WECAN SmartStart in Cancer Patient Advocacy

WECAN MASTERCLASS in Cancer Patient Advocacy

Patient Generated Data

The patient organisation experience of using technology

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EURORDIS – Rare Diseases Europe (Barcelona)

What is Patient Generated Data (PGD)

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Patient-generated data are health-related data **created, recorded, or gathered by or from patients** (or family members or other caregivers) to help support and manage disease state.

- Patient generated data include, but are not limited to:
  - health related events / symptoms
  - medication adherence
  - biometric data (wearable devices)
  - Patient Reported Outcomes (PROs)

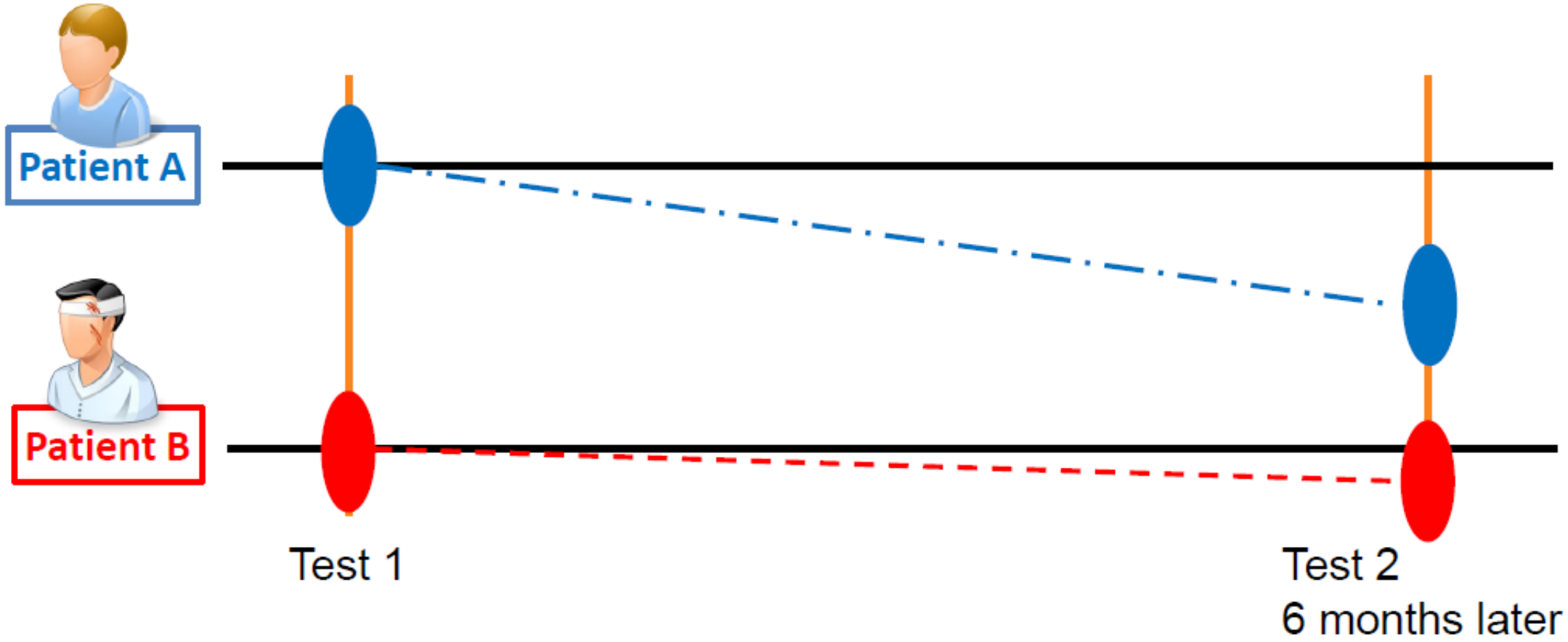
Patient-generated data are **distinct from data generated in clinical settings** and through encounters with health care providers in two important ways:

- Patients, not providers, are responsible for capturing or recording these data.
- Patients decide what data to share, and with which health care providers / researcher to do so.

Examples include physical activity using wearable devices, and medication adherence and ePRO using a mobile app.

