



ISMRD Annual Report 2024

The International Advocate for Glycoprotein Storage Diseases

Our Mission

The International Society for Mannosidosis and Related Diseases (ISMRD) is the leading advocate for families worldwide affected by a Glycoprotein Storage Disease. Through partnerships built with medicine, science and industry, we seek to detect and cure these diseases, and to provide a global network of support and information.

Our Vision

We seek a future in which children with Glycoprotein Storage Diseases can be detected early, be treated effectively, and go on to live long, healthy and productive lives.

Board of Directors (All non-salaried)

Carolyn Paisley-Dew	President	Australia
Danielle Forsman	Treasurer	USA
Nathan Hubbard	Secretary	USA
Sarah Forsman	Communications Officer	USA
Darko Jamnik	Board Member	Slovenia
Patricia Gribel	Board Member	USA
Laurel Gregier	Board Member	USA
Lama Khalil	Board Member	Saudi Arabia
Tareq Qashou	Board Member	Saudi Arabia
Hussein Peeran	Board Member	USA
Cagdas Canbolat	Board Member	United Kingdom
Abby Lindenberg	Board Member	USA
Erin Hubbard	Board Member	USA

The ISMRD Board represents four continents and six of the nine Glycoproteinoses.

ISMRD Professional Advisory Board (All non-salaried)

Prof Richard Steet: Scientific Chair	USA	Sara Cathey	USA
Steve Walkley	USA	Dag Malm	Norway
Alessandra d'Azzo	USA	Charles Vite	USA
Marc Patterson	USA	Amelia Morrone	Italy
Thomas Braulke	Germany	Vish Koppaka	USA
Enrico Moro	Italy	Jenny Klein	USA

Our Professional Advisory Board is an essential and invaluable source of scientific and medical guidance for the Board and the President. We extend our sincere gratitude to each member of the PAB for their contributions.

OUR ACTIVITIES IN 2024

Activity 1

25 years ago, a family's hope for a cure gave birth to ISMRD. In March 2024, we celebrated a quarter-century of uniting families and scientists across the globe. This **25th anniversary** is an amazing milestone for ISMRD and all the families it has served through its 25 years.

Activity 2

ISMRD was invited by the National Organization for Rare Disorders (NORD) to provide commentary on the impact of Lamzede, an enzyme replacement therapy for Alpha-Mannosidosis introduced in the U.S. in 2023. As a result of ISMRD's testimony, Chiesi Global Rare Diseases was honored with a prestigious **NORD Rare Impact Award**. This award recognizes companies that have developed groundbreaking treatments and individuals who advocate for rare diseases. ISMRD President Carolyn Paisley-Dew and Communications Officer Sarah Forsman were invited to Los Angeles to present the award to Giacomo Chiesi, Head of Chiesi Global Rare Diseases, at Hollywood Studios on June 8, 2024.

Activity 3

ISMRD became a founding member of the **Global LSD Collaborative**.

Activity 4

Leading up to **Rare Disease Day**, we sent daily emails to our members featuring a rare disease fact and highlighting a child with a different Glycoprotein Storage Disease each day. Six of our nine diseases were represented. Additionally, Carolyn Paisley-Dew attended a Rare Voices Rare Disease Day event at Parliament House in Canberra, Australia.

Activity 5

Thanks to a grant from Chiesi Global Rare Diseases, ISMRD **updated its website**, creating a fresh, modern, and accessible source of information for newly diagnosed families, clinicians, and researchers.

Activity 6

ISMRD Board Member Cagdas Canbolat was nominated as a **RARE Champion of Hope**, for his hard work on Galactosialidosis. RARE Champion of Hope Awards are given at the Global Genes RARE Advocacy Summit. This nomination is given to those in the rare disease community who are raising the bar through their innovative approaches to research, programming and advocacy to create meaningful impact.

Activity 7

We celebrated the **40th birthday of founder Paul Murphy's daughter, Taryn**. She was the catalyst for the establishment of ISMRD. Danielle, Jeff and Sarah Forsman traveled to Baltimore, MD to share in the birthday festivities with the Murphy family.

