Thursday - BETTER

Making Life Better... Knowing more, being educated, knowing what to look for, what to ask, and where to go

12:00

Harborside Ballroom Welcome – Duchenne: Life Lessons on Taking the Sting of out the Diagnosis – Pat Furlong, PPMD President & CEO

12:40

Six Important Areas of Care
– James Dowling, MD, PhD

1:00

The Importance of Bone Health, Endocrine, Oral Health, GI, and GU – Garey Noritz, MD

1:20

Q&A

1:40

Report : Transition Care Meeting
– Kathi Kinnett, MSN, CNP

1:50

Parents and Clinicians Weigh In: Making the Clinical Trial Process Better – Holly Peay, MS

2:00

Encouraging Independence: A Panel Discussion

Moderator: Doug Biggar, MD

Panelists: Max Gravenstein, Patrick Denger, Ben Dupree, Tayius Surampudi, Jonathan Piacentino, Ben Cumbo

2:45

Q&A

3:00 BREAKOUTS

Breakout 1: Harborside Ballroom

Sex and Duchenne Evelyn Resh, CNM,

MPH

(This session is for parents only)

Breakout 2: Essex Rooms A-C

Behavior Issues in Duchenne

Jos Henricksen, PhD

Breakout 3: Kent Rooms A-C

Navigating Medicaid and Insurance Brian Denger

4:00 BREAKOUTS

Breakout 1: Harborside Ballroom

Adaptive

Technologies & IEPsJill Castle, M.Ed.

Breakout 2: Essex Rooms A-C

Home Remodeling
Scott Scherbarth

Breakout 3: Kent Rooms A-C

Marriage & Relationships Rachel Poysky James Poysky, PhD

Friday - FASTER

Moving Faster... Understanding research, advocacy and elements that will help move the field faster

7:30 BREAKFAST

8:00

Harborside Ballroom Better, Faster, Now: Our Lives with Duchenne – Ivy Scherbarth

8:20

Keynote Address: The Medicinal Value of Pleasure and Touch – Evelyn Resh, CNM, MPH

9:30

Better, Faster, Now: Research with a Mission – Sharon Hesterlee, PhD

10:10

Beyond 51: Rare Deletions, Duplications, and Multi-Exon Skipping

Moderator: Sharon Hesterlee, PhD

Panelists: Toshifumi Yokota, PhD; Steve Wilton, PhD; Adeline Vulin-Chaffiol, PhD

11:00

Q&A 11:20

Natural History and MRI as Biomarker
– Krista Vandenborne, PhD

11:40

Moving Research from Bench to Bedside: The Role of Research Review

This interactive mock review panel will provide and example of how Duchenne research is presented for review and offer insight and a behind the scenes look at the complexity of review.

Panelists: Sharon Hesterlee, PhD; Lee Sweeney, PhD; Bob McDonald, MD; Linda Cripe, MD; Annemieke Aartsma-Rus, PhD

12:30

A&D

12:40 LUNC

At today's lunch you are welcome to go and escape for a bit or join one of our discussion lunches. The four groups meeting will be:

Dads (Moderators: James Poysky, Chris Fanning) in the Laurel Room

Grandparents (Moderators: Gail McVicker, Anita Bullers) in the Kent Room

Moms (Moderators: Stefanie Killian, Debbie Dupree) in the Harborside Ballroom

Teens and Adults with Duchenne (Moderators: Pat Moeschen, Michael Gaglianone) in the Essex Room

1:40

Department of Defense Duchenne Funding – Marielena McGuire, PhD

Saturday - NOW

What is Now... Discussing what is happening now, and how it impacts the Duchenne community

7:30 BREAKFAST

8:00

Harborside Ballroom Mitchell's Journey and Our Mission – Chris Jones

8:30

Current Issues and Developments in Duchenne Cardiac Care

Moderator: Linda Cripe, MD

Panelists: Charles E. Canter, MD; Naomi Kertesz, MD; W. Reid Thompson, MD; David Morales, MD

9:45

Q&A

10:00

What to Know in an Emergency – Doug Biggar, MD

10:20

The Trouble with Oxygen
– Jonathan Finder, MD

10:40

Genetics and Proteomics: DuchenneConnect Registry, Biomarkers, Modifiers – Stan Nelson, MD, PhD

11:00

Q&A 11:10

Where Are We Now: Clinical Trial Updates Introduction – Sharon Hesterlee, PhD

11:20

Follistatin Gene Therapy – Jerry Mendell, MD

11:40

Reveragen (VBP-15)

– Eric Reeves, PhD

12:00

PTC Therapeutics (Ataluren)
- Robert Spiegel, MD

12:20

Results of REVERSE-DBMD (Revatio™) Study – Kathryn Wagner, MD, PhD

12:40

Q&A

1:00 LUNCH

At today's lunch we invite you to make it possible!

