

**Wednesday, June 26**

11 am Registration opens  
7pm-10pm Newly Diagnosed Meeting (private)  
7pm-10pm Teen and Adult Social (private)

**Thursday, June 27**

7:30-8:15 Yoga

**Attendees are welcome to join either session at any time.**

<b><u>Understanding Duchenne from the start</u></b> <b><u>9:15-12:00 Moderator: Pat Furlong</u></b>		<b><u>Adults and Teens Session</u></b> <b><u>9:15-12:00 Moderator: Annie Kennedy</u></b>	
9:15-9:40	Introduction... So it begins Pat Furlong	<b><u>Carving Your Personal Path - WORKSHOP SESSIONS</u></b> Identifying Options Beyond High School -- and Resources to Help you Get There Facilitators: Annie Kennedy, Jill Castle MEd, PAAC Member <i>This session is open to everyone age 14 and above.</i>	
9:40-10:00	Making a Care Plan & lining up your team Christina Trout, MD	<u>College Workshop</u> Love learning? Ready to tackle college? Come hear about community college and four year university options, disability resources, scholarships and creative supports for assistance once you've found your perfect campus setting.	
10:00-10:20	Why Physical Therapy is so necessary Claudia Senesac, PT,PhD,PCS	<u>Job Workshop</u> Ready to just get to work (with or without a college degree)? By utilizing our interest survey info and strength based assessments, come up with creative job ideas that set your soul on fire.	
10:20-10:40	Diet & Duchenne: simple Nutrition advice Kent Williams, MD	<u>Entrepreneur Workshop</u> Academics not your passion? Need more flexibility than full time employment offers? Use your interests and strengths to start your own endeavor!	
10:40-11:00	Q & A and Break		
11:00-11:20	Talking to School, Family & children about Duchenne James Poysky, PhD		
11:20-11:40	Early Identification of boys with developmental and socio- emotional delays Amy Wagner, MD		
11:40-12:00	Navigating Challenges at School and Home Denise Gruender, BS		
POLLING QUESTION #1 FOCUSED ON NEW DIAGNOSIS			

**12:00-1:00 Lunch**

**1:00 OPENING PPMD' 2019 Annual Conference**

**Where we have come from/ Where we are going**

Pat Furlong, PPMD

PPMD's Journey video and presentation

<b>1:40-3:00</b>	<b>The Complexities of Duchenne</b>	<b>Moderator, Pat Furlong</b>
1:40-2:00	Duchenne: a Multisystem Disease	Stan Nelson, MD, PhD
2:00-2:20	Variability: Modifiers to the Gene	Kevin Flanigan, MD
2:20-2:40	Measuring Outcomes in the face of variability	Cliff Bechtold
2:40-3:00	Challenges in Designing Trials	Craig McDonald, MD
3:00-3:15	Q&A	
<b>3:15-3:30</b>	<b>Break</b> (3:15-5:15 Kids Track Session 1 – group/ presenter)	
<b>3:30-5:00</b>	<b>Addressing the Complexities of Duchenne</b>	<b>Moderator: Anessa Fehsenfeld</b>
3:30-3:40	Attacking Duchenne from every angle	Anessa Fehsenfeld, PPMD Chairman
3:40-4:00	<b>Duchenne Research and Clinical Trials:</b>	Abby Bronson
	<ul style="list-style-type: none"><li>• Current Therapeutic Approaches and Clinical Trials</li><li>• Strategies to Accelerate the process</li></ul>	
4:00-4:20	<b>Advocacy: Pushing the Timeline to treatments</b>	Annie Kennedy
	<ul style="list-style-type: none"><li>• Influencing Policy that drives Duchenne research and treatments</li><li>• Working with regulators to assure access to therapies</li></ul>	
4:20-4:40	<b>Improvements in Care: Extending quality of life with a focus on the future</b>	Kathi Kinnett
	<ul style="list-style-type: none"><li>• The Certified Duchenne Care Center network accomplishments</li><li>• Integrating Clinic, Industry and Registry to build a solid network</li></ul>	
4:40-5:00	<b>The Patient's Voice: Why YOU are the most important part of the Community</b>	Ryan Fischer
	<ul style="list-style-type: none"><li>• Your Preferences and Participation drives innovation and change</li></ul>	
5:00-5:10	<b>POLLING QUESTION #2, 3, &amp; 4– General Demographics and STRETCH</b>	
<b>5:15-6:15</b>	<b>Quality of Life and Emotional Health of the Family:</b>	
	<b>A Panel Discussion</b>	<b>Moderator: Erik Henricson, PhD</b>
	(Panelists: James Poysky, PhD, Rachel Poysky, Denise Gruender, BS, Jaime Jackson, MD, and Jessica Rownd)	
	<ul style="list-style-type: none"><li>• Behaviors and Duchenne</li><li>• Your Family Dynamic</li></ul>	<ul style="list-style-type: none"><li>• Making Time for Yourself</li><li>• Your extended family</li></ul>
	<b>POLLING QUESTION #5</b>	
<b>6:15-8:45</b>	<b>Welcome Reception/ Poster Session / Vendor Fair</b>	

