



Press invitation – International congress of Myology 2019 At Bordeaux (France) from 25 to 28 March 2019

Twenty years after the first International Congress of Myology in March 2000, the first innovative drugs (gene therapy, ...) are available to patients, the number of clinical trials grows around the world, involving hundreds of patients. Myology, this little-known science dedicated to the study of muscles, is, today, one of the big therapeutic innovation field. During the 3 days of plenary sessions and thematic symposia of Myology 2019, more than 70 scientist speakers from the United States, Japan, France, Italy, England, Germany, Spain, the Netherlands, Russia, Brazil... will testify before 800 scientists.

Would you like to attend ? Sign up, we'll take care of everything !

I participate !

6th edition of the International Myology Congress of the AFM-Telethon

Name :

Media :

Phone :

Email :

Will attend the congress on march 25

Will attend the congress on march 26

Will attend the congress on march 27

Will attend the congress on march 28

Note :

AFM-Telethon's Press office :

Stéphanie Bardon / Marion Delbouis – (+33)1.69.47.29.01 / 06.45.15.95.87

presse@afm-telethon.fr

Program in details

Monday, 25 March 2019

Opening Ceremony • 17:30 > 18:45 • Opening Lecture • Amphi A

18:00 • S01-01 • **From soil to brain, journey in a glass of wine**
> Axel MARCHAL (Bordeaux, France)

Tuesday, 26 March 2019

26-AM-Plenary • 09:00 > 10:30 • Transational Myology • Amphi A

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

09:00 • S02-01 • **Molecular mechanisms regulating muscle satellite cell function**
> Frédéric RELAIX (Creteil)

09:30 • S02-02 • **Approaches to delay the progression of Muscular Dystrophy**
> Graziella MESSINA (Milan, Italy)

10:00 • S02-03 • **Expression and functional analyses of Dlk1 in muscle stem cells and mesenchymal progenitors during regeneration**
> So-Ichiro FUKADA (Osaka, Japan)

26-AM-Coffee • 10:30 > 11:00 • Coffee Break & Exhibition (Poster / Booth) • Pluriel Room

26-AM-Parallel-1 • 11:00 > 12:30 • Congenital Myopathies • Amphi A

• Chairman : Ana FERREIRO (Paris, France)

11:00 • S03-01 • **Modulation of amphiphysin and dynamin rescues severe congenital myopathies**
> Jocelyn LAPORTE (Illkirch, France)

11:30 • S03-02 • **The expanding phenotypical spectrum of RYR1-related neuromuscular disorders**
> Heinz JUNGBLUTH (London, United Kingdom)

12:00 • S03-03 • **Sarcomere contractility in nemaline myopathy**
> Coen Ac OTTENHEIJM (Amsterdam, Netherlands)

26-AM-Parallel-2 • 11:00 > 12:30 • Cardiomyology • Amphi B

• Chairman : Denis DUBOC (Paris, France)

11:00 • S04-01 • **High Risk of Fatal and Non-Fatal Venous Thromboembolism in Myotonic Dystrophy**
> Karim WAHBI (Paris, France)

11:30 • S04-02 • **Altered cytoskeleton in cardiac disease caused by nuclear A-type lamins gene mutations**
> Antoine MUCHIR (Paris, France)

12:00 • S04-03 • **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, United Kingdom)

26-AM-Symposium • 12:30 > 14:00 • INDUSTRY SYMPOSIUM: AVEXIS • Amphi C

26-AM-Lunch • 12:30 > 14:00 • Free Time for Lunch - Exhibition (Poster / Booth) • Pluriel Room

26-PM-YOUNG • 14:00 > 15:00 • YOUNG INVESTIGATORS SYMPOSIUM • Amphi A

- Chairman : Serge BRAUN (Evry, France)

14:00 • S05-01 • **Small non-coding RNAs of intron origin in Myotonic Dystrophy type 1: new candidate drivers of splicing defects**
> Baptiste BOGARD (Paris, France)

