The Patient Voice

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.

Ryan Fischer
SVP Community Engagement

“The value placed on the benefits and risks of any treatment can only *truly be understood* by the **patients/caregivers** themselves, for it is *they* who have to make the treatment choices and it is *they* who bear any associated risks.”

(~ *quote from a health economist at a recent conference*)

Gap between scientific data and patient experience

- Disease burden & unmet need
- Outcome measures and endpoints
- Preferences w/ risks, benefits, uncertainty
- Symptom priorities
- Quality of life
- Meaningful benefit

Patient/Caregiver Data Takes Many Forms…

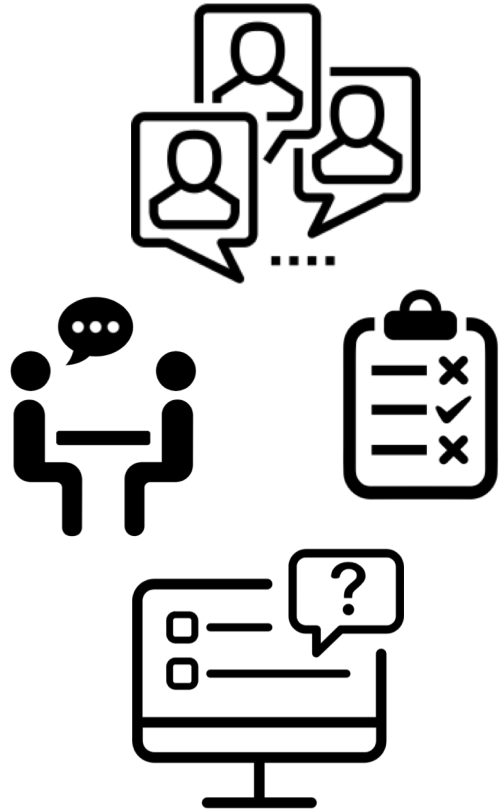
Qualitative Data *(Describes)*

- Focus Groups
- Open Ended Survey Questions
- Testimonies
- Interviews (in person or phone)
- Town halls

Quantitative Data *(Defines)*

- Patient Registries
- Patient Reported Outcomes
- Quality of Life Instruments
- Polling data
- Surveys

Preference Data (can do both)



Preference Studies – Quantifying the patient voice

5 studies completed to date

- Caregivers preferences
- Symptom priorities
- Meaningful pulmonary outcomes
- Multi-stakeholder preferences
- Preferences for emerging gene therapies

Underway

- Global preferences for treatments

Academic collaborators

Dr. John Bridges (The Ohio State)
Nonie Crossnohere (JHU)
Holly Peay (RTI International)

Funding and participation on advisory groups:

- Pfizer
- Everylife Foundation
- Santhera Pharmaceuticals
- Solid Biosciences

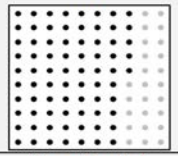
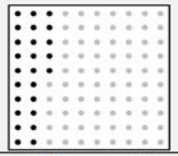
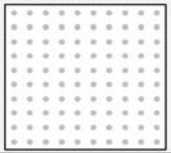
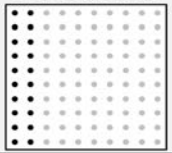
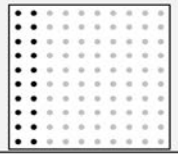
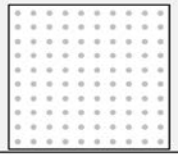
Stated Preference Methods

- Method … what people …

  Quantita… in …

  - How …
  - ***How*** … ***fit?***
  - ***How do*** … ***ng a decisior***

Task 2

	Drug A	Drug B
Does it slow disease progression?	Slows for 5 years	Slows for 3 years
How many people would benefit?	75% 	25% 
What is the extra risk of kidney damage?	No extra risk 	20% extra risk 
What is the extra risk of fracture?	20% extra risk 	No extra risk 
Which drug would you choose?	☐	☐

