



**eNMD**  
**2023**

**4<sup>th</sup>**

**ENMD**

**Congress**

CONGRESS IN E-HEALTH & INNOVATION  
TO OVERCOME BARRIERS IN NEUROMUSCULAR  
DISEASES

2<sup>nd</sup> - 4<sup>th</sup> november 2023

**MUNICH**

Digital biomarkers and digital therapies:  
New solutions for New Avenues  
in Neuromuscular Disorders





# 4<sup>th</sup> ENMD Congress

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## Scientific Committee

Benedikt Schoser (DE)  
Sabrina Sacconi (FR)  
Gabriele Siciliano (IT)

## Local Organizing Committee

Federica Montagnese (DE)

## eNMD Advisory Board

Gabriele Siciliano, (IT)  
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Sandrine Segovia-Kueny (FR)  
Antonio Toscano (IT)

## Speakers and Chairs

Paola Alberti (IT)  
Corrado Angelini (IT)  
António Marques Atalaia (FR)  
Mohammad Bakhtiari (DE)  
Jan Baumbach (DE)  
Stephen Dando (UK)  
Martin Daumer (DE)  
Nicolas Dubuisson (BE)  
Katrin Hahn (DE)  
Katy Hamana (UK)  
Jean-Yves Hogrel (FR)  
Tiffany House (USA)  
Olivier Lambercy (FR)

Sophie Lehnerer (DE)  
Hanns Lochmüller (CA)  
Marco Lorenzi (IT)  
Claudia Mazzà (IT)  
Arthur Miller (UK)  
Federica Montagnese (DE)  
Anne Mühe (DE)  
Yann Pereon (FR)  
Niklas Probul (DE)  
Francesco Saccà (IT)  
Valeria Sansone (IT)  
Angelo Schenone (IT)  
Benedikt Schoser (DE)

Eric Schott (DE)  
Gabriele Siciliano (IT)  
Susan Sparks (USA)  
Barbara Tettenborn (CH)  
Antonio Toscano (IT)  
John Vissing (DK)  
Nicole Voet (NL)  
Stephan Wenninger (DE)  
Heinz Wiendl (DE)  
Andreas Ziegler (DE)  
Jana Zschüntzsch (DE)





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## Thursday, 2<sup>nd</sup> of November

11:00 Registration

### Pre-Industry Satellite Symposium | apps and tools for NMD

Chair: *Benedikt Schoser (DE)*

12:30 New digital tools for neuromuscular disease patients: where do we stand?

Focus on myotonia

**Sponsored by LUPIN NEUROSCIENCES**

*Yann Pereon (FR)*

13:00 "Tiny Steps" towards a more active lifestyle in the realm of neuromuscular diseases

**Sponsored by ALEXION**

*Anne Mühe (DE)*

Sponsored session

13:30 Snack Lunch

### 14:00 Welcome and Introduction

*Benedikt Schoser (DE)*

### Topic 1 Digital monitoring and databases

Chairs: *Gabriele Siciliano (IT), Benedikt Schoser (DE)*

14:15 Disease monitoring in 2030

*Angelo Schenone (IT)*

14:45 Data sharing on web-based platforms - how to overcome classic hurdles?

*Jan Baumbach (DE)*

15:15 Treatabome database: a variant-based decision-making aid for treating rare and neuromuscular disorders

*António Marques Atalaia (FR)*

15:45 Industry demands in clinical trials

*Susan Sparks (USA)*

16:15 Coffee break

## Topic 2 Digital biomarkers

Chairs: Valeria Sansone (IT), Stephan Wenninger (DE)

**16:45** How to develop digital biomarkers  
*Martin Daumer (DE)*

## Oral Communications

Chairs: Valeria Sansone (IT), Stephan Wenninger (DE)

**17:15 Oral 1** | Sensorless Computer Vision-Based Motor Function Assessment: Revolutionizing Patient Evaluation with AI and Videos Taken by Smart Devices  
*Qi Tang*

**17:30 Oral 2** | Objective Monitoring of Facioscapulohumeral Dystrophy During Clinical Trials using a Smartphone Application and Wearables: Observational Study  
*Ghobad Maleki*

**17:45 Oral 3** | Evaluation of the nation-wide implementation of telemonitoring in the care for patients with ALS: ALS Home Monitoring and Coaching  
*Esther Kruitwagen van Reenen*