## Friday, June 28

6:45 Early Morning Yoga

8:00- 8:10 **Our Stories Matter (Challenges I Faced)** **Lance Hester**

8:15-9:30 **Gene Therapy: Moderator: Tim Cripe**  
**What we know today and what we will need to know tomorrow**

8:15-8:35 Understanding Gene therapy – what we know today Barry Byrne MD, PhD

8:35-9:10 Panel Discussion: Current hurdles in Gene Therapy Moderator: Tim Cripe, MD  
 (Panelists: Lee Sweeney, Kevin Flanigan, Kathryn Wagner, Barry Byrne)

9:10-9:30 Q&A Audience  
 (9:30-12:30 Kids Track breakout sessions)

9:30 **Gene Therapy Trials Moderator: Lance Hester**

9:30-9:50 Solid

9:50-10:10 Pfizer

10:10-10:30 Sarepta

10:30-10:50 Q & A

10:50-11:00 **POLLING QUESTIONS 6 & 7**

11:00-11:20 **BREAK**

11:20-1:00 **Downstream Moderator: Lance Hester**

11:20-11:40 Roche/Genentech

11:40-12:00 Santhera

12:00-12:20 Fibrogen

12:20-12:40 Capricor

12:40-1:00 Q & A

**POLLING QUESTION #8**

1:00-2:20 **Lunch (Vendor Fair Open)**

**Our Stories Matter**

2:20-2:30 **Our Stories Matter Stephen Marchesani**

2:30-4:00 **Duchenne and the Heart Moderator: Stephen Marchesani**

2:30-2:40 Introduction and overview Linda Cripe, MD

2:40-3:00 Outcome Measures for the Heart Kan Hor, MD

3:00-3:20 2nd Generation Micro dystrophin Dongshen Duan, PhD

3:20-3:40 Understanding Gene Therapy for the heart Lee Sweeney, PhD

3:40-4:00 Cumberland Pharmaceuticals

**Interaction Alley**  
**open 11:00- 2:30 &**  
**4:30-6:30**  
 Duchenne Registry  
 Registration  
 School Issues  
 Private counseling  
 Physical Therapy  
 Private Coaching  
 Xbox Gaming Room

POLLING QUESTION #9 and #10

4:00-4:20 Q & A and Break

4:20-4:30 Our Stories Matter

Stacie Al-Chokhaci

TRACK 1 – General Plenary Session	TRACK 2 ( Teens and Adults closed session)
<p><b>4:30 Inflammation and Fibrosis Moderator: Staci Al-Chokhaci</b></p> <p>4:30-4:50 Catabasis</p> <p>4:50-5:10 PTC</p> <p>5:10-5:30 Italfarmaco</p> <p>5:30-5:50 Reveragen</p> <p>5:50-6:10 Mallinckrodt</p> <p>6:10-6:30 Q&amp;A</p>	<p><b>4:30 Quarterbacking Your Care</b></p> <p>Closed discussion session for teens and Adults living with Duchenne (age 14 and up)</p> <p><u>Kathryn Wagner, MD (Neuro)</u></p> <p><u>Christina Trout, MD (Transitions)</u></p> <p><u>Linda Cripe, MD (cardiology)</u></p> <p><u>Richard Shell, MD (pulmonary)</u></p> <p><u>Claudia Senesac, PT, PhD, PCS (PT)</u></p> <p><u>Rob Benjamin, MD (Duke, Endocrine)</u></p>

