



# 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)

**Draft Agenda (9 October 2024)**

## Saturday, 2 November

- 9:00 am Registration/Coffee**
- 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 Shindler, 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**  
KD Men, Carriers, Spouses/Caregivers (In-person only)
- 5:00 Conference Sessions End for the Day**
- 6:00 Reception and Beginning of Silent Auction**
- 7:00 Dinner (included with registration)**  
Ballroom D
- 8:00 Live auction and completion of silent auction**

**Sunday, 3 November**  
*Note: Time Change to EST*

- 7:30 am Breakfast Buffet**
- 8:20 Welcome to Day 2 and Announcements**  
KDA President
- 8:30 Tips and Tricks for KD Men, with Q&A**  
Moderator: Jameson Parker, KDA Vice President  
KDA Board Members
- 9:00 Tips from an Occupational Therapist**  
Kathleen Farrell, OTR/L, Occupational Therapist, NIH
- 9:25 NIH Natural History Study: Results and Implications**  
Chris Grunseich, MD, NIH/NINDS
- 9:50 Break**
- 10:20 Advice from a KD Clinician, with Q&A**  
Tahseen Mozaffar, MD, University of California, Irvine  
*With NIH clinicians Angela Kokkinis, Chris Grunseich, Abdullah AlQahtani*
- 11:00 A Primer on KD Research**  
Ed Meyertholen, PhD
- 11:30 pm Lunch**
- 1: 00 Keynote Address for Research**  
Kurt Fischbeck, MD, NIH (emeritus)  
Al La Spada, MD, PhD, University of California, Irvine
- 1:20 Unexpected Prevalence of KD Gene Mutation**  
Matteo Zanovello, University College, London
- 1:40 Clinical Trials Update**  
**Overview:** Chris Grunseich, MD, NIH/NINDS  
Vissia Viglietta, MD, PhD, Nido Biosciences – *Nido-361 Update*  
Andy Chen, PhD, AnnJi Pharmaceutical Co.  
Gianni Soraru, MD, University of Padua – *Update on Clenbuterol trial*  
Milan Barnes, NIH – *Use of wearable sensors in SBMA*
- 2:30 Break**
- 3:00 Reports from KDA Grant Recipients**
- **Carlo Rinaldi**, MA, MD, PhD, Oxford University – *Harnessing polyQ AR transcriptional activity to advance a gene therapy strategy in the SBMA clinic*
  - **Shinichiro Yamada**, MD, PhD, Nagoya University – *Pathophysiology and development of symptomatic therapy for cold paresis in SBMA*
  - **Alyson Sujkowski**, PhD, Wayne State University – *Using drosophila exercise for therapeutic discovery in Kennedy's Disease*
  - **Kerri Schellenberg**, MD, University of Saskatchewan – *Clinical characteristics and prevalence of KD in the indigenous populations of western Canada*
  - **Silvia Fenu**, MD, Besta Institute – *Connecting SBMA national registries and databases*
- 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**

## Monday, 4 November

- 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, LaSpada Lab, UC Irvine – *Delineating novel pathways of motor neuron demise in SBMA*
  - Caterina Marchioretto, PhD, University of Padua – *From molecular pathogenesis to pharmacological intervention targeting skeletal muscle*
  - Diane Merry, PhD, Thomas Jefferson University
  - Helen Miranda, PhD, Case Western Reserve – *Stem cell modeling of SBMA*
  - Heather Montie, PhD, Philadelphia College of Osteopathic Medicine – *Does CD38 disrupt skeletal muscle function in KD?*
  - Gianni Soraru, MD, University of Padua - *Hearing function in KD: a case control study from a tertiary referral center*
- 10:15** Break
- 10:45** **Panel 2: Translational Targets**  
Moderator: Diane Merry, PhD, Thomas Jefferson University
- Riccardo Cristofani, PhD, University of Milan – *A new degradative pathway for AR: the role of chaperone-mediated autophagy*
  - 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, Oxford University – *RNA editing dysregulation in SBMA: molecular mechanisms and therapeutic implications*
  - Brad Turner, PhD, and Jack Juan, University of Melbourne, Florey Institute – *Developing a brain-penetrating antisense treatment approach for SBMA*
- 12:00 pm** **Conference Wrap-up, with Q&A**  
KDA President
- 12:30** **Conference Ends for All but Researchers**
- 1:00** **Researcher Luncheon and Discussion**
- 3:00** **End of KDA 2024 Conference**