



**XV INTERNATIONAL CONFERENCE
ON DUCHENNE AND BECKER MUSCULAR DYSTROPHY**
18 – 19 February 2017
Ergife Palace Hotel, Rome- Italy

Draft agenda

Saturday, February 18

8:30 REGISTRATION

9:00 WELCOME AND INTRODUCTION

Filippo Buccella, Parent Project Onlus, Rome, Italy

Luca Genovese, Parent Project Onlus, Rome, Italy

Stefano Mazzariol, Parent Project Onlus, Rome, Italy

9:05 CURRENT SCENARIO OF THERAPEUTIC STRATEGIES

Fernanda De Angelis, Parent Project Onlus, Rome, Italy

SESSION 1: PRE-CLINICAL STUDIES

Chair: Pier Lorenzo Puri

Genome editing for DMD

Annemieke Aartsma-Rus, Leiden University Medical Center LUMC, Leiden, Netherland

Stereopure antisense for exon skipping

Wendy Erler, Wave Life science

Gene Therapy for DMD

Carl Morris, Solid Biosciences

Use of microencapsulated Sertoli cells in Duchenne muscular dystrophy

Guglielmo Sorci, University of Perugia, Perugia, Italy

A different approach to delay the progression of Muscular Dystrophy: silencing Nfix slows twitching and regeneration of dystrophic muscles and rescues the pathologic phenotype

Graziella Messina, University of Milan, Milan, Italy

Question & Answer

COFFEE BREAK



SESSION 2: ROUND TABLE "TOWARDS NEW THERAPIES"

Chair: Filippo Buccella

During this session we will talk about pre-clinical research and how to optimize this important phase of drug development in order to improve the chances of success of clinical trials in patients. During the session we will talk also about the costs of the disease

Participants:

Annemieke Aartsma Rus; Leiden University Medical Center LUMC, Leiden, Netherland

Elena Serena, Università degli Studi di Padova, Padova, Italy

Luca Emili, Promeditec

Andrea Marcellusi, Università di Tor Vergata, Rome, Italy

Laura Bianconi, Senatrice

13:00 LUNCH

SESSION 3: ROUND TABLE "ADULT POPULATION"

Chair: Luca Genovese

During this session the children and young adults will talk about independent life and related aspects like school, work, passions, sports and feelings

COFFEE BREAK

SESSION 4: OUTCOME MEASURE

Chair: Eugenio Mercuri

URINE DERIVED STEM CELLS CAN RECAPITULATE DMD PHENOTYPE AND ARE TOOLS TO STUDY MUTATIONS AND DRUGS EFFECT.

Alessandra Ferlini, Dip. di Riproduzione e Accrescimento & Dip. di Scienze Mediche, Ferrara, Italy

MEASURE FOR MEASURE

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

IMPLEMENTATION PROJECT OF THE RESPIRATORY DATA BASE OF PARENT PROJECT ITALY THROUGH SNIP MEASURE

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

Question & Answer



SESSION 5: PHASE 1 – 1/2 CLINICAL TRIALS

Chair: Filippo Buccella

Update: BMS Anti-Myostatin Adnectin Program

Leslie Jacobsen, Bristol-Myers Squibb

CAP-1002

Linda Marban, Capricor Therapeutics

RIM 4DMD study: update and next steps

Stefano Previtali, San Raffaele Hospital, Milan, Italy

Update On Mitochondrial Biogenesis As a Novel Therapy for Duchenne and Becker Muscular Dystrophy

George Schreiner, Cardero Therapeutics

Question & Answer

20:30 DINNER



Sunday, February 19

8:30 WELCOME AND INTRODUCTION

*Luca Genovese, Parent Project Onlus, Italy
Stefano Mazzariol, Parent Project Onlus, Italy*

SESSION 6: PHASE 2 CLINICAL TRIALS

Chair: TBD

Ezutromid/SMT C1100

Ralf Roskamp, Summit

CAT 1004

Joanne M. Donovan, Catabasis

Vamorolone/VBP 15

Eric Hoffman, ReveraGen

Domagrozumab: Pivotal trial update and beyond

Michael Binks, Pfizer

Question & Answer

COFFEE BREAK

SESSION 7: PHASE 3 CLINICAL TRIALS

Chair: Francesco Muntoni

Givinostat

Paolo Bettica, Italfarmaco

The role of Raxone[®] (idebenone) for pulmonary care in DMD

Thomas Meier, Phd Santhera

Eteplirsen, SRP 4053, SRP 4045

Edward Kaye, Sarepta

Updates on Nonsense Mutation

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

Question & Answer



SESSION 8: ROUND TABLE "CLINICAL TRIALS AND EXPECTATIONS"

Chair: Eugenio Mercuri and Francesco Muntoni

During this session we will talk about critical issues related to advanced clinical trials.

Invited to participate:

Edward Kaye, Sarepta Therapeutics

Mark Rothera - Sonmi Park, PTC Therapeutics

Paolo Bettica, Italfarmaco

Thomas Meier, Santhera

Valeria Ricotti, Solid Biosciences

13:30 END OF WORKS