



“I joined DuchenneConnect because the site gave me a lot of easy to understand information about Duchenne. When my son was first diagnosed I was so lost. I found DuchenneConnect and I was able to learn so much about what I needed to do for him as well as what to expect.”

– Kristen (mother of a child registered in DuchenneConnect)

What is DuchenneConnect?

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 muscular dystrophy, 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.

Recruitment for Clinical Trials and Research Studies

In 2016, DuchenneConnect was asked to recruit for 10 clinical trials and 13 research studies. Methods of recruitment include targeted emails to registrants who appear to match inclusion criteria, newsletter articles, social media posts, webinars, and website postings under Clinical Trial News.

Data Sharing

In 2016, we had 8 separate data inquiries and we provided data to pharmaceutical industries, academic researchers, and research organizations. The data provided included raw data for research, as well as feasibility data for optimal planning of clinical trials.

Educational Resources

As part of our educational outreach we:

- Post frequent updates on Facebook, Twitter, and Instagram
- Publish a bimonthly newsletter to all patients and professional registrants
- Create Clinical Trial FAQ Sheets for PPMD's Annual Connect Conference
- Respond to numerous phone calls, emails, and “Ask an Expert” contact requests

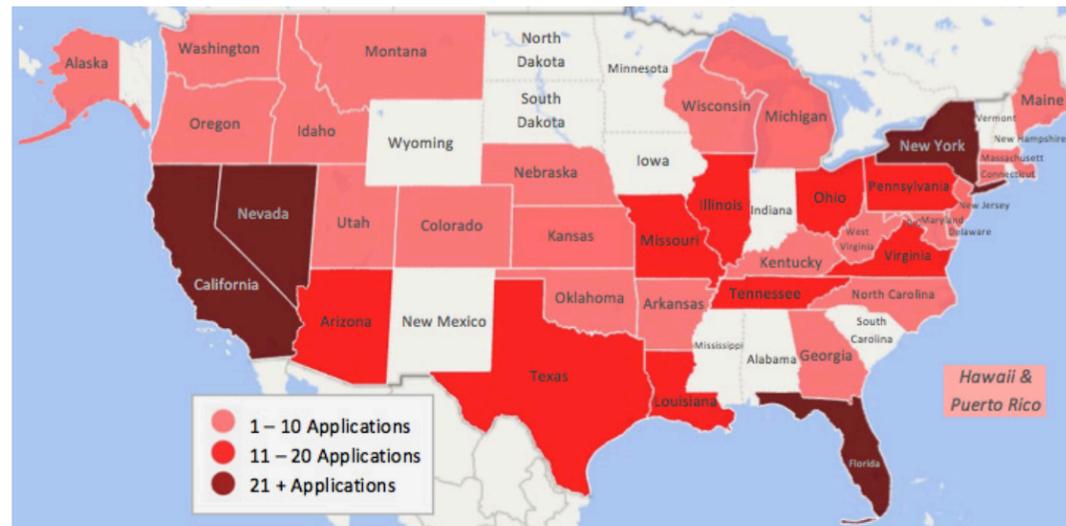
Collaboration with Certified Duchenne Care Centers

Thanks to an industry-awarded grant in 2016, PPMD created a process for all Certified Duchenne Care Centers (CDCCs) to apply for grants to support a PPMD Liaison Position to 1) help ensure that families are informed of all relevant research projects and clinical trials, 2) understand the importance of DuchenneConnect and assist with registration, and 3) encourage completion of the Clinical Experiences Survey, which provides us with the family perspective of medical care and services. In addition, DuchenneConnect provides an iPad to every CDCC, to assist patients and families with registering in DuchenneConnect and updating their accounts while in clinic.

Decode Duchenne

Decode Duchenne had a very successful year in 2016, and is receiving ongoing support from Sarepta Therapeutics and PTC Therapeutics. Decode Duchenne provides free genetic testing through EGL Genetics (formerly Emory Genetics) to patients who could not otherwise afford this important testing. In 2016, 285 participants were approved for free genetic testing. The total number of participants from the program launch in December 2013 through December 2016 was 474, with referrals from 38 different states or territories of the United States and Canada. The program also provides free genetic counseling and test result interpretation.

Decode Duchenne Application by State



DuchenneConnect and PCORnet

DuchenneConnect is currently in year two of a three year award for Phase Two of PCORnet. What does this mean? The funding from this award allows us to continue growing the registry and continuing our outreach. Some of our projects in 2016 have included:

- Continuing to provide incentives for registering and completing surveys (12 iPads and 24 Amazon.com gift cards were given away in 2016!)
- Developing a clinical trial decision aid tool along with video and written educational content (to go live Summer 2017)
- Continuing partnership with other members of PCORnet on research projects, including OneFlorida, who will have preliminary data available Summer 2017
- Development of data dashboard to launch Summer 2017
- Ongoing engagement with the PCORnet Family Advisors who have provided invaluable contributions to our PCORnet projects and DuchenneConnect

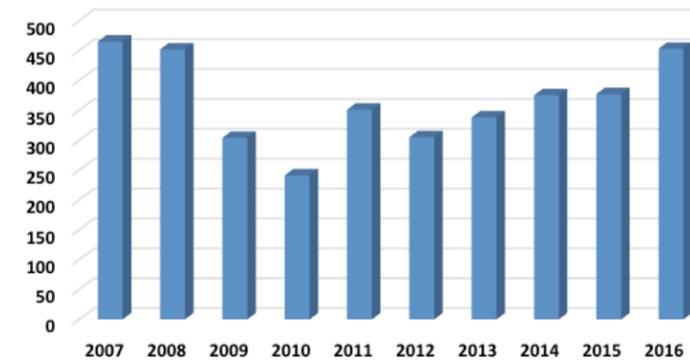
"We're finally getting our son's genetic testing done through Decode Duchenne. Thank you for providing such an awesome service for so many families struggling with this diagnosis. I am forever grateful."

