2021
ANNUAL
REPORT

Global DARE Foundation
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#CureRefsum
A LETTER FROM THE PRESIDENT

Global DARE Foundation has had another amazing year. Again in 2021 we established a set of measurable goals focused on improving treatment and care, advancing research, raising awareness, and collaborating. As you will see in our accomplishments, we have achieved everything we set out to do this year.

Shortly after the year started, we established a partnership with Kennedy Krieger Institute to deliver on our objective to test more foods to understand their fatty acid content. Only 150 foods had been tested in the past. Susan Kuranoff, DARE’s Vice President and Ann Moser of Kennedy Krieger deserve special recognition for their hard work in moving forward this project. A big win for our community!

We spent much of the year increasing the awareness of Refsum Disease around the world. This was done with the release of our easy-to-understand white boarding video in 5 languages, more strategic partnerships built by our Country Ambassadors, Google Ad campaigns, and our educational webinars.

Our Medical and Scientific Advisory Board has been very engaged with us this year discussing how we move forward treatment guidelines for Refsum, determining the translational research to pursue and made several grant submissions to advance our mouse model studies at Johns Hopkins. In 2022, we expect to begin some preliminary mouse model studies.

We launched the first annual DARE to Believe 5K fundraiser in June. It was a great success! We were fortunate to have two matching contributions that allowed us to raise just over $33K. A special thanks goes to my co-workers at TD Bank who helped us receive a $8K matching grant from TD Bank.

In October, we were excited to be awarded a one-time $50,000 general operating support award from the Chan Zuckerberg Initiative for the impressive and inspiring work we are doing. This will go a long way to helping us fulfill our mission in 2022.

We are very grateful to have such strong supporters and volunteers helping us achieve our mission. None of this would have been possible without all of you. We are positioned well for continued success in 2022!

Kristie DeMarco | President
Global DARE Foundation

www.DeatAdultRefsumEverywhere.org
Board of Directors

When we established Global DARE Foundation back in 2019, we had 5 Board members. This small and mighty board has accomplished a lot over the last 2 years. This year we have begun to grow our board to ensure we bring a diverse set of skills and perspectives to everything we do. We are proud of the strong diverse team we have built with our 12-person board. This will set Global DARE up for long term sustainability and success.

Medical & Scientific Advisory Board

Our Medical & Scientific Advisory Board (MSAB) is made up of the foremost researchers and clinicians in the field of Refsum Disease, peroxisomal disorders and Retinitis Pigmentosa. The MSAB is constantly collaborating with us on how to improve treatment and care and direct the areas of focus for Refsum research. One of the exciting things the MSAB is working on is developing treatment guidelines. Having published treatment guidelines will have a big impact on influencing better quality of care for people with Refsum Disease.
COUNTRY AMBASSADOR PROGRAM

Our Country Ambassador program was established to raise awareness of Refsum disease around the world and is vital to our mission. Country Ambassadors are responsible for establishing and building relationships within their country to increase awareness and support for those with Refsum disease. Ambassadors serve as official liaisons between their country and the Global DARE Foundation and are essential in achieving our mission. Our hope is that the program will help people with Refsum receive an earlier diagnosis and connect people who have already been diagnosed with the Refsum community.

2021 Key Accomplishments

**Driving Awareness**

- Rare Bones Article in Rare Revolution Magazine
- Took over social media for one day on Rare Revolution Magazine sites
- Refsum Disease pages build out on Blindness foundation websites (Retina UK, Foundation Fighting Blindness, Retinal International, ProRetina)
- Inherited Retinal Disease specialists and other specialists (e.g., neurologists, podiatrists) are being contacted worldwide to bring awareness of Refsum Disease
- An ambassador’s life story was published on Retina UK

**Advocacy**

- Reached and met with politicians concerning Refsum disease to bring recognition as well as support to our mission.
- Attended many virtual rare disease events, meetings, and conferences (NORD, Rare Disease Week at Capitol Hill, Global Genes Patient Summit, NIH Rare Disease conference).
- Initiated work with Guy’s and St. Thomas' Hospital in London to improve access to PA testing so patients won’t have travel to London to have blood work completed.
- Provided endorsement to several US Rare disease legislative bills (newborn screening, increased NIH/FDA funding)
DRIVING EDUCATION AND AWARENESS

As part of Global DARE Foundation's goals, we look to raise awareness and conduct educational webinars and videos to all that are interested in learning more about Refsum Disease.

**White Boarding Video:** Global DARE Foundation collaborated with Youreka Science and our Medical and Scientific Advisory Board to create this easily understood whiteboarding video about Refsum Disease. It is being used to help raise awareness of Refsum Disease through use on social media and other media channels. Our hope is it will educate clinicians and people experiencing these symptoms to enable earlier diagnosis. Early diagnosis is so critical to improve the quality of life of people with Refsum disease.

**Webinars:** Global DARE Foundation hosted three webinars in 2021 discussing the Ophthalmology side of Refsum Disease and the benefits of early diagnosis by clinicians, the clinical practice for treating people with Adult Refsum Disease and Phytanic Acid analysis.

**Refsum Disease Campaign:** Rare Disease Day® takes place on the last day of February each year. The week leading up to that day Global DARE Foundation ran a Refsum Awareness Campaign. We raised awareness for Refsum disease by sharing facts about the disease and personal stories on social media.
AREAS OF FOCUS FOR REFSUM RESEARCH

Global DARE’s Research team is investigating several areas of research to both better understand Refsum Disease and begin discovery into better therapies.