14:15 • S05-02 • **Downregulation of Phosphodiesterase 10A mitigates the manifestation of DMD phenotype in zebrafish model**
> Matthias LAMBERT (Boston, USA)

14:30 • S05-03 • **Using Human Pluripotent Stem Cells Derived Motor Neurons to address the Pathogenesis of Spinal Muscular Atrophy**
> Camille JANUEL (Corbeil-Essonnes, France)

14:45 • S05-04 • **Genetic control of skeletal muscle fiber type**
> Matthieu DOS SANTOS (Paris, France)

26-PM-Plenary • 15:00 > 16:00 • Outcome Measures and Biomarkers • Amphi A

- Chairmen : Nathalie GOEMANS (Leuven, Belgium), Volker STRAUB (Newcastle, United Kingdom)

15:00 • S06-01 • **Quantitative muscle MRI as a reliable outcome measure**
> Jordi DIAZ-MANERA (Barcelona, Spain)

15:30 • S06-02 • **Identification of prognostic and pharmacodynamic serum biomarkers in Duchenne and Becker muscular dystrophies**
> Pietro SPITALI (Leiden, Netherlands)

26-PM-Coffee • 16:00 > 16:30 • Coffee Break & Exhibition (Poster / Booth) • Pluriel Room

26-PM-Parallel-1 • 16:30 > 18:00 • Update in Muscular Dystrophies [LGMD, CMD, DMD] • Amphi A

- Chairman : Bruno EYMARD (Paris, France)

16:30 • S07-01 • **AAV-gene transfer in Limb-girdle muscular dystrophies**
> Isabelle RICHARD (Evry, France)

17:00 • S07-02 • **Mutation Specific Precision Therapy: The COL6-Related Dystrophies and Beyond**
> Carsten BONNEMANN (Washington, USA)

17:30 • S07-03 • **Two in vitro muscular dystrophy modelling approaches for development and verification of gene therapy methods**
> Ivan YAKOVLEV (Moscow, Russia)

17:45 • S07-04 • **Residual very low dystrophin levels mitigate dystrophinopathy towards Becker muscular dystrophy**
> Helge AMTHOR (Montigny-Le-Bretonneux, France)

26-PM-Parallel-2 • 16:30 > 18:00 • Muscle & Central Nervous System • Amphi B

- Chairman : Alvaro RENDON (Paris, France)

16:30 • S08-01 • **Genotype-phenotype relationships underlying visual, cognitive and neuropsychiatric disorders in Duchenne muscular dystrophy**
> Francesco MUNTONI (London, United Kingdom)

17:00 • S08-02 • **Neurobehavioral dysfunctions and therapeutic approaches in DMD mouse models lacking brain dystrophins**
> Cyrille VAILLEND (Orsay, France)

17:30 • S08-03 • **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)

17:45 • S08-04 • **Defective visual information processing in Duchenne muscular dystrophy**
> Dora Fix VENTURA (Sao Paulo, Brazil)

26-PM-Symposium • 18:00 > 20:00 • INDUSTRY SYMPOSIUM: SAREPTA • Amphi C

Wednesday, 27 March 2019

27-AM-Plenary • 09:00 > 10:30 • Lessons Learned from Negative Trials • Amphi A

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

09:00 • **S09-01 • GNE Myopathy Therapy Trial With Sialic Acid Supplementation: Why did phase 3 'fail'?**

> Zohar ARGOV (Jerusalem, Israel)

09:30 • **S09-02 • Lessons Learned From Myostatin Trials**

> Kathryn WAGNER (Baltimore, USA)

10:00 • **S09-03 • Cell Therapies**

> Jennifer MORGAN (London, United Kingdom)

27-AM-Coffee • 10:30 > 11:00 • Coffee Break & Exhibition (Poster / Booth) • Pluriel Room

27-AM-Parallel-1 • 11:00 > 12:30 • Motoneuron / NMJ / Membrane • Amphi A

- Chairman : Bruno ALLARD (Lyon, Paris)

11:00 • **S10-01 • Regulation of neuromuscular connectivity by Wnt signaling: from signaling molecule to therapeutic strategies**

