Kennedy's Disease Association

Annual Report
2023

UNITING FOR

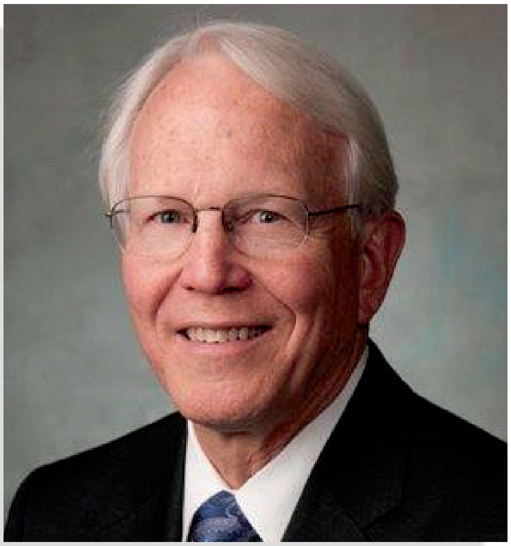
PATIENTS
FAMILIES
A FUTURE WITHOUT KD

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, friends and researchers. 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 raised 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

KDA and the KD community had a very successful 2023. Thanks to the continuing generosity of the KD family and friends, we were able to contribute more to research than in any other year in our history, awarding four grants and a fellowship to five researchers and sponsoring an SBMA workshop with specialists from several related disciplines. We saw the first clinical trial of a potential treatment developed by AnnJi Pharmaceuticals and the announcement of another potential treatment by Nido Biosciences. We strengthened our alliance with KD patient advocacy groups, especially KD-UK, who hosted an international KD conference in November, and we expanded our relationship with the Muscular Dystrophy Assocation. We were saddened by the loss of our Treasurer, David Yelton, in December, but glad to welcome new Board members Joan Sorensen, Randy Soo Hoo, and John Lauber. We look forward to an equally productive 2024 as we continue the search for a cure.

Terry Thompson, President

2023 | YEAR IN REVIEW

2023 was a very busy, productive and eventful year as the KDA continues to focus on and strive to find a cure for Kennedy's Disease. Below is a snapshot of some of the year highlights.

2023 GOALS

Raise $200,000 to give to research and support our cause.

Include one or more Fellowships in the 2023 Grants Program.

KDA Awards $437,000.00 In Research Grants For 2023

The KDA awarded $437,000 in research grants in 2023. As of October 2023, the KDA has awarded $2,453,617.00 in research grants to help find a cure or treatment for Kennedy's Disease.

11th Annual KD Golf Scramble – 4/22/2023

The 11th annual KD Golf Scramble was held on April 22 with great success. It was fun for all and the scramble raised a yearly high donation amount of around $43,000 to help find a cure for Kennedy's disease. Over the past 11 years. The KD Golf Scramble has raised over $343,000 for research to find a cure for Kennedy's Disease. The goal of our 11th annual tournament was to not only provide much-needed funds to help find a cure for Kennedy's Disease, but also to raise awareness of the disease and give HOPE to anyone affected by it that we WILL find a cure. To learn more about the KD Golf Scramble and view photos, click here.

Thank you to the Montie family and friends who raised over $10,000 this year in their annual Ed Montie 5K event.

Thank you for Fred Briones and large community of supporters who raised over $20,000 during their annual Bay Area Rare Disease Day.

Together, we'll find a cure to this rare genetic progressive neuro-muscular disease!

2023 GOALS

Support the efforts of the NIH and other research labs that are dedicated to understanding KD and finding a cure or treatment for KD.

Support clinical trials by maintaining relationships with pharmaceutical companies and sharing information with the KD community.

The First-In-Patient Trial of a Drug to Treat Kennedy's Disease

In 2023, AnnJi tested AJ201 on its first patient for the KD clinical trial. Avenue Therapeutics and AnnJi were excited to announce that the first patient was dosed in the clinical trial of AJ201. There were six AnnJi drug trial distribution sites set up to distribute the drug. For more information about the drug, click here.

