

PROGRAM

Tuesday Nov 30, 18:00 – 20:30 CET

Care

- Challenges and opportunities in improving DMD/BMD care
- Patients' and carers' experience of medical care for their rare disease
- What patient organizations can do to improve care

Wednesday Dec 1, 18:00 – 20:30 CET

Capacity & Policy

- Mindsets and strategies for negotiation success
- Professional communication
- Navigating global policy

Thursday Dec 2, 18:00 – 20:30 CET

Therapy Development

- Why don't we have gene and stem cell therapy yet?
- The importance of publishing negative results
- Improving the patient voice in therapy development

Friday Dec 3, 18:00 – 20:30 CET

Data & Scientific Information

- Contributing to collecting and connecting health data
- Available tools to collect and connect health data
- Organizing individual data return from clinical trials
- Finding, following and understanding scientific information

Saturday Dec 4, 15:00 – 19:00 CET

Project updates

- Various presentations

Returning individual clinical trial results back to patients

- Patient preference on returning individual clinical trial data
- The role of CRO's in returning individual clinical trial data
- Which tools are available to facilitate patient data empowerment?

Company updates to the community

- Presentations and panel discussion

Closing ceremony

TUESDAY

NOVEMBER 30

18:00 – 18:10

Welcome

- Welcome and introduction
George Paliouras, Chair Duchenne Data Foundation
Elizabeth Vroom, Chair World Duchenne Organization

18:30 – 19:30

Care

- An introduction to Care
Elizabeth Vroom, Chair World Duchenne Organization
- Interactive session: what are the challenges and opportunities in your country with implementing the Standards of Care?
- Patients' and carers' experience of medical care for their rare disease
Jessie Dubief, Rare Barometer Research Executive at EURORDIS
- What can POs organize to improve care?
Learnings from the Latvian oncology community
Olga Valcina, founder Steps Ahead With Melanoma
DMD Pathfinders
Dr. Jon Rey-Hastie, joint CEO Pathfinders Alliance
Argentina- Programa de Atención Extramuros
Dr. Alberto Dubrovsky, Prof. Neurology at University of Buenos Aires

19:30 – 19:45

Break

19:45 – 20:30

- Questions & Answers, moderated
Elizabeth Vroom, Chair World Duchenne Organization
- Interactive session: How to translate these insights to your patient organization?
- Sharing insights, plenary wrap-up

WEDNESDAY

DECEMBER 01

18:00 – 18:30

Introduction

- An introduction to capacity and policy
Juan Fernández, Policy Advisor at European Patient's Forum
- Interactive session: what are the challenges and opportunities in your country regarding capacity and policy?

18:30 – 19:30

Capacity

- Mindsets and strategies for negotiation success
Lousin Mehrabi, professional negotiator and motivational speaker
- Professional Communication
Dante Di Iulio, Communication Manager at European Patient's Forum
- Questions & Answers, moderated
Ryan Fischer, Chief Advocacy Officer at PPMD USA

19:30 – 19:45

Break

19:45 – 20:30

Policy

- Panel discussion: Navigating global policy
Chaired by Dimitrios Athanasiou, MDA Hellas, Board Member WDO
Mary Wang, Science Policy Manager at Rare Diseases International
Ryan Fischer, Chief Advocacy Officer at PPMD USA
Klair Bayley, Founder Duchenne Australia, Board Member DDF
- Conclusion, plenary wrap-up

THURSDAY

DECEMBER 02

18:00 – 18:10

Therapy Development

- An introduction to Therapy Development
Pat Furlong, President at PPMD USA, WDO Board Member
- Why don't we have gene and stem cell therapy yet?
Prof. Dr. Annemieke Aartsma-Rus, dept. Human Genetics at LUMC
- Why is it important to publish negative results?
Prof. Dr. Annemieke Aartsma-Rus, dept. Human Genetics at LUMC
- Questions & Answers, moderated
Pat Furlong, President at PPMD USA, WDO Board Member

19:30 – 19:45

Break

19:45 – 20:30

- Improving the patient voice in therapy development
Elizabeth Vroom, Chair World Duchenne Organization
- Questions & Answers, moderated
Pat Furlong, President at PPMD USA, WDO Board Member
- Sharing insights, plenary wrap-up

FRIDAY

DECEMBER 03

18:00 – 18:10

Data

- The importance of data seen from various perspectives
George Paliouras, Chair Duchenne Data Foundation
Dr. BS Ajai Kumar, Exec. Chair at HealthCare, DDF Board Member
Elizabeth Vroom, Chair World Duchenne Organization
- How patient organizations can contribute to the collection and connection of health data
Elizabeth Vroom, Chair World Duchenne Organization
- Which tools are available to optimise the (re)use of health data?
Nawel van Lin, FAIR Project Manager
- What patient organizations can do to organize individual data return from clinical trials
Dr. Eric Hoffman, President & CEO at ReveraGen
Suzanne Gaglianone, Patient Coordinator at ReveraGen
Ritchie Head, Managing Director at Ceratium

19:30 – 19:45

Break

19:45 – 20:30

Scientific Information

- Finding, following and understanding scientific information
Deborah Robins, co-founder of Parent Project Australia
Elizabeth Vroom, Chair World Duchenne Organization
- Interactive session: How to translate these insights to your patient organization?
- Sharing insights, plenary wrap-up

SATURDAY

DECEMBER 04

15:00 – 15:45

Project updates

- DDF & WDO Project update pitches
Various presenters

Break

15:45 – 16:00

Returning individual clinical trial data

16:00 – 16:45

- An introduction to returning individual clinical trial data
Elizabeth Vroom, Chair World Duchenne Organization
- Patient preference on returning individual clinical trial data
Ritchie Head, Managing Director at Ceratium
- The role of CRO's in returning clinical trial patient data
*Dr. Martine Dehlinger-Kremer, Vice President of Scientific Affairs
Pediatric Subject Matter Expert, ICON Plc*
- Which tools are available to facilitate this work?
Sarah Longman, Technology Projects at Action Duchenne
- Panel discussion, moderated
*Elizabeth Vroom, Chair World Duchenne Organization
Dimitrios Athanasiou, MDA Hellas, Board Member WDO*

Break

16:45 – 17:00

Company updates

17:00 – 19:00

- Introduction to company updates and clinical trials
George Paliouras, Chair Duchenne Data Foundation
- Company updates to the community
Various companies
- Panel discussion, moderated
*George Paliouras, Chair Duchenne Data Foundation
Alessandra Gaeta, Director of Research Duchenne UK*
- Plenary wrap-up