



# Advancing the management of Duchenne muscular dystrophy: the role of real-world evidence

A virtual symposium at ICNMDigital

Sunday 13 September, 16:00–17:00 CEST



**Professor Juan Vilchez (Chair)**

Hospital Universitario y Politécnico La Fe  
CIBERER  
Valencia, Spain



**Professor Annemieke Aartsma-Rus (Speaker)**

Department of Human Genetics  
Leiden University Medical Center  
Leiden, Netherlands



**Professor Eugenio Mercuri (Speaker)**

Professor of Pediatric Neurology  
Università Cattolica del Sacro Cuore  
Rome, Italy

**Please join our expert faculty in a peer-to-peer interactive discussion on the diagnostic considerations and recent real-world evidence for the management of nonsense mutation Duchenne muscular dystrophy (nmDMD).**

This virtual symposium has been designed to explore the following areas:

- the impact of delaying Duchenne muscular dystrophy (DMD) milestones on disease progression
- the importance of obtaining an accurate genetic diagnosis for patients with DMD
- the benefits of long-term real-world evidence (RWE) for evaluating treatment effects and informing future management of patients
- the use of propensity score matching for comparing RWE and natural history data
- the latest RWE regarding ataluren use in patients with nonsense mutation DMD.<sup>a</sup>

We hope that this engaging symposium will provide you with insights relevant to your practice in managing patients with DMD.

<sup>a</sup>Ataluren is indicated for the treatment of nmDMD in ambulatory patients aged  $\geq 2$  years in member states of the European Union, and Iceland, Israel, Kazakhstan, Liechtenstein, Norway and the Republic of Korea, or aged  $\geq 5$  years in Brazil and Chile. Registration conditions differ internationally and prescribing information may vary depending on local approval in each country.

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16:00–16:05	Welcome and introduction Professor Juan Vilchez, Valencia, Spain
16:05–16:25	Obtaining an accurate DMD genetic diagnosis Professor Annemieke Aartsma-Rus Leiden, Netherlands
16:25–16:50	Examining real-world evidence for the treatment of nmDMD Professor Eugenio Mercuri, Rome, Italy
16:50–17:00	Q&A session Professor Juan Vilchez, Valencia, Spain