

Patient Generated Data

The patient organisation experience of using technology

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What is Patient Generated Data (PGD)

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:

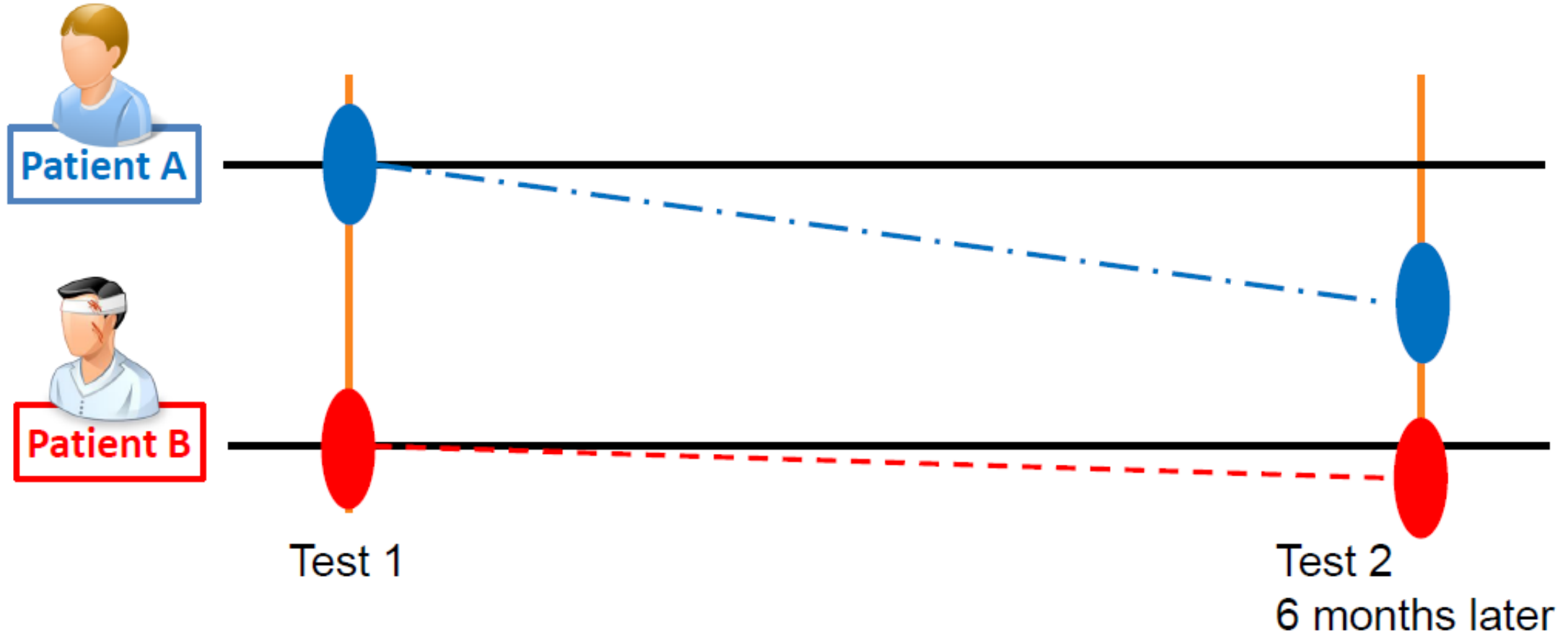
- Patients, not providers, are responsible for capturing or recording these data.
- Patients decide what data to share, and with which health care providers / researcher to do so.

