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European Medicines Agency

Real-world data collection in Duchenne muscular dystrophy workshop

High-level summary

On 25 September 2026, the Heads of Medicines Agencies (HMA) and the European Medicines Agency (EMA), in collaboration with World Duchenne Organization (WDO), organised a multistakeholder workshop. The aim was to discuss current challenges and gaps in clinical development and real-world data collection in Duchenne muscular dystrophy (DMD), and to explore how stakeholders can work together to generate evidence on the effectiveness and safety of medicines for regulatory purpose.

As for all rare, severe and progressive neuromuscular diseases, DMD continues to place an immense burden on patients, their families and caregivers throughout life. Timely access to safe, effective, and of high-quality medicines is therefore critical. Whilst clinical trials remain the cornerstone of evidence generation, these are challenging in DMD due to the small population of patients available for recruitment. In addition, the long and heterogenous disease progression makes their design particularly difficult.

Real-world data (RWD) can help in addressing knowledge gaps across the entire patient journey from the understanding of the disease natural history to the impact of the evolving therapeutic landscape on the disease trajectory in different population subgroups, as well as being a reliable tool for long-term observation. Numerous well-established RWD sources exist in the DMD field. However, to support regulatory decision-making, these must be fit for purpose to answer questions on medicines, while remaining meaningful to patients.

To address the above challenges and requirements, the workshop brought together representatives from patient organisations, regulatory agencies, healthcare professional organisations, data holders, academia, non-for-profit research institutions, pharmaceutical companies and health technology assessment bodies.

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After listening to the lived experience from an adult patient, learning about the challenges in clinical development and data collection, and hearing the perspectives from all stakeholder groups on the evidence gaps and the opportunities offered by RWD to address these gaps, the participants agreed on a series of key principles.

- RWD are critical to accelerate development and support post-approval evidence generation of DMD medicines. No single stakeholder alone can unlock the full value of Real-World Evidence (RWE). Therefore, collective efforts and shared commitments will help build a scientifically robust, trustworthy, sustainable and interoperable DMD data ecosystem capable of supporting regulatory, HTA and clinical decisions.
- Stakeholders share a common vision on the need to create high-quality, patient-centred, fit-for-purpose evidence across the entire DMD journey, from diagnosis through adulthood. This should be supported by the development of a realistic core dataset and harmonised outcome definitions drawn from clearly defined priority regulatory questions and taking account of outcomes which are meaningful to patients. The EMA funded project on *Description and assessment of fitness-for-purpose of RWD sources on DMD for regulatory decision-making* was considered the starting point on which to build upon.
    - Stakeholders, in particular patients, caregivers and healthcare professionals must be involved in the development of harmonised outcome definitions and core dataset to be collected in RWD sources. This will ensure that the data reflect what truly matters to patients in daily life, and that they are representative of real clinical practice through the full lifespan of DMD including relevant disease milestones.
    - Given the evolving treatment landscape and disease progression trajectories, the outcome definitions and related core dataset should be flexible to remain relevant over time. They need to be updated periodically under scientific consensus to ensure their suitability to respond to arising research questions.
- Training opportunities for patients, clinicians, and data holders should be created and maximised to promote the value of data collection in a standardised manner, improve precision of data entry and facilitate data sharing.

Next steps

The workshop participants converged on a number of collective actions to be translated into a **roadmap** with detailed deliverables, related timelines and roles/responsibilities to advance evidence generation and support the development and monitoring of medicines that improve clinical care and the quality of life of DMD patients.

In addition, EMA plans to establish a multistakeholder Duchenne muscular dystrophy platform to take the workshop recommendations and actions forward.

Ultimately, success should be measured not by the amount of data collected, but by their quality and the collective ability to turn these data into better evidence, supporting better therapy developments, better decisions, better access, and better lives for people living with Duchenne muscular dystrophy.