



## 5<sup>th</sup> International Conference on Cardiomyopathy in Children Virtual Meeting Agenda

March 5, 2021 (12:00-4:10 p.m.) – March 6, 2021 (9:00-2:15 p.m.)

**Times listed are Eastern Standard Time**

### **Friday, March 5, 2021**

**12:00 p.m. - 12:10 p.m.**

#### **Welcome**

Lisa Yue, Steven Lipshultz, MD, and Teresa Lee, MD

**12:10 p.m. - 1:25 p.m.**

#### **Dilated Cardiomyopathy**

Session Leaders: Steve Lipshultz, MD, Daphne Hsu, MD, and Paul Kantor MBBCh, MSc

12:10 p.m. - 12:15 p.m.

#### Risk Stratification and Treatment

Case presentation: 2-year-old presenting with newly diagnosed DCM in acute decompensated heart failure

- Molly Weisert

12:15 p.m. - 12:30 p.m.

#### Risk stratification & treatment

- Daphne Hsu: Evaluation and risk stratification
- Melanie Everitt: Acute medical management
- Robert Shaddy: Long term management with look to future

12:30 p.m. - 12:35 p.m.

#### Care Delivery and Research Studies

Case presentation: Research drug X is shown to be effective in the chronic care of adults with heart failure. How do we design a drug of efficacy in children?

- Induja Gajendran

12:35 p.m. - 12:50 p.m.

#### Challenges to Drug Studies in DCM

- Kimberly Lin: Study population, inclusion/exclusion criteria
- Bill Mahle: Challenges to study design: pharmacokinetics, study interventions (blood draws, advanced imaging, time for parents and children)
- Chris Almond: Statistical approaches to randomized, observational trials

12:50 p.m. - 1:05 p.m.	FDA perspective clinical trials in children with DCM <ul style="list-style-type: none"> <li>○ Lynne Yao</li> </ul>
1:05 p.m. - 1:25 p.m.	Panel discussion <ul style="list-style-type: none"> <li>○ Moderators: Daphne Hsu and Paul Kantor</li> <li>○ Panel: Melanie Everitt, Robert Shaddy, Kimberly Lin, Bill Mahle, Chris Almond, Lynne Yao</li> </ul>
<b>1:25 p.m. - 1:35 p.m.</b>	<b>Break</b>
<b>1:35 p.m. - 2:50 p.m.</b>	<b>Noncompaction Cardiomyopathy</b> Session Leaders: Melanie Everitt, MD, Brian Feingold, MD, and John Lynn Jefferies, MD, PhD
1:35 p.m. - 1:45 p.m.	<u>Phenotypes of Ventricular Noncompaction in Children and Adults</u> Spectrum of phenotypes of ventricular noncompaction in children <ul style="list-style-type: none"> <li>○ Jeff Towbin</li> </ul>
1:45 p.m. - 1:55 p.m.	Spectrum of phenotype of ventricular noncompaction in adults <ul style="list-style-type: none"> <li>○ Eloisa Arbustini</li> </ul>
1:55 p.m. - 2:10 p.m.	Panel discussion <ul style="list-style-type: none"> <li>○ Moderator: Melanie Everitt</li> <li>○ Panel: Jeff Towbin, Eloisa Arbustini</li> </ul>
2:10 p.m. - 2:20 p.m.	<u>Histopathologic and Imaging Features</u> Molecular and histopathology of ventricular noncompaction <ul style="list-style-type: none"> <li>○ Weinian Shou</li> </ul>
2:20 p.m. - 2:30 p.m.	The role of imaging in diagnosis and evaluation of noncompaction <ul style="list-style-type: none"> <li>○ Carolyn Taylor</li> </ul>
2:30 p.m. - 2:45 p.m.	Panel discussion <ul style="list-style-type: none"> <li>○ Moderator: John Lynn Jefferies</li> <li>○ Panel: Weinian Shou, Carolyn Taylor</li> </ul>
2:45 p.m. - 2:50 p.m.	Summary and wrap up <ul style="list-style-type: none"> <li>○ Brian Feingold</li> </ul>
<b>2:50 p.m. - 3:00 p.m.</b>	<b>Break</b>
<b>3:00 p.m. - 4:00 p.m.</b>	<b>Arrhythmogenic Cardiomyopathies</b> Session Leaders: Jeff Towbin, MD, and Teresa Lee, MD
3:00 p.m. - 3:05 p.m.	Case presentation: No Longer Just Arrhythmogenic Right Ventricular Cardiomyopathy <ul style="list-style-type: none"> <li>○ Teresa Lee</li> </ul>

- 3:05 p.m. - 3:15 p.m. Overview of Current Knowledge  
Clinical and genetic features of arrhythmogenic cardiomyopathy: heart failure perspective
  - Luisa Mestroni
- 3:15 p.m. - 3:25 p.m. Clinical and genetic features of arrhythmogenic cardiomyopathy: electrophysiology perspective
  - Dominic Abrams
- 3:25 p.m. - 3:35 p.m. Immune signaling and targeted therapy
  - Jeffrey Saffitz
- 3:35 p.m. - 4:00 p.m. Panel discussion
  - Moderator: Jeff Towbin
  - Panel: Luisa Mestroni, Dominic Abrams, Jeffrey Saffitz, Teresa Lee, Pamela Woodard, Joseph Rossano