Consistent themes learned from the data

- Stopping or slowing disease progression are *both* valued treatment outcomes, patients and caregivers are willing to take on risk and uncertainty to achieve these outcomes.
- The community prioritizes protecting muscle function and treatments targeting secondary symptoms of cardiac and pulmonary function most and are willing to take on risks, burdens, and uncertainties for benefit.
- An appropriate balance of benefits and risks may be different for people with Duchenne or Becker, their caregivers, and the professionals who develop treatments and manage medical care.
- Decision making for clinical trial participation is mainly driven by the chance for benefit (skeletal, cardiac, pulmonary) over risks, burdens and uncertainties.

Why this matters

- These studies allow us to engage a large group of community members in patient centered research
- Your contributions to these studies enable us to better communicate your preferences to a range of stakeholders in the drug development ecosystem
- Preference change over time, we will continue doing these studies in order to accurately reflect the current environment
    - Global study underway (6 countries)
    - Thank you to everyone who has contributed data!

THE DUCHENNE REGISTRY

Parent Project Muscular Dystrophy

JOIN THE FIGHT.
END DUCHENNE.

The Duchenne Registry

Ann Martin, MS, CGC

Jen Ely, MS, CGC

- Online self-reported registry for individuals with Duchenne or Becker, as well as carrier females
- Established by PPMD in 2007, in collaboration with Emory Genetics and the Centers for Disease Control
- Largest patient reported registry globally

PPMD

#PPMDConference

Inform understanding on NH of DBMD

- Family Hx and Location
- Genotype/phenotype
- Care interventions
- Collect PROs on disease

Patient Resources

- Free genetic counseling
- Free genetic testing
- Clinic trial education
- Trial Finder
- Decision making tool

Drug Development

- Recruit for studies
- Prep to trial
- PROs on standard of care

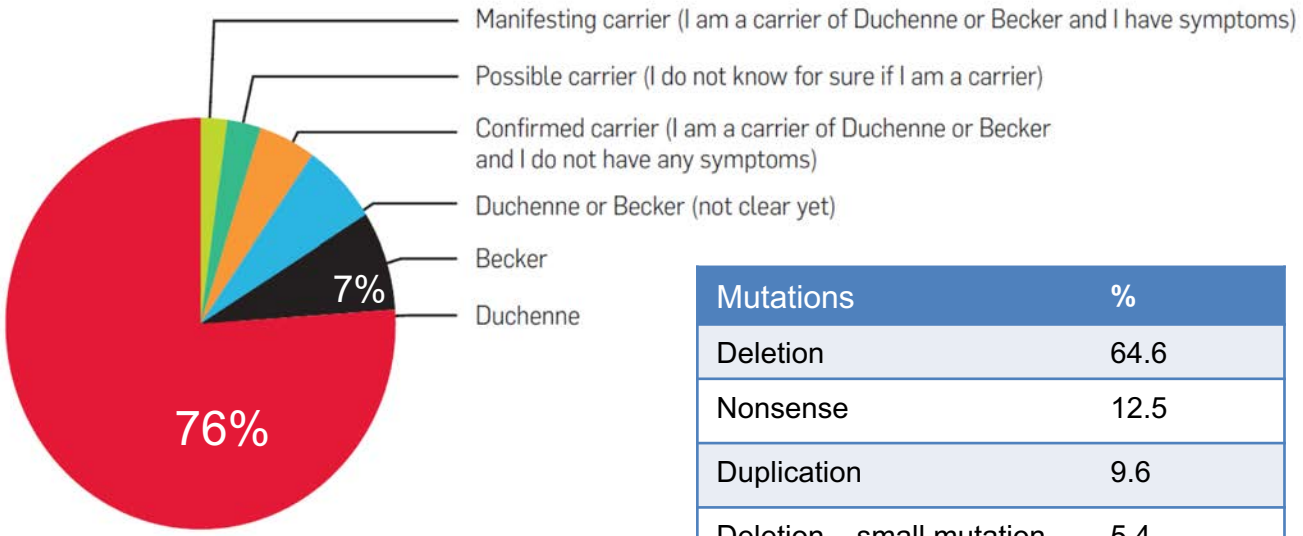
Share information

- Individuals with DBMD
- Families and caregivers
- Healthcare providers
- Researchers
- Industry
- Regulators
- Payers

