



**2.3.4TH
MAY 2025**

PROGRAM

Endorsed by:



NEUMOPED
Asociación Española de Neumología Pediátrica



FRIDAY MAY 2

(Only Spanish)

13:00-15:45 REGISTRATION

15:45-16:00 WELCOME

Silvia Ávila. President of Duchenne Parent Project Spain.

16:00-18:00 ROUNDTABLE: FAMILIES-CLINICIANS-PATIENTS

This roundtable will address topics related to care standards.

Panel Chair: **Dr. Marisol Montolio**. Director of Research and Technological Department Duchenne Parent Project Spain.

Dr. **Sergi Cesar**. Hospital Sant Joan de Déu (Barcelona).

Dr. **Julita Medina**. Hospital Sant Joan de Déu (Barcelona)

Dr. **Esther Benito**. Hospital Virgen del Rocío (Sevilla).

Dr. **Andrés Nacimiento**. Hospital Sant Joan de Déu (Barcelona).

Dr. **David Gómez**. Hospital Vall d'Hebrón (Barcelona).

Dr. **Carlos Ortez**. Hospital Sant Joan de Déu (Barcelona)

Dr. **Patricia Aguilera**. Hospital Materno infantil Princesa Leonor_H.U. Torrecárdenas (Almería).

Dr. **Pia Gallano**. Hospital de la Santa Creu i Sant Pau (Barcelona).

Dr. **Marcos Madruga**. Neurolinkia (Sevilla).

Dr. **Belén Carnero**. FEA Anestesiología HULA (Hospital Universitario Lucus Augusti).

Dr. **Adolfo López de Munain**. Hospital Universitario Donostia (Donostia).

Dr. **Pilar Martínez**. Neurofisiología Clínica. Hospital de la Arreixaca (Murcia).

18:00-18:30 COFFEE BREAK

18:30-19:30 ROUNDTABLE: ACCESS TO TREATMENTS UNDER SPECIAL CIRCUMSTANCES IN DUCHENNE AND BECKER MUSCULAR DYSTROPHIES

19:30- 20:15 SOCIAL RIGHTS: INTEGRATED CARE

20:15-21:15 INTRODUCTION SESSION – WELCOME WORKSHOP FOR DUCHENNE/ BECKER FAMILIES

21:30 DINNER

SATURDAY MAY 3

Sessions with simultaneous translation between English and Spanish (both directions)

9:00-9:10 INTRODUCTION AND WELCOME

Silvia Ávila. President of Duchenne Parent Project Spain.

9:10-9:40 DENTISTRY IN DUCHENNE MUSCULAR DYSTROPHY

Elizabeth Vroom. World Duchenne Organization.

9:00-13:00 PARALLEL SESSION: POSTERS

9:40-9:50 THERAPEUTIC STRATEGIES: CURRENT SCENARIO WHAT IS A CLINICAL TRIAL?

In this session, the strategy carried out by Duchenne Parent Project Spain to expedite clinical trials for DMD/DMB will be explained: Patient Registry, SARA Program.

Dra. Marisol Montolio. Director of Research and Technological Department, Duchenne Parent Project Spain.

9:50-11:00 CLINICAL TRIALS: GENE THERAPY

Moderators: **Dra. Marisol Montolio**, **Dr. David Gómez**

CIFFREO Trial: What We've Learned and What Still Needs to Be Done in Gene Therapy Trials for DMD, Dr. David Gómez, Hospital Vall d'Hebron

Update on the Delandistrogene Moxeparvec Gene Therapy Program, Dr. Carmen Martín, Roche Farma

SGT-003: A Next Generation Microdystrophin Gene Therapy Candidate, Dr. Patrick González, Solid Biosciences

RegenXBio Clinical Development Updates in DMD, Dr. Naz Dastgir, RegenXbio

• Q&A

11:00-11:30 COFFEE BREAK**11:30-13:00 CLINICAL TRIALS:
EXON SKIPPING**

Moderator: **Dr. Marisol Montolio and Ángel Ayuga**

RNA Therapy Update (PMO and PPMO) (Sarepta).
Hospital Sant Joan de Déu, Barcelona

Advancement of FORCETM in Search for
Transformative Therapies for People Living with
DMD. Dr. Esther Aix, Dyne

