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FasterCures

Vital Voices Webinar Series: Patient Organization Strategies to Inform CMS Access and Coverage Decisions

Part 2: Just the Facts: Patient Experience Data to Inform CMS Decision-Making

September 30, 2025

FasterCures Programs

Mission: To build a biomedical innovation system that is effective, efficient, and driven by a clear vision: patient needs above all else

R&D Environment

- ENRICH-CT (Enabling Networks of Research Infrastructure for Community Health Through Clinical Trials)
- Representation in Clinical Trials
- Future of Biomedical Innovation

Policy

- CMS/FDA Alignment: Accelerating Treatments to Patients
- Building Patient Engagement Capabilities at CMS
- Prevention-First Health

Patient Engagement

- TRAIN (The Research Acceleration and Innovation Network)
- LeadersLink
- Patient Engagement in Medtech Development
- Vital Voices: Patient Engagement with CMS

Innovation

- Future of Cancer Care in the US
- Cell, Gene, and RNA Therapies
- Emerging Technologies
- Data and AI

International

- Project Prevent
- Global Cancer Care
- Anti-Microbial Resistance
- Early Warning System

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TRAIN: The Research Acceleration & Innovation Network

The objectives of TRAIN are to:

- **To encourage more entrepreneurial philanthropy in medical research**
- **To build more and better networks with other R&D stakeholders**
- **To enhance the influence of the network**

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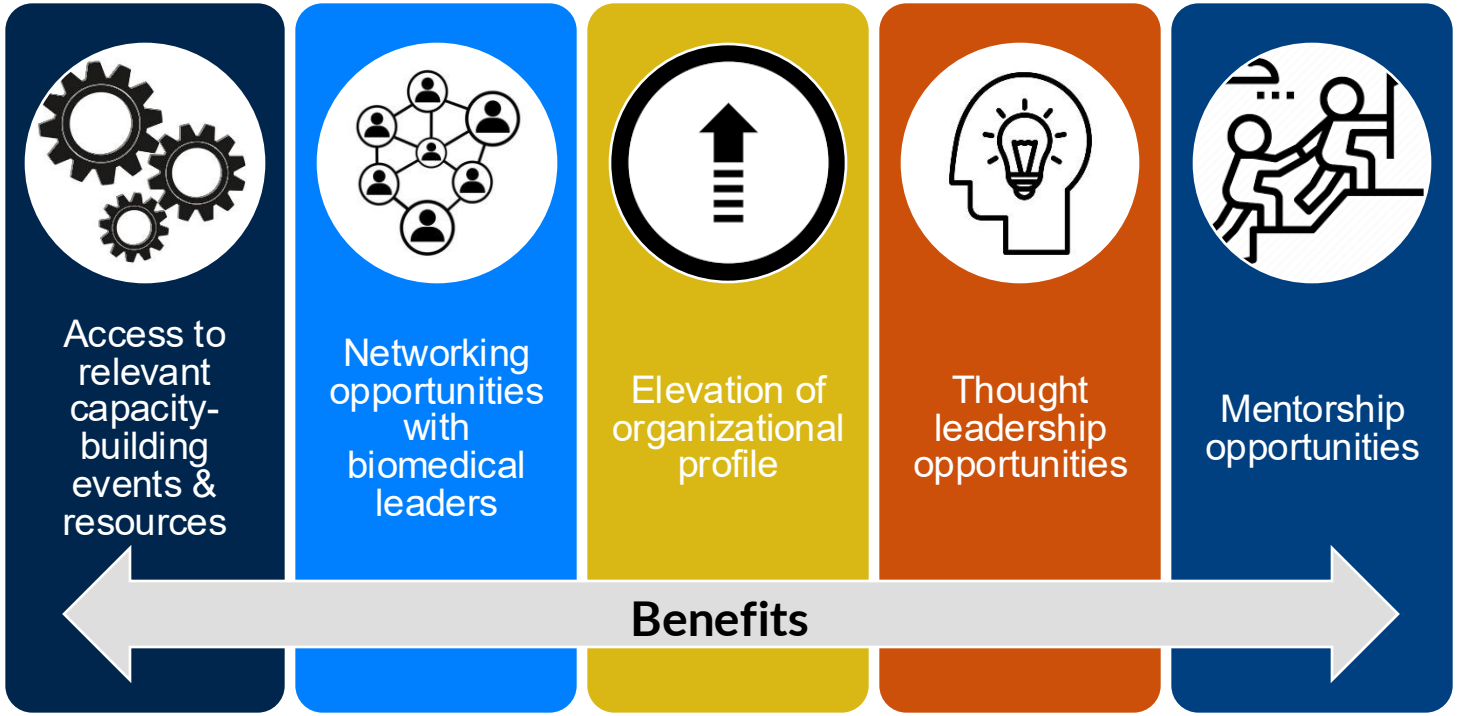
Join TRAIN and/or serve as a mentor for a rare disease org!

