



**13-14-15TH
MAY 2022**

PROGRAM

Endorsed by:



With the collaboration of:



FRIDAY MAY 13th

(only in Spanish)

13:00-16:00 REGISTRATION.

16:00-16:15 WELCOME & INTRODUCTION.

Silvia Ávila. Duchenne Parent Project Spain Chair.

16:15-18:00 ROUND TABLE: "PARENTS- PATIENTS- PHYSICIANS".

Moderators: **Dr. Marisol Montolio.**
Scientific Director Duchenne Parent Project Spain.

Dr. Jaume Colomer.
Hospital Sant Joan de Déu (Barcelona)

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

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

Dr. Emilia Barrot. Hospital Virgen del Rocío (Sevilla).

Dr. Marcos Madruga. Neurolinkia y Hospital Viamed
Santa Ángela De la Cruz (Sevilla).

Dr. Carmina Díaz. Hospital General Universitario
de Alicante (Alicante).

Dr. Itxaso Martí. Hospital Universitario Donostia
(San Sebastián).

Dr. Francina Munell. Hospital Vall d'Hebrón
(Barcelona).

Dr. David Gomez. Hospital Vall d'Hebrón (Barcelona).

Dr. Andrés Nascimento. Hospital Sant Joan de Déu
(Barcelona)

Dr. Juan Vílchez. Hospital La Fe (Valencia).

Dr. Cristina Dominguez.
Hospital 12 de Octubre (Madrid).

Dr. Ana Camacho. Hospital 12 de Octubre (Madrid).

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

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

18:00-18:30 COFFEE BREAK.

18:30-18:45 OFFICIAL INTRODUCTION.

Silvia Ávila. Duchenne Parent Project Spain Chair.

Esther Sabando. CEO Duchenne Parent
Project Spain.

Marta Marbán de Frutos. Diputada en la
Asamblea de Madrid por el Partido Popular.
Presidenta de la Comisión de Sanidad.

Paula Gómez-Angulo Amoros. Directora
General de Humanización y Atención al Paciente
de la Comunidad de Madrid.

18:45-20:15 ACCESS TO ORPHAN DRUGS IN DMD/BMD.

Dr. Juan Estévez. Departamento de Medicamentos
de Uso Humano. Agencia Española de Medicamentos
y Productos Sanitarios (AEMPS).

Dr. Jose Luis Poveda. Presidente de la Comisión
Nacional de Farmacia Hospitalaria.

Dr. Álvaro Lavandeira. Doctor en Derecho Sanitario
y Farmacéutico.

Manuel Pérez. Presidente de la Fundación Mehuer
Medicamentos huérfanos y enfermedades raras.

Marian Corral. Directora de la Asociación
Española de Medicamentos Huérfanos y Ultra
Huérfanos Aelmhu.

Silvia Ávila. Duchenne Parent Project Spain Chair.

21:30 DINNER.

SATURDAY MAY 14th

(English and Spanish with simultaneous translation)

9:00-9:10 WELCOME & INTRODUCTION.

Silvia Ávila. Duchenne Parent Project Spain Chair.

9:10-9:20 DUCHENNE PARENT PROJECT SCIENTIFIC STRATEGY: A GLOBAL VISION FOR CURE.

Dr. Marisol Montolio. Scientific Director Duchenne Parent Project Spain.

9:20-10:40 PRE-CLINICAL STUDIES.

Moderator: Dr. Marisol Montolio. Scientific Director Duchenne Parent Project Spain.

Improvements for gene therapy. Dr. Salvador Borrós.

Utrophin repurposing. Dr. Gal.ía Pericot.

miRNAs: modulate muscle differentiation and regeneration. Dr. A. Aránega.

Muscle in a chip. Dr. Juan Manuel Fernández.

Senolytics in DMD. Dr. Pura Muñoz.

10:40-11:15 BRAIN & DYSTROPHIN.

BIND: Brain Involvement in Dystrophinopathies
Dr. Rubén Miranda García. Universidad Complutense de Madrid.

