Fall 2026

Fall 2025

THE RAY

The Association for Glycogen Storage Disease

President's Message

As we look back on the past few months, I am filled with gratitude and pride for what our community accomplished together at the 2026 AGSD Annual Conference in Cleveland. Hosting this year's event in my hometown made the experience especially meaningful, and the turnout exceeded all expectations. We welcomed a record number of attendees, including many first-time participants and newly diagnosed families.

We were additionally fortunate to have perfect weather, which made our annual group photo and our Topgolf event all the more memorable. Even more valuable were the connections made during the social events—often one of the most treasured parts of the conference. These moments remind us why gathering as a community matters so deeply.

This summer also brought exciting news for the GSD community. In August, Ultragenyx announced FDA approval of Genglycose, a new treatment for GSD Ia. This milestone offers hope for improved quality of life for individuals with Ia, and it also signals something larger: research and clinical trials are possible—and progressing—for all types of GSD. Moments like this reinforce the importance of advocacy, collaboration, and continued investment in scientific discovery.

Looking ahead, 2027 will be a unique year for our community. The International GSD (IGSD) organization will host the International GSD Conference in June 2027, held in the Netherlands. With a major global gathering already planned, the AGSD Board felt it made sense to take a 'bye year' from hosting our own annual conference. Therefore, please note that AGSD will not hold a conference in 2027. We will return refreshed and ready in June 2028, and once details are finalized, we will share them on our website.

For information about the 2027 International Conference, you can visit: https://www.igsd2027.com/home.

I also want to acknowledge the tremendous generosity shown by our community this year. While the annual conference is our largest fundraiser—and most funds raised go directly toward making the event possible—we were thrilled to see record sales in our on site store, several significant donations, and even an impromptu live auction featuring a one of a kind hand crocheted liver created by Ambreen Mian. Thanks to this collective support, AGSD was able to provide financial assistance to more than 10 individuals and families to attend the conference and award nine secondary school scholarships to deserving applicants.

Thank you for your continued dedication, compassion, and belief in our mission. Our community is strong because of each of you,

Warm regards,

Kim Burney
President, Association for Glycogen Storage Disease

DID YOU KNOW?!

WE HAD **244** ATTENDEES
(OUR BIGGEST YET!)

29 SPEAKERS

OVERALL, OUR PARTICIPANTS TRAVELED FROM **37** DIFFERENT STATES, AND **10** DIFFERENT COUNTRIES!

AND THERE WERE **12** DIFFERENT TYPES OF GSD REPRESENTED

A Conference Perspective written by Conference attendee - Joan Eisele-Cooper (Type 15)

The 48th annual AGSD conference brought our community together once again, creating opportunities for connections between families, doctors and researchers. It is the third year I have attended along with my adult daughter, Jocelyn.

I have one of the ultra rare GSDs. It took me many years after my diagnosis was confirmed at the Mayo Clinic to finally choose to participate. I am so glad I did.

It is here each year that I have found a community of doctors and specialists from many different fields that I can actually listen and speak to, as well as other families and individuals affected by this disease. It is here that I have found personal connection and understanding and it is here that I have found hope, as I hear about the new breakthroughs being investigated and learn important information that will help me to live a better life.

Every year the conference closes with a community event. This year we all shared a meal and some fun activities at a local Topgolf. It was here that I had an opportunity to chat with participants about their experiences.

A first time attendee said, **"It is very profound how they have gathered everyone together into a community with professionals to empower the GSD community-to be encouraged and optimistic about the future."**

Other Opined :

"It was great to be a part of the AGSD community. You're learning from others, especially for recently diagnosed parents, exchanging contacts and socializing."

"It's so nice not to be alone."

"It's always heartwarming to see so many new families and even more heartwarming to see young adults thriving in so many areas."

"This community is like a family where we share information and caring."

"The providers have great compassion and collaboration. This community is real."

"It is a relief to know that I'm not the only one to have surgery - that it is not a personal failure."

