

## **RevEal the burdeN on daily life for myotonic dyStrophy patients due to myotoniA: the ENSA survey.**

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**Introduction:** Myotonia is a symptom of myotonic dystrophy (DM) type 1 and 2. This can be debilitating and affects patients' everyday living, with a significant burden on Quality of Life (QoL) (1). Impact of DM on QoL has been evaluated (2,3), however, the specific contribution of myotonia remains unclear. The ENSA survey will assess the impact of myotonia on DM patients' daily lives.

**Methods:** Patients living in Europe, UK and North America, aged  $\geq 18$  years with a confirmed diagnosis of DM1/DM2, (or caregivers) will be invited to complete an anonymised online survey. Questions will explore the patient's description of DM symptom onset, time to medical consultation, the nature, frequency and location of myotonia, muscle weakness, fatigue, daytime sleepiness, gastrointestinal, and cardiorespiratory symptoms, along with disease management, treatment history and impact on daily life.

**Results:** Findings will be available in Q2-2023 and will aim to provide insight into the burden of myotonia on the daily life for DM patients, as well as increasing understanding of symptoms to support future clinical-trial outcome measures.

**Conclusions:** The ENSA survey will quantify the impact of myotonia on DM1 and DM2 patients' daily life and raise awareness of the need for appropriate management.

### **Disclosures:**

Zozulya-Weidenfeller is employed by Lupin. Other authors received honoraria from Lupin as consultants during the ENSA creation.

### **References.**

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