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

Courtesy of Dr. Elin Haf Davies aparito.com



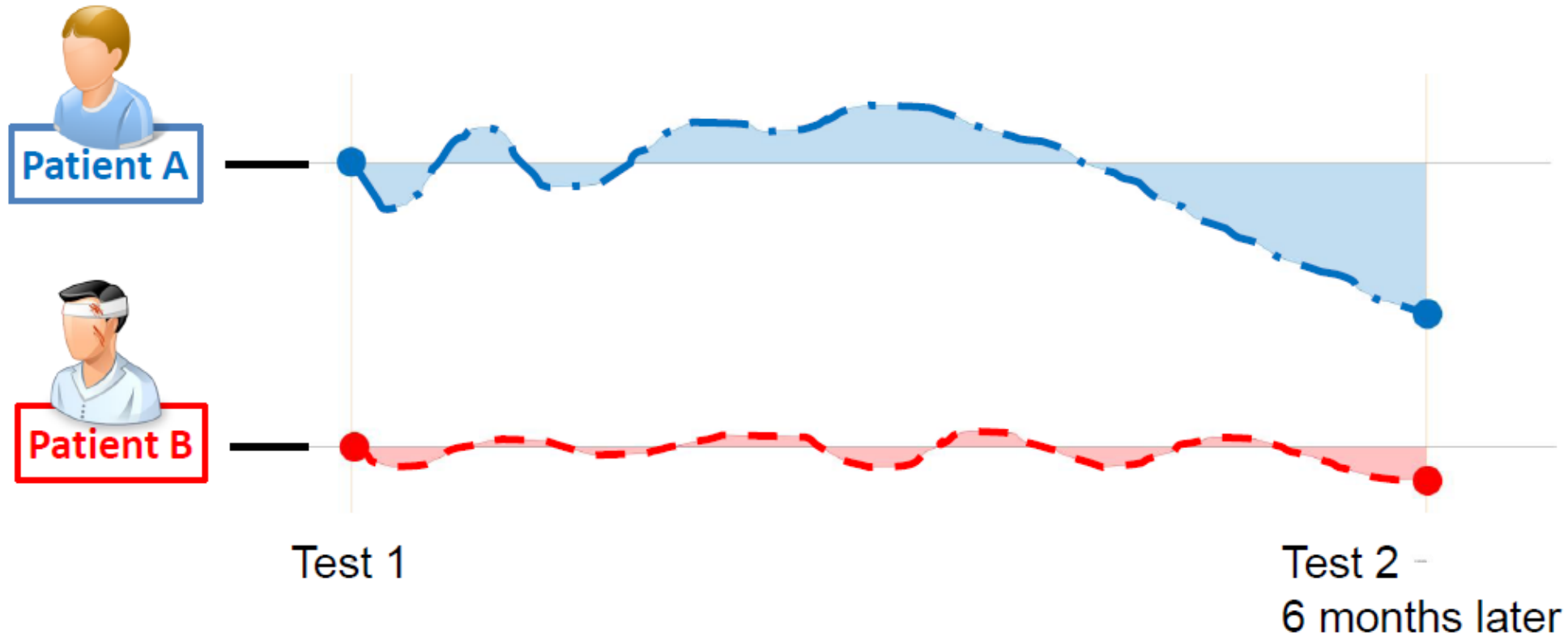
What the Doctor **sees**...



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 ?

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

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, MS¹; Amylou C. Dueck, PhD⁴; [et al](#)

» [Author Affiliations](#) | [Article Information](#)

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

Patient Generated Data using aparito.com



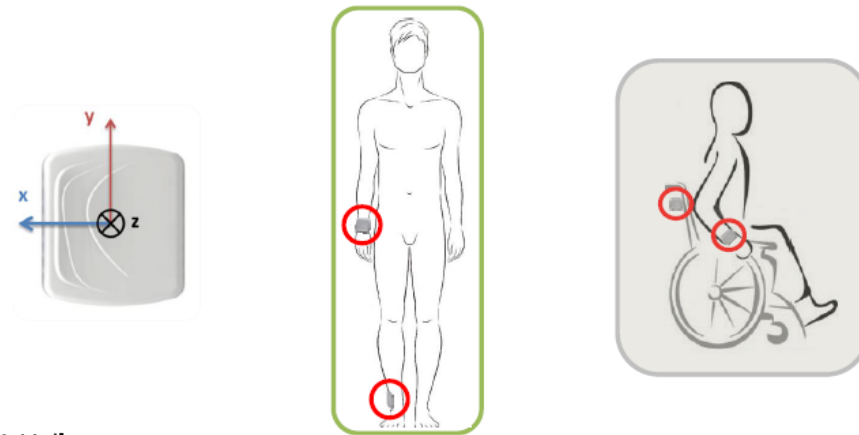
- ✓ Vastly improved understanding of diseases
- ✓ Increased chance of regulatory approval
- ✓ Better, cheaper & faster drug development
- ✓ Higher patient empowerment & quality of life
- ✓ More participants & engagement
- ✓ Lives Saved & Improved

