Kennedy's
Disease
Association

2022 ANNUAL REPORT

The Kennedy's Disease Association
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The Kennedy's Disease Association is an all volunteer, non-profit corporation

The people who make the KDA possible are all people impacted by Kennedy's Disease. We are men with KD, carriers of KD, caregivers, parents and children, family and friends. We give our time and talents to help fulfill the mission of the KDA - to inform, support, educate, fund research, and find a treatment and cure for Kennedy's Disease.

The Mission of the Kennedy's Disease Association

- Raise funds with a target of contributing at least 90% of every dollar spent for Kennedy's Disease research and education.
- Share information about Kennedy's Disease with those who seek it.
- Create a support system for those living with the disease.
- Increase public awareness of Kennedy's Disease and its effect upon families.
- Increase awareness of Kennedy's Disease in the medical community.

A Message from the President

2022 was a busy – and successful – year for KDA. Our association continues to grow and evolve, enhancing our relationships with the SBMA research community and with our counterpart patient advocacy groups in several countries. Here are the highlights:

- We held our first-ever hybrid conference and first in-person conference since 2019. "KDA 2022," was based on the theme "The Global KD Family: Together Again." Over 350 people registered for the conference, mostly on Zoom but with a significant presence in San Diego, especially by the KD research community.
- We learned about AnnJi's Phase 1/2a clinical trial of AJ201, a curcumin-based therapy designed to enhance degradation of mutant Androgen Receptor (AR) genes, reduce cellular oxidative stress, and suppress inflammation. The trial will take place in 2023 at six clinical sites in the United States. We also heard about two other research efforts that may lead to clinical trials.
- We conducted a Patient-Focused Drug Development (PFDD) meeting at KDA 2022. Two panels of KD men discussed their lived experiences with KD and things they are doing to manage the disease until effective treatments can be developed. Patient testimony from the PFDD meeting will be documented in a "Voice of the Patient" report to be used by the Food and Drug Administration (FDA) in their evaluation of clinical trial results.

- We experienced exceptional growth in the CoRDS patient registry that was launched in June 2021 in response to patient and researcher requests discussed at the 2020 virtual KDA conference. The number of registrants in CoRDS increased from 261 in December 2021 to 356 in December 2022. The current registry includes people from 41 U.S. states and 22 countries, with 91% KD men and 9% female carriers. We know that KD researchers are using the registry: NIH used CoRDS to recruit patients for their Quality of Life study, and AnnJi is using CoRDS to recruit people for the clinical trial of AJ201.
- We initiated the Waite-Griffin SBMA Fellowship program to serve as an incentive for junior researchers at the post-doctoral level. Recipients agree to devote at least 70% of their time to SBMA research for the period of their fellowship. We awarded two fellowships for $75,000 each at KDA 2022.
- We also refreshed the Scientific Review Board (SRB), the group of senior researchers and clinicians who review and evaluate submissions for KDA's annual grants program. Leadership of the SRB transitioned from Dr. Kenneth Fischbeck of the NIH to Dr. Andy Lieberman from the University of Michigan Medical School.
- We benefited from three highly successful fundraising events: the Bay Area Rare Disease Day event, the Texas Golf Scramble, and the Great Road Trip in partnership with KD-UK. More than $162,000 from these three events alone is helping to fund global research into Kennedy's disease.
- We supported the Carriers group in their monthly meetings under the leadership of Joan Sorensen and Dr. Heather Montie.
- We strengthened relationships with other patient advocacy groups including KD-UK, AIMAK, KD-Downunder, SBMA Japan, and SBMA Russia. Information sharing and collaboration among these groups continues to improve, mirroring the exceptional collaboration among scientists in the global Kennedy's disease research community.

In 2023, we will continue the momentum in building our membership, increasing our fundraising, and supporting research. We also expect announcements of more clinical trials as research moves closer to the clinic in developing effective treatments for KD. For more about our plans, please see our 2023 Goals below.

Looking Toward the Future

The KDA's Board of Directors has approved the following goals for 2023.

