chairwoman: Ana FERREIRO (Paris - France)

Modulation of amphiphysin and dynamin rescues severe congenital myopathies

Jocelyn LAPORTE (Illkirch - France)

The expanding phenotypical spectrum of RYR1-related neuromuscular disorders

Heinz JUNGBLUTH (London - UK)

Sarcomere contractility in nemaline myopathy

Coen Ac OTTENHEIJM

(Amsterdam - The Netherlands)

AMPHI A

## • Cardiomyology

Chairman: Denis DUBOC (Paris - France)

High Risk of Fatal and Non-Fatal Venous Thromboembolism in Myotonic Dystrophy

Karim WAHBI (Paris - France)

Altered cytoskeleton in cardiac disease caused by nuclear A-type lamins gene mutations

Antoine MUCHIR (Paris - France)

The DMD Heart Protection Trial: A double blind randomised, placebo-controlled, multi-centre trial of combined ACE-inhibitor and beta-blocker therapy in preventing the development of cardiomyopathy in genetically characterised males with DMD without echo-detectable left ventricular dysfunction

John BOURKE (Newcastle - UK)

AMPHI B

12:30-14:00

Free time for lunch and exhibition



PLURIEL ROOM

*Industry symposium: 12:30-14:00*

> AveXis

Gene therapy in neuromuscular diseases:  
a journey from bench to bedside

AMPHI C

14:00-15:00

*Symposium*

AMPHI A

## • Young investigators symposium

Chairman: Serge BRAUN (Evry-Courcouronnes - France)

Small non-coding RNAs of intron origin in Myotonic Dystrophy type 1: new candidate drivers of splicing defects  
Baptiste BOGARD (Paris - France)

Downregulation of Phosphodiesterase 10A mitigates the manifestation of DMD phenotype in zebrafish model  
Matthias LAMBERT (Boston - USA)

Using Human Pluripotent Stem Cells Derived Motor Neurons to address the Pathogenesis of Spinal Muscular Atrophy  
Camille JANUEL (Corbeil-Essonnes - France)

Genetic control of skeletal muscle fiber type  
Matthieu DOS SANTOS (Paris - France)

15:00-16:00



*Plenary session*

AMPHI A

## • Outcome Measures and Biomarkers

Chairpersons: Nathalie GOEMANS (Leuven - Belgium), Volker STRAUB (Newcastle - UK)

Quantitative muscle MRI as a reliable outcome measure  
Jordi DIAZ-MANERA (Barcelona - Spain)

Identification of prognostic and pharmacodynamic serum biomarkers in Duchenne and Becker muscular dystrophies  
Pietro SPITALI (Leiden - The Netherlands)

16:00-16:30 Coffee break and exhibition



PLURIEL ROOM

16:30-18:00

*Parallel symposia*

## • Update in muscular dystrophies [LGMD, CMD, DMD]

Chairman: Bruno EYMARD (Paris - France)

AAV-gene transfer in Limb-girdle muscular dystrophies  
Isabelle RICHARD (Evry-Courcouronnes - France)

Mutation Specific Precision Therapy: The COL6-Related Dystrophies and Beyond  
Carsten BONNEMANN (Bethesda - USA)

Two in vitro muscular dystrophy modelling approaches for development and verification of gene therapy methods  
Ivan YAKOVLEV (Moscow - Russia)

Residual very low dystrophin levels mitigate dystrophinopathy towards Becker muscular dystrophy  
Helge AMTHOR (Montigny-Le-Bretonneux - France)

AMPHI A

## • Muscle & Central Nervous System

Chairman: Alvaro RENDON (Paris - France)

Genotype-phenotype relationships underlying visual, cognitive and neuropsychiatric disorders in Duchenne muscular dystrophy  
Francesco MUNTONI (London - UK)

Neurobehavioral dysfunctions and therapeutic approaches in DMD mouse models lacking brain dystrophins  
Cyrille VAILLEND (Orsay - France)

Electroretinographic anomalies as biomarkers of CNS dysfunction and for evaluation of gene therapy efficacy: Insights from the preclinical study of Dp71-null mice  
Jan KREMERS (Erlangen - Germany)

Defective visual information processing in Duchenne muscular dystrophy  
Dora Fix VENTURA (Sao Paulo - Brazil)

AMPHI B

Industry symposium: 18:00-20:00

> Sarepta

Precision genetic medicines for duchenne muscular dystrophy

AMPHI C

9:00-10:30



Plenary session

AMPHI A

## • Lessons Learned from Negative Trials

Chairmen: Enrico BERTINI (Rome - Italy), François RIVIER (Montpellier - France)

GNE Myopathy Therapy Trial With Sialic Acid Supplementation: Why did phase 3 'fail' ?

