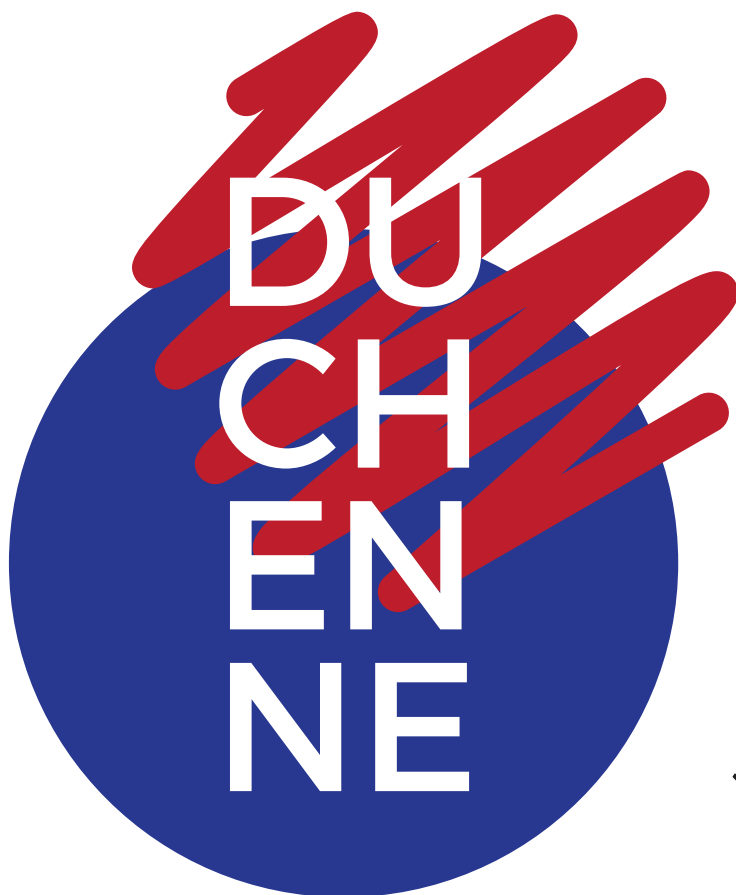


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7.8.9 JUNIO 2019

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FRIDAY, JUNE 7th (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 8th

09:00-09:15 WELCOME AND INTRODUCTION

Silvia Ávila, Duchenne Parent Project Spain

09:15-09:30 DUCHENNE PARENT PROJECT SCIENTIFIC STRATEGY: A GLOBAL VISION FOR CURE

Dr. Marisol Montolio, Scientific Director Duchenne Parent Project Spain

09:30-11:00 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*

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. Lucía Gregorio*

Improvements for Gene Therapy. *Dr. Salvador Borrós*

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

Q&A

11:00-11:30 COFFEE BREAK

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**11:30-11:45 THERAPEUTIC STRATEGIES: CURRENT SCENARIO
WHAT IS A CLINICAL TRIAL?**

Dr. Marisol Montolio, Scientific Director Duchenne Parent Project Spain

11:45-13:15 CLINICAL TRIALS: RESTORING DYSTROPHIN

Moderator: Dr. Marisol Montolio, Dr. Juan Vilchez

Gene Therapy: Microdystrophin. *Dr. Madeleine Billeter. Sarepta Therapeutics*

An update of the gene therapy program. *Nuria Mir. Director Medical Affairs
Rare Disease Spain Pfizer*

Exon Skipping. *Dr. Lucía Quintana-Gallardo. Sarepta Therapeutics*

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

**15:00-16:30 CLINICAL TRIALS: IMPROVING MUSCLES AND INFLAMMATORY
PROCESSES**

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

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

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

Dr. Kristina Nygren - Dr. Vanessa dos Reis. Santhera

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Givinostat in DMD and DMB. Dr. Sara Cazzaniga. *Italfármaco*

Tamoxifen in DMD. Dr. Dirk Fisher. *Universitäts-Kinderspital Basel (UKBB)*

Q&A

16:30-17:00 **COFFEE BREAK**

17:00-18:30 **CARE IN DUCHENNE AND BECKER part I**

Moderator: *Dr. Marisol Montolio, Elizabeth Vroom*

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

Technological Solutions: Dr. Ofir Arad. *Duchenne Parent Project Spain*

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

6MWT+. Eudald Mas. *Eurecat*

Exorapi. Dr. Sebastián Idelshon. *Eurecat*

Q&A

21:00 **DINNER**

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SUNDAY, JUNE 9th (only in Spanish)

09:30-11:30 **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

11:30-11:40 **CLOSURE**

Silvia Ávila, Duchenne Parent Project Spain