



Duchenne
**Parent
Project**
onlus

IT TAKES TEAMWORK TO CHANGE THE WORLD

Participation makes a difference



**XVII INTERNATIONAL CONFERENCE
ON DUCHENNE AND BECKER MUSCULAR DYSTROPHY**

Saturday, February 16 | 08:00 REGISTRATION

8:30

Welcome and introduction

Luca Genovese, *Parent Project onlus*

Ronald Cohn, *SickKids, Canada*

Cell therapy for DMD: why it may finally work

Giulio Cossu, *University of Manchester*

9:00

Current Scenario of Therapeutic Strategies

Francesca Ceradini, *Parent Project onlus*

10:30 Questions & answers

9:15 SESSION 1:

RESTORING DYSTROPHIN PRODUCTION - PART 1

Chairs: Francesco Muntoni and Filippo Buccella

11:00 Break



11:15 SESSION 2: ROUND TABLE

When complexity increases:

DMD/BMD and related issues

Chair: Marica Pugliese, *Parent Project onlus*

Gene Therapy - AAV-MINI DYS

Michael Binks, *Pfizer*

Microdystrophin Gene Therapy - SGT-001

Carl Morris, *Solid Biosciences*

Microdystrophin Gene Therapy as a treatment for Duchenne Muscular Dystrophy

Rachel Potter, *Sarepta*

Updates about genome editing for Duchenne muscular dystrophy

In this session, the topic of DMD/BMD-related issues will be tackled. Starting from studies that show that in one third of cases patients may present difficulties in neuropsychological functions, learning, behavior and mood disorders, we will outline the neuro-rehabilitative approaches designed on the specificity of the individual. Particular attention will be dedicated to the ways in which this ecological approach is performed and

*adapted to contexts of daily life, at home
and at school.*

Interventions:

Marika Pane, *Centro Nemo Pediatrico
- Policlinico Universitario "A. Gemelli",
Rome*

Maria Elena Lombardo, *UILDM Sezione
Lazio*

Rosy Galbo, *UILDM Sezione Lazio*

Serena Calabrese, *speech therapist*

Giancarlo Onger, *C.N.I.S. Brescia*

Sabrina Parazzini, *Istituto Comprensivo
via Luigi Rizzo, Rome*

Serena Calabrese, *speech therapist*

Families' experiences

Domagrozumab

Michael Binks, *Pfizer*

Long term efficacy of idebenone on respiratory function outcomes in patients with DMD – real world evidence

Thomas Meier, *Santhera*

Pharmaceuticals

15:00 Questions & answers

12.45: Lunch



Poster session

15:30 Break



14:00 SESSION 3: IMPROVING MUSCLES

*Chairs: Valeria Sansone and Stefano
Mazzariol*

Givinostat: clinical update in Duchenne and Becker

Paolo Bettica, *Italfarmaco*

Roche Anti-myostatin RG6206 in Duchenne

Roxana Dreglici, *Roche*



15.45 ROUND TABLE

Taking care of caregivers: women who meet DMD/BMD

Chair: Emanuela Giulitti, Parent Project onlus

In this session the role of women, either mothers or sisters, who are confronted with the diagnosis of DMD/BMD, will be emphasized. The interventions will focus on the importance of being aware of the inheritance of the pathology and of the possibility of being a carrier, underlining the strategies of clinical management in the event of a carrier female. Particular attention will be dedicated to the psycho-emotional state connected with the fulfillment of maternal duties, through personal life experiences.

Interventions:

Alessandra Ferlini, *University of Ferrara*

Elena Pegoraro, *University of Padua*

Families' experiences

17:15 "New lives:
stories linked by
Duchenne and Becker
muscular dystrophy" –
DOCUMENTARY FILM

18:00 SESSION 5:

Patients: the extra support in the lab

Stefano Mazzariol, *Parent Project onlus*

Pier Lorenzo Puri, *Fondazione Santa*

Lucia - Rome and Sanford Burnham

Medical Research Institute - California,

USA

18:45 Emergency card

Fabrizio Racca, *"SS. Antonio e Biagio e*

Cesare Arrigo" Hospital, Alessandria

19:00 CLOSE

20:30 Dinner

Dj Set



Sunday, February 17 | 08:00 REGISTRATION

8:15

Welcome and introduction

**8:30 SESSION 6:
RESTORING DYSTROPHIN
PRODUCTION - PART 2**

*Chairs: Annemieke Aartsma-Rus and
Filippo Buccella*

**Exon skipping development
program: PMO and PPMO**

*Madeleine Billeter, Sarepta
Therapeutics*

**Investigational stereopure
oligonucleotides for DMD:
clinical trial update**

Mike Panzara, Wave Life Science

Update on non-sense mutations

*Eugenio Mercuri, Policlinico
Universitario "A. Gemelli", Rome*

**09:15 Questions &
answers**

**09:45 SESSION 7:
ROUND TABLE**

**Accessibility made possible: a
conscious design of living spaces**

*Chair: Tiziana D'Auria, Parent Project
onlus*

*The interventions in this session will
outline the procedures to be followed to*

*design accessible living spaces based
on individual needs.*

*Starting from the definition of the
guidelines on housing renovation, the
possibilities offered by the new domotics
technologies will be evaluated. With the
aim of demonstrating the feasibility of
a good design, particular attention will
be dedicated to the stories of housing
reorganizations already implemented or
in progress.*

Interventions:

*Michele Marchi, Department of
Architecture, University of Ferrara
Luca Bertazzoni, Ambient Assisted
Living (AAL) Quadrifoglio
Patients' and families' experiences*

15:30 Break



11:30 SESSION 8:

**Improving muscles & opposing
inflammation processes**

*Chairs: Giuseppe Vita and Luca
Genovese*

Update on vamorolone

Eric Hoffman, ReveraGen BioPharma

**PolarisDMD, a Phase 3 Trial of
Edasalonexent, a Novel NF-κB
Inhibitor**

*Joanne Donovan, Catabasis
Pharmaceuticals*

The BRAVE Study: A Phase 2 Trial to Assess the Efficacy and Safety of MNK-1411 in Duchenne Muscular Dystrophy

Jacqueline Delfgaauw, Mallinckrodt Pharmaceuticals

TAMOXIFEN IN DUCHENNE MUSCULAR DYSTROPHY: Rationale and protocol for a multicentre, randomised, double-blind, placebo-controlled, phase 3 safety and efficacy 48-week trial

Dirk Fisher, Universitäts-Kinderspital Basel (UKBB)

continue with a debate that will involve representatives of all figures involved in the process: patients, clinicians, experts and institutions.

Interventions:

Sandra Petraglia, *Pre-authorization area - AIFA, Rome – To be confirmed*

Francesco Macchia, *Orphan Drugs Observatory*

Valeria Sansone, *Centro Clinico Nemo - "Niguarda" Hospital, Milan*
Families' experiences

12:15 Questions & answers

14.00 Close

12:45 SESSION 9:
ROUND TABLE

Drugs early access programs

Chairs: Filippo Buccella and Stefano Mazzariol, Parent Project onlus

This session is dedicated to the regulatory aspects that control early access to experimental drugs. The table will open with a presentation of the possible early access programs and will



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