



**XVIII INTERNATIONAL CONFERENCE
ON DUCHENNE AND BECKER MUSCULAR DYSTROPHY
February 17-21, 2021**

Draft Agenda

Wednesday 17

16:45 OPENING

17:00-18:30 PARALLEL SESSIONS

When complexity increases. DMD/BMD and related issues

Paolo Alfieri and Francesca Cumbo – Ospedale Pediatrico Bambino Gesù, Roma

I'm here too. Being a sibling of a DMD/BMD patient

Carla Ferrazzoli – Comitato Siblings

Motor physiotherapy for kids and adults

Cristina Bella – Parent Project aps

18:30 SESSION 1: RESEARCH PROJECTS SUPPORTED BY PARENT PROJECT

Presentation of results of completed research projects

Employment of microencapsulated Sertoli Cells as a new tool to treat Duchenne Muscular Dystrophy

Guglielmo Sorci e Sara Chiappalupi - Università di Perugia

Biobanking Urine Cells

Maria Sofia Falzarano – Università di Ferrara

Role of histone deacetylase inhibitor (HDAC) givinostat on cardiac remodeling

Roberto Rizzi – CNR, Institute of Biomedical Technologies - Milano

Targeting PKC theta to counteract late hallmarks in muscular dystrophy

Marina Bouché – Università di Roma La Sapienza

Epigenetic and transcriptomic profiling of Fibro Adipogenic Progenitors during Duchenne Muscular Dystrophy progression and histone deacetylase inhibitors treatment

Pier Lorenzo Puri and Luca Tucciarone - Sanford Burnham Medical Research Institute, California e Fondazione Santa Lucia, Roma

19:30 Q&A

20:00 CLOSE



Thursday 18

16:45 OPENING

17:00 SESSION 2: ADVANCED THERAPIES: EXPECTATIONS AND REALITY

Hopes and challenges in advanced therapies

Annemieke Aartsma-Rus, Leiden University Medical Center

Path from Bench-to-Bedside: iPS Cell-Based Therapy for Muscular Dystrophies

Rita Perlingeiro, University of Minnesota

Development of CRISPR/Cas9-based therapies for Duchenne Muscular Dystrophy

Eleonora Maino, Hospital for Sick Children in Toronto, Canada

How to escape the immune response: current observations and mitigation strategies

Berry Byrne and Manuela Corti, Powell Gene Therapy Center, University of Florida

18:00 Q&A

18:30 - 20:00 PARALLEL SESSIONS

Laws, rights and bureaucracy (Italian only)

Carlo Giacobini, journalist

Affectivity and sexuality

Max Ulivieri, Comitato Love Giver

20:00 CLOSE

Friday 19

16:30 OPENING

17:00 SESSION 3: ADDRESSING DYSTROPHIN PRODUCTION: FROM DMD/BMD DIAGNOSIS TO GENE THERAPY

DMD/BMD diagnosis

Eugenio Mercuri, Policlinico Gemelli - Roma

CIFFREO: Pfizer's Phase 3 study of gene therapy for DMD

Beth Belluscio, Pfizer

IGNITE DMD Clinical Trial Update

Carl Morris, Solid Biosciences

SRP-9001



Alex Murphy, Roche - Teji Singh, Sarepta

An Introduction to REGENXBIO and RGX-202, a new gene therapy program for Duchenne Muscular Dystrophy (DMD)
Olivier Danos, REGENXBIO

18:00 Q&A

18:30 SESSION 4: PSYCHOLOGICAL ASPECTS IN THE COMMUNICATION OF DIAGNOSIS: THE FAMILY PERSPECTIVE

CAD Parent Project aps

20:00 CLOSE

Saturday 20

9:45 OPENING

10:00 SESSION 5: GENETIC MATTERS

As genetic diagnosis addresses personalized therapies

Alessandra Ferlini, Università di Ferrara

RNA-Targeted Therapies: PMO & PPMO update

Luigi Picaro, Sarepta Therapeutics

NS Pharma Inc and the Viltolarsen Clinical Development Program

Benjamin Yungheer, NS Pharma

Dyne Therapeutics: Committed to Delivering Transformative Therapies for Duchenne Muscular Dystrophy Patients

Romesh Subramanian, Dyne Therapeutics

Mesoangioblast mediated exon skipping

Giulio Cossu, University of Manchester

Nonsense mutations

Luca Bello, Università degli Studi di Padova

11:30 Q&A

12:00 LUNCH BREAK

15:45 OPENING



16:00 SESSION 6: CLINICAL AND DRUG MANAGEMENT: PRESENT AND FUTURE

Clinical management

Adele D'Amico, Ospedale Pediatrico Bambino Gesù – Roma

Phase 3 PolarisDMD trial of edasalonexent in Duchenne

Joanne M. Donovan, Catabasis Pharmaceuticals

Vamorolone as a potential safer alternative to corticosteroids in DMD

Eric Hoffman, ReveraGen BioPharma

Pamrevlumab Program Development in DMD

Bassem Elmankabadi, FibroGen

An update of Givinostat clinical development

Paolo Bettica, Italfarmaco

FOR DMD

Michela Guglieri, University of Newcastle

Digital endpoints in rare disease: measuring ease of movement in Duchenne Muscular Dystrophy

Mindy Leffler, Casimir

17:30 Q&A

18:00 SESSION 7: PSYCHOLOGICAL ASPECTS OF THE TRANSITION FROM CHILDHOOD TO ADOLESCENCE: THE FAMILY PERSPECTIVE

CAD Parent Project aps

19:30 CLOSE

Sunday 21

10:00-11:30 PARALLEL SESSIONS

By integrating we learn: school integration (ITALIAN ONLY)

Giancarlo Onger – C.N.I.S. Brescia

What we eat matters. Nutritional aspects in DMD and BMD

Simona Bertoli – Università degli Studi di Milano

Easy life. Home automation at the service of the community

Luca Bertazzoni – Domotica Quadrifoglio

11:30 SESSION 8: FAMILY GUIDELINES AND TRIALS FOR BMD



Developing family guidelines for BMD

Elena Pegoraro, Università degli Studi di Padova

Givinostat in BMD

Giacomo Comi, Fondazione IRCCS Cà Granda Ospedale Maggiore Policlinico, Milano

A Mitochondrial Approach to Becker Muscular Dystrophy

Ransi Somaratne, Epirium Bio

Introduction to Edgewise Therapeutics: Our company and our plans

Alan Russel, Edgewise Therapeutics

12:30 Q&A

13:00 LUNCH BREAK

15:00 SESSION 9: PSYCHOLOGICAL ASPECTS OF ADULT LIFE: THE PATIENT PERSPECTIVE

CAD Parent Project aps

16:30 SESSION 10: HEART AND LUNG FUNCTION

Cardiac management

Rachele Adorisio, Ospedale Pediatrico Bambino Gesù - Roma

Respiratory management and Emergency Card

Fabrizio Racca, SS Antonio Biagio e Cesare Arrigo di Alessandria

Santhera: Learnings from the SIDEROS trial and enduring commitment to the Duchenne community

Jodi Wolff, Santhera Pharmaceuticals

The FIGHT DMD Trial

Ines Marcias-Perez, Cumberland Pharmaceuticals

CAP-1002 (cardiac cell therapy) for the treatment of upper limb and cardiac function in later stage DMD patients

Linda Marbàn, Capricor Therapeutics

17:30 Q&A

19:00 CLOSE