August 2025

Honoring Our Past, Empowering Our Future

This August marks a meaningful milestone—the AMDA turns 30.

We invite you to take a moment with us to reflect on the story of how the Acid Maltase Deficiency Association came to be, the tireless work of our late President Tiffany House, and the collective progress we've made as a community. Our featured article, "*Three Decades of Impact: Celebrating 30 Years of the AMDA,*" is more than a timeline—it's a tribute to a legacy of advocacy, collaboration, and hope.

Read the Full Article

As we honor the road behind us, we also look ahead—and this month, we're focusing on **education**. Whether you're preparing for IEP meetings with the help of COPAA, or looking to better understand pediatric Pompe care through Duke's upcoming virtual session, we've gathered several resources this month to support you and your family through the back-to-school season.

Disclaimer:

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AMDA Welcomes New Director and Lead Social Media Manager

The Acid Maltase Deficiency Association (AMDA) is proud to announce two important updates to our leadership team:

Andrea Faris has been named Director, and **Tara Mancine** has joined as Lead Social Media Manager. Both bring deep passion, personal commitment, and professional expertise that will strengthen our mission to serve the Pompe disease community.

Andrea is moving into this new leadership role with a strong sense of purpose and personal connection to our mission. Tara joins us with fresh energy and valuable expertise. Together, they bring a powerful combination of lived experience and strategic insight that will help shape the future of our organization and deepen our impact within the Pompe community.

DIRECTOR

Andrea Faris

A passionate advocate and sibling ally whose life was deeply shaped by growing up alongside Tiffany House (beloved late AMDA President and Pompe Disease patient). From an early age, Andrea witnessed the challenges and strength that come with living with

