

Global DARE Foundation 2025 ANNUAL REPORT



REFSUM WITHOUT BORDERS
Romania - August 2025



Refsum without Borders
Community Gathering in Cologne

"Meeting Chantal and Stephan not only strengthened our bond but also reminded me of the power of community."

Kristie DeMarco, President
Global DARE Foundation



LUNCH AT SHARON'S

WELCOME TO MELBOURNE

Refsum Without Borders

FEIBAN, JOY, SHARON



**CELEBRATING
6 YEARS
OF SUCCESS!**



JABATAN OPHTHALMOLOGI
UNIVERSITI KEBANGSAAN MALAYSIA

JABATAN OPHTHALMOLOGI
UNIVERSITI KEBANGSAAN MALAYSIA

Kristie DeMarco
President
Global Dare Foundation

Refsum Without Borders
Malaysia



The Lions Club of Greenwich is Making a Difference.



The Global DARE Foundation is working to Defeat Refsum Disease.

Together We Are Stronger.



Global DARE Foundation
443 Western Ave #1054
South Portland, ME 04106
United States
info@globaldare.org

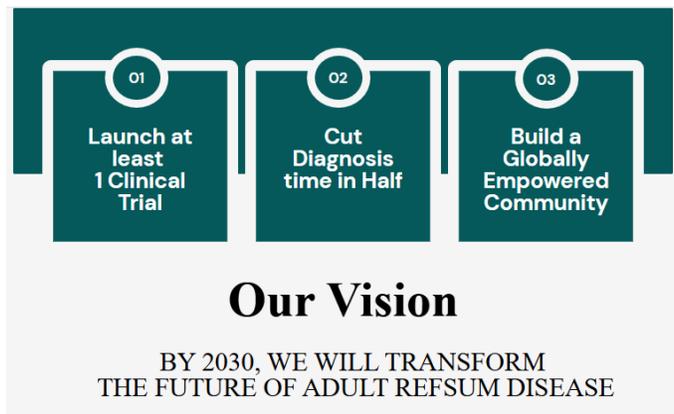
A LETTER FROM THE PRESIDENT

Over the past year, with your support, Global DARE Foundation reached a pivotal moment in advancing its mission to improve the lives of individuals affected by Adult Refsum Disease. We accelerated our scientific efforts by hiring a Scientific Director and approving funding for the first-ever Natural History Study in Refsum Disease, an essential step toward understanding disease progression and enabling future clinical trials. In parallel, our Medical and Scientific Advisory Board submitted the first clinical care guidelines for Refsum Disease for publication, providing clinicians worldwide with a much-needed framework for diagnosis and management.



Global awareness and community engagement expanded significantly. Global DARE connected with individuals in 22 countries, hosted scientific and community meetings, established a new Center of Excellence in Germany and deepened engagement with patients to better understand lived experiences and priorities. Early diagnosis efforts were advanced through targeted outreach to retinal specialists and an important awareness milestone was achieved by exhibiting for the first time at Euretina in Paris, engaging ophthalmologists from around the world. Our Country Ambassador Program continued to grow, driving local impact through initiatives such as securing grant funding in Germany to support food testing and launching new clinical awareness efforts in Australia.

Global DARE's Vision for 2030, a defining step forward launched at the end of 2025, focuses on initiating the first clinical trial for Refsum Disease and cutting diagnosis time in half. We successfully restarted and expanded the Food Testing Program, delivering immediate, practical support for individuals managing Refsum Disease through diet, and launched the Patient Collaborator Program to ensure lived experience is embedded in research and advocacy. Securing Chan Zuckerberg Initiative funding enabled Global DARE to transition from a volunteer-led model toward a more professionally supported and sustainable organization. Together, these accomplishments position Global DARE as a globally connected, patient-centered, and research-driven organization, moving us closer to earlier diagnosis, better care, and effective therapies for Refsum Disease.



None of this would be possible without the incredible support of our community, and for that, I am deeply grateful. Thank you.

With gratitude,
Kristie DeMarco | President
 Global DARE Foundation

BOARD OF DIRECTORS

Over the past year, the Global DARE Foundation Board of Directors continued to provide strong strategic leadership, guiding the organization through a period of meaningful growth and momentum. The Board remains deeply committed to advancing Global DARE's mission to improve care, accelerate research, raise global awareness, strengthen community, and ensure long-term organizational sustainability.