Join TRAIN and enjoy the following **benefits:**



There is no cost to apply and membership is free!

Apply to TRAIN today!

Questions? TRAIN@milkeninstitute.org

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Milken Institute Future of Health Summit

In Service of Better Health

November 4-6, 2025

Salamander Washington DC

The Summit will amplify innovative partnerships, targeted research, thought leadership, and policy priorities emerging from the Institute's commitment to the fields of public health, food, aging, strategic philanthropy, and biomedical innovation.

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Background

- The Centers for Medicare and Medicaid Services (CMS) is the bellwether to which other payers and value assessors look for guidance and leadership.
- Patient organizations bring unique, real-world insights that can shape CMS's decisions on treatment access, yet many groups are unaware of how or when to effectively engage.
- Providing patient organizations with practical tools and peer-driven examples strengthens their ability to advocate for equitable, patient-centered care policies.

About the Webinar Series

The FasterCures Vital Voices three-part webinar series explores how patient organizations can inform and potentially influence CMS activities and state-level coverage decisions. Each session focuses on a key strategy for engagement:

- **Part 1:** Engaging in coalitions to impact CMS decision-making (August)
- **Part 2:** **Collecting and leveraging patient experience data (September)**
- **Part 3:** Navigating state-level policy and coverage (November)

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Access the Vital Voices workshop materials here

Today's Webinar

•Objective: Explore effective methods for gathering, analyzing, and presenting patient experience data to shape coverage policies and pathways

•Key topics will include:

- Generating patient-generated evidence to inform CMS national and local coverage pathways
- Real-world case examples of successful data-driven advocacy
- Actionable tips for patient organizations beginning or expanding their data collection efforts

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Speakers

Kate Davidson, MSW

Director, Learning and Diffusion Group

Center for Medicare and Medicaid Innovation (CMMI)

Joe Vandigo, MBA, PHD

Principal Scientist and Director of Value

Applied PX

Megan Freed, MPH

Chief of Strategic Initiatives

Parent Project Muscular Dystrophy (PPMD)

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Kate Davison, MSW
Director, Learning and Diffusion Group
Center for Medicare and Medicaid Innovation (CMMI)

Joe Vandigo, MBA, PHD
Principal Scientist and Director of Value
Applied PX

Just the Facts: Patient Experience Data to Inform CMS Decision-Making

30 September 2025

1 – 2 PM ET

A multi-year, multi-stakeholder process to solve a problem of fragmentation in patient-centered evidence

2022

Identify solution

- Formed advisory board
- Environmental scan
- Stakeholder interviews
- Patient community workshop
- PE Dossier concept emerges

2023

Co-develop preliminary template and socialize concept

- Advisory board
- Workshop at PEOF
- Patient community workshops (n=2)
- Stakeholder interviews
- ISPOR Workshop
- Concept described in Health Affairs Forefront

2024

Pilot dossier template and develop implementation strategy

- Collaborate with patient groups to produce dossiers
- Socialize PE dossiers among key audiences (e.g., life science industry, academic researchers, regulators, payers, HTA bodies)

2025

Pilot dossier template

- Continue collaborations to product dossiers
- Identify opportunities for patient groups to submit PED through existing and emerging processes
- Framework described in Health Affairs Forefront

Patient experience dossiers compile patient-centered evidence, highlight gaps, and inform patient-focused value narratives.

1. Patient, healthcare provider, manufacturer, payer, and other stakeholder Input
2. Gray literature
3. Targeted literature review

Patient Experience Dossiers

are narrative reports in which patient groups consolidate and contextualize disease-specific experience data from various sources, covering topics including the patient journey, disease burden, treatment experiences, patient-centered outcomes, and unmet needs.