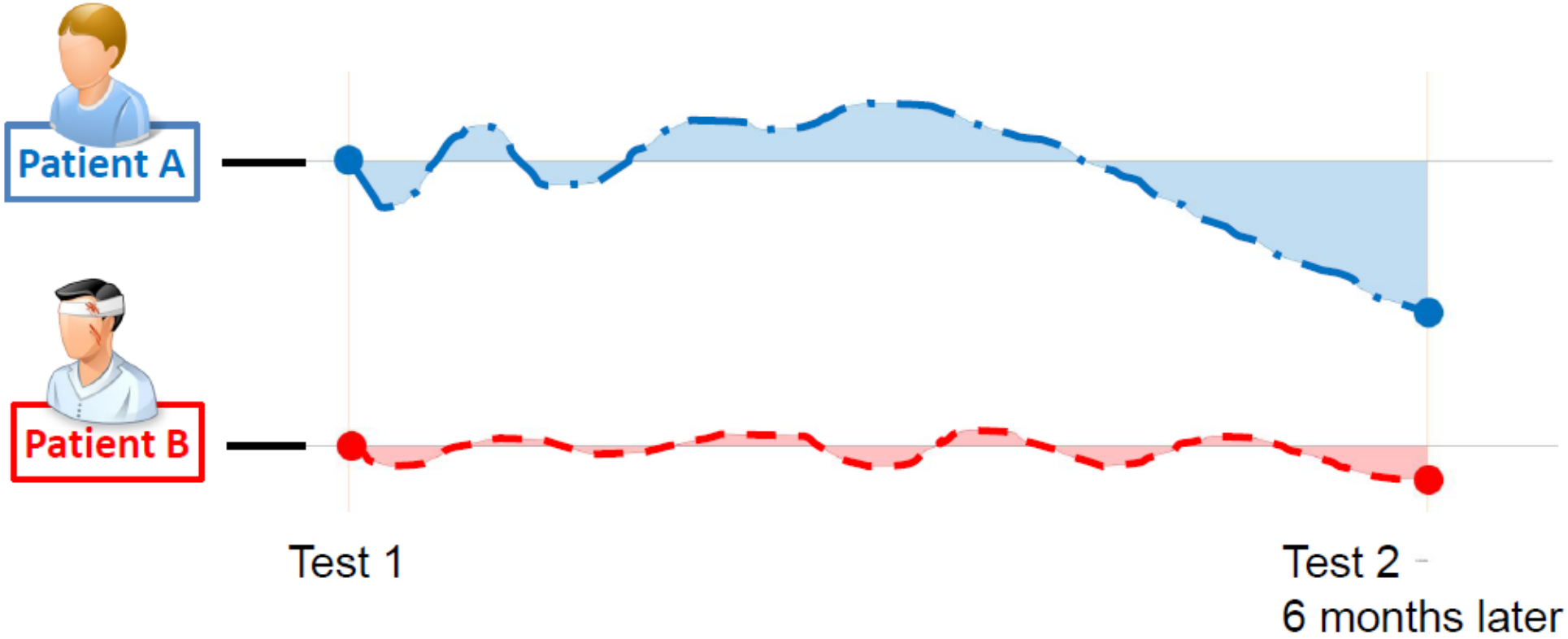
Courtesy of Dr. Elin Haf Davies aparito.com

What the Doctor **sees…**



Courtesy of Dr. Elin Haf Davies aparito.com

What the Patient **experiences…**



"99 percent of patient activity happens outside of the hospital or clinic, beyond the scope of the [electronic health record] EHR"

Courtesy of Dr. Elin Haf Davies aparito.com

Why do we need technology to foster Patient Generated Data ?

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Increasing the probability of success and lowering costs

- Improve remote patient monitoring, and natural history understanding
- Drive value from Patient Generated Data – demonstrate what's important to patients
- Reduce the amount of monitoring intervention that contributes to a lower quality of data captured.
- Conduct studies anywhere in the world where there's an Internet or 3G connection.
- Conduct studies in geographies without having to be too concerned about the costs or practicalities of that locations infrastructure.
- For selected studies / diseases / drug type - reduce the number of patient visits and reduce the overall cost of running a study.
- Bring objective, outcome based data to Drug Application and increase the probability of success.

Growing interest in Patient Generated Data

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Research Letter

ONLINE FIRST FREE

June 4, 2017

Overall Survival Results of a Trial Assessing Patient-Reported Outcomes for Symptom Monitoring During Routine Cancer Treatment

Ethan Basch, MD, MSc[1,2]; Allison M. Deal, MS[1]; Amylou C. Dueck, PhD[4]; et al

Author Affiliations | Article Information

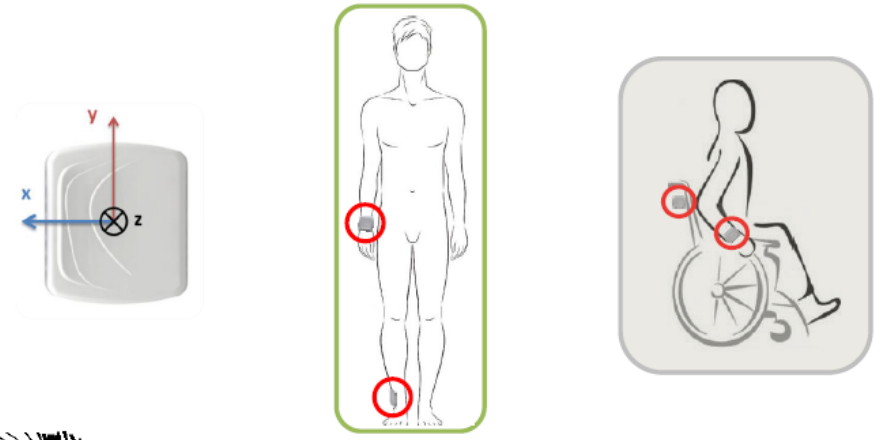
JAMA. Published online June 4, 2017. doi:10.1001/jama.2017.7156

Patient Generated Data using aparito.com

Patient Generated Data via wearables

ACTIMYO PLACEMENT AND DATA MEASURED

- ActiMyo contains a magneto-inertial measurement unit
- Ambulant patients : ankle and wrist
- Non ambulant patients : wheelchair and wrist



