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DUCHENNE
DATA FOUNDATION

Bringing data to life to improve
the world of dystrophinopathies

Dear stakeholders, partners, and community members,
As 2025 comes to a close, we reflect on a year defined by scientific progress, strategic collaborations, and meaningful steps forward for the dystrophinopathy community. Below is a recap of what we achieved together and what lies ahead.

New Strategic Development: DDF becomes a member of EPTRI

One of the year's significant milestones: DDF is now an official member of the European Paediatric Translational Research Infrastructure (EPTRI).
This membership strengthens our contribution to:

- Paediatric data innovation
- Harmonisation of protocols for dystrophinopathy research
- Translational pipelines for rare paediatric neuromuscular disorders
- Access to a pan-European network of specialised research platforms.

It also positions DDF as a key player in the upcoming EPTRI paediatric data initiatives.

More information on EPTRI can be found here.

ePTRI
EUROPEAN PAEDIATRIC TRANSNATIONAL RESEARCH INFRASTRUCTURE

World Muscle Society Congress 2025

The Congress took place in Vienna (7-11 October), where the DDF team presented a poster spotlighting our work to harness real-world data and digital platforms in the dystrophinopathy field. This presence underscores our commitment to ensuring that data-driven insights are not just academic but actionable for care, therapy development, and patient outcomes.

Strong presence in the 2nd General Assembly meeting of the ERDERA project

This October (29-31) in Amsterdam, DDF demonstrated its ongoing engagement and active participation within the ERDERA project, which aims to unify rare-disease research, data infrastructures, and clinical readiness across Europe. In particular, our roles include:

- Developing data-management & FAIR (Findable, Accessible, Interoperable, Reusable) frameworks for dystrophinopathies.

- Delivering patient and advocate-oriented training and capacity-building in rare-disease data science.

Find more on the ERDERA project here.

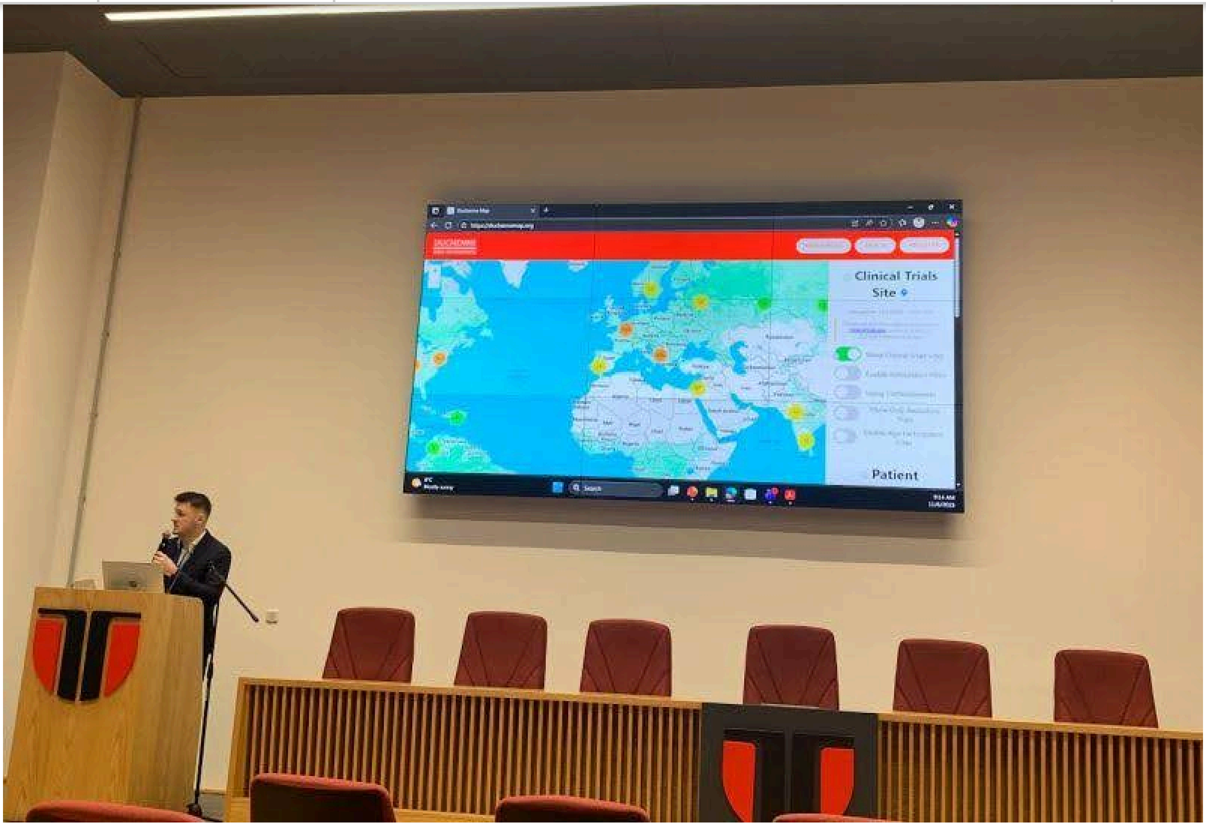
Highlights from the MAGIC General Assembly

During 28-30 October, the MAGIC consortium met in Lisbon for its annual General Assembly, bringing together partners from around the world to review progress and shape the next phase of the project. Discussions ranged from the challenges of immune responses in AAV-based gene therapy to emerging gene-editing strategies that could enhance muscle resilience to the development of advanced 3D muscle organoids that promise faster, more accurate preclinical testing. Throughout these exchanges, DDF, in collaboration with MDUK, played a central role, ensuring the patient perspective was embedded across all activities. By leading the project’s communications and dissemination efforts, DDF's primary focus is to translate complex scientific developments into transparent, patient-centered formats and to organize dedicated webinars for families. This way, DDF continues to steer MAGIC toward meaningful, real-world impact for the dystrophinopathy community.

