

PPMD 2018 CONFERENCE AGENDA

Parent **JOIN THE FIGHT.**
Project **END DUCHENNE.**
Muscular
Dystrophy

KEY:



RESTORING OR REPLACING
DYSTROPHIN



INFLAMMATION



IMPROVING MUSCLE
MECHANICS

See page 9 of your Conference Booklet for corresponding clinical trial and research study FAQ sheets

Wednesday, June 27th

7:00- 9:30p Teen Adult Social

Estrella Theatre

Coordinated by Buddy Cassidy, Michael Counterman, Alex Toalson, & PPMD's Adult Advisory Committee (PAAC)

7:00- 9:30p Newly Diagnosed Meet and Greet

Camelback H & I

For our newly diagnosed families only, please

Thursday, June 28th

Choose one of the sessions below

9:00- 12:15p Understanding the Diagnosis of Duchenne Sessions

Camelback Ballroom K, L, M, N

9:00 Duchenne: Now What?

Pat Furlong

Putting Your Plan in Place and Lining Up Resources

9:20 Understanding Genetics, Mutation and Other Details

Stan Nelson, MD

9:40 Making A Care Plan and Lining Up Your Team

Kathi Kinnett

10:00 The Value of Physical Therapy

Tina Duong

10:20 Your Data at Work: The Duchenne Registry

Ann Martin

10:40 Break

11:00 Talking to Family, Friends and School

Catherine Butz, PhD

11:20 Navigating the Challenges of School

Denise Gruender, ABC Educational Services Of NC/SC

11:40 Taking Care of You

Eric M. Butter, PhD

12:00p Q&A

12:15 Adjourn

9:00- 12:15p Transitions meeting: Planning for a Lifetime of Happiness – Not just a Happy Childhood Focus on Teens and Adults

Ballrooms E, F, G

9:00 Welcome

Annie Kennedy & Colin Rensch

9:15 Navigating Benefit Eligibility

Marilyn Morrison, VACCESS
(Buddy Cassidy, facilitator)

10:00 Knowing Your Rights & Responsibilities

Conrad Reynoldson

10:30 Financial Planning

Michael Wilkinson & John Borchers

11:15 Managing & Hiring PCAs

Buddy Cassidy and Michael Counterman

11:45 Panel: Life After High School

Colin Rensch, Alex Toalson, Ben Dupree,
Jenn Bauer, Michael Counterman

12:15p Adjourn

12:00–1:00 Lunch

Ballroom D

Thursday continues >

Annual Conference begins:

Camelback Ballroom K, L, M, N

1:00p	Welcome Address Pat Furlong
1:30	Panel Presentation: What we know and don't know about Duchenne Moderator: Pat Furlong What do we know about the biology and variability of Duchenne Kevin Flanigan, MD What do we know about Genotype/Phenotype Stan Nelson, MD Impact of care, nutrition, PT and supplements Brenda Wong, MD
2:30	Q&A
2:40	Pat's Pearls – Head, Shoulders, Knees & Toes Pat Furlong
3:00	Dental Considerations in Duchenne Elizabeth Vroom
3:20	Digestion and Elimination Kent Williams, MD
3:40	Panel Presentation: Cardiac Health in Duchenne Moderator: Kathi Kinnett Pediatrics Linda Cripe, MD Adult Care & Carrier Study findings May Ling Mah, MD What we are learning from cardiac MRI Kan N. Hor, MD Understanding cardiac medications Larry Markham, MD
4:40	Break
5:00	Panel Presentation: Clinical Trial Readiness Moderator: Eddie Smith, MD Panel: Erica Goude, MD, Tina Duong, MPT, Ann Martin, Amber Sapp, Tara McDonnell Moorehead – Panel discussion on understanding clinical trial information. Expectations. – What makes you choose a clinical trial? – How do you discuss clinical trials? – What is informed consent?
6:00	Master Protocol: What is it and why are we talking about it? Abby Bronson
6:30p	Welcome Reception and Poster Session in the East Foyer

