

PPMD 2019 CONFERENCE AGENDA

Parent Project Muscular Dystrophy JOIN THE FIGHT. END DUCHENNE.

KEY:	 RESTORING OR REPLACING DYSTROPHIN	 COMBATING FIBROSIS	 REDUCING INFLAMMATION
	 IMPROVING MUSCLE GROWTH AND PROTECTION	 RESTORING THE CELLS ENERGY	 IMPROVING HEART FUNCTION

See pages 10-11 of your Conference Booklet for corresponding clinical trial and research study FAQ sheets.

Wednesday, June 26th

11:00a Registration Opens

7:00-10:00p Newly Diagnosed Meet & Greet
Room: Crystal A/B
 For our newly diagnosed families only, please

7:00-10:00p Teen and Adult Social
Room: Longboat
 Coordinated by PPMD's Adult Advisory Committee (PAAC)

Thursday, June 27th

7:30-8:15a Yoga
Room: Merritt

8:45-10:45 Coffee

9:15-12:00p Understanding Duchenne from the Start
 Moderator: Pat Furlong
Room: Crystal C/D/E

9:15a **Introduction: So It Begins**
 Pat Furlong

9:40 **Making a Care Plan & Lining up Your Team**
 Christina Trout, RN, MSN

10:00 **Why Physical Therapy Is so Necessary**
 Claudia Senesac, PT, PhD, PCS

10:20 **Diet & Duchenne: Simple Nutrition Advice**
 Kent Williams, MD

10:40 **Q&A and Break**

11:00 **Talking to School, Family, & Children about Duchenne**
 James Poysky, PhD

11:20 **Early Identification of Boys with Developmental & Socio-Emotional Delays**
 Amy Wagner, PhD, MAEd

11:40 **Navigating Challenges at School & Home**
 Denise Gruender, BS

9:15-12:00p Teens & Adults Workshop Sessions
 Moderator: Annie Kennedy
Room: Crystal A/B

Carving Your Personal Path: Identifying Options Beyond High School – and Resources to Help you Get There
 Facilitators: Annie Kennedy; Jill Castle, MEd; PAAC Members
 This session is open to everyone age 14 and above.

– **College Workshop**
 Austin LeClaire, Buddy Cassidy, Colin Rensch, Alex Toalson
Love learning? Ready to tackle college? Come hear about community college and four-year university options, disability resources, scholarships & creative supports for assistance once you've found your perfect campus setting.

– **Job Workshop**
 Ben Dupree, Michael Counterman, Jonathan Piacentino, Colin Werth
Ready to just get to work (with or without a college degree)? By utilizing our interest survey info & strength-based assessments, come up with creative job ideas that set your soul on fire.

– **Entrepreneur Workshop**
 Jill Castle & Sean Crosby
Academics not your passion? Need more flexibility than full time employment offers? Use your interests & strengths to start your own endeavor!

12:00-1:00p Lunch

Thursday continues >

Annual Conference begins:

Room: Crystal C/D/E

Thursday Kids Track Hours

Kids Track
3:15-5:15p
Amazing Animals: Hands on Experience
Room: Tarpon

1:00p	Where We Have Come From/ Where We Are Going Moderator: Pat Furlong
1:40	The Complexities of Duchenne Pat Furlong
1:40	Duchenne: A Multisystem Disease Stan Nelson, MD, PhD
2:00	Variability: Modifiers to the Gene Kevin Flanigan, MD
2:20	Measuring Outcomes in the Face of Variability Cliff Bechtold
2:40	Challenges in Designing Trials Craig McDonald, MD
3:00	Q&A
3:15	Break
3:30	Addressing the Complexities of Duchenne – What You Should Know Moderator: Anessa Fehsenfeld
3:30	Attacking Duchenne from Every Angle Anessa Fehsenfeld, PPMD Chairman
3:40	Duchenne Research and Clinical Trials Abby Bronson – Current therapeutic approaches and clinical trials – Strategies to accelerate the process
4:00	Working to Facilitate a Favorable Access Environment Annie Kennedy – Influencing policy and infrastructure that drives Duchenne research, treatments, and access – Producing evidence to reflect the Duchenne community's experience to inform decision-making by all stakeholders within the access ecosystem

