



# 2024 CONFERENCE

Supporting the KDA Family & Bringing Hope for a Cure.

**NOVEMBER 2-4 (Sat. - Mon.)**

Orlando, FL  Rosen Plaza Hotel



[KENNEDYSDISEASE.ORG](http://KENNEDYSDISEASE.ORG)

- 8:45 am Registration/Coffee, Pastries and Fruit**
- 9:30 Welcome**  
KDA President
- 9:45 KDA Keynote Address**  
Ms. Susanne Waite, Founder and President Emeritus
- 10:00 The Ten Things I've Learned in My 25 Years with KD**  
Ed Meyertholen, PhD
- 10:30 The KD Family: Genetics, Testing, and the Implications**  
Alice Schindler, Lead Genetics Counselor, NIH/NINDS
- 10:55 Break**
- 11:15 What We Are Learning About Carriers**  
Abdullah AlQahtani, MD, MPH  
Johns Hopkins University
- 11:40 What's New in Mobility Devices?**  
Jim Kissling, President, AutoFarm Mobility  
Note: Mobility devices will be on display in Salon 4.
- 12:00 pm Lunch**
- 1:30 How to Increase the Impact of the KDA**  
Terry Thompson, KDA President  
Lew Myers, KDA Fundraising Advisor
- 1:45 KDA Umbrella Organizations: Value to KD Patients**  
Brian Lin, MDA,  
Alli Ward, NORD
- 2:10 An Emotional Roadmap for the Journey with Kennedy's Disease**  
Dr. John Weaver, Psy.D.  
Owner, Stress Management & Mental Health Clinics
- 3:00 Break**
- 3:30 Breakout Sessions (in-person only)**  
KD Men – Moderator: Jameson Parker  
KD Carriers – Moderator: Heather Montie  
KD Spouses/Caregivers - Moderator: JoDee Coulter
- 5:00 Conference Sessions End for the Day**
- 6:00 Reception and Beginning of Silent Auction**
- 7:00 Dinner (included with registration)**  
Ballroom D

7:30 am	<b>Breakfast Buffet</b>
8:20	<b>Welcome to Day 2 and Announcements</b> KDA President
8:30	<b>Tips and Tricks for KD Men, with Q&amp;A</b> Moderator: Jameson Parker, KDA Vice President KDA Board Members
9:00	<b>Tips from an Occupational Therapist</b> Kathleen Farrell, OTR/L, Occupational Therapist, NIH
9:25	<b>NIH Natural History Study: Results and Implications</b> Chris Grunseich, MD, NIH/NINDS
9:50	<b>Break</b>
10:20	<b>Advice from a KD Clinician, with Q&amp;A</b> Tahseen Mozaffar, MD, University of California, Irvine <i>With NIH clinicians Angela Kokkinis, Chris Grunseich, Abdullah AlQahtani</i>
11:00	<b>A Primer on KD Research</b> Ed Meyertholen, PhD
11:30 pm	<b>Lunch</b>
1:00	<b>Keynote Address for Research</b> Kurt Fischbeck, MD, NIH (emeritus) Al La Spada, MD, PhD, University of California, Irvine
1:20	<b>Unexpected Prevalence of KD Gene Mutation</b> Matteo Zanovello, University College, London
1:40	<b>Clinical Trials Update</b> <b>Overview:</b> Chris Grunseich, MD, NIH/NINDS Vissia Viglietta, MD, PhD, Nido Biosciences – <i>Nido-361 Update</i> Gianni Soraru, MD, University of Padua – <i>Update on Clenbuterol trial</i> Milan Barnes, PT, Kruthika Doreswamy, MPH, and Michael Sheehan, PT, NIH – <i>Use of wearable sensors in SBMA</i>
2:30	<b>Break</b>
3:00	<b>Reports from KDA Grant Recipients</b> <ul style="list-style-type: none"><li>• <b>Carlo Rinaldi</b>, MA, MD, PhD, Oxford University – <i>Harnessing polyQ AR transcriptional activity to advance a gene therapy strategy in the SBMA clinic</i></li><li>• <b>Shinichiro Yamada</b>, MD, PhD, Nagoya University – <i>Pathophysiology and development of symptomatic therapy for</i></li></ul>

*cold paresis in SBMA*

- **Alyson Sujkowski**, PhD, Wayne State University – *Using Drosophila exercise for therapeutic discovery in Kennedy’s Disease*
- **Kerri Schellenberg**, MD, MMedEd, University of Saskatchewan – *Clinical characteristics and prevalence of KD in the indigenous populations of western Canada*
- **Silvia Fenu**, MD, Carlo Besta Institute, Milan – *Connecting SBMA international registries/databases: a retrospective study*

**4:00**

**Research Summary and Q&A**, with senior researchers and lab heads

Moderator: Diane Merry, PhD, Thomas Jefferson University

**5:00**

**Conference Ends for the Day**

- 7:30 am** Breakfast Buffet  
Foyer
- 8:20** **Conference Updates and Introduction of Research Panels**  
KDA President  
Panel Coordinator: Andy Lieberman, MD, PhD, University of Michigan
- 8:30** **Panel 1: Disease Mechanisms**  
Moderator: Andy Lieberman, MD, PhD, University of Michigan
- Janghoo Lim, PhD, Yale University – *Protein Homeostasis in the Pathogenesis of SBMA*
  - David Luong, La Spada Lab, UC Irvine – *Delineating novel pathways of motor neuron demise in SBMA*
  - Diane Merry, PhD, Thomas Jefferson University, *Tissue-specific repeat instability in SBMA*
  - Caroline Rodrigues, PhD, Miranda Lab, Case Western Reserve University – *Stem cell modeling of SBMA*
  - Heather Montie, PhD, Philadelphia College of Osteopathic Medicine – *Does CD38 disrupt skeletal muscle function in KD?*
  - Laura Bott, PhD, Northwestern University – *Interindividual variation in protein misfolding disease: Lessons from the model organism C. elegans*
- 10:00** **Break**
- 10:30** **Panel 2: Translational Targets**  
Moderator: Diane Merry, PhD, Thomas Jefferson University
- Gianni Soraru, MD, University of Padua - *Hearing function in KD: a case control study from a tertiary referral center*
  - Caterina Marchioretta, PhD, University of Padua – *From molecular pathogenesis to pharmacological intervention targeting skeletal muscle*
  - Riccardo Cristofani, PhD, University of Milan – *Proteostasis of androgen receptor*
  - Ramesh Narayanan, PhD, University of Tennessee – *Update on the preclinical development of AR N-Terminus domain binding degrader ONCT-505 for the treatment of KD*
  - Agamjot Sangotra and Andy Lieberman, University of Michigan – *Harnessing protein quality control to degrade polyglutamine AR in SBMA*
  - Ambra Speciale, DPhil candidate, Oxford University – *RNA editing dysregulation in SBMA: molecular mechanisms and therapeutic implications*
  - Brad Turner, PhD, and Jibao Yuan, University of Melbourne, Florey Institute – *Developing a brain-penetrating antisense treatment approach for SBMA*
- 12:15 pm** **Conference Wrap-up**  
KDA President
- 12:30** **Conference Ends for All but Researchers**
- 1:00** **Researcher Luncheon and Discussion**
- 3:00** **End of KDA 2024 Conference**

