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Acid Maltase Deficiency Association

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## February / 2026

# AMDA Newsletter

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**Rare Disease Month is a reminder that no one should navigate a rare disease alone.**

The AMDA is proud to announce its formal and emboldened mission statement.

These principles have guided us since our inception, and we are proudly carrying these ideals forward, shining a light on connection, advocacy, and the everyday strength of the Pompe community. This February, we'd like to outline ways to participate in Rare Disease Day, join upcoming community conversations, and stay plugged into research-focused learning.

## AMDA Updates

15TH ANNUAL

amda

PCMA

PULL FOR POMPE

Sporting Clay Fundraiser for Pompe Disease Research
Benefiting AMDA & families living with Pompe disease

**DATE:**
MAY 2, 2026

**NATIONAL SHOOTING COMPLEX**
5931 ROFT RD SAN ANTONIO, TX 78253

**TIME:**
8:00AM - 2:00PM

**REGISTER & SPONSOR**
PULLFORPOMPE.ORG

**SPECIAL TOURNAMENT**
**TEAM CHALLENGE**
PRIZE $5,000, TEAM NAME TROPHY AND TRADE BRAGGING RIGHTS

The Precast Concrete Manufacturers Association (PCMA) invites you to a day of family-friendly fun at a sporting clay event welcoming all ages and experience levels.

Honoring the Legacy
Tiffany Laurel House
1983–2025

READY TO TAKE AIM FOR A CURE?

## 2026 PCMA Pull for Pompe Fundraiser

We are excited to announce that the 15th Annual PCMA's Pull for Pompe will take place on Saturday, May 2, 2026, from 8 a.m. to 2 p.m. CST at the National Shooting Complex in San Antonio, Texas!

The Pull for Pompe clay shooting event is designed for all ages and ability levels and is a family-friendly fundraiser to support Pompe disease research. All proceeds from the Pull for Pompe fundraiser will go towards the AMDA's 2026 Pompe Disease Research Grant!

Register today

Download flyer

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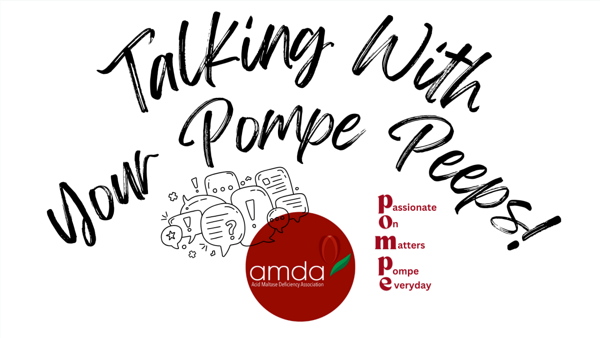
## The AMDA is Seeking Infantile-Onset Pompe Parents, Your Voice Matters



We know this journey can feel isolating, and sometimes it's easier to talk to other parents who truly "get it."

Our Talking With Your Pompe Peeps (TWYPP) series is a community-led support meeting where we discuss all aspects of living with Pompe disease. We are looking for Infantile-Onset Pompe parents who would be willing to

- Share their story;
- Participate in small-group conversations;
- Help shape topics that matter most to IOPD families;
- Be part of a supportive, judgment-free space,

You don't need to be an expert.

You don't need perfect words.

You just need lived experience and a willing heart.

If you are interested, please contact: Marsha Zimmerman (marzim50@gmail.com)

If you've ever thought "I wish I could talk to someone who understands this life," this space is for you!

## Community & Events

### Streaming For Rare – Rare Disease Day 2026

The National Organization for Rare Disorders (NORD) is looking for **creators, streamers**, and **community members** to join them for a charity streaming initiative in support of Rare Disease Day called **Stream for Rare**!

“On February 28, supporters across the community will host a live stream doing what they love and invite their audiences to donate directly to the National Organization for Rare Disorders (NORD).

By registering your live stream event, you will be able to create a personalized fundraising page and submit your event for review to be published on our Rare Disease Day website.

How it works in three easy steps:

1. Register your stream
2. Stream what you enjoy
   - NORD is a charity partner on Twitch, Tiltify, YouTube, & Meta
3. Invite your audience to donate during the stream

Your stream helps reduce isolation, amplify rare voices, and show that rare does not mean alone."

