

**21st INTERNATIONAL CONFERENCE ON DUCHENNE AND BECKER MUSCULAR DYSTROPHY
16-18 February 2024 – Ergife Palace Hotel, Rome**

Draft agenda

FRIDAY FEBRUARY 16th

14:00 REGISTRATIONS AND WELCOME

15:00 Parent Project Meets The New Families (Italian only)

Ezio Magnano - Ilaria Zito - Sara Guglielmi - Sara Billai - Cristina Bella, Parent Project aps

17:00 PARALLEL SESSIONS (Italian only)

ABC of science in DMD/BMD

Fernanda De Angelis, Parent Project aps

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

Laura Gentile, OMAR - Maria Caterina Pugliese, Parent Project aps

School aids and technologies

Fabrizio Corradi, Istituto Leonarda Vaccari LUMSA, Rome - Daniela Frigerio, Centro Clinico Nemo, Milan - Cristina Bella, Parent Project aps

What we eat has a weight. Nutritional aspects in DMD/BMD

Simona Bertoli, University of Milan, IRCCS AUXOLOGICO, Milan - Valentina Bionda, Parent Project aps

Cognitive and emotional-behavioral aspects: evaluation, rehabilitation and monitoring

Francesca Cumbo and Michele Tosi, Ospedale Pediatrico Bambino Gesù, Rome - Emanuela Giulitti, Parent Project aps

BMD group sharing

Giada Perinel, Sara Guglielmi and Antonio Bellon Parent Project aps

Testimonies from patients



SATURDAY FEBRUARY 17th

PLENARY

PARALLEL ROOMS

**LABORATORIES
(Italian only)**

8:30 A SPECIAL WELCOME

9:30 Scientific session

Strategies in early development

Moderation: Ilaria Zito, Parent Project aps

- Claudio Bruno, Istituto Gaslini, Genoa: DYNE-251, PGN-EDO51, WVE-N531
- Valeria Sansone, Ospedale Niguarda, Milan: ENTR-601-44, BMN-351, AOC-1044
- Stefano Previtali, Ospedale San Raffaele, Milan: RGX-202, SGT-003, DMD06

Q&A with: Dyne Therapeutics, PepGen, Wave Life Sciences, Entrada Therapeutics, BioMarin, Avidity Biosciences, RegenXbio, Solid Biosciences, Prof. Giulio Cossu

9:30 Parallel Session (Italian only)

Laws, bureaucracy and rights

Sara Guglielmi, Parent Project aps - Carlo Giacobini, Consulente Parent Project aps

10:45 PaLaDIn Project presentation

David Allison, Treat-NMD

10:30 Videogames lab

Davide Carotti
AIV, Italian Videogame Academy

11:00 Poster Ciak

11:15 COFFEE BREAK

11:30 Panel discussion

Socialization - bullying - resilience - discrimination

Maria Caterina Pugliese and Sara Billai, Parent Project aps

Alessia Cinotti, University Bicocca, Milan

Testimonies from families

With the participation of Lulu Rimmel, artist

11:30 Parallel Session

Focus on Becker muscular dystrophy

Moderation: Giada Perinel and Fernanda De Angelis, Parent Project aps

- Daniele Velardo, Policlinico of Milan: BMD clinical management
- Luca Bello, University Hospital of Padua: Updates on vamorolone and EDG-5506 clinical trials
- Elisa Minuti, Policlinico of Milan: Physiotherapy and assisted devices in BMD



- Francesca Cumbo,
Ospedale Pediatrico
Bambino Gesù, Rome:
Neuropsychological
aspects in BMD
- Q&A with: Edgewise
Therapeutics, Santhera
Pharmaceuticals

13:00 LUNCH BREAK

13:00 Poster session

14:15 Panel discussion

Affectivity and sexuality, comparing two worlds

Maximiliano Ulivieri, Comitato Lovegiver -
Lulu Rimmel, artist

14:30 Parallel Session (Italian only)