- **Topic 1**. Patient Journey & Access to Care
- **Topic 2**: Burden of Disease & Impacts on Daily Life
- **Topic 3**: Patient Perspectives on Current & Future Treatments
- **Topic 4**: Patient-Centered Outcomes & Measurement Tools

Enhance regulatory submissions and global value dossiers with patient-centered narratives and data.

Develop a tool for patient organizations to effectively communicate patient-centered evidence to decision-makers.

Identify gaps in patient-centered evidence to guide future research priorities.

Each dossier is a "living document" updated over time.

Source: Oehrlein EM, Escontrías O, Chapman H, Vandigo J. Patient Experience Dossiers: Connecting Decision Makers With Patient-Centered Evidence. Health Affairs Forefront, September 9, 2025. [Link]

Patient experience dossiers are built on a framework of topics where decision-makers value patient experience evidence.

Patient journey and access to care

- Disease onset and diagnosis
- Signs and symptoms
- Defining characteristics and different patient populations
- Clinical care and navigating the health system
- Barriers and facilitators of high-quality care
- Access, adherence, and affordability barriers among different populations

Burden of disease and impacts on life

- Patients' day-to-day experiences living with the condition(s), including psychosocial impacts
- Economic impacts
- Caregiver and family impacts
- Direct medical costs
- Non-clinical healthcare costs
- Social impacts
- Ability to work and job impacts
- Education impact

Patient perspectives on current and future treatments

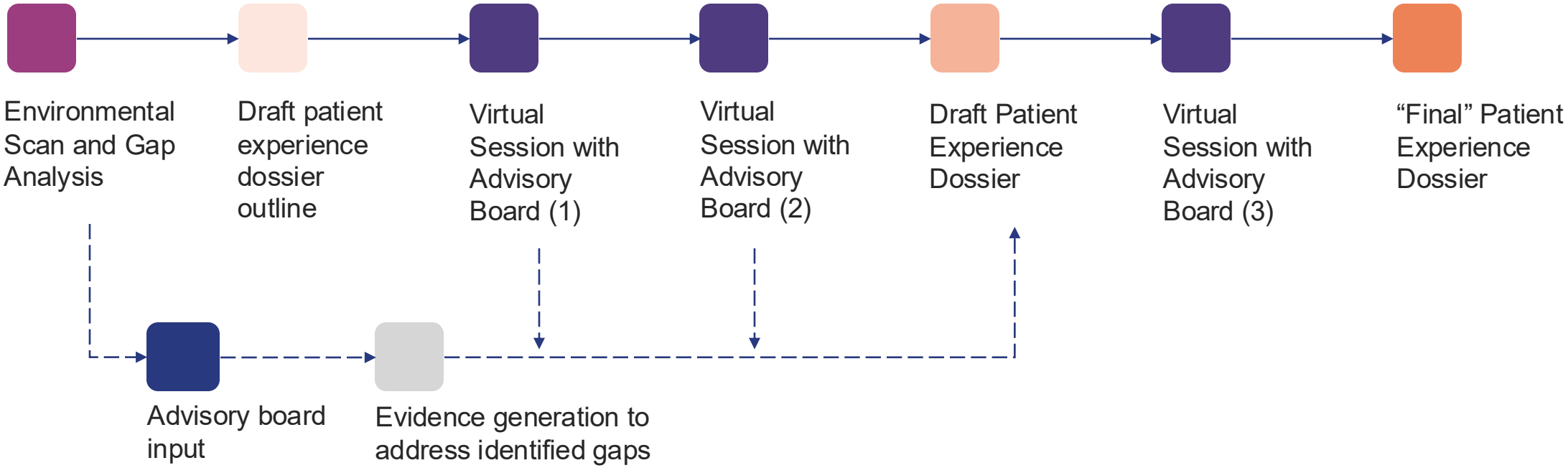
- Patient's perception of the benefits and side effects of current treatment option(s)
- Unmet medical needs
- Attributes of an ideal treatment
- Considerations when patients are choosing among different treatment options

Patient-centered outcomes and measurement tools

- Outcomes that matter most to patients with the condition(s)
- Patient perspectives on meaningful improvements
- Patient perspectives on tools used to measure clinical outcomes, quality of life, or health-related quality of life

This framework was developed based on input from an advisory board, environmental scan, and key informant interviews, patient community workshops, the ISPOR workshop, and patient engagement open forum workshops.