> Laure STROCHLIC (Paris, France)

11:30 • **S10-02 • An in vivo model for the functional validation of polymorphisms in genes required for AChR synthesis**

> Jean-Louis BESSEREAU (Lyon, France)

12:00 • **S10-03 • Sarcolemmal membrane repair activated by conserved intracellular signaling responses can compensate for membrane fragility in muscular dystrophy**

> Noah WEISLEDER (Columbus, USA)

27-AM-Parallel-2 • 11:00 > 12:30 • Myotonic Dystrophies • Amphi B

- Chairman : Geneviève GOURDON (Paris, France)

11:00 • **S11-01 • Myotonic dystrophies: current core clinical phenotypes**

> Giovanni MEOLA (Milano, Italy)

11:30 • **S11-02 • RNA-based approaches to reverse repeat expansion toxicity in Myotonic Dystrophy**

> Denis FURLING (Paris, France)

12:00 • **S11-03 • 3D reconstruction of DM1 patients' myoblasts: studying the relationship between CTG repeats, RNA foci and MBNL1 in single cells**

> Alfonsina BALLESTER-LOPEZ (Badalona, Spain)

27-AM-Symposium • 12:30 > 14:30 • INDUSTRY SYMPOSIUM: BIOGEN • Amphi C

27-AM-Lunch • 12:30 > 14:30 • Poster Lunch • Pluriel Room

27-PM-Plenary • 14:30 > 16:00 • Gene Therapies • Amphi A

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

14:30 • **S12-01 • Alternative AAV-based gene therapies for dystrophinopathies**

> Kevin FLANIGAN (Columbus, USA)

15:00 • **S12-02 • Advancements in AAV-based gene therapy strategies for familial ALS forms**

> Maria-Grazia BIFERI (Paris, France)

15:30 • **S12-03 • Gene Therapy of Myotubular Myopathy**

> Anna BUJ-BELLO (Evry, France)

27-PM-Coffee • 16:00 > 16:30 • Coffee Break & Exhibition (Poster / Booth) • Pluriel Room

27-PM-Parallel-2 • 16:30 > 18:00 • Myogenesis & Muscle Ageing • Amphi A

- Chairman : Gillian BUTLER-BROWNE (Paris, France)

16:30 • S14-01 • Regulation of the mouse skeletal muscle stem cell niche during homeostasis and regeneration

> Shahragim TAJBAKHSI (Paris, France)

17:00 • S14-02 • Recovery macrophages secrete pro-fusogenic effectors during skeletal muscle regeneration

> Bénédicte CHAZAUD (Lyon, France)

17:30 • S14-03 • Dynamics of muscle growth, regeneration and hypertrophy provide an essential quantitative basis to understanding muscle stem cell function

> Terence PARTRIDGE (Washington DC, USA)

17:45 • S14-04 • Attenuation of myostatin/activin signaling delay aging signs in progeric mice model

> Khalid ALYODAWI (Reading, United Kingdom)

27-PM-Parallel-1 • 16:30 > 18:00 • Fighting Diagnostic Odysseys • Amphi B

- Chairman : Emmanuelle LAGRUE (Tours, France)

16:30 • S13-01 • Targeted therapies for rare neuromuscular disorders – first steps towards a treatable disease

> Hanns LOCHMULLER (Ottawa, Canada)

17:00 • S13-02 • Genetic Landscape of Limb Girdle Muscular Dystrophies

> Madhuri HEGDE (Waltham, USA)

17:30 • S13-03 • Spinal Muscular Atrophy : A challenging disease for Newborn screening ?