"This is an isolating disease. It was so good to talk to doctors, to make friends, to build connections that will support you for the rest of your life."

"It is really good to meet people with the same struggles."

And finally, **"It's a nice break from life."**

Join the AGSD Community United for Progress in Glycogen Storage Disease

Whether you are a patient, a caregiver, a clinician, a researcher, or an advocate, the Association for Glycogen Storage Disease (AGSD) is here to support, connect, and empower you.

Why become a member?

- **Affordable Dues:** Just $25/year — because support should be accessible to all.
- **Scholarship Opportunities:** After 1 year active members are eligible for post-secondary education scholarships.
- **Discounted Conference Registration:** Enjoy member rates for our annual AGSD Conference — a hub for learning, networking and community.
- **Your Voice Shapes Our Events:** We consider member locations when planning our annual conference.

Join AGSD today and be part of a community that understands, uplifts and advocates.

Donations Thank You for Fueling Our Mission

The Association for Glycogen Storage Disease (AGSD) is a fully volunteer-run organization. Every program we offer — including our annual conference — is made possible through the generosity of donors like you.
We simply couldn't do this work without your support.
With heartfelt appreciation, we thank the following for their recent donations:

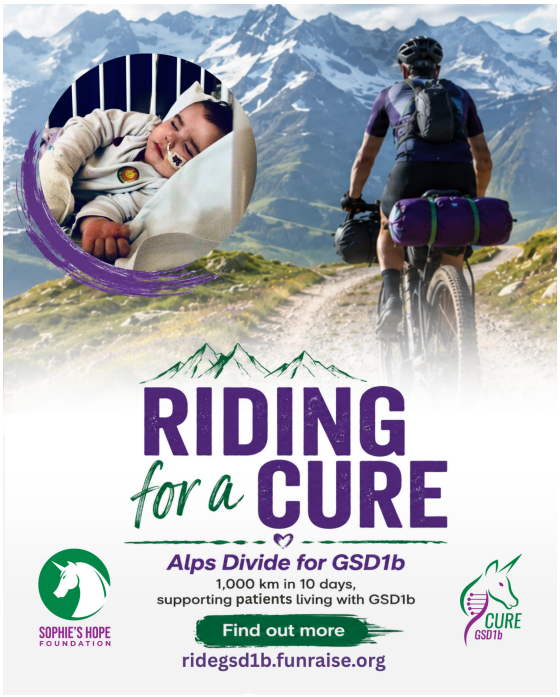
Patricia Oetzel
(in Memory of Marylyn Neary)
Carol Taylor
Pete Bailey
John Adams
Deidre McElroy
(in memory of Marylyn Neary)
Rodney Haun
(in memory of Marylyn Neary)
Jewish Communal Fund
Novartis Corp
Carol Gaylord

Carol Gaylord
Dwight Koeberl
Dona La Costa Gomez
Deborah Kozak
David Watkins
Payal Rastogi
Richard & Nancy Tapia
(in memory of Marylyn Neary)
Heather Edwards
Nick Buck
Sophie Tepperman

Your contributions help us connect families, empower patients, educate clinicians, and advance research — all while keeping our events accessible and inclusive.

Stories from our GSD Community

GSD1B Ride Goes the Distance!



What started as a 1,000 km bike riding challenge across the Alps became an incredible show of strength and support for the GSD1b community. In August, Phil and his friend Toby took on the demanding 10-day journey from the Mediterranean Sea to Lake Geneva, dedicating each day of the ride to a different person living with GSD1b.

Their goal was to raise awareness and support research and families through Sophie's Hope Foundation/cureGSD1b—and thanks to the incredible generosity of the GSD community, they surpassed their fundraising goal and raised more than $10,000!
Every mile ridden and every dollar donated represents a community pushing forward together toward better treatments and, ultimately, a cure.
Congratulations to Phil and Toby, and thank you to everyone who supported this amazing effort!

Learn more about their Journey !