The patient experience dossier process is patient-centered and structured to maximize engagement.



This is a simplified overview of the process. In practice, the dossier can be created around an instigating event (e.g., a PFDD meeting or a life science product entering clinical trials) and include opportunities for external stakeholder review and additional touch-points with advisors.

Joe Vandigo, MBA, PhD
Vice President, Market Access and Value Strategy
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Linked in

Patient Experience Dossiers: Connecting Decision Makers With Patient-Centered Evidence

Elisabeth M. Oehrlein, Omar Escontrias, Hayley Chapman, Joe Vandigo

SEPTEMBER 9, 2025 10.1377/forefront.20250908.298842

apx
applied patient experience

Megan Freed, MPH
Chief of Strategic Initiatives
Parent Project Muscular Dystrophy (PPMD)

The Duchenne Registry & Patient Experience Data

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.

Megan Freed, MPH

Chief of Strategic Initiatives

BROADLY ENGAGING THE DRUG DEVELOPMENT PIPELINE

For 30 years, PPMD has contributed to each stage of the drug development pipeline, awarding grants, filling in critical gaps, convening stakeholders, and redefining the clinical trial landscape.

Leveraging support from PPMD:

- Expertise
- Community reach & connections
- Data & research capabilities
- Diverse programs & meetings
- Multi-faceted strategy

BROADLY ENGAGING THE DRUG DEVELOPMENT PIPELINE

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DISCOVERY & PRECLINICAL

- Exploratory research awards
- PPMD Venture Pathways – venture philanthropy investments
- Validation and replication study services
- Facilitate KoL/SME outreach
- Support novel endpoint design

- The Duchenne Registry – data access
- Partnership with federal agencies (NIH/DoD)
- Updated Duchenne Care Consideration Guidelines

TRIAL READINESS/ PHASE 1

- Comprehensive clinical trial readiness services
- Protocol/IFC Review
- Patient focus groups
- Identification of Phase 1 study centers
- IND Meeting support

- Duchenne Drug Development Roundtable – engaging sponsors in pre-competitive space
- Duchenne FDA Guidance for industry
- PPMD/C-Path Duchenne Regulatory Science Consortium

PHASE 2/3 & RECRUITMENT

- The Duchenne Registry – targeted patient recruitment
- Duchenne community engagement
- webinars and newsletters
- outreach recommendations
- CDCC Network of Trial Sites
- CDCC Lunch and Learn with clinicians

- PPMD for You – individualized patient trial opportunities
- Drug Development Pipeline and Trial Finder listings for increased community visibility

REGULATORY APPROVAL

- Multichannel community outreach
- Leading patient and caregiver preference studies for publication
- Advisory committee meeting support
- Regulatory engagement with FDA CBER
- Regulatory engagement with FDA CDER

- Pediatric Gene Therapy & Medical Ethics working group
- Think tanks and listening sessions
- Partner meeting preparation and attendance

POST-MARKET & ACCESS

- Pioneering access, coverage, and reimbursement strategy
- Duchenne Outcomes Research Interchange – data warehousing infrastructure for post-market surveillance
- Patient engagement initiatives
- Post-market strategy development
- Payer engagement
- State-based advocacy & DUR meeting support

- Newborn Screening – leading federal and state-based efforts
- Decode Duchenne – free genetic testing
- ChildMuscleWeakness.org
- PPMD For You – personalized benefits investigation and case management support

PPMD

Engagement & Expertise

PPMD PERSPECTIVES

PPMD fosters a comprehensive range of activities that assist all therapy development efforts, from pre-clinical work through post-market data collection. PPMD can facilitate engagement through face-to-face meetings, focus groups, virtual meetings, and direct conversation.

