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

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.

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

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

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).

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

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