**Mouse Model Studies:** The Refsum mouse model has been brought to Johns Hopkins Research center to begin pilot studies in gene therapy and conduct other studies that will help to better understand Refsum Disease.

**Expanding Omega Oxidation Pathway:** People with Refsum can process small quantities of phytanic acid through the Omega Oxidation pathway. Prior research showed the possibility of enhancing the pathway to process more phytanic acid. Our research team has begun the work to revive this promising research.

**Metabolomic Profiling:** The research team is investigating the use of metabolomic profiling on blood samples of people with Refsum to understand if there are other biomarkers besides Phytic Acid that should be considered.

FOOD TESTING PROGRAM

Until better therapies are found for Refsum Disease, a diet low in phytanic acid forms the cornerstone for managing the condition and improving quality of life. Given the importance, at the beginning of the year, Global DARE Foundation entered into partnership with the Kennedy Krieger Institute (KKI) of Baltimore to carry out fatty acid testing in food items. A total of 60 food items identified by the Refsum community are currently being tested for phytanic acid content, and its precursors phytol and phytyl fatty esters according to new methodology established by KKI, which will help to better inform diet choices for people impacted by Refsum. The DARE Foundation plans to continue testing additional food items on an annual basis to expand the existing repertoire of food suitable for people with Refsum which will be a big win for the community.

Collaborating with Em. Prof. Ann Moser at the KKI has enabled the Foundation to harness many years of rich experience of testing fatty acids in disorders of peroxisomal metabolism, including Refsum Disease. Ann has since become a member of the DARE MSAB and plays a leading role in the DARE research group.

Continue Reading article in appendix
REFSUM PATIENT REGISTRY

In August of 2022 Global DARE Foundation rolled out the first ever patient registry. A patient registry would bring together the Refsum community and researchers to gain a better understanding of Refsum Disease

We believe that the registry will help us do the following:

- Develop treatment guidelines to improve the care people receive
- Drive specific research to develop better therapies
- Provide Global DARE Foundation information to focus support efforts
- Represent a link between Researchers and the Refsum community
- Create interest from investors that can help fund therapy development
- Identify patients for future research studies and clinical trials

We have already begun to learn from the data being collected in the registry.

WHAT HAVE WE LEARNED SO FAR?

Long Time to Diagnosis

2 years → 38 years
Average: 19 Years

79% Diagnosed through Genetic Testing

43% have a Phytanic Acid Level done only yearly

Ophthalmologists, Neurologists, Dietitians, Audiologists, and Metabolic Specialists are the most frequently seen specialists

You can learn more about the Refsum Patient Registry at our website.

www.DeatAdultRefsumEverywhere.org
FINANCIAL UPDATE

Thanks to the Chan Zuckerberg general operating support grant and our generous donors, Global DARE Foundation raised $94,000* in 2021 which helps us to continue our mission of improving the lives of people diagnosed with Refsum Disease. Our leadership continues to be focused on maintaining low overhead and maximizing investments in research, education, and awareness.

*2021 Financials are as of October 31st

Fundraising Efforts

www.DefeatAdultRefsumEverywhere.org
FINANCIAL UPDATE CONTINUED

Year over Year Operating / Fundraising Expense

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*2021 Expenses are as of October 31st

Expense Breakdown – % of Income

Notes of interest:

- 2021 we paid no payment process fees for donations by using platforms that either charge no fees or fees that can be paid by the donor.
- 2021 program expenses included delivery of our whiteboarding video and food testing program.
- 2021 fundraising expense was for the t-shirt, masks, and shipping for the DARE to Believe 5K.
- We continue to keep operating expenses very low and continue to increase our funding towards executing our programs.
APPENDIX – FOOD TESTING PROGRAM

Continuation...

A normal daily diet consists of approximately 60 mg of phytanic acid. People with Refsum should not consume more than 10 mg per day to avoid harmful accumulation of phytanic acid. This means it is extremely important to understand not only the phytanic acid content of each food making up part of the diet but also some of its relevant precursors. A diet comprised of foods low in phytanic acid content is suspected to slow the progression of some of the more debilitating disease symptoms such as retinitis pigmentosa, peripheral neuropathy and hearing loss.

It’s been almost 30 years since initial phytanic acid testing in approximately 150 food items was published and formed the basis for a low phytanic acid diet (Brown et al., 1993). Since then farming practices and food manufacturing processes have changed dramatically as well new precursors to phytanic acid were identified by the Institute of Food Chemistry in Stuttgart, Germany (Krauss et al., 2017). Krauss’s results suggest that the consumption of phytol fatty esters leads to the release of trans-phytol during the digestion process while trans-phytol is then metabolized by oxidation into phytanic acid. This would imply that foods high in phytol fatty ester content could contribute to the total daily amount of phytanic acid recommended for consumption. Due to the complexity of this subject matter, the DARE research team is consulting with the authors of the Krauss et al manuscript to tap into their expertise on the topic.

The basic foods currently being tested by the DARE research group include items that have never been tested before such as seeds, legumes, an expanded selection of nuts, fish and fruit. Some foods tested in the past are being repeated using today’s newest testing methodology to update previous results. This testing methodology will form the basis for all future testing, providing better overall understanding of optimal diet choices for the Refsum community. Once the final testing results are available, the existing diet sheet will be updated and shared publicly via an educational webinar.