Activity 8

Preparations continued for the **7th ISMRD Family and Scientific Conference** to be held in Minneapolis in August 2025. Registration and sponsorship opportunities for the conference opened on November 1.

Activity 9

Beta-Mannosidosis parents **Nathan and Erin Hubbard joined the Board**, with Nathan taking on the role of Secretary.

RESEARCH

ISMRD announced the outcome of two ISMRD-Sponsored **Mucopolipidosis Research Projects**:

1. To evaluate AAV Gene Therapy in the feline model of ML II.
2. To evaluate the cardiovascular manifestations of ML II in a feline colony through genetic sequencing, cardiac ultrasound, and pathology.

Research continued into the long-term effects of **bone marrow transplant for Alpha-Mannosidosis**, a project funded by the Al-Ansari family being conducted by .

JCR Pharmaceuticals and Medipal continued their work on JR-471, a blood-brain barrier-penetrating **enzyme replacement therapy for Fucosidosis**.

ISMRD Board Member, Laurel Gregier, has been pivotal in establishing the **Lost Enzyme Project** for Beta-Mannosidosis, a foundation dedicated to supporting research and treatment development for Beta-Mannosidosis. TLEP has partnered with Kimonis Labs through the University of California Irvine, as well as JCR Pharmaceuticals, who will create the Beta-Mannosidase enzyme using their patented J-Brain Cargo technology to be tested in an animal model. JCR will train a Kimonis Lab principle researcher in Japan on specialized testing techniques. Over \$150,000 of the \$520,000

total needed to complete these preclinical studies has been raised by the Lost Enzyme Project throughout 2024.

Cagdas Canbolat, Galactosialidosis parent and an ISMRD Board member, has also been working diligently on a treatment for **Galactosialidosis**. His efforts have led to a significant breakthrough: the establishment of a Galactosialidosis registry in Germany. He's also building a global network, forging connections with doctors worldwide and working on translations to ensure his work reaches as many people as possible. He's actively seeking funds to launch a Galactosialidosis clinical trial. In a collaborative effort, he has partnered with the Sialidosis community to raise funds for an AAV9 gene therapy, which would benefit both the Galactosialidosis and Sialidosis communities.

FUNDRAISING, DONATIONS AND GRANTS

Giving Season Fundraiser

ISMRD held a Giving Season fundraiser from November 12, 2024 - December 31, 2024, with a campaign of emails and social media posts on Facebook, Instagram, and LinkedIn. featuring some of the glycoproteinoses and ISMRD families. The campaign raised over \$6,000.

Facebook Fundraisers

Facebook offers a very simple and effective way to fundraise for ISMRD. On your birthday, Facebook asks if you would like to hold a birthday fundraiser. Once you say yes, you can nominate ISMRD as your chosen charity, and the rest is easy. This seems to be a fairly new Facebook feature. Four of our Board members combined raised over \$1,600 for their birthdays in 2024.

Instagram Fundraisers

In honor of his sister who passed from Alpha-Mannosidosis, Houtan Yaghmai-Hasti, requested in lieu of flowers that donations be sent to ISMRD. Houtan raised over \$3,200 through Instagram to honor his sister's legacy.

CouponBirds

CouponBirds is a useful and regular fundraiser for ISMRD. Donations are small, but steady, and we hope that they will increase as more of our members begin to utilise CouponBirds. Join CouponBirds [here](#).

Donations

Thank you to all those who donated to ISMRD in 2024:

