DuchenneConnect, a registry for individuals with Duchenne and Becker muscular dystrophy, is a program of Parent Project Muscular Dystrophy (PPMD). The overall goal of the Registry is to expand the knowledge base of Duchenne and Becker, to connect patients with actively recruiting clinical trials and research studies, and to educate patients and families about Duchenne and Becker research. At the same time, DuchenneConnect is a valuable resource for researchers, allowing access to data provided by patients and their families—information that is vital to advances in the care and treatment of Duchenne.

We have expanded our services over the past year. While we still focus on speeding up the clinical trial process, we have widened our reach by addressing other important clinical areas.

Highlights from 2015 include:

- Continuation of our PCORI funding for Phase II of PCORnet, the National Patient-Centered Clinical Research Network
- Expansion of our Decode Duchenne genetic testing program
- Collaboration with PPMD’s Certified Duchenne Care Centers and FACES groups
- Enhanced outreach through social media
- Launch of whiteboard videos and better online presence

DuchenneConnect Priorities for 2016:

1. Continuation of our PCORI funding for Phase II of PCORnet
2. Expansion of our Decode Duchenne genetic testing program
3. Collaboration with PPMD’s Certified Duchenne Care Centers and FACES groups
4. Enhanced outreach through social media
5. Launch of whiteboard videos and better online presence
Collaboration with CDCCs

In order to assist families with registration and updating of medical history information, and to increase the registration of underserved patients and families, DuchenneConnect provides an iPad to every Certified Duchenne Care Center (CDCC) in the US. There are currently 11 CDCCs with several more planned for 2016. Site coordinators are trained at each clinic to assist patients and families with registering in DuchenneConnect and updating their accounts while in clinic.

Decode Duchenne

In 2015, the Decode Duchenne program was expanded with support from Biomarin Pharmaceutical, PTC Therapeutics, and Sarepta Therapeutics. Decode Duchenne provides free genetic testing through Emory Genetics to patients who could not otherwise afford this important testing. In 2015, 110 participants from 20 different states were approved for free genetic testing. The program also provides free genetic counseling and test result interpretation for Duchenne and Becker.

DuchenneConnect and PCORnet

DuchenneConnect completed Phase I of the PCORnet award and we were delighted to be awarded a Phase II award to continue our participation in PCORnet. As part of this project, we continue to work on ways to improve the Registry experience for everyone. We are excited to have a new member on our team, Alan Chaulet, who is serving as our Patient Engagement Liaison. Alan is an adult with Duchenne who also serves on the PPMD Adult Advisory Committee (PAAC). We continue to gain valuable insight from our Patient and Family Advisory Committee, a group of parents and adults with Duchenne/Becker who volunteer their time to guide the PCORnet project. To learn more about this remarkable group of people, go to www.duchenneconnect.org/pcornet.

Some of our accomplishments with the PCORnet project in 2015 include:

• Launching a revised consent and more user-friendly Medical History Surveys
• Providing incentives for registering and completing surveys (iPads and Amazon.com gift cards)
• Translating the consent and surveys into Spanish (to go live in 2016)
• Creating Facebook and Twitter accounts
• Developing two whiteboard videos highlighting DuchenneConnect with more in production for 2016
• Conducting two online focus groups
• Working with a Patient Reported Outcomes (PRO) expert to incorporate PROs in the Registry
• Collaborating with several partners within PCORnet on potential research projects, including our partnership with the OneFlorida CDRN who chose Duchenne as their rare disease focus

“I just wanted to express my gratitude and thanks for this awesome program that DuchenneConnect has created for families like ours. We highly appreciate the services that are being offered and look forward to working with you!”

-Kiska

( Parent of a child registered in DuchenneConnect)
Recruitment for Clinical Trials
In 2015, DuchenneConnect was asked to recruit for nine clinical trials. Methods of recruitment include targeted emails to registrants who appear to match inclusion criteria, newsletter articles, website postings on homepage, webinars, social media (Facebook and Twitter), and FAQ Sheets (family-friendly summaries for website and Annual Connect Conference program book). The following trials were recruited for in 2015:

• Aldosterone Inhibition Trial (OSU)
• Becker Natural History Study (CINRG)
• BMS-986089 Trial (Bristol-Myers Squibb)
• Eteplirsen Confirmatory Trial (Sarepta)
• Genetic Modifier Study (UCLA)
• Move DMD Trial (Catabasis)
• Pfizer PF-06252616 Trial (Pfizer)
• Robotic Arm Study (NJIT)
• Strength Training Protocol Study (U of FL)

