Multistakeholder workshop on Real-World Data collection in Duchenne Muscular Dystrophy

25 September 2026, 09:00 – 17:30 (CET/CEST)
In-person at the EMA building, Amsterdam (Room TBD) + virtual enabled

Organised in collaboration with the World Duchenne Organisation

Objectives of the workshop

This joint HMA/EMA hybrid workshop is anchored in the Network Data Steering Group workplan and follows on stakeholders' interactions to support medicines regulation and access in the field of Duchenne Muscular Dystrophy (DMD).

Drug development in DMD remains complex. Despite existing and past efforts on several levels, the challenges persist, including the generation of relevant[1] and high-quality evidence to support regulatory decisions. This workshop, supported by the World Duchenne Organisation, aims to discuss current gaps and hurdles in data collection, and to explore how we can all collaborate to enable the generation of meaningful evidence on the effectiveness and safety of medicines.

The event brings together representatives from patient organisations, regulatory agencies, healthcare professional organisations, data holders, academia, non-for-profit research institutions, pharmaceutical companies, health technology assessment bodies and payers with the following objectives:

- Taking stock on current challenges from the perspectives of different stakeholders and lessons learnt from past experiences in using Real-World Data (RWD) in DMD.
- Discussing how to leverage the output of the EMA-funded project on key data elements for regulatory use.
- Aligning on actionable recommendations and collaboration to support the collection and use of fit-for-purpose RWD for regulatory decision-making in this disease area, with a focus on data quality, patient consent, governance for data access and sharing, and registry interoperability.

[1] As defined in the Data Quality Framework for EU medicines regulation: application to Real-World Data.

Multistakeholder workshop on Patient Registries for Duchenne Muscular Dystrophy - Programme

08:30 Joining and technical checks

09:00 Welcome and opening remarks

Welcome to the workshop	**5'**
Opening remarks from EMA Executive Director	**5'**
Opening remarks from HMA	**5'**
Opening remarks from the European Commission	**5'**

09:20 Session 1: Current challenges in clinical development and regulatory decision-making

The lived experience of DMD - Patient voice	**20'**
Introduction on DMD and current therapies	**20'**
Stakeholders' perspectives	**80'**

11:20 Coffee Break

11:50 Session 2: Potential of real-world data in DMD

EMA funded project on the fitness for use of RWD sources	**50'**
Stakeholders' perspectives on the project's findings	**35'**
Instructions for the afternoon breakout sessions	**5'**

13:20 Event photo and lunch break

14:00 Session 3: Parallel breakout sessions (no live broadcast)

Participants will be divided into three parallel breakout sessions	**90'**

15:30 Coffee break and move back to plenary room

16:00 Session 4: Panel discussion and next steps

Feedback from breakout sessions	**30'**
Input from the Audience (slido)	**50'**
Panel discussion and next steps	
Questions & Answers	

17:20 Closing remarks from the Co-Chairs

10'