Data Platforms

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EMA Multi-stakeholder Workshop
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Position of the DMD Patient community

• Optimal (re)use of data for drug development and care
  https://www.worldduchenne.org/fair-data-for-duchenne/

• Collection of data relevant to patients (PROs and PROMs)
  ‘Need for Natural History of PROMs’

• Placebo data should be made available

• Clinical trial data should be returned to participants

• Patients should decide about the (re)use of their own data

• Patients and citizens are willing and interested to share data

• Data centralization and the old data transfer process are roadblocks for scientific research. Let’s change that
Data platform(s)

• To be used for questions relevant to the patient community whether it is development of new drugs, new technologies or about daily life
• Give patients the power and control over the use of their own data
• Allow to have all their data with them at all times (emergencies)
• To give patients the option to ‘get their data together’. See it as ‘storing in a personal locker’
• To facilitate the adoption of emerging technologies regarding data collection and enable their optimal application in health research, care and drug development
Classified as public by the European Medicines Agency

- Conforming to GDPR obligations
- DMD data was made (FAIR)
- International Standards recommended at European level were used (European Joint Programme on Rare Diseases (EJP-RD))
1st September, 2022 - Announcement by Joint Press Release

INTEROPERABILITY ACHIEVED!
Between two FAIR Rare Diseases Registries
Another initiative by Foundation 29

- Uses PODs technology that empowers rare disease patients with their data to be able to activate applications and connections with organizations such as pharmaceutical companies and regulators
- Patient Organizations aggregate anonymous data from Raito’s network of patients
- Patient Organizations can send questionnaires to collect data
- Patient Organizations can share these data for drug development or other projects
Thank you for your attention!

(Also on behalf of Julián Isla, Foundation 29)