aparito

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

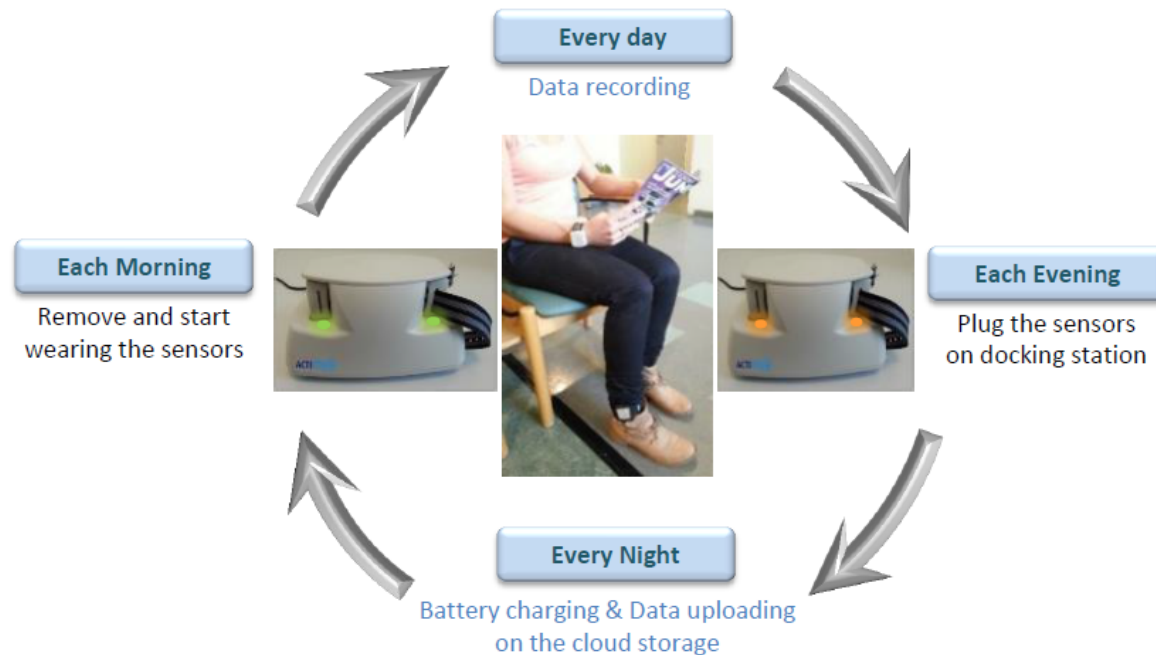


Example : Ankle trajectory during a 6WT

Patient Generated Data via wearables

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.



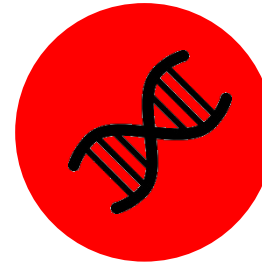
Patient Generated Data via Health29 (www.foundation29.org)



Social Q&A



PROMs



Genotype



Phenotype

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

Source: <https://www.foundation29.org>





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

The screenshot displays the 'Duchenne Data Platform' interface. On the left is a navigation menu with items: Home, Personal information, My clinical history, Anthropometry, Course of the disease (highlighted), Medical care, Medication, Clinical trials, My genetic information, FAQ, and Privacy policy. The main content area is titled 'Course of the disease' and lists various categories: Diagnosis, Physical outcomes, Heart condition, Respiratory condition, Nutrition, Gastrointestinal, Urinary tract (highlighted), Bone health and orthopedics, (Technical) support, Learning and behaviour outcomes, and Problems and comorbidity. The 'Urinary tract' form asks the user to 'Please, choose which options are applicable to you, if any'. It includes three toggle switches: 'NO' (off), 'DRIBBLE' (on), and 'FEELING THAT BLADDER IS NOT COMPLETELY EMPTY (URINARY RETENTION)' (off). Below these are two date input fields labeled 'START DATE' and 'END DATE', both with the placeholder 'mm/dd/yyyy'. At the bottom right, there is an 'INCONTINENCE' toggle switch (off) and a red 'Assista' chat button.