LEAD SOCIAL MEDIA MANAGER

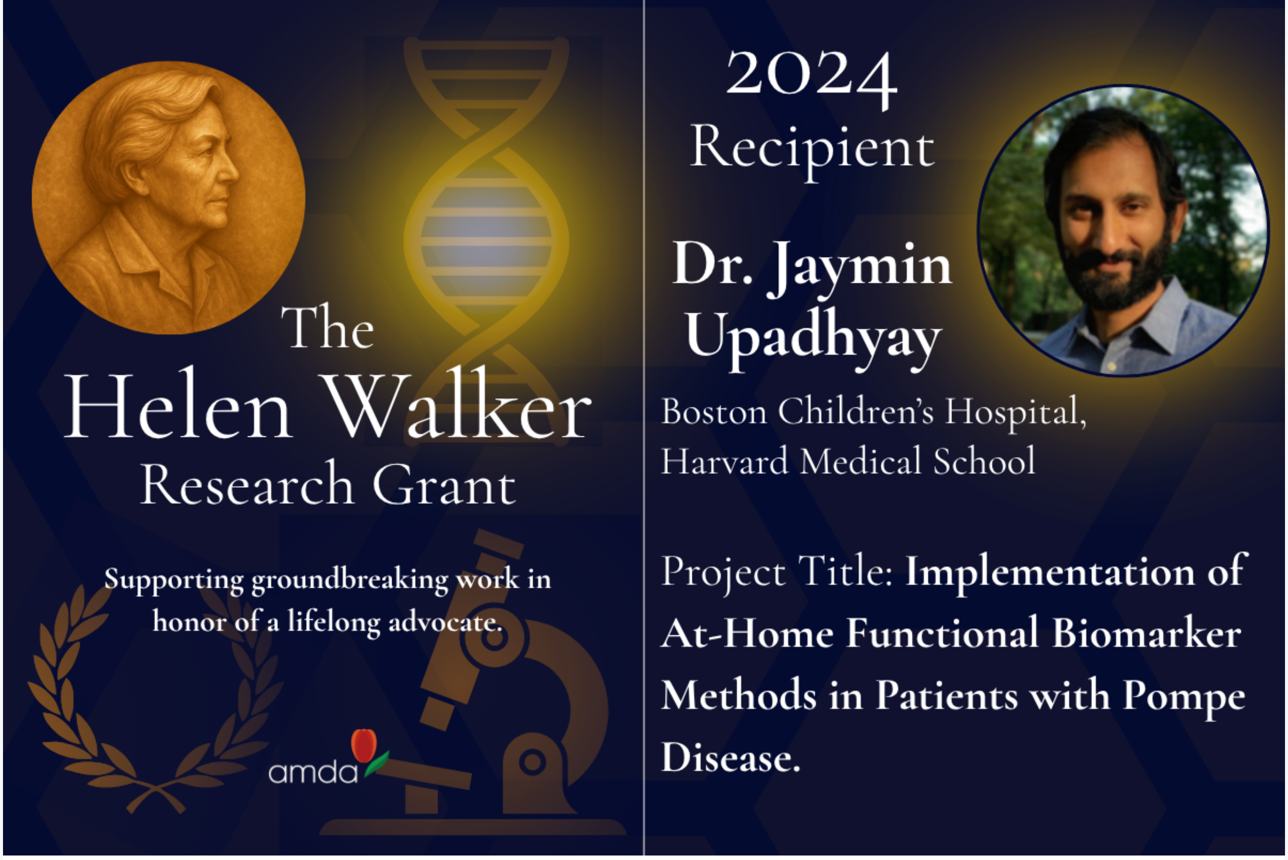
Tara Mancine

With over 15 years of experience as an entrepreneur and business owner, she brings a unique blend of strategic insight, brand development, and audience engagement expertise to her role in the nonprofit space. She's a rare, progressive condition. Her experience adapting to changing family dynamics and supporting Tiffany’s journey has fueled her commitment to raising awareness, building community, and creating space for patients, families, and sibling voices in rare disease conversations. Today, Andrea continues to honor Tiffany’s legacy and support families walking a similar path.

led marketing and communication efforts across multiple channels, developing educational content and impactful campaigns that connect with diverse audiences and drive long-term engagement.

Now, as the lead social media manager for the AMDA (Acid Maltase Deficiency Association), she's applying her skills to amplify awareness, build community, and support advocacy for individuals affected by Pompe disease. She approaches digital strategy with the same passion she brings to everything she does: rooted in research, driven by purpose, and designed to connect meaningfully with real people.

Whether it’s creating compelling content, distilling complex medical concepts into accessible language, or mapping out long-term engagement strategies—she thrives where impact meets creativity.



2024 Helen Walker Research Grant Recipient Announcement

Dr. Jaymin Upadhyay of Boston Children's Hospital, Harvard Medical School

The AMDA is pleased to announce the recipient of the 2024 Helen Walker Research Grant!

This year's grant has been awarded to Dr. Jaymin Upadhyay of Boston Children's Hospital, Harvard Medical School for his proposed research project titled:

"**Implentation of At-Home Functional Biomarker Methods in Patients with Pompe Disease.**"

Learn more about Dr. Upadhyay's below.

Read More

Let's Talk Back-to-School: A Pompe Parent Conversation

You're invited to our **8th Talking With Your Pompe Peeps session** on **Wednesday, August 13, 2025 at 9 AM CST / 10 AM EST**. This live discussion will be moderated by **Colleen Sackos**, a mother of three and a seasoned educator with over 15 years of classroom and administrative experience. Colleen also led last year’s back-to-school session and brings both professional insight and lived experience as a Pompe parent navigating the school system.

*****This session will be recorded and posted on the AMDA YouTube Channel, by attending, you are giving the AMDA permission to post your image on it’s channel.***

EVENT DETAILS:

Title: Back to School with Pompe: Real Talk for Real Parents

Date: Tuesday, May 27, 2025

Time: 9 AM CT / 10 AM ET

Moderator:
Colleen Sackos

Summary:
This time of year is a stressful time for every parent, as kids all over the country start preparing to go back to school. Add on the stress of managing Pompe disease, and it can easily become overwhelming. You are not alone! Come bringing your questions, concerns, and even your expertise,

and let's talk them out. Whether it’s setting up 504 plans, educating school staff, or balancing academics and medical care—this is a supportive space for real conversation and community connection.

