PPMD’s End Duchenne [Remote] Tour: St. Louis, MO
Who is PPMD?

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SVP, Clinical Care
Director, PPMD’s Certified Duchenne Care Center Program (CDCC)
1994 Questions

- What was the current state of research & care in Duchenne?
- What was the federal government investment in Duchenne?
- What data existed about the Duchenne population?
- What are the gaps we need to fill in order to move the needle toward better care & treatments for Duchenne?
Impact on Research

$50 million+ invested by PPMD into Duchenne research & therapy to date

Over 260 research grants awarded, supporting nearly every major therapeutic approach

~35 clinical trials in Duchenne at any given time

3 U.S. drug approvals with additional promising therapies in development
The Drug Development Pipeline is full of potential treatments that are being tested. These include therapeutic approaches that restore or replace dystrophin and those that treat Duchenne symptoms. The goal? To test combinations of these therapies to create the best “cocktail” for each patient.
For over two decades, Parent Project Muscular Dystrophy (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.

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<th>DISCOVERY &amp; PRECLINICAL</th>
<th>TRIAL READINESS/PHASE 1</th>
<th>PHASE 2/3 &amp; RECRUITMENT</th>
<th>REGULATORY APPROVAL</th>
<th>POST-MARKET &amp; ACCESS</th>
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<tr>
<td>- Exploratory research awards</td>
<td>- Certified Duchenne Care Center program &amp; Clinical Trial Awareness program</td>
<td>- Clinical trial support &amp; drug development research awards</td>
<td>- Pioneering access, coverage, &amp; reimbursement strategy</td>
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<td>- Validation &amp; replication study services</td>
<td>- Duchenne Specialty Care Workshops</td>
<td>- Duchenne community engagement</td>
<td>- Decode Duchenne, free genetic testing</td>
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<td>- Updated Duchenne Care Consideration Guidelines &amp; Family Guide</td>
<td>- PPMD / C-Path Duchenne Regulatory Science Consortium</td>
<td>- Leading creation of forward thinking expert publications, i.e.: Putting Patients First: Patients are Waiting, &amp; numerous patient &amp; caregiver preference study publications</td>
<td>- Patient engagement initiatives</td>
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<td>- Duchenne Newborn Screening Program</td>
<td>- Duchenne Drug Development Roundtable – engaging sponsors in pre-competitive space</td>
<td>- Advisory Committee &amp; IND meeting support</td>
<td>- Post-marketing strategy development</td>
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<td>- The Duchenne Registry</td>
<td>- Partnering with federal agencies (MDCC, FDA, CDC, NIH, DoD, CMS, SSA)</td>
<td>- Leading passage of 5 federal bills, securing Duchenne-specific federal funding, &amp; supporting rare disease legislation</td>
<td>- Payer engagement</td>
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<td>- ChildMuscleWeakness.org – an early diagnosis program</td>
<td>- The Duchenne Registry trial readiness services</td>
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<td>- AAP motor delay tool</td>
<td>- Duchenne FDA Guidance for industry</td>
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<td>- ICD-10 code refinement</td>
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Impact on Advocacy

- 4 bills signed into law, reshaping the Duchenne landscape
- 12,000 messages & action alerts sent to Members of Congress
- Over $600 million in federal funding leveraged by PPMD community into research
- More than 2,000 face-to-face with Members of Congress

#EndDuchenne
How YOU Can Advocate

- Sign up to become an advocate to receive action alerts in your inbox at ParentProjectMD.org/AdvocacyAlerts
- PPMD can assist in organizing in district meetings with your local representatives
- Contribute to our preference studies and represent our community at meetings with federal agencies
- Adults living with Duchenne were the first patient representatives to serve on an FDA Advisory Committee for FDA decisions
- Attend PPMD’s Advocacy Conference in Washington, DC
  - Will take place February 28 – March 2nd with scheduled visits on Capitol Hill are arranged for all attendees to meet with the offices of their congressional members and tell their stories

