Patient-managed registries
How can we get high quality data from patients for precision medicine?

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Why patient-managed registries are needed?
Clinical data

Patient reported data
Patient Orgs
Technology barrier

1. **def** binarySearch(arr, l, r, x):
2.     # Check base case
3.     if r >= l:
4.         mid = l + (r - l) // 2
5.     # If element is present at the middle itself
6.     elif arr[mid] == x:
7.         return mid
8.     # If element is smaller than mid, then it can only be present in left subarray
9.     elif arr[mid] > x:
10.        return binarySearch(arr, l, mid - 1, x)
11.    # Else the element can only be present in right subarray
12.    elif arr[mid] < x:
13.        return binarySearch(arr, mid + 1, r, x)
14.    else:
15.        return binarySearch(arr, mid + 1, r, x)
16.     # Element is not present in the array
17.     return -1

Low-hanging fruit effect
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
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
DUCHEENNE
FAIR DATA
DECLARATION

1. Patient derived or provided data are not owned by those who collected them.

2. Consent should be provided by the patient, unless under exceptional circumstances.

3. Data should be shared for the betterment of health care and the massive saving of costs to keep optimal healthcare affordable for all.

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 their 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 Europe
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.
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
Health Data Locker

• Patients need a safe place to store their information.

• A health data locker provides:
  • Interoperability
  • Ownership
  • Portability

• **FAIR** principles implementation:
  • Findable, Accessible, Interoperable, Reusable

• *Patients don’t give data; they allow requestors to visit it*

• A Blockchain registry can help with this feature
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
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.
Thank you

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