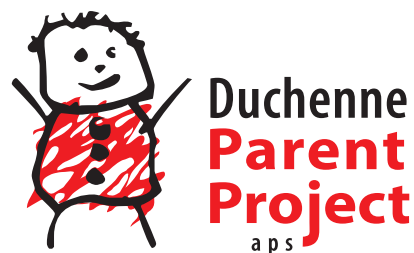




**28
February
2 March
2025**



**22nd International
Conference on Duchenne
and Becker muscular
dystrophy**

Accoglienza
Speranza
Passione
Appartenenza
Comunità
Presenza
Condizione
Gioia

Trasformare i confini in orizzonti.
Ogni giorno è un'opportunità.

Turning boundaries into horizons.
Each day is an opportunity.

2:00 pm

REGISTRATION AND WELCOME

3:00 pm

**Parent Project meets the new families
(Italian only)**

Parent Project aps

ROOM BAALBEK

**5:00 pm - PARALLEL SESSIONS
(Italian only)**

ABC of Science in DMD/BMD

Parent Project aps

ROOM MERIDA

**Cognitive and emotional-behavioural
aspects: evaluation, rehabilitation and
monitoring**

Francesca Cumbo and Michele Tosi, OPBG Rome

Relationships and sexuality

Maximiliano Ulivieri - Comitato Love Giver

ROOM POLA

**BMD: group for discussion among patients
over 16**

Parent Project aps

ROOM SABRATHA

**Laws, bureaucracy and rights for people
with DMD/BMD**

*Carlo Giacobini - Journalist, Publicist and Consultant for Parent
Project aps*

ROOM ORANGE 1

5:30 pm

School aids and technologies

*Fabrizio Corradi - Istituto Leonarda Vaccari, Roma – LUMSA,
Roma*

Daniela Frigerio - Istituto Giannina Gaslini, Genova

ROOM ORANGE 2

8:30 pm

DINNER



SATURDAY, MARCH 1 - PLENARY SESSION

8:30 am

A SPECIAL WELCOME

9:30 am

SCIENTIFIC SESSION - FLASH

How to alter the disease progression

Moderator: Ilaria Zito, *Parent Project aps*

Updates on:

CAP-1002

Marika Pane - *Policlinico Gemelli, Rome*

EDG-5506

Giacomo Comi - *Policlinico of Milan*

Satralizumab

Riccardo Masson - *Istituto Besta, Milan*

Q&A with: *Capricor Therapeutics, Edgewise Therapeutics, Roche*

10:10 am

ROUND TABLE

The culture that DisAbilitates: Countering Ableism in all its forms

Moderator: Marica Pugliese - Sara Billai - Carlotta

Falaschi - *Parent Project aps*

Rachel Callander - *Speaker. Author. Trainer. Artist*

Rosa Bellacicco - *University of Turin*

Maximiliano Ulivieri - *Comitato Love Giver*

Testimonies from families and patients:

Valentina Esposito

Samuele Ragona

Domitilla Mezzanotte

11:25 am

How activism is changing: the new opportunities of 2025

Massimo Coen Cagli - *Fundraising School, Rome*

11:40 am COFFEE BREAK



12:00 am

SCIENTIFIC SESSION

What we have learned: feedback from respiratory and endocrine meetings

Moderator: Fernanda De Angelis, *Parent Project aps*

Fabrizio Racca - *Ospedale Mauriziano, Turin*

Tommaso Aversa - *Policlinico of Messina*

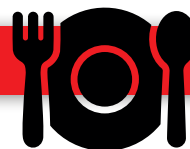
Q&A

12:45 pm

The Power of Words

Rachel Callander - *Speaker. Author. Trainer. Artist.*

1:00 pm LUNCH BREAK



2:00 pm

Rare Disease Day

Annalisa Scopinaro - *UNIAMO*

Neuromuscular Diseases Board

Fabio Amanti - *Parent Project aps*

Francesco Ieva - *Altrodomani*

2:15 pm

SCIENTIFIC SESSION - FOCUS

Exon skipping

Moderator: Ilaria Zito, *Parent Project aps*

Updates on:

SRP-5051, casimersen, golodirsen, eteplirsen, viltolarsen,

DYNE-251, WVE-N531, BMN 351, ENTR-601-44, AOC 1044, PGN-

ED051, SQY51

Annemieke Aartsma-Rus - *Leiden University Medical Center*

Discussion with:

Sarepta Therapeutics, Dyne Therapeutics, Wave LifeScience,

BioMarin Pharmaceutical, Entrada Therapeutics

3:15 pm

Integrating the patient's voices into registries to improve care and research: the PaLaDIn Project