PPMD Team Insights:

- Trial Design
- Protocol Review
- Regulatory strategy
- Outreach & communication guidance
- Access & Payer strategy
- Customizable based on need

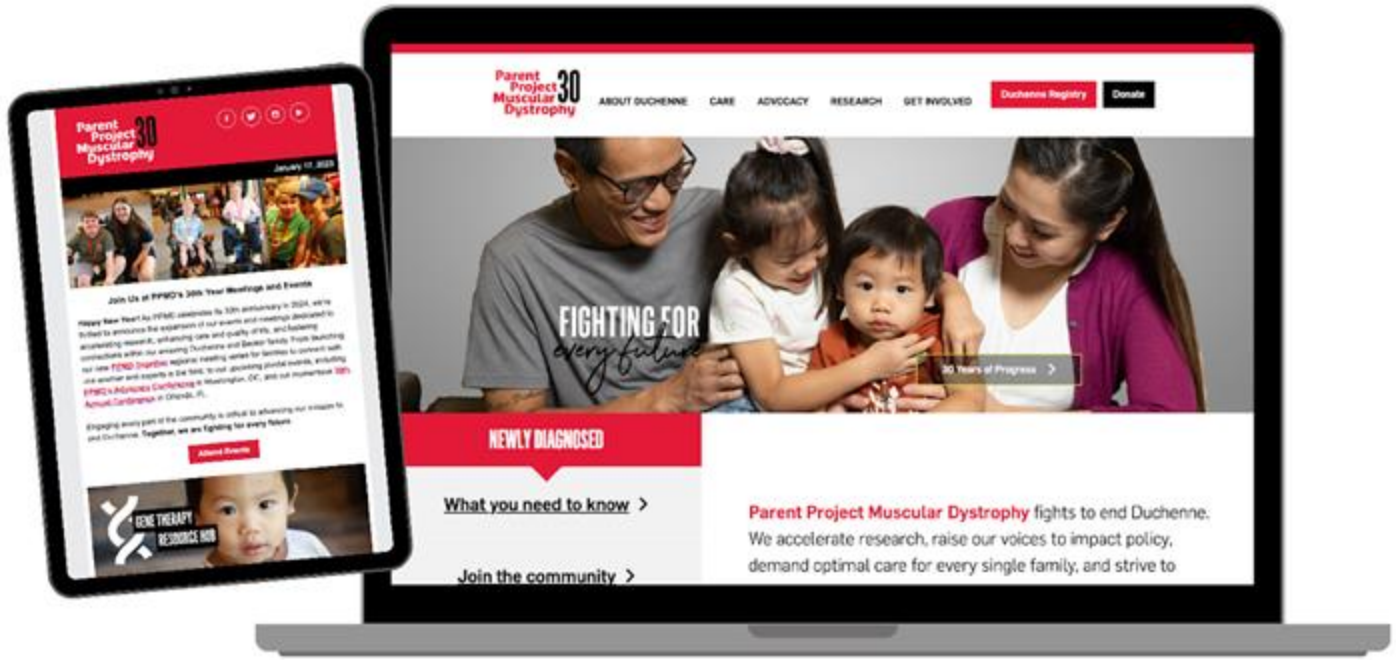
PPMD Network Insights:

- CDCC Network
- Caregivers & Parents
- Patient insights
- PPMD Connect

CONNECT TO THE COMMUNITY

PPMD employs a diverse range of core communication channels that can be leveraged to educate the community about research, therapy development, and trial recruitment.

- Webinar Series
- Blogs
- Newsletter
- PPMD Website
- Social Media



PPMD TOGETHER

PPMD ANNUAL CONFERENCE

PPMD Together is a series of regional meetings designed to be responsive to the needs of the community.

- Focused on connection, discussion, and learning
- Attendees include families, industry partners, care providers, and advocates

PPMD's Annual Conference is the largest global gathering of Duchenne families. Together, we address the most relevant issues, challenges and opportunities, share our collective triumphs and tribulations, our communities hopes and fears—in the most meaningful way—as ONE.

Participant Identification and Recruitment

THE DUCHENNE REGISTRY

The Duchenne Registry is the largest, most comprehensive patient-reported registry for Duchenne and Becker worldwide. The Registry is powered by more than 15 years of robust data shared by patients and families with Duchenne and Becker as well as carrier females.