**4:00 p.m. - 4:10 p.m. Next Steps & Meeting Adjourns for The Day**  
Teresa Lee, MD and Steven Lipshultz, MD

**Saturday, March 6, 2021**

**9:00 a.m. - 9:10 a.m. Welcome**  
Lisa Yue, Steven Lipshultz, MD, and Teresa Lee, MD

**9:10 a.m. - 10:30 a.m. Hypertrophic Cardiomyopathy**  
Session Leaders: Charlie Canter, MD, Steve Colan, MD, and Robert Weintraub, MB.BS

Diagnosis and Management  
9:10 a.m. - 9:20 a.m. Differences between non-sarcomeric versus sarcomeric HCM. Should they have their own management scheme
 

- Anne Murphy

9:20 a.m. - 9:30 a.m. Role of genetic testing in clinical management. Are P-G+ normal, diseased, or somewhere in between?
 

- Carolyn Ho

9:30 a.m. - 9:40 a.m. Optimal imaging strategy for pediatric HCM: echo, MRI, or a hybrid approach? What is the optimal evaluative and surveillance protocol for pediatric HCM, and the minimum dataset for clinical care
 

- Elfriede Pahl

Sudden Death Prevention  
9:40 a.m. - 9:50 a.m. What is the optimal management strategy to minimize sudden death in pediatric HCM
 

- Juan Kaski

9:50 a.m. - 10:00 a.m.	Pro pediatric HCM±ICD participation in competitive athletics <ul style="list-style-type: none"> <li>○ Jeffrey Kim</li> </ul>
10:00 a.m. - 10:10 a.m.	Con pediatric HCM±ICD participation in competitive athletics <ul style="list-style-type: none"> <li>○ George Van Hare</li> </ul>
10:10 a.m. - 10:30 a.m.	Panel Discussion <ul style="list-style-type: none"> <li>○ Moderators: Steve Colan and Charlie Canter</li> <li>○ Panel: Robert Weintraub, Anne Murphy, Carolyn Ho, Elfriede Pahl, Juan Kaski, Jeffrey Kim, George Van Hare</li> </ul>
<b>10:30 a.m. - 10:40 a.m.</b>	<b>Break</b>
<b>10:40 a.m. - 11:30 a.m.</b>	<b>Restrictive Cardiomyopathy</b> Session Leaders: Joseph Rossano, MD and Michael Burch, MB ChB, MD
10:40 a.m. - 11:00 a.m.	Overview of current knowledge <ul style="list-style-type: none"> <li>○ Susan Denfield</li> </ul>
11:00 a.m. - 11:30 a.m.	Panel discussion <ul style="list-style-type: none"> <li>○ Moderators: Mike Burch and Joe Rossano</li> <li>○ Panel: Susan Denfield, Jeff Towbin, Shelley Miyamoto, Yuk Law, Paul Kantor</li> </ul>
<b>11:30 a.m. - 11:40 a.m.</b>	<b>Break</b>
<b>11:40 a.m. - 12:50 a.m.</b>	<b>Genetics of Cardiomyopathy</b> Session Leaders: Wendy Chung, MD, PhD, Shelley Miyamoto, MD, and Stephanie Ware, MD, PhD
11:40 a.m. - 11:50 a.m.	<u>Genetic Causes and Diagnosis of Cardiomyopathy</u> Breaking news: Genetics of the primary cardiomyopathies <ul style="list-style-type: none"> <li>○ Stephanie Ware</li> </ul>
11:50 a.m. - 12:00 p.m.	Clinical utility of genetic testing in pediatric cardiomyopathy <ul style="list-style-type: none"> <li>○ Andrew Landstrom</li> </ul>
12:00 p.m. - 12:10 p.m.	Models of care and integrating genetic services into care of patients with cardiomyopathy <ul style="list-style-type: none"> <li>○ Erin Miller and Benjamin Helm</li> </ul>
12:10 p.m. - 12:20 p.m.	<u>Treatments for Genetic Subtypes of Cardiomyopathy</u> Update on gene-directed therapy for primary cardiomyopathies <ul style="list-style-type: none"> <li>○ Anjali Owens</li> </ul>
12:20 p.m. - 12:30 p.m.	New gene therapies under investigation <ul style="list-style-type: none"> <li>○ Elizabeth McNally</li> </ul>

12:30 p.m. - 12:50 p.m.

**Panel discussion**

- Moderators: Wendy Chung and Shelley Miyamoto
- Panel: Stephanie Ware, Anjali Owens, Elizabeth McNally, Erin Miller, Benjamin Helm, Andrew Landstrom

**12:50 p.m. - 1:00 p.m.**

**Break**

**1:00 p.m. - 2:05 p.m.**

**Future Directions for Pediatric Cardiomyopathy**

Session recaps and discussion: short and long term research and treatment priorities

- Moderator: Steve Colan
- Panel: session leaders and steering committee members

**2:05 p.m. - 2:15 p.m.**

**Meeting Adjourns**

Lisa Yue and Steven Lipshultz, MD

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