



13 - 14 February 2016
The Church Palace, Rome – Italy

XIV International Conference **on Duchenne and Becker** **Muscular Dystrophy**

Parent Project Onlus



Agenda - Saturday, February 13

8:15 REGISTRATION

8:45 **WELCOME**

Filippo Buccella, *Parent Project Onlus, Rome, Italy*

9:00 INTRODUCTION

Giuseppe Chinè, *Capo di Gabinetto, Ministero della Salute, Rome, Italy*

Eugenio Gaudio, *Magnifico Rettore Università "Sapienza" di Roma, Rome, Italy*

Walter Ricciardi, *Presidente Istituto Superiore di Sanità, Rome, Italy*

9:20 **ROLE OF TREAT-NMD FOR DMD/BMD THERAPY DEVELOPMENT**

Annemieke Aartsma-Rus, *Leiden University Medical Center LUMC, Leiden, Netherlands*

9:35 **CURRENT SCENARIO OF THERAPEUTIC STRATEGIES**

Fernanda De Angelis, *Parent Project Onlus, Rome, Italy*

TO EVERYTHING THERE IS A SEASON: A TIME TO BE BORN

9:45 **SESSION 1: PRE-CLINICAL RESEARCH**

Chair: *Pier Lorenzo Puri*

Duplications

Kevin Flanigan, *Center for Gene Therapy, Nationwide Children's Hospital, Ohio, USA*

Cell therapy for DMD: steps towards clinical efficacy

Giulio Cossu, *Institute of Inflammation and Repair, University of Manchester, Manchester, UK*

Mitochondria as a new target for drug development in MD

George Schreiner, *Cardero Therapeutics*

CRISPR genome editing and DMD

Rhonda Bassel-Duby, *Department of Molecular Biology UT Southwestern Medical Center, Dallas, USA*

Genetic modifiers

Elena Pegoraro, *Dip. Di Neuroscienze, Università di Padova, Padua, Italy*

11:00 **QUESTION & ANSWER**

11:30 **BREAK**

Agenda - Saturday, February 13

11:45 **SESSION 2:**

CLINICAL TRIALS IN AMBULANT PATIENTS OLDER THAN 5 YEARS

Chair: *Francesco Muntoni*

Outcome measure in ambulant patients older than 5 years

Eugenio Mercuri, *Dip. Neuropsychiatria Infantile, Policlinico Agostino Gemelli, Rome, Italy*

The potential for PDE5 inhibitors in Duchenne muscular dystrophy

Michele Guadagna, *Ely Lilly*

Update: BMS Anti-Myostatin Adnectin Program

Leslie Jacobsen, *Bristol-Myers Squibb*

PF 06252616

Michael Binks, *Pfizer*

Update on Givinostat

Paolo Bettica, *Italfarmaco*

13:00 **QUESTION & ANSWER**

13:30 **LUNCH**

14:30 **SESSION 3:**

CLINICAL TRIALS IN AMBULANT PATIENTS OLDER THAN 5 YEARS

Chair: *Eric Hoffman*

Rimeporide for the treatment of Duchenne muscular dystrophy: starting of a phase 1 multicentric study

Stefano Previtali, *Ospedale San Raffaele, Milan, Italy*

Utrophin upregulation in DMD

Francesco Muntoni, *Dubowitz Neuromuscular Centre and MRC Centre for Neuromuscular Diseases UCL Institute of Child Health and Great Ormond Street Hospital for Children (GOSH), Dept. Of Developmental Neuroscience, London, UK*

SRP 4053

Genevieve Laforet, *Sarepta*

BMN 044; 045; 053

Giles Campion, *BioMarin*

Ataluren

Eugenio Mercuri, *Dip. Neuropsychiatria Infantile, Policlinico Agostino Gemelli, Rome, Italy*

15:50 **QUESTION & ANSWER**

Agenda - Saturday, February 13

TO EVERYTHING THERE IS A SEASON: A TIME TO BUILD UP

16:30 **SESSION 4:**

ROUND TABLE “ADDRESSING YOUNGER POPULATION”

Chair: *Filippo Buccella*

During this session we will discuss the following topics: newborn screening, mean age of diagnosis today, early diagnosis and cognitive impairment, carrier, trial in younger patients, natural history/placebo controlled trials, informed consent/assent form.

