



**2-3-4TH
JUNE 2023**

PROGRAM

Endorsed by:



FRIDAY, JUNE 2TH

(only Spanish)

13:00-16:00 REGISTRATION.

16:00-16:15 WELCOME AND INTRODUCTION.

Silvia Ávila. Duchenne Parent Project Spain.

16:15-18:00 ROUND TABLE

"Parents- Patients- Physicians"

18:00-18:30 COFFEE BREAK.

18:30-20:00 ACCES TO ORPHAN DRUGS: DMD AND BMD.

20:00-21:00 DMD/DMB FAMILIES. PSYCHO-EMOTIONAL WORKSHOP.

21:30 DINNER.

SATURDAY, JUNE 3TH

9:00-9:10 WELCOME AND INTRODUCTION.

Silvia Ávila. Duchenne Parent Project Spain.

9:00-13:00 Satellite Session. London Room.
Physiotherapy in Duchenne and Becker muscular dystrophies.

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

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

9:20-11:00 PRE-CLINICAL STUDIES.

During this session, we will talk about preclinical research and how to optimize this important phase of drug development in order to become more successful and generate more clinical trials in the future.

Moderator: **Dr. Marisol Montolio.**

Possible use of a deletion as a model for gene therapy. Dr. Juan Vílchez.

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

Muscle in a chip. Dr. Juan Manuel Fernández.

Repurposing bortezomib for DMD. Dr. Patricia Rico.

The european DARTER network and Duchenne Parent Project Spain: five years of results.. Dr. Virginia Arechavala.

BIND project. Dr. Rubén Miranda.

• Q&A

11:00-11:30 COFFEE BREAK.

11:30-11:40 THERAPEUTIC STRATEGIES: CURRENT SCENARIO, WHAT IS A CLINICAL TRIAL?

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

11:40-13:00 CLINICAL TRIALS: RESTORING DYSTROPHIN.

Moderators: **Dr. Carlos Ortez, Dr. Marisol Montolio.**

A joint commitment with the DMD. Kim Hae Kyung, Pfizer.

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

RNA Program Update (Sarepta). Dr. Laura Carrera. Hospital Sant Joan de Déu, Barcelona.

Advancing FORCE™ in the Pursuit of Transformative Therapies for People Living with DMD. Dr. Ash Dugar, Dyne.

Non-sense Mutations: Ataluren. Dr. Carlos Ortez. Hospital Sant Joan de Déu, Barcelona.

• Q&A

13:00-15:00 LUNCH.

15:00-15:15 RESEARCH: WHERE WE ARE?**15:15-16:15 IMPROVING MUSCLES AND INFLAMMATORY PROCESSES.**

Moderator: **Dr. Sara Cazzaniga, Dr. Marisol Montolio.**

Givinostat in DMD and DMB.

Dr. Sara Cazzaniaga, Italfármaco.

Vamorolone: study outcomes and introduction in Europe. Dr. Ana de Vera, Santhera.

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

EDG-5506: A Novel Approach to Protect Muscle in Duchenne and Becker Muscular Dystrophy.

Dr. Abby Bronson, Edgewise.

16:15-16:45 COFFEE BREAK.**16:45-17:30 TECHNOLOGY IN DUCHENNE AND BECKER.**

Dr. Marisol Montolio. Duchenne Parent Project Spain.

Dr. Ofir Arad. Duchenne Parent Project Spain.

Dr. Sebastian Idelsohn. Eurecat/Ephion Health.

Bsc. Aaron Vilalta. Duchenne Parent Project Spain.

17:30-19:00 POSTER SESSION: DUCHENNE AND BECKER.**19:00-19:30 SHORT FILM PRESENTATION: A RACE AGAINST DUCHENNE****19:30-20:30 PAEDIATRIC PHYSIOTHERAPY WORKSHOP AND RESPIRATORY PHYSIOTHERAPY WORKSHOP**

(only for families, Spanish).

21:30 DINNER.**SUNDAY,
JUNE 4TH**

(only Spanish)

9:00-9:05 WELCOME AND INTRODUCTION.

Silvia Ávila. Duchenne Parent Project Spain.

9:05-9:30 PSYCHOLOGICAL IMPACT ON DUCHENNE Y BECKER MUSCULAR DYSTROPHY.

Dña. Sara Osuna. Responsable del servicio psicológico Duchenne Parent Project España.

9:30-9:50 DIFFICULTIES IN THE EDUCATIONAL STAGE IN DMD/DMB.

D. David Díaz-Pinto. Servicio Psicológico Duchenne Parent Project España.

9:50-10:10 RESOURCES FOR DISABILITY. NEW SCALES FOR ASSESSING THE DEGREE OF DISABILITY.

Dr. Álvaro Lavandeira. Abogado y asesor jurídico Duchenne Parent Project España.

10:10- 11:00 A LIFE WITH DUCHENNE/BECKER. TESTIMONIES OF AFFECTED AND THEIR FAMILIES.

Dña. Marisa Parra, Dña. María Bernabéu, D. Quique Grávalos, Dña. Juana Rodríguez.

11:00-12:00 PARALLEL SESSIONS.

- > PAEDIATRIC PHYSIOTHERAPY WORKSHOP
- > RESPIRATORY PHYSIOTHERAPY WORKSHOP
- > TECHNOLOGY WORKSHOP

13:00 CLOSURE.

Esther Sabando. CEO Duchenne Parent Project Spain. **Silvia Ávila.** Presidenta Duchenne Parent Project Spain.

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