Example : Ankle trajectory during a 6WT

Patient Generated Data via wearables

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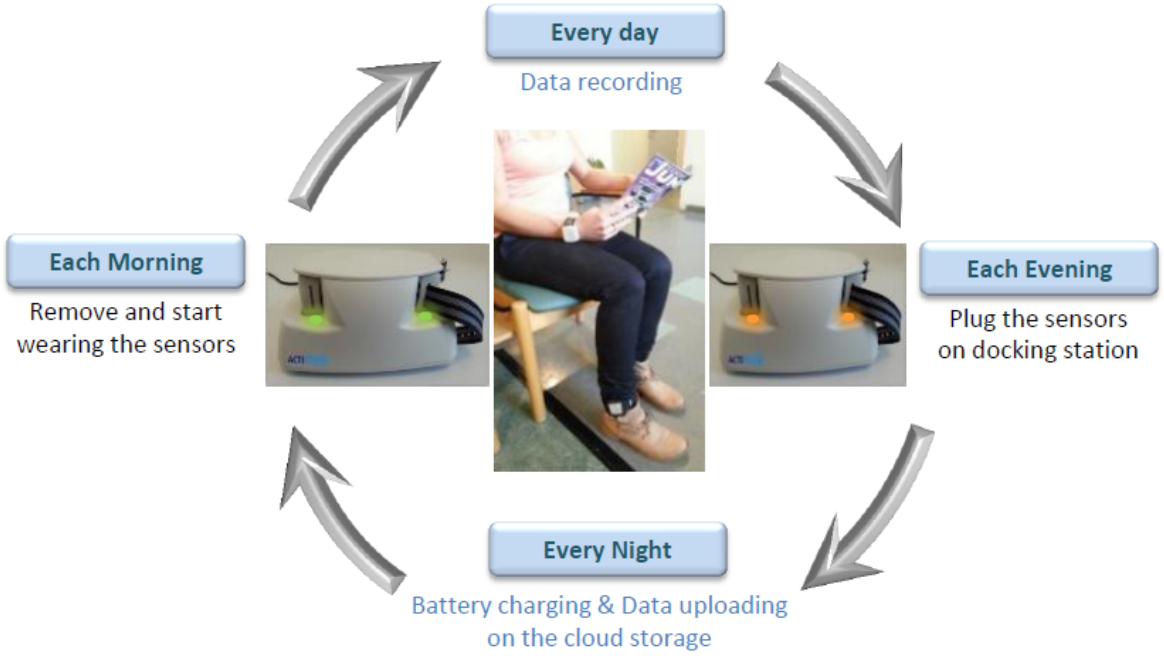
ACTIMYO USE

- ActiMyo is used in children from the age of 5
- Use every day for one month at every study time point
- Data have been gathered in ambulant DMD, SMA, FSHD, LGMD2B and Parkinson. It was also used in non ambulant, DMD, SMA and X-MTM.



Patient Generated Data via Health29 (www.foundation29.org)