Visit the MAGIC website to learn more.

Invited talk for the value of using AI in healthcare, with a special focus on Rare Diseases

During 5-6 November, we took part in the “Frontiers in Artificial Intelligence: Challenges & Prospects” workshop, reflecting our commitment to pushing boundaries in how data, machine learning, and modelling can help the dystrophinopathy community. As big data and AI become increasingly integral in rare-disease research, DDF aims to ensure that dystrophinopathy data is leveraged ethically, transparently, and in ways that respect and benefit patients.



Thrilled to begin the EFFecT Project journey as an associated partner!

The Kick-off Meeting took place on 3-4 November in Porto. Genuinely impressed by the high level of expertise and competencies demonstrated by the PhD candidates. All PhD candidates share remarkable journeys through their early research careers, enriching their CVs with internships at top pharma companies and research institutes across the EU, USA, and New Zealand! It's an honor to be part of this dynamic group. I couldn't be more excited to have the opportunity to train such an experienced and motivated cohort of early-career researchers.

Learn more about the EFFecT Project here.

Showcasing Innovation at eNMD 2025: Presenting Duchenne Map & the Duchenne Data Repository

Our team recently participated in the 5th eNMD Congress (27-29 November), this year focusing on an AI ecosystem for neuromuscular diseases. We were proud to present two of our flagship digital tools - Duchenne Map and the Duchenne Data Repository - and showcase how they support global collaboration, improve data accessibility, and empower the dystrophinopathy community. Attending eNMD provided valuable opportunities to connect with clinicians, researchers, patient groups, and technology innovators who share our mission to advance care and accelerate research for neuromuscular diseases.

New publication: The FAIR journey of a patient-driven registry - The Duchenne Data Platform

Thrilled to announce our new research article, in collaboration with the World Duchenne Organization and the Dutch Duchenne Parent Project. The purpose of this research article was to recount and reflect on the process and challenges encountered during the FAIRification of the patient registry, the Duchenne Data Platform. Practical solutions for applying the FAIR data principles are also presented. We hope that these solutions will inspire and empower other rare disease communities to join the Duchenne FAIR efforts - ultimately working together for the benefit of patients.

Access the article here.

Duchenne Patient Academy: Connecting DMD/BMD patient advocates of the future (13-14 December 2025)

The Chairman of the DDF Board, George Paliouras, was invited to discuss **Why data are important to patients and patient organizations**. In this presentation, the speaker walks us through the crucial questions that families face the moment a Duchenne Muscular Dystrophy (DMD) diagnosis enters their lives - what we call the two dragons: **the Care Dragon** and **the Cure Dragon**. Each demands different decisions, different timelines, and different kinds of strength. The video highlights the global initiatives shaping today's DMD data landscape and presents the Duchenne Data Foundation's two flagship projects:

community-driven research. Before you dive in, take a moment to reflect on how these tools can support your own journey, and **join us by using them, sharing them, and helping spread high-quality information across the Duchenne community**.

👉 **Click below to watch the video**

What's coming in 2026: Key priorities

We encourage stakeholders and community members to support our efforts in expanding the Duchenne Data Repository, participate in training programs, and collaborate on AI projects to accelerate progress together.

- **Scaling the DDF Data Repository**: We will continue to populate the Duchenne Data Repository with more datasets, metadata, and links to sources to support research and treatment development, as well as improve care.
- **Patient & Advocate Training**: We will roll out workshops and training programs (in collaboration with ERDERA partners) to empower patients, families, and advocacy groups with knowledge of data science, FAIR principles, and translational research.
- **AI-Enabled Insights**: We will pilot projects applying AI / predictive modelling to integrated dystrophinopathy data (e.g., natural history data, clinical trial data, etc.), always with patient-centric ethics and transparency at the core.
- **Global Engagement**: While rooted in the dystrophinopathy community, we will continue to engage with the broader rare disease ecosystem through ERNs, European projects, and international groups to share

networks.

- **Policy & Advocacy**: Data doesn’t just inform science - it informs regulation, policy, and patient rights. We will advocate for responsible data sharing, interoperability, and patient-driven research in Europe and beyond.

How you can engage

We invite community members, researchers, clinicians, and families to actively participate in upcoming projects, training, and events to strengthen our collective impact and foster collaboration.

- Please stay connected with us on LinkedIn, Facebook, and Bluesky to receive updates, webinar invites, and calls for action.
- If you are a researcher, clinician, patient organisation, or industry partner working in dystrophinopathies, reach out to explore how you can collaborate with the DDF ecosystem.
- If you are a patient or family member, ask us about our training modules and how you can engage with the data community in meaningful ways.
- Encourage your national centres, registries, or datasets to align with FAIR principles; join us in our mission to make data work for better care and treatment.

Community Spotlight

To the global community: patients, families, clinicians, scientists, and advocates, thank you for driving this mission forward. Thank you for being part of the journey with DDF.

Closing & Holiday Message

As we wrap up 2025, we extend warm wishes for a peaceful holiday season filled with rest and connection. Thank you for supporting our mission to make data a catalyst for solutions in Duchenne and Becker muscular dystrophy.
We look forward to an impactful year ahead in 2026.

With appreciation,
The Duchenne Data Foundation Team

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Wishing you a joyous holiday season and a prosperous New Year ahead!

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