



THE GLOBAL KD FAMILY: TOGETHER AGAIN 2022 KDA CONFERENCE

Virtual and In-Person
San Diego, California | November 9-11, 2022

For more information go to: kennedysdisease.org



Note: All times are Pacific Time.

Wednesday, November 9	
From 7:15 am	Registration/Sign-in/Continental Breakfast
8:15 am	Welcome and Introductory Remarks Terry Thompson, PhD, KDA President
8:30	Patient Focused Drug Development (PFDD) Meeting Introduction <ul style="list-style-type: none"> • KDA Welcome and PFDD overview (Terry Thompson) • FDA Welcome and Opening Remarks (Michelle Campbell, PhD) (via Zoom) • Clinical Overview of Kennedy's Disease (Christopher Grunseich, MD, National Institutes of Health)
9:00	Panel 1: Health Effects and Daily Impacts of Kennedy's Disease Moderator/Discussant: Brian Ahangar, MD Panelists: Fred, Micah, Scott, James Zoom: Maria, George, Lou, Randy, Tim Open discussion
10:30	Break
11:00	Panel 2: Patient Perspectives on Potential Treatments Moderator/Discussant: Ed Meyertholen, PhD Panelists: Lew, Chris, Mike Zoom: Andrew, Jurgen, Dan Open discussion
12:30 pm	Lunch
2:00	NIH Quality of Life Study Abdullah Al Qahtani, MD, MPH, National Institutes of Health (NIH)
2:15	Caregivers: The Unsung Heroes of KD Jason Resendez, President and CEO, National Alliance for Caregiving (via Zoom)
2:30	Conference Participant Introductions and General Discussion
3:00	Break
3:15	Breakout sessions for KD men, women carriers, and spouses/caregivers Note: Breakout sessions will be for in-person attendees only

Thursday, November 10

8:15 am	Conference Updates and Plan for the Day Terry Thompson
8:30	The Biology of Kennedy's Disease Ed Meyertholen
9:10	Exercises to Improve Speech and Swallowing Beth Solomon, MS, CCC-SP, National Institutes of Health (via Zoom)
9:40	Exercise, KD, and You Joe Shrader, PT, CPed, National Institutes of Health (Via Zoom)
10:00	Break
10:30	The French National Protocol for Kennedy's Disease Pierre François Pradat, MD, PhD, Assistance Publique – Hôpitaux de Paris, Sorbonne University (via Zoom)
11:00	Managing KD: A Roundtable and Q&A with Clinicians Chair: Christopher Grunseich, MD, National Institutes of Health Carlo Rinaldi, MD, PhD, Oxford University Tahseen Mozaffar, MD, University of California Irvine Health (via Zoom) John Ravits, MD, University of San Diego Health Brian Ahangar, MD, Kaiser Permanente Pierre François Pradat, MD, PhD, Assistance Publique – Hôpitaux de Paris, Sorbonne University (via Zoom) Beth Solomon, National Institutes of Health (via Zoom) Joe Shrader, National Institutes of Health (via Zoom)
12:00 pm	Lunch
1:30	KDA Registry: Update and Analytics Alyssa Mendel, Research Project Manager, Center of Rare Diseases Sanford (CoRDS) (via Zoom)
1:40	Introduction to Research Agenda Terry Thompson
1:45	Presentations by 2020 KDA Grant Recipients Gerry Pfeffer, MD, PhD, University of Calgary Antonella Falconieri, PhD, University of Padova (via Zoom) Manuela Basso, PhD, University of Trento (via Zoom) Mariarita Galbiati, PhD, University of Milan (via Zoom)
2:25	Announcement of 2022 KDA Grants and Waite-Griffin Fellowship Awards
2:30	Break
3:00	Panel: Clinical Trials and Trends Chair: Andy Lieberman, MD, PhD, University of Michigan <ul style="list-style-type: none"> • <i>An AAV-miRNA for Androgen Receptor Knockdown in Spinal and Bulbar Muscular Atrophy</i> Eileen Workman, PhD, University of Pennsylvania • <i>Progress Toward a Disease Modifying Therapy for SBMA</i> Jeremy Springhorn, PhD, Nido Biosciences • <i>A Phase 1/2a First In-Patient Clinical Trial of AJ201 for Treatment of Kennedy's Disease</i> Yumo Michael Chan, PhD, AnnJi Pharmaceuticals
4:00	General Discussion and Q&A Kenneth Fischbeck, MD, National Institutes of Health
4:30	Set up for Silent Auction and Dinner
6:00 pm	Reception and Beginning of Silent Auction
7:00	Dinner (included with registration)
8:00	Live Auction and Completion of Silent Auction <i>Mingling with old and new friends</i>