The Cornstarch Chronicle

Amanda Boudreaux Roser

We're excited to share a wonderful new resource created by a member of our GSD community. This special children's book was written by a mom about her son, who lives with GSD-0 and ketotic hypoglycemia (KH).
After struggling to find a children's book that explained KH, GSD, and why her son drinks cornstarch, she decided to create one herself. The result is a story filled with adventure, education, and awareness, designed to help children better understand why some bodies work differently while showing that those differences don't define who they are.
The book is also a wonderful tool for families, schools, and caregivers to help start conversations and build greater understanding of GSD-0 and KH.
We're proud to share a resource created by and for our community, and hope you'll help spread the word!
📖 Find the book on Amazon

All proceeds from the book will benefit Ketotic Hypoglycemia International, helping support awareness and the KH community.

Stories from our GSD Community Cont...

Camp Hole in the Wall Gang - Family Camp

This October, the Maryland location is hosting a Family Camp specifically for families affected by metabolic disorders, including Glycogen Storage Disease (GSD)!

October 23–25, 2026
Queenstown, Maryland

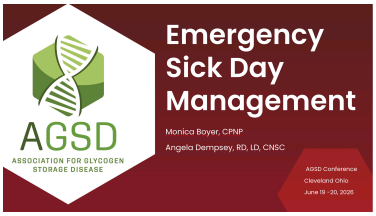
The weekend is designed for the whole family and includes a variety of traditional camp activities in a safe, supportive environment. Family Camp is free of charge, including meals and lodging.

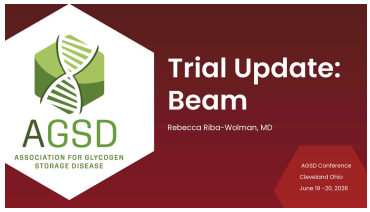
If you're a GSD family, this could be a wonderful opportunity to connect with other metabolic families and experience a little bit of Camp magic! ✨
Check out the Camp's website for eligibility and more information:
Hole in the Wall Gang Camp – Maryland Family Camps

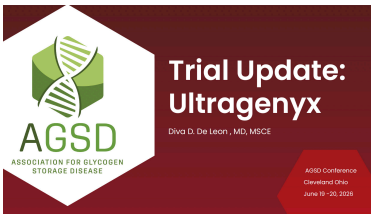
Help us Stay in the Know!

Have GSD news, events, research updates, fundraisers, or community stories to share?

We want to hear from you!
Reach out to us through Messenger or email us with your news. We'd love to help keep our GSD community connected and make sure everyone stays up to date on the amazing things happening within our community!







Conference Videos

Check out these NEW videos from the 2026 AGSD Conference!!

We have made great efforts this year to bring you some recordings from our speakers who presented at the 2026AGSD conference in Cleveland, OH..

We aim to record key talks to bringing the most updated GSD news to those who were not able to attend. View them all now on our YouTube page!

