

AGENDA

DUCHENNE
PATIENT ACADEMY

Monday Nov 30, 6.00pm – 8.30pm CET

Welcome

Room 1: Capacity building

Room 2: Community engagement

Tuesday Dec 1, 6.00pm – 8.30pm CET

Patient voice in regulation

Patient voice in policy

Wednesday Dec 2, 6.00pm – 8.30pm CET

Improving the patient's position in data collection

Thursday Dec 3, 6.00pm – 8.30pm CET

Room 1: Clinical trial preparedness

Room 2: Grassroots fundraising and impact investing

Friday Dec 4, 6.00pm – 8.30pm CET

DMD/BMD care update

Saturday Dec 5, 3.00pm – 8.00pm CET

Project updates

Community priorities

Optimise DMD coalition

Networking and poster session

Sunday Dec 6, 3.00pm – 6.00pm CET

Clinical trials update & COVID impact

Panel discussion on pipeline

Closing ceremony



MONDAY

NOVEMBER 30

6.00pm – 6.15pm

Welcome & Introduction

6.15pm – 7.45pm

Room 1: Capacity Building

- Community building, creating a common voice
Nicoletta Madia, WDO
- Advancing a Patient Organisation to the next level
Nicoletta Madia, WDO

7.45pm – 8.00pm

Break

8.00pm – 8.30pm

- Interactive session

6.15pm – 7.45pm

Room 2: Community Engagement

- How to create a community for young people with NMD
Jana Popova, SMA Europe
- How to build a research ecosystem in your disease
Bettina Ryll, Melanoma Patient Network Europe
- Dissemination of reliable information
Elizabeth Vroom, WDO

7.45pm – 8.00pm

Break

8.00pm – 8.30pm

- Interactive session

TUESDAY

DECEMBER 01

6.00pm – 7.15pm

Patient Voice in Regulation

- Patient voice in regulation
Kaisa Immonen, European Patient's Forum
- Presentation about the European Medicines Agency (EMA)
Elizabeth Vroom, WDO
- Presentation about the US Food and Drug Administration (FDA)
Pat Furlong, WDO
- Panel discussion
Moderator Dimitrios Athanasiou, WDO

7.15pm – 7.30pm

Break

7.30pm – 8.30pm

Patient Voice in Policy

- Coordinating global policy
Durhane Wong-Rieger, Chair Rare Disease International
- Patient voice in policy
Kaisa Immonen, European Patient's Forum
- Panel discussion
Moderator Durhane Wong-Rieger, Chair Rare Disease International

WEDNESDAY

DECEMBER 02

6.00pm – 6.50pm

Improving the Patient's Position in Data Collection

- Real World Evidence- importance for patient communities
Karen Facey, RWE4
- Data and patients
Julian Isla, Foundation29

6.50pm – 7.05pm

Break

7.05pm – 7.50pm

- DMD Community initiatives on data
FAIR data
Duchenne Map
Duchenne Data Repository
EURO-NMD Registry Hub
Share4Rare

7.50pm – 8.30pm

Challenges & Opportunity on NMD Data

- Panel: Challenges and opportunities on NMD data
Moderator Ryan Fischer, DDF; Elvina Sakellariou, DDF

THURSDAY

DECEMBER 03

6.00pm – 7.30pm

Room 1: Clinical Trial Preparedness

- What is clinical trial preparedness?
Dr. Martine Dehlinger-Kremer, European CRO Federation & PRA Health Sciences
- Clinical trial preparedness in your country: a HCP perspective
Dr. Jana Haberlova, Motol University Hospital
- Selection criteria for clinical trial centers: a CRO perspective
Nefeli Panousis, CRO advisor
- How patient advocacy groups can become involved
Alex Johnson, WDO

7.30pm – 7.45pm

Break

7.45pm – 8.30pm

- Panel: How to attract, start and run a clinical trial in your country
Moderator Dr. Martine Dehlinger-Kremer, European CRO Federation & PRA Health Sciences

Room 2: Grassroots Funding & Impact Investing

6.00pm – 7.30pm

- Grassroots funding and fundraising
Nicoletta Madia, WDO
- Access to impact investing and corporate sponsorship
Eleni Theodorou, WDO

7.30pm – 7.45pm

Break

7.45pm – 8.30pm

- Interactive session

FRIDAY

DECEMBER 04

6.00pm – 6.45pm

Care Update & Implementation

- Hurdles in implementation of Standards of Care
Elizabeth Vroom, WDO
Yvonne Krom, Duchenne Center Netherlands
- FAQ
Elizabeth Vroom, WDO

6.45pm – 7.00pm

Break

7.00pm – 8.30pm

- Why endocrinology is important
Dr. Jarod Wong, Glasgow University
- Adult care and smooth muscle
Prof. Ros Quinlivan, Great Ormond Street Hospital
- DMD and the brain
Dr. Jos Hendriksen, Kempenhaeghe

SATURDAY

DECEMBER 05

3.00pm – 4.10pm

Project Updates

- Update per project, various presenters

4.10pm – 4.25pm

Break

4.25pm – 5.10pm

Community Priorities

- Certified Duchenne Care Center: experience from Motol Hospital
Dr. Jana Haberlova, Motol University Hospital
- Newborn screening
Elizabeth Vroom, WDO

5.10pm – 6.10pm

Break

6.10pm – 6.55pm

Data & Post-Marketing Registries

- Challenges and opportunities in Data and Post-Marketing Registries
Anja Schiel, Chair EMA Scientific Advice Working Party
- The need for a patient centric Post Marketing Registry for Duchenne
Ryan Fischer, DDF
- Panel discussion: Data and Post Marketing Registry
Moderator Anja Schiel, Chair EMA Scientific Advice Working Party

6.55pm – 7.40pm

Optimise DMD Coalition

- The need for CT optimisation and regulatory harmonisation
Anja Schiel, Chair EMA Scientific Advice Working Party
- Optimising DMD: From research to access
Dimitrios Athanasiou, WDO
- Panel discussion: DMD Coalition
Moderator Dimitrios Athanasiou, WDO

7.40pm – 8.00pm

- Poster pitch sessions

SUNDAY

DECEMBER 06

3.00pm – 4.30pm

DMD Clinical Trials Update & COVID Impact

- Update per company
Various presenters

4.30pm – 4.45pm

Break

4.45pm – 5.45pm

- Panel discussion on drug development pipeline
Moderator Prof. Dr. Annemieke Aartsma-Rus

5.45pm – 6.00pm

Closing Ceremony

- Reflections, wrap-up
- Certificates