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Social Q&A

PROMs

Genotype

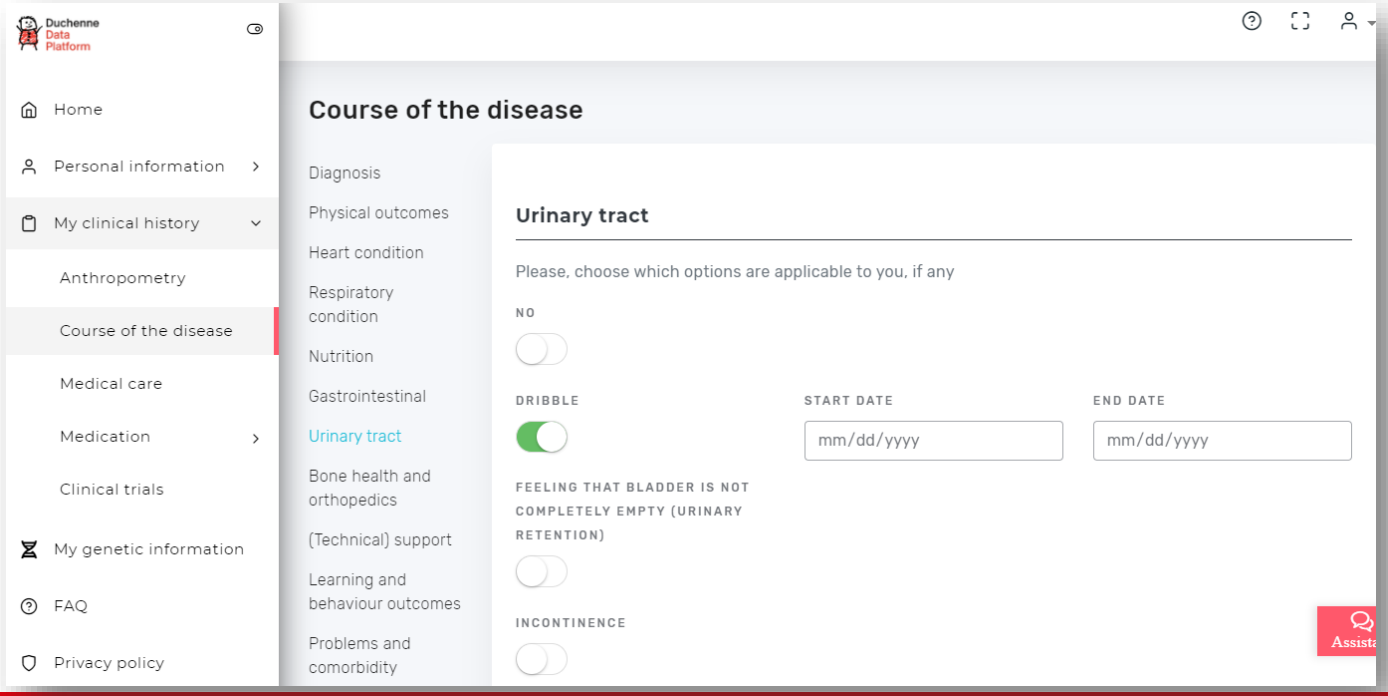
Phenotype

"Health29 is a platform with patient health data, by and for people"

Source: https://www.foundation29.org

Health29

- Full set of clinical data
- Customized by patient organization
- Standardized and with quality controls
- Easy to fill by users
- API for interoperability



- Deep phenotyping using CNLP
- Standard HPO
- Based on AI for symptoms identification
- Phenotype can be initiated by patients

8 Symptoms

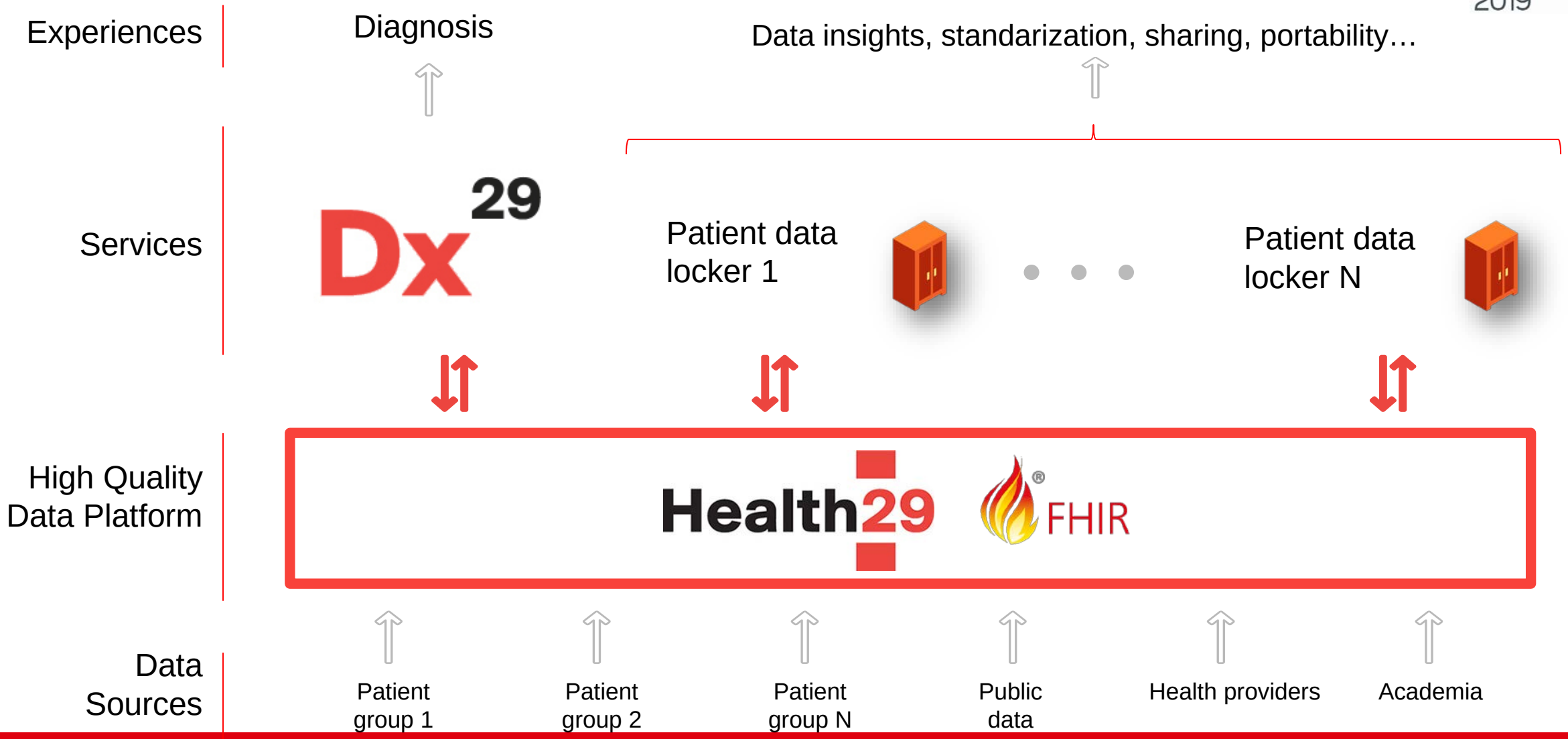
Name	Max. Similarity	# / Apparitions	Select/deselect
Calf muscle pseudohypertrophy	0.83	#: 1 dystrophy with characteristic pseudohypertrophy of the calves	
EMG: myopathic abnormalities	0.985	#: 1 myopathic changes by electromyography	
Elevated serum creatine phosphokinase	0.956	#: 2 elevation of creatine kinase levels in the , Serum creatine kinase was particularly elevated in	
Fatty replacement of skeletal muscle	0.93	#: 1 and fatty infiltration on muscle	
Limb-girdle muscular dystrophy	0.947	#: 2 autosomal recessive limb-girdle muscular dystrophy , of females with limb-girdle muscular	

