



MSMDS Conference Agenda

May 5 - 7th ♦ Boston, MA
The Simches Building ♦ 185 Cambridge St
Room: MGH-CRP 3110



Friday, May 5th, 2023

- 8:00AM: **Breakfast**
- 8:15AM: **Conference Welcome** [Warren Anderson, President YGSA]
 - Community Organization Overview
 - Family Introductions
 - Conference Goals
- 8:45AM: **Keynote Speaker – State of the Research**
 - Introduction to ACTA2/MSMDS Clinical & Translational Research [Dr. Patricia Musolino (MGH) & Dr. Mark Lindsay (MGH)]
- 9:30AM: **Break**
- 9:45AM: **MSMDS Fundamentals and Q&A: Bladder and Prune Belly Syndrome**
 - Family Story
 - Urology [Dr. Jack Elder (MGH)]
 - Nephrology [Dr. Weizhen Tan (MGH)]
 - Bladder Research [Dr. Rosalyn Adam (BCH)]
 - Breakout Q&A
- 11:15AM: **Break (Switch Rooms)**
- 11:30AM: **MSMDS Fundamentals and Q&A: Gastroenterology**
 - Family Story
 - Gastroenterology [Dr. Christopher Moran (MGH)]
 - Gut Motility and Smooth Muscle [Dr. Ahmed Rahman (MGH)]
 - Breakout Q&A
- 12:30PM: **Lunch**
- 1:30PM: **MSMDS Fundamentals and Q&A: Heart-Aorta and Lungs**
 - Family Story
 - Heart/Aorta & POTS [Dr. Mark Lindsay (MGH)]
 - Cardiovascular Surgery [Dr. Duke Cameron, (MGH)]
 - Lungs [Dr. Bernard Kinane (MGH)]
 - Breakout Q&A
- 3:30PM: **Break**
- 3:45PM: **MSMDS Fundamentals and Q&A: Rehabilitation**
 - Family Story
 - Rehabilitation and Exercise Management [Dr. Brian Wishart (Spaulding Rehab)]
 - Breakout Q&A
- 4:30PM: **Rare Disease Research Roadmap**
 - Treatment Development Pipeline in Rare Diseases [Dr. Rebecca Oberman, PhD (MGH)]
- 5:00PM: **Closing Remarks with Cocktail Hour and Invitation to Dinner**

Saturday, May 6th, 2023

- 8:00AM: **Breakfast**
- 8:30AM: **MSMDS Fundamentals and Q&A: Brain, Eye, and Dysautonomia**
 - Family Story
 - Brain Development & Dysautonomia [Dr. Patricia Musolino (MGH)]
 - Ophthalmology [Dr. Brian Gise (MEEI, BCH)]
 - Breakout Q&A
- 9:30AM: **Break**
- 9:45AM: **MSMDS Fundamentals and Q&A: Critical Care**
 - Family Story
 - Critical Care Kids/Adults [Dr. Ryan Carroll (MGH)]
 - Anesthesiology [Dr. Nicholas Houska (Colorado Children's Hospital)]
 - Role of Care Coordination in Critical Care [Dr. Jessica Schnell, Children's Wisconsin]
 - Breakout Q&A
- 11:00AM: **Patient Learning Academy** [June Ganley (Notre Dame Pedi-Pals), Jess Kennen (Notre Dame Pedi-Pals)]
 - Family Story
 - Coping Skills
 - Having a child with MSMDS
 - Helping Your Children Speak about MSMDS
 - Breakout Q&A
- 12:00PM: **Lunch**
- 1:00PM: **Patient-Centered Therapeutic Development in Rare Disease**
 - Dr. Casey McGuire (MGH)
- 2:00PM: **Monitoring & Management Recommendations Summary** [Dr. Patricia Musolino (MGH)]
- 3:00PM: **Break**
- 3:10PM: **ACTA2 Alliance & YGSA**
 - Accomplishments
 - Road Ahead
- 3:45PM: **Road to Diagnosis & Living with MSMDS**
 - Parent Panel
 - Patient Panel
 - Patient Videos
- 6:00PM: **Closing Remarks**

Sunday, May 7th, 2023

Family Event: TBD