

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. Neuropsichiatria 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. Neuropsichiatria 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 Artsma-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- κ B 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 Campion, BioMarin

Eteplirsen
Petra Duda, Sarepta

13:15 QUESTION & ANSWER

XIV International Conference on DMD/BMD

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