Results:

The most distinctive feature of Duchenne muscular dystrophy is a progressive proximal muscular dystrophy with characteristic pseudohypertrophy of the calves . The bulbar (extraocular) muscles are spared but the myocardium is affected. There is massive elevation of creatine kinase levels in the blood, myopathic changes by electromyography and myofiber degeneration with fibrosis and fatty infiltration on muscle biopsy.The onset of Duchenne muscular dystrophy usually occurs before age 3 years, and the victim is chairridden by age 12 and dead by age 20. The onset of Becker muscular dystrophy is often in the 20s and 30s and survival to a relatively advanced age is frequent. Moser and Emery (1974) found that some female heterozygotes had myopathy resembling autosomal recessive limb-girdle muscular dystrophy (253600). Serum creatine kinase was particularly elevated in these patients. In most populations, the frequency of manifesting heterozygotes is about the same as that of females with limb-girdle muscular dystrophy

- Full genetic information
- Standard HGVS nomenclature
- Clustering patients according genetic variations + phenotype

Patients driving research



Patients driving research

DEVELOPMENTAL MEDICINE & CHILD NEUROLOGY ORIGINAL ARTICLE

Development of the Performance of the Upper Limb module for Duchenne muscular dystrophy

ANNA MAYHEW[1]* | ELENA S MAZZONE[2]* | MICHELLE EAGLE[1]* | TINA DUONG[3] | MARIA ASH[4] | VALERIE DECOSTRE[5] | MARLENE VANDENHAUWE[6] | KATRIJN KLINGELS[7] | JULAINE FLORENCE[8] | MARION MAIN[4] | FLAVIANA BIANCO[2] | ERIK HENRIKSON[9] | LAURENT SERVAIS[5] | GILES CAMPION[10] | ELIZABETH VROOM[11] | VALERIA RICOTTI[4] | NATALIE GOEMANS[6] | CRAIG MCDONALD[9] | EUGENIO MERCURI[2] | ON BEHALF OF THE PERFORMANCE OF THE UPPER LIMB (PUL) WORKING GROUP

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***These authors contributed equally to the study.**

PUBLICATION DATA
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ABBREVIATIONS

AIM An international Clinical Outcomes Group consisting of clinicians, scientists, patient advocacy groups, and industries identified a need for a scale to measure motor performance of the upper limb. We report the steps leading to the development of the Performance of the Upper Limb (PUL), a tool specifically designed for assessing upper limb function in ambulant and non-ambulant patients with Duchenne muscular dystrophy (DMD).

Duchenne Data Platform

- Home
- Personal information
- My clinical history
- My genetic information
- FAQ
- Privacy policy

Duchenne Data Platform (DDP) is a collaboration between Duchenne Parent Project and Foundation 29, a non-profit organization, with the aim to bring the data back to the patients and their parents / caregivers..

DDP is a data platform for storing biomedical data. DDP has been developed to store data in a very safe environment in a simple secure way. (Security policy)

The platform is available via the internet or as

Assistant

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940
patient advocacy groups

400
moderators

RareConnect.org

12
languages

180
global communities

2,000+
posts & comments per month

40,000
members
+20-30 new users/day

74K
translated posts & comments

13K
followers

15K
followers

60%
mobile users

2,000
patient testimonials

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RareConnect

Social

My Studies

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RareConnect.org

https://www.rareconnect.org/en

MyStudies. RareConnect.org

https://mystudies.rareconnect.org

(De-identified data)