POLLING QUESTIONS #11 AND #12

**Saturday. June 29**

7:15 Yoga

**8:30 Our Stories Matter Storyteller 4**

PAAC Member - Buddy Cassidy

**8:40-9:20 CRISPR - Future Strategies in Gene**

**Moderator: Buddy Cassidy**

8:40-9:00 Pre-clinical research in CRISPR

9:00-9:20 Moving CRISPR toward the clinic

9:20-9:40 Q & A

Melissa Spencer, PhD

Charles Gersbach, PhD

POLLING QUESTIONS 13

**9:40 Living Duchenne in 2019**

**Moderator: Jonathan Finder, MD**

9:40-10:00 TBA

10:00-10:20 Social Media: the good, the bad, and the ugly

10:20-10:40 Citizen Science: Duchenne Registry- get your phones out and ready!!!

10:40-11:00 The Duchenne Outcomes Research Interchange: cutting edge for the future

TBD

Will Nolan

Ann Martin

Ryan Fischer

POLLING QUESTIONS #14

11:00-11:20 Break

Track 1 – General Plenary Session	Track 2 - Teen and Adult Closed session
<b>11:20-1:00</b> <b>Gene Correction</b> <b>Moderator: Richard Klein</b> 11:20-11:40     PTC 11:40-12:00     Sarepta 12:00-12:20     Wave 12:20-12:40     NS Pharma 12:40-1:00     Q&A POLLING QUESTION #15 and #16	<b>Topics TBD 11:20</b> Closed Session for Teens and Adults living with Duchenne ( ages 14 and up )

**1:00-2:30**     *Lunch*

**2:30-5:00**     **Care Breakout Session**

Early Childhood (Moderator: Linda Cripe, Cardiology, NW)	Childhood/Tweens (Moderator: Richard Shell, Pulmonary, NW)	Teens/Young Adults (Moderator: Chet Villa, Cardiology, CCHMC)	Kids Track #3	Sib Shop Led by: Jill Castle
<b>NMS</b> Mena Scavina, MD, Nemours Dupont  <b>Learning and Behavior</b> Jamie Jackson, MD, NW  <b>School and Behavioral issues</b> Denise Gruender, BS	<b>NMS</b> Kathryn Wagner, MD KKI  <b>GI and Nutrition</b> Kent Williams, MD, NW  <b>Cardiology</b> Steve Kindell, MD, CHW  <b>Endocrine- growth, puberty, bone health</b> David Repaske, MD, UVA	<b>Endocrine- puberty, bone health</b> Rob Benjamin, MD, Duke  <b>NMS/Rehabilitation</b> Vamshi Rao, MD , Lurie  <b>Transition Planning</b> Christina Trout, MD, U of Iowa  <b>Psychosocial Issues</b> James Poysky, PhD  <b>Pulmonary</b> Jonathan Finder, MD LeBonheur	Movie and Lego Building gathering	A workshop for our Duchenne and Becker siblings attending the conference.

**5:15-6:15**     **Our Voice Matters**     **Duchenne Town Hall Forum**     **Moderator: Pat Furlong**  
 All presenters, panelists, and guests are invited to participate in this interactive forum.

**7:00**     *Reception / 7:30 Dinner Celebration and Dance Party*

**Sunday, June 30<sup>th</sup>**

**8:30-10:30**     *Breakfast*

**9:30-11:30**     **Hands on PT sessions**

Claudia Senesac, PT,PhD,PCS