September / 2026

AMDA Newsletter

Related by Muscle, Not by Name

September is Muscular Dystrophy Awareness Month, and this year we're using it to celebrate something the Pompe community sometimes forgets: we belong to a much bigger family. This month's newsletter features Talking With Your Pompe Peeps (TWYPP) moderator and Muscular Dystrophy Association (MDA) Ambassador, Dwayne Wilson, starts the countdown to Grant's Giants' 4th Annual Pompe Family Meet Up, and previews our September webinar with the Duke team on brain involvement in Pompe disease. No matter how muscle weakness has touched your life, we're glad you're here with us this month.

This Month's Highlights

Related by Muscle, Not by Name

Muscular Dystrophy Awareness Month tends to bring Duchenne and Becker to mind first, and understandably so. But Pompe disease has its own place in that same family. The MDA includes acid maltase deficiency in its network of covered diseases, and many Pompe families already share MDA care centers, mobility equipment programs, and clinical specialists with people carrying a very different diagnosis. Different root cause, same lived experience of progressive muscle weakness.

No one embodies that overlap better this month than Dwayne Wilson. Diagnosed with late-onset Pompe disease (LOPD) in 2018, Wilson serves as both an MDA Ambassador and a Pompe Champion with Amicus, and he's moderating this month's TWYPP session on resilience (See AMDA Updates below). He didn't choose a side between these two communities; he's been showing up for both.

Whichever diagnosis brought you to this newsletter, Muscular Dystrophy Awareness Month is a chance to recognize you're not navigating muscle weakness alone. That's the whole idea behind this issue.

Read Dwayne's Story

AMDA Updates

September Webinar: Biomarkers of Central Nervous System Involvement in Pompe

Disease

WEBINAR • THURSDAY, SEPTEMBER 24, 2026 • 10 a.m. CT / 11 a.m. ET

Back in July, we shared early research on GFAP, a blood biomarker that may help doctors detect brain involvement in Pompe disease. Join us for our September webinar, featuring the Duke team behind that work. Kristen Hagarty-Waite, PhD, RDN, LDN, a postdoctoral associate in Duke's Division of Medical Genetics, will explain why central nervous system symptoms can persist even with enzyme replacement therapy (ERT), which does not cross the blood-brain barrier. Two emerging biomarkers, GFAP and neurofilament light chain (NfL), may help monitor central nervous system involvement, especially in infantile-onset Pompe disease (IOPD).

Learn More

Register Now

Talking With Your Pompe Peeps (TWYPP): The Importance of Being Resilient

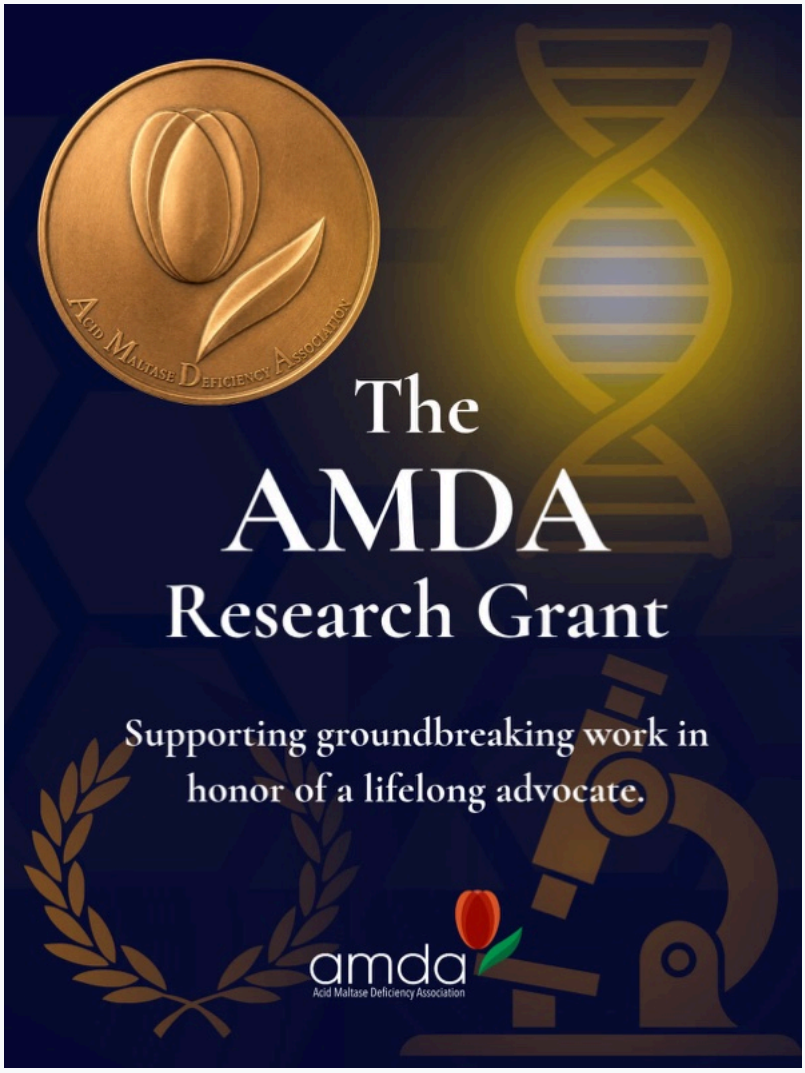
TWYPP • TUESDAY, SEPTEMBER 29, 2026 • 6 p.m. CT / 7 p.m. ET

