

# PPMD DUCHENNE HEALTHCARE PROFESSIONALS SUMMIT JANUARY 18–20, 2023

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

InterContinental New York Times Square  
New York City, New York

Thank you to our sponsors for your support of this meeting.



## Tuesday, January 17

**8:00a – 4:00p** **Early Care Meeting**  
Gotham Ballroom

**6:00p – 8:00p** **Welcome Reception**  
The Stinger Lounge

## Wednesday, January 18

**7:15a – 8:00a** **Registration**  
**Breakfast Available**  
Gotham Foyer

**8:00a – 8:15a** **Welcome and About PPMD**  
Pat Furlong  
Gotham Ballroom

**8:15a – 8:45a** **Complexities of Delivery of AAV-Based Therapies**  
Sue Apkon, MD; Christine Caneva, BS;  
Kaitlin Haug, RN

**8:45a – 10:15a** **Session I**  
**Setting the Stage**  
Eric Camino, PhD  
Gotham Ballroom

**8:45a – 9:05a** **Dystrophinopathy: A Current Landscape**  
Annemieke Aartsma-Rus, PhD

**9:05a – 9:20a** **The State of Gene Therapy in Dystrophinopathy**  
Kevin Flanigan, MD

**9:20a – 9:40a** **Updates on the Clinical Trial Landscape**  
Craig McDonald, MD

**9:40a – 10:00a** **Defining Dystrophinopathy: Do We Need a New Disease Definition?**  
Emma Ciafaloni, MD

**10:00a – 10:15a** **Discussion and Open Q&A**

**10:15a – 10:30a** **Break**

**10:30a – 12:15p** **Session II**  
**The Words We Use, The Ways We Feel**  
Alexis Hazlett MSN, RN, CPN  
Gotham Ballroom

## Wednesday, January 18 continued

**10:30a – 10:45a** **Balancing Act: Physician and Father**  
Stan Nelson, MD

**10:45a – 11:15a** **Palliative Care in the Current Landscape**  
Moshe Cohn, MD

**11:15a – 11:30a** **The Burden of Caring**  
Russell Butterfield, MD, PhD

**11:30a – 12:00p** **Finding Resiliency**  
Brandon Kozar, PsyD, MBA

**12:00p – 12:15p** **Discussion and Open Q&A**

**12:15p – 1:15p** **Lunch: Finding Your People**  
Enjoy lunch with your subspecialist peers from across the country  
Gotham Foyer and surrounding rooms

**1:15p – 1:45p** **Intensive Care, Looking Back**  
Mary-Lou Weisman  
Gotham Ballroom

**1:45p – 3:30p** **Session III**  
**Caring for Our Carriers**  
Mena Scavina, DO  
Gotham Ballroom

**1:45p – 2:05p** **Impact as a Caregiver**  
Maria Jose Contreras, PhD

**2:05p – 2:15p** **Case Presentation of an Adult Carrier**  
Pradeep Mammen, MD

**2:15p – 2:25p** **Clinical and Research Experience in Carriers**  
May Ling Mah, MD

**2:25p – 3:00p** **Discussion: Care and Treatment of the Female Carrier**  
Pradeep Mammen, MD; May Ling Mah, MD;  
Alison Barnard, DPT, PhD; Teresa Wang, MD

**3:00p – 3:30p** **Break**

**3:30p – 5:00pm** **Session IV**  
**What's New in 2023**  
Jon Soslow, MD, MSCI  
Gotham Ballroom

3:30p – 3:50p	<b>Overview of ACTION/Duchenne</b> Chet Villa, MD
3:50p – 4:10p	<b>Learnings from Gene Therapy</b> Krista Vandenborne, PT, PhD
4:10p – 4:30p	<b>Imaging the Bone: What Lies Ahead</b> Leanne Ward, MD
4:30p – 5:00p	<b>Discussion and Open Q&amp;A</b>
<b>5:00p</b>	<b>Daily Recap &amp; Dismissal</b>
<b>6:00p – 7:00p</b>	<b>Welcome Cocktail Reception</b> Gotham Foyer
<b>7:00p – 8:30p</b>	<b>Dinner and Keynote Address</b> Carsten Bönemann, MD
<b>8:30p – 9:30p</b>	<b>Special Interest Group: Female Carriers of Duchenne</b> Mena Scavina, DO Central Park Room

## Thursday, January 19

<b>7:00a – 7:45a</b>	<b>Breakfast Session #1</b> <b>Combating Burnout, Compassion Fatigue, and Finding Purpose</b> Brandon Kozar, PsyD, MBA; Mosche Cohn, MD Union Square Park Room <b>OR</b>
<b>7:00a – 7:45a</b>	<b>Breakfast Session #2</b> <b>Battling Ego During Emergencies: How to Change the Culture</b> Sue Apkon, MD; Richard Shell, MD; Linda Cripe, MD Washington Square Park Room
<b>8:00a – 9:00a</b>	<b>Keynote Presentation: The Haves and The Have Nots: A Global Perspective on Health Equity</b> Tareq Albanai, MBA (United Nations) Gotham Ballroom
<b>9:00a – 9:15a</b>	<b>Late Breaking: Duchenne RUSP Nomination</b> Niki Armstrong, MS, CGC
<b>9:15a – 10:30a</b>	<b>Session V</b> <b>The Future of Access to Clinical Trials</b> Eric Camino, PhD Gotham Ballroom
9:15a – 9:35a	<b>Pushing Clinical Trials Forward: Challenges and Barriers</b> Sue Apkon, MD
9:35a – 9:55a	<b>A New Model: Building an Independent Trial Site Focused on Rare Diseases</b> Han Phan, MD