REGISTRY AIMS AND GOALS

Diagnosis

5000+ Registrants



Mutations	%
Deletion	64.6
Nonsense	12.5
Duplication	9.6
Deletion – small mutation	5.4
Splice site	3.9
Duplication small mutation	2.2
Insertion	0.8
Missense	0.5
other	0.4

Registry Modules	
Genetic information	Behavior
Medications	Bone Health
Muscle Function	Family History
Insurance	Pain
Cardiac	Steroids
Respiratory	Diagnosis

PPMD

#PPMDConference

10-Year Registry Report

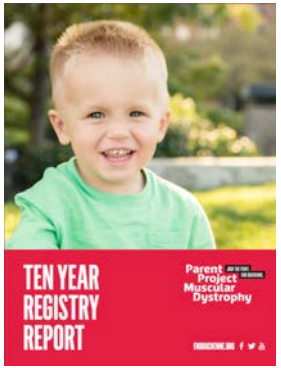
IMPACT

Over 5000 Registrants

115 countries

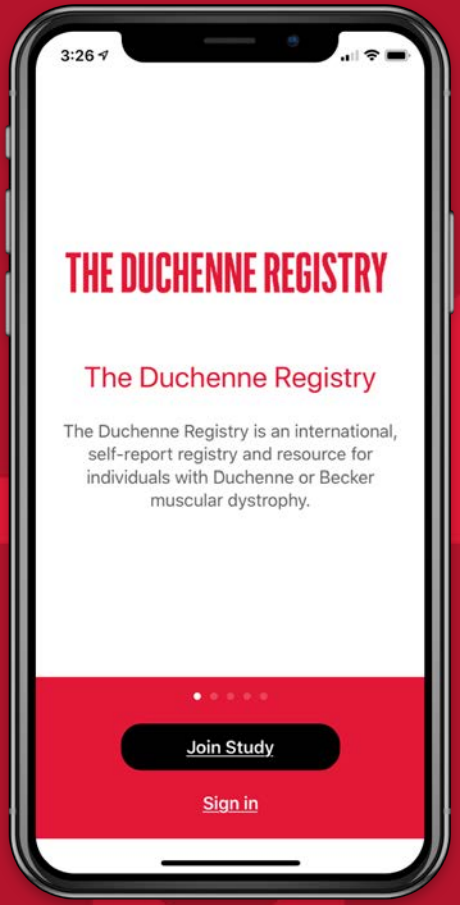
100+ Trials and Studies Recruited

Data collected over 12 years



PPMD

#PPMDConference

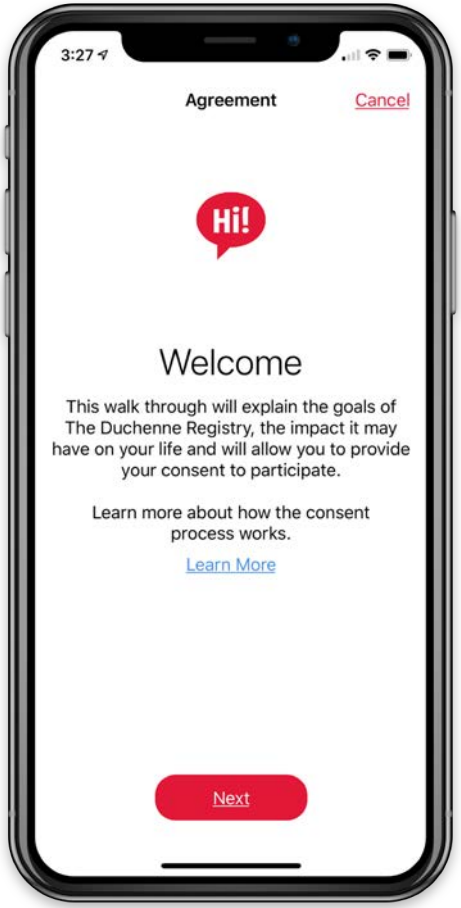


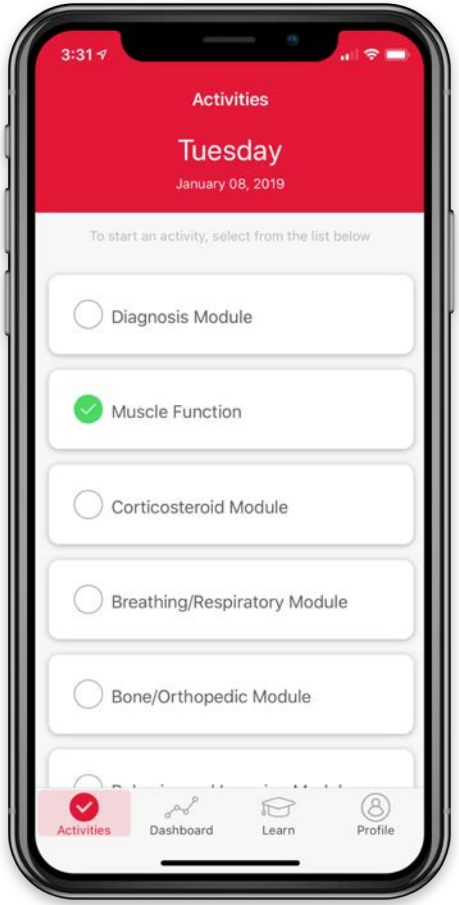
The NEW Duchenne Registry Platform (app based)