Mary Alice Abbot	Amy Elton	Berfin Ozturk
Pat Baird	Forsman Family	Paisley-Dew Family
CJ & Betti Barbalaci	Gary & Angela Frangoules	Rosemary Saunders
Serkan Bastas	Deborah Glass	Elizabeth Savoca
Jan Bateman Perkins	Dave & Judi Glenn	Tom Scurria
Linda Bell	Kaya Guven	Dave & Barb Shields
Debbie Bruce	Esra Hosain	Rhonda Skipper
Amy Buchanan	Cihan Kama	Dianne Smith
Filiz Bulut	Daria Karlsen	Kathy & Eric Stanek
Cagdas Canbolat	Eren Kepez	Diana Sweeney
Santiago Cerna	Atilla Kopan	Nazmije Thaqi
Philip Cufaro	Abby Lindenberg	Kathleen Tyree
Barry Dickman	Steve LoCastro	Margaret White
Wayne Diluigi	David Maxwell	John Wolfe
David Dollins	Ailsa Morgan	Woolley Family
Bryan Durnan	Anita Muonio	Houtan Yaghmai-Hasti
Lisa Ehret	Paul Murphy	Maxwell Forsman Investments
Tali Elfassy	Libia Elena Nahas	The Philadelphia Phillies
Eray Eltan	Eva Nordskog	Rosanti Flowers

Grants

ISMRD received **two grants from Chiesi Global Rare Diseases**:

- \$9,500 to upgrade the website, and
- \$6,000 for community outreach.

We are very grateful to Chiesi GRD for these generous and valuable grants, which allowed us to modernise our website, attend conferences, maintain software for the ISMRD Doctor Database and host an Alpha-Mannosidosis webinar.

COMMUNICATIONS

Newsletters

Over 20 newsletters were sent to members in 2024, with a focus on family stories and promoting all nine of the Glycoproteinoses. A quarterly newsletter was sent every three months. Other e-blasts were sent out for Rare Disease Day, a new website, condolences for families as loved ones passed away, and a press release for Mucopolidosis II research projects funded by ISMRD.

Social Media

An ISMRD Instagram account was created in February 2024. In March 2024, ISMRD created a Facebook Schindler Disease page for families to connect. Utilization of the public Facebook page and disease pages were maintained.

Rare Disease Day Campaign

For a week leading up to Rare Disease Day, ISMRD sent an email blast out to our subscribers each day with a rare disease fact and featuring different kids with Glycoprotein Storage Diseases each day. During this campaign, we had six of nine diseases represented. On Rare Disease Day we made mention of the nine Glycoprotein Storage Diseases we represent.

25 Years

On March 10th, 2024 ISMRD celebrated 25 years since the creation of this organization. It's been 25 years of community and advancements in research, and a few treatment options being available. To celebrate this milestone, ISMRD included this in the March Newsletter and also posted on social media accounts about what ISMRD has

done. To commemorate this milestone anniversary, ISMRD's Communication Officer, Sarah Forsman created a new logo featuring the ISMRD penguins with "25". This will stay up until the 25th year is completed.

New ISMRD Website

The ISMRD website was upgraded through work with Holly from Daypack Digital, creating a new modern version of the prior ISMRD website. The project work was from March 2024 until August 2024. This project was made possible thanks to a grant from Chiesi Global Rare Diseases.

New Printed Materials

With the help of ISMRD's volunteer graphic designer, Debbie Bruce, a tri-fold brochure was created to showcase ISMRD's Mission, Vision, history and diseases. Also, an Individual information handout page was created for Fucosidosis.

PATIENT ADVOCACY

We have advocated for increased awareness, equitable access to healthcare, and supportive policies for individuals with Glycoprotein Storage Diseases. Our **new partnerships** in 2024 include:

- SkyHope Patient Air Lift Services
- Metabolic Support UK
- Galactosialidosis Network
- Lost Enzyme Project (Beta-Mannosidosis)

ISMRD also became a **founding member** of the **Global LSD Collaborative**.

PATIENT SUPPORT

We have provided invaluable resources and support services to patients and families, empowering them to navigate the complexities of their conditions. Our **initiatives** include:

- Connecting with newly diagnosed families to provide support and resources, and, where possible, linking them with similar families.
- An upgraded website with more information and family stories.
- A webinar with Alpha-Mannosidosis families to enable them to share their experiences, challenges, and triumphs, and support each other.
- A partnership with Probably Genetic to identify undiagnosed American Alpha-Mannosidosis patients earlier in their diagnostic journey and offer them free whole-exome sequencing. Probably Genetic also tests for most of the Glycoproteinoses.

