Patient-managed registries

How can we get high quality data from patients for precision medicine?

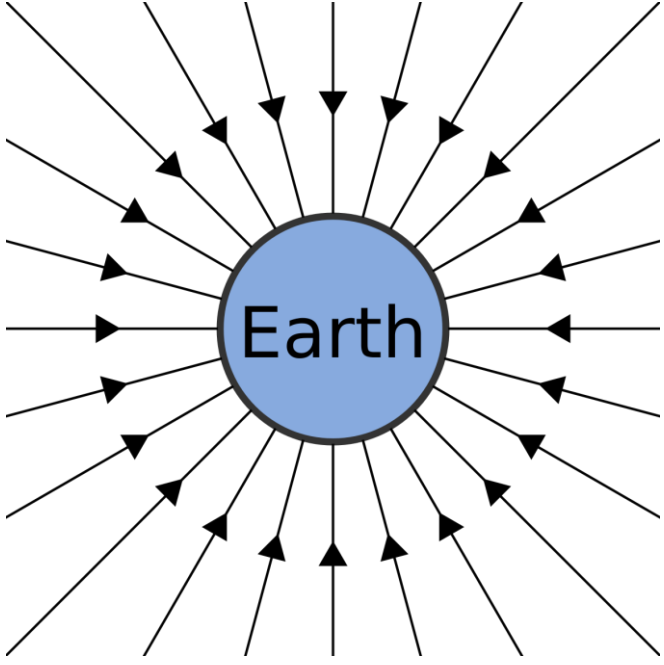
Elizabeth Vroom
Duchenne Parent Project

Julian Isla