Patients & Caregivers

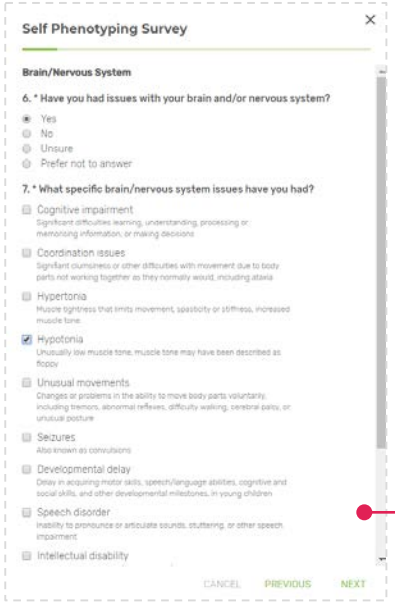
Advocacy Organizations

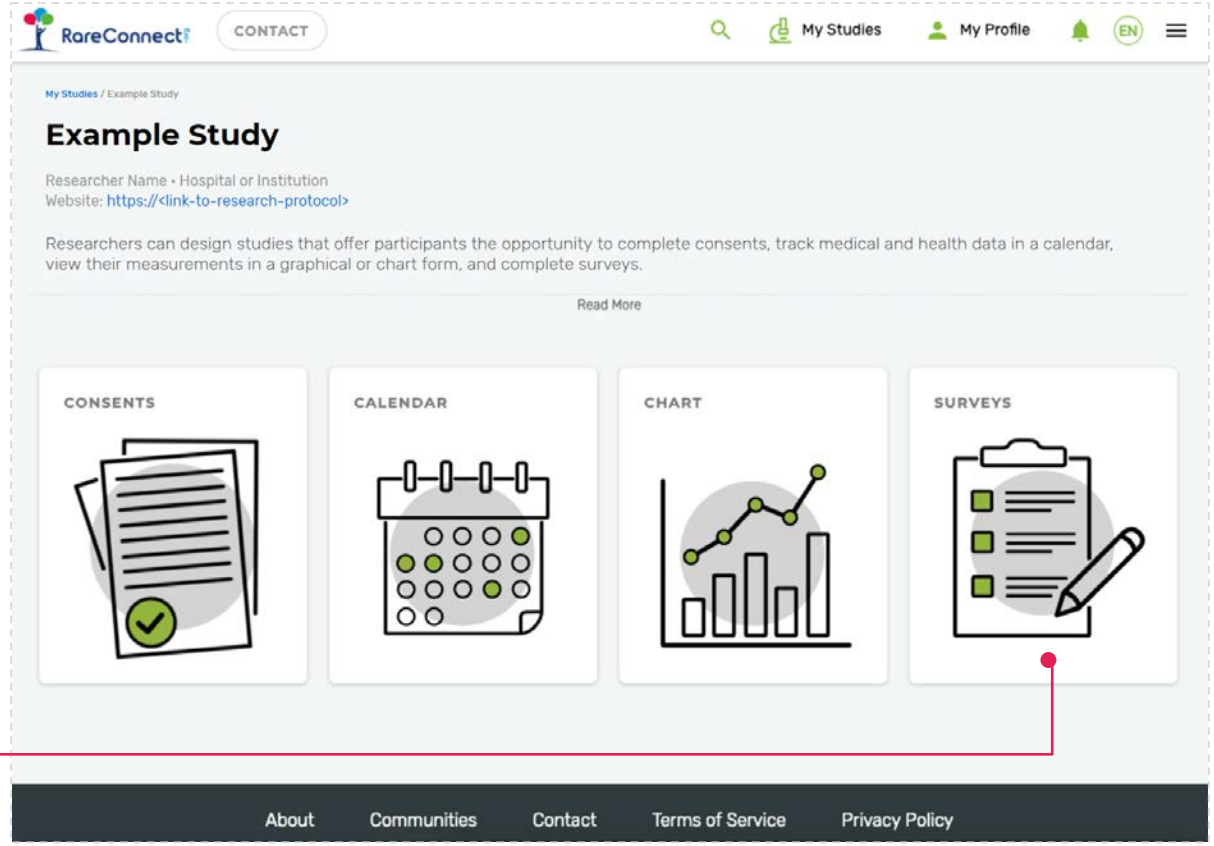
Researchers

Clinicians

Patient Reported Outcomes (PROs)

Designed to provide researchers with a customizable survey and questionnaire system that they can push to their study participants to complete.