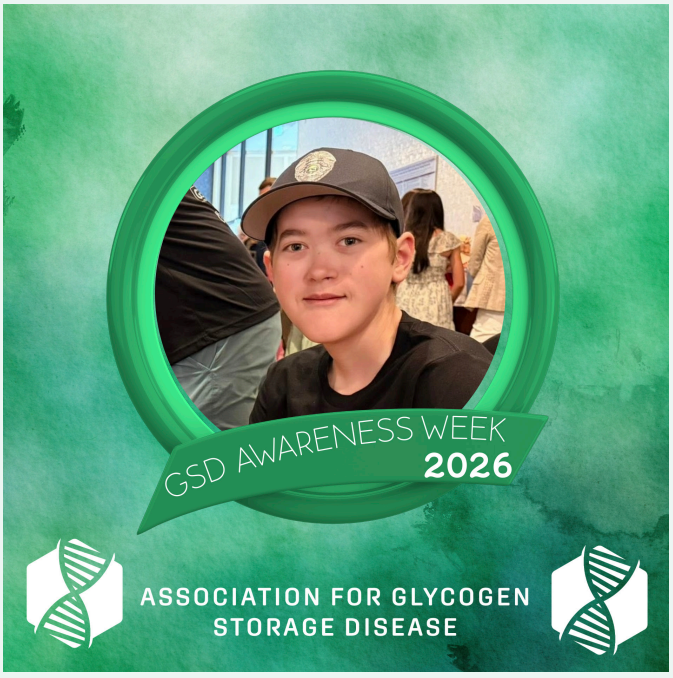
www.youtube.com/@agsd-agsd

GSD Awareness Week October 19th - 25th

As we get closer, keep an eye out for ways to participate, help spread awareness, and celebrate our GSD community. We'll be sharing more information and social media posts as Awareness Week approaches, so be sure to follow along!

You can also send in your photo through Facebook Messenger to participate. Just a reminder: we will not be sending out photo frames until a couple of days before GSD Awareness Week, so please don't worry if you haven't received one yet.

We hope you'll join us, participate, and help us make GSD Awareness Week a wonderful celebration of our GSD community!



Drug Trial Updates

Ultragenyx

- **August 19th**
  - Ultragenyx Announces U.S. FDA Approval of GENGLYCOS™ Gene Therapy, the First-Ever FDA-Approved Treatment Designed to Treat the Underlying Cause of Glycogen Storage Disease Type Ia (GSDIa) Learn more Here
    - Visit https://www.genglycos.com/ for more information on safety , prescribing, and
    - treatment centers

- **September 1st, 2026**
  - Ultragenyx Announces the Publication of a Successful 96-Week Randomized, Placebo-Controlled Trial with Crossover Treatment of GENGLYCOS™ (also known as DTX401) AAV Gene Therapy in GSDIa in The Journal of Inherited Metabolic Disease

Beam Therapeutics

- Beam Therapeutics is continuing to enroll adult participants in a Phase 1/2 trial evaluating BEAM-301 for the potential treatment of GSD1a. There are three clinical study sites open across the United States. Please contact clinicalinfo@beamtx.com for more information.
  - https://beamtx.com/pipeline_candidates/beam-301/

Other Clinical Trials

- Accuracy of Home Lactate Meter and Accu-chek Glucometer in Patients With Glycogen Storage Disease. More information here .
- Sleep and Quality of Life in Patients With Glycogen Storage Disease on Standard Versus Modified Uncooked Cornstarch. More Information here.

to view more ongoing trial, those recruiting or complete please visit. www.clinicaltrials.gov

Visit our website under "News and Articles" for more up to date information.

New: GSD Research & Publications

In collaboration with our Scientific Advisory Board, we're excited to introduce a new segment to our newsletter highlighting recent publications related to GSD. Our goal is to keep you informed and up to date on the latest research, findings, and developments in the GSD community.