We will continue to:

- Raise money to give to research and support our cause.
    - This year we set a goal of $200,000 for this purpose.
- Include one or more Fellowships in the 2023 Grants Program.
- Collaborate with KD/UK on a joint KDA-KD/UK conference and educational symposium in Fall 2023.
- Support the efforts of the NIH and other research labs that are dedicated to understanding KD and finding a cure or treatment for KD.
- Publish quarterly KDA email updates on what is happening in the world of Kennedy's Disease. This includes information about fundraisers, research, the annual conference, and other information that supports the KD community.
- Build a new KDA website and continue to provide updates about research and clinical trials, and emphasize the 'KDA Family' theme.
- Support clinical trials by maintaining relationships with pharmaceutical companies and sharing information with the KD community.

In addition, we will:

- Seek new sources of funding, including but not limited to foundations aimed at helping people with rare diseases.
- Expand the talent pool of the KDA volunteers (board members and/or leaders) to include people who can broaden the social media reach of the KDA and expand KDA's fundraising efforts.
- Expand on the successful Carrier Group Zoom sessions to include sessions for KD Men and Caregivers.
- Continue and expand our working relationships with counterpart advocacy groups in the UK, Australia, Italy, Japan, and other countries.

2022 KDA Awards, Grants and Fellowships

In 2022, the KDA awarded two Waite-Griffin SBMA Fellowships, named in honor of the people who founded the KDA. This is a one-year stipend of $75,000 for a junior PhD or MD interested in a career in SBMA research and/or patient care. An additional $2,000 is provided for travel and/or educational expenses. The awardee is expected to devote at least 70% of their time to SBMA research. The fellowship may be extended with demonstrated progress in SBMA research.

Following is a summary of the 2022 Waite-Griffin Fellowships

Abdullah AlQahtani, MD, MPH
Senior Clinical Research Fellow, NGB, NINDS
“Developing an imaging biomarker in spinal and bulbar muscular atrophy (SBMA)”

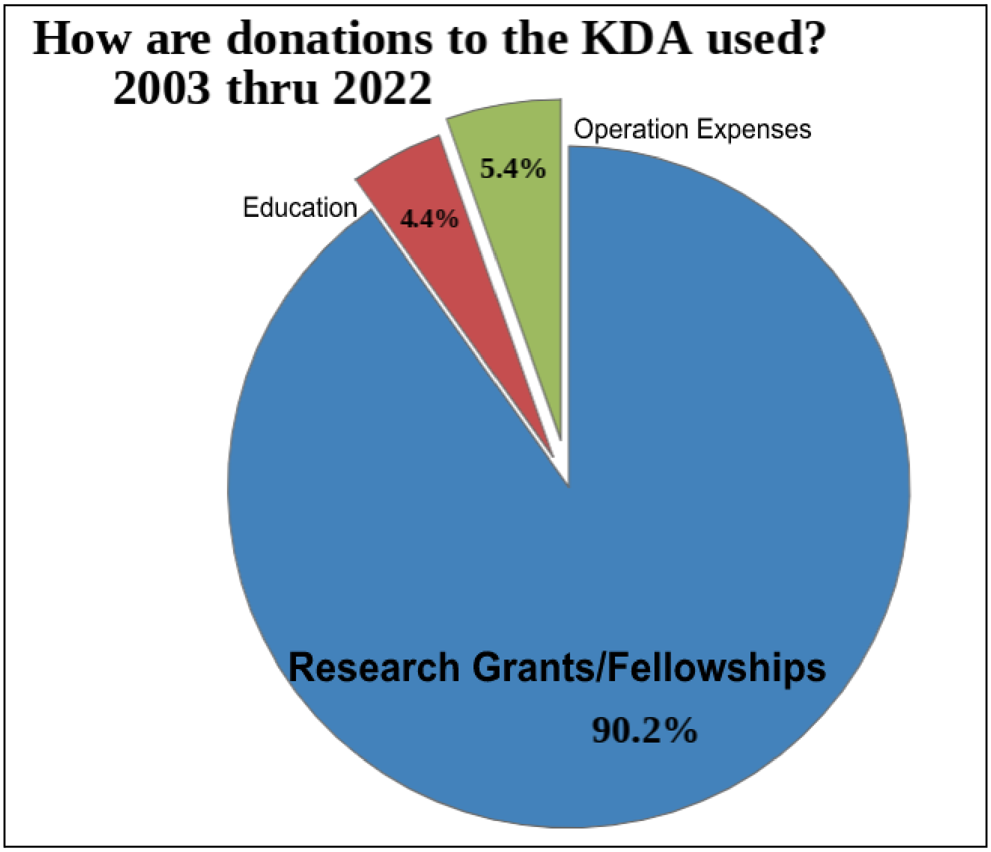
Candidate therapeutic strategies have been tested in preclinical models of SBMA, and potential disease-modifying therapies will soon be evaluated in future clinical trials. However, without appropriate biomarkers to assess disease progression reliably over a short period of time, studies would require a large sample size and long duration to evaluate efficacy. The goal of this study is to identify neuromuscular ultrasound (NMU) parameters that serve as sensitive, reproducible, and cost-effective disease biomarkers for evaluating efficacy in future therapeutic studies in spinal and bulbar muscular atrophy (SBMA). In addition, the project will develop an ultrasound protocol to be used by neuromuscular specialists in clinical practice to improve diagnostic accuracy and result in an earlier diagnosis.