**18:00 Oral 4** | Computational Models for new Patients Stratification Strategies of Neuromuscular Disorders: a new strategy to tackle hereditary neuromuscular disorders  
*Rossella Tupler*

## Evening Keynote Lecture

**18:15 Generative AI creates art and literature but is it the real thing?**  
*Arthur Miller (UK)*

**19:00 Welcome Cocktail**

## Friday, 3<sup>rd</sup> of November

### Industry Satellite Symposium Sponsored by BIOGEN

**08:00** New digital solutions for SMA patient From disease monitoring to treatment support  
*Andreas Ziegler (DE)*

**08:30** Assessing the clinical validity of Konectom™ in adults living with Spinal Muscular Atrophy  
*Claudia Mazzà (IT)*

**09:00** End of the session

Sponsored session

**09:15 Introduction of Day 2**  
*Benedikt Schoer (DE)*

## Topic 3 Device Development

Chairs: Jean-Yves Hogrel (FR), Katrin Hahn (DE)

**09:20** Neuromuscular devices - what do we have in place already?  
*Jean-Yves Hogrel (FR)*



## Oral Communications

Chairs: *Jean-Yves Hogrel (FR), Katrin Hahn (DE)*

**09:50 Oral 1** | Exploring the role of Mobile Health Technologies in detecting subtle motor impairment in paucisymptomatic Late-onset Pompe Disease: a preliminary study  
*Baetrice Labella (IT)*

**10:05 Oral 2** | Wearable devices for the biomechanical investigation of DMD during the 6MWT: preliminary results  
*Elisa Panero (IT)*

**10:20 Oral 3** | InGene 2.0: How a technological platform supports the clinicians in the diagnosis and treatment of neuromuscular disorders  
*Alessandro Tonacci (IT)*

**10:35 Oral 4** | Usability of a mobile health application to support home-based aerobic exercise in neuromuscular diseases  
*Eric Voorn (NL)*

**10:50** Discussion

**11:00** Coffee break

## Topic 4 Remote monitoring and telemedicine

Chairs: *António Marques Atalaia (FR), Antonio Toscano (IT)*

**11:30** Remote monitoring - an unmet need for patients and clinicians  
*Gabriele Siciliano (IT)*

**12:00** Digital monitoring of myasthenia gravis  
*Sophie Lehnerer (DE)*

**12:30** Public and private incentives for medical innovation: an ecosystem perspective  
*Eric Schott (DE)*

**13:00** End of the session

## Industry Lunch Satellite Symposium

Sponsored by AMICUS

Harnessing Digital Technology to Optimise Patient Care in the Long-Term Management of NMD

**13:30** Welcome and introduction

**13:35** Introduction to patient-centric disease management  
*Stephen Dando (UK)*

**13:40** Co-creation of digital content by HCPs and patients/caregivers  
*Katy Hamana (UK)*

**13:50** Case study: An example of how digital technology has supported patients with their physiotherapy  
*Stephen Dando (UK)*

**14:00** How can we harness digital technologies to optimise patient management?  
*Stephen Dando (UK), Katy Hamana (UK)*

