

# PROGRAM

## Thursday December 1, 15:00 – 19:00 CET

### Research

- Future considerations of dystrophin restoring therapies: What do we know, what can we expect?
- Updates from companies
- Moderated panel discussion

### Innovation

- Poster session: Innovation in Duchenne muscular dystrophy

## Friday December 2, 15:00 – 19:00 CET

### Advocacy

- How do we optimize patient advocacy?
- Case study presentations
- Moderated panel discussion

### Networking

- Changing perspectives on patient value in R&D
- Case study presentations
- Moderated panel discussion

## Saturday December 3, 15:00 – 19:00 CET

### Care Management

- Standards of Care and Care Guidelines
- Case study presentations
- Moderated panel discussion

### Allied Therapies

- What are allied therapies and why are they so important?
- Case study presentations
- Moderated panel discussion

15:00 – 17:45

17:45 – 18:00

18:00 – 19:00

19:00

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PATIENT ACADEMY

# THURSDAY

DECEMBER 01

*Times are shown in Central European Time*

## Topic 1: Research

- Welcome, introduction  
*Suzie-Ann Bakker, World Duchenne Organization*
- Keynote: Future considerations of dystrophin restoring therapies: What do we know, what can we expect?  
*Prof Annemieke Aartsma-Rus, Leiden University Medical Centre*
- Company updates  
*Various presenters*
- Moderated panel discussion with companies  
*Prof Annemieke Aartsma-Rus, Leiden University Medical Centre*

Break

## Topic 2: Innovation

- Introduction to poster session  
*Nawel Lalout, World Duchenne Organization*
- Poster presentations  
*Various presenters*
- Wrap-up & Closing  
*Suzie-Ann Bakker, World Duchenne Organization*

Wrap-up, closing

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## TOPIC 1

15:00 – 15:30

15:30 – 16:15

16:15 – 16:45

16:45 – 17:00

## FRIDAY

DECEMBER 02

*Times are shown in Central European Time*

## ADVOCACY

### Introduction

- How do we optimize patient advocacy?  
*Elizabeth Vroom, World Duchenne Organization*

### Presentations

- The Téléthon model  
*Francois Lamy, AFM Téléthon*
- Duchenne as a disability: using the UN Health Resolution as leverage to advocate for rights  
*Flaminia Macchia, Rare Diseases International*
- Connecting foundations in one country: experience of Chile  
*Nicolas Schongut Grollmus, Fundacion Duchenne Chile*
- The importance of communication for advocacy  
*Nicoletta Madia, World Duchenne Organization*
- How patient preference shapes clinical trials  
*Pat Furlong, Parent Project Muscular Dystrophy USA*
- Don't 'invite me to the table'. It's my table  
*Pat Furlong, Parent Project Muscular Dystrophy USA*

### Panel discussion

- Moderated panel discussion  
*Elizabeth Vroom, World Duchenne Organization*

Break

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## TOPIC 2

17:00 – 17:10

17:10 – 18:30

18:30 – 18:55

19:00

## FRIDAY

DECEMBER 02

*Times are shown in Central European Time*

## NETWORKING

### Introduction

- Changing perspectives on patient value in R&D  
*Dimitrios Athanasiou, World Duchenne Organization*

### Presentations

- Patient value for patient partnerships in project development  
*Nicoletta Madia, World Duchenne Organization*
- Engaging with clinical networks- the EURO-NMD and Registry Hub  
*Antonio Atalaia, ERN EURO-NMD*
- How to start engaging with policy-makers  
*Labrini Papageorgiou, Roche*
- Engaging regulatory & HTA bodies  
*Anja Schiel, Norwegian Medicines Agency*
- Engaging with actors involved in drug development  
*Martine Dehlinger-Kremer, ICON plc*

### Panel discussion

- Moderated panel discussion  
*Dimitrios Athanasiou, World Duchenne Organization*

Wrap-up, closing

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## TOPIC 1

15:00 – 15:25

15:25 – 16:30

16:30 – 17:00

17:00 – 17:15

## SATURDAY

DECEMBER 03

*Times are shown in Central European Time*

## CARE MANAGEMENT

### Keynote

- Standards of Care and Care Guidelines in Duchenne  
*Dr. Karolína Podolská, Parent Project Czech Republic*

### Presentations

- Patient-centered care and listening to the patient needs  
*Elizabeth Vroom, World Duchenne Organization*
- Collaboration between the clinic and patient organization  
*Dr. Marisol Montolio, Duchenne Parent Project Spain*
- How to keep the medical community up to date  
*Elizabeth Vroom, World Duchenne Organization*
- Patient perspective on gender specific medicine  
*Lizanne Schreur*
- Accredited Duchenne Center Program  
*Dr. Imelda de Groot, World Duchenne Organization*
- Inspiring the medical community in your area to become interested in Duchenne care  
*Dr. B.R.Lakshmi, MDCRC India*

### Panel discussion

- Moderated panel discussion  
*Dr. Karolína Podolská, Parent Project Czech Republic*

Break

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## TOPIC 2

17:15 – 17:30

17:30 – 18:30

18:30 – 18:55

19:00

## SATURDAY

DECEMBER 03

*Times are shown in Central European Time*

## ALLIED THERAPIES

### Keynote

- What are allied therapies and why are they so important?  
*Dr. Imelda de Groot, World Duchenne Organization*

### Presentations

- Physiotherapy in Duchenne  
*Dr. Linda Lowes, Nationwide Children's Hospital*
- Martial arts training  
*Mariska Janssen, Klimmendael*
- The role patient organizations play in accessible sports  
*René Břečťan, Parent Project Czech Republic*
- Brain training  
*Sam Geuens, KU Leuven*
- Hydrotherapy: surfing with Duchenne  
*Merav Hadar-Frumer, Little Steps*
- Speech and swallowing  
*Dr. Jodi Allen, University College London*

### Panel discussion

- Moderated panel discussion  
*Dr. Imelda de Groot, World Duchenne Organization*

Wrap-up, closing

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