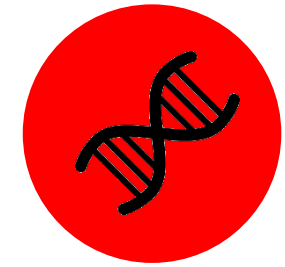
- Deep phenotyping using CNLP
- Standard HPO
- Based on AI for symptoms identification
- Phenotype can be initiated by patients

8 Symptoms

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

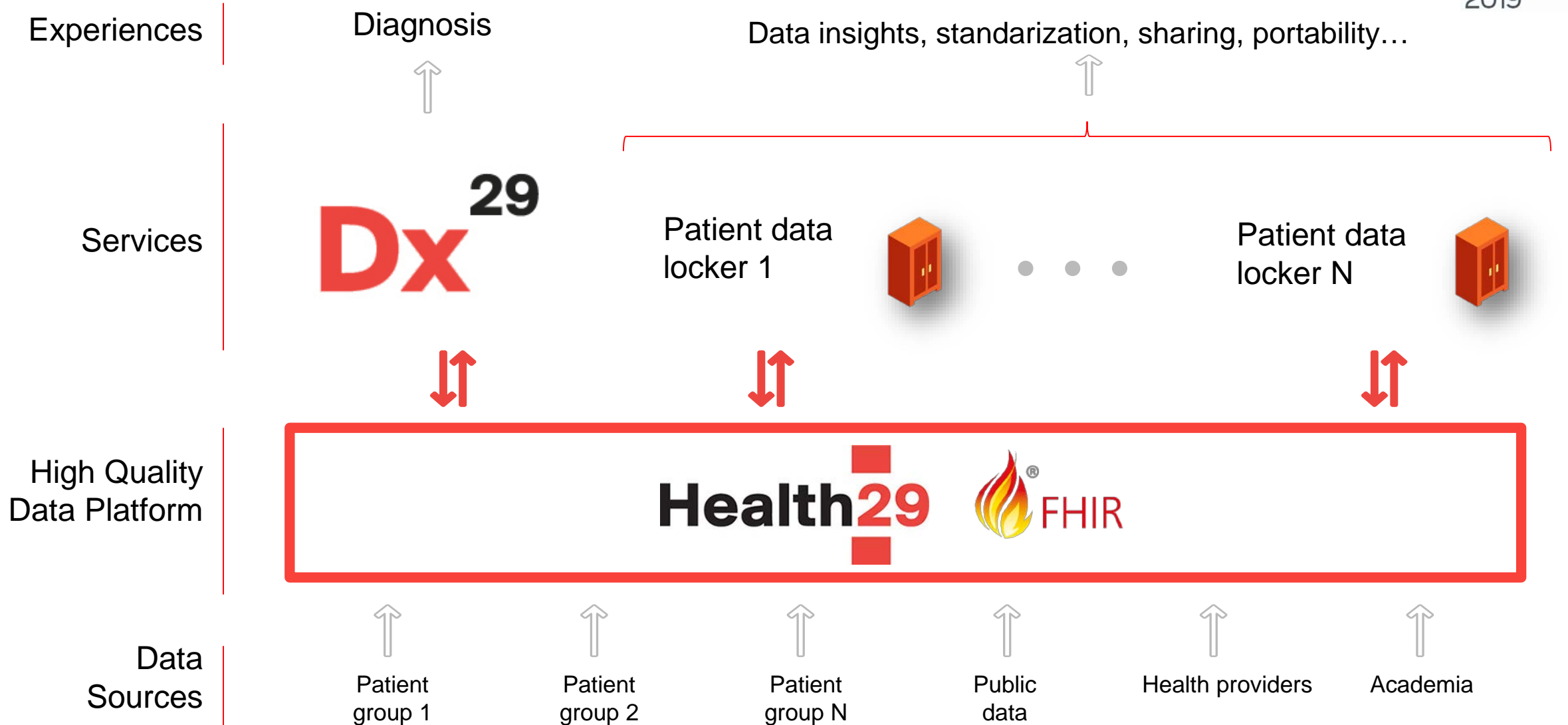
Name	Max. Similarity	# / Apparitions	Select/deselect
Calif muscle pseudohypertrophy	0.83	#: 1 Q dystrophy with characteristic pseudohypertrophy of the calves	<input checked="" type="checkbox"/> <input type="checkbox"/>
EMG: myopathic abnormalities	0.985	#: 1 Q myopathic changes by electromyography	<input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



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



Patients driving research



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

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VROOM¹¹ | VALERIA RICOTTI⁴ | NATALIE GOEMANS⁶ | CRAIG MCDONALD⁹ | EUGENIO MERCURI² | ON BEHALF
OF THE PERFORMANCE OF THE UPPER LIMB (PUL) WORKING GROUP

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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).