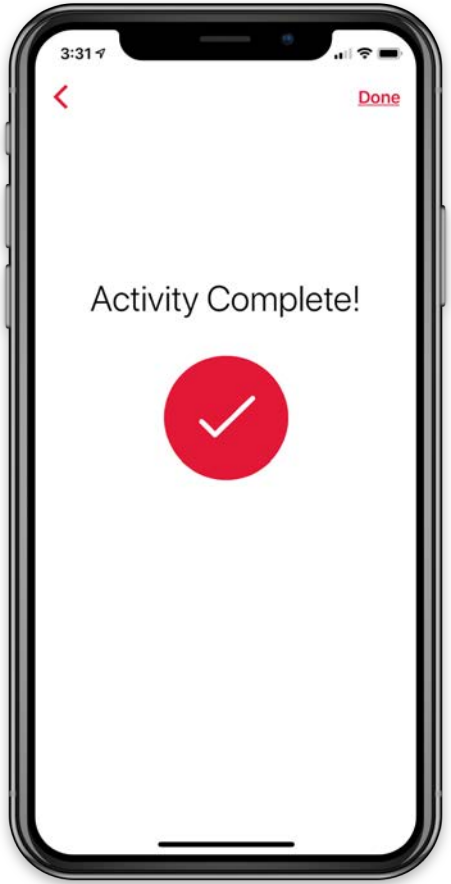
Set to launch late summer

In partnership with

THREAD™

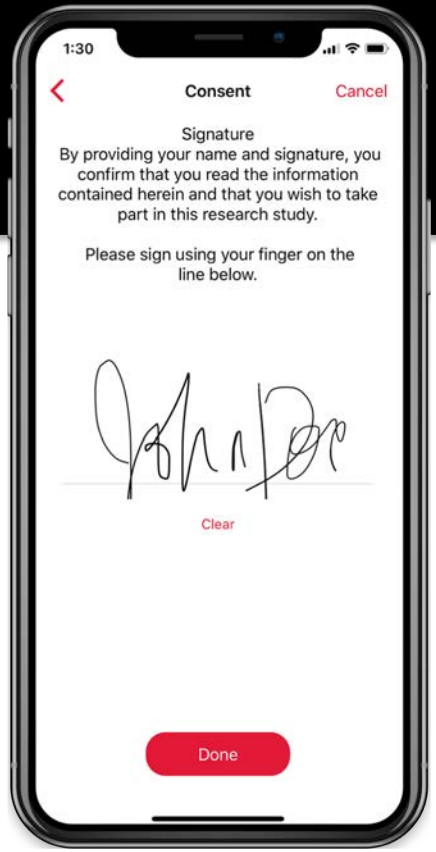

3:27
Agreement
Cancel
Hi!
Welcome
This walk through will explain the goals of The Duchenne Registry, the impact it may have on your life and will allow you to provide your consent to participate.
Learn more about how the consent process works.
Learn More
Next


3:31
Activities
Tuesday
January 08, 2019
To start an activity, select from the list below
Diagnosis Module
Muscle Function
Corticosteroid Module
Breathing/Respiratory Module
Bone/Orthopedic Module
Activities
Dashboard
Learn
Profile


3:31
Done
Activity Complete!

Consent Process

- Quick and straight forward consent process to allow for your de-identified (anonymous) data to be used for research purposes



Parent Project Muscular Dystrophy

DUCHENNE OUTCOMES RESEARCH INTERCHANGE

DUCHENNE OUTCOMES RESEARCH INTERCHANGE

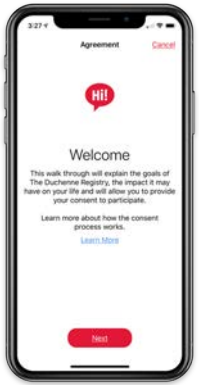
The Interchange combines **patient-reported data**, **clinician-reported data** (on approved therapies), and data from **electronic health records** (care visits), to analyze and understand *real world evidence* about the Duchenne and Becker progression.

- PPMD partnered with Sarepta Therapeutics to launch the Interchange
- Data from the Duchenne Registry is the first patient reported data included in the Interchange
  We are looking to marry the PRO data with Electronic Health Records.
- Data from patients on Exondys 51 (eteplirsen) will be the first post-market surveillance data in the Interchange
- The Interchange is built as a model for additional Industry partners to be added on after the pilot of Exondys 51 (Sarepta's EVOLVE Registry)

PPMD approach to capturing Electronic Health Records with Clinical Care Centers –

How PPMD will enhance the new Duchenne Registry

You will complete an informed consent in the new PPMD THREAD platform giving PPMD permission to pull Electronic Health Records



Consented List
Patient A
Patient B
Patient C

PPMD prepares a list for each institution of all consented patients seen at that institution

HIPPA Compliant Data Transfer

Institution shares with PPMD data elements in EHR for these patients through a secure API