This month's TWYPP session turns to the emotional side of living with Pompe disease: staying resilient through diagnosis, loss, physical changes, and the everyday challenges that come with a rare, progressive condition. Moderated by Dwayne Wilson, a member of our community and an MDA Ambassador, this is a space to share experiences and hear how others have found strength and hope.

Note: *This session will be recorded for internal review and possible educational use. If you'd rather not be recorded, you're welcome to turn off your camera, use an anonymous display name, or participate by chat only. This is a peer support space, not medical advice.*

Learn More

Register Now



The AMDA Research Grant Returns to Its Original Name in Honor of Tiffany L. House

The AMDA is renaming its research grant program back to the AMDA Research Grant. This was the name it carried when the program was first established in 2010. Then, the program was renamed the Helen Walker Research Grant in 2013, honoring founding Australian Pompe Association president Helen Walker. The change honors Tiffany L. House, who served as president of the AMDA and chair of the International Pompe Association (IPA) before her passing on May 25,

2025. Walker's legacy remains an essential part of the AMDA's history and will continue to be honored in the AMDA's work.

> *"Returning the AMDA Research Grant to its original name is how we carry her legacy forward: through research and the people it helps, not just a name on a program," says Andrea Faris, AMDA Director.*

Read the full announcement for the story behind the name, and visit Tiffany's memorial page to share a memory, a photo, or simply your name on her Memory Wall.

Read the Full Announcement

Visit Tiffany's Memorial Page

AMDA Research Grant Program: Call for Letters of Intent

The AMDA is now accepting Letters of Intent for the 2026-2027 AMDA Research Grant Program. One grant of up to $150,000 will be awarded to support research that advances the understanding, diagnosis, treatment, and management of Pompe disease, with basic, translational, clinical, and patient-centered research all eligible for a 12- or 24-month project period. Full guidelines, key dates, and application instructions are on our website.

Read the Full Announcmeent and Apply

Pompe Kitchen – Apple Cinnamon Baked Oatmeal Cups

As the weather starts to cool, this month's Pompe Kitchen recipe brings warm spice to your morning routine. Apple Cinnamon Baked Oatmeal Cups are a make-ahead breakfast you can freeze and reheat one at a time, perfect for mornings when energy is in short supply.

Community & Events

WORLDFair 2026: A Gathering for the Lysosomal Disease Community

THURSDAY, SEPTEMBER 18, 2026 • 9:30 a.m. – 4:00 p.m. CT | UNIVERSITY OF MINNESOTA LANDSCAPE ARBORETUM, CHASKA, MN

Mark your calendar: the 12th annual WORLDFair Annual Meeting is just weeks away, bringing together patients, families, health care providers, and researchers from across the lysosomal disease community for a day of education on lysosomal disease and current treatments, plus a chance to connect. Can't attend in person? You can register to join remotely via Zoom. A box lunch will be provided for in-person attendees.

Note: This resource is shared for your information. It is provided by an independent organization and is not affiliated with or endorsed by the AMDA.

Learn More and Register

Grant's Giants 4th Annual Pompe Family Meet Up

OCTOBER 2–4, 2026 | BRADFORD WOODS, MARTINSVILLE, IN

The countdown is on. The team at Grant's Giants Pompe Nonprofit is bringing the community together for their 4th Annual Pompe Family Meet Up, and it promises to be a weekend worth traveling for. The event is designed for pediatric Pompe patients (both IOPD and LOPD) and their families, though adult patients are warmly welcome. Set at Bradford Woods, a universally accessible camp about 30 minutes from Indianapolis International Airport, the weekend will be filled with community, fun, and education. Travel stipends are available because Grant's Giants wants every interested family to be able to experience this weekend together.

