

2017 ANNUAL CONNECT CONFERENCE AGENDA

PRE-CONFERENCE: WEDNESDAY, JUNE 28

Registration Opens

11:00am • Salon 1 Alcove, 7th Floor

Private Event: Newly Diagnosed Families – Download Duchenne and Drinks

6:00pm–9:00pm • Room TBA

PPMD invites all newly diagnosed families and families attending the conference for the first time to gather and get to know each other. This informal gathering is private and a safe place to ask questions, express concerns, and take a breath.

Private Event: Teens and Adults with Duchenne Social

6:00pm–9:00pm • Lincolnshire Room, 6th Floor

This is a private event for teens and adults living with Duchenne/Becker hosted by PPMD's Adult Advisory Committee (PAAC).

DAY 1: THURSDAY, JUNE 29

KIDS TRACK

3:30pm–6:00pm • Northwestern Room, 6th Floor

Must be registered for Kids Track.
See p. 3 of your Conference Booklet for more information.

RESOURCE FAIR & POSTER SESSION HOURS

6:00pm–9:00pm • Salon 3, 7th Floor

Registration Opens

7:30am • 7th Floor Registration Desk

Newly Diagnosed Program Meeting – Getting Through the Basics

8:30am–12:00pm • Salon 3, 7th Floor

This session is open to any conference attendee, but presentations are geared toward newly diagnosed families and families attending the conference for the first time.

- Here We Are / 8:30am
Speaker: Pat Furlong
- Understanding Your Genetic Mutation & What That Means / 9:00am
Speaker: Kathryn Wagner, MD
- Setting Up Your Teams – Care, Support, & Family / 9:20am
Speaker: Kathi Kinnett, MSN, CNP
- DuchenneConnect Registry & Other Tools / 9:40am
Speaker: Ann Martin, MS, CGC
- Q & A / 10:00am
- Break / 10:20am
- Understanding the Areas of Duchenne Research / 10:40am
Speaker: Melissa Spencer, PhD
- Looking at Clinical Trials / 11:00am
Speaker: Pat Furlong
- Navigating School / 11:20am
Speaker: Jill Castle, M Ed
- Q & A / 11:40am

Teens and Young Adult Track

8:30am–12:00pm • Chicago Ballroom D & E, 5th Floor

This session is open to any conference attendee, but will be led by and geared toward teens and adults living with Duchenne.

- Life Hacks & Technology Adaptations / 8:30am
Panelists: Alan Chaulet; Jonathan Piacentino; Michael Counterman
- Experiencing Depression & Anxiety? It's Not 'Just You' – Mental Health in Duchenne/Becker & What You Can Do to Thrive / 9:00am
Co-Facilitators: Buddy Cassidy; Ben Dupree; James Pinsky, PhD; Danielle Sheypuk, PhD
- Break / 9:45am
- Life After Highschool / 10:00am
Panelists: Colin Werth; Colin Rensch; Jonathan Piacentino; Sean Crosby; Michael Counterman
- Navigating Adulthood – Information About Critical Financial Considerations & Benefit Eligibility / 11:00am
Speakers: Jenny Kean, LCSW; Annie Kennedy

Lunch

12:00pm–1:00pm • 7th Floor Foyer

Connect Conference Begins

12:30pm–1:50pm • Salon 1 & 2, 7th Floor

- Welcome Address – Duchenne 2017 / 12:30pm
Speaker: Pat Furlong
- Taking Care of Our Children, Our Community, Ourselves / 1:00pm
Speaker: Chris Jones
- Pillars and Progress – PPMD's Programs and Priorities / 1:30pm
Speaker: John Killian

Our New Duchenne Basics – Care, Data, Trials, and Outcomes

1:50pm–3:40pm • Salon 1 & 2 • Moderator: Pat Furlong

- Care – Why Care Matters / 2:00pm
Speaker: Kathi Kinnett, MSN, CNP
- Data – Why Data Makes the Difference / 2:20pm
Speaker: Stan Nelson, MD
- Trials – How to Know What Trial is Right for You / 2:40pm
Speaker: Pat Furlong
- Outcomes – Current and Forthcoming Outcome Measures / 3:00pm
Speaker: Tina Duong, MPT
- Q & A / 3:20pm

Break

3:40pm–4:00pm

Accessing Our First Two US Approved Treatments for Duchenne

4:00pm–5:00pm • Salon 1 & 2 • Moderator: Stan Nelson, MD

- EXONDYS 51 / 4:00pm
Speaker: Ed Kaye, MD
- EMFLAZA / 4:20pm
Speaker: Stuart Peltz, PhD
- Q & A / 4:40pm

The Clinical Trial Experience

5:00pm–6:15pm • Salon 1 & 2 • Moderator: Tim Cripe MD, PhD

Panelists: Chris Curran; Michael Gaglianone; Dylan Miceli; Tammy Cate; Alison Greiner

Welcome Reception / Resource Fair & Poster Session

6:15pm–8:30pm • Salon 3

DAY 2: FRIDAY, JUNE 30

KIDS TRACK

9:30am–12:30pm • Northwestern Room, 6th Floor

Must be registered for Kids Track.
See p. 3 of your Conference Booklet for more information.