4:15 **Improvements in Care: Extending Quality of Life with a Focus on the Future**
Kathi Kinnett, MSN, APRN

- The Certified Duchenne Care Center network accomplishments
- Integrating clinics, industry, and The Duchenne Registry to build a solid network

4:30 **The Patient Voice: Your Data Informing the Drug Development Ecosystem**
Ryan Fischer

- Your preferences and participation drives innovation and change
- The Duchenne Registry and The Duchenne Outcomes Research Interchange

4:55 **Polling Questions**

5:00 **Stretch Break**

5.15-6:15 **Quality of Life and Emotional Health of the Family: A Panel Discussion**

Moderator: Erik Henricson, PhD
Panel: James Poysky, PhD; Rachel Poysky; Denise Gruender, BS; Jamie Jackson, PhD; Jessica Rownd

- Behaviors and Duchenne
- Your Family Dynamic
- Making Time for Yourself
- Your Extended Family

6:15 **Polling Questions**

6:15-8:45p **Welcome Reception, Poster Session, and Vendor Fair**

Interaction Alley

Friday, June 28: 11:00-2:30p & 4:30-6:30p

Saturday, June 29: 11:00-4:00p

Rooms: Merritt and Longboat

- The Duchenne Registry: Registration & Update
- Genetic Counselor: Individual Counseling (by appointment)
- School Issues: Private Counseling (by appointment)
- Physical Therapy: Private Coaching (by appointment)
- Xbox Gaming Room (Friday only)
- Microsoft Adaptive Technology (Friday only)

Friday Kids Track Hours






Kids Track Room: Tarpon A/B
9:30-12:30p

6:45a	Early Morning Yoga Room: Merritt
7:30-8:45	Breakfast
8:00	Our Stories Matter Lance Hester
8:10	Polling Questions
8:15-9:30	Gene Therapy: What We Know Today & What We Will Need to Know Tomorrow Moderator: Tim Cripe, MD, PhD
8:15	Understanding Gene Therapy – What We Know Today Barry Byrne MD, PhD
8:35	Panel Discussion: Current Hurdles in Gene Therapy Moderator: Tim Cripe, MD, PhD Panel: Lee Sweeney, PhD; Kevin Flanigan, MD; Kathryn Wagner, MD; Barry Byrne, MD, PhD
9:10	Q&A
9:30	Gene Therapy Trials Moderator: Richard Klein
9:30	Solid  Genevieve A. Laforet, MD, PhD
9:50	Pfizer  Michael Binks, MD
10:10	Sarepta  Doug Ingram
10:30	Q&A
10:50	Polling Questions
11:00	Break
11:20	Downstream Moderator: Lance Hester
11:20	Roche/Genentech  Roxana Dreghici, MD
11:40	Santhera  Kristina Nygren, MD
12:00	FibroGen  Bassem Elmankabadi, MD
12:20	Capricor  Linda Marban, PhD
12:40	Q&A
1:00	Polling Questions
1:00p-2:20	Lunch & Vendor Fair
2:20	Our Stories Matter Stephen Marchesani

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2:30	Duchenne & the Heart Moderator: Abby Bronson
2:30	Introduction & Overview Linda Cripe, MD
2:40	Outcome Measures for the Heart Kan Hor, MD
3:00	Second Generation Micro-Dystrophin Dongsheng Duan, PhD
3:20	Understanding Gene Therapy for the Heart Lee Sweeney, PhD
3:40	Cumberland Pharmaceuticals  Larry Markham, MD
4:00	Polling Questions
4:00	Q&A and Break
4:20	Our Stories Matter Stacie Al-Chokhachi
TRACK 1	
General Plenary Session Room: Crystal C/D/E	
4:30	Inflammation and Fibrosis Moderator: Abby Bronson
4:30	Catabasis  Joanne Donovan, MD, PhD
4:50	PTC Brian Pfister, PhD
5:10	Italfarmaco  Paolo Bettica, MD, PhD
5:30	ReveraGen  Paula Clemmons, MD
5:50	Mallinckrodt  Jacqueline Delfgaauw, PhD
6:10	Q&A
6:30p	Polling Questions
TRACK 2	
Teens & Adults Closed Session Room: Nomeus	
4:30	Quarterbacking Your Care Closed discussion session for only teens and adults living with Duchenne (age 14 and up), featuring: – Kathryn Wagner, MD (Neurology) – Christina Trout, RN, MSN (Transitions) – Linda Cripe, MD (Cardiology) – Richard Shell, MD (Pulmonary) – Claudia Senesac, PT, PhD, PCS (Physical Therapy) – Rob Benjamin, MD (Endocrine) – Tina Duong, PT, PhD (Physical Therapy)