 Home Personal information > My clinical history > My genetic information FAQ Privacy policyDuchenne Data Platform 

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



RareConnect.org

940

patient advocacy groups

400

moderators

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



RareConnect



Social

My Studies



<https://www.rareconnect.org/en>



Patients &
Caregivers



Advocacy
Organizations

(De-identified data)



<https://mystudies.rareconnect.org>



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.

Self Phenotyping Survey

Brain/Nervous System

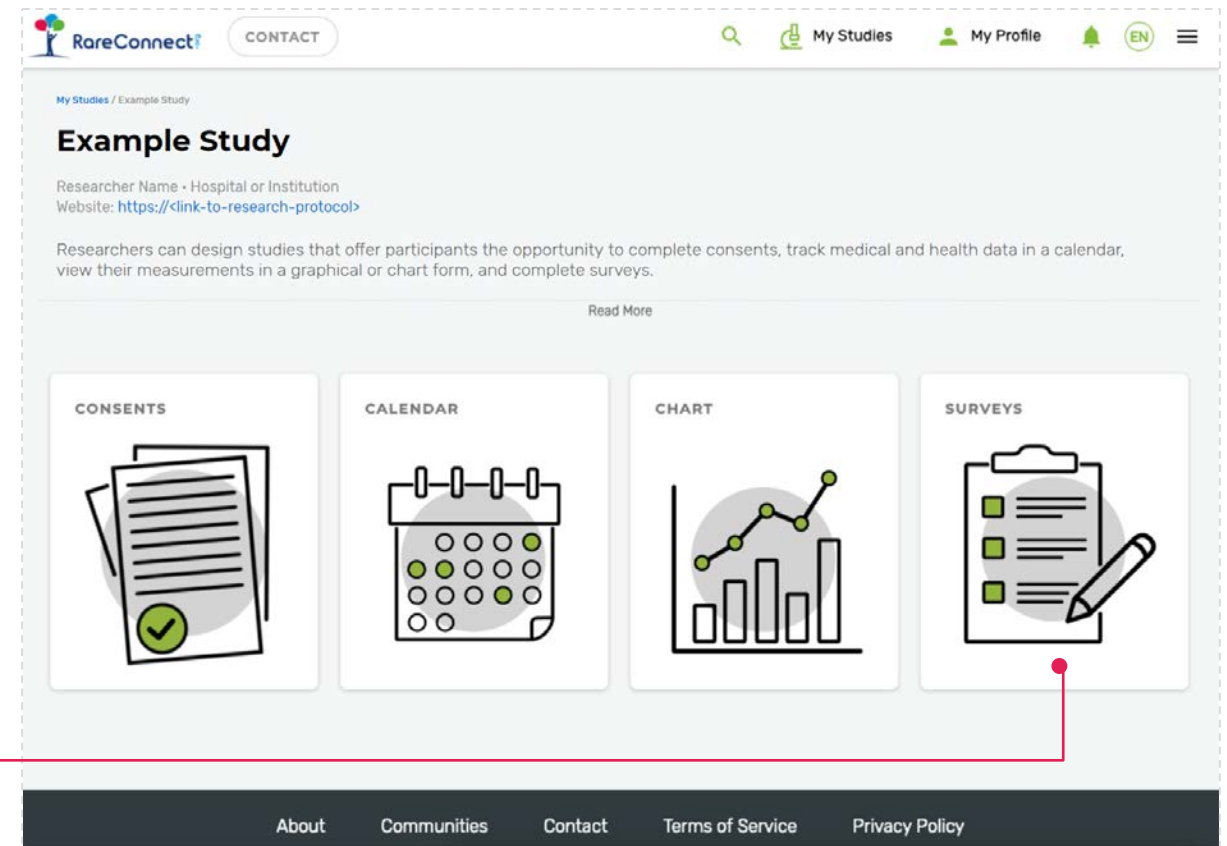
6. * Have you had issues with your brain and/or nervous system?

- Yes
- No
- Unsure
- Prefer not to answer

7. * What specific brain/nervous system issues have you had?

