Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.

PPMD Duchenne Healthcare Professionals' Summit 2019
Addressing Challenges and Seeking Solutions in Duchenne

January 23-25, 2019
Sanibel Harbour Marriott Resort and Spa, Sanibel, FL
17260 Harbour Pointe, Fort Myers, FL 33908

The goal of this meeting is to create an opportunity for industry, clinicians and regulatory to convene and discuss challenges, barriers and solutions to providing care, developing novel therapies and meeting the growing needs of the Duchenne community. In addition, this effort will expand our existing network, between and beyond the Certified Duchenne Care Centers, allowing our community to work more closely together to meet challenges we face.

The audience of this meeting will include healthcare providers from each of the 20+ Certified Duchenne Care Centers and providers from Academic Centers who provide care for patients and their families and Industry partners

Wednesday January 23

8:00 – 8:30	**Registration, Breakfast**
8:30 – 9:00	**Introduction**
9:00 – 12:00	**Session I:** **What Are We Learning from Natural History?** **(Moderator: Pat Furlong)**
9:00 – 9:30	CPATH Disease Progression Model: What have we learned?
9:30 – 10:00	Variations in Phenotype and Genotype
10:00 – 10:30	**Break**
	(Session I Cont'd)
10:30 – 11:00	What have we learned from Imaging DMD?
11:00 – 11:30	What have we learned from CINRG?
11:30 – 12:00	Findings from MD STARnet
12:00 – 12:30	Panel Q&A
12:00 – 1:00	**Lunch (provided)**
1:00 - 4:30	**Session II** **What Are Health Care Professionals Seeing in Clinic: Complicated Cases** **(Moderator: Kathi Kinnett, PPMD)**
1:00 – 1:45	Case I
1:45 – 2:30	Case II
2:30 –3:00	**Break**
3:00- 3:45	Case III
3:45 – 4:30	Case IV
5:00 – 6:00	**COCKTAIL RECEPTION**
6:00 – 8:00	**Dinner (Provided)**

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Working dinner: The Impact of Social Media on Care, Clinical Trials, and Medical Decision Making

Thursday January 24

7:30 – 8:00	**Breakfast**
	Session 3
8:00 – 10:00	**What Are We Learning about Conducting Clinical Trials in Duchenne? (Moderator: Annie Kennedy, PPMD)**
8:00 – 9:00	Challenges faced by industry in clinical trials
9:00 – 10:00	Challenges faced by clinical teams in clinical trials
10:00 – 10:30	Panel discussion and Q&A: Potential solutions for mitigating those challenges and barriers
10:00 – 10:30	**Break**
	Session 4
11:00 – 1:00	**What Have We Learned from the First Duchenne Approvals? – How has this impacted data collection and aggregation? (Moderator: Ryan Fischer, PPMD)**
11:00 – 11:30	Case studies of our Duchenne Approvals
11:30 – 12:00	Novel Approaches to Data Collection
12:00 – 1:00	What is "Real World Data" and how do we use it?
1:00 – 4:00	**Lunch and Break (box lunch will be available to carry out or eat in)**
4:00 – 7:00	**Session 5 Platform Trials (Moderator: Abby Bronson, PPMD)**
4:00 – 4:30	Relevance of Platform Trials
4:30 – 5:00	Improving the Clinical Trial Structure: A Platform Study for Duchenne - Design/Protocol Considerations
5:00 – 5:30	A Platform Trial for Duchenne: Operations and Infrastructure
5:30 – 6:00	A Clinicians Perspective
6:00 – 7:00	Panel Discussion

(Session 5: Parallel session: Duchenne 101 presented by PPMD team)

7:00 – 8:30 **Dinner (on your own)**

Friday January 25

7:30 – 8:00	**Breakfast (Provided)**
	Session 6 Regulatory and Access Issues (Moderator TBD)
8:00 – 8:45	Regulatory Challenges in CDER
8:45 – 9:30	Regulatory Challenges in CBER
9:30 – 10:30	Panel Q&A
10:30 – 11:30	Access and Reimbursement – working through challenges
11:30 - 12:00	Closing remarks-END