Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

Duchenne Intensive

About the Duchenne Intensive:
PPMD recognizes and appreciates the growth of our partner companies and has identified the need to provide comprehensive education about the current landscape of Duchenne for those joining the field. To that end, we have developed the Duchenne Intensive, a focused meeting designed for field staff, MSLs, and other employees of industry or biotechnology companies actively engaged in therapy development for Duchenne and Becker muscular dystrophy. This full-day Intensive will precede the Annual Summit and provide an overview of the basics of Duchenne, review current standards of care, ethics in medical care, active clinical trials and ongoing research as well as current learnings around access and reimbursement.

Time	Presentation	Speakers
8:00 am – 8:30 am	Breakfast, Registration	
8:30 am – 9:00 am	Welcome and Introductions	Pat Furlong & Kathi Kinnett *Parent Project Muscular Dystrophy*
9:00 am – 10:15 am	**Session I: Genetics and Variability**	
9:00 am – 9:20 am	**Basic Introductions to Genetics of Duchenne** Duchenne is caused by changes in a single gene in our body. Duchenne can be passed from parent to child, or it can be the result of random spontaneous genetic mutations, which may occur during any pregnancy. In fact, about one out of every three cases occurs in families with no previous history of Duchenne. This presentation will dive into the genetic of Duchenne and Becker and differences with gene mutations.	Jen Ely *Parent Project Muscular Dystrophy*
9:20 am – 9:40 am	**Incidence, Prevalence of Duchenne learnings from MD-STARnet** MD STARnet is the Muscular Dystrophy Surveillance, Tracking and Research Network. MD STARnet is the only research program designed to collect health information on everyone with muscular dystrophies (MD) living in specific areas of the United States. Findings from MD STARnet are reliable and can paint a more complete picture of MD for patients, their families, providers, researchers, drug developers, and policymakers. This presentation will discuss learnings from this multi-year, multi-site program surveillance program.	Emma Ciafaloni, MD *University of Rochester*

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9:40 am – 10:00 am	**Genotype/phenotype - what influences progression** The dystrophin gene is one of the largest in the human body. With 79 exons, the potential mutations that can occur, resulting in mild to severe dystrophinopathy, are too numerous to count. Different mutations can result in distinct variabilities in presentation and symptoms. There are also genetic modifiers playing a role in Duchenne. This talk will describe how Duchenne occurs and the significance of mutations regarding phenotype, natural history and therapeutic approach, as well as the role of genetic modifiers.	Stanley Nelson, MD *UCLA*
10:00 – 10:15	**Q&A Panel**	Pat Furlong – moderator *Parent Project Muscular Dystrophy*
10:15 – 10:30	**BREAK**	
10:30 am – 12:00 pm	**Session II: Duchenne Care and Management**	
10:30 am – 10:50 am	**Duchenne Care Considerations** As science and medicine are advancing, people with Duchenne are living longer; therefore, their care throughout life is evolving as well. These care considerations, funded by the Centers for Disease Control and Prevention (CDC) and supported by PPMD, are intended to raise the standards of care, help clinicians provide the best possible care to people with Duchenne, and give families and caregivers the necessary information to manage their care.	Rachel Schrader, CPNP-PC *Parent Project Muscular Dystrophy*
10:50 am – 11:05 am	**Certified Duchenne Care Centers Overview** PPMD's Certified Duchenne Care Center Program helps to ensure that centers comply with the standards of care and services that have been established in the Duchenne Care Guidelines. All Certified Duchenne Care Centers have met the requirements for, and agree to provide, optimal standardized care and services.	Kathi Kinnett MSN, APRN *Parent Project Muscular Dystrophy*
11:05 am – 11:20 am	**Challenges in Care for Underserved Populations** The term 'vulnerable' is often used interchangeably with 'underserved'. While underserved consumers may have limited access to health care services, vulnerable population consumers tend to experience additional barriers to getting care. In 2008, PPMD spent time in Jackson, MS to better understand the factors related to barriers to diagnosis and to appropriate healthcare, this effort guided a series of key initiatives around diagnosis and care.	Pat Furlong *Parent Project Muscular Dystrophy*

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11:20 am – 11:40 am	**Establishing and maintaining ethical relationships with the Neuromuscular community** Ethical relationships in the field of healthcare are critical. There are many ethical issues that may arise in regards to patient healthcare. These major issues, as well as ways to manage them, will be discussed. Issues include: Confidentiality, privacy, aggressive marketing, patient welfare, access to trials and therapies.	Allison Bateman-House *NYU Langone Health*
11:40 am – 12:00 pm	**Q&A Panel**	
12:00 pm – 12:30 pm	**Lunch**	
12:30 pm – 2:30 pm	**Session III: Research, Clinical Trials, and Access**	
12:30 pm – 12:50 pm	**Current and Active Clinical Trials and Research Strategies**	Abby Bronson *Parent Project Muscular Dystrophy*
12:50 pm – 1:10 pm	**Access and Reimbursement** In September 2016 Duchenne entered into a new era of approved therapies. To date there are two FDA approved therapies with more hopefully on the horizon. This presentation will cover lessons learned to date from our first two approved therapies in Duchenne and how PPMD supports both healthcare professionals and the patient community on supporting access to these therapies.	Ryan Fischer *Parent Project Muscular Dystrophy*
1:10 pm – 1:30 pm	**Expanded Access and Compassionate use** Expanded access, also referred to as “compassionate use,” provides a pathway for patients to gain access to investigational drugs, biologics, and medical devices used to diagnose, monitor, or treat patients with serious diseases or conditions for which there are no comparable or satisfactory therapy options available outside of clinical trials. Recently, “Right to Try” has been added as a potential pathway for patients. Here we will discuss all programs related to obtaining access to medicines outside of the traditional trial and approval pathway.	Richard Klein, MD

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1:30 – 1:50	**Natural History of Duchenne and Learnings from Critical Path** The Duchenne Regulatory Science Consortium (D-RSC) was formed to develop tools to accelerate therapy development for Duchenne Muscular Dystrophy. Many clinical trials are currently underway for potential therapies for the disease, but further work is needed to optimize trial protocols to ensure that such trials are as effective and informative as possible. D-RSC aims to develop new tools to accelerate and improve trial protocol development and to reduce the numbers of patients needed to demonstrate the effect of new therapies. The ultimate goal is to help clinical trial sponsors make informed decisions on groups of patients most appropriate to take part in specific clinical trials, endpoints to select, and how to analyze data from those trials. C-Path is seeking regulatory endorsement for tools developed by the consortium from both the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA).	Jane Larkindale, D.Phil *Critical Path Institute*
1:50 – 2:10	**FDA Regulatory Process and Labels – What that means to you** The number of FDA approvals of rare disease therapies has grown in recent history. Companies pursuing these therapies have incentives and pathways aimed at speeding development and access for patients. Here we will discuss the FDA regulatory process for rare conditions and how labels impact access to therapies.	Tim Franson, MD *Faegre Baker Daniels Consulting*
2:10 – 2:30	**Q&A Panel**	
2:30 – 2:45	**BREAK**	
	Session IV: PPMD	
2:45 – 3:00	**PPMD Resources & Community Education**	Amanda Wilkison, RN *Parent Project Muscular Dystrophy*
3:00 – 3:20	**How PPMD works with Industry**	Kathi Kinnett MSN, APRN *Parent Project Muscular Dystrophy*
3:20 – 4:00	**Round Table Discussion - All Attendees**	Pat Furlong & Kathi Kinnett *Parent Project Muscular Dystrophy*
4:00	Conclude	