**14:15** Q&A

Sponsored session



## Workshop 1 | Digital platforms for data sharing and decentralized mining

Chair: *Jan Baumbach (DE)*

**14:30** FeatureCloud: Federated Learning in Biomedicine  
*Mohammad Bakhtiari (DE)*

**15:00** Applied Federated Learning for Colorectal Cancer Detection  
*Niklas Probul (DE)*

**15:30** Coffee break

## Workshop 2 | e-Education in eNMD and beyond

Chair: *Corrado Angelini (IT)*

**16:00** EAN educational platform  
*Barbara Tettenborn (CH)*

**16:20** PNS educational platform  
*Paola Alberti (IT)*

**16:40** Discussion

### Industry Satellite Symposium

Sponsored by **Argenx**

Innovations to overcome barriers in myasthenia gravis

**17:00** Welcome and introduction  
*Heinz Wiendl (DE)*

**17:05** Innovations in diagnosis and assessment  
*Francesco Saccà (IT)*

**17:20** Innovations in therapy  
*Jana Zschüntzsch (DE)*

**17:35** Innovations in practice  
*Heinz Wiendl (DE), Francesco Saccà (IT), Jana Zschüntzsch (DE)*

**17:50** Q&A and closing  
*Heinz Wiendl (DE), Francesco Saccà (IT), Jana Zschüntzsch (DE)*

**18:00** Speed dating - poster session - networking sessions

Chairs: *Eric Schott (DE), Benedikt Schoser (DE), Hans Lochmuller (CA)*

**19:00** End of the session

Sponsored session



## Saturday, 4<sup>th</sup> of November

09:00 Introduction of Day 3  
*Benedikt Schoser (DE)*

### Topic 5 Technical therapeutics

Chairs: *Federica Montagnese (DE), Nicolas Dubuisson (BE)*

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09:05 Technical solutions for mobility and rehabilitation  
*Olivier Lambercy (CH)*

09:35 Poster pitches  
Chair: *Hans Lochmuller (CA)*

10:15 Coffee break and Poster Viewing

### Topic 6 eNMD in the real world

Chairs: *John Vissing (DK), Nicole Voet (NL)*

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10:45 Biomedical data integration in neurodegenerative disorders: challenges and promise  
*Marco Lorenzi (IT)*

11:15 Digital biomarkers and digital therapies for neuromuscular diseases: high-tech and low touch?  
*Nicole Voet (NL)*

11:45 *Digital integrated care solutions for the neuromuscular community - presence and future*  
*Andreas Ziegler (DE)*

12:15 Patients voices on e-health  
*Tiffany House (USA)*

12:45 Best Oral & Best Poster Presentation Awards  
Congress wrap-up and farewell  
*Benedikt Schoser (DE)*



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# POSTER SESSION >>



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## POSTER SESSION

Chair: *Hans Lochmuller (CA)*

### Technical therapeutics

**P01 - Dose selection and clinical development of efgartigimod PH20 subcutaneous (SC) in patients with generalized myasthenia gravis (gMG)**

*George Li, Yuebing Li, Tuan Vu, Denis Korobko, Marek Smilowski, Krzysztof Banaszkiwicz, Li Liu, Sophie Steeland, Benjamin Van Hoorick, Jana Podhorna, Jenna Casey, Jan Noukens, Tonke Van Bragt, Kimiaki Utsugisawa, Heinz Wiendl, Jan L. De Bleecker, Renato Mantegazza, James F. Howard, Jr, and the ADAPT-SC Study Group*

**P02 - Long-term safety, tolerability, and efficacy of subcutaneous (SC) efgartigimod PH20 in patients with generalized myasthenia gravis (gMG): interim results of the ADAPT-SC+ study**

*Heinz Wiendl, James F. Howard, Jr, George Li, Tuan Vu, Denis Korobko, Marek Smilowski, Krzysztof Banaszkiwicz, Li Liu, Sophie Steeland, Jan Noukens, Benjamin Van Hoorick, Jana Podhorna, Yuebing Li, Kimiaki Utsugisawa, Francesco Sacca, Jan L. De Bleecker, Renato Mantegazza, and the ADAPT-SC Study Group*

### eNMD in the real world

**P03 - Innovation in Treatment of Generalized Myasthenia Gravis: From Trial Design to Real-World Clinical Practice**

*Andreas Meisel, Kathy Perez, Cynthia Qi, Filip Callewaert, Sabine Holzleitner*

**P04 - Development of an auto-questionnaire to measure the impact of non-dystrophic myotonia on patients' daily living and quality of life**

*Ahmed A., Zozulya-Weidenfeller A., Ezaru A., Pini J., Sacconi S.*

**P05 - unMASC NDM Myotonia Symptom Checker:**

**A new tool to raise public awareness of myotonia**

*Péron Y., Roddate M., Dominguez-Gonzalez C., Zozulya-Weidenfeller A., Mongini T.*

### Remote monitoring and telemedicine

**P06 - Multimodal tele-rehabilitation in two patients with DMD**

*Parravicini Stefano; Gardani Alice; Vacchini Valeria; Berardinelli Angela*

**P07 - Development of a Myasthenia Gravis Symptom Tracking Mobile Application**

*Elena Cortes, Bibiana Da Rocha, Julien Lebleu*

**P08 - Facilitators of and Barriers to integrating telemonitoring in ALS care: a nationwide implementation study of ALS Home Monitoring & Coaching**

*A.K. Schmidt, E.T. Kruitwagen-van Reenen, M.L. Dontje, E. van Wijk, E.C.T. Baars, R.P.A. van Eijk, J.M.A. Visser-Meily, A. Beelen on behalf of the Study Group ALS Home monitoring & Coaching*