Zohar ARGOV (Jerusalem - Israel)

Lessons Learned From Myostatin Trials

Kathryn WAGNER (Baltimore - USA)

Cell therapies & Muscle: an update

Jennifer MORGAN (London - UK)

10:30-11:00 Coffee break and exhibition



PLURIEL ROOM

11:00-12:30

Parallel symposia

## • Motoneuron / NMJ / Membrane

Chairman: Bruno ALLARD (Lyon, Paris)

Regulation of neuromuscular connectivity by Wnt signaling:  
from signaling molecule to therapeutic strategies

Laure STROCHLIC (Paris - France)

An in vivo model for the functional validation of polymorphisms  
in genes required for AChR synthesis

Jean-Louis BESSEREAU (Lyon - France)

Sarcolemmal membrane repair activated by conserved intra-  
cellular signaling responses can compensate for membrane  
fragility in muscular dystrophy

Noah WEISLEDER (Columbus - USA)

AMPHI A

## • Myotonic Dystrophies

Chairwoman: Geneviève GOURDON  
(Paris, France)

Myotonic dystrophies: current core clinical phenotypes  
Giovanni MEOLA (Milano - Italy)

RNA-based approaches to reverse repeat expansion toxicity  
in Myotonic Dystrophy

Denis FURLING (Paris - France)

3D reconstruction of DM1 patients' myoblasts: studying  
the relationship between CTG repeats, RNA foci and MBNL1  
in single cells

Alfonsina BALLESTER-LOPEZ (Badalona - Spain)

AMPHI B

12:30-14:30

Posters lunch



PLURIEL ROOM

Industry symposium: 12:45-14:30

> Biogen

Nusinersen in SMA: 6 years and over  
6,600 patients  
What have we learned? What questions  
remain to be answered?

AMPHI C

14:30-16:00



Plenary session

AMPHI A

## • Gene Therapies

Chairmen: Serge BRAUN (Evry-Courcouronnes - France), Giuseppe RONZITTI (Evry-Courcouronnes - France)

Alternative AAV-based gene therapies for dystrophinopathies

Kevin FLANIGAN (Columbus - USA)

Advancements in AAV-based gene therapy strategies for familial ALS forms

Maria-Grazia BIFERI (Paris - France)

Gene Therapy of Myotubular Myopathy

Anna BUJ-BELLO (Evry-Courcouronnes - France)

16:00-16:30 Coffee break and exhibition



PLURIEL ROOM

16:30-18:00

Parallel symposia

## • Myogenesis & Muscle Ageing

Chairwoman: Gillian BUTLER-BROWNE  
(Paris - France)

Regulation of the mouse skeletal muscle stem cell niche during homeostasis and regeneration

Shahragim TAJBAKSH (Paris - France)

Recovery macrophages secrete pro-fusogenic effectors during skeletal muscle regeneration

Bénédicte CHAZAUD (Lyon - France)

Dynamics of muscle growth, regeneration and hypertrophy provide an essential quantitative basis to understanding muscle stem cell function

Terence PARTRIDGE (Washington - USA)

Attenuation of myostatin/activin signaling delay aging signs in progeric mice model

Khalid ALYODAWI (Reading - UK)

AMPHI A

## • Fighting Diagnostic Odysseys

Chairwoman: Emmanuelle LAGRUE  
(Tours - France)

Targeted therapies for rare neuromuscular disorders – first steps towards a treatable disease

Hanns LOCHMULLER (Ottawa - Canada)

Genetic Landscape of Limb Girdle Muscular Dystrophies

Madhuri HEGDE (Waltham - USA)

Spinal Muscular Atrophy: A challenging disease for Newborn screening?