- 17 years of data collection
- 6000 registrants
- 2500 test reports
- 100+ countries represented
- 100+ studies and trials recruited

Download on the App Store

GET IT ON Google Play

PPMD

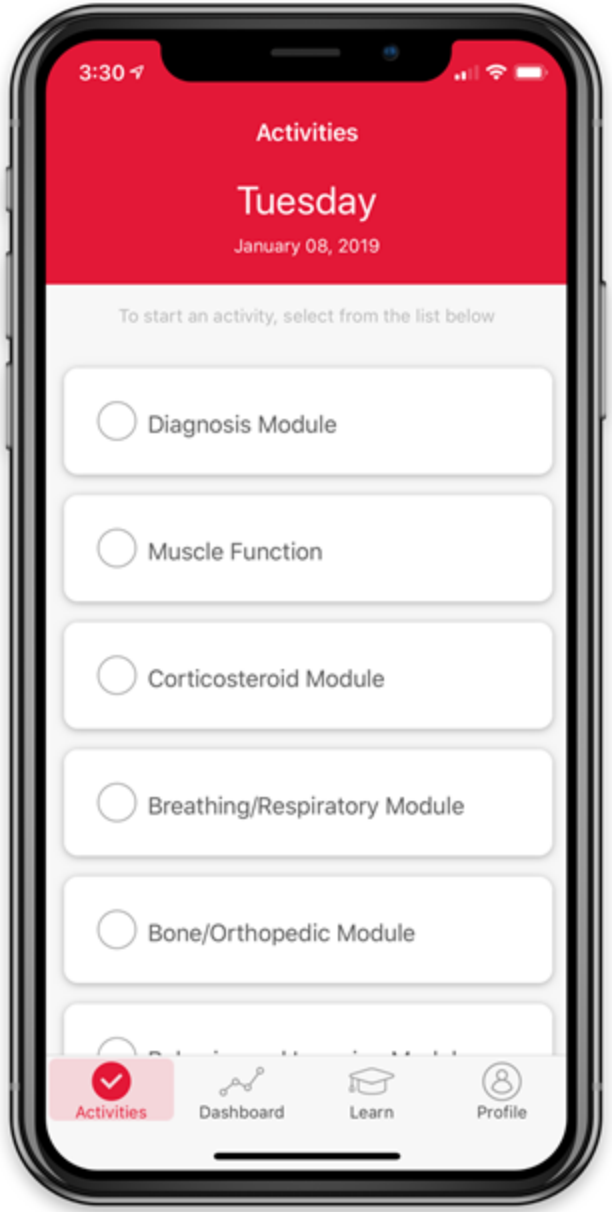
REGISTRY SURVEYS

Patient-Reported Surveys

1. About Me (Diagnosis and Demographics)
2. Muscle Function (PODCI)
3. Corticosteroids
4. Genetic Testing and Family History
5. Gene Therapy
6. Heart
7. Breathing/Respiratory
8. Bone/Orthopedic
9. Behavior and Learning
10. Therapy Services
11. Nutrition and Supplements
12. Comorbidities
13. Pain Inference (PROMIS)
14. Clinical Trials
15. Insurance
16. Female Carrier

Curator-Entered Surveys

17. Genetic Test Curator Form



THE DUCHENNE REGISTRY SERVICES

The Registry provides targeted information about the Duchenne community, available for:

- ✓ Trial Design Feasibility
- ✓ Targeted Research
- ✓ Surveys
- ✓ Mutation-Specific Data Set Purchase
- ✓ Longitudinal Data Set Purchase
- ✓ Targeted Recruitment
- ✓ **Approved Therapies**

