# Workshop Presentation: "Advancing Together: Shaping a Brighter Future for NMD Patients"

On October 25-26, 2024, Ljubljana hosted a workshop entitled “Advancing Together: Shaping a Brighter Future for NMD Patients,” an event dedicated to addressing the challenges and opportunities in the field of neuromuscular disorders (NMD) across Europe.

## Day 1: Challenges and Opportunities in Neuromuscular Disorders in Europe

The workshop commenced with an official opening where the organisers presented the event’s objectives and the agenda for the day. This introduction highlighted the importance of collaboration between specialists and patient organisations to improve the lives of those affected by NMD.

The first panel, titled “The Diagnostic Odyssey and Newborn Screening (NBS),” tackled the discrepancies in diagnostic processes for NMDs across various European countries. Discussions focused on the role of newborn screening in the early detection of these conditions. Speaker Kacper Rucinski provided a detailed perspective on the challenges faced in diagnostics and the benefits of implementing screening programs.

The next panel, “Standards of Care and Access to Therapies for NMD Patients,” led by Sandra Blum, underscored the variability in patient care standards and the solutions needed to ensure equitable access to necessary treatments. This session was crucial for identifying gaps in care and for establishing more rigorous standards.

Panel 3 was dedicated to “Research and Clinical Trials,” where Grainne Crowley presented current research initiatives and the ways in which patients can access relevant clinical studies for their conditions. This discussion highlighted the importance of collaboration between researchers and patients in the development of new therapies.

In the afternoon session, discussions revolved around “Building Bridges in the NMD Field.” An important presentation was dedicated to European Reference Networks, where François Lamy discussed the role of EURO-NMD ERN and its impact at the European level. The concept of patient-centred care at the European level was explored, along with a presentation on the EAMDA NMD Resource Hub project, a centralised resource system aimed at improving support for patients across Europe.

At the end of the day, a recap of the discussions was conducted, offering a preview for the following day and reinforcing the participants' commitment to ongoing collaboration.

## Day 2: Strengthening Organisations and Advancing Together

On the second day of the workshop, activities began with a group discussion for patient organisations. Catalina Crainic, representing Dystonia Europe, contributed a presentation on organisational aspects. The participating organisations discussed the services provided to their members, the challenges and needs at both the organisational and national levels, as well as the distinctive characteristics and strengths of each organisation.

The educational sessions continued with presentations dedicated to sharing knowledge for patient representatives. Arabela Acalinei highlighted initiatives such as EUPATI, which aims to empower patients in clinical trials, and the EURORDIS Open Academy, which has developed advocacy skills for rare diseases. Additionally, the EPF program provided training in leadership and management for patient advocates, while EFNA's e-learning modules focused on enhancing patient involvement in health research. The experience of the Czech Republic in establishing an academy for patient organisations served as an example of best practices.

The workshop concluded with an official closing session, summarising the main outcomes of the event and expressing gratitude to the participants.

This workshop represented a valuable opportunity to advance together in support of patients with neuromuscular disorders, strengthening the networks and resources available at the European level.