Register for This Session

Moderator Biography

Colleen Sackos

Colleen Sackos is a proud mother of three and educator with over 15 years of experience as a teacher and administrator in the elementary school setting in both Oregon and Arizona. Her youngest son, Axel, has Infantile Onset Pompe Disease and just finished kindergarten. She lives in Happy Valley, OR, and is grateful to be a part of the Pompe community: together we are strong!

By the Numbers

AMDA This Quarter

- 1 Webinar Produced
- 1 Community Fundraiser
- 2 Internal Strategy Meetings
- 2 Non-Industry Meetings
- 9 Cross-Organization Efforts
- 10 Industry Dialogues

Behind the Numbers: A Snapshot of Our Work

From bringing our community together for a powerful webinar and fundraising event, to sitting down with researchers and rare disease collaborators around the world, AMDA has been actively advancing conversations that matter.

This past quarter, we hosted our *Talking With Your Pompe Peeps* support session, connected with 10 pharmaceutical leaders, and contributed to 9 different working groups, policy coalitions, and research efforts.

Our goal remains the same: to advocate, educate, and amplify the Pompe community’s voice — one meeting, one collaboration, and one story at a time.

POMPE NEWS

Pompe Disease Added to Texas' Newborn Screening Panel in August

We're celebrating a historic win for the Pompe community! Texas has officially added Pompe disease to its newborn screening panel—a milestone that reflects years of advocacy and coincides with AMDA's 30th anniversary. This progress honors the lasting impact of Tiffany House, who played a vital role in advancing newborn screening efforts in the state.

Read More

Upcoming Opportunities to Learn and Connect—Virtually!

Two major virtual events are on the horizon this fall: Duke's

Virtual Pediatric Pompe meeting and WORLDFair 2025, University of Minnesota's Advance Therapies Department's annual conference on lysosomal diseases. Whether you're looking to deepen your understanding of Pompe or connect with the broader rare disease community, both events are free to attend online—and registration is now open.
Images link to registration pages.

SAVE THE DATE

DUKE POMPE DISEASE CLINICAL AND RESEARCH PROGRAM PRESENTS:

Pediatric Conference
August 23rd, 2025
Fully virtual

Agenda and Registration Info
Coming Soon

VIRTUAL ONLY

Duke's 2025 Virtual Pediatric Pompe Conference

The **Duke Pompe Disease Clinical and Research Program** will host its **2025 Pediatric Pompe Conference virtually on Saturday, August 23, 2025—**and **registration is now open.**

This year's meeting will be held **exclusively online,** and **no clinic appointments will be scheduled** during the event.

University of Minnesota
Driven to Discover®
Crookston Duluth Morris Rochester Twin Cities

WORLDFair™ 2025

September 19, 2025 I 10 AM – 2 PM CST
Pacific Standard Time: 8:00 AM until noon
Eastern Standard Time: 11:00 AM until 3:00 PM

Minnesota Landscape Arboretum

3675 Arboretum Drive, Chaska, MN 55318

WORLD Fair 2025 will be a hybrid meeting, which will invite patients, caregivers and healthcare providers from around the world to attend remotely through zoom.

HYBRID

WORLDFair 2025

A Gathering Focused on Lysosomal Diseases

The **11th Annual WORLDFair**, hosted by the **Advanced Therapies Department** at the University of Minnesota, will take place on:

Date: September 19, 2025

Time: 10:00 AM - 2:00 PM CST

Location:
University of Minnesota Landscape Arboretum
3675 Arboretum Drive, Chaska, MN 55318

The conference will feature the latest updates on **Pompe disease research and clinical care**, and provide a space for individuals and families in the Pompe community to connect with one another and leading experts.