The KDA held its First KD Banbury Conference in September.

The KDA tried a new approach to researcher collaboration. In September, the KDA held its first Banbury Conference. Researchers from around the world gathered at the Banbury Center to stimulate additional research into the mechanisms of SBMA pathology, and to identify potential therapeutic approaches. A key goal of the workshop was to invite fresh perspectives from research experts with a diversity of backgrounds and to encourage new collaborations among scientists leading to new areas of SBMA research and/or therapy development. Banbury Center in New York is a gathering spot for scientific think tanks. "The meetings are recognized internationally as being amongst the world's best discussion workshops for topics in molecular biology, molecular genetics, human genetics, neuroscience and science policy."

Nido Biosciences announced the development of NIDO-361

Nido Biosciences, a Boston-based company specializing in precision treatments for neurological diseases, announced the development of NIDO-361, a clinical stage therapeutic targeting the gene mutation that causes Kennedy's disease (Spinal and Bulbar Muscular Atrophy, or SBMA). NIDO-361 is based on original research by Dr. J. Paul Taylor of St. Jude Children's Research Hospital and a former member of KDA's Scientific Review Board. To read more, click here.

The KDA Supports Gordon Conference on CAG Repeat Diseases

Support for research and education on Kennedy's Disease (SBMA) is one of the main functions of the KDA. This year, in addition to funding our annual grants and fellowships programs, KDA provided $5,000 for scholarships to allow researchers to attend the 2023 Gordon Conference on "CAG Triplet Repeat Disorders" held in June in Vermont. This conference is part of the Gordon Research Conferences, an internationally recognized program for scientists to present and discuss new research in many fields. The Vermont conference will focus on SBMA in addition to related, CAG repeat diseases such as Huntington's disease, spinocerebellar ataxias and dentatorubral-pallidoluysian atrophy. Conference attendees heard presentations from senior SBMA researchers and participated in discussions about potential treatments.

The French National Protocol for Kennedy's Disease (SBMA) is Published

The Kennedy's Disease Association Board of Directors & Scientific Review Board encourages clinicians to consult the French National Protocol for Kennedy's Disease (SBMA) for guidance on diagnosis and management of Kennedy's Disease (KD). To read the document, click here.

Kennedy's Disease/SBMA Voice of the Patient Report is Published

This report is based on the "Patient-Focused Drug Development (PFDD)" meeting held at the KDA 2022 conference and related patient input. Voice of the Patient reports are used by the U.S. Food and Drug Administration as an important source of information used to evaluate clinical trial results. To read the report, click here.

2023 GOAL

Build a new KDA website and continue to provide updates about research and clinical trials, and emphasize the 'KDA Family' theme. Also publish quarterly newsletters.

The KDA publishes 4 newsletters

In 2023, we published a newsletter with each of the seasons.

The KDA launches a new website

In the Spring of 2023, the KDA launched a new, improved, and updated website! The new site features streamlined displays that are easier to read, topics that are easier to find, updated information on Living with KD and KD Research, an Events calendar, notes from Board of Directors meetings, and more.

The KDA develops Short KD Reference Guides

The KDA has started to develop a series of short (1-2 page) reference guides on different aspects of KD including symptoms and management approaches. These documents will provide information to patients as well as medical personnel who may not be familiar with KD. To access these guides, click here.

2023 GOALS

Collaborate with KD/UK on a joint KDA-KD/UK conference and educational symposium in Fall 2023.

Continue and expand our working relationships with counterpart advocacy groups in the UK, Australia, Italy, Japan, and other countries.

