



## Invitation presse Congr  s Myology 2019

**Bordeaux 25 - 28 Mars 2019**

Vingt ans apr  s le premier congr  s international de myologie en mars 2000, les premiers m  dicaments de th  rapie innovante sont disponibles pour les malades, les essais se multiplient et des centaines de malades    travers le monde y participent. La myologie, cette science m  connue, est devenue le fer de lance de l'innovation th  rapeutique. Plus de 70 conf  renciers des Etats-Unis, du Japon, de France, d'Italie, d'Angleterre, d'Allemagne, d'Espagne, des Pays-Bas, de Russie, du Br  sil... en t  moigneront [durant les 3 jours de pl  ni  res et symposia th  matiques](#).

**Vous souhaitez y assister ? Inscrivez-vous, on s'occupe de tout !**

### Je participe !

**6  me du congr  s international de myologie de l'AFM-T  l  thon**

Nom : .....

M  dia : .....

T  l : .....

Email : .....

**Assistera au congr  s le 25 mars**

**Assistera au congr  s le 26 mars**

**Assistera au congr  s le 27 mars**

**Assistera au congr  s le 28 mars**

Commentaires :

**Service de presse AFM-T  l  thon :**

St  phanie Bardon / Marion Delbouis – 01.69.47.29.01 / 06.45.15.95.87

[presse@afm-telethon.fr](mailto:presse@afm-telethon.fr)

# Le programme en détail

## 25 Mars 2019

Opening Ceremony • 17h30 > 18h45 • Cérémonie d'Ouverture • Amphi A

18h00 • S01-01 • De la terre à la tête, voyage dans un verre de vin  
> Axel MARCHAL (Bordeaux, France)

## 26 Mars 2019

26-AM-Plenary • 09h00 > 10h30 • Myologie Translationnelle • Amphi A

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

09h00 • S02-01 • Molecular mechanisms regulating muscle satellite cell function  
> Frédéric RELAIX (Creteil)

09h30 • S02-02 • Approaches to delay the progression of Muscular Dystrophy  
> Graziella MESSINA (Milan, Italy)

10h00 • 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 • 10h30 > 11h00 • Pause Café & Exhibition (Poster / Stand) • Pluriel Room

26-AM-Parallel-1 • 11h00 > 12h30 • Myopathies Congénitales • Amphi A

• Modérateur : Ana FERREIRO (Paris, France)

11h00 • S03-01 • Modulation of amphiphysin and dynamin rescues severe congenital myopathies  
> Jocelyn LAPORTE (Illkirch, France)

11h30 • S03-02 • The expanding phenotypical spectrum of RYR1-related neuromuscular disorders  
> Heinz JUNGBLUTH (London, United Kingdom)

12h00 • S03-03 • Sarcomere contractility in nemaline myopathy  
> Coen Ac OTTENHEIJM (Amsterdam, Netherlands)

26-AM-Parallel-2 • 11h00 > 12h30 • Cardiomyologie • Amphi B

• Modérateur : Denis DUBOC (Paris, France)

11h00 • S04-01 • High Risk of Fatal and Non-Fatal Venous Thromboembolism in Myotonic Dystrophy  
> Karim WAHBI (Paris, France)

11h30 • S04-02 • Altered cytoskeleton in cardiac disease caused by nuclear A-type lamins gene mutations  
> Antoine MUCHIR (Paris, France)

12h00 • 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 • 12h30 > 14h00 • SYMPOSIUM INDUSTRIE: AVEXIS • Amphi C

26-AM-Lunch • 12h30 > 14h00 • Déjeuner Libre - Exhibition (Poster / Stand) • Pluriel Room

26-PM-YOUNG • 14h00 > 15h00 • SYMPOSIUM JEUNES CHERCHEURS • Amphi A

• Modérateur : Serge BRAUN (Evry, France)

14h00 • S05-01 • Small non-coding RNAs of intron origin in Myotonic Dystrophy type 1: new candidate drivers of splicing defects  
> Baptiste BOGARD (Paris, France)

14h15 • S05-02 • Downregulation of Phosphodiesterase 10A mitigates the manifestation of DMD phenotype in zebrafish model  
> Matthias LAMBERT (Boston, USA)

14h30 • S05-03 • Using Human Pluripotent Stem Cells Derived Motor Neurons to address the Pathogenesis of Spinal Muscular Atrophy  
> Camille JANUEL (Corbeil-Essonnes, France)

14h45 • S05-04 • Genetic control of skeletal muscle fiber type  
> Matthieu DOS SANTOS (Paris, France)

**26-PM-Plenary • 15h00 > 16h00 • Outils d'Évaluation et Biomarqueurs • Amphi A**

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

15h00 • S06-01 • **Quantitative muscle MRI as a reliable outcome measure**

> Jordi DIAZ-MANERA (Barcelona, Spain)

15h30 • S06-02 • **Identification of prognostic and pharmacodynamic serum biomarkers in Duchenne and Becker muscular dystrophies**

> Pietro SPITALI (Leiden, Netherlands)