Kristie DeMarco
President



Susan Kuranoff
Vice President



Marissa Rossouw
Secretary



Darrell Gunter
Director



Mark Liquorish
Director



Heidi Page
Director



Donald Samulack
Director



Kerstin Reichert
Director

As part of our commitment to transparency and community engagement, Global DARE hosted two public webinars to share program updates, accomplishments, and future plans. [*Global DARE 2025: Driving Change, Shaping the Future of Refsum*](#) provided a comprehensive overview of progress across research, care, and community initiatives, while [*Rare to Recognize: Path to 2030*](#) outlined the Foundation's bold Vision for 2030, focused on initiating the first clinical trial for Refsum Disease and cutting diagnosis time in half. In both sessions, Board members reflected on the organization's growth and shared strategic priorities.

Looking ahead, Global DARE is preparing for its next phase of growth and plans to expand its Board of Directors in 2026. Those interested in learning more about Board service are encouraged to reach out and join us in shaping the future of Global DARE Foundation. Please contact us at info@globaldare.org.

MEDICAL & SCIENTIFIC ADVISORY BOARD

The Global DARE Foundation's Medical & Scientific Advisory Board (MSAB) is comprised of leading clinicians and researchers with expertise in Adult Refsum Disease, peroxisomal disorders, and Retinitis Pigmentosa.

Over the past year, the MSAB played a central role in advancing Global DARE's research and care agenda. Board members were instrumental in the development and submission of the first-ever clinical care guidelines for Adult Refsum Disease, providing clinicians worldwide with a critical framework for diagnosis and management. The MSAB also helped design the Foundation's Natural History Study, a foundational milestone that will deepen understanding of disease progression and enable future clinical trials.

In addition, the MSAB supported expanded engagement with ophthalmologists and researchers globally, helping raise awareness of Refsum Disease within the retinal and rare disease communities. Members continued to advance translational research through ongoing work with the Refsum mouse model, generating insights to inform future therapeutic pathways. The Board also contributed to the continued development and expansion of Global DARE's Centers of Excellence, strengthening access to specialized, coordinated care.

Through their leadership, expertise, and unwavering commitment, the Medical & Scientific Advisory Board remains a driving force behind Global DARE's progress, helping bridge research and care, accelerate discovery, and move the field closer to effective therapies and earlier diagnosis.



Joe Hacia, PhD
Chair



Paul Watkins,
MD, PhD,
Co-chair



Mousumi
Bose, PhD
Co-Chair



Ronald
Wanders, PhD



Florian
Eichler, MD



Radha
Ramachandran,
MD, PhD



Anthony
Wierzbicki, PhD



Sarah Firman
Dietitian



Rachel
Huckfeldt, MD,
PhD



Ann Moser,
PhD



Sacha
Ferdinandussa,
PhD



Bart Leroy,
MD, PhD



Wedad Fallatah,
MD, PhD



Reinhard
Klingel, MD,
PhD

COUNTRY AMBASSADOR PROGRAM

The Country Ambassador Program remains a cornerstone of Global DARE Foundation's mission to raise awareness of Adult Refsum Disease worldwide. Ambassadors serve as trusted local leaders—building relationships with clinicians, researchers, advocacy organizations, and patients—while acting as vital liaisons between their countries and the global Refsum community. Through their efforts, Global DARE continues to expand awareness, foster earlier diagnosis, and strengthen international collaboration.



Susan Kuranoff,
Switzerland



Kristie DeMarco,
United States



Alan Gunzburg,
United States



Mark Liquorish,
United Kingdom



Kerstin Reichert,
Germany



Bob Wyborn,
Australia



Susie Breuer,
Australia



Sharon Burstin,
Australia



Michel Veys,
Belgium



Tali Mirsky
Lachman, Israel

Key Accomplishments

This year marked significant progress in both Australia and Germany. In Australia, Country Ambassador Sharon Burstin played a pivotal role in elevating awareness and strengthening clinical connections. She convened a collaborative meeting with the Centre for Eye Research Australia and Retina Australia, participated in awareness webinars, and contributed to articles and papers highlighting Refsum Disease and the challenges of delayed diagnosis. Sharon also hosted a Refsum community meet-up at her home in Melbourne, creating an important opportunity for connection and peer support. Susie Breuer further strengthened community ties by hosting a meet-up that brought together individuals in Sydney.

In Germany, Country Ambassador Kerstin Reichert continued to expand both community engagement and research collaboration. She facilitated Refsum-focused meetings in Germany to better support local patients and families, played a key role in initiating a new Center of Excellence in Leipzig, and successfully secured a \$20,000 grant to support Global DARE's Food Testing Program—directly benefiting the global Refsum community.