Register your stream

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## Talking With Your Pompe Peeps Session 11: Our Adaptations with Pompe Disease

**Date**: Wednesday, February 11, 2026 • 12 p.m. CST / 1 p.m. EST

**Moderator**: George Fox

Living with Pompe disease often means finding new ways to adapt. In this **Talking With Your Pompe Peeps** session, George, a dad of a young adult diagnosed with Pompe disease, facilitates a peer-led conversation about the everyday adaptations families and individuals make to support fuller, more meaningful lives. Participants are invited to share what has worked for them and learn from one another's experiences.

**Note:** *This discussion is for informational and community support purposes and is not medical advice.*

**Recording Notice**: *This session will be recorded for internal review and possible educational use. If we share a recording publicly (including YouTube), we will post an edited version focused on general information and community tips, and we will remove participant personal stories and identifying details when possible. If you prefer not to be recorded, you're welcome to keep your camera off, change your display name, and/or participate via chat. If you have concerns, please contact us in advance.*

Learn more and register

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## Upcoming Webinar: Making Sense of Research Studies and Registries

**Date**: Thursday February 19, 2026 • 7 p.m. CST / 8 p.m. EST

**Speakers**: Karmen Trzupek & Ryan Colburn

Have you heard about research studies and patient registries for Pompe disease? These studies are a critical tool in capturing the patient experience, which improves disease understanding, management, and treatment options.
Join this session to learn about the different types of research studies and patient registries for Pompe disease. What are the differences between them? How is the data shared and used? Karmen Trzupek (Senior Director of Scientific Programs at Global Genes) and Ryan Colburn (Principal at odimm) cover what you may want to consider when choosing to participate, a few of the historical registries that have contributed to progress in Pompe, and share an innovative patient-led registry, Progress4Pompe, where patients own their data and choose how to share it.

**Disclaimer**: *The content provided in this webinar is for informational purposes only and does not constitute an endorsement by the AMDA.*

Learn more and register

## Message from Raquel van Gool to the AMDA and the Pompe Community

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95 followers

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We were recently honored to receive a message from Dr. Jaymin Upadhyay sharing words written by his PhD student, Raquel van Gool, as she prepares to defend her doctoral thesis. Raquel has been deeply involved in Pompe disease research, and her acknowledgements reflect the heart behind the science.

In her own words, Raquel expressed gratitude to the patients and families who placed their trust in research, including parents who continue to give even after unimaginable loss. She spoke of children for whom life has been unfair, of brave parents, and of a community whose shared experiences continue to move science forward. Her words are a powerful reminder that progress in rare disease research is built on courage, connection, and collaboration.

She also acknowledged the role of patient advocacy organizations, including Acid Maltase Deficiency Association, for helping connect families, clinicians, and researchers. A special tribute was shared for Tiffany House, whose dedication to the Pompe community continues to inspire researchers and advocates alike.

Pompe disease is rare. Support shouldn’t be.
We are proud to stand alongside researchers and families as a trusted resource, helping move studies forward and supporting patient outreach that leads to real progress.

If you ever have questions or want to learn more about research opportunities, you can always reach out to us through our website or on social media. We are here to help.

#pompepower #togetherwearestrong #amda #PompeDisease #RareDiseaseResearch PatientAdvocacy

view all comments

Add a comment...

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## Pompe Alliance Women's Retreat

**Pompe Alliance**
2026-01-22

🌿 We’re just three months away from the very first Pompe Alliance Women’s Retreat! 🌿
A few spots are still available — register soon so you don’t miss this special weekend of connection, rest, and empowerment.

✨ Retreat highlights include:
- Friday night meet-and-greet dinner
- A hands-on cooking demonstration
- Massage therapy
- Stretching and movement sessions tailored for Pompe patients
- A visit to the beautiful Cleveland Botanical Garden

💚 Financial assistance is available, and the retreat is open to women living with Pompe disease and women caregivers.