Alyson Sujkowski, PhD
“Using Drosophila exercise for therapeutic discovery in Kennedy’s Disease”

Endurance exercise is a potent intervention with widespread benefits proven to reduce disease incidence across species. While endurance exercise supports neural plasticity, enhanced memory, and reduced neurodegeneration, less is known about the effect of chronic exercise on the progression of movement disorders like Kennedy’s Disease (KD). Initial work has revealed protection of speed and maximal lifespan in exercised KD model flies. Based on these preliminary observations, there are two specific aims. First, evaluate the role of endurance exercise on a broad spectrum of KD phenotypes and second, determine molecular mechanisms of protection from exercise that may be leveraged in the future toward the development of targeted therapeutics.

Here is a link to find out more or to see previous years grant recipients.
https://kennedysdisease.org/research/research-grants.

As of November 2022, the KDA has awarded $2,016,617 in research grants and fellowships to help find a cure or treatment for Kennedy's Disease.



Over the course of the last 18 years, over 90¢ of every dollar received by the KDA is spent on Kennedy’s Disease research and education. This is only possible because the KDA is an all-volunteer organization, including the board of directors and everyone who engages in fundraising and our day-to-day operations.

2022 Statement of Activities

Income	2022 $ (U.S.)	2021 $ (U.S.)
Conference	-4,172	11,065
Donations - Corporate and Matching	200	2,609
Facebook	13,629	16,698
General	191,406	204,974
In Memory/Honor	17,215	22,530
Proceed Partners	1,949	1,965
Misc. Fundraisers	-	3,915
The Great Road Trip	35,523	50
Dim Sum - Give Sum	100	5,000
Rare Disease Day - Bay Area (fundraiser)	22,422	20,554
Golf Tournament - Texas (fundraiser)	40,210	2,361
Tacos and Tussles	1,195	-
Gift of Stock	-	2,267
Sales	575	140
Total Income	320,252	294,128

Charitable Operations/Expenditures	2022 $ (U.S.)	2021 $ (U.S.)
Research Grants/Fellowships	154,000	175,000
Education	7,737	2,265
Total Charitable Expenses	161,737	177,265

Expenses	2022 $ (U.S.)	2021 $ (U.S.)
Bank and Credit Card Fees	3,832	2,724
Insurance/Licensing/Membership	2,001	2,041
Web and Web hosting	1,832	1,475
Total Office (Phone/Postage/Equip and Software)	645	737
Total Expenses	8,455	6,977

Gain/(Loss)	**150,012**	**109,886**

Complete financial statements are available upon request from the KDA

2022 KDA Officers and Board of Directors

- Terry Thompson, President
- Jameson Parker, Vice President
- David Yelton, Treasurer
- Simon Hill, Secretary
- Ron Moffett, Director
- Steven Rittmaster, Director
- Kathy Thompson, Director
- Dale Traxler, Director

Retired Board members who continue to assist:
Ed Meyertholen, Patient Advocate to the Scientific Review Board