Friday, June 29th

Other Friday meetings	
9:30a-12:30p	Kids Track <i>Ballroom G</i>
12:30-1:45p	Clinician lunch (Closed meeting) <i>Camelback H & I</i>
12:30-1:45p	PPMD's SAC (Closed meeting) <i>Camelback J</i>
6:45-10:00p	DDDR Meeting (Closed meeting) <i>Estrella Room</i>
7:00-9:30p	Teen/Adult hang out <i>Camelback H</i>
8:00a	Breakfast served <i>Ballroom Foyer</i>
8:30	Duchenne Research Overview: the landscape and the opportunities Pat Furlong
9:00	Conference Polling Begins Ryan Fischer – Duchenne Citizen Science – contributing data to advance research – During this presentation we will discuss the value of contributing data. – We will also then pose live polling questions to the audience as well as the audience watching via live stream. This interactive polling will continue throughout the main session on Friday and Saturday.
9:20	Intro to the session – Restoring or Replacing Dystrophin ☒ Moderator: Abby Bronson
9:20	NS Pharma ☒ Paula Clemens, MD; Hirofumi Komaki, MD
9:40	WAVE Life Sciences ☒ Wendy Erler, VP Patient Adv.
10:00	Sarepta Therapeutics ☒ Doug Ingram, CEO
10:20	Summit. Plc ☒ Michelle Avery, PhD
10:40	PTC Therapeutics ☒ Stuart Peltz, CEO
11:00	Q&A
11:15	Polling Time #1 and then a Break
11:30	Panel Presentation: Studies currently underway in Gene Therapy ☒ Moderator: Tim Cripe, MD, PhD Panel: Jerry Mendell, MD, Nationwide Children's; Jorge Quiroz, CMO, Solid BioSciences; Tara McDonnell Moorhead, Dir. Clinical Research, Pfizer; Kevin Flanigan, MD, Nationwide Children's

Friday continues >

Friday, June 29th continues

12:30-1:45p **Lunch (lunch is a nonscheduled time, however, you are also invited to one of our discussion lunch sessions):**

<p>Duchenne Carriers <i>Ballroom E</i> Facilitated by Jerry Mendell, MD; Linda Cripe, MD; Jaime Jackson, PhD; May Ling Mah, MD; Kan N. Hor, MD; Pat Furlong</p>	<p>Teen/Adults are invited to join a Closed Lunch Session: Dating & Relationships (must be an individual with Duchenne or Becker in high school or above - <u>no parents</u>) <i>Ballroom F</i> Moderator: Pat Moeschel</p>
--	--

1:50 **Intro to the Session: Inflammation** ⚡
Moderator: Abby Bronson

2:00 **Reveragen** ⚡
Eric Hoffman, MD, PhD

2:20 **Catabasis** ⚡
Joanne Donovan, MD, PhD

2:40 **Italfarmaco** ⚡
Paolo Bettica, MD, PhD

3:00 **Mallinckrodt** ⚡
Bryan Due, PhD

3:20 **Capricor** ⚡
Deborah Ascheim, MD

3:40 **Q&A followed by Polling Time #2 and then a Break**


4:00 **Why Advocacy is more important than ever**
Ryan Fischer and Annie Kennedy

Polling time #3

4:45 **Panel Presentation: Today's Tomorrow – Looking at the future**
Moderator: Pat Furlong
Panelists: Annie Kennedy, Abby Bronson, Jane Larkindale, PhD, Stan Nelson, MD, Kathi Kinnett

What is needed to move the needle in the Duchenne landscape? How is PPMD working to make this happen?

- Duchenne protein signature
- Duchenne Regulatory Science Consortium
- PPMD's Certified Duchenne Care Centers
- Duchene Newborn Screening
- Master Protocol
- Duchenne Registry 2.0
- Preclinical Assessment Lab

6:00p **Race to End Duchenne .1K (You won't want to miss this!)** 

Saturday, June 30th

Other Saturday meetings

8:45-11:00a **Kids Track**
Ballroom H and I
Individual age group sessions with crafts and projects

1:30-3:30p **Kids Track**
Ballroom H and I
Movie and Lego time

7:45- 9:00a **Breakfast available**
Camelback Foyer

8:30- 9:45 **Breakfast Breakouts:**

▶ **Moms in Motion**
Estrella room
Michelle Furlong, Pat Furlong, plus the community

Mom's yoga class then a discussion on the pressures of being a Duchenne mom. How to keep balance.