Join informal discussions around DuchenneConnect, community & fundraising events, taking action through advocacy, and other interactive ways to support the Duchenne community

2:10

Introduction of Clinical Trial Updates
Part II – Sharon Hesterlee, PhD

Thursday — BETTER

Harborside Ballroom Living Duchenne: Life Lessons for Every Age and Stage

(A panel discussion) This panel will discuss problems and practical solutions: Talking to babysitters, playdates, grocery store lines, PE teachers, nosy people, peers, bullies, etc.

Moderator: Pat Furlong

Panelists: Rachel Poysky, Ivy Scherbarth, Jill Castle, Ellen Wagner

Come listen and share your experiences and your solutions.

6:15 - 7:30

Meet and Greet

in the Harborside Ballroom Foyer

Join fellow guests and presenters in an informal reception with cocktails and conversation.

Friday – FASTER

Putting Patients First: The White Paper - Julianna Belelieu

2:20

Benefit/Risk: What We Have Learned – Holly Peay, MS; John Bridges, PhD

2:40

Advocacy: Our Weapon in the Fight to End Duchenne

- Rvan Fischer

3:00

FDA Perspective

- Robert Temple, MD, Deputy Director for Clinical Science

3:30

Q&A

4:00

Laying the Groundwork for GalNac Transferase Gene Therapy - Paul Martin, PhD

Biomarker Collaboration with Somalogic - Sally Nelson, PhD

4:40

Beyond Steroids: Blocking Inflammation in Duchenne

– Denis Guttridge, PhD

5:00

PPMD's Pre-Clinical Efforts and the Case for Tadalafil

- Lee Sweeney, PhD

5:20

30 Meters Matter™ : What Natural History Data is Showing Us, and Why it is Critical to Clinical Trials – Craig McDonald, MD (via Skype)

5:40

O&A

KIDS TRACK

9:00 - 12:30

Camp Friendship

Galena Room

This morning session is for all kids registered for Kids Track. There are three groups: ages 5-8, 9-12, and 13 & up. Qualified adult supervision is provided, as

2:30 - 5:00

Camp Friendship

Laurel Room

This afternoon session is designed for those diagnosed with Duchenne. During the afternoon, there will be fun, interactive discussions around friendship building skills. Plus other fun activities.

2:30 - 5:00

Duchenne SibShop

Kent Room

The Duchenne SibShop is a special session only for siblings who have brothers with Duchenne.

Host: Jill Castle, M.Ed.

This workshop will provide an opportunity for brothers and sisters to meet other siblings in a relaxed, recreational setting and discuss common joys and concerns.

Saturday - NOW

Prosensa

- Giles Champion, PhD

2:40

GSK (Drisapersen)

- Carlo Russo, MD

3:00

Sarepta (Eteplirsen)

- Chris Garabedian

3:20

Halo (HT-100)

– Diana Escolar, MD

3:40

Q&A

4:00

For DMD Study (Corticosteroids) – Kathryn Wagner, MD

4:20

Pfizer (Anti-GDF-8 Antibody)

- Carl Morris, PhD

4:40

Summit (SMT-C1100)

Jon Tinsley, MD

5:00

Lilly (Cialis™)

– Ron Victor, MD

5:20

Tivorsan (Biglycan)

- Joel Brausnstein, MD

Catabasis (CAT-1000)

- Michael Jirousek, PhD

6:00

Q&A

7:15 COCKTAILS

Harborside Ballroom Foyer

8:00 DINNER

Harborside Ballroom Guest Speaker

Mary-Lou Weisman, author of Intensive Care

Sunday

Physical Therapy is a very important part of Duchenne management and care. Come and listen to top Duchenne Physical Therapists, ask questions, and see stretching demonstrations.

9:00 – 10:30

Kent Room

PT Hands on Sessions – Helen Posselt, PT; Laura Case, PT, DPT, MS, PCS; Jennifer Hernandez, PT

10:30 - 12:00

Kent Room

PT Hands on Sessions - Helen Posselt, PT; Laura Case, PT, DPT, MS, PCS; Jennifer Hernandez, PT

SIDE MEETINGS: FRIDAY

6 PM – 10 PM

SAC meeting

(Closed meeting of the PPMD Scientific Advisory Committee)

SIDE MEETINGS: SATURDAY

8 AM – 11 AM

Drug Development Round Table (Closed meeting)