CONFERENCES AND OTHER BOARD DEVELOPMENT

ISMARD was represented at several key conferences and events throughout the year:

- **WORLDSymposium** in San Diego, California (February 4-9, 2024): Attended by President Carolyn Paisley-Dew, Treasurer Danielle Forsman, Communications Officer Sarah Forsman, and Board Member Cagdas Canbolat.
- **NORD Rare Impact Awards** in Los Angeles (June 2024): Attended by President Carolyn Paisley-Dew, Treasurer Danielle Forsman, and Communications Officer Sarah Forsman.
- **Lysosomal Disease Summit** in Melbourne, Australia (October 2024) and **Rare Voices Australia Biennial Summit** in Brisbane, Australia (November 2024): Attended by President Carolyn Paisley-Dew.
- **NORD Breakthrough Summit** in Washington, D.C. (October 2024): Attended by Treasurer Danielle Forsman and Communications Officer Sarah Forsman. They also participated in NORD's Annual Member Meeting.

- **Ultragenyx Rare Entrepreneur Bootcamp** in Novato, California (November 12-14, 2024): Attended by Board Member Abby Lindenberg.
- **Chronicle of Philanthropy “Planning a Year-End Fundraising Strategy”** (July 18, 2024): Attended by Communications Officer Sarah Forsman.

In May 2024, several **Alpha-Mannosidosis** families and President Carolyn Paisley-Dew joined a three-hour **virtual Advisory Board meeting**. This joint Chiesi/ISMRD meeting provided an opportunity for families across the U.S. to share their journeys and connect with one another.

Attendance at these conferences and events is crucial for several reasons: it keeps board members current on the latest research and developments in rare diseases, provides opportunities to network and form important connections, and supports the overall professional growth of the Board.

FINANCIAL STATEMENTS

Income Statement for the 2024 Calendar Year

Attachment A

Balance Sheet as of December 31, 2025

Attachment B

ISMRD CONTACT DETAILS

Address:

1302 Lincoln Ave Ste 205
San Jose, CA 95125
USA

Mailing Address:

PO Box 683
Turnersville, NJ 08012
USA

E-mail: info@ismrd.org

Website: www.ismrd.org

Income Statement (Profit and Loss)
International Society for Mannosidosis & Related Diseases
For the year ended December 31, 2024

Income	
Contributed Income	
Corporate & Foundation Grants	15,500.00
Donations directed by Individuals	6,914.20
Donations directed by Companies	3,885.00
Donations - PayPal	6,186.57
Donations - Facebook (Meta)	1,716.82
Donations - Website Click & Pledge	3,120.00
Donations - Misc	6,000.00
Total Income	43,322.59
Gross Profit	43,322.59
Operating Expenses	
Advertising & Marketing	11,054.24
Charity Registration	70.00
Bank & Merchant Fees	374.37
Bereavement Flowers	70.02
Conferences & Meetings	10,322.00
Consulting & Accounting	3,000.00
ISMRD 2025 Conference Deposits	30,193.50
Membership Fees	150.00
Postage & Shipping	200.00
Software & Database	647.25
Total Operating Expenses	56,081.38
Operating Income	-12,758.79
Net Income	-12,758.79

Statement of Financial Position
International Society for Mannosidosis and Related Diseases
As of December 31, 2024

DISTRIBUTION ACCOUNT	TOTAL
Assets	
Current Assets	
Bank Accounts	
Business Adv Fundamentals - 0576 - 1	76,043.59
Total for Bank Accounts	\$76,043.59
Accounts Receivable	
Other Current Assets	
Total for Current Assets	\$76,043.59
Fixed Assets	
Other Assets	
Total for Assets	\$76,043.59
Liabilities and Equity	
Liabilities	
Current Liabilities	
Accounts Payable	
Credit Cards	
Other Current Liabilities	
Total for Current Liabilities	
Long-term Liabilities	
Total for Liabilities	
Equity	
Opening balance equity	0.00
Retained Earnings	88,802.38
Net Income	-12,758.79
Total for Equity	\$76,043.59
Total for Liabilities and Equity	\$76,043.59