



Duchenne
Data
Platform

Foundation
29

Data Platforms

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Chair, World Duchenne Organization

EMA Multi-stakeholder Workshop

21 September 2022



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**

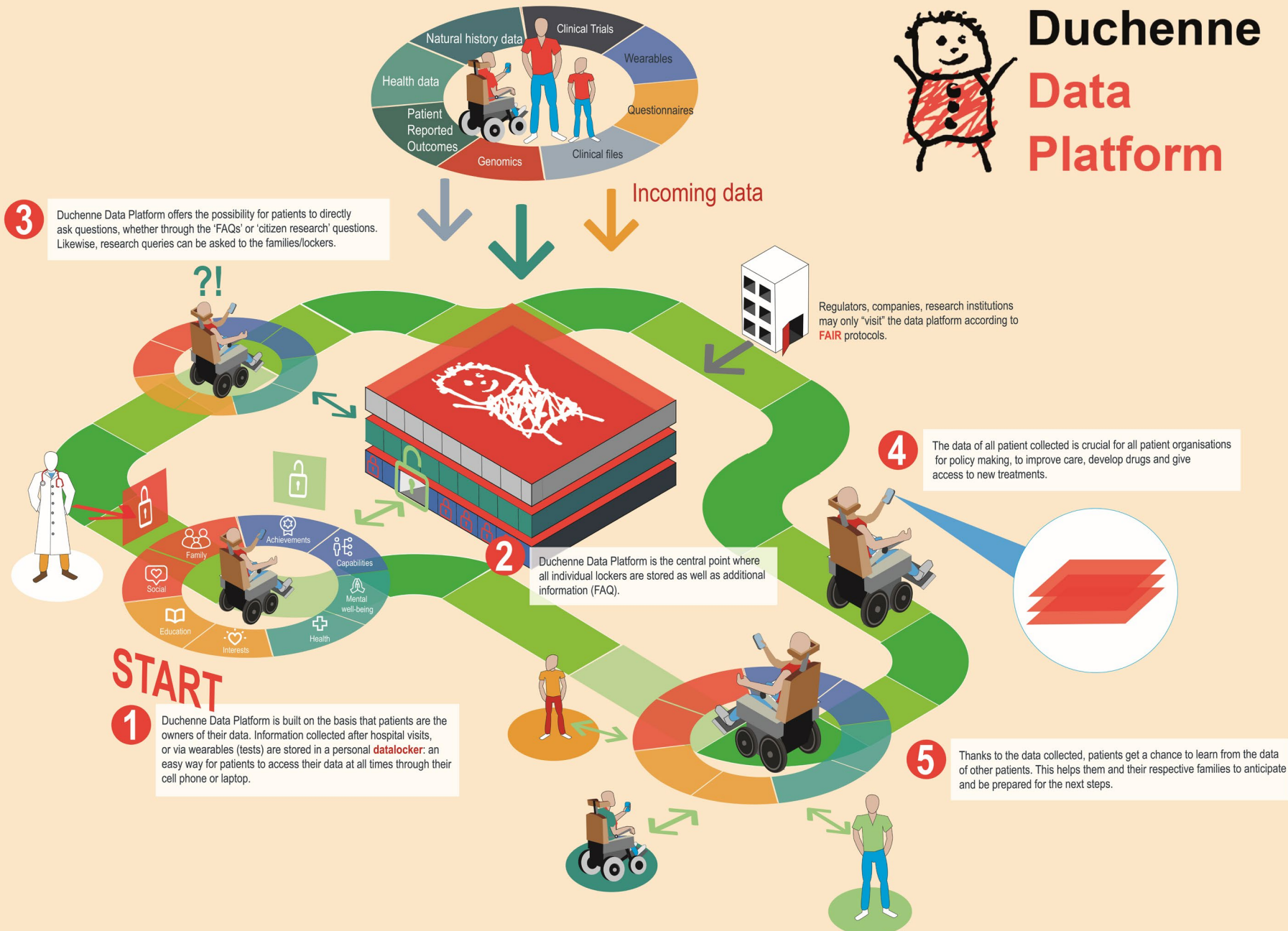
A group of diverse people, including children and adults, are gathered together, many with their hands raised in celebration. There are balloons and confetti in the air, suggesting a festive or successful event. The background is slightly blurred, focusing attention on the group.

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
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- 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**



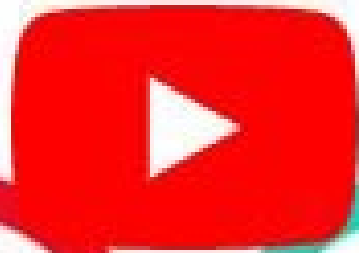
Duchenne Data Platform



EHDS

- 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))

FAIR Data for Duchenne (what Justus wants)



1st September, 2022 - Announcement by Joint Press Release

FAIR

**INTEROPERABILITY
ACHIEVED!**

Between two
FAIR Rare Diseases Registries

**ERN
EURO-NMD**

**Duchenne
Data
Platform**

FAIR

FAIR

Another initiative by Foundation 29

RAITO

Decentralized patient registries



- 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)