48-week Results from FORWARD-53 Study of
WVE-N531 in Duchenne Muscular Dystrophy, Li-Jung
Tai, Wave

Actualización sobre el ensayo clínico BMN 351-201
Dr. Ángel Ayuga, Biomarin

Clinical trial update on EXPLORE44 (Avidity).
Dr. Angela Guerra, Avidity

13:00-15:00 LUNCH**15:00-17:00 PARALLEL SESSION: POSTERS****15:00- 15:10 DUCHENNE PARENT PROJECT
SPAIN SCIENTIFIC STRATEGY: A GLOBAL
VISION FOR THE CURE**

Dr. Marisol Montolio. Director of Research and
Technological Department Duchenne Parent Project
Spain.

15: 10-15:50 PRECLINICAL STUDIES

During this session, we will discuss preclinical re-
search and how to optimize this important phase of
drug development for more successful clinical trials
in the future.

Moderator: **Dr. Marisol Montolio.** Director of Research
and Technological Department Duchenne Parent
Project Spain.

miRNAs: Modulating Muscle Differentiation and
Regeneration. Dr. A. Aránega, Universidad Jaén

Muscle on Chip. Dr. Juan Manuel Fernández, IBEC

Brain Comorbidities in Dystrophinopathies: First
Results from the European BIND Study. Dr. Rubén
Miranda, Universidad Complutense de Madrid

• Q&A

**15:50-16:00 RESEARCH: WHERE WE ARE AND
WHERE WE ARE GOING**

Dr. Marisol Montolio. Director of Research and
Technological Department Duchenne Parent Project
Spain.

**16:00-17:10 IMPROVING MUSCLES, BONES
AND INFLAMMATORY PROCESSES**

Moderator: **Dr. Marisol Montolio and Laura García.**

Sevasemten: A New Approach to Protecting Muscle
in Duchenne and Becker Muscular Dystrophy.
Dr. Katherine Krieger . Edgewise

SHIELD DMD: Study on the impact of satralizumab
on bone and muscle health in Duchenne,
Dr. Maitea Guridi, Roche

Givinostat Development Update for Duchenne and
Becker Muscular Dystrophies.
Laura García, Italfarmaco

Update on Glucocorticoids in the Treatment of DMD.
Dr. Kerry Nip, Santhera

17:10-17:40 COFFEE BREAK

Time to rest and visit the networking area to
meet exhibitors, talk with experts, and chat with
Duchenne and Becker families.

**17:40-18:00 TECHNOLOGY: A NECESSARY SU-
PPORT FOR DUCHENNE AND BECKER MUS-
CULAR DYSTROPHIES**

During this session, we will discuss the implementa-
tion of different devices that can facilitate the course
of the disease. Additionally, we expect knowledge
exchange on the use of digital outcomes that will be
used in Duchenne research, drug development, and
daily management.

Dr. Marisol Montolio. Duchenne Parent Project Spain

Dr. Ofir Arad. Duchenne Parent Project Spain

Dr. Sebastian Idelsohn. Eurecat/Ephion Health

MSc. David Díaz-Pinto. Duchenne Parent Project
Spain

BSc. Aaron Vilalta. Duchenne Parent Project Spain

MSc. Raquel Ávalos. Duchenne Parent Project Spain

18:00-18:30 SCIENTIFIC POSTERS

Posters will be displayed throughout the congress to
provide a space for students, researchers, and profes-
sionals to present their work to all attendees. We will
proceed to vote for the best work in this space.

21:30 DINNER

SUNDAY**MAY 4** (Only in Spanish)**9:55-10:00 WELCOME**

Ms. Silvia Ávila. President of Duchenne Parent Project Spain.

10:00-10:40 CARE IN DUCHENNE AND BECKER MUSCULAR DYSTROPHY**10:40-11:20 LABOR INTEGRATION AND INCLUSION FOR DMD/DMB****11:20- 12:00 ROUND TABLE: EXPERIENCES IN THE ACADEMIC AND WORK ENVIRONMENT OF DMD/DMB****12:00-13:00 ANNUAL GENERAL ASSEMBLY FOR DUCHENNE PARENT PROJECT SPAIN MEMBERS****13:00 CLOSING REMARKS CLOSING REMARKS**

Ms. Silvia Ávila. President of Duchenne Parent Project Spain.

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