RESOURCE FAIR & POSTER SESSION HOURS

8:00am–3:00pm • Salon 3, 7th Floor

Registration Opens

7:30am • 7th Floor Registration Desk

Race To End Duchenne .1K

7:45am • Salon 3

Breakfast / Resource Fair & Poster Session

8:15am–9:15am • Salon 3

Intro to Clinical Trial Update Sessions

9:15am–9:30am • Salon 1 & 2 • Moderator: Abby Bronson

Clinical Trial Updates – Part 1

9:30am–10:45am • Salon 1 & 2 • Moderator: Abby Bronson

- Givinostat / 9:30am
Speaker: Italfarmaco – Paolo Bettica, MD, PhD
- Raxone / 9:45am
Speaker: Santhera – Thomas Meier, PhD
- Vamorolone / 10:00am
Speaker: ReveraGen – Eric Hoffman, PhD
- CAP-1002 / 10:15am
Speaker: Capricor – Linda Marban, PhD
- Q & A / 10:30am

Break

10:45am–11:00am

Clinical Trial Updates – Part 2

11:00am–12:00pm • Salon 1 & 2 • Moderator: Jonathan Finder, MD

- Domagrozumab / 11:00am
Speaker: Pfizer – Michael Binks, MD
- Anti-Myostatin Adnectin / 11:15am
Speaker: BMS – Cliff Bechtold, MS
- HT-100 / 11:30pm
Speaker: Akashi – Diana Escolar, MD
- Q & A / 11:45pm

Lunch / Resource Fair & Poster Session

12:00pm–1:30pm • Salon 3

NOTE: Lunch for neuromuscular care team (nurses, physicians, therapists, nutritionists, etc) is in the Denver/Houston room (5th floor).

Gene Transfer – How to Understand the Best Situation For My Family

1:30pm–2:40pm • Salon 1 & 2 • Moderators: Melissa Spencer, PhD and Jeff Chamberlain, PhD

Panelists: Louise Rodino-Klapac, PhD; Eric Olson, PhD; Katherine Beaverson, MS; Carl Morris, PhD; Amy Martin; Ben Dupree; Rose Juhasz

Break / Last Chance for Resource Fair & Poster Session

2:40pm–3:00pm

Care Complications – Going Beyond the More Common Elements of Care

3:00pm–4:10pm • Salon 1 & 2 • Moderator: Jonathan Finder, MD

Panelists: Linda Cripe, MD (Chest Pain); Lauren Camarda, MD (Flu and Airway Emergencies); Sue Apkon, MD (Fat Embolism); Pransanth Surapudi, MD (Bone Health)

Clinical Trial Updates – Part 3

4:30pm–5:45pm • Salon 1 & 2

- Edasalonexent / 4:30pm
Speaker: Catabasis – Joanne Donovan, MD, PhD
- Pamrevlumab / 4:45pm
Speaker: Fibrogen – Elias Kouchakji, MD
- MTB-1 / 5:00pm
Speaker: Mitobridge – George Mulligan, PhD
- Q & A / 5:15pm

Private Session: Dating & Relationships

5:00pm–7:30pm • Indiana Room, 6th Floor • Moderators: Danielle Sheypuk, PhD and Tina Duong, MPT

This session is for teens (age 15+) and adults living with Duchenne/Becker. It is a private meeting. No parents or PCA's.

DAY 3: SATURDAY, JULY 1

KIDS TRACK

2:20–5:00pm • Northwestern Room, 6th Floor

Must be registered for Kids Track.
See p. 3 of your Conference Booklet for more information.

Breakfast

8:00am • 7th Floor Foyer

Morning Address: Approvals in Duchenne – A Moment of Recognition & Appreciation

8:15am–8:45am • Salon 1 & 2

Speaker: Pat Furlong

Clinical Trial Updates – Part 4

8:45am–10:40am • Salon 1 & 2 • Moderator: Abby Bronson

- Ezutromid / 8:45am
Speaker: Summit – Michelle Avery, PhD
- Translarna / 9:00am
Speaker: PTC Therapeutics – Stuart Peltz, PhD
- Biglycan / 9:15am
Speaker: Tivorsan – Justin Fallon, PhD
- Exon Skipping / 9:30am
Speaker: WAVE Life Sciences – Wendy Erler
- Exon 53 NS-065/NCNP-01 / 9:45am
Speaker: NS Pharma – Paula Clemens, MD
- Exon 45 and 53 / 10:00am
Speaker: Sarepta – Lisa Borland, PharmD
- Q & A / 10:15am

Break

10:40am–11:00am

Wellness Workshops

11:00am–1:00pm • Various Rooms

Physical Therapy

Marriott Ballroom, 4th Floor

- PT & Stretching – Ambulatory / 11:00am
Speakers: Claudia Senesac PT, PhD, PCS; Laura Case, PT, DPT, MS, PCS, C/NDT
- Break / 11:55am
- PT & Stretching – Non-Ambulatory / 12:05pm
Speakers: Claudia Senesac, PT, PhD, PCS; Laura Case, PT, DPT, MS, PCS, C/NDT