In addition, in the US, Kristie DeMarco, presented at the Rare Disease Spotlight event sponsored by the Pharma Neighborhood Network, raising awareness of Refsum Disease and fostering early connections with the pharmaceutical community.

Through their dedication and leadership, Global DARE's Country Ambassadors continue to drive meaningful local impact around the world.

REFSUM DEUTSCHLAND E.V.

Refsum Deutschland e.V. is a key international partner of Global DARE Foundation, playing an increasingly important role in strengthening community connection, clinical awareness, and research collaboration across Germany, Austria, and Switzerland. In 2025, the organization continued to grow in reach and visibility, now serving 47 members and actively connecting 16 individuals living with Refsum Disease across the region.

Throughout the year, Refsum Deutschland expanded relationships with leading experts in genetics, neurology, and ophthalmology, helping ensure patients are connected to both specialized care and community resources. A major milestone was the deepening collaboration with Global DARE during an in-person visit to Germany, including joint meetings at the Apheresis Research Institute in Cologne and University Hospital Leipzig. These efforts supported the establishment of Leipzig as one of only three global Centers of Excellence for Refsum Disease, with Refsum Deutschland leading local patient engagement and awareness. Through strong advocacy and patient engagement, Refsum Deutschland exemplifies the power of global partnership in advancing care and research for an ultra-rare disease.



REFSUM AWARENESS WEEK

June's Annual Refsum Awareness Week brought the global Refsum community together through virtual events focused on awareness, education, and connection. 2025 activities highlighted patient experiences, research progress, and Global DARE's growing momentum toward earlier diagnosis and improved care.

The week featured a series of webinars offering patient perspectives, research updates, and an organizational overview, providing the community with meaningful insight into both lived experience and scientific advancement. Link to webinars:

[Summer Fest 2025](#)

In addition, Global DARE hosted a virtual 5K, encouraging participants to run, walk, bike, swim, or roll in support of Refsum awareness, and published *Real People, Real Stories*, a blog series amplifying patient voices. Together, these activities strengthened community connection, elevated awareness, and reinforced the importance of collaboration in advancing research and improving care for those affected by Refsum Disease.



PATIENT COLLABORATOR PROGRAM

The Patient Collaborator Program is a new and important initiative designed to ensure that the voices of individuals living with or caring for someone with Adult Refsum Disease are embedded directly into research, clinical planning, and advocacy. By elevating lived experience alongside scientific expertise, Global DARE is strengthening its commitment to truly patient-centered progress.

Although still in the early stages, the program has already identified 11 Patient Collaborators from multiple countries who are eager to contribute. These collaborators bring firsthand insight into daily challenges, unmet needs, and what meaningful progress looks like from a patient perspective. Their input helps ensure that research priorities, study design, and future therapies reflect real-world realities, not just scientific endpoints.

Patient Collaborators play a vital role across several areas. They contribute to research and clinical development by helping identify gaps in knowledge and defining outcomes that matter most to patients. They support awareness and advocacy efforts by sharing lived experiences and highlighting the importance of earlier diagnosis and better care. As Global DARE advances toward clinical trial readiness, collaborators will also provide critical feedback on what makes potential therapies meaningful, manageable, and tolerable for patients and families.

To support this work, Global DARE is developing a structured training curriculum that equips collaborators to participate confidently in scientific and advocacy discussions. Several foundational training modules have already been released, with additional modules planned as the program continues to mature. The training videos are available for the full community on YouTube. Link: [Patient Collaborator Training](#)



The Patient Collaborator Program reflects Global DARE's belief that lasting progress happens when patients are not just participants but partners. By embedding lived experience into every stage of our research roadmap, this program strengthens the path toward better care, earlier diagnosis, and effective therapies for Adult Refsum Disease.

REFSUM WITHOUT BORDERS

Because Adult Refsum Disease is often highly disabling, long-distance travel to a single annual gathering is not possible for many individuals and families. Yet in-person connection is essential for building trust, understanding lived experience, and shaping research that reflects real needs. Through the Refsum Without Borders Initiative, Global DARE brings connection directly to the community by meeting people where they live.

Over the past several years, Global DARE has hosted in-person patient meet-ups across Europe, Australia, and the United States, including gatherings in the UK, Germany, Romania, the Netherlands, and Australia. These meetings provide rare opportunities for patients and families to connect, share experiences, and inform Global DARE's research and advocacy priorities. They consistently reinforce the urgency of earlier diagnosis and better therapies.