The KDA Joins KD-UK in London for a Kennedy's Disease Conference

The Kennedy's Disease Association and KD-UK sponsored the London International Conference on Kennedy's Disease in London. This Kennedy's Disease conference was held in November at the Bloomsbury Event Venue in London. The first two days of the conference were focused on people living with Kennedy Disease, carriers, carers and for people wishing to learn more about the disease. The second two days were predominately aimed at researchers, clinicians and scientists.

2023 GOAL

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.

The KDA Board adds new members and says good bye to a friend

The board was able to add three new board members. Joan Sorensen, Randy Soo Hoo and John Lauber, who took over the role of treasurer.

In 2023, the board had to say goodbye to a friend and long time board member, David Yelton, who served as treasurer of the KDA. David passed away in late 2023.

2023 GOAL

Expand on the successful Carrier Group Zoom sessions to include sessions for KD Men and Caregivers.

KDA started is hosting a Zoom chat for men.

In the Spring of 2023, the KDA started hosting a Zoom chat for men to share experiences and just catch up with old friends. Chats are typically held the 2nd Saturday of each month - MensZoomChat@kennedysdisease.org

2023 GRANT & FELLOWSHIP AWARDS.

In 2023, the KDA awarded four research grants and one fellowship totaling $437,000. Award recipients are listed below.

$100,000 "Identification and characterization of kinase(s) responsible for androgen receptor phosphorylation at serine 16", Masoud Shekarabi, Thomas Jefferson University.

$69,000 "Connecting SBMA national registries databases: a retrospective study" Davide Pareyson, Besta Institute, Milan, Italy (two-year award) $46,000 (2023) and $23,000 (2024).

$91,350 "Polyglutamine expansion in aggregation AR in cells", Xavier Salvatella Giralt, IRB Barcelona, Spain (two-year award) $49,350 (2023) and $42,000 (2024).

$100,000 "Investigating the role of CD38 in metabolic dysregulation in Kennedy's Disease", Heather Montie, Philadelphia College of Osteopathic Medicine

$75,000 "Investigating differences in polyQ-AR genomic activity leading to muscle atrophy in SBMA", Anastasia Gromova, University of California, Irvine. Recipient of the 2023 Waite-Griffin SBMA Fellowship

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

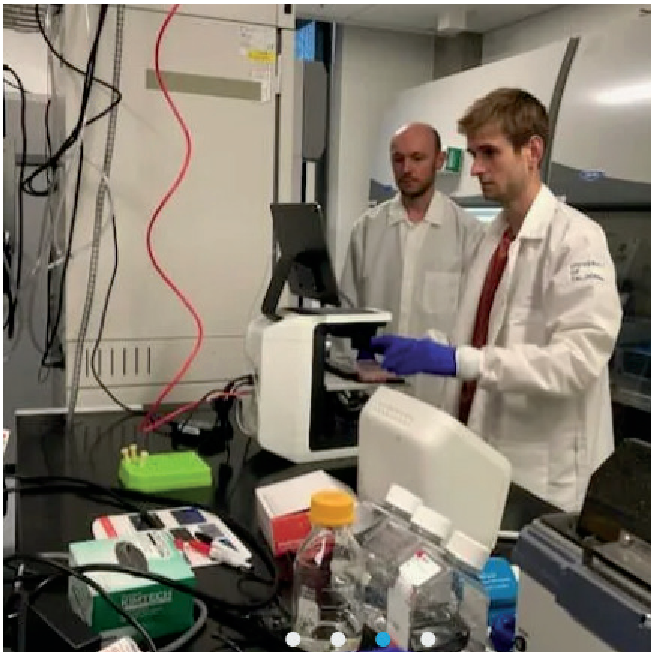
As of October 2023, the KDA has awarded $2,453,617.00 in research grants to help find a cure or treatment for Kennedy's Disease.

LOOKING TOWARD THE FUTURE

The KDA's Board of Directors has approved the following goals for 2024.