Sibling & Family

Indiana Room, 6th Floor

- Siblings – Balancing Their Needs / 11:00am
Speakers: Molly Colvin, PhD; Jill Castle, M Ed
- Break / 11:55am
- What We Are Learning About Carriers / 12:05pm
Speakers: Kan Hor, MD; Linda Cripe, MD

Private: Teen & Young Adult

Open only to those age 14+ living with Duchenne/Becker. No parents or PCA's.
Avenue Room, 4th Floor

- Quarterbacking Your Care – An Opportunity to Talk Openly With an Expert Multi-Disciplinary Team About Care Considerations for Teens & Adults with Duchenne/Becker / 11:00am
Speakers: Sue Apkon, MD; Cristina Trout, MSN, Jane Taylor, MD; James Poysky, PhD; Tina Duong, MPT; Elizabeth McNally, MD, PhD

Lunch

1:00pm–2:15pm • 7th Floor Foyer

Care Breakout Sessions

2:30pm–5:00pm • Various Rooms

Diagnosis – Childhood (Ambulatory)

Indiana Room, 6th Floor
Moderator: Sue Apkon, MD

- Learning & Behavior / 2:30pm
Speaker: Molly Colvin, PhD
- Diagnosis, Steroids, & Steroid Alternatives / 2:50pm
Speaker: Sue Apkon, MD
- Exercise, Activity, & Assistance Devices / 3:10pm
Speaker: Claudia Senesac PT, PhD, PCS
- Hearts / 3:30pm
Speaker: Katheryn Gambetta, MD
- Nutrition & Supplements / 3:50pm
Speaker: Zoe Davidson, PhD
- Living with Duchenne / 4:10pm
Speaker: Jessica Curran
- Q&A / 4:30pm

Adolescents & Early Teens (Transitioning to Non-Ambulatory)

Marriott Ballroom, 4th Floor
Moderator: Linda Cripe, MD

- GI, GU, & Nutrition / 2:30pm
Speaker: Umme Salma Vahanvaty
- Pulmonary / 2:50pm
Speaker: Jane Taylor, MD
- Cardiology / 3:10pm
Speaker: Linda Cripe, MD
- Keeping Bones Healthy / 3:30pm
Speaker: Sasi Bowden, MD
- Learning, Behaviors, & Establishing Independence / 3:50pm
Speaker: James Poysky, PhD
- Planning for Transition to Adult Care / 4:10pm
Speaker: Christina Trout, MSN
- Q&A / 4:30pm

Late Teens & Adults

Avenue Room, 4th Floor
Moderator: Beth McNally, MD, PhD

- Endocrine & Bone Health / 2:30pm
Speaker: Pransanth Surampudi, MD
- Pulmonary / 2:50pm
Speaker: Lauren Camarda, MD
- Living with Duchenne / 3:10pm
Speaker: Buddy Cassidy
- Cardiac Health / 3:30pm
Speaker: Elizabeth McNally, MD, PhD
- SSI, Medicaid, Etc / 3:50pm
Speaker: Anna Goldberger, LCSW
- GI & GU / 4:10pm
Speaker: Kent Williams, MD
- Q&A / 4:30pm

Life with Duchenne/Becker through the Lens of PPMD's Adult Advisory Committee (PAAC)

5:15pm–6:15pm • Salon 1 & 2

Panelists: Buddy Cassidy; Colin Rensch; Colin Werth; Michael Counterman; Jonathan Piacentino; Sean Crosby

CONNECT CON! Celebrating the Superheroes of Our Community

7:30pm • Salon 1, 2, & 3

Join us for this fun celebration dance party honoring the superheroes living among us. Costume (or costume accessories) optional.

DAY 4: SUNDAY, JULY 2

Breakfast

8:00am–9:00am

Now What? Advocacy Initiatives Update – Regulatory, Congressional, Patient Preference Research, Access and Reimbursement

9:00am–9:45am • Salon 1 & 2

Speakers: Annie Kennedy; Ryan Fischer

Robotics – Innovation for Our Community

9:50am–11:00am • Salon 1 & 2 • Moderator: Buddy Cassidy

- Solid Bio / 9:50am
Speaker: Tina Duong, MPT
- NJIT / 10:10am
Speaker: Madeline Corrigan, PhD
- City University of NY / 10:30am
Speaker: Hao Su, PhD
- Q&A / 10:50am

Duchenne Hacks – Parent to Parent Open Mic to Ask Advice

11:00am–12:30pm • Salon 1 & 2 • Moderator: Jill Castle, M Ed

- Family Time – Holding It Together / 11:00am
Panelists: Mike Gaglianone; Gretchen Egner; Buddy Cassidy
- Schools – Navigating School, IEPs, Etc. / 11:30am
Panelists: Annie Kennedy; Molly Colvin, PhD; Jan Bonner; Buddy Cassidy
- Resources – The Who, How, and Where / 12:00pm
Panelists: Ryan Fischer; Annie Kennedy; Ann Lucas, MS, CGC

2017 CONNECT CONFERENCE ADJOURNED