

Dec 06 – 07, 2024  
duchennepatientacademy.org

DUCHENNE

PATIENT

Connecting DMD/BMD patient advocates  
of the future



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# PROGRAM

## DAY 1 FRIDAY DECEMBER 6

16:00 – 19:00 CET

*Welcome & introduction*  
*Updates on drug development*  
*Proving optimal care*

## DAY 2 SATURDAY DECEMBER 7

14:00 – 20:00 CET

*Welcome*  
*Research: Challenges in drug development*  
*Improving care*  
*Improving advocacy*  
*Patient involvement in clinical trials and regulatory processes*  
*Crisis communication*  
*Best practices in fundraising*  
*Reflection & Graduation*

# SPONSORS



FRIDAY

DECEMBER 06

*Times are shown in Central European Time*

16:00 – 16:15

Welcome

- Welcome  
*Elizabeth Vroom, World Duchenne Organization*
- Introduction to Duchenne Patient Academy 2024  
*Nicoletta Madia, World Duchenne Organization*

TOPIC 1

## DRUG DEVELOPMENT

16:15 – 18:00

Updates on Duchenne Drug Development

- Introduction to the session  
*Elizabeth Vroom, World Duchenne Organization*
- Company presentations  
*Various presenters*
- Panel discussion, moderated  
*Elizabeth Vroom, World Duchenne Organization*

18:00 – 18:15

Break

FRIDAY

DECEMBER 06

*Times are shown in Central European Time*

TOPIC 2

## PROVIDING OPTIMAL CARE

18:15 – 18:55

Providing Optimal Care

- Introduction to the session  
*Elizabeth Vroom, World Duchenne Organization*
- Why it's important to have Standards of Care in place  
*Prof Eugenio Mercuri, Policlinico Gemelli*
- Accredited Duchenne Centers (ADC) Program  
*Dr Karolina Podolska*
- Duchenne Care Conference  
*Elizabeth Vroom, World Duchenne Organization*
- Questions & Answers  
*Elizabeth Vroom, World Duchenne Organization*

18:55 – 19:00

Wrap-up

- Wrap-up & Agenda for next day  
*Elizabeth Vroom, World Duchenne Organization*

# SATURDAY

DECEMBER 07

*Times are shown in Central European Time*

## Welcome

- Welcome and introduction  
*Elizabeth Vroom, World Duchenne Organization*

14:00 – 14:10

## TOPIC 3 RESEARCH & DEVELOPMENT

### Challenges in Drug Development

- Introduction to the session  
*Dr Karolina Podolska, World Duchenne Organization*
- Challenges in Duchenne drug development  
*Prof Annemieke Aartsma-Rus, Leiden University Medical Center*
- Questions & Answers  
*Dr Karolina Podolska, World Duchenne Organization*

14:05 – 14:50

## TOPIC 4 IMPROVING CARE

### Providing Optimal Care

- Introduction to the session  
*Elizabeth Vroom, World Duchenne Organization*
- What POs can play a role in improving care  
*Prof Ros Quinlivan, University College London*
- Questions & Answers  
*Elizabeth Vroom, World Duchenne Organization*

14:50 – 15:20

Break

15:20 – 15:40

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# SATURDAY

DECEMBER 07

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## TOPIC 5 IMPROVING ADVOCACY

15:40 – 16:10

### National & International Community Building

- Introduction to the session  
*Nicoletta Madia, World Duchenne Organization*
- Case study presentations  
*Various speakers*
- Questions & Answers  
*Nicoletta Madia, World Duchenne Organization*

## TOPIC 6 PATIENT INVOLVEMENT

16:10 – 17:15

### In Drug Development and Regulatory Processes

- Introduction to the session  
*Dimitrios Athanasiou, World Duchenne Organization*
- Patient involvement in the EMA  
*Maria Mavris, European Medicines Agency*
- Patient involvement in the FDA  
*Pat Furlong, PPMD USA*
- Managing Conflict of Interest  
*Dimitrios Athanasiou, World Duchenne Organization*
- Duchenne Community Advisory Board  
*Sally Hofmeister, World Duchenne Organization*
- Questions & Answers  
*Dimitrios Athanasiou*

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# SATURDAY

DECEMBER 07

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Break

## CRISIS COMMUNICATION

### Communicating in Urgent Situations

- Introduction to the session  
*Elizabeth Vroom, World Duchenne Organization*
- Crisis communication  
*Nicoletta Madia, World Duchenne Organization*
- How to handle crises in drug development  
*Pat Furlong, PPMD USA*
- Questions & Answers  
*Elizabeth Vroom, World Duchenne Organization*

## FUNDRAISING

### Best Practices in Fundraising

- Introduction to the session  
*Nicoletta Madia, World Duchenne Organization*
- Case study presentations  
*Various speakers*
- Questions & Answers  
*Nicoletta Madia, World Duchenne Organization*

# SATURDAY

DECEMBER 07

*Times are shown in Central European Time*

19:35 – 19:55

### Sharing Insights & Graduation Ceremony

- Roundtable: Lessons Learnt  
*Suzie-Ann Bakker, World Duchenne Organization*
- Graduation Ceremony  
*Elizabeth Vroom, World Duchenne Organization*

19:55 – 20:00

### Wrap-up

- Wrap-up & Goodbye  
*Elizabeth Vroom, World Duchenne Organization*

17:15 – 17:35

## TOPIC 7

17:35 – 18:30

## TOPIC 8

18:30 – 19:35