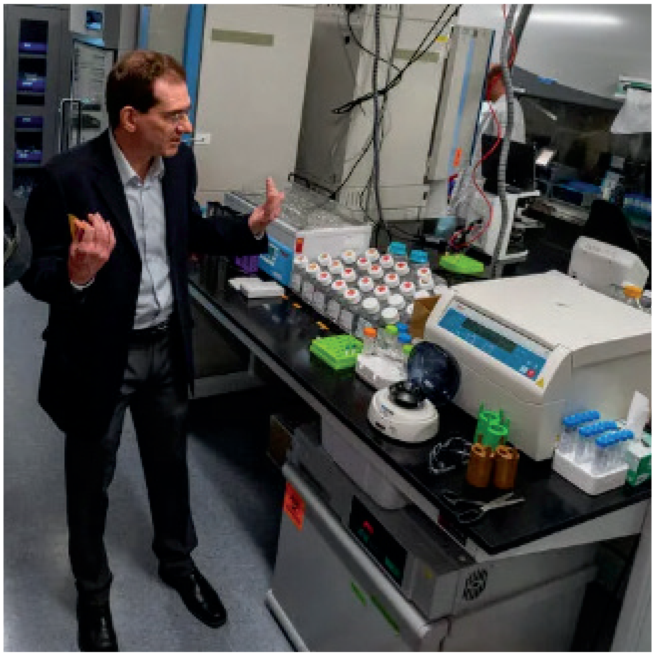
WE WILL CONTINUE TO:

- 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 2024 Grants Program.
- Offer a patient support conference and educational symposium in Fall 2024.
- Support the efforts of the NIH and other research labs that are dedicated to understanding KD and finding a cure or treatment for KD.
- 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.
- Continue and expand our working relationships with counterpart advocacy groups in the UK, Australia, Italy, Japan, and other countries.

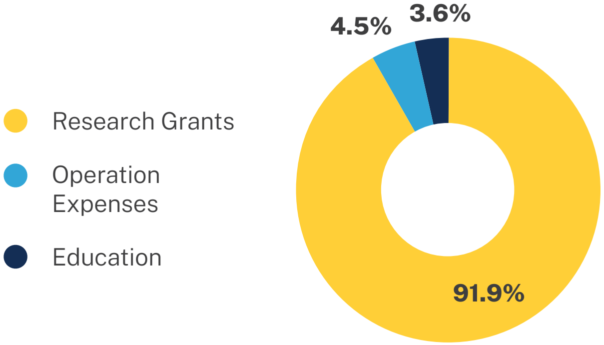


FINANCIALS

HOW ARE DONATIONS TO THE KDA USED?

Over the course of the last 19 years, over 95¢ of every dollar received by the KDA is used 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.

2003 – 2023



2023 STATEMENT OF ACTIVITIES

	Year to date 12/31/2023	**Year to date** 12/31/2022
REVENUES		
Conference - Net	(10,000)	(4,172)
Donations	381,589	224,041
Fundraising Events	69,739	99,450
Other	10	4,973
TOTAL REVENUE	**441,338**	**324,292**
EXPENDITURES		
Awards, Grants And Related Support	447,753	451,000
Admin & Fundraising		
Bank & Credit Card Fees	3,216	3,819
Business Registration Fees	3,585	–
Direct Fundraising Expense	836	–
Insurance	1,572	1,572
Office Expense & Other	1,457	9,004
Website Expenses	10,730	1,832
Total Admin & Fundraising	21,396	16,227
Percent of Total Revenue	4.8%	5.0%
TOTAL EXPENDITURES	**499,149**	**170,227**
Investment Income (Expense) & Other	13,200	(124)
NET REVENUE	**(44,611)**	**153,941**

2023 KDA OFFICERS AND BOARD OF DIRECTORS

The Kennedy's Disease Association

P.O. Box 1105, Coarsegold, CA 93614
Telephone: 1-855-KD-ASSOC
(855) 532-7762 or (734) 288-5580
info@kennedysdisease.org
www.kennedysdisease.org