Laurent SERVAIS (Liege - Belgium)

AMPHI B

20:30 Gala dinner  
Information page 19

Industry symposium: 18:00-19:30

> Akcea

Recent advances in genetic neuropathies

AMPHI C

# Thursday, March 28th - morning -

9:00-10:30



*Plenary session*

AMPHI A

## • Therapies on the Horizon

Chairmen: Jean-Marie GILLIS (Brussels - Belgium), J. Andoni URTIZBEREA (Hendaye - France)

Nucleic acid-based therapies for neuromuscular disease

Matthew WOOD (Oxford - UK)

Tamoxifen as a treatment for muscular diseases: an unexpected facet of a repurposed anticancer drug

Olivier DORCHIES (Geneva - Switzerland)

Vamorolone retains efficacy and reduces safety concerns of glucocorticoid drugs

Eric HOFFMAN (Rockville - USA)

10:30-11:00

Coffee break and exhibition



PLURIEL ROOM

11:00-12:30

*Parallel symposia*

## • FSHD

Chairwoman: Sabrina SACCONI (Nice - France)

Facioscapulohumeral muscular dystrophy

Silvere VAN DER MAAREL (Leiden - The Netherlands)

Applying genome-wide CRISPR screens for therapeutic discovery in FSHD

Angela LEK (New Haven - USA)

PAX7, DUX4 and Facioscapulohumeral muscular dystrophy

Peter ZAMMIT (London - UK)

AMPHI A

## • Glycosylation / Sialisation

Chairwoman: Cecilia JIMENEZ-MALLABRERA (Esplugues de Lobregat - Spain)

Glycosylation and sialylation in congenital muscular diseases: the glycobiologist's point of view

Arnaud BRUNEEL (Paris - France)

Molecular mechanisms involved in MDC1A / LAMA2 MD and development of possible treatment options

Markus RUEGG (Basel - Switzerland)

Genotype-phenotype correlation in GNE myopathy

Oksana POGORYELOVA (Newcastle - UK)

AMPHI B

12:30-14:00

Free time for lunch and exhibition



PLURIEL ROOM

*Industry symposium: 12:30-14:00* > Roche

Are we watching the drop or the ripple it creates: Clinical and practical insights which could shape the future for all SMA patients

AMPHI C

14:00-15:30

Parallel symposia

• **Metabolic Myopathies**

Chairman: Pascal LAFORET (Paris - France)

Investigational liver gene transfer for secretable GAA in the treatment of Pompe disease

Federico MINGOZZI (Evry-Courcouronnes - France)

Exercise training and pathophysiology of exercise in metabolic myopathies

John VISSING (Copenhagen - Denmark)

Thymidine Kinase 2 Deficiency: review of 16 Spanish patients with a late onset form

Carmen PARADAS (Antequera - Spain)

**AMPHI A**

• **Innovative Therapies**

Chairman: Shahram ATTARIAN (Marseille - France)

A decoy trapping DUX4 for the treatment of FacioScapulo-Humeral Muscular Dystrophy

Julie DUMONCEAUX (London - UK)

CRISPR/Cas9 genome editing to generate new zebrafish models of centronuclear myopathy

Éléonore DUPUIS (Brussels - Belgium)

TcDNA-ASO-mediated exon skipping approach for brain dystrophins restoration and compensation of cognitive/behavioral deficits in mouse models of DMD

Faouzi ZARROUKI (Montigny-le-Bretonneux - France)

Gene Therapy For Peripheral Neuropathy CMT1A

Nicolas TRICAUD (Montpellier - France)

**AMPHI B**

15:30-16:00 Coffee break and exhibition



PLURIEL ROOM

16:00-17:00

Late breaking news

**AMPHI A**

Chairman: Bertrand FONTAINE (Paris - France)