Date: Saturday, August 23, 2025

Location: Virtual only

For updates on this and future Duke Pompe conferences, follow the **Duke Pompe Disease Clinical and Research Program** on Facebook.

Don't miss this opportunity to tune in and learn from one of the leading programs in the field.

This annual event brings together patients, families, healthcare providers, researchers, and industry representatives to exchange knowledge, share experiences, and explore the latest developments in lysosomal disease research and treatment.

- Remote attendance via Zoom
- Box lunch provided for in-person attendees

Intended Audience:

- Individuals diagnosed with lysosomal diseases
- Family members, caregivers, and friends
- Medical professionals and researchers
- Industry professionals
- Anyone with an interest in lysosomal diseases

Program Highlights Include:

- Educational presentations on lysosomal disorders and emerging treatments
- Patient and caregiver stories

- Networking opportunities across the lysosomal disease community
- A presentation from the **Pompe Warrior Foundation** on dietary intervention, supplementation, and ongoing research in Pompe disease

A full program with speakers and topic details can be found here.

2025 Pediatric Conference Registration

WORLDFair 2025 Registration

COMMUNITY NEWS

Heading Back to School?

COPAA Can Help You Advocate with Confidence

As students prepare to return to the classroom, families of children with Pompe disease are also preparing education plans that ensure the right support is in place. The **Council of Parent Attorneys and Advocates (COPAA)** is an invaluable resource for navigating IEPs, 504 Plans, and understanding your child's educational rights.

COPAA empowers parents through advocacy training, legal resources, and a national network of experts committed to protecting the civil rights of students with disabilities. Their goal: to help every child access a free, appropriate, and inclusive education.

Learn more at www.copaa.org and start the school year equipped with the tools you need to advocate effectively.

Grant's Giants Providing Infusion Role Play Kits to Pompe Children Receiving ERT

Grant's Giants is providing Infusion Role Play kits to children with Pompe disease that are currently receiving ERT or are about to start ERT. Each kit includes a stuffed animal with a port or IV access and everything needed to role play an infusion.
Email grantsgiants@gmail.com with any questions!

Grant's Giants is providing Infusion Role Play kits to Pompe children that are currently receiving ERT (enzyme replacement therapy) or are about to begin ERT worldwide. Each kit will include a stuffed animal with a port or IV access and everything needed for a child to role play an infusion. For children under the age of 3 the kit must be used with adult supervision due to the small pieces and strong magnets.

> "When a child is able to engage in role-playing an

infusion, it helps normalize the process and makes it feel much less intimidating. Currently, enzyme replacement therapy, delivered via IV infusion, is the only treatment for Pompe disease. That’s why it’s crucial to normalize this lifelong treatment for children, helping them feel more at ease with it." - Alison Breitbarth of Grant's Giants

Request Play Kit

SAVE THE DATE:

Pompe Alliance Women's Retreat

Calling all women in the Pompe community! The *Pompe Alliance Women's Retreat* is back—and it's more than just a weekend getaway. It's a powerful, transformative experience created just for women living with or impacted by Pompe disease.

Whether you're a patient, caregiver, or advocate, this retreat is about *you*—your strength, your story, and your voice.

What to expect:

- Guided workshops on **mental health, advocacy, and personal growth**
- A safe, supportive space to **connect and share your story**
- Wellness activities, art sessions, and moments to recharge
- A weekend full of **laughter, learning, and sisterhood**

Registration is now open!

For questions, contact Pompe Alliance directly at pompealliance@gmail.com.

You can also visit the *Pompe Alliance Facebook page* for more details and updates.

Let's come together to celebrate the power of women in rare disease—we hope to see you there!

Registration Page

Astellas FORTIS Trial UPDATE: August 2025

Astellas, a company that researches, develops, manufactures, and commercializes innovative medicines, is sharing an update about our investigational gene therapy treatment called AT845, which is being tested for the potential treatment of late-onset Pompe disease (LOPD). This update is for the Pompe community and responds to a request for information about our current clinical trial from the AMDA.

