



**28  
February  
2 March  
2025**



Duchenne  
**Parent  
Project**  
aps



**22<sup>nd</sup> International  
Conference on Duchenne  
and Becker muscular  
dystrophy**



**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 Olivieri - *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 Olivieri - *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*

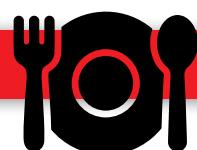
**Q&A**

**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 Artsma-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*

**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

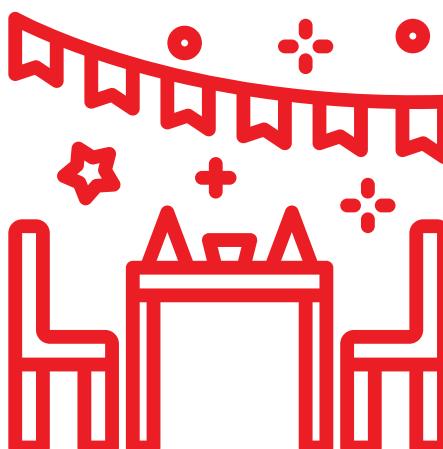
**4:50 pm COFFEE BREAK****5:20 pm****SCIENTIFIC SESSION - FOCUS****GENE THERAPY****Moderator:** Fernanda De Angelis, Parent Project aps**Updates on:**

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****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**

**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:** Lalina 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

**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

# PATRONAGES

*under the patronage*



Con il patrocinio di  
**ÜNIAMO**  
Federazione Italiana Malattie Rare



Università  
degli Studi  
di Ferrara



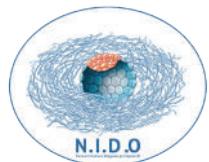
UNIVERSITÀ  
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2 March  
2025**



Accoglienza  
Speranza  
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Comunità  
Presentanza  
Condivisione  
Giusta



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**PARENT PROJECT** aps  
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Tel 0666182811 - 800943333

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

**Codice Fiscale  
05203531008**