> Laurent SERVAIS (Liège, Belgium)

27-PM-Symposium • 18:00 > 19:30 • INDUSTRY SYMPOSIUM: AKCEA • Amphi C

Thursday, 28 March 2019

28-AM-Plenary • 09:00 > 10:30 • Therapies on the Horizon • Amphi A

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

09:00 • S15-01 • Nucleic acid-based therapies for neuromuscular disease

> Matthew WOOD (Oxford, United Kingdom)

09:30 • S15-02 • Tamoxifen as a treatment for muscular diseases: an unexpected facet of a repurposed anticancer drug

> Olivier DORCHIES (Geneva, Switzerland)

10:00 • S15-03 • Vamorolone retains efficacy and reduces safety concerns of glucocorticoid drugs

> Eric HOFFMAN (Rockville, USA)

28-AM-Coffee • 10:30 > 11:00 • Coffee Break & Exhibition (Poster / Booth) • Pluriel Room

28-AM-Parallel-1 • 11:00 > 12:30 • FSHD • Amphi A

- Chairman : Sabrina SACCONI (Nice, France)

11:00 • S16-01 • Facioscapulohumeral muscular dystrophy

> Silvere VAN DER MAAREL (Leiden, Netherlands)

11:30 • S16-02 • Applying genome-wide CRISPR screens for therapeutic discovery in FSHD

> Angela LEK (New Haven, USA)

12:00 • S16-03 • PAX7, DUX4 and Facioscapulohumeral muscular dystrophy

> Peter ZAMMIT (London, United Kingdom)

28-AM-Parallel-2 • 11:00 > 12:30 • Glycosylation / Sialisation • Amphi B

- Chairman : Cecilia JIMENEZ-MALLABRERA (Esplugues De Lobregat, Spain)

11:00 • **S17-01 • Glycosylation and sialylation in congenital muscular diseases: the glycobiologist's point of view**
> Amaud BRUNEEL (Paris, France)

11:30 • **S17-02 • Molecular mechanisms involved in MDC1A / LAMA2 MD and development of possible treatment options**
> Markus RUEGG (Basel, Switzerland)

12:00 • **S17-03 • Genotype-phenotype correlation in GNE myopathy**
> Oksana POGORYELOVA (Newcastle, United Kingdom)

28-AM-Symposium • 12:30 > 14:00 • SYMPOSIUM INDUSTRIE: ROCHE • Amphi C

28-AM-Lunch • 12:30 > 14:00 • Free Time for Lunch & Exhibition (Poster / Booth) • Pluriel Room

28-PM-Parallel-1 • 14:00 > 15:30 • Metabolic Myopathies • Amphi A

- Chairman : Pascal LAFORET (Paris, France)

14:00 • **S18-01 • Investigational liver gene transfer for secretable GAA in the treatment of Pompe disease**
> Federico MINGOZZI (Philadelphia, USA)

14:30 • **S18-02 • Exercise training and pathophysiology of exercise in metabolic myopathies**
> John VISSING (Copenhagen, Denmark)

15:00 • **S18-03 • Thymidine Kinase 2 Deficiency: review of 16 Spanish patients with a late onset form**
> Carmen PARADAS (Antequera, Spain)

28-PM-Parallel-2 • 14:00 > 15:30 • Innovative Therapie • Amphi B

- Chairman : Shahram ATTARIAN (Marseille, France)

14:00 • **S19-01 • A decoy trapping DUX4 for the treatment of FacioScapuloHumeral Muscular Dystrophy**
> Julie DUMONCEAUX (London, United Kingdom)

14:30 • **S19-02 • CRISPR/Cas9 genome editing to generate new zebrafish models of centronuclear myopathy**
> Eléonore DUPUIS (Brussels, Belgium)

15:00 • **S19-03 • 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)

15:15 • **S19-04 • Gene Therapy For Peripheral Neuropathy CMT1A**
> Nicolas TRICAUD (Montpellier, France)

28-PM-Coffee • 15:30 > 16:00 • Coffee Break & Exhibition (Poster / Booth) • Pluriel Room

28-PM-Surprise Box • 16:00 > 17:00 • Surprise Box • Amphi A

- Chairman : Bertrand FONTAINE (Paris, France)

16:00 • **S20-01 •**

16:15 • **S20-02 •**

16:30 • **S20-03 •**

16:45 • **S20-04 •**

28-SPM-Closing • 17:00 > 17:30 • Closing Ceremony • Amphi A

17:00 • **S21-01 •**