Secure API

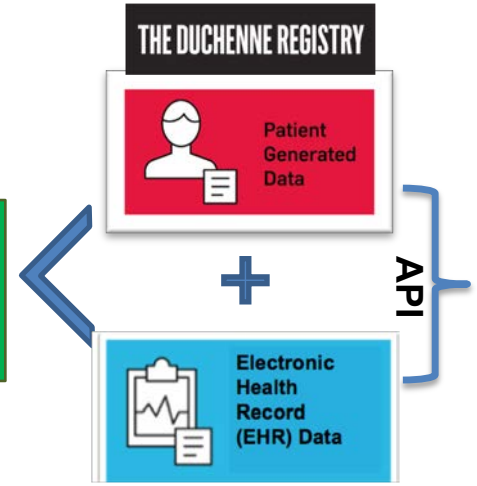
Data is ingested into new Interchange

Duchenne Interchange

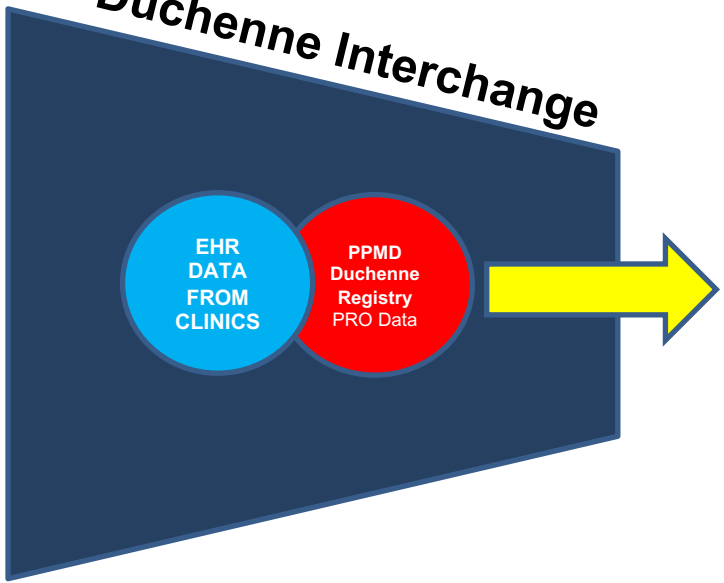
PPMD Electronic Health Record Capture

Example of how it works:

Patient consents with **The Duchenne Registry** and **"Springfield Hospital"** to push their EHR data to Interchange



(EHR data elements