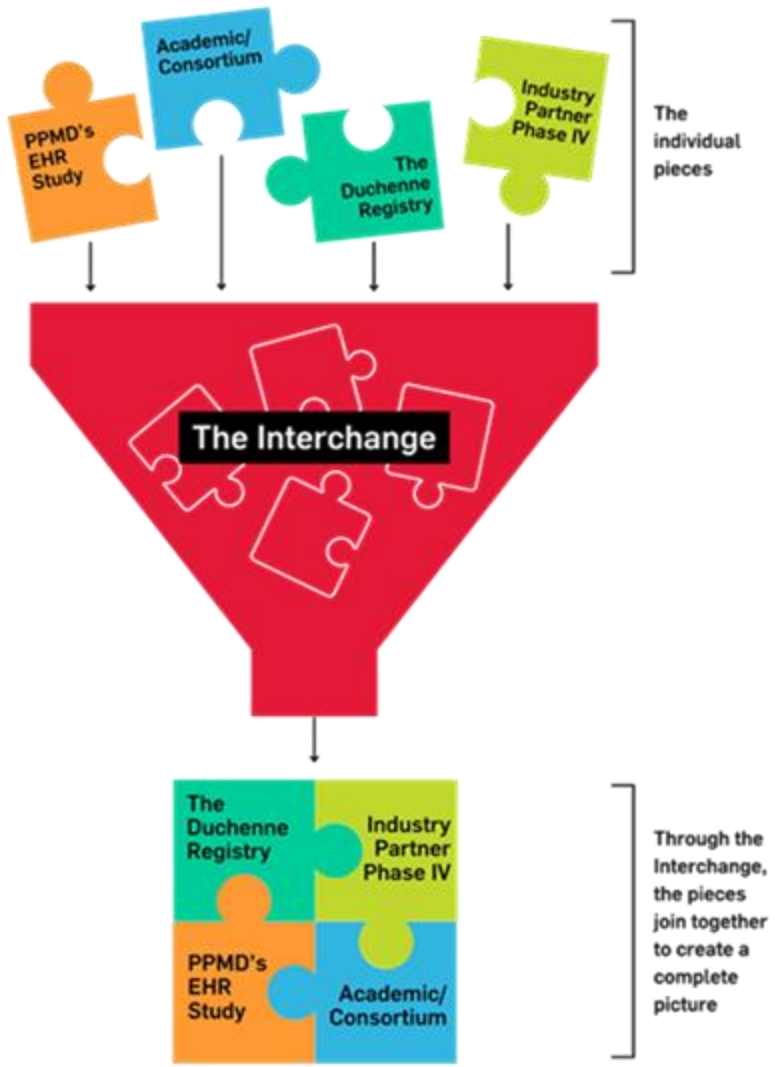
Connecting Data, Enhancing Research

DUCHENNE OUTCOMES RESEARCH INTERCHANGE

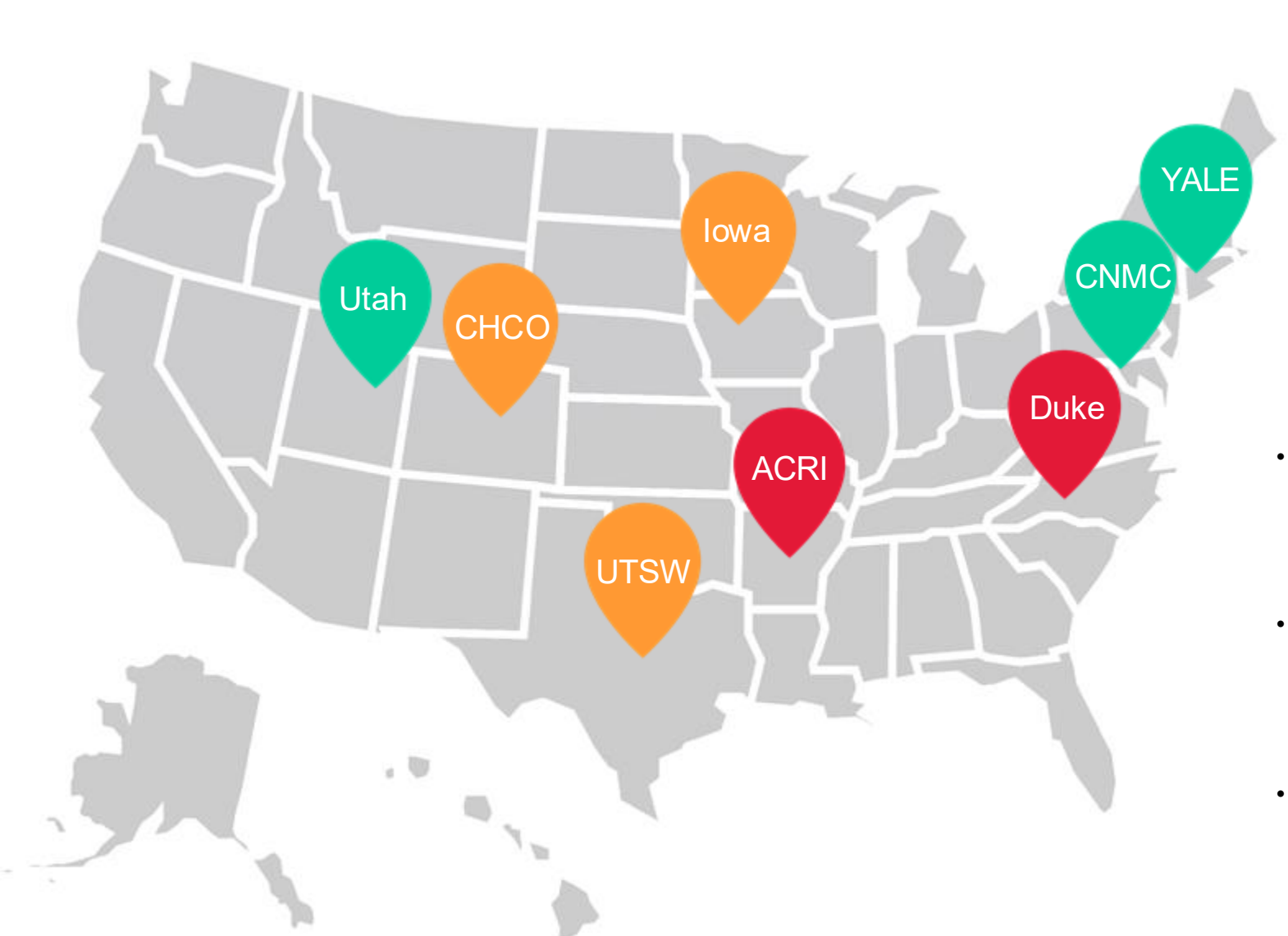
The Duchenne Outcomes Research Interchange is a centralized data hub that combines disparate sources of data to analyze and understand real-world evidence about the Duchenne and Becker progression.

