Kennedy's Disease ASSOCIATION

OUR MISSION

inform, support, educate,
fund research, and find a cure
for Kennedy's Disease

KDA News

KDA Conference 2020
Goes Virtual!

In the face of the global pandemic, the KDA has decided to move this year's 2020 KDA conference ONLINE!

Tentative date: Middle or late October 2020.
Tentative format: A live, three day Zoom meeting.

Why this change? Several factors have gone into this decision, including: the difficulties of long distance travel, the many requirements to safely engage in social distancing, the uncertainty of the level of infection a few months from now and the KD community's vulnerability to the Covid-19 virus.

What you can count on? As with past conferences, we are working hard to ensure that this conference is an excellent opportunity to meet others who are impacted by KD. We will learn from each other, lean on each other and share our collective struggles. You can also count on engagement with researchers and medical professionals who provide updates on the latest research and information tailored specifically for KD patients and caregivers.

These are challenging times. And though we may have to stay home for this conference, we can still connect and continue the fight for a cure!

More details coming soon!

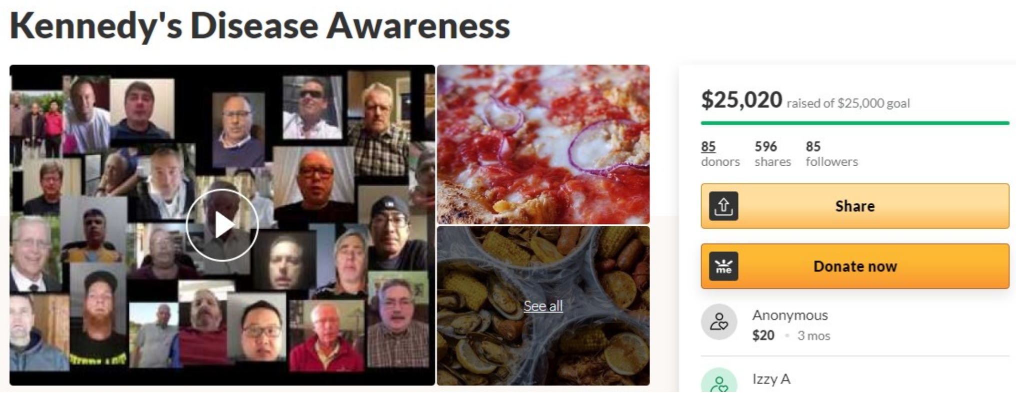
Thank you to Louise Goforth

who is retiring from the KDA board. For several years now, Louise has lead our fundraising efforts and with her leadership has brought our ability to fund research amazing new heights. Multiple $50,000 grants given to researchers can be attributed directly to her efforts to mobilize the KD community. Fortunately, she will continue to work doing the Texas Golf Scramble which has been the biggest fundraising effort in the history of the KDA. Louise, your steady leadership, positive outlook and joyful disposition will be missed on the board of directors. On behalf of the entire KD community, we thank you.

The "Go Fund Me " Master!

Huge shoutout to Ralph Briones, who recently completed a $25,000 fundraiser for the KDA. We encourage all KDA members to check out Ralph's Gofundme page to see the difference that one person can make.

Ralph, you engaged the KDA community and your efforts created an amazing campaign to raise awareness about Kennedy's Disease. Your work to help find a cure is an inspiration to us all. Thank you!


Kennedy's Disease Awareness

$25,020 raised of $25,000 goal

85 donors 596 shares 85 followers

Anonymous
$20 · 3 mos

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[Visit the new and improved KDA website!]

Research Updates

Updates and summaries provided by Todd Allen - KDA Board Member.

Arginine is a disease modifier for polyQ disease models that stabilizes polyQ protein conformation
In this study, we identified arginine as a potent polyQ aggregation inhibitor that acts by inhibiting the formation of misfolded and oligomeric toxic protein species before the formation of insoluble aggregates. We also confirmed its therapeutic effects on neurological symptoms and protein aggregation pathology using two different animal models of polyQ diseases. In addition, we showed that arginine may exert a therapeutic effect on the dendritic arborization of the Purkinje cell in the cerebellum.

Muscle BDNF: A Potential Therapeutic Target for Kennedy’s Disease
Novel to this study, the authors developed a 97Q mouse with Cre-dependent overexpression of BDNF (97Q/BDNF) specifically in muscle. This resulted in significant increases in muscle-specific BDNF expression by 150-fold in the fast-twitch tibialis anterior (TA) and 45-fold in the slow-twitch soleus. The hang test, used to determine disease progression, demonstrates neuromuscular impairment and motor coordination in mouse models by measuring how long a mouse can hold their body weight using an overhanging bar. In the current study, disease onset was defined as a hang time < 120 seconds for two consecutive days and a hang-time < 30 seconds represented disease end-stage. Overexpression of BDNF was found to significantly increase time to disease onset, end stage and further, doubled survival time.

Cell-Clearing Systems Bridging Repeat Expansion Proteotoxicity and Neuromuscular Junction Alterations in ALS and SBMA
The coordinated activities of autophagy and the ubiquitin proteasome system (UPS) are key to preventing the aggregation and toxicity of misfold-prone proteins which manifest in a number of neurodegenerative disorders. These include proteins which are encoded by genes containing nucleotide repeat expansions. In the present review we focus on the overlapping role of autophagy and the UPS in repeat expansion proteotoxicity associated with chromosome 9 open reading

frame 72 (C9ORF72) and androgen receptor (AR) genes, which are implicated in two motor neuron disorders, amyotrophic lateral sclerosis (ALS) and spinal-bulbar muscular atrophy (SBMA), respectively. At baseline, both C9ORF72 and AR regulate autophagy, while their aberrantly-expanded isoforms may lead to a failure in both autophagy and the UPS, further promoting protein aggregation and toxicity within motor neurons and skeletal muscles. Besides proteotoxicity, autophagy and UPS alterations are also implicated in neuromuscular junction (NMJ) alterations, which occur early in both ALS and SBMA. In fact, autophagy and the UPS intermingle with endocytic/secretory pathways to regulate axonal homeostasis and neurotransmission by interacting with key proteins which operate at the NMJ, such as agrin, acetylcholine receptors (AChRs), and adrenergic beta2 receptors (B2-ARs). Thus, alterations of autophagy and the UPS configure as a common hallmark in both ALS and SBMA disease progression.

Other News

Power of CRISPR

One of the inventors of CRISPR is hopeful that in the near future, this technology can help people like those impacted by Kennedy's Disease!

To learn more, listen to a recent Clear and Vivid podcast, hosted by Alan Alda. In this episode, the co-inventor of the powerful gene editing tool called CRISPR, Jennifer Doudna describes for Alan her hopes for new treatments and possibly even cures for diseases that are today incurable.

Do you have a message to share with us? Let me know it! jameson411@gmail.com

KDA Memorial Page

If you have a loved one with Kennedy's Disease who has passed away and would like to have them added to the KDA memorial page, let us know at Contact the KDA with the information and a picture.

Click here to see the KDA Memorial Page

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KDA - Donation

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