

Duchenne Data Platform

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D U C H E N N E

A 360° DOCUMENTARY

Why we need a platform

- Currently, there is a severe underutilisation of patient-derived and patient-provided data.
- Data are collected and kept in Silo's.
- Modern technologies, including personal wearable sensors, health apps and federated learning over large numbers of distributed data sets fully enable the dovetailing of classical research data with data from patients and healthy citizens.
- However the adoption of emerging technologies that enable all this and their optimal application in health research care and drugdevelopment are lagging severely behind.

Why we need a platform

- The role patient participation, expert annotation and crowd sourcing, collective hypothesis-analysis and citizen science in general can play in the acceleration of the development, testing and adoption of new interventions is frequently ignored
- Educated patients and their family members feel a dire need for much more effective (re)use of their data, as well as their own time and skills in knowledge discovery and the fast-track translation of knowledge derived from studies into health practice.

What is a data platform

- A data platform is an infrastructure to facilitate the process of data collection
- The data platform has to be able to collect data from different sources of data
- Interoperability is key
- Many data platforms are a minimum registry with mainly demographic data and some mandatory fields
- Our goal is to build a platform to be used in drug development which is interactive with patients/parents and the DMD community

Why we need a Duchenne platform

- Data of DMD patients are collected in silo's
- Huge variety of data collected
- Some include PROM's others are not
- Network of DMD registries exist.
- No data sharing other than through queries on a specific question (from industry)
- Lack of 'Natural History of PROMs'
- Patients don't benefit (enough) from their own data as they are scattered
- Patients should benefit more from the total of data collected in registries

Data platform modules

Basic patient information (age, country, residence, etc)

Clinical data (information to describe the condition)

PROM datapoints. Information to capture the condition of the patient in real life.

Phenotype. A module to describe the phenotype of the patient using HPO

Genotype. A module to describe the genotype of the patient using HGVS.

Genotype and phenotype are following IRDiRC recommendations

An illustration of various medical supplies on a teal background. In the top left is a white bottle with a red base. Next to it is a single white pill. To the right is a brown bottle with a white label. Further right is a taller white bottle with a yellow cap and a red and yellow pill on its label. In the center is a white blister pack containing four red pills. Below this is a dark grey blister pack containing four dark grey pills. At the bottom is a syringe with a yellow plunger and a grey needle. To the right of the syringe are two capsules, one red and yellow, and one red and white. The text "All is about adherence" is overlaid in white on a dark grey horizontal band across the middle of the image.

All is about adherence

Adherence

- Families don't have a lot of time to fill out long questionnaires.
- Asking irrelevant questions does not help.
- If you don't get in return what is promised or could be expected does not help either (for example, being recruited or informed about upcoming trials)

Adherence

- What to ask when and how is crucial
- Our goal is to have patients engaged from day one
- Patients need an incentive to be interacting with the platform
- We are implementing a social Q&A solution where patients can find answer to their common questions
- Patients can upload and download their own information

Focusing on conversations

- We don't want to expose Duchenne patients to a form with hundred of data items
- We are exploring conversational interfaces where the form is replaced by a chat bot
- The chat bot can ask questions to the patient and other interfaces like Alexa from Amazon, Siri or Google Assistant can be used.



Dashboard

Basic Info >

Clinical Info >

Phenotype

Genotype

FAQ



Bot in english

Chat

Type your message...



Assistant



Percentage



Assistant

Copyri

Type your message...



The importance of data

- Data relevant for patients are relevant for drug development
- We need answers to relevant questions, asked by for example regulators, not based on opinions but on collective data
- Data in silo cannot do for patients what collective data can do

Data

- Patient derived or provided data are not owned by those who collected them, and their reuse should be primarily controlled by the donors of these data. Researchers and Health professionals are custodians.
- Optimal care should be taken to restrict the need to reveal the actual identity of individuals associated with certain data, and to protect privacy with all possible means, but we realise privacy is subordinate in many cases to fast-tracking of better solution for the diseases we are suffer from.
- 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.

Data platform

- Gives patients the power and control over the use of their own data.
- 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