Supplements in DMD/BMD

Moderation: Ilaria Zito and
Francesca Mattei, Parent
Project aps

- Annamaria De Luca,
University of Bari

15:30 Scientific session

Strategies in an intermediate stage of development

Moderation: Fernanda De Angelis, Parent
Project aps

- Adele D'Amico, Ospedale Pediatrico
Bambino Gesù, Rome: PF-06939926,
Ifetroban
- Maria Grazia D'Angelo, IRCCS E. Medea
Bosisio Parini of Lecco: Pamrevlumab,
CAP-1002, ATL1102
- Federica Ricci, Ospedale Regina
Margherita, Turin: SRP-5051, EDG-5506

Q&E with: Pfizer, Cumberland
Pharmaceuticals, Fibrogen,
Capricor Therapeutics, Percheron
Therapeutics, Sarepta Therapeutics,
Edgewise Therapeutics

15:30 Parallel Session (Italian only)

Discussion group on sexuality and affectivity (for boys only)

Maximiliano Ulivieri,
Comitato Lovegiver
Vittorio Montixi, Project: Il
viaggio di Enea
Lulu Rimmel, artist

16:45 COFFEE BREAK

17:15 Scientific session

Let's build the future

Moderation: Annemieke Aartsma-Rus,
Leiden University

- Francesco Saverio Tedesco, UCL, GOSH
& The Francis Crick Institute of London:
3d muscle models and the Magic project
- Adrian Westhaus, Genethon, Evry:
advancements on viral vectors

17:00 Mindfulness workshop for parents

Luisa Barbato, SIAR

17:00 Drone workshop for kids

Oscar
Chinello and
Guido Magni,
SKY Italy





- Francesco Chemello, University of Bologna: Gene editing strategies for Duchenne

17:30 Pet therapy workshop for children
ASD "A TEMPO DI CODA"

18:30 Closing

20:30 DINNER WITH SURPRISE AND POSTER AWARDS



SUNDAY FEBRUARY 18th

PLENARY

8:45 OPENING

9:00 Scientific session

Strategies in an advanced stage of development

Moderation: Fernanda De Angelis, Parent Project aps

- Eugenio Mercuri, Policlinico A. Gemelli, Rome: SRP-9001, Givinostat
- Elena Pegoraro, University Hospital of Padua: Vamorolone, nonsense mutation therapy
- Alessandra Ferlini, University Hospital Sant'Anna of Ferrara: Eteplirsen, Golodirsen/Casimersen, Viltolarsen

Q&A with: Roche, Italfarmaco, Santhera Pharmaceuticals, Sarepta Therapeutics

10:30 Panel discussion

The diagnostic path of preimplantation genetic test in planning a pregnancy for carrier women

Ilaria Zito and Emanuela Giulitti, Parent Project aps

Daniela Zuccarello, University Hospital of Padua, PGT Italian Network (N.I.D.O.)

Andrea Gallinelli, AUSL of Ferrara

Marina Forte, IVI Rome

12:00 Scientific session

Regulatory aspects: feedback from the thematic webinars

Moderation: Filippo Buccella, Parent Project aps

Ramona Belfiore-Oshan, Duchenne Regulatory Science Consortium, C-Path, USA

13:00 LUNCH BOX AND CLOSING

PARALLEL ROOMS

9:00 Parallel Session (Italian only)

Travel and Accessibility

Luigi Passetto, LP viaggi

Roberto Vitali, Village for all

10:30 Parallel Session

Emergencies and how to deal with them

Moderation: Cristina Bella and Nefeli Vourou, Parent Project aps

- Cristina Bella, Parent Project aps: Emergency Card
- Luca Bello, University Hospital of Padua: P.J. Nicholoff protocol and emergencies in fractures
- Fabrizio Racca, AO Ordine Mauriziano, Turin: Respiratory emergencies and anesthesia
- Rachele Adorisio, Ospedale Pediatrico Bambino Gesù, Rome: Cardiac emergencies