Foundation 29

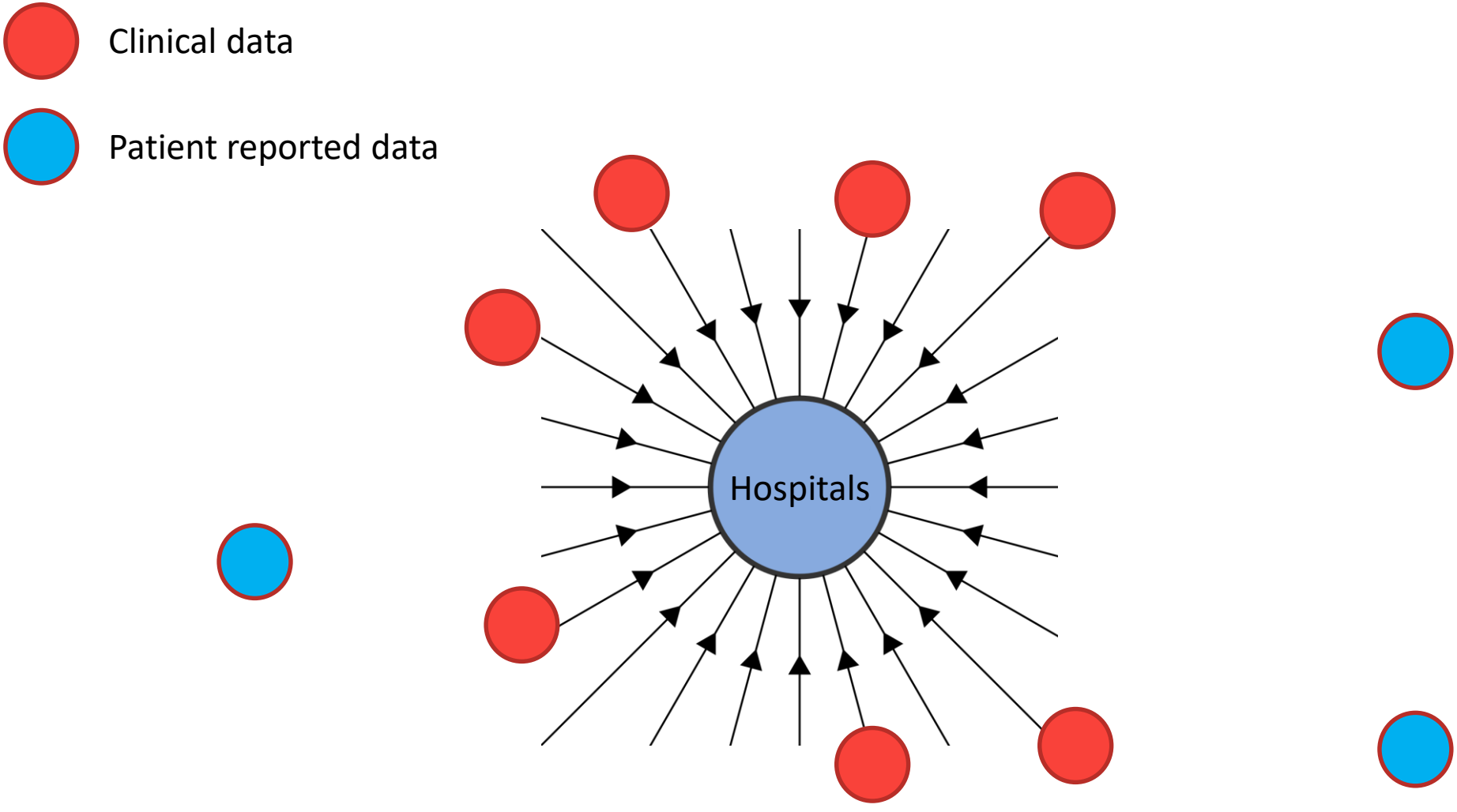
Duchenne Parent Project

29 foundation twenty-nine

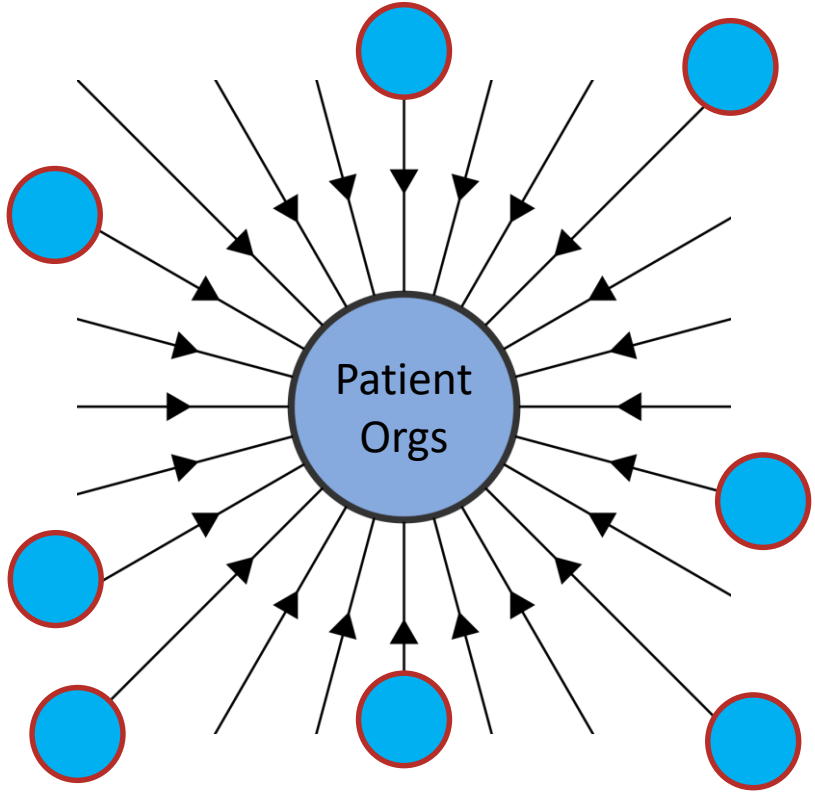
Why patient-managed registries are needed?