GSD Type	Publication	Summary
Ia	Turner-Bowker DM et al. Trial Interviews to Explore Glycogen Storage Disease Type Ia Patient Experiences Following Gene Therapy. J Health Econ Outcomes Res. 2026 Feb 12;13(1):39-47. 155666. PMID: 41694477	This qualitative study explored GSD Type Ia patient experiences following gene therapy through trial interviews. The interviews aimed to understand patient perspectives on gene therapy as an emerging treatment option. Free article
II	Regmi N et al. Plasma glial fibrillary acidic protein (GFAP) is a biomarker for central nervous system involvement in infantile-onset Pompe disease. EBioMedicine. 2026 Jan;123:106096. PMID: 41483684	This study identified plasma glial fibrillary acidic protein (GFAP) as a biomarker for central nervous system involvement in infantile-onset Pompe disease. This discovery would potentially allow monitoring neurological complications in these patients, potentially allowing earlier detection and intervention for CNS manifestations that may not be adequately addressed by current enzyme replacement therapies. Free article
IV	Taylor A et al. Predicting subtypes of glycogen storage disease type IV: Challenges of hepatic subtypes and genotype-phenotype correlation. Mol Genet Metab. 2025 Dec;146 (4):109293. PMID: 41308240	The authors presented a 4-year-old patient with GSD IV and performed a comprehensive literature review of GSD IV genotype-phenotype analysis to address the challenges in predicting hepatic phenotypes, noting that liver disease progression exists on a spectrum with some patients developing progressive/severe hepatic forms while others stabilize with attenuated disease. Free article
IX	Baronio E et al dynamics in glycogen storage disease type IXa with novel PHKA2 variants: insights from our experience and a comprehensive review of the disease spectrum. Hormones (Athens). 2025 Dec;24(4):1209-1216. PMID: 40866742	This article presents novel PHKA2 variants in GSD Type IXa and provides insights into glucose dynamics through comprehensive disease spectrum review. The authors describe their clinical experience with patients with novel PHKA2 variants and conducted a comprehensive literature review of the disease spectrum, as the severity of hypoglycemia varies considerably between patients and subtypes, with some patients experiencing minimal metabolic derangement while others require intensive dietary management similar to GSD Ia. Free article
V	Erdoğan B et al. A disease that is difficult to predict: regional distribution and phenotypic, histopathological and genetic findings in McArdle disease. J Pediatr Endocrinol Metab. 2025 Sep 9;38(11):1187-1194. PMID: 40960216	This study examined regional distribution and phenotypic, histopathological, and genetic findings in McArdle disease, emphasizing that it is "a disease that is difficult to predict." The authors' findings highlight the challenges in predicting disease severity and outcomes based on genotype alone, underscoring the need for individualized management approaches. Free Article

In Memoriam: Areeg Hassan El-Gharbawy, MD, DSc

Areeg Hassan El-Gharbawy, MD, DSc
Professor of Pediatrics,
Duke University School of Medicine
Member, AGSD Scientific Advisory Board

We honor the memory of Dr. Areeg ElGharbawy, a cherished member of the AGSD community who passed away on May 12, 2026. Dr. ElGharbawy was a respected physicianscientist whose career was devoted to improving the lives of children and adults with inborn errors of metabolism. Her compassion, expertise, and unwavering dedication made her a trusted clinician, mentor, and advocate for families affected by rare diseases.

Dr. ElGharbawy completed her medical and graduate training at Cairo University and pursued advanced training in internal medicine, endocrinology, and genetics at the Medical College of Wisconsin, the National Institutes of Health, UCLA Medical Center, and Duke University. At Duke, she became a leader in patient care, research, and education.

Her scientific contributions spanned innovative therapies, biomarkers, and genebased approaches for inherited metabolic disorders, with a special focus on glycogen storage diseases. Known for her extraordinary depth of knowledge and her passion for teaching, she mentored trainees across disciplines and was widely regarded as a brilliant and generous educator.

Dr. ElGharbawy served on the AGSD Scientific Advisory Board and was a valued and frequent speaker at our annual conferences. Her legacy lives on through the patients she cared for, the colleagues and students she inspired, and the scientific advancements she helped shape. She will be deeply missed by the AGSD community.

See you in 2028!

We're taking a break from hosting our conference next year so that we can support and attend the International GSD Conference. We're excited about the opportunity to connect with the broader GSD community and support this important event.

Stay tuned for the big reveal of our 2028 conference destination! We can't wait to share where we'll be gathering next.

If you'd like to learn more about the International GSD Conference, please visit

https://www.igsd2027.com/home

Member HIGHLIGHTS

Back to School, GSD Rockstars! 💙

As we head into fall, we want to wish all of our GSD families a wonderful start to the new school year!

We know life with GSD can come with its own unique challenges, and back-to-school season can bring a few extra ones. After hospital stays, strict regimens, endless labs, and plenty of cornstarch, getting that backpack packed and walking through those school doors is a BIG deal! 💙

We're cheering on all of our GSD Rockstars as they take on another school year. Here's to a year filled with new adventures, new memories, and plenty of reasons to celebrate!