- Cognitive impairment
Significant difficulties learning, understanding, processing or memorizing information, or making decisions
- Coordination issues
Significant clumsiness or other difficulties with movement due to body parts not working together as they normally would, including ataxia
- Hypertonia
Muscle tightness that limits movement, speedily or stiffness, increased muscle tone
- Hypotonia
Unusually low muscle tone, muscle tone may have been described as floppy
- Unusual movements
Changes or problems in the ability to move body parts voluntarily, including tremors, abnormal reflexes, difficulty walking, strange gait, or unusual posture
- Seizures
Also known as convulsions
- Developmental delay
Delay in acquiring motor skills, speech/language abilities, cognitive and social skills, and other developmental milestones, in young children
- Speech disorder
Inability to pronounce or articulate sounds, stuttering, or other speech impairment
- Intellectual disability

CANCEL PREVIOUS NEXT



The screenshot shows the 'Example Study' page on the RareConnect platform. At the top, there is a navigation bar with the RareConnect logo, a 'CONTACT' button, and user options for 'My Studies', 'My Profile', and a language selector set to 'EN'. Below the navigation bar, the page title is 'Example Study', followed by the researcher's name and a link to the research protocol. A brief description states that researchers can design studies with consents, calendars, charts, and surveys. A 'Read More' link is provided. The main content area features four large, interactive cards: 'CONSENTS' (with a document icon and a checkmark), 'CALENDAR' (with a calendar icon), 'CHART' (with a bar and line graph icon), and 'SURVEYS' (with a clipboard and pencil icon). A red line connects the 'SURVEYS' card to the 'Self Phenotyping Survey' form shown in the adjacent image. At the bottom, a footer contains links for 'About', 'Communities', 'Contact', 'Terms of Service', and 'Privacy Policy'.