Classified as public by the European Medicines Agency

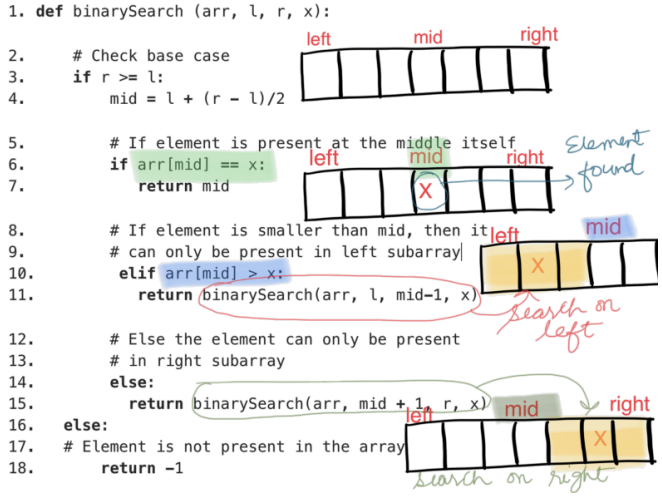


Classified as public by the European Medicines Agency



Classified as public by the European Medicines Agency

Technology barrier



Low-hanging fruit effect

Classified as public by the European Medicines Agency

Duchenne Data

Genetic defect Duchenne Muscular Dystrophy (DMD) discovered in 1986

Natural History studies started as early as 1969

Patients see approx 10 different health care professionals per year

Data collected by HCP, patient registries and research databases.

Clinicaltrials.gov shows 271 clinical trials for DMD data kept by companies

Classified as public by the European Medicines Agency

Position of the DMD Patient community

Optimal (re)use of data for drug development and care

Collection of data relevant to patients (PROMs)

Placebo data should be made available

Patients deciding about the use of their own Data

Patients are willing and interested to share data

Clinical trial data should be returned to participants

Classified as public by the European Medicines Agency

DUCHENNE
FAIR DATA
DECLARATION

EURORDIS
RARE DISEASES EUROPE

Rare Barometer
A EURORDIS INITIATIVE

SHARE AND PROTECT OUR HEALTH DATA!

Rare disease patients' preferences on data sharing and protection

January 2020

30 MILLION
people are living with a rare disease in Europe and 300 million worldwide

NO CURE
for the vast majority of diseases and few treatments available

Sharing health data to advance scientific research and improve clinical benefits are of particular importance in the **field of rare diseases** where knowledge and expertise are limited and **patient populations are geographically dispersed.**

Understanding what patients want and need from rare disease research and data sharing is **important to ensure their participation and engagement in the process**, and to ensure that these wishes and needs are embedded within research and healthcare delivery design.

WITHIN THIS REPORT YOU WILL FIND:

1. Key results from research conducted by EURORDIS, outlining rare disease patients' preferences with regards to data sharing
2. Seven recommendations designed to inform and support stakeholders involved in data sharing initiatives
3. Research and recommendations methodology

Classified as public by the Europea

Patient 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. (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.

Classified as public by the European Medicines Agency

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’

Have all their data with them at all times (emergencies)

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

Classified as public by the European Medicines Agency

Health Data Locker

- Patients need a safe place to store their information.
- A health data locker provides:
  - Interoperability
  - Ownership
  - Portability
- **FAIR** principles implementation:
  - **F**indable, **A**ccessible, **I**nteroperable, **R**eusable
- *Patients don't give data; they allow requestors to* ***visit*** *it*
- A Blockchain registry can help with this feature

Classified as public by the European Medicines Agency

Privacy and security by design

Privacy and security are critical features for DDP. A security breach could put the project at risk. Privacy and security design is being done from the beginning:

- GDPR assessment using a PIA (Privacy Impact Assessment).
- Security certification. An external provider with expertise in security runs technical security tests.
- A DPO is responsible for data protection

Classified as public by the European Medicines Agency

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.

Classified as public by the European Medicines Agency

Thank you

elizabeth.vroom@worldduchenne.org

julian.isla@foundation29.org

Classified as public by the European Medicines Agency