



2021 ANNUAL REPORT

The Kennedy's Disease Association

P.O. Box 1105

Coarsegold, CA 93614

Telephone: 1-855-KD-ASSOC

(855) 532-7762 or (734) 288-5580

Email: info@kennedysdisease.org

Web Site: www.kennedysdisease.org

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 include men with KD, carriers of KD, caregivers, parents and children, family and friends. We all 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 Board of Directors

As the global pandemic of Covid-19 marched on, so did the KDA. We continue to work to support those impacted by Kennedy's Disease as well as raise funds to support research and increase awareness of Spinal Bulbar Muscular Atrophy.

Due to the ongoing Covid-19 pandemic, the KDA opted to hold its annual conference entirely virtual for the second year in a row. We also did not require any fees, asking instead for donations. The result is the largest conference in the history of the KDA. Our live conferences usually draw about 100 participants. Our first virtual conference brought 250 viewers. For the 2021 conference, "Global Momentum: Bringing the KD Community Together" we had an unprecedented 371 registrations.



We are amazed at how the KD community has come together and continues to provide generous funding for research. Even though many of the major fundraising efforts of years past could not be held due to the Covid pandemic, the community gave and gave generously. With that money, we support new research projects and have also added efforts to promote even more interest by starting a comprehensive patient registry.

Accomplishments for 2021

With your help and support, the following is a review of what happened with the KDA this year.

- Annual Conference: The KDA held its second all-virtual conference, hosting over 200 participants.
- Fund Research: KDA commits \$175,000 to fund research on finding a treatment or cure for Kennedy's Disease.
- Started a patient registry for men and women with the SBMA gene: After several months of collaborative work with the NIH and Sanford Health, the KDA has started the CoRDS patient registry. The development of this registry is crucial to future research and a key moment in our quest for a treatment or cure. We learned at the 2020 KDA conference that multiple pharmaceutical companies are considering clinical trials of potential treatments for Kennedy's Disease. However, they need KD patients enrolled in a patient registry in order to move forward. There are **over 200** people registered so far.
- Support of Research by communicating the need for participants.
 - The NIH has launched a new study titled "Clinical, Molecular and Imaging Biomarkers in Spinal and Bulbar Muscular Atrophy (SBMA)"
 - A Quality-of-Life survey is being conducted by the National Institute of Health (NIH) along with the University of Rochester. They are studying the issues and symptoms that are most important to patients with spinal bulbar muscular atrophy (SBMA).
- Published seasonal KDA email updates on what is happening in the world of Kennedy's Disease
- Regular KDA website updates with information on research and clinical trials.

We are thankful for all of you who support the Association, our cause, and Kennedy's Disease research. Your kindness and generosity are greatly appreciated.

**“Working together to find a cure ...
for our generation, and for our children and our grandchildren”**

2021 KDA Awards Grants

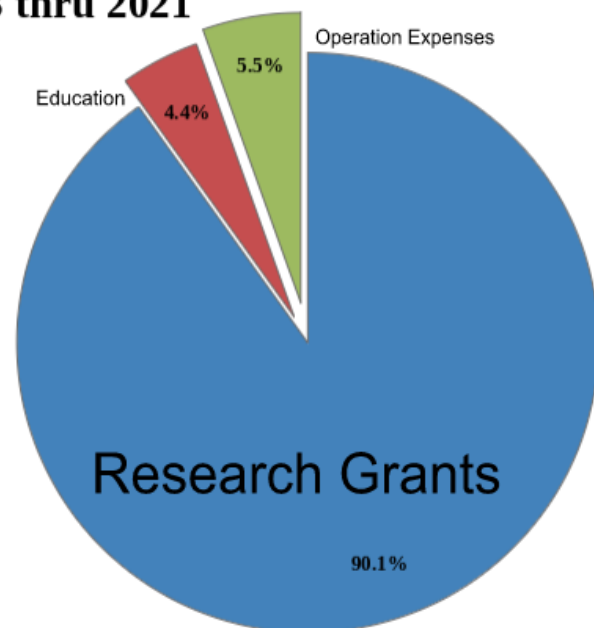
In 2021, research grants were awarded totaling \$175,000. Award recipients are listed below along with a brief description of their research proposal. Here is a link to find out more or to see previous years grant recipients. <https://kennedysdisease.org/research/research-grants>

Carlo Rinaldi, Oxford University (\$75,000 for one year): We plan to elucidate polyQ AR transcriptional altered activity and its role in SBMA pathogenesis, filling a fundamental gap in the understanding of this disease, and to unravel the therapeutic mechanism of action of AR45, a naturally-occurring AR isoform able to fine-tune AR transcriptional activity. Ultimately our goal is to bring a gene therapy approach based on therapeutic delivery of AR45 into first-in-man clinical testing for SBMA patients.

