The French National Dystrophinopathies Registry (DYS)

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Background

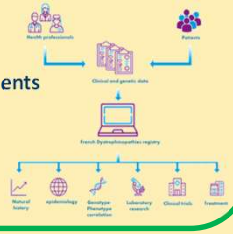
Dystrophinopathies, including Duchenne (DMD) and Becker (BMD) muscular dystrophies, are rare, progressive neuromuscular disorders caused by pathogenic variants in the dystrophin gene. In the absence of curative treatments, advances in patient care and clinical research rely on detailed, high-quality and standardized clinical and genetic data.

Objectives of the registry

The DYS registry aims to centralize harmonized national data on dystrophinopathies. It supports clinical research, improves understanding of disease presentation and progression, and contributes to clinical trials preparation through structured longitudinal data collection

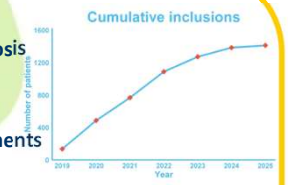
Registry Organization

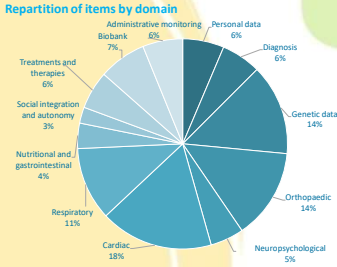
- National registry established by AFM-Téléthon in 2019
- Patients followed in 35 neuromuscular expert centers of the FILNEMUS network
- Standardized collection of clinical, biological and genetic data
- Data quality ensured by a dedicated Clinical Research Associate pool
- Full compliance with GDPR and French data protection authority requirements
- AFM-Téléthon is the data controller of the registry

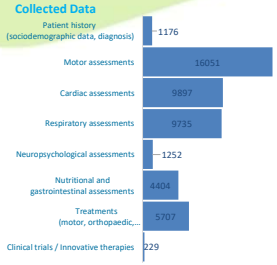


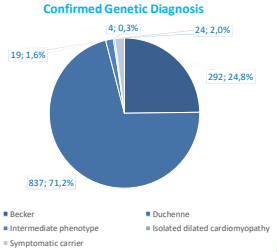
Results (as of May 2026)

- 1,413 patients included, including 1,176 with a confirmed genetic diagnosis
- More than 400 standardized variables collected
- Over 30,000 case report forms completed, covering motor, cardiac, respiratory, neuropsychological and digestive assessments, as well as treatments









Uses and Perspectives

The DYS registry provides real-world data on disease progression, supports clinical research, strengthens national epidemiological resources and facilitates the identification of patients eligible for clinical studies. It represents a key tool for the preparation of future clinical trials and for improving care organization.

Conclusion

The DYS registry represents a major national platform for clinical and epidemiological studies, supporting advances in knowledge and in the management of dystrophinopathies in France.

Acknowledgements / Contact
- Registry DYS
- Filnemus Network
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