



**2024 CONFERENCE**  
**NOVEMBER 2-4**

Orlando, FL



# THANKS TO OUR SPONSORS



John and Debby Lauber

Anonymous



## Saturday, 2 November

**8:45 am**      **Registration/Coffee, Pastries and Fruit**

**9:30**            **Welcome**  
KDA President

**9:45**            **KDA Keynote Address**  
Ms. Susanne Waite, Founder and President Emerita

**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

**8:00**            **Live auction and completion of silent auction**

## Sunday, 3 November

*Note: Time Change to Eastern Standard Time*

**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*  
[Gianni Soraru](#), MD, University of Padua –  
    *Update on Clenbuterol trial*  
[Milan Barnes](#), PT, NIH, [Kruthika Doreswamy](#), MPH, NIH,  
and [Michaele Sheehan](#), PT, 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, 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



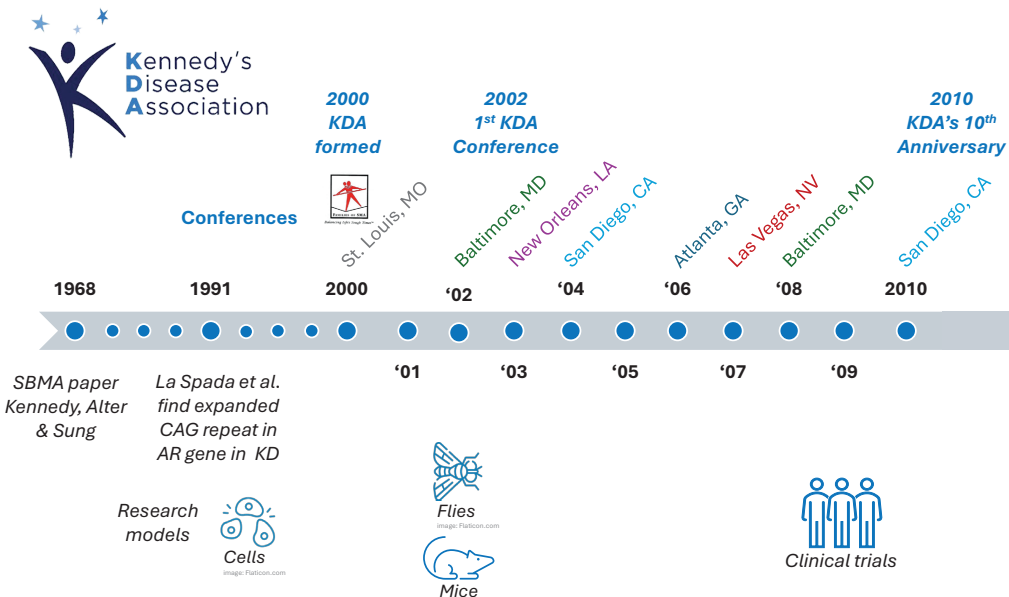
# Monday, 4 November

**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**, 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 Marchioretto**, 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

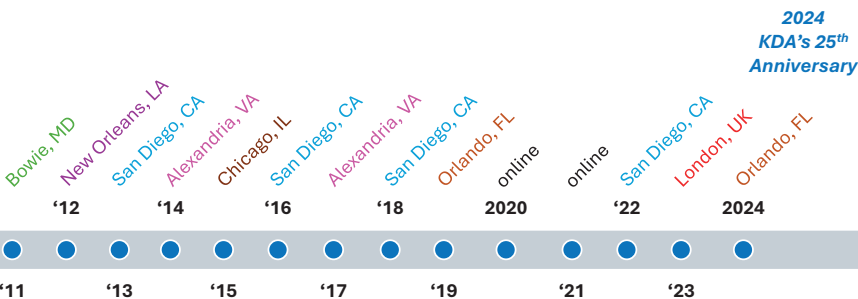
**Conference Ends for All but Researchers**

1:00

**Researcher Luncheon and Discussion**

3:00

**End of KDA 2024 Conference**





## THE KDA TEAM

### Board of Directors

Terry Thompson, President  
Jameson Parker, Vice President  
John Lauber, Treasurer  
Kathy Thompson, Secretary  
Ronald Moffett, Director  
Simon Hill, Director  
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Dale Traxler, Director  
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### Scientific Review Board

Andy Lieberman, MD, PhD, Chair  
Kurt Fischbeck, MD  
Al La Spada, MD, PhD  
Diane Merry, PhD  
Pietro Fratta, PhD  
Charlotte Sumner, MD  
Alexander McCampbell, PhD  
Christopher Grunseich, MD

### Retired Board members who continue to assist:

Ed Meyertholen, Patient Advocate to the Scientific Review Board



## The Kennedy's Disease Association

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