

7.8.9 JUNIO 2019

**MADRID**

HOTEL: EUROSTARS  
MADRID CONGRESS \*\*\*\*



Duchenne  
PARENT  
PROJECT  
España



**CONGRESO  
INTERNACIONAL  
DUCHENNE**

PARENT PROJECT  
ESPAÑA

*(Draft of the Program)*

## FRIDAY, JUNE 7<sup>th</sup> (only in Spanish)

15:00-16:00 **REGISTRATION**

16: 00-16:15 **WELCOME AND INTRODUCTION**

*Silvia Ávila, Duchenne Parent Project Spain*

16:15-18:00 **ROUND TABLE** "Parents- Patients- Physicians"

Moderators: Dr. Francina Munell, Dr. Marisol Montolio

Dr. Adolfo López de Munain. *IIS Biodonostia (San Sebastián)*

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

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

Dr. Juan Vilchez. *Hospital La Fe (Valencia)*

Dr. Cristina Domínguez. *Hospital 12 de Octubre (Madrid)*

Dr. Jordi Díaz-Manera. *Hospital de la Santa Creu i Sant Pau (Barcelona)*

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

18:00-18:30 **PAUSE**

18:30-19:00 **DEFICIT OF DYSTROPHIN IN THE BRAIN**

19:00-19:20 **AEMPS**

19:20-20:30 **PARALLEL WORKSHOPS**

21:30 **DINNER**

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## SATURDAY, JUNE 8<sup>th</sup>

### 9:00-9:20 WELCOME AND INTRODUCTION

*Silvia Ávila, Duchenne Parent Project Spain*

### 9:20-9:30 DUCHENNE PARENT PROJECT SCIENTIFIC STRATEGY: A GLOBAL VISION FOR CURE

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

### 9:30-11:15 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*

New compounds and Calcium. *Dr. Ainara Vallejo*

miRNAs as molecular tools modulating muscle differentiation and regeneration. *Dr. A. Aránega*

Repurposing Utrophin. *Dr. Raúl Insa*

New pro-regenerative and anti-fibrotic compounds. *Dr. Antonio Ferrer*

Improvements for Gene Therapy. *Dr. Salvador Borrós*

Nintedanib: Repurposing for DMD. *Dr. Jordi Díaz-Manera*

Q&A

### 11:15-11:45 COFFEE BREAK

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**11:45-12:00 THERAPEUTIC STRATEGIES: CURRENT SCENARIO  
WHAT IS A CLINICAL TRIAL?**

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

**12:45-14:00 CLINICAL TRIALS: RESTORING DYSTROPHIN**

*Moderator: Dr. Marisol Montolio, Dr. Juan Vilchez*

Gene Therapy: Microdystrophin. Dr. xxx. Sarepta Therapeutics

Exon Skipping. Dr. xxx. Sarepta Therapeutics

Gene Therapy and Pipeline. Dr. xxx. Pfizer

Exon Skipping. Dr. Matthew Anderton. Wave Life Sciences

Non-sense mutations: Ataluren. Dr. Juan Vilchez. Hospital La Fe, Valencia

Q&A

**13:15-15:00 LUNCH**

**15:00-17:00 CLINICAL TRIALS: IMPROVING MUSCLES AND INFLAMMATORY  
PROCESSES**

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

MNK-1411. Dr. Jacqueline Delfgaauw. Mallinckrodt Pharmaceuticals

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

Clinical development for DMD led by Santhera (SIDEROS on spotlight).

Dr. Kristina Nygren. Santhera

Givinostat in DMD and DMB. Dr. Sara Cazzaniga. Italfarmaco

Tamoxifen in DMD. Dr. Sirk Fisher. Universitats-Kinderspital Basel (UKBB)

Q&A

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17:00-17:30 **COFFEE BREAK**

17:30-19:00 **CARE IN DUCHENNE AND BECKER part I**

Moderator: *Dr. Marisol Montolio, Elizabeth Vroom*

**Dental Considerations.** Elizabeth Vroom. *Chair of World Duchenne Organization*

**Technological Solutions:**

**6MWT+.** Dr. Ofir Arad. *Duchenne Parent Project Spain*

**Exorapi.** Dr. Sebastián Idelshon. *Eurecat*

**Supportive devices to improve the quality of life.** Dr. Joan Lobo. *CSIC.*

21:30 **DINNER**

**SUNDAY, MAY 27<sup>th</sup> (only in Spanish)**

9:30-10:00 **The journey of a Pharmaceutical Company to effectively engage with patient groups in clinical trials.** Dra. Vanessa dos Reis. *Santhera Pharmaceuticals*

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10:00-12:00 **CARE IN DUCHENNE AND BECKER part II**

Moderator Dr. Marisol Montolio, Dr. Emilia Barrot.

Pneumological Care. Dr. Emilia Barrot. *Hospital Virgen del Rocío*

Physiotherapeutic Care. Lidia parra. *Interactúa. Duchenne Parent Project España*

Care of the heart. Dr. Sergi Cesar. *Hospital Sant Joan de Dèu de Barcelona*

Nutritional Care. Dr. Nuria Monfuleda, Dr. Carles Miralles. *Centro de Nutrición*

*Loveyourself*

Neuropsychological Care. Sara Osuna. *Duchenne Parent Project Spain*

12:00-12:10 **CLOSURE**

*Silvia Ávila, Duchenne Parent Project Spain*