#EndDuchenne
Impact on Care

- Added to average lifespan due to PPMD-led advances
- Awarded certification by PPMD across the US as of January 2020
- In PPMD network of certified clinics
- Of identifying & addressing gaps in care through specialty workshops & consensus meetings

#EndDuchenne
Duchenne Care Considerations

- In partnership with the CDC
- First published in January 2010, with updated guidelines published in 2018 in *Lancet Neurology*
- Diagnosis and management of Duchenne muscular dystrophy (3 parts):
  - Part 1: Diagnosis, neuromuscular, rehabilitation, endocrine, & gastrointestinal and nutritional management
  - Part 2: Respiratory, cardiac, bone health, & orthopedic management
  - Part 3: Primary care, emergency management, psychosocial care, & transitions of care across the lifespan
- Led to the development of the Certified Duchenne Care Center program (2014)
  - Care Considerations = requirements for certification
  - Certification requirements were adapted for adult certification, with the first adult CDCC certified in 2019
  - Expanded program globally to include 2 international centers
- PPMD also led the effort to an ICD-10 code approval specifically for Duchenne/Becker to
  - Goal: improve longitudinal surveillance of data

#EndDuchenne
PPMD’S CERTIFIED DUCHENNE CARE CENTER PROGRAM

27 CENTERS across the United States
700 CARE PROVIDERS caring for individuals with Duchenne
3,300 PATIENTS served at certified centers
79 CLINICAL TRIALS located at certified centers
+1,150 TRIAL PARTICIPANTS currently enrolled or in active recruitment

*As of January 2020

SEATTLE CHILDREN'S HOSPITAL
UCSF
UC DAVIS
UCLA
STANFORD CHILDREN'S HOSPITAL
PRIMARY CHILDREN'S HOSPITAL
CHILDREN'S HOSPITAL COLORADO
CHILDREN'S MERCY HOSPITAL
CHILDREN'S HEALTH & UT SOUTHWESTERN
AMERICAN FAMILY CHILDREN'S HOSPITAL
UNIVERSITY OF IOWA CHILDREN'S HOSPITAL
CHILDREN'S HOSPITAL WISCONSIN
AKRON CHILDREN'S HOSPITAL
NATIONWIDE CHILDREN'S HOSPITAL
YALE NEW HAVEN CHILDREN'S HOSPITAL
NEMOURS/ALFRED I. DUPONT HOSPITAL FOR CHILDREN
KENNEDY KRIEGER INSTITUTE
CHILDREN'S HOSPITAL OF THE KING'S DAUGHTERS
CHILDREN'S NATIONAL HOSPITAL
CHILDREN'S MERCY HOSPITAL
CINCINNATI CHILDREN'S HOSPITAL
UNIVERSITY OF IOWA
CHILDREN'S HOSPITAL
ARKANSAS CHILDREN'S HOSPITAL
UNIVERSITY OF MISSOURI
LURIE CHILDREN'S HOSPITAL
DUKE UNIVERSITY MEDICAL CENTER
AMERICAN FAMILY CHILDREN'S HOSPITAL
UNIVERSITY OF ROCHESTER
NEMOURS/ALFRED I. DUPONT HOSPITAL FOR CHILDREN
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UNIVERSITY OF MISSOURI
UCK CHILDREN'S HOSPITAL
LURIE CHILDREN'S HOSPITAL
DUKE UNIVERSITY MEDICAL CENTER

*As of July 2020

Plus, two Global Certified Duchenne Care Centers: Red Cross War Memorial Children's Hospital (South Africa) and Motol University Hospital in (Czech Republic)
Engagement in the Community

- 26 PPMD Connect locations across the United States, providing local families outreach & mentoring
- Over 5,000 patients & carriers across the globe have registered in The Duchenne Registry since launch
- $36 million+ raised through Race to End Duchenne & family-led grassroots events since 1994
- 14,310 families reached in person, through 25 Annual Conferences, End Duchenne Tour stops, Roundtable discussions, & Advocacy Conferences

#EndDuchenne
Coach To Cure MD

- Coach to Cure MD, presented by Werner Ladder, is a partnership between the American Football Coaches Association (AFCA) and PPMD.