**26-PM-Coffee • 16h00 > 16h30 • Pause Café & Exhibition (Poster / Stand) • Pluriel Room**

**26-PM-Parallele-1 • 16h30 > 18h00 • Dystrophies Musculaires des Ceintures et Dystrophies Musculaires Congénitales • Amphi A**

- Modérateur : Bruno EYMARD (Paris, France)

16h30 • S07-01 • **AAV-gene transfer in Limb-girdle muscular dystrophies**

> Isabelle RICHARD (Evry, France)

17h00 • S07-02 • **Mutation Specific Precision Therapy: The COL6-Related Dystrophies and Beyond**

> Carsten BONNEMANN (Washington, USA)

17h30 • S07-03 • **Two in vitro muscular dystrophy modelling approaches for development and verification of gene therapy methods**

> Ivan YAKOVLEV (Moscow, Russia)

17h45 • S07-04 • **Residual very low dystrophin levels mitigate dystrophinopathy towards Becker muscular dystrophy**

> Helge AMTHOR (Montigny-Le-Bretonneux, France)

**26-PM-Parallele-2 • 16h30 > 18h00 • Muscle et Système Nerveux Central • Amphi B**

- Modérateur : Alvaro RENDON (Paris, France)

16h30 • S08-01 • **Genotype-phenotype relationships underlying visual, cognitive and neuropsychiatric disorders in Duchenne muscular dystrophy**

> Francesco MUNTONI (London, United Kingdom)

17h00 • S08-02 • **Neurobehavioral dysfunctions and therapeutic approaches in DMD mouse models lacking brain dystrophins**

> Cyrille VAILLEND (Orsay, France)

17h30 • 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)

17h45 • S08-04 • **Defective visual information processing in Duchenne muscular dystrophy**

> Dora Fix VENTURA (Sao Paulo, Brazil)

**26-PM-Symposium • 18h00 > 20h00 • SYMPOSIUM INDUSTRIE: SAREPTA • Amphi C**

**27 Mars 2019**

**27-AM-Plenary • 09h00 > 10h30 • Les leçons à tirer des essais thérapeutiques négatifs • Amphi A**

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

09h00 • S09-01 • **GNE Myopathy Therapy Trial With Sialic Acid Supplementation: Why did phase 3 'fail'?**

> Zohar ARGOV (Jerusalem, Israel)

09h30 • S09-02 • **Lessons Learned From Myostatin Trials**

> Kathryn WAGNER (Baltimore, USA)

10h00 • S09-03 • **Stem cell therapies for muscular dystrophies – lessons from failed trials**

> Jennifer MORGAN (London, United Kingdom)

**27-AM-Coffee • 10h30 > 11h00 • Pause Café & Exhibition (Poster / Stand) • Pluriel Room**

**27-AM-Parallele-1 • 11h00 > 12h30 • Motoneurone - Jonction Neuromusculaire et Membrane Musculaire • Amphi A**

- Modérateur : Bruno ALLARD (Lyon, Paris)

11h00 • S10-01 • **Regulation of neuromuscular connectivity by Wnt signaling: from signaling molecule to therapeutic strategies**

> Laure STROCHLIC (Paris, France)

11h30 • S10-02 • **An in vivo model for the functional validation of polymorphisms in genes required for AChR synthesis**

> Jean-Louis BESSEREAU (Lyon, France)

12h00 • 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 • 11h00 > 12h30 • Dystrophies Myotoniques • Amphi B**

- Modérateur : Geneviève GOURDON (Paris, France)

**11h00 • S11-01 • Myotonic dystrophies: current core clinical phenotypes**

> Giovanni MEOLA (Milano, Italy)

**11h30 • S11-02 • RNA-based approaches to reverse repeat expansion toxicity in Myotonic Dystrophy**

> Denis FURLING (Paris, France)

**12h00 • 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 • 12h30 > 14h30 • SYMPOSIUM INDUSTRIE: BIOGEN • Amphi C**

**27-AM-Lunch • 12h30 > 14h30 • Déjeuner Poster • Pluriel Room**

**27-PM-Plenary • 14h30 > 16h00 • Thérapies Géniques • Amphi A**

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

**14h30 • S12-01 • Alternative AAV-based gene therapies for dystrophinopathies**

> Kevin FLANIGAN (Columbus, USA)

**15h00 • S12-02 • Advancements in AAV-based gene therapy strategies for familial ALS forms**

> Maria-Grazia BIFERI (Paris, France)

**15h30 • S12-03 • Gene Therapy of Myotubular Myopathy**

> Anna BUJ-BELLO (Evry, France)

**27-PM-Parallel-2 • 16h30 > 18h00 • Du muscle en développement au muscle vieillissant • Amphi A**

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

**16h30 • S14-01 • Regulation of the mouse skeletal muscle stem cell niche during homeostasis and regeneration**

> Shahragim TAJBAKHSI (Paris, France)

**17h00 • S14-02 • Recovery macrophages secrete pro-fusogenic effectors during skeletal muscle regeneration**

> Bénédicte CHAZAUD (Lyon, France)