17:00-17:30

Closing ceremony

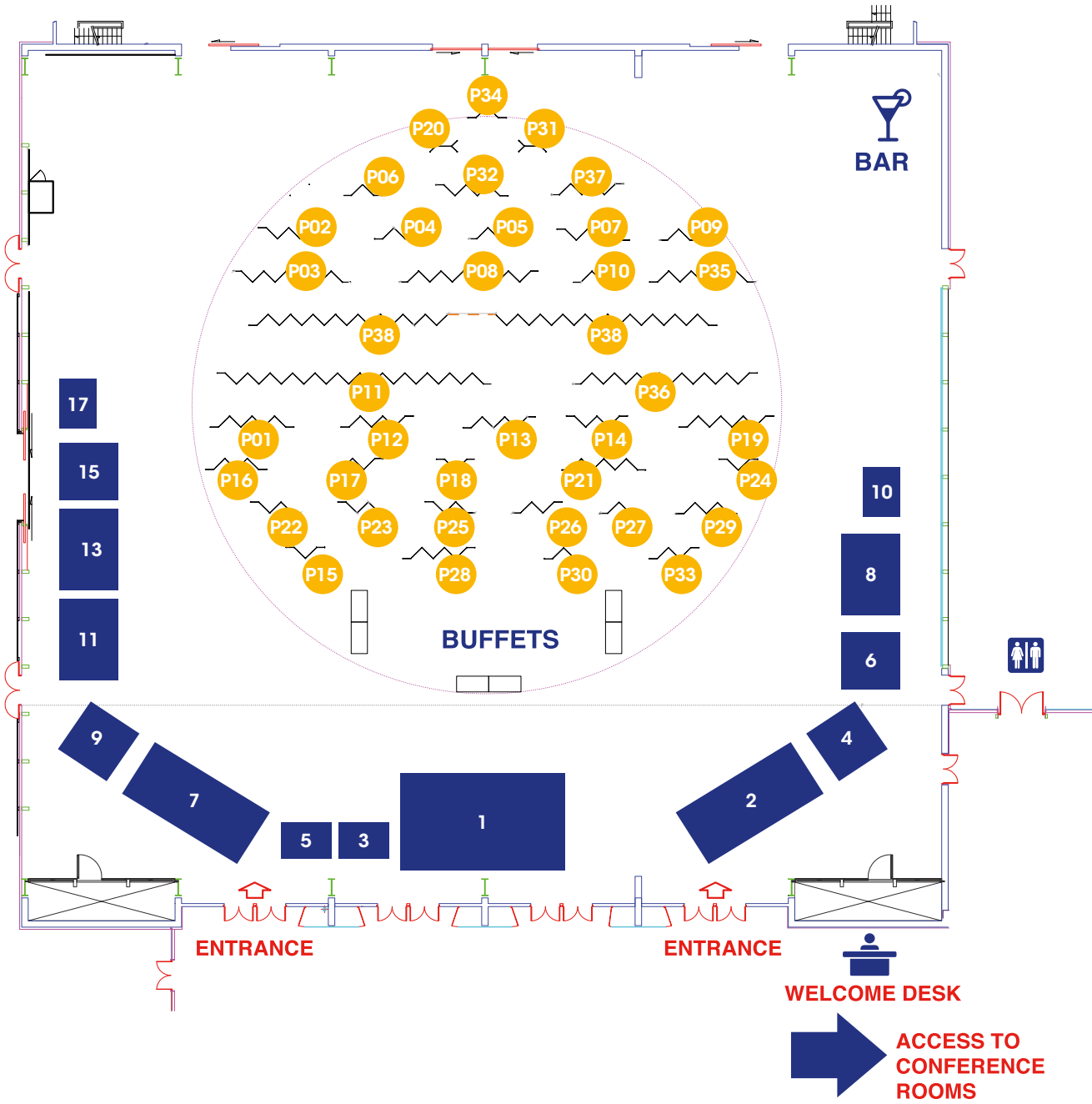
**AMPHI A**

## Posters - pluriel room -

- P01.** Adult muscle regeneration >> **N° 001 / N° 012**
- P02.** ALS and other motor neuron diseases (except SMA) >> **N° 013 / N° 020**
- P03.** Animal models >> **N° 021 / N° 038**
- P04.** Cardiac stem cells and cardiogenesis >> **N° 039 / N° 042**
- P05.** Cardiomyopathies >> **N° 043 / N° 044**
- P06.** Congenital muscular dystrophies (other than dystroglycanopathies) >> **N° 045 / N° 046**
- P07.** Congenital myasthenic syndromes >> **N° 0 c**
- P08.** Congenital myopathies >> **N° 056 / N° 076**
- P09.** Distal myopathies >> **N° 077 / N° 079**
- P10.** Dystroglycanopathies >> **N° 080 / N° 083**
- P11.** Dystrophinopathies (Duchenne, Becker, others) >> **N° 084 / N° 129**
- P12.** Facioscapulohumeral dystrophy (FSHD1, FSHD2) >> **N° 130 / N° 139**
- P13.** Gene therapies (except exon-skipping) >> **N° 140 / N° 149**
- P14.** Gene therapies (exon-skipping) >> **N° 150 > N° 157**
- P15.** Glycogenoses (except Pompe) and other metabolic myopathies >> **N° 158 / N° 160**
- P16.** Hereditary neuropathies >> **N° 161 / N° 166**
- P17.** Homeostasis in the adult muscle >> **N° 167 / N° 172**
- P18.** Inflammatory myopathies >> **N° 173 / N° 176**
- P19.** Limb girdle muscular dystrophies >> **N° 177 / N° 190**
- P20.** Metrology >> **N° 191**
- P21.** Miscellaneous >> **N° 192 / N° 202**
- P22.** Mitochondrial disorders >> **N° 203 / N° 208**
- P23.** Muscle function >> **N° 209 / N° 214**
- P24.** Muscle regeneration >> **N° 215 / N° 217**
- P25.** Myasthenia gravis >> **N° 218 / N° 223**
- P26.** Myofibrillar myopathies >> **N° 224 / N° 228**
- P27.** Myotonias (except DM1 and DM2) >> **N° 229 / N° 231**
- P28.** Myotonic dystrophies type 1 (DM1) and type 2 (DM2, PROMM) >> **N° 232 / N° 240**
- P29.** NGS >> **N° 241 / N° 247**
- P30.** Nuclear envelopathies (lamin A/C, emerin, others) >> **N° 248 / N° 251**
- P31.** Oculo-pharyngeal muscular dystrophy >> **N° 252**
- P32.** Outcome measures >> **N° 253 / N° 262**
- P33.** Pharmacological therapy of neuromuscular disease >> **N° 263 / N° 268**
- P34.** Pompe disease >> **N° 269 / N° 271**
- P35.** Skeletal muscle development >> **N° 272 / N° 286**
- P36.** Spinal muscular atrophy (including variants) >> **N° 287 / N° 317**
- P37.** Stem cells therapies >> **N° 318 / N° 327**
- P38.** Young Investigator Poster >> **N° 328 / N° 403**