– Mother of Decode Duchenne participant

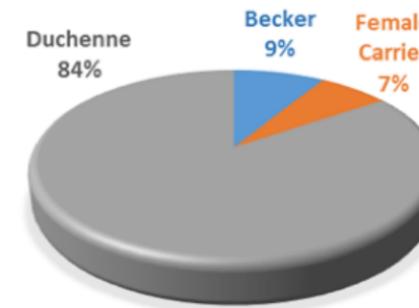
Registration Data

In 2016, we had 455 new DuchenneConnect registrants. DuchenneConnect is an international registry, and we have registrants from 108 countries! Non-US registrants make up approximately 30% of DuchenneConnect participants.

Total New Registrants per Year

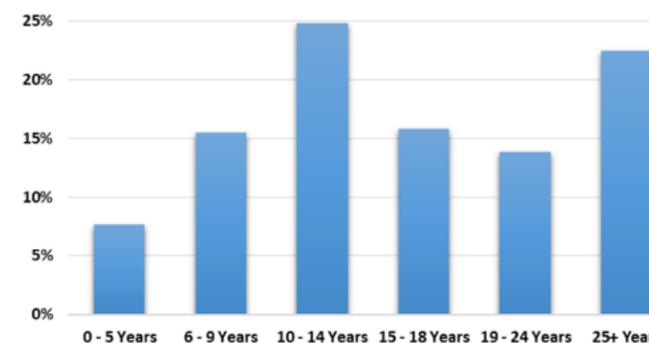


Registrants by Diagnosis



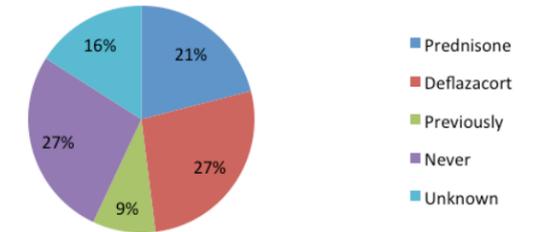
* Registrants who were unsure of their diagnosis were excluded.

Registrants by Age

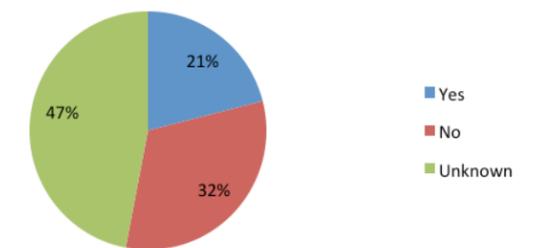


Data Snapshot of Duchenne/Becker males registered in DuchenneConnect and living in the United States or Canada (n = 2,376):

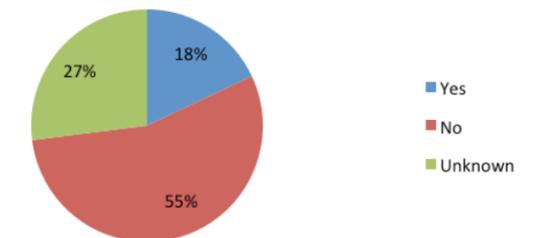
Corticosteroid Use



Use of Cardiac Medication



Use of Breathing Device



"I joined DuchenneConnect to keep up-to-date with trials — I love getting the emails when my son matches criteria for a trial. And we joined to provide longitudinal data so research can progress faster."

– Jessica (mother of a child registered in DuchenneConnect)

Outreach to the Professional Community

In 2016 data from DuchenneConnect allowed us to present 6 posters at 3 professional meetings. Our team was honored to have an educational session on Duchenne at the Annual National Society of Genetic Counselors meeting in Seattle, WA. More broadly, members from our team presented at 9 additional professional forums which helped to increase awareness of DuchenneConnect in the professional community.

DuchenneConnect Priorities for 2017

- Continue to find ways return meaningful data back to you, our Registrants!
- Continue to grow the Registry in the number of new Registrants as well as increasing the number of underserved people who are a part of our community
- Explore new ways to have people contribute data to the Registry
- Continue to strengthen our research collaborations

Meet the DuchenneConnect Team

The DuchenneConnect team is ready to help you with any questions you may have. We have three board-certified genetic counselors: Ann Martin, Ann Lucas, and our newest counselor Jen Ely who leads the Decode Duchenne program. In addition, we welcomed Elizabeth Habeeb-Louks as a program manager recently.

Contact the DuchenneConnect team at coordinator@duchenneconnect.org or call 888-520-8675.

Chat live with a genetic counselor from DuchenneConnect. Login to your DuchenneConnect account to learn more!

 facebook.com/duchenneconnect

 instagram.com/duchenneconnect

 twitter.com/duchenneconnect

Parent Project Muscular Dystrophy

LEADING THE FIGHT TO END DUCHENNE

“DuchenneConnect is an amazing tool. I think its greatest asset is the wealth of information that is collected from us, the parents! The information gathered helps not only researchers, but it’s beneficial as a caregiver too.”

– Lauren (mother of a child registered in DuchenneConnect)

