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Stakeholders' perspectives

•German DMD

HMA/EMA Multistakeholder Workshop on Real-World Data Collection in September 25, 2026 | Marcel Heidemann



Stakeholders' perspectives

German DMD Registry

- Patient-reported registry established in 2008 at Friedrich-Baur-Institut Munich (PI Prof. Maggie Walter)
- Today over 2,000 participants incl. Becker and carriers
- TREAT-NMD membership provides crucial support and alignment with key stakeholders
- Initially aimed at trial readiness with focused dataset; now shifting to collection of RWD
- Patient-reported data collection is a vital component of RWD, but challenging in terms of reliability and extensiveness
- Possible resources for registries:
  - Data quality objectives that prospectively guide registries
  - Platforms, toolkits and best practices for patient-facing data collection (cf. PaLaDIn)
  - Infrastructure for privacy-preserving data linkage and data visiting
  - Long-term financial support for registry setup and sustainable operation (independent of data access fees)

Classified as internal/staff & contractors by the European Medicines Agency