Note: This is a third-party event hosted by a fellow Pompe disease nonprofit. Inclusion does not constitute an endorsement by the AMDA.

Register Now

Connor Cares: Sibling Support Grant

Siblings carry their own quiet weight in this journey, and Grant's Giants' Connor Cares initiative was built with them in mind. The grant offers a one-time $250 award to help siblings (under 18 years old) of children with Pompe disease receiving ERT access enrichment, self-care, educational resources, and a special experience of their own during their family's treatment journey. Families in the U.S. can apply through the online form.

Note: This is a third-party resource offered by a fellow Pompe disease nonprofit. Inclusion does not constitute an endorsement by the AMDA.

Learn More and Apply

Advocacy & Science

Boston Children's Hospital Is Recruiting for a Home-Based Pompe Study

Boston Children's Hospital is looking for participants for a home-based research study on Pompe disease. Eligible participants must be 5 to 21 years old with an IOPD or LOPD diagnosis who have been on ERT for at least 3 months. The study is safe and non-invasive, and includes a speech and muscle assessment, cognitive and motor tasks, and breathing and swallowing tests. Participation is free of charge and involves four visits over one year (about once every three months). The first visit happens in person at your home, with the remaining visits done remotely over Zoom. Families who complete all visits can receive up to $275 in compensation.

Note: This is a third-party research study shared for your information. Inclusion does not constitute an endorsement by the AMDA.

Interested?
Contact Raquel Van Gool (Raquel.VanGool@childrens.harvard.edu | 617-301-0920)
or Jaymin Upadhyay (Jaymin.Upadhyay@childrens.harvard.edu | 917-736-1541).

View Study Flyer

Beyond the Diagnosis: Rare Care Series (Give an Hour)

Give an Hour's continuing education–accredited Beyond the Diagnosis training series explores the emotional and psychological realities faced by individuals, caregivers, and families navigating life with a rare condition. Through trauma-informed, evidence-based modules, mental health professionals gain practical tools to recognize the emotional and cultural factors shaping rare disease mental health needs, strengthen therapeutic communication, and better understand the systemic barriers families face. The eight-part series offers up to twelve continuing education hours for NBCC, ASWB, and APA participants through the end of 2026.

Next Live Module: September 2, 2026 | 11:30 a.m. CT / 12:30 p.m. ET | 1.5 Continuing Education Hours — Bridging the Gaps in Care: Rare Disease Mental Health Strategies

Modules 1 through 5 are already available on-demand for anyone catching up.

Note: This is a third-party resource shared for your information. Inclusion does not constitute an endorsement by the AMDA.

Explore the Series

Face the Five: Signs for Rare Caregivers to Notice

Rare caregiving can take a significant emotional toll. Give an Hour's Face the Five resource helps families and loved ones recognize when a caregiver may be struggling, and offers simple language for how to check in, respond with care, and connect them to support.

Note: This is a third-party resource shared for your information. Inclusion does not constitute an endorsement by the AMDA.

Download the Resource

Observances

Monthly Observances

Muscular Dystrophy Awareness Month: This month, we're thinking about everyone whose muscles carry more than their share of the load, whatever the diagnosis on the chart. Pompe disease has its own place in the wider community, and this issue's Featured Content digs into what that connection actually looks like.

Daily Observances

Fight Procrastination Day | September 6: Procrastination looks different when the task itself takes real energy to face, whether that's returning a call from the clinic, filling out another form, or having a hard conversation with someone you love. Today isn't about guilt for what's still on your list. It's a gentle nudge to pick just one small thing and let that be enough.

National Grateful Patient Day | September 7: For many in the Pompe community, gratitude often has a name attached to it. Few names come up as often as Dr. Priya Kishnani's, whose decades of work at Duke have shaped how Pompe disease is diagnosed, treated, and understood. Today is a good day to say thank you to her and to every clinician who shows up for this community.

Positive Thinking Day | September 13: It's hard to stay positive with a progressive disease, and pretending otherwise doesn't help anyone. Positive Thinking Day isn't about forcing optimism; it's a small nudge to notice the moments that still feel good, even inside a hard season.

National Pancake Day | September 26: In honor of Tiffany, who never turned down a stack of pancakes, today we're flipping a batch and thinking of the people whose warmth still shapes how we gather.

https://amda-pompe.org

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