Patients from "Springfield Hospital")



Doctors at "Springfield Hospital's" are provided access to secure *Data* in Interchange (PPMD PRO + EHR data elements)

The good news for clinics!
We are NOT asking clinics to enter anything into a *new* database

PPMD

#PPMDConference

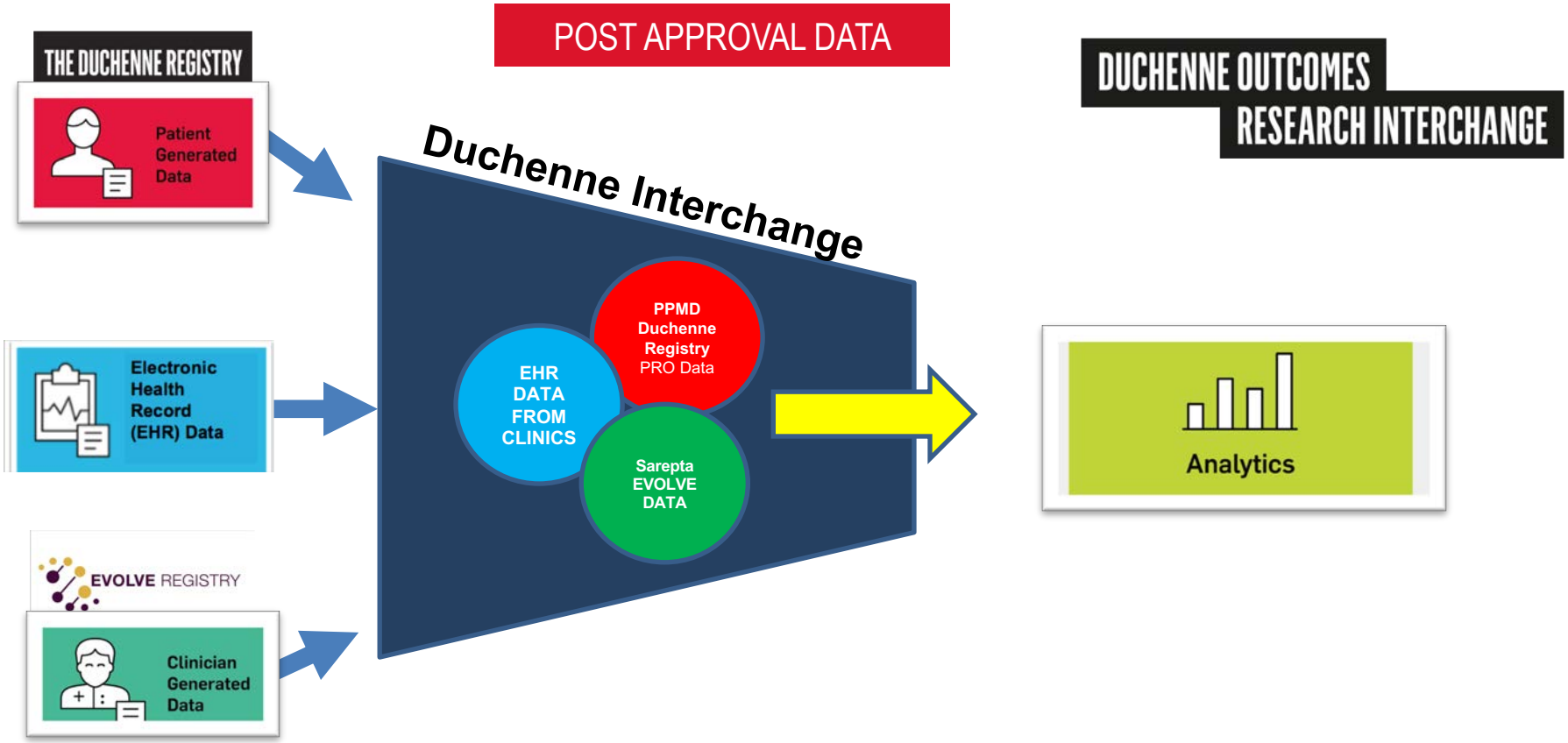
EVOLVE - Sarepta's Registry on Approved Therapies

