

Putting Real
World
Evidence in
Practice

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World Duchenne Organization

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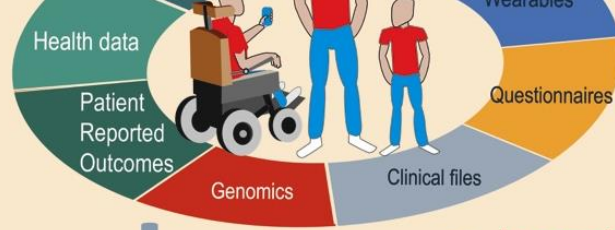
Position of the DMD Patient community

- Optimal use and reuse of data for drug development and care
- Collection of data relevant to patients (PROs and PROMs)
- Placebo data should be made available
- Clinical trial data should be returned to participants
- Patients should decide about the use of their own data
- Patients and citizens are willing and interested to share data

Data platform

- Gives patients the power and control over the use of their own data.
- Have all their data with them at all times (emergencies)
- Gives patients the option to 'get their data together'. See it as 'storing in a locker'
- Patient data can be used for questions relevant to the patient community whether it is development of new drugs, new technologies or about daily life.
- Will facilitate the adoption of emerging technologies regarding data collection and enable their optimal application in health research, care and drug development

Data Platform



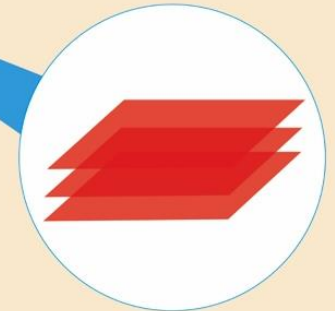
Incoming data

3 Duchenne Data Platform offers the possibility for patients to directly ask questions, whether through the 'FAQs' or 'citizen research' questions. Likewise, research queries can be asked to the families/lockers.



Regulators, companies, research institutions may only "visit" the data platform according to FAIR protocols.

4 The data of all patient collected is crucial for all patient organisations for policy making, to improve care, develop drugs and give access to new treatments.

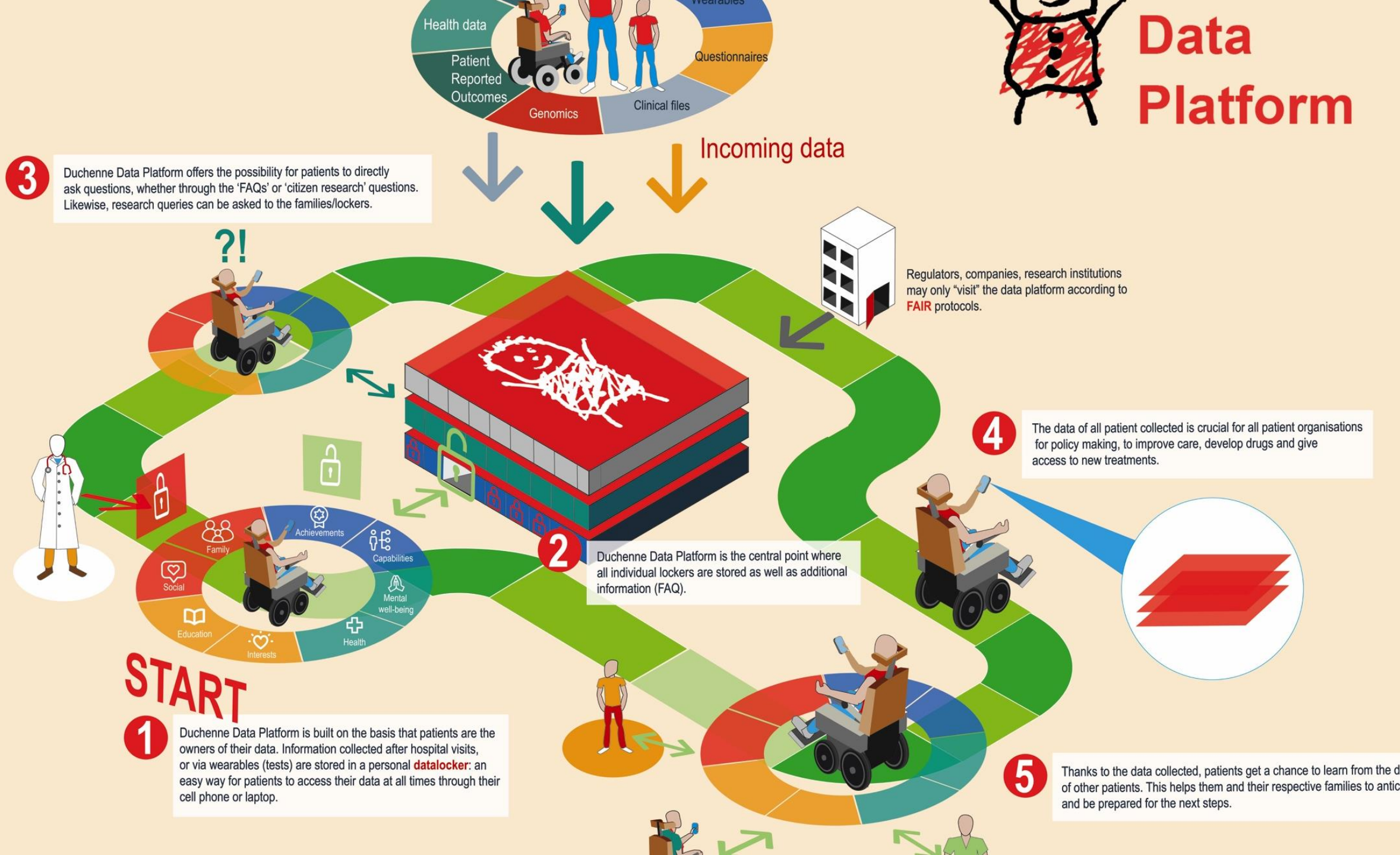


2 Duchenne Data Platform is the central point where all individual lockers are stored as well as additional information (FAQ).

START

1 Duchenne Data Platform is built on the basis that patients are the owners of their data. Information collected after hospital visits, or via wearables (tests) are stored in a personal **data locker**: an easy way for patients to access their data at all times through their cell phone or laptop.

5 Thanks to the data collected, patients get a chance to learn from the d of other patients. This helps them and their respective families to antic and be prepared for the next steps.



Patient Data

- Patient derived or provided data are not owned by those who collected them, and their (re)use should be primarily controlled by the donors of these data. Researchers and Health professionals are custodians. (GDPR)
- To enable the optimal reuse of real world data, the data needs to be Findable, Accessible, Interoperable and reusable by Medical professionals, Patients and in particular also by machines.

Optimal use and reuse of data for drug development and access

- How to use these patient relevant data in the regulatory environment other than through publications in scientific papers?
- What is the 'status' of these data collected by PO's? Patient representatives in all committees at EMA.
- Ongoing discussion with EMA and starting with HTA
- Need to create a 'pathway' to bring in RWD collected by patients.