Dystrophinopathies: cognitive implications. A Spanish cohort. Dr. Maria Damià. Hospital La Fe (Valencia).

11:15-11:40 COFFEE BREAK.

11:40-11:50 THERAPEUTIC STRATEGIES: CURRENT SCENARIO, WHAT IS A CLINICAL TRIAL?

Dr. Marisol Montolio. Scientific Director Duchenne Parent Project Spain.

11:50-13:20 RESTORING DYSTROPHIN.

Moderators: Dr. Marisol Montolio. Scientific Director Duchenne Parent Project Spain.

Microdystrophin for Gene Therapy in Duchenne Muscular Dystrophy. Dr. Maitea Guridi, Roche.

Pfizer on DMD: Pfizer's Commitment to Patients. Kim Hae Kyung, Pfizer.

Update of the RNA program (PMO and PPMO). Rachel Salazar, Sarepta Therapeutics.

Update in Nonsense Mutations: Ataluren. Dr. Carlos Ortez. Hospital Sant Joan de Deu. Barcelona.

• Q&A

13:20-15:30 LUNCH.

15:30-17:00 IMPROVING MUSCLES & INFLAMMATORY PROCESSES.

Moderators: Dr. Marisol Montolio. Scientific Director Duchenne Parent Project Spain. Dr. Andrés Nascimento. Hospital Sant Joan de Déu (Barcelona).

Givinostat in development for DMD and DMB. Dr. Nicoletta Coceani. Italfarmaco.

CAP-1002, a cell therapy for the treatment of non-ambulant DMD. Dr. Linda Marban. Capricor.

Vamolorone (ReveraGen-Santhera). Dr. Andrés Nascimento. Hospital Sant Joan de Déu (Barcelona).

Tamoxifen in DMD. Dr. Jessica Expósito. Hospital Sant Joan de Déu (Barcelona).

Anti-Myostatin (RG6206). Dr. Maitea Guridi. Roche.

• Q&A

17:00-17:10 RESEARCH: WHERE ARE WE NOW? WHERE ARE WE GOING?

Dr. Marisol Montolio. Scientific Director Duchenne Parent Project Spain.

17:10-17:40 COFFEE BREAK.

17:40-18:30 TECHNOLOGY IN DUCHENNE AND BECKER.

Moderators: Dr. Ofir Arad & Dr. Marisol Montolio.

Dr. Ofir Arad. Duchenne Parent Project Spain.

Dr. Sebastian Idelshon. Eurecat/ Duchenne Parent Project Spain /Ephion Health.

Dr. Aaron Vilalta. Duchenne Parent Project Spain.

18:30-19:30 POSTERS SESSION RESEARCH & TECHNOLOGY IN DUCHENNE AND BECKER.

21:30 DINNER.

SUNDAY MAY 15th

(only in Spanish)

9:30-9:45 ADULT ADVISORY COMMITTEE DUCHENNE PARENT PROJECT SPAIN.

9:45-10:15 TRANSITION TO THE ADULT STAGE: DEPENDENCE, DISABILITY, AND PROTECTED HERITAGE.

Dr. Álvaro Lavandeira. Departamento Legal.
Duchenne Parent Project Spain.

10:15-12:15 PARALLEL WORKSHOPS.

- › **Duchenne Revolution.** Technological Department and Adult Advisory Committee.
 - › **Sexuality Workshop.** Sara Osuna y David Díaz-Pinto. Psicólogos Duchenne Parent Project Spain. Dr. Thais Pousada García. Grupo investigación TALIONIS. CITIC- Universidade da Coruña.
 - › **Respiratory Physiotherapy Care.** Beatriz Sánchez Fernández. Fisioterapeuta respiratoria. Formadora.
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12:15-12:45 ANNUAL GENERAL ASSEMBLY ONLY FOR MEMBERS OF DUCHENNE PARENT PROJECT SPAIN.

12:45-13:00 CLOSURE.

Esther Sabando. CEO Duchenne
Parent Project Spain.

Silvia Ávila. Duchenne Parent Project Spain Chair.

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