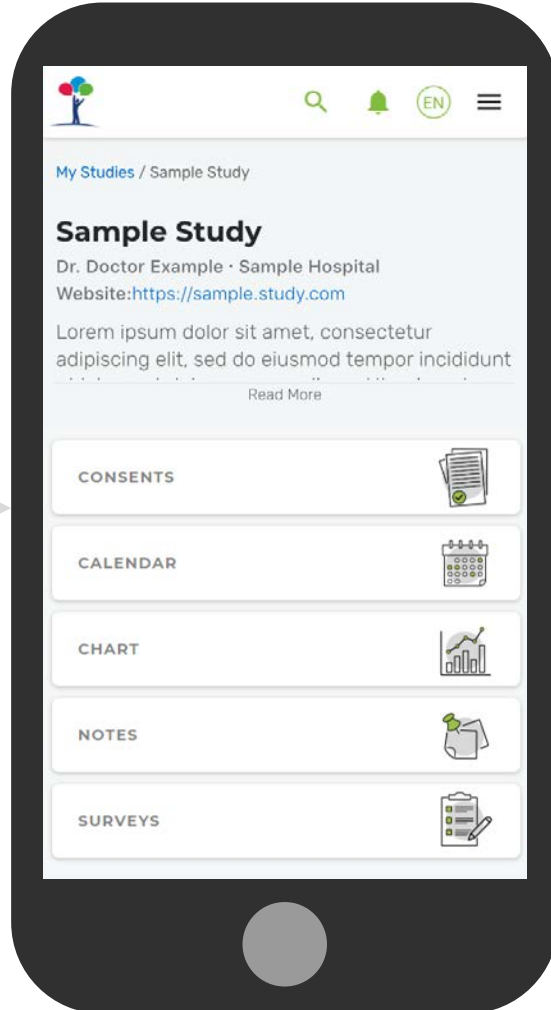
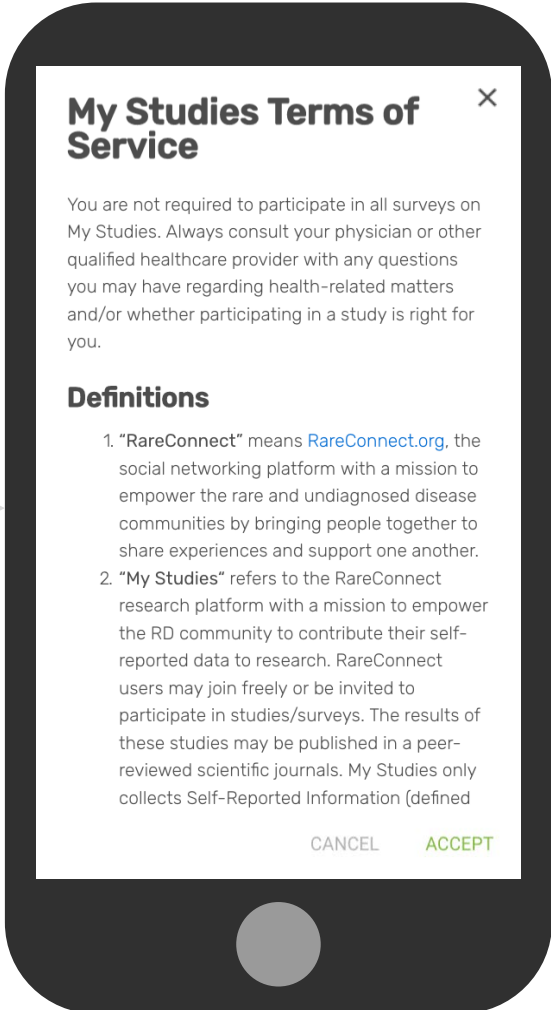
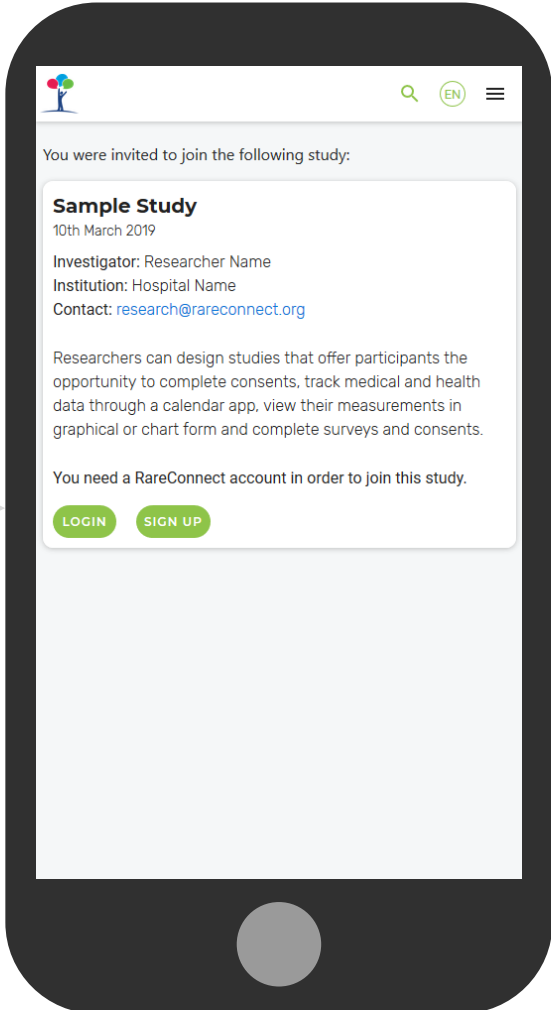
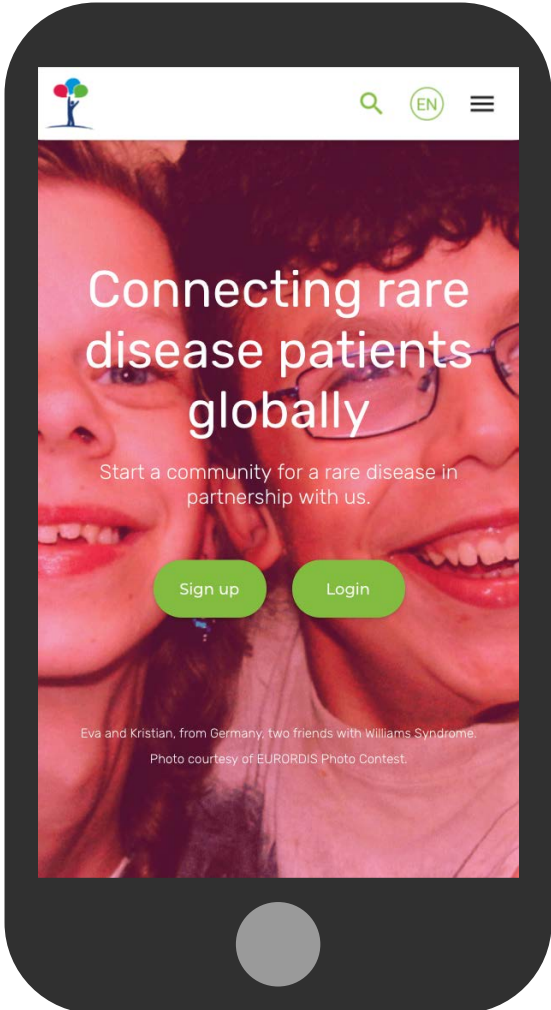
Patient Reported Outcomes (PROs)