- The Evolve Registry is owned and operated by Sarepta and is an important and exciting project to gather real world evidence data in DMD patients who are on their approved medications.

- The Evolve Registry has started enrolling sites and recruiting patients.

EVOLVE REGISTRY

SAREPTA THERAPEUTICS


POST APPROVAL DATA
THE DUCHENNE REGISTRY
Patient Generated Data
Electronic Health Record (EHR) Data
EVOLVE REGISTRY
Clinician Generated Data
Duchenne Interchange
EHR DATA FROM CLINICS
PPMD Duchenne Registry PRO Data
Sarepta EVOLVE DATA
DUCHENNE OUTCOMES RESEARCH INTERCHANGE
Analytics

What do I need to know and do?

- The NEW Duchenne Registry App is launching this fall!
  - **Visit The Duchenne Registry at Interaction Alley at Conference!**
  - Be sure to indicate with us which clinic you are currently receiving care
  - Provide us with the most up to date email for your account(s)
  - Be on the look out for emails from us this summer! (check your email!)
- For those on Exondys 51, you will be notified by your clinic if they are participating in the EVOLVE registry.

Conference Polling

Conference Polling Goals

- **Gain a better understanding** from families on a range of topics
- **Provide additional insights for discussions** taking place during conference
- **Inform drug development, trials, and care** through the lens of patients, caregivers, and families

Polling Guidelines

Polling will take place on Thursday, Friday, and Saturday in between sessions

Those watching the stream can also participate

To participate visit: pollev.com/ppmd

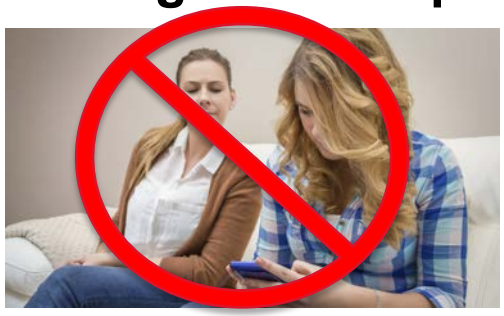
Wireless code: PPMD2019

Polling Questions

- Questions are for people with Duchenne and Becker, their caregivers and family members only
- Some questions require only one response per family; we will indicate when we only need one response
- Answers are anonymous

Polling Advice

- **Don’t fall victim to *peer pressure* with the answers on screen**
  - Answer the polling question on your phone **before** looking at the results on the larger screen
- **Don’t look at your neighbors responses**



Polling warm up