Shinichiro Yamada, Nagoya University: (\$100,000 total - \$50,000 per year for two years): Most patients with SBMA experience cold exposure, a worsening of muscle movement under cold temperature, which is caused by muscle membrane hyperexcitability due to abnormal sodium current alteration. Based on the clinical and basic studies, we carried out a placebo-controlled, randomized, double-blind, multicenter, crossover exploratory clinical study of the efficacy and safety of mexiletine hydrochloride, a sodium channel blocker, in SBMA patients. ALSFRS-R which reflect comprehensive motor function and quantitative muscle strength in SBMA patients tend to be improved in the mexiletine group. Therefore, we will prepare a confirmatory clinical trial with change of ALSFRS-R as the primary endpoint. This study will be the first trial to confirm the efficacy of mexiletine hydrochloride administration in SBMA patients.

As of November 2021, the KDA has awarded \$1,880,339 in research grants 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.

How are donations to the KDA used? 2003 thru 2021



2021 Statement of Activities

Income	2021 \$ (U.S.)	2020 \$ (U.S.)
Conference Registration/Donations	11,065	11,285
Donations	2,609	0
Facebook	16,698	19,305
General	204,974	114,021
In Memory/Honor	22,530	7,143
Proceed Partners	1,965	1,443
Misc. Fundraisers	3,915	6,742
Rare Disease Day - Bay Area (fundraiser)	20,554	-
Dim Sum Give Sum (fundraiser)	5,000	35,100
Golf Tournament - Texas (fundraiser)	2,361	335
Gift of Stock	2,267	-
Sales	140	-
Other	50	250
Total Income	294,128	\$195,674

Charitable Operations/Expenditures	\$ (U.S.)	\$ (U.S.)
Research Grants	175,000	196,200
Education	2,265	0
Conference Costs	0	0
Total Charitable Expenses	\$177,265	\$196,200

Expenses	\$ (U.S.)	\$ (U.S.)
Bank and Credit Card Fees	2,724	981
Insurance/Licensing/Membership	2,041	1,746
Web and Web hosting	1,475	2,726
Total Office (Phone/Postage/Equip and Software)	737	733
Total Expenses	\$6,977	\$6,186

Gain/(Loss)	109,886	\$(6,712)
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Complete financial statements are available upon request from the KDA.

Worth recognizing with the fundraisers for this year is that many events did not actually happen due to the covid pandemic. However, groups that normally have an event still managed to raise funds! Thank you to Louise Goforth and the Texas Crew, and Dan Jacobs with Dim Sum Give Sum who manage to power through these tough times and still give. And special shout out to Fred Briones who started the Rare Disease Day - Bay Area fundraiser, with GREAT SUCCESS.

Looking Toward the Future

The KDA's Board of Directors has approved the following goals for 2022.

We will continue to:

- Raise money to give to research and support our cause (a goal of \$200,000).
- Review other ways to support research, such as Fellowship Grants.
- Sponsor a LIVE/VIRTUAL KDA conference and educational symposium in the fall of 2022.
- Support the efforts of the NIH groups that are dedicated to understanding KD and finding a cure or treatment for KD.
- Publish seasonal 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.
- Continue to update the KDA website with information on research and clinical trials, create a greater level of security software for our associates, and emphasize the 'KDA Family' theme.

In addition, we also look to:

- Work with the KD/UK to organize and conduct "The Great Road Trip" which has a Citroen, 2CV drive several thousand miles to raise funds and awareness for KD. The trip starts in Los Angeles, California - drives across the United States - continues in the UK and concludes in Rome, Italy.
- 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
- broaden the social media reach of the KDA
- lead Zoom sessions for different groups with the KDA
- specialize in supporting fundraising efforts
- Continue and expand our working relationships with counterpart advocacy groups in the UK, Australia, Italy, and other countries.

2021 KDA Officers and Board of Directors

February 2021, the Kennedy's Disease Association announces Terry Thompson as the new KDA president. He is passionate about finding treatments and a cure for KD, a disease that directly affects his family. Doctor Thompson comes to this role of president with a long track record of leadership and experience. He has served as an officer in the U.S. Marine Corps and went on to become a Senior Executive with the National Security Agency. He joined Booz Allen Hamilton where he rose to the position of Vice President and currently teaches graduate courses in cyber-security at Johns Hopkins University. Terry has a BA and MA from Wayne State University in Detroit, Michigan, and a PhD from Georgetown University. Though Terry is new to the KDA board, he and his wife Kathy (who has been successfully running our conferences for a number of years) have invested several years as active members in the KD community. They are powerful advocates to bring awareness and funding for Kennedy's Disease. The board of directors of the KDA is thankful and welcomes Terry to lead us in our mission to inform, support, educate, fund research and find a cure for Kennedy's Disease.

We also thank Maria Monte who has served as interim president for the past year and a half and who continues to volunteer as our treasurer. We are greatly thankful to your service and talents for our cause!

- Terry Thompson, President
- Jameson Parker, Vice President
- Maria Monte, Treasurer
- David Yelton, Secretary
- Ron Moffett, Member
- Steven Rittmaster, Member
- Kathy Thompson, Member
- Dale Traxler, Member

Retired Board members who continue to assist:

Ed Meyertholen, Patient Advocate to the Scientific Review Board