## Digital monitoring and data bases, Digital biomarkers

### **P09 - FAIR from the start: FISMA for high quality and interoperable real-world data on dystrophinopathy**

*[Y.D.Krom](#); N.M. van de Velde; S. Houwen-van Opstal; M. van der Holst; E.H. Niks; R.R. Snijder*

### **P10 - From concept to reality: FISMA as a framework for the Dutch biobank for dystrophinopathies**

*[Y.D.Krom](#); N.M. van de Velde; S. Houwen-van Opstal; M. van der Holst; R.R. Snijder; E.H. Niks;*

### **P11 - Development of AUTOMA 2.0: an innovative wearable platform to assess the muscular activity of patients affected by Neuromuscular Disorders**

*Andrea Vannini, Roberto Lazzarini, Mario Milazzo, [Francesco Sansone](#), Guja Astrea, Silvia Frosini, Francesca Torri, Roberto Chiappini, Giulia Ricci, Gabriele Siciliano, Filippo Maria Santorelli, Raffaele Conte, Stefano Roccella*

### **P12 - Screen4Care: A project to change the diagnostic landscape of rare diseases in Europe**

*Zschüntzsch J., Lochmüller H., Garnier N., Ferlini A., Alves F., Rudolf Blankart, Richard Röttger, Janbernd Kirschner, Lizbeth Goodman and Screen 4Care consortium*

### **P13 - Amyotrophic Lateral Sclerosis Holistic Outpatient Exercise (ALS HOPE) Group**

*[Virginia Kudritzki](#), DPT*

*Co-author: Ileana Howard, MD*

### **P14 - Supporting Physical Exercise in patients with Neuromuscular Diseases: AI- assisted training**

*Berardina De Carolis, [Giuseppe Palestra](#), Erika Schirinzi, Gabriele Siciliano, Mario A. Bochicchio  
Department of Informatics University of Bari Aldo Moro - Italy*

### **P15 - An ICT suite of tools for for deep phenotyping and genotype correlation in neuromuscular diseases: the Phenotype module**

*Francesco Sansone, Gianluca Diodato, Maria Cristina Scudellari, Alessandro Tonacci, Stefano Roccella, Andrea Vannini, Bianca Buchignani, Anna Rubegni, [Giulia Ricci](#), Gabriele Siciliano, Francesca Torri, Gian Nicola Gallus, Diego Lopergolo, Alessandro Malandrini, Sabrina Matà, Martina Sperti, Corrado Angelini, Raffaele Conte*

### **P16 - Artificial-Intelligence fosters the muscular MRI interpretation by the clinician: a proof-of-concept study**

*Raffaele Conte, Gianluca Diodato, Maria Cristina Scudellari, Alessandro Tonacci, Stefano Roccella, Mario Milazzo, [Guja Astrea](#), Filippo Maria Santorelli, Giulia Ricci, Gabriele Siciliano, Francesca Torri, Giacomo Aringhieri, Diego Lopergolo, Alessandro Malandrini, Sabrina Matà, Martina Sperti, Corrado Angelini, Francesco Sansone*

### **P17 - Application of digital tools in a management model for transition phase from paediatric to adult care in dystrophinopathies**

*[Francesca Torri](#), Alessandro Tonacci, Francesco Sansone, Michele Sacchini, Chiara Ticci, Guja Astrea, Roberta Battini, Roberto Chiappini, Gabriele Vadi, Raffaele Conte, [Giulia Ricci](#), Gabriele Siciliano*



## Digital biomarkers, Device Development

### **P18 - Wearable sensors to measure indirect fatigue in Myotonic Dystrophy (DM1A)**

*Eleonora Diella, Fabio Storm, Luca Molteni, Morena Delle Fave, Rossella Cima and Maria Grazia D'Angelo*

### **P19 - Wearable-devices assisted clinical protocol for assessment of fatiguability in SMA**

*Giulia Ricci, Stefano Roccella, Francesca Torri, Roberto Chiappini, Alessandro Tonacci, Francesco Sansone, Raffaele Conte, Gabriele Siciliano*

### **P20 - A novel technology-based tool for performing motor assessment in neuromuscular disorders**

*Raffaele Conte, Gianluca Diodato, Maria Cristina Scudellari, Alessandro Tonacci, Stefano Roccella, Andrea Vannini, Silvia Frosini, Roberto Chiappini, Giulia Ricci, Gabriele Siciliano, Francesca Torri, Diego Loperголо, Alessandro Malandrini, Sabrina Matà, Martina Sperti, Corrado Angelini, Francesco Sansone*



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# SPEED DATING >>



**eNMD**  
**2023**



**November 3<sup>RD</sup>, FROM 18:00 to 19:00**

This "Speed Dating" session will create an opportunity of a one-to-one meeting between you and the major Key Opinion Leaders on the Neuromuscular Diseases.