▶ **Dads De-stress**
Ballroom G
Jason Minx, John Killian, plus the community

Dad's yoga class and then a discussion on the pressures of being a Duchenne dad. How to keep balance.


▶ **Sibshop Workshop**
Camelback J
Jill Castle

Workshop for brothers and sisters who have a brother with Duchenne.

▶ **Kids Track**
Camelback H & I
Rachel Poysky and team

Kids Track sessions will be split up into 2-3 age groups with different activities for each group.

10:00 **Keynote Address**
Camelback Ballroom K, L, M, N
Chris Jones, PPMD Board Member

10:30 **Intro to the Session: Improving Muscle Mechanics** 
Moderator: Abby Bronson

10:40 **Roche/Genentech** 
Michelle Krishnan, MD, PhD

11:00 **Pfizer** 
Shannon Marraffino, Dir. Clinical Research

11:20 **Santhera** 
Kristina Nygren, CMO

11:40 **Phrixus** 
John Lynn Jeffries, MD

12:00p **Fibrogen** 
Elias Kouchakji, MD; Tro Sekayan, MD

12:20 **Q&A and then Polling Time #4**

Saturday, June 30th continues

12:40–2:00 Lunch (lunch is a nonscheduled time, however, you are also invited to one of our discussion lunch sessions):

Lifelong physical therapy needs in Duchenne
Estrella room
Presenters: Leslie Vogel, Tina Duong

2:00-4:00p **Care Breakouts:**

▶ **Diagnosis and Early Childhood**

Ballroom E

Moderator: Larry Markham, MD

– **Learning and Behavior**

Andrea Miele, MD, CHC

– **Physical Therapy**

Leslie Vogel, MSPT, Seattle Children's

– **NMS**

Brenda Wong, MD, U Mass

▶ **Childhood**

Ballroom F

Moderator: Linda Cripe, MD

– **NMS, Growth and Puberty**

Eddie Smith, MD, Duke

– **Genes, Mutations and Supplements**

Stan Nelson, MD, UCLA

– **Learning and Behavior**

Eric Butter, PhD, Nationwide Children's

– **Assessing Educational Plans and Resources**

Karen Carpenter, MS, CRC, LPC,

Univ. of Wisconsin Hospitals and Clinics

– **Pulmonary**

Rich Kravitz, Duke

▶ **Tweens and Teens**

Ballroom G

Moderator: Sue Apkon, MD

– **Pulmonary**

Richard Shell, MD, NW

– **NMS/Rehabilitation**

Sue Apkon, MD, Seattle Children's

– **Transition Planning**

Ann Modricin, MD, CM

– **GI and Nutrition**

Kent Williams, MD, NW

– **Endocrine and Bone Health**

Sara DiVall, MD, Seattle Children's

▶ **Quarterbacking Your Care**

Camelback J

Closed Session for Teens & Adults

Moderator: Annie Kennedy

– **Care Guidelines & New Transition Tools**

Christina Trout, MD, Iowa

– **Benefits & Resources**

Marilyn Morrison, VACCESS

– **Pulmonary**

Jonathan Finder, MD, Children's Hospital Pitt

– **Cardiology**

Nancy Halnon, MD, UCLA

– **Endocrine**

Pransanth Surampudi, MD, UC Davis

– **Physical Therapy**

Tina Duong, MPT, Stanford

4:10-5:15

Panel Presentation: Gene Editing – A strategy for the future (CRISPR)

Moderator: Kevin Flanigan, MD

Panelists: Dongsheng Duan, PhD,

Jeff Chamberlain, PhD,

Melissa Spencer MD, PhD

Polling Time #5

5:15-6:15

Panel Presentation: Living Duchenne from different perspectives

Moderator: Pat Furlong

Panelists: Gretchen Egner, Rich Romito,

Diane Friar, Brayden Hester,

Dee Dee M. Castle, Colin Rench

Polling Time #6 and Word Cloud #2

6:30p

Reception

7:15–9:30p

Celebration Dinner