- **Centralized data hub combines:**
  - Patient-reported data
  - Clinician-reported data
    - Phase IV
    - Post-market surveillance
  - Electronic health records
    - Care visits

- **Infrastructure allows for:**
  - Patient matching between studies so The Duchene Registry can be matched to other ongoing studies for comparative analysis
  - Individual ownership and full control of data set with opportunity to combine data through Steering Committee request.



PPMD'S ELECTRONIC HEALTH RECORD STUDY



PPMD's Electronic Health Record (EHR) Study is a research study that shares clinical data from a patient's clinical visits in the EHR with PPMD. Data from consented patients' health records is securely sent to PPMD's Duchenne Outcomes Research Interchange.

- Currently includes 8 CDCC sites
  - *2025-26 preliminary evaluation of the program*
- Sites consent patients, and an IT process using the Cerner/Epic orchard is built to allow data to be pushed into the Interchange
- Pushed data includes:
  - *USCDI v2 data elements*
  - *Clinical notes*

More Programs, More Impact

BROADLY ENGAGING THE DRUG DEVELOPMENT PIPELINE

PPMD CARE CONSENSUS MEETINGS

DUCHENNE DRUG DEVELOPMENT ROUNDTABLE

PPMD ADULT ADVISORY COMMITTEE

PATIENT-PREFERENCE STUDY: STABILITY

NEWBORN SCREENING FOR DUCHENNE

PATIENT-PREFERENCE STUDIES

Collecting patient-preference data since 2012

- Inaugural study: caregivers of Duchenne patients revealed a strong willingness to accept risks for therapies that halt or slow progression
- Since then, conducted more than 10 patient-preference studies with academic partners on:
  - Pulmonary outcomes
  - Steroid experience
  - Gene therapy
- Answering a critical need to redefine meaningful endpoints in DMD with STABILITY Study

IMPROVING ACCESS

PPMD tracks access to therapeutics in the rare disease space with a focus on Duchenne/Becker.

PPMD Team:

- •Engage with CMS and private insurers on broader coverage and policy decisions
- •Refer individual cases (e.g. denials, prior authorization issues) to **Little Hercules Foundation** for 1:1 case management support
- •Collaborate with the Duchenne community to lead on broader systemic issues
- –Sr Director of Advocacy
- –Community Engagement Coordinator

Who to contact:

Federal & state advocacy, all things access & payer focused

Lauren Stanford
Senior Director, Advocacy
Lauren@parentprojectmd.org

Lauren Bogue
Lead Curator, The Duchenne Registry
LBogue@parentprojectmd.org
Coordinator@parentprojectmd.org

Registry, data curation, nerdy genetics questions

Thank you!

Moderated Q & A

Raymond Puerini

Director,

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Thank You!

For additional questions or to learn more about TRAIN, please contact TRAIN@milkeninstitute.org