All sessions will be 6 minutes long and will focus on the topics and questions you are most fascinated about.

If interested, you need to book an appointment in the speed date agenda at the following link: <https://enmd-neuromuscular.eu/one-to-one/>

Here following, the professors and professionals involved in this activity:

**Benedikt Schoser** - Professor of Neurology at Friedrich-Baur-Institute, LMU Munich

■ *Topics: Digital monitoring and databases, Digital Biomarkers, Clinical trials*

**John Vissing** - Professor of Neurology at the University of Copenhagen

■ *Topics: eNMD in real world, Digital Therapies, Clinical trials*

**Gabriele Siciliano** - Director of Neurology at the Pisa Hospital University, Pisa

■ *Topics: Remote Monitoring and Telemedicine, Device Development, Clinical trials*

**Eric Schott** - CEO of Campana & Schott and honorary professor for innovation management at the TU Berlin and the Goethe University Frankfurt, Germany

■ *Topics: E-commerce, AI and digital solutions, Startups*

**Susan Sparks** - Global Head, Medical Rare Neurometabolic, Sanofi, USA

■ *Topics: Industry clinical trials, Digital tools for Metabolic Myopathies, Registries*

**Björn Schmid** - Medical Lead Neuromuscular Diseases, New Customer Solutions & Pipeline, Biogen Germany

■ *Topics: Digital solutions in SMA, patient centricity*

**Markus Peceny** - Senior Country Medical Director, Amicus Therapeutics, Germany, Austria & Switzerland

■ *Topics: Industry clinical trials and independent clinical trial support, Automated patient database screening, Registries*



# General Info

## CONGRESS VENUE

### MBW MEDIENBERATUNG DER WIRTSCHAFT

Max-Joseph-Straße 5,  
80333 München, Germany



## ORGANIZING SECRETARIAT

First Class - Meetings and Conferences  
Via Vittoria Colonna 40 - 20149 Milan  
Ph. +39 02 30066329  
segreteria\_milano@fclassevents.com  
www.fclassevents.com

The Organizing Secretariat will be available to the participants at the congress venue during the following times:

- Thursday, November 2<sup>nd</sup> from 10:00 to the end of the works
- Friday, November 3<sup>rd</sup> from 07:00 to the end of the works

## REGISTRATION

Congress attendance is subject to online registration by Monday October 30th, 2023. After the deadline, it will be possible to register directly at the congress venue.

Regular

€ 275,00

Under 35

€ 200,00

*All prices mentioned are in Euro, VAT 19% is not included*

## Registration includes:

- Participation in all scientific sessions
- Congress materials and a name badge
- Access to the exhibition area and poster area
- Coffee breaks and lunches, according to the programme
- Certificate of Attendance

## PAYMENT METHOD

- Online - Bank transfer and credit card
- Onsite - ATM and credit card

## BADGE

At the time of registration, each registered participant will be given a name badge which must always be shown in order to access the classrooms where the scientific sessions are held, the exhibition area and the catering services.

## CERTIFICATE OF ATTENDANCE

The certificate of participation can be downloaded online after completing the evaluation questionnaire. The Organizing Secretariat will send an e-mail to all participants on November 6th containing useful information to download it.

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**EXHIBITION**  
**AREA >>**



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- E-Poster Totem
- One to one Meeting

**BOOTH LEGEND**

- 1 - ALEXION
- 3 - AMICUS THERAPEUTICS
- 4 - ARGENX
- 5 - BIOGEN
- 2 - LUPIN
- 7 - KEDRION
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Max-Joseph-Straße 5,  
80333 München, Germany

[www.enmd-neuromuscular.eu](http://www.enmd-neuromuscular.eu)

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Organizing Secretariat / CME Provider



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