# EXHIBITION AREA



## BOOTHS

1	AFM-TELETHON	3	MYOBANK
13	AKCEA THERAPEUTICS	5	MYOTOOLS
9	AUDENTES THERAPEUTICS	15	REVERAGEN
8	AVEXIS	11	ROCHE
7	BIOGEN	2	SAREPTA THERAPEUTICS
4	BIOPHYTIS	10	TREAT-NMD
17	ENMC (European Neuromuscular Centre)	6	YPOSKESI



# Château Lafitte





Chateau Lafitte  
Chemin du Loup  
33370 Yvrac  
France



Haven of peace among its vines, located only 15 minutes from the centre of Bordeaux and 20 min by bus from the Convention centre, this prestigious estate will be the setting for our Gala dinner. To the rhythm of the dishes and wines of this Bordeaux chateau, let's share this gastronomic journey in a dressed stone huge reception room, with its panoramic view over the vineyard and its remarkable barrel store. For your enjoyment, we'll combine music and lights to invite you to round off the evening on the dance floor. Let the party begin!



Havre de paix au milieu de ses vignes, situé à 15 min seulement du centre de Bordeaux et à 20 min du Palais des congrès, ce domaine de prestige sera l'écrin de notre Dîner de gala. Au rythme des mets et vins de ce château bordelais, partageons ce voyage gastronomique dans une grande salle de réception en pierres de taille, avec sa vue panoramique sur le vignoble et son chai à barriques remarquables. Pour votre plaisir, combinons musiques et lumières pour vous inviter sur la piste de danse en fin de soirée. Que la fête commence !



## only on registration



**Bus departure from the Bordeaux convention centre at 20:00**

**Returns / first bus at 23:00, last bus at 1:00**

2 stops served :

- Convention centre
- Quinconces place (Downtown)

**Départs des bus du Palais des congrès à 20 h**

**Retour / premier bus à 23 h, dernier bus à 1 h**

2 arrêts desservis :

- Palais des congrès
- Place des Quinconces (centre-ville)



**Access by car**

On ring road exit n° 26 direction Libourne. Take exit n° 2 direction Yvrac. 2<sup>nd</sup> road on the left after Intermarché.

**Accès en voiture**

Sur rocade sortie n° 26 direction Libourne. Prendre sortie n° 2 direction Yvrac. 2<sup>e</sup> route à gauche après l'Intermarché.

## Bordeaux convention centre

**Avenue Jean Gabriel Domergue  
33300 Bordeaux**

Tramway Line C

Stop: Palais des congrès



Association reconnue d'utilité publique

1, rue de l'Internationale - BP 59  
91002 Évry-Courcouronnes cedex  
Tél. : 33 (0)1 69 47 28 28 - Fax : 33 (0)1 60 77 12 16  
Siège social : AFM - Institut de Myologie  
47-83, boulevard de l'Hôpital - 75651 Paris cedex 13  
[www.afm-telethon.fr](http://www.afm-telethon.fr)