Recruitment for Research Studies
DuchenneConnect assisted with recruitment for eight research studies in 2015:

• PPMD’s Second Benefit/Risk Study (Santhera)
• Deflazacort Survey (Marathon)
• Epilepsy and Duchenne Study (Netherlands)
• GI/GU Survey (Nationwide Children’s Hospital)
• Ipsos Interview Study (for pharmaceutical company)
• Parental Beliefs about Research Survey (Johns Hopkins School of Public Health)
• Sanguine Biosciences Blood Donation (for myostatin inhibition study)
• Sanguine Biosciences Urine Donation (for biomarker study)

“I joined the registry so that I could strengthen my understanding of existing research and do our part to support clinicians and researchers…. I like receiving the alerts about recruitment for new studies. That is how we learned of the study in which our son is now participating.”

-Amy

(Parent of a child registered in DuchenneConnect)

Educational Resources
Educational information is available on the DuchenneConnect website, social media sites, and through email communication. As part of our educational outreach we:

• Post frequent updates on Facebook and Twitter, with the addition of Instagram in 2016
• Provide up-to-date news on the DuchenneConnect website
• Publish a bimonthly newsletter to all patient and professional registrants
• Create Clinical Trial FAQ Sheets for PPMD’s Annual Connect Conference
• Contribute to PPMD’s monthly Direct Access Webinar Series
• Respond to numerous phone calls, emails, and “Ask an Expert” contact requests

Data Sharing
DuchenneConnect provides de-identified data* to researchers upon request. A Data Access Application must be completed and approved by the DuchenneConnect team, and the Data Sharing Agreement must be signed by both parties. In 2015, we provided data to nine institutions: five pharmaceutical industries, two academic researchers, and two research organizations. The data provided included raw data for research as well as feasibility data for optimal planning of clinical trials.

* de-identified data is information from the registry that does not contain any information such as date of birth, name, etc that could be used to identify someone.
Meet the DuchenneConnect Team

Who is doing the work behind the scenes at DuchenneConnect? We have a dedicated group of PPMD staff and family advisors who work diligently to make DuchenneConnect a great registry and resource for our community.

This photo was taken at the DuchenneConnect/PCORnet Family Advisory Committee meeting during PPMD’s Connect Conference in June 2015.

Front row: Matt Walsh (Community Engagement Coordinator), Margie Heger (Family Advisor), Jason Abramowitz (Family Advisor), Ben Dupree (Family Advisor)

Back row: Ann Martin (Co-Director), Brian Denger (Family Advisor), Ann Lucas (Co-Director & Co-PI PCORnet), Adele Abramowitz (Family Advisor), Holly Peay (Co-PI PCORnet), Tom Cosgrove (Family Advisor)

Our DuchenneConnect Team is growing! New team members added in 2016 include:

- Lauren Bogue, Genetic Counselor and Decode Duchenne Program Manager
- Jenifer Lavigne, DuchenneConnect/PCORnet Program Manager

Contact the DuchenneConnect Team at coordinator@duchenneconnect.org or call 888-520-8675. We are here to help you!

DuchenneConnect Priorities for 2016

- Increase the number of research collaborations with PCORnet partners
- Implement integration of electronic health records into DuchenneConnect
- Improve visualization of data for patients and families
- Continue developing videos for clinical trial education
- Expand the Decode Duchenne genetic testing program
- Focus on the needs of female carriers through our Carrier Initiative
- Grow our recently launched online “Live Chat” to increase access to our genetic counselors
- Continue to increase registration in DuchenneConnect, especially for underserved patients, through a variety of outreach strategies

“I am 20 years old and joined the registry to keep track of my medical information while contributing to research.”

-Alex

(Self-registered DuchenneConnect participant)

Outreach to the Professional Community

- In 2015, the DuchenneConnect team presented about DuchenneConnect, Decode Duchenne, and PCORnet in over 15 professional forums to ensure that clinicians, researchers, sponsors, and the broader community are aware of the capabilities of the Registry.
- Data from DuchenneConnect was used in a publication by TREAT-NMD in Human Mutation (Jan 2015): “The TREAT-NMD DMD Global database: Analysis of More Than 7000 Duchenne Muscular Dystrophy Mutations.”
- DuchenneConnect’s Professional Portal had 37 new professional registrations in 2015.