Refsum Without Borders also advances global awareness by engaging retinal specialists, blindness organizations, and ophthalmology trainees, often the first clinicians to encounter Refsum Disease. Even in regions such as Southeast Asia and Japan with no known patients, these conversations are expanding recognition and opening doors for future education and collaboration. Together, these global, in-person connections have become one of Global DARE's most powerful tools for building community, shaping patient-centered research, and driving progress across borders.

PROGRESS ON REFSUM DISEASE RESEARCH

Research Convenings & Scientific Collaboration

Cologne, Germany – Apheresis Research Institute

Global DARE leaders met with the team at the Apheresis Research Institute (AFI) in Cologne, a long-standing center of expertise in apheresis treatment for Adult Refsum Disease. These discussions focused on defining when and how apheresis can best be used to improve quality of life and how this approach fits into future care pathways.



Leipzig, Germany – University Hospital Leipzig

At University Hospital Leipzig, Global DARE convened with clinical and research leaders to advance two major initiatives: the establishment of a new Center of Excellence in Germany, discuss areas of research, and participation in the natural history study. This collaboration significantly expands both care access and research participation for individuals living with Refsum Disease in Germany and beyond.



United States – Kennedy Krieger Institute

At Kennedy Krieger Institute, Global DARE brought together researchers to review progress across food testing, biomarker development, and translational research using the Refsum mouse model. These discussions informed dietary guidance, explored emerging therapeutic approaches, and helped refine priorities for the broader Refsum research roadmap.

International Natural History Study

As a cornerstone of Global DARE's Vision for 2030, the Foundation funded the first-ever international Natural History Study in Adult Refsum Disease, led by **Florian Eichler, MD** at **Massachusetts General Hospital** and **Radha Ramachandran, MD, PhD** at **Guy's & St Thomas' NHS Foundation Trust**. This multi-center study provides the foundational data needed to understand disease progression, define meaningful clinical outcomes, and enable future clinical trials, directly advancing Global DARE's goal of initiating the first Refsum clinical trial and accelerating earlier diagnosis by 2030.

FINANCIAL UPDATE

In 2025, Global DARE Foundation continued to grow with purpose, thanks to the generosity and commitment of our community. We raised \$290,000, and were able to invest deeply in research, awareness, and community connection while remaining thoughtful stewards of every dollar. We closed the year on strong financial footing, allowing us to look ahead with confidence as we support ongoing programs and planned growth through 2026. In 2025, the vast majority of our resources were directed where they matter most: advancing research, raising global awareness of Refsum Disease, and bringing the community together. Of the \$176,000 spent during the year, 84% percent directly supported programs, with the largest investment focused on research. We invested in management and operating expenses at a responsible level to ensure we could effectively deliver and continue to grow the programs that support our mission and vision.



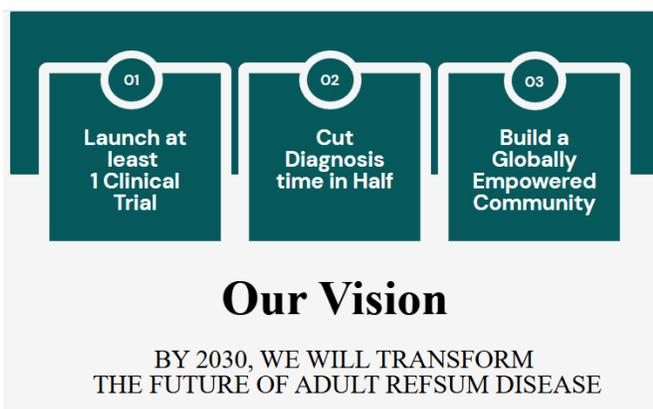
Research & Care
\$127K (72%)

Conferences &
Raising Awareness
\$20K (12%)

Management &
Operations
\$29K (16%)

Thank you for standing with Global DARE Foundation as we work to Defeat Adult Refsum Everywhere and transform what it means to live with this ultra-rare disease.

Together, we are advancing our mission to promote worldwide awareness and a better quality of life for all who are diagnosed with Adult Refsum Disease. Your partnership fuels research, education, awareness, and advocacy at the very heart of this work.



Because of you, we dare to believe in a future where every person with Adult Refsum Disease is diagnosed earlier, supported more fully, and empowered to live a better life. This shared commitment has shaped our 2030 vision to cut the time to diagnosis in half and help initiate at least one clinical trial by 2030.