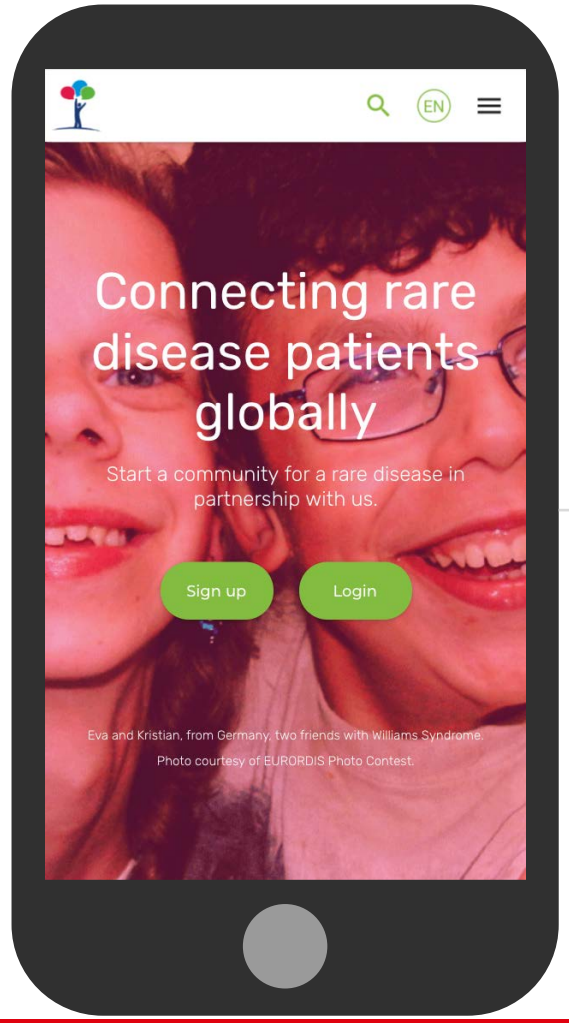


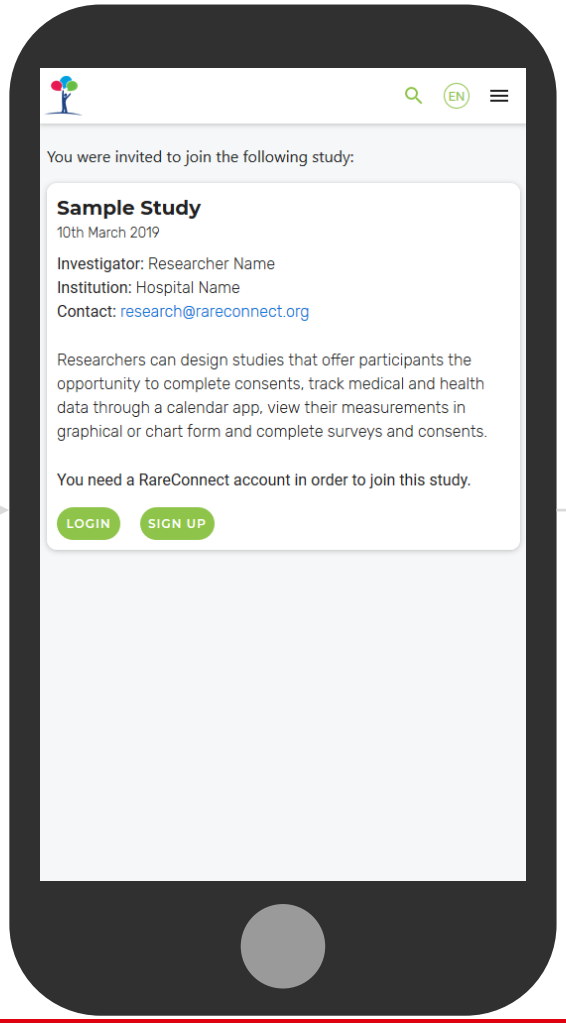
Patient Reported Outcomes (PROs)

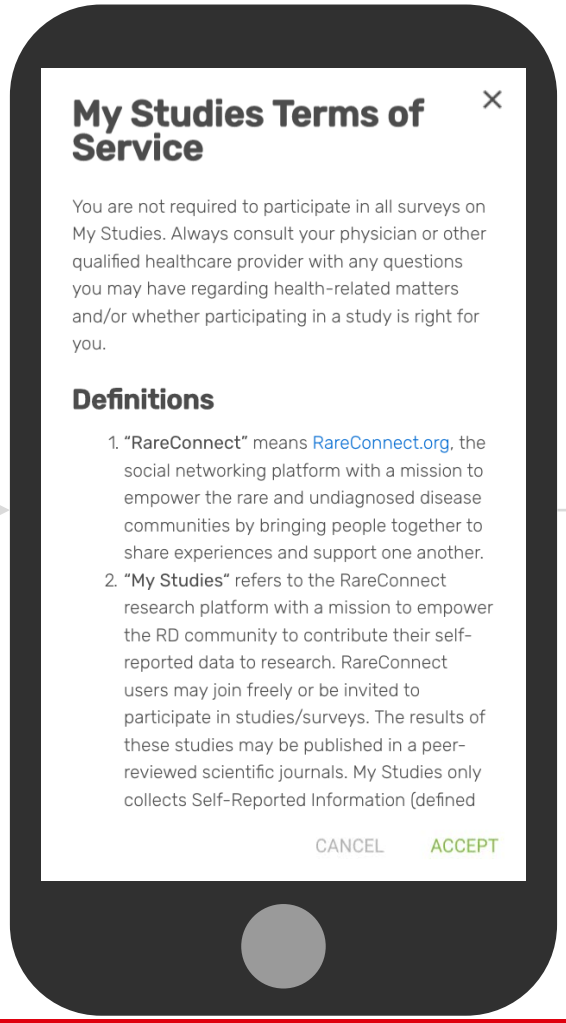
In addition to surveys, participants can also track medical events, tests, and medications as longitudinal data.

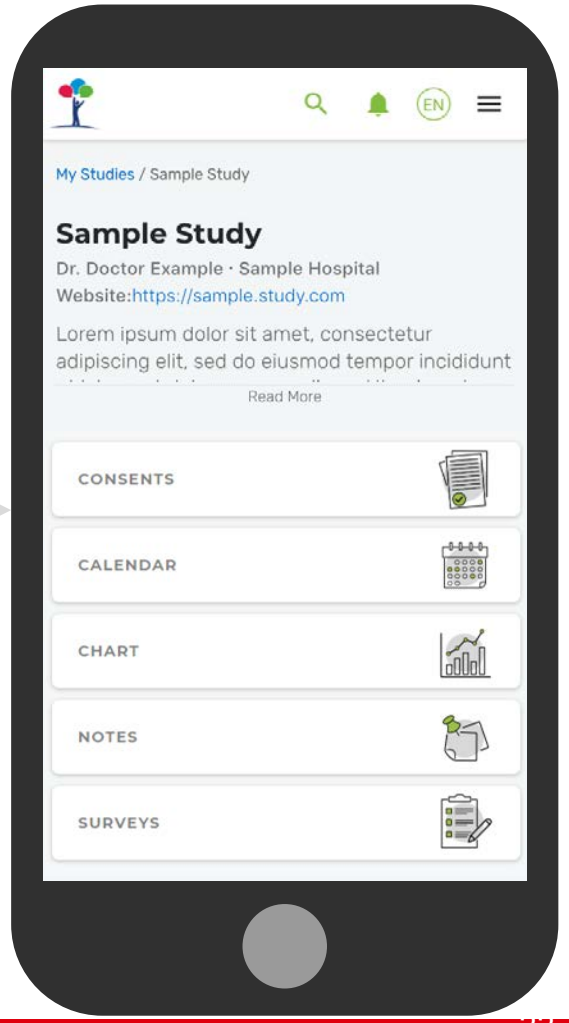
Future versions of My Studies will enable participants to share their outcome measures with their clinicians.