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.

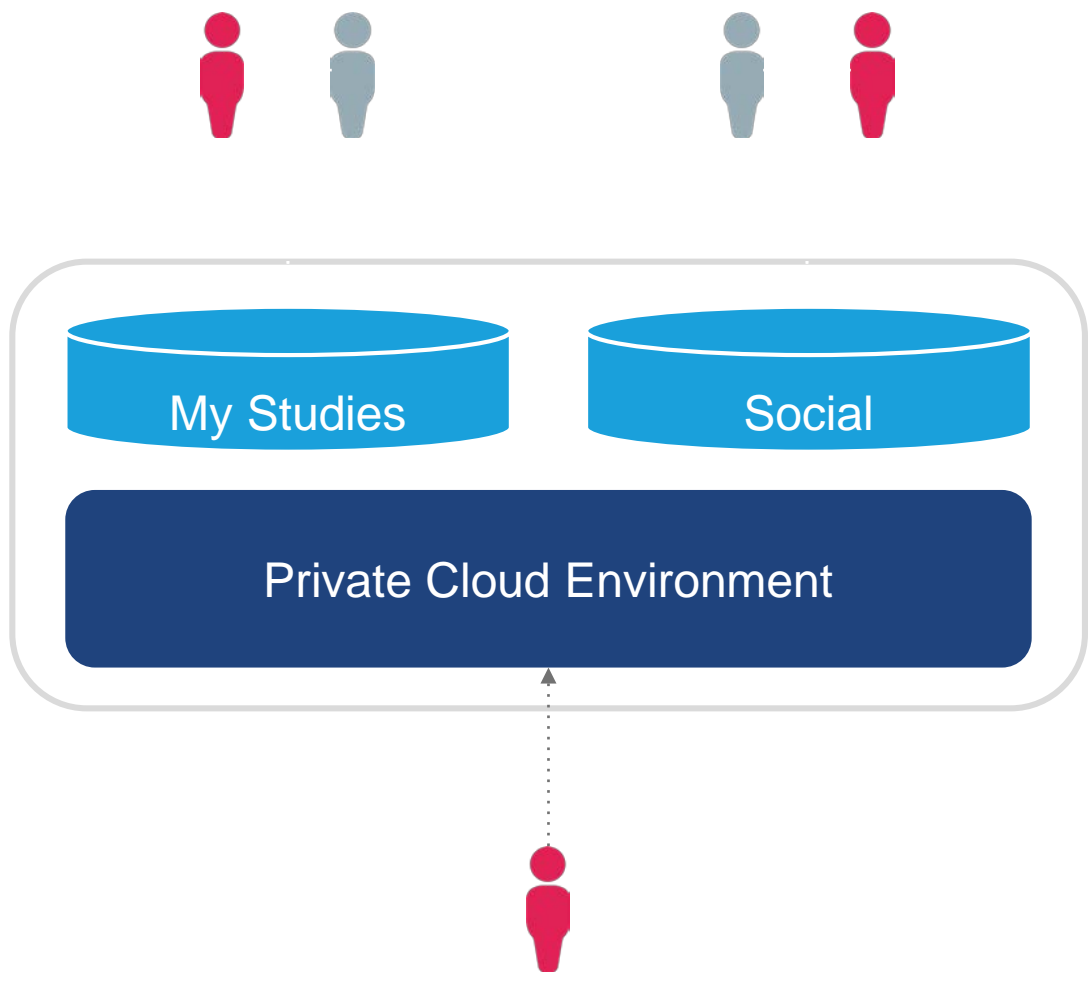


Participant registration



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

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 Assessment)
- Consider how much resources you will need. Can't give you a figure as each case is different!

Thank you!



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