Participants:

Adriana Ceci, *Coordinatore Scientifico Network TEDDY/ Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF)*

Adele D'Amico, *UO malattie neuromuscolari e neurodegenerative, Ospedale Pediatrico Bambino Gesù, Rome, Italy*

Alessandra Ferlini, *Department of Reproduction and Growth & Department of Medical Science, Ferrara, Italy*

Marilena Lastella, ** AIFA Ufficio Ricerca e Sperimentazione Clinica – *To be confirmed*

Eugenio Mercuri, *Dip. Neuropsichiatria Infantile, Policlinico Agostino Gemelli, Rome, Italy*

Francesco Muntoni, *Dubowitz Neuromuscular Centre and MRC Centre for Neuromuscular Diseases UCL Institute of Child Health and Great Ormond Street Hospital for Children (GOSH), Dept. Of Developmental Neuroscience, London, UK*

Marika Pane, *Dip. Neuropsichiatria Infantile, Policlinico Agostino Gemelli, Rome, Italy*

20:30 **STANDING COCKTAIL**

Agenda - Sunday, February 14

8:30 WELCOME AND INTRODUCTION

Filippo Buccella, *Parent Project Onlus, Rome, Italy*

8:40 SESSION 5: CLINICAL TRIALS INCLUDING YOUNGER OR NON AMBULANT PATIENTS

Chair: *Annemieke Aartsma-Rus*

Outcome measure for young and non ambulant patients

Marika Pane, *Dip. Neuropsichiatria Infantile, Policlinico Agostino Gemelli, Rome, Italy*

Giovanni Baranello, *UO Neurologia dello Sviluppo Fondazione IRCCS Istituto Neurologico "Carlo Besta", Milan, Italy*

Respiratory outcome

Fabrizio Racca, *Rep. Di Anestesia Pediatrica AO Nazionale, SS. Antonio e Biagio e Cesare Arrigo di Alessandria, Alessandria, Italy*

Efficacy of Raxone® (idebenone) on respiratory function in DMD

Thomas Meier, *Santhera*

Aldosterone inhibition in Duchenne muscular dystrophy: design and rationale of a multicenter clinical trial (AIDMD Trial)

Kan N. Hor, *Nationwide Children's Hospital, Ohio, USA*

VBP 15: A safer and more effective steroid?

Eric Hoffman, *ReveraGen*

CAT 1004, an oral agent targeting NF-κB in development for treatment of Duchenne muscular dystrophy

Joanne M. Donovan, *Catabasis*

FG-3019

Ewa Carrier, *FibroGen*

10:30 QUESTION & ANSWER

11:00 BREAK

Agenda - Sunday, February 14

11:10 SESSION 6:

ROUND TABLE “ADDRESSING ADULT POPULATION”

Chair: *Filippo Buccella*

During this session we will discuss the following topics: outcome measure, wearable, risk/benefit assesment, biopsy/RMN, natural history/placebo controlled trials.

Participants:

Nathalie Goemans, *Dept. of Pediatrics and Child Neurology, University Hospitals, Leuven, Belgium*

Eric Hoffman, *ReveraGen*

Eugenio Mercuri, *Dip. Neuropsichiatria Infantile, Policlinico Agostino Gemelli, Rome, Italy*

Fabrizio Racca, *Rep. Di Anestesia Pediatrica AO Nazionale, SS. Antonio e Biagio e Cesare Arrigo di Alessandria, Alessandria, Italy*

Valeria Ricotti, **Medical Director**, *Clinical Sciences BioMarin Pharmaceutical Inc.*

Honorary Lecturer, Biomedical Research Centre, UCL Institute of Child Health, London, UK

Elizabeth Vroom, *Duchenne Parent Project, Netherlands - United Parent Project Muscular Dystrophy*

TO EVERYTHING THERE IS A SEASON: A TIME FOR LOVE

12:30 SESSION 7:

CLINICAL TRIALS INCLUDING MULTIPLE POPULATIONS

Chair: *Filippo Buccella*

HALO-DMD-01/02/03: Interim safety and efficacy results of ongoing trial

Diana Escolar, *Akashi*

Drisapersen

Giles Champion, *BioMarin*

Eteplirsen

Petra Duda, *Sarepta*

13:15 QUESTION & ANSWER

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