Ilaria Zito, *Parent Project aps*

SATURDAY, MARCH 1 - PLENARY SESSION

3:20pm ROUND TABLE

Siblings: the transition from the wings to the stage

Moderator: Silvia Bencivelli and Marica Pugliese - Valeria Catarraso -

Carmenrocca Sileo - *Parent Project aps*

Andrea Dondi - *Fondazione Paideia, Milan*

Alessia Cinotti - *University of Milan - Bicocca*

Testimonies from families and patients

Angela Pirozzi

Federica Verardo

Francesco Marinelli

4:35pm

The new and final website of Parent Project aps

Mirco Natili - *Parent Project aps*

4:50 pm COFFEE BREAK



5:20 pm

SCIENTIFIC SESSION - FOCUS GENE THERAPY

Moderator: Fernanda De Angelis, *Parent Project aps*

Updates on:

PF-06939926, SRP-9001, SGT-003, RGX-202, GNT-0004

Eugenio Mercuri - *Policlinico Gemelli, Rome*

Discussion with:

Roche, Solid Biosciences, Genethon

6:30 pm

Closing

8:30 pm DINNER WITH SURPRISE



PARALLEL ROOMS (ITALIAN ONLY)

2:30 pm

Sexuality and relationship discussion group

(for Duchenne and Becker boys
over 16)

Parent Project aps

Parent Project aps

Vittorio Montixi - *Educatore - VideA -*

Il viaggio di Enea

Claudio Montixi

ROOM POLA

2:30 pm

PET THERAPY LAB for children and teenagers

5:00 pm

I'm there too! Being the sibling of a DMD/BMD patient (for siblings of patients only)

Parent Project aps

ROOM MERIDA

5:00 pm

Laboratorio Cinema Lab: Every view is Unique

(for Duchenne and Becker boys
over 11)

Federica Spanò

Gianluca Mazzarella

Experia asp, progetto

"Doppio Sorriso"

ROOM SPALATO

SUNDAY, MARCH 2 - PLENARY SESSION

8:45 am
OPENING OF DAY 2

9:00 am
Scientific Session - FLASH
Innovativeness

Moderator: Ilaria Zito, *Parent Project aps*

First clinical trial of MyoPAXon, an induced pluripotent stem cell-based therapy for DMD
Karim Azzag - *University of Minnesota*
Split intein for gene therapy
Jeffrey Chamberlain - *School of Medicine, University of Washington*
Precise gene editing strategies for DMD and BMD

Francesco Chemello - *University of Bologna*

Q&A

9:40 am
ROUND TABLE: Becker
Muscular Dystrophy

Moderator: Giada Perinel - *Carlotta*

Falaschi - *Parent Project aps*

Francesca Cumbo - *OPBG, Rome*

Elena Pegoraro - *University Hospital Padua*

Michela Caroli - *University Hospital Padua*

Carlo Giacobini - *Journalist, Publicist and Consultant for Parent Project aps*

Testimonies from families and patients:

Fabio Superti

Michele Celebrin

10:55
The Easter Campaign: updates, news, and goals
Mirco Natili - *Parent Project aps*



11.10 am COFFEE BREAK



11:30 am
Scientific Session - FOCUS
Molecules at the end of the journey

Moderator: Fernanda De Angelis, *Parent Project aps*

Vamorolone

Luca Bello - *University Hospital Padua*

Givinostat

Emilio Albamonte - *NeMO Clinical Center, Milan*

Q&A with Santhera Pharmaceuticals, *Italfarmaco*

12:00 pm
Scientific Session - PANEL
DISCUSSION
Problems, challenges, successes: how to optimize the development path of a drug

Moderator: Ialina Vinci e Alberto Bottaro, *Parent Project aps*

Open discussion with

Eugenio Mercuri - *Policlinico Gemelli Rome*

Luca Bello - *University Hospital Padua*

Valeria Sansone - *NeMO Clinical Center, Milan*

Ramona Belfiore-Oshan - *C-Path*

Italfarmaco, Santhera Pharmaceuticals, Roche, Sarepta Therapeutics

PARALLEL ROOMS (ITALIAN ONLY)

9:00
Space for Well-Being: Practical Techniques for Self-care

Sara Billai – Giuseppe Giardina - *Parent Project aps*

ROOM SPALATO

10:30 am
Parallel Session: Sleep and sleeping strategies

Moderator: Cristina Bella - *Parent Project aps*

Giordana Donvito, *Occupational Therapist*

Grazia Crescimanno - *Institute for Biomedical Research and Innovation (IRIB) of the CNR - Palermo*

Maria Grazia D'Angelo - *mo*

Maria Grazia D'Angelo - *IRCCS E.*

Medea, La Nostra Famiglia Association-Bosisio Parini, Lecco

ROOM ORANGE 1

1:30 pm

CLOSING REMARKS
AND LUNCH BOX

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under the patronage



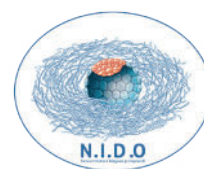
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Parent
Project**
aps

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February
2 March
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PARENT PROJECT aps

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info@parentproject.it - www.parentproject.it
Tel 0666182811 - 800943333

Ente con personalità giuridica iscritto al RUNTS
dal 17/10/22 al n.57282

**Codice Fiscale
05203531008**