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

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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
Barry 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

What we eat matters. Nutritional aspects in DMD and BMD
Simona Bertoli – Università degli Studi di Milano

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

Micro-dystrophin gene therapy

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 Yungher, 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 MANAGMENT: 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

Establish clinical and genetic characteristics in young, steroid naïve subjects with Duchenne muscular dystrophy: the FOR DMD study
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

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 managment
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