**17h30 • 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)

**17h45 • S14-04 • Attenuation of myostatin/activin signaling delay aging signs in progeric mice model**

> Khalid ALYODAWI (Reading, United Kingdom)

**27-PM-Parallel-1 • 16h30 > 18h00 • La lutte contre l'errance diagnostique • Amphi B**

- Modérateur : Emmanuelle LAGRUE (Tours, France)

**16h30 • S13-01 • Targeted therapies for rare neuromuscular disorders – first steps towards a treataboloome**

> Hanns LOCHMULLER (Ottawa, Canada)

**17h00 • S13-02 • Genetic Landscape of Limb Girdle Muscular Dystrophies**

> Madhuri HEGDE (Waltham, USA)

**17h30 • S13-03 • Spinal Muscular Atrophy : A challenging disease for Newborn screening ?**

> Laurent SERVAIS (Liège, Belgium)

**27-PM-Symposium • 18h00 > 19h30 • SYMPOSIUM INDUSTRIE: AKCEA • Amphi C**

**28 Mars 2019**

**28-AM-Plenary • 09h00 > 10h30 • Thérapies à l'Horizon • Amphi A**

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

**09h00 • S15-01 • Nucleic acid based therapies for neuromuscular disease**

> Matthew WOOD (Oxford, United Kingdom)

**09h30 • S15-02 • Tamoxifen as a treatment for muscular diseases: an unexpected facet of a repurposed anticancer drug**

> Olivier DORCHIES (Geneva, Switzerland)

**10h00 • S15-03 • Vamorolone retains efficacy and reduces safety concerns of glucocorticoid drugs**

> Eric HOFFMAN (Rockville, USA)

**28-AM-Coffee • 10h30 > 11h00 • Pause Café & Exhibition (Poster / Stand) • Pluriel Room**

28-AM-Parallel-1 • 11h00 > 12h30 • **Dystrophie musculaire Facio-Scapulo-Humérale** • Amphi A

- Modérateur : Sabrina SACCONI (Nice, France)

11h00 • **S16-01 • Facioscapulohumeral muscular dystrophy**

> Silvere VAN DER MAAREL (Leiden, Netherlands)

11h30 • **S16-02 • Applying genome-wide CRISPR screens for therapeutic discovery in FSHD**

> Angela LEK (New Haven, USA)

12h00 • **S16-03 • PAX7, DUX4 and Facioscapulohumeral muscular dystrophy**

> Peter ZAMMIT (London, United Kingdom)

28-AM-Parallel-2 • 11h00 > 12h30 • **Glycosylation / Sialisation** • Amphi B

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

11h00 • **S17-01 • Glycosylation and sialylation in congenital muscular diseases: the glycobiologist's point of view**

> Arnaud BRUNEEL (Paris, France)

11h30 • **S17-02 • Molecular mechanisms involved in MDC1A / LAMA2 MD and development of possible treatment options**

> Markus RUEGG (Basel, Switzerland)

12h00 • **S17-03 • Genotype-phenotype correlation in GNE myopathy**

> Oksana POGORYELOVA (Newcastle, United Kingdom)

28-PM-Parallel-1 • 14h00 > 15h30 • **Myopathies Métaboliques** • Amphi A

- Modérateur : Pascal LAFORET (Paris, France)

14h00 • **S18-01 • Investigational liver gene transfer for secretable GAA in the treatment of Pompe disease**

> Federico MINGOZZI (Philadelphia, USA)

14h30 • **S18-02 • Exercise training and pathophysiology of exercise in metabolic myopathies**

> John VISSING (Copenhagen, Denmark)

15h00 • **S18-03 • Thymidine Kinase 2 Deficiency: review of 16 Spanish patients with a late onset form**

> Carmen PARADAS (Antequera, Spain)

28-PM-Parallel-2 • 14h00 > 15h30 • **Thérapies Innovantes** • Amphi B

- Modérateur : Shahram ATTARIAN (Marseille, France)

14h00 • **S19-01 • A decoy trapping DUX4 for the treatment of FacioScapuloHumeral Muscular Dystrophy**

> Julie DUMONCEAUX (London, United Kingdom)

14h30 • **S19-02 • CRISPR/Cas9 genome editing to generate new zebrafish models of centronuclear myopathy**

> Eléonore DUPUIS (Brussels, Belgium)

15h00 • **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)

15h15 • **S19-04 • Gene Therapy For Peripheral Neuropathy CMT1A**

> Nicolas TRICAUD (Montpellier, France)

28-PM-Surprise Box • 16h00 > 17h00 • **Surprise Box** • Amphi A

- Modérateur : Bertrand FONTAINE (Paris, France)

16h00 • **S20-01 •**

16h15 • **S20-02 •**

16h30 • **S20-03 •**

16h45 • **S20-04 •**

28-SPM-Closing • 17h00 > 17h30 • **Cérémonie de Clôture** • Amphi A

17h00 • **S21-01 •**