



Sunday, June 27, 2021

Syndrome-Specific Sessions

MPS I: Hurler, Hurler-Scheie, Scheie

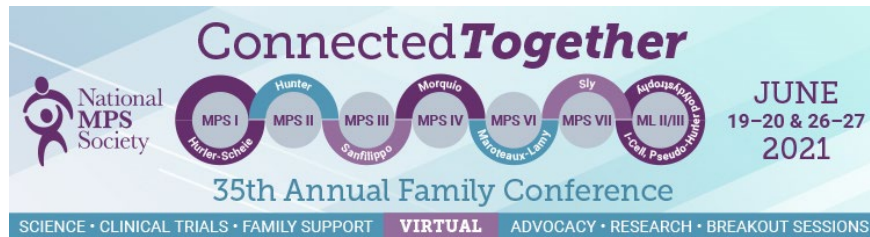
MPS II: Hunter

MPS III: Sanfilippo

MPS IV: Morquio

MPS VI/VII: Maroteaux-Lamy, Sly

ML II, II/III, III: I-Cell, Pseudo-Hurler Polydystrophy



Sunday, June 27: 12:00 p.m. – 3:00 p.m. EST

MPS I: Hurler, Hurler-Scheie, Scheie

Session begins in 6Connex/main conference

12:00 p.m. – 12:30 p.m. Our Impact: Fundraisers Make a Difference
Newman Family, MPS III
Bohley Family, MPS I
Fundraising Committee

12:30 p.m. – 1:00 p.m. Genetics and Family Planning
Barbara Burton, MD

Session transitions to Zoom platform

1:00 p.m. – 1:15 p.m. Break

1:00 p.m. – 1:20 p.m. Optional scientific session, in main conference platform on
 6Connex: Predicting Pathogenicity of IDUA and IDS Variants
Richard Steet, PhD

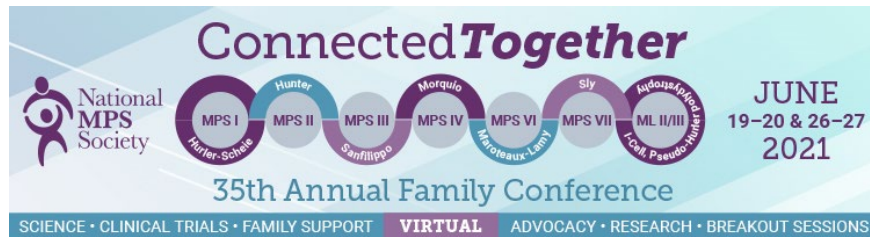
1:15 p.m. – 2:15 p.m. Breakout sessions, live on Zoom

- **MPS I (Hurler-Scheie; Scheie; non-transplant care)**
 - Moderators: Steve Holland, Samantha Slawson
 - Discussion topics: family and personal experiences with clinical trials, transitions to adulthood, unique considerations with attenuated diagnoses
- **MPS I (Hurler; transplant care)**
 - Moderators: Amber Mongan, Brooke Thomas
 - Discussion topics: family and personal experiences with HCT, post-transplant follow-up care, parenting, supporting siblings

Session transitions to 6Connex/main conference

2:20 p.m. – 3:00 p.m. Anti-inflammatory Biologics: experimental adjunctive treatments
 of MPS
Michelina Iacovino, PhD
Lynda Polgreen, MD, MS

3:00 p.m. Conference concludes



Sunday, June 27: 12:00 p.m. – 3:00 p.m. EST

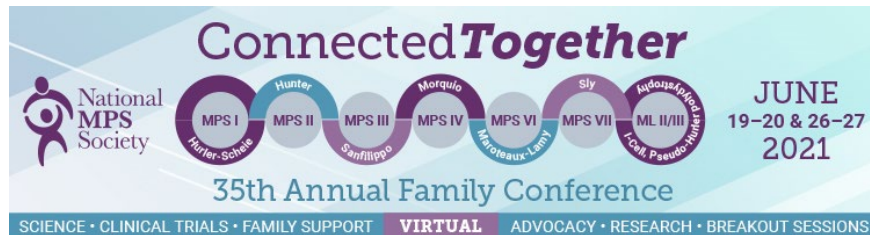
MPS II: Hunter

Session begins in 6Connex/main conference

- 12:00 p.m. – 12:30 p.m. Our Impact: Fundraisers Make a Difference
Newman Family, MPS III
Bohley Family, MPS I
Fundraising Committee
- 12:30 p.m. – 1:00 p.m. Genetics and Family Planning
Barbara Burton, MD
- 1:00 p.m. – 2:00 p.m. Scientific sessions
- 1:00 p.m. – 1:20 p.m. Predicting Pathogenicity of IDUA and IDS Variants
Richard Steet, PhD
 - 1:20 p.m. – 1:40 p.m. Classification of IDS gene variants using saturation
genome editing
Patricia Dickson, MD
 - 1:40 p.m. – 2:00 p.m. Q&A session, break

Session transitions to Zoom platform

- 2:00 p.m. – 3:00 p.m. Breakout session, live on Zoom**
- Moderators: Mike Hu, Avram Joseph
 - Discussion topics: review of current clinical trials,
understanding genetic variants, care management
- 3:00 p.m. Conference concludes