Saturday, June 29th

Saturday Kids Track & Sib Shop Hours

Kids Track 2:30-5:00p	Movie and Lego Building <i>Room: Tarpon A/B</i>
Sib Shop 2:30-5:00p	A workshop for our Duchenne and Becker siblings attending the conference. Led by: Jill Castle <i>Room: Nomeus</i>

7:15a **Yoga**
Room: Merritt

8:00-9:15 Breakfast

8:45 **Our Stories Matter**
PAAC Member - Buddy Cassidy

9:00 **CRISPR - Future Strategies in Gene Therapy**
Moderator: Buddy Cassidy

9:00 **Pre-Clinical Research in CRISPR**
Melissa Spencer, PhD

9:20 **The Path to the Clinic**
Charles Gersbach, PhD

9:40 **Q&A**

10:00 **Understanding Immunity**
Moderator: Sharon Hesterlee

10:00 **Innate and Acquired Immunity**
Carrie Miceli, MD, PhD

10:20 **Overcoming Neutralizing Antibodies for Broader Inclusion**
Jude Samulski, PhD

10:40 **Q&A**

11:00 **Polling Questions**

11:00-11:20 Break

TRACK 1
General Plenary Session
Room: Crystal C/D/E

TRACK 2
Teens & Adults Closed Session
Room: Nomeus

11:20 **Gene Correction**
Moderator:
Richard Klein

11:20 **PTC** ⌘
Richard Able Jr., PhD

11:40 **Sarepta** ⌘
Gilmore O'Neill,
M.B., M.M.SC.

12:00 **Wave** ⌘
Michael Panzara,
MD, MPH

12:20 **NS Pharma** ⌘
Leslie Magnus, MD

12:40 **Q&A**

1:00p **Polling Questions**

1:00-2:30 Lunch

2:30-5:00 Care Breakout Sessions

► Early Childhood

Room: Canaveral

Moderator: Linda Cripe, MD - Cardiology (NW)

– NMS

Mena Scavina, MD (Nemours Dupont)

– Learning & Behavior

Jamie Jackson, MD (NW)

– School & Behavioral Issues

Denise Gruender, BS

► Childhood/Tweens

Room: Crystal A/B

Moderator: Richard Shell, MD - Pulmonary (NW)

– NMS

Kathryn Wagner, MD (KKI)

– GI & Nutrition

Kent Williams, MD (NW)

– Cardiology

Steve Kindell, MD (CHW)

– Endocrine: Growth, Puberty, & Bone Health

David Repaske, MD (UVA)

► Teens/Young Adults

Room: Longboat

Moderator: Chet Villa, MD - Cardiology (CCHMC)

– Endocrine: Growth, Puberty, & Bone Health

Rob Benjamin, MD (Duke)

– NMS/Rehabilitation

Vamshi Rao, MD (Lurie)

– Transition Planning

Christina Trout, RN, MSN (U of Iowa)

– Psychosocial Issues

James Poysky, PhD

– Pulmonary

Jonathan Finder, MD (Le Bonheur)

5:15-6:15

Our Voice Matters:

Duchenne Town Hall Forum

Room: Crystal C/D/E

Moderator: Pat Furlong

All presenters, panelists, and guests are

invited to participate in this interactive forum.

7:00

Reception

7:30p

Dinner Celebration and Dance Party

Room: Peninsular 1-4

Sunday, June 30th

8:30-10:30a Breakfast

9:30-11:30 Hands-On PT Sessions

Room: Merritt

Claudia Senesac, PT, PhD, PCS