



**XVIII INTERNATIONAL CONFERENCE
ON DUCHENNE AND BECKER MUSCULAR DYSTROPHY
28-29 February – 1 March 2020 Ergife Palace Hotel, Rome - Italy**

DRAFT AGENDA

Friday, February 28

15:00 to 19:00 Parallel session for families (Italian only)

19:30 DINNER (by reservation at the following link <http://conferenza.parentproject.it/>)

Saturday, February 29

8:00 REGISTRATION

8:30 Welcome and Introduction

Luca Genovese, President of Parent Project aps

9:00 SESSION 1: RESTORING DYSTROPHIN PRODUCTION PART 1: GENE THERAPY

Chairs: Eugenio Mercuri and Fernanda De Angelis

Update on PF-06939926, a mini-dystrophin gene therapy for boys with DMD

Beth Belluscio, Pfizer

AAV Gene Therapy for Duchenne

Carl Morris, Solid Biosciences

Microdystrophin Gene Therapy as a treatment for Duchenne Muscular Dystrophy

Sarepta Therapeutics

Microdystrophin Gene therapy

Jean Francois Briand, AFM-Téléthon

Critical issues in Gene therapy

Francesco Muntoni, Dubowitz Neuromuscular Centre UCL Institute of Child Health & GOSH, London

9:55 QUESTIONS AND ANSWERS WITH A PANEL OF EXPERTS

10:30 Break



11:00 SESSION 2: ROUND TABLE "WELLNESS CAN BE AN ART: NEW POSSIBLE SCENARIOS"
Chair: Emanuela Giulitti

The round table aims to disseminate information about recreational activities children and young adults with DMD/BMD can benefit from. Parents and young patients who participate in these activities will share their experiences, highlighting how activities such as figurative arts, dance, music and drama can positively influence children's lives, laying the ground for building personal self-esteem and inclusion within a larger social context. Experts in pet therapy, dance therapy, art therapy and music therapy will be involved as technicians.

Participants: Lucrezia Salucci - Patrizia Cecinini - Daniele Amanti - Francesco Pavese - Ilaria Martino

12:30 Lunch

POSTER SESSION

14:00 SESSION 3: DOWNSTREAM/IMPROVING MUSCLES

Chairs: Claudio Bruno and Ilaria Zito

Results from the interim analysis of a Phase 2/3 study of RG6206 in boys with Duchenne muscular dystrophy

Roxana Dreghici, Roche

Long term evolution of respiratory function with Idebenone treatment

Shabir Hasham, Santhera Pharmaceuticals

Givinostat: clinical update in Duchenne and Becker Muscular Dystrophy

Sara Cazzaniga, Italfarmaco

CAP 1002

Capricor Therapeutics

14:45 QUESTIONS AND ANSWERS

15:15 SESSION 4: ROUND TABLE "BEYOND THERAPIES: leisure TIME"

Chair: Tiziana D'Auria

The round table aims to let children and parents talk about their experiences on the topic of leisure time, with a special focus on sport and travelling activities.

Participants: Stefano Carducci - Lorenzo Diosi - Roberto Vitali - Maria Concetta Del Gaudio

16:45 SESSION 5: DEVELOPING FAMILY GUIDELINES FOR BMD

Participants: *Elena Pegoraro, Alessandra Ferlini, Antonio Bellon and Fernanda De Angelis*



17:15 COFFEE BREAK

17:45 SESSION 6: OPTIMIZING THE RESEARCH FLOW

Chairs: Annemieke Aartsma-Rus and Gloria Antonini

Planning and conducting a comprehensive research study
Annamaria De Luca Università degli Studi di Bari "Aldo Moro"

Use of microincapsulated Sertoli cells in DMD
Guglielmo Sorci – Sara Chiappalupi Università di Perugia

Characterization of the fibroadipogenic progenitors genetic profile during the progression of the DMD and the treatment with HDAC inhibitors
Lorenzo Puri – Luca Tucciarone Fondazione Santa Lucia IRCCS/ Sanford Burnham Prebys Medical Discovery Institute, La Jolla, California – US

Warning and recommendation about Information and Communication
Francesca Ceradini, Osservatorio Terapie Avanzate

18:30 Project HERCULES (HEalth Research Collaboration United in Leading Evidence Synthesis)

Emily Crossley, Duchenne UK

19:00 CLOSE

20:30 Dinner and surprise evening



Sunday, 1st March

9:00 Opening sessions

9:15 SESSION 7: RESTORING DYSTROPHIN PRODUCTION - PART 2

Chairs: Gianluca Vita and Ilaria Zito

Genome editing for Duchenne muscular dystrophy: where are we?

Fernanda De Angelis, Parent Project aps

Exon skipping

Sarepta Therapeutics

Exon Skipping Gene Therapy for DMD

Fulvio Mavilio, Audentes Therapeutics

Gene and Stem Cell Therapy in combination

Giulio Cossu, University of Manchester

Nonsense mutations

Marika Pane, Centro Nemo Pediatrico Policlinico Agostino Gemelli

10:05 QUESTIONS AND ANSWERS

10:30 BREAK

11:00 SESSION 8: ROUND TABLE "AFFECTIVITY"

Chairs: Maria Caterina Pugliese and Luca Buccella

The round table has the objective to discuss love and emotions from several points of view. The session will be managed by operators who have been reflecting and working on this topic for a long time.

Participants: Max Ulivieri - Fabrizio Quattrini

12:30 SESSION 9: OPPOSING INFLAMMATION PROCESSES

Chairs: Michela Catteruccia and Gloria Antonini

Edasalonexent, an oral NF- κ B inhibitor in Phase 3 development for the treatment of Duchenne muscular dystrophy, regardless of mutation

Joanne Donovan, Catabasis Pharmaceuticals



An update on clinical trials of vamorolone for the treatment of Duchenne muscular dystrophy

Laurie Conklin, Reveragen BioPharma

12:50 QUESTIONS AND ANSWERS

13:30 CLOSE