PPMD 2018 CONFERENCE AGENDA

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)

Thursday, June 28th

Choose one of the sessions below

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- 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

12:00–1:00 Lunch
Ballroom D
### Conference Schedule

**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  
Kevin Flanigan, MD  
Stan Nelson, MD  
Brenda Wong, MD

**2:30** Q&A

**2:40** Pat’s Pearls – Head, Shoulders, Knees & Toes  
Pat Furlong

**3:00** Dental Considerations in Duchenne  
Elisabeth Vroom

**3:20** Digestion and Elimination  
Kent Williams, MD

**3:40** Panel Presentation: Cardiac Health in Duchenne  
Moderator: Kathi Kinnett  
Linda Cripe, MD  
May Ling Mah, MD  
Kan N. Hor, MD  
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 Moorhead  
Doug Ingram, CEO  
Michelle Avery, PhD  
Stuart Peltz, CEO

**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

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### Friday, June 29th

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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| 8:00a      | Breakfast served  
Ballroom Foyer
| 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

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**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):
  Duchenne Carriers
  Ballroom E
  Facilitated by Jerry Mendell, MD; Linda Cripe, MD; Jaime Jackson, PhD; May Ling Mah, MD; Kan N. Hor, MD; Pat Furlong
  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 - no parents)
  Ballroom F
  Facilitated by Jerry Mendell, MD; Linda Cripe, MD; Jaime Jackson, PhD; May Ling Mah, MD; Kan N. Hor, MD; Pat Furlong

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
- Duchenne 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.

8:00p Race to End Duchenne .1K
(You won’t want to miss this!)

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 continues >
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