## Thursday, January 19 continued

9:55a – 10:15a	<b>Clinical Trial Management: Wins and Woes</b> Erica Goude, MS, CCRP
10:15a – 10:30a	<b>Panel Discussion and Q&amp;A</b>
<b>10:30a – 10:45a</b>	<b>Break</b>
<b>10:45a – 11:00a</b>	<b>PPMD Commercial Break: Decode Duchenne</b> Ann Martin, MS, CGC
<b>11:00a – 12:00p</b>	<b>Session VI</b> <b>Harnessing the Power of Data</b> Ryan Fischer Gotham Ballroom
11:00a – 11:15a	<b>Real-World Data in Duchenne</b> James Signorovitch, PhD
11:15a – 11:30a	<b>Data Standardization and Considerations on Regulatory Rigor</b> Romona Belfiore–Oshan, PhD
11:30a – 11:45a	<b>Interoperability and Opportunity: PPMD's EHR Extraction Initiative</b> Megan Freed, MPH
11:45a – 12:00p	<b>Discussion and Open Q&amp;A</b>
<b>12:00p – 1:00p</b>	<b>Lunch</b> Gotham Foyer and surrounding rooms
<b>12:00p – 1:00p</b>	<b>Working Lunch Special Interest Group: Duchenne Hearts</b> Deip Nandi, MD, MSc; Chet Villa, MD; Linda Cripe, MD Central Park Room
<b>1:00p – 2:30p</b>	<b>Session VII</b> <b>Ethical Considerations for Carrier Reproduction</b> Ann Martin, MS, CGC Gotham Ballroom
1:00p – 1:25p	<b>Setting the Stage: PPMD Experiences in Expanded Carrier Screening</b> Niki Armstrong, MS, CGC
1:25p – 1:50p	<b>Challenges in the Interpretation of Deletions And Duplications in DMD in ECS</b> Bradford Coffee, PhD
1:50p – 2:15p	<b>Ethical Considerations Around Reproduction</b> Brian Carter, MD
2:15p – 2:30p	<b>Discussion and Open Q&amp;A</b>
<b>2:30p – 3:00p</b>	<b>Change How You See, See How You Change</b> Rick Guidotti
<b>3:00p</b>	<b>Daily Recap and Dismissed for Evening/ Enjoy NYC</b>

## Friday, January 20

7:00a – 7:45a	<b>Breakfast Session #1</b> <b>Diversity, Equity, and Inclusion in Health Care</b> Tareq Albanai, MBA Union Square Park Room <b>OR</b>
7:00a – 7:45a	<b>Breakfast Session #2</b> <b>Partnering Together: Industry, CROs, and the Clinics</b> Joanne Donovan MD, PhD; Aravind Veerapandiyan, MD; Erica Goude, MS, CCRP Washington Square Park Room
8:00a – 9:15a	<b>Session VIII</b> <b>The Weight of Decision Making</b> Art Caplan, PhD Gotham Ballroom
8:00a – 8:20a	<b>Quality vs. Quantity: Navigating Invasive Interventions</b> Art Caplan, PhD
8:20a – 9:15a	<b>Panel Discussion</b> Angelena Edwards, MD; Kent Williams, MD; Courtney Gushue, DO; Deip Nandi, MD, MSc
9:15a – 10:20a	<b>Session IX</b> <b>Testosterone: More than Puberty</b> Rob Benjamin, MD Gotham Ballroom
9:15a – 9:30a	<b>Inducing Puberty: Who Decides?</b> Leanne Ward, MD
9:30a – 9:45a	<b>Testosterone and Impact on Bone</b> Kim Phung, MD
9:45a – 10:00a	<b>Navigating Sexuality and Gender</b> Deanna Adkins, MD
10:00a – 10:20a	<b>Discussion and Open Q&amp;A</b>
10:20a – 10:45a	<b>Break</b>
10:45a – 12:30p	<b>Session X</b> <b>The Truth About Individualized Medicine</b> Carsten Bönnemann, MD Gotham Ballroom
10:45a – 11:00a	<b>Federal Perspectives on Small Trials</b> Peter Pitts
11:00a – 11:15a	<b>Experiences in “N=Few” Trials</b> Kevin Flanigan, MD
11:15a – 11:30a	<b>Crowdfunding for a Cure: Ethical Considerations</b> Alison Bateman–House, MPH, PhD

## Friday, January 20 continued

11:30a – 12:30p	<b>Panel Discussion: Perspectives on Accessing Inaccessible Treatments</b> Kevin Flanigan, MD; Peter Pitts; Alison Bateman–House, MPH, PhD; JC Lopez, PhD
12:30p – 1:00p	<b>Moving Forward: Summary, Themes and Discussion</b> Peter Pitts; Tim Franson, MD
1:00p	<b>Closing Remarks and Dismissal</b> Pat Furlong