Sunday, June 27: 12:00 p.m. – 3:00 p.m. EST

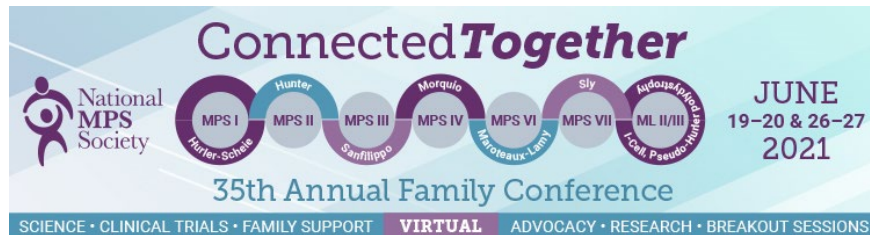
MPS III: Sanfilippo

Session begins in 6Connex/main conference

- 12:00 p.m. – 12:30 p.m. Our Impact: Fundraisers Make a Difference
Newman Family, MPS III
Bohley Family, MPS I
Fundraising Committee
- 12:30 p.m. – 1:00 p.m. Genetics and Family Planning
Barbara Burton, MD

Session transitions to Zoom platform

- 1:00 p.m. – 1:45 p.m. MPS III research updates, live presentation, and Q&A**
Kim Hemsley, PhD
- 1:45 p.m. – 3:00 p.m. Breakout session, live on Zoom**
- Moderators: Sasha and Jimmy Holder, Austin Noll
 - Discussion topics: review of current clinical trials, care management
- 2:20 p.m. – 3:00 p.m. Optional scientific session, in main conference platform on 6Connex: Anti-inflammatory Biologics: experimental adjunctive treatments of MPS
Michelina Iacovino, PhD
Lynda Polgreen, MD, MS
- 3:00 p.m. Conference concludes



Sunday, June 27: 12:00 p.m. – 3:00 p.m. EST

MPS IV: Morquio

Session begins in 6Connex/main conference

12:00 p.m. – 12:30 p.m. Our Impact: Fundraisers Make a Difference
Newman Family, MPS III
Bohley Family, MPS I
Fundraising Committee

12:30 p.m. – 1:00 p.m. Genetics and Family Planning
Barbara Burton, MD

Session will transition to Zoom

1:00 p.m. – 1:15 p.m. Break

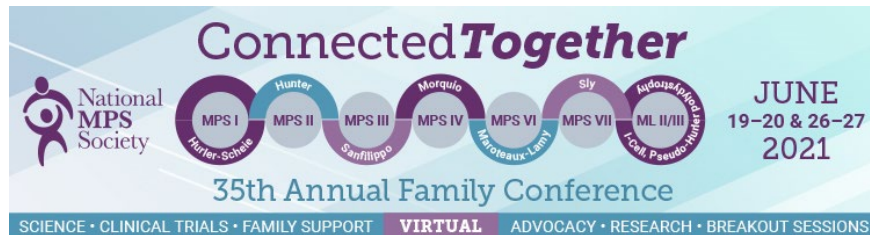
1:15 p.m. – 2:15 p.m. Breakout session, live on Zoom

- Moderators: Stephanie Bozarth, Chris Tippet
- Discussion topics: orthopedic interventions, care management, supporting siblings

Session transitions to 6Connex/main conference

2:20 p.m. – 3:00 p.m. Scientific session: Anti-inflammatory Biologics: experimental
 adjunctive treatments of MPS
Michelina Iacovino, PhD
Lynda Polgreen, MD, MS

3:00 p.m. Conference concludes



Sunday, June 27: 12:00 p.m. – 3:00 p.m. EST

MPS VI: Maroteaux-Lamy and VII: Sly

Session begins in 6Connex/main conference

12:00 p.m. – 12:30 p.m. Our Impact: Fundraisers Make a Difference
Newman Family, MPS III
Bohley Family, MPS I
Fundraising Committee

12:30 p.m. – 1:00 p.m. Genetics and Family Planning
Barbara Burton, MD

Session transitions to Zoom platform

1:00 p.m. – 1:15 p.m. Break

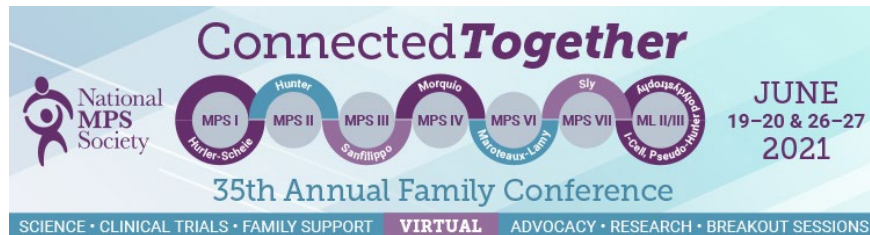
1:15 p.m. – 1:45 p.m. MPS VI/VII updates and care management presentation
Paul Harmatz, MD

1:45 p.m. – 3:00 p.m. Breakout session, live on Zoom

- Moderators: Kendra Gottsleben, Belinda Whorton
- Discussion topics: orthopedic and surgical interventions, enzyme replacement therapy, care management, transitions to adulthood

2:20 p.m. – 3:00 p.m. Optional scientific session, in main conference platform on 6Connex: Anti-inflammatory Biologics: experimental adjunctive treatments of MPS
Michelina Iacovino, PhD
Lynda Polgreen, MD, MS

3:00 p.m. Conference concludes



Sunday, June 27: 12:00 p.m. – 3:00 p.m. EST

ML: I-Cell, Pseudo-Hurler Polydystrophy

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Fundraising Committee

12:30 p.m. – 1:00 p.m. Genetics and Family Planning
Barbara Burton, MD

Session transitions to Zoom platform

1:00 p.m. – 2:00 p.m. ML updates and care management, live presentation, and Q&A
Jennifer Klein, MS

2:00 p.m. – 3:00 p.m. Breakout session, live on Zoom

- Moderators: Jennifer Klein, Terri Klein
- Discussion topics: research in ML, orthopedic and surgical interventions, care management

3:00 p.m. Conference concludes