Friday, November 11

8:15 am	Conference Updates and Introduction of Research Panels Terry Thompson										
8:30	Panel: Disease Mechanisms Chair: Kenneth Fischbeck, MD, National Institutes of Health <ul style="list-style-type: none"> • <i>Cytotoxicity of ligand-free mitochondria-localized mutant AR</i> Xia Feng, PhD, Massachusetts General Hospital • <i>Mutant ARpolyQ during motoneuron development</i> Angelo Poletti, PhD, University of Milan (via Zoom) • <i>USP7 and AR post-translational modifications – a novel mechanism and potential therapeutic target in SBMA</i> Diane Merry, PhD, Jefferson University • <i>Identification of kinases and phosphatases targeting polyQ-expanded AR as disease modifiers</i> Maria Pennuto, PhD, University of Padova, (via Zoom) • <i>Neuromuscular junction pathology is correlated with differential motor unit vulnerability in spinal and bulbar muscular atrophy</i> Elana Molotsky, PhD Candidate, Jefferson University • <i>Neuromuscular junction dysfunction in an in-vitro human model of SBMA</i> Helen Miranda, PhD, Case Western Reserve • <i>La Spada lab update</i> Anastasia Gromova, PhD, University of California, Irvine 										
9:50	Break										
10:20	Panel: Emerging Therapeutic Targets Chair: Diane Merry, PhD, Jefferson University <ul style="list-style-type: none"> • <i>Novel AR N-Terminus-binding Degradors</i> Ramesh Narayanan, PhD, University of Tennessee • <i>CNS penetrating peptide-ASOs</i> Brad Turner, PhD, University of Melbourne • <i>Can SIRT3 fix metabolic problems in KD muscle?</i> Heather Montie, PhD, Philadelphia College of Osteopathic Medicine • <i>Targeting muscle to prevent neuromuscular degeneration in SBMA</i> Andy Lieberman, MD, PhD, University of Michigan • <i>AR transcriptional activity: from mechanism to therapeutic target</i> Carlo Rinaldi, MD, PhD, Oxford University • <i>AI-based drug screening for SBMA</i> Masahisa Katsuno, MD, PhD, Nagoya University (via Zoom) 										
11:30	Looking Ahead: Summary of Trends in KD Research, Research Q&A with Lab Directors Chair: Kenneth Fischbeck, MD, National Institutes of Health <table style="width: 100%; border: none;"> <tr> <td style="width: 50%;">Andy Lieberman, MD, PhD</td> <td style="width: 50%;">Diane Merry, PhD</td> </tr> <tr> <td>Al La Spada, MD, PhD</td> <td>Carlo Rinaldi, MD, PhD</td> </tr> <tr> <td>Maria Pennuto, PhD (via Zoom)</td> <td>Masahisa Katsuno, MD, PhD (via Zoom)</td> </tr> <tr> <td>Heather Montie, PhD</td> <td>Brad Turner, PhD</td> </tr> <tr> <td>Helen Miranda, PhD</td> <td></td> </tr> </table>	Andy Lieberman, MD, PhD	Diane Merry, PhD	Al La Spada, MD, PhD	Carlo Rinaldi, MD, PhD	Maria Pennuto, PhD (via Zoom)	Masahisa Katsuno, MD, PhD (via Zoom)	Heather Montie, PhD	Brad Turner, PhD	Helen Miranda, PhD	
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12:30	Conference Wrap-up Dr. Fischbeck, Terry Thompson										
Afternoon	Researchers' Luncheon and Discussion Harborview Room, Building A, 5th Floor										