- This year on **November 14, 2020**, coaches nationwide will wear a Coach to Cure MD patch and mention the program during on and off-field interviews.

- To help raise awareness and funds, visit coachtocure.org

#EndDuchenne
PPMD’s 2020 Annual Conference

• Join over 700 families, healthcare professionals, and industry partners at PPMD’s annual conference to learn the latest progress in the fight to end Duchenne
• SAVE THE DATE: June 23-27, 2021 at the Omni Orlando Resort at Champion’s Gate in Orlando, FL
• ParentProjectMD.org/Conference

#EndDuchenne
PPMD’s Adult Advisory Committee (PAAC)

• The PPMD Adult Advisory Committee (PAAC) is a group of 22 young men living with Duchenne & Becker and 1 female carrier of Duchenne

• The PAAC is on a mission to elevate the lives of individuals living with Duchenne and Becker muscular dystrophy through advocacy, education, mentorship, and awareness. We represent the voice of people living with Duchenne and Becker as an extension of PPMD

• Applications for the 2021 PAAC will open on November 1st, email amanda@parentprojectmd.org if interested

• For more information about the PAAC and how to get involved, visit parentprojectmd.org/PAAC
Other Ways to Get Involved

Find a local PPMD Connect group

Race to End Duchenne

Local DIY Events
Get Involved
Jessica Divin & Kate Vanderweele
Coordinators, PPMD’s Connect Illinois
PPMD’s Connect Program

• PPMD’s Connect is the official family outreach program of Parent Project Muscular Dystrophy.

• Led by volunteer parents and grandparents, PPMD’s Connect groups serve as regional points of contact for families and individuals affected by Duchenne and Becker.

• PPMD’s Connect groups provide:
  – **Family Mentoring**: To connect families with local resources, help them better navigate the diagnosis of Duchenne or Becker, and provide ways to connect with other families both in-person and online.

  – **Outreach opportunities**: To participate in national awareness and advocacy campaigns.

  – **Fundraising opportunities**: From lemonade stands to silent auctions to endurance events, there’s a way for everyone to make a difference and support PPMD’s mission.
How to Find Us

• Facebook:
  www.facebook.com/groups/PPMDCoectllinois/

• Email: ppmdconnectillinois@gmail.com
Let’s Get Together!

We are planning a Zoom get together for families and will send out the details soon!
PPMD’s Sibs Connect
Abbie Killian
Coordinator
PPMD’s Sibs Connect

• Our goal is to actively engage individuals that have siblings with Duchenne and Becker Muscular Dystrophy to build a community that fosters connecting with other siblings, sharing concerns and ideas, and supporting siblings at every step of their journey with Duchenne and Becker.

• The group is currently for siblings 16 and older, with plans to expand to younger siblings later on.

• We are currently growing and engaging our group on our Facebook page, and planning some virtual get-togethers this year. We also have plans for in-person socials and more formal sibling-focused programming at the Annual Conference in June.
How to Find Us

● Facebook: Search PPMD’s Sibs Connect (www.facebook.com/groups/PPMDSibsConnect/)
● Email: siblings@parentprojectmd.org
● Interest Survey for more information: join.parentprojectmd.org/sibsconnectsurvey
Let’s Get Together!

• Plans are in the works for our first Zoom social!
• Please fill out the interest survey or join the Facebook group to receive information on date and time
• Join us and get to know some other awesome sibs!
Race to End Duchenne Virtual Series

• Starts TODAY and runs through November 1st
• 5K, 10K and 15K distances (walk or run your own course, at your own pace)
• Download the RaceJoy app to track your route and be cheered on by our community throughout your race
• Register at www.RunSignUp.com
• Every participant receives a t-shirt and medal in the mail