Participant registration



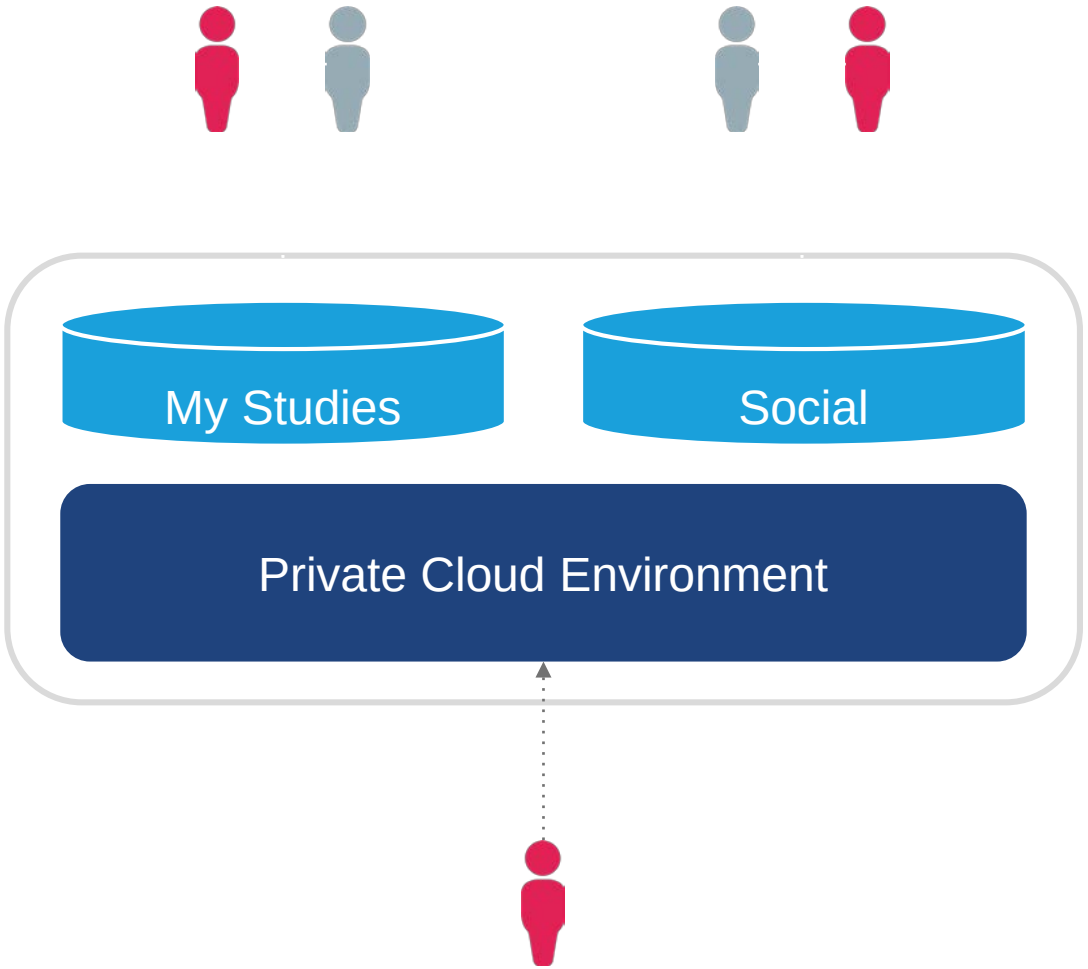






Research data

Data is stored on an instance of HPC4Health owned by CHEO. HPC4Health is a secure facility that has undergone external Privacy Impact Assessment (PIA) and Threat Risk Assessment (TRA).



Conclusions

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Some final thoughts:

- Consider which data is most important and impactful for your patient group/community to generate
- Don’t be afraid to work with experts to develop new data instruments which can determine unmet medical need or clinical added value for example
- Where possible do not build from scratch but make use of existing, compliant, affordable, reliable and well supported platforms (e.g. Health29 or others)
- Learn from what other patient groups are doing, via webinars, conferences, newsletters etc
- Develop a data plan which encompasses governance, access, sustainability, compliance (GDPR, Privacy Impact Assesment)
- Consider how much resources you will need. Can’t give you a figure as each case is different!

Thank you!

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