Currently, Astellas is running a study called FORTIS. This study is in the early stages (Phase 1/2) and is testing AT845 in adults who have LOPD. The main goal of this study is to check if the treatment is safe, and a secondary goal is to see if it works well. So far, Astellas has given the treatment to 11 patients: 2 received a lower dose and 9 received a higher dose. The FORTIS trial is no longer looking for new participants, and Astellas expects to finish testing whether the treatment works by the end of March 2026. This process is called proof of concept.

Additionally, AT845 has received a special designation from the U.S. Food and Drug Administration (FDA) called Regenerative Medicine Advanced Therapy (RMAT). This designation can help speed up the process of developing and reviewing new treatments for serious diseases. It means Astellas can have more regular discussions with the FDA as we continue to evaluate the safety and efficacy of this potential treatment for adults with LOPD. Astellas also wants to thank the people who participated in the FORTIS trial, as their involvement is crucial for the research.

It is important to note that the safety and effectiveness of AT845 have not been proven. There is no guarantee that AT845 will be approved for use or available for sale in any country.

Formulation Survey on Pompe Disease

This 15-minute online survey, conducted by InCrowd on behalf of a pharmaceutical company, aims to understand experiences with Pompe disease and current treatment gaps. If the patient is under 18, a caregiver must complete the survey.

The survey is currently open to patients in Italy, Japan, Germany, the UK, the Netherlands, and the US.

Take Survey

Monthly Observances

Back-to-School Month: August is Back-to-School Month—a perfect time to ensure children with Pompe disease are supported and set up for success. For families navigating school with an Individualized Education Program (IEP), preparation is key. Be sure to check out our *Talking With Your Pompe Peeps* session on education plans, and explore valuable resources like the Center for Parent Information and Resources (CPIR) and the Council of Parent Attorneys and Advocates (COPAA). Plus, don't miss Pompe Canada's helpful blog post on how to explain Pompe disease to teachers—a great tool for building understanding and support in the classroom. Together, we can help students with Pompe disease thrive both in and out of the classroom.

National Immunization Awareness Month: National Immunization Awareness Month is a reminder of the critical role vaccines play in protecting our health and communities. For those living with Pompe disease, staying up to date on immunizations can be especially important, as infections may pose greater risks. Talk with your healthcare provider about recommended vaccines, including flu and pneumonia shots. Protecting yourself also helps protect others.

Weekly Observances

International Assistance Dog Week | August 4 - 10: International Assistance Dog Week celebrates the incredible impact service dogs have on the lives of people with disabilities

—offering support, independence, and companionship. For individuals with Pompe disease, a service dog can assist with mobility, alerting, and everyday tasks. In our *Talking With Your Pompe Peeps* series, don't miss the session *Have You Ever Considered Getting a Service Dog?* to hear real experiences and learn what to consider when exploring this option. These remarkable dogs do more than assist—they empower.

Daily Observances

National Lazy Day | August 10: Honestly... we were too lazy to finish this write-up. You get it. #NationalLazyDay

National Nonprofit Day | August 17: Today we celebrate the mission-driven nonprofits that uplift, advocate, and support communities. If you've been impacted by the work of a nonprofit—maybe even one like the AMDA—consider donating to your favorite today. Every gift helps us continue serving the Pompe community. Donations can be made on our website. #NationalNonprofitDay #SupportPompeProgress

National Grief Awareness Day | August 30: Grief takes many forms and journeys, especially in rare disease communities like ours. Today is a moment to recognize those who are grieving, to offer support, and to remind each other that no one has to face it alone. #GriefAwarenessDay #PompeCommunityCares

#PompePower

Passionate
On
Matters
Pompe
Everyday

https://amda-pompe.org

30th
AMDAVERSARY

Acid Maltase Deficiency Association

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