Secure your spot using the link below. We can’t wait to welcome you.
https://forms.gle/nUgiiPnEeML8c7MWA

JOIN POMPE ALLIANCE FOR OUR

Women's Retreat

Friday-Sunday | April 24th - 26th | Crowne Plaza Cleveland at Playhouse Square

Use the link below to register!

https://forms.gle/tbP7jeEZjy
cPjr4x5

Pompe Alliance

Like Comment Share

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## Save The Dates

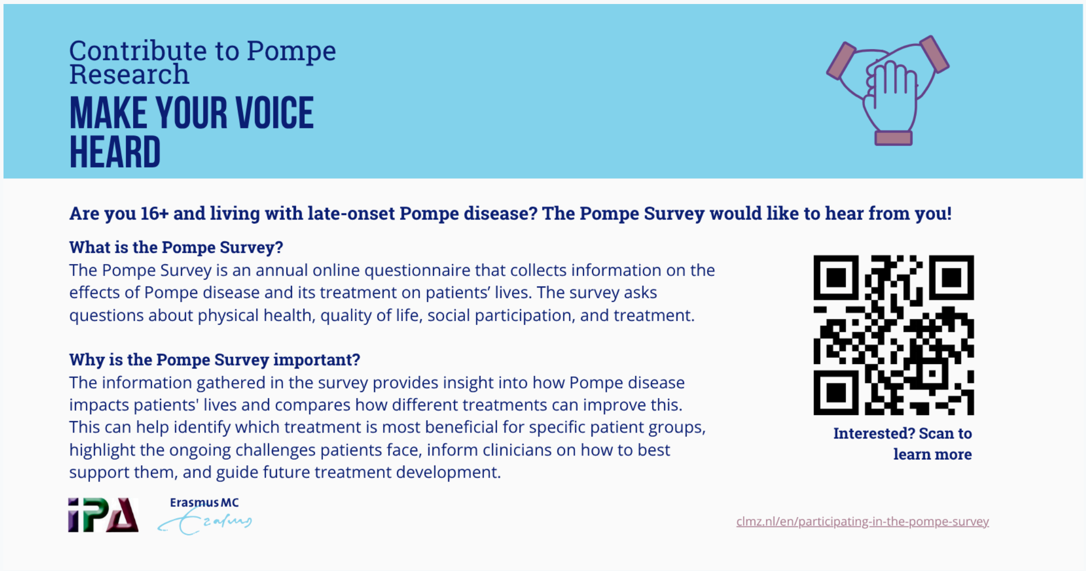
Rare Disease Day | February 28

International Pompe Day | April 15

Pull for Pompe | May 2

# Advocacy & Science

## Erasmus MC Pompe Survey Now Supports E-Consent!



Contribute to Pompe Research

**MAKE YOUR VOICE HEARD**

**Are you 16+ and living with late-onset Pompe disease? The Pompe Survey would like to hear from you!**

**What is the Pompe Survey?**
The Pompe Survey is an annual online questionnaire that collects information on the effects of Pompe disease and its treatment on patients' lives. The survey asks questions about physical health, quality of life, social participation, and treatment.

**Why is the Pompe Survey important?**
The information gathered in the survey provides insight into how Pompe disease impacts patients' lives and compares how different treatments can improve this. This can help identify which treatment is most beneficial for specific patient groups, highlight the ongoing challenges patients face, inform clinicians on how to best support them, and guide future treatment development.

**Interested? Scan to learn more**

iPA Erasmus MC

clmz.nl/en/participating-in-the-pompe-survey

The Pompe Survey is an annual online questionnaire that collects information on the effects of Pompe disease and its treatment on patients' lives. The survey asks questions about physical health, quality of life, social participation, and treatment. Participating helps ensure that patient experiences remain central to Pompe research.

The Pompe Survey Team is especially seeking participants from the UK, US, Australia, and Canada. Interested in participating? Registration only takes minutes!

Complete the form here to receive more information and enroll.

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## NORD Rare Impact Awards Nominations Now Open!

The National Organization for Rare Disorders (NORD) has just opened nominations for its 2026 NORD Rare Impact Awards! Do you know someone making a difference for people with rare disorders? This is your chance to celebrate those who inspire change in the rare disease community.

**Deadline**: Nominations close Friday, February 13, 2026, at 11:59 p.m. EST.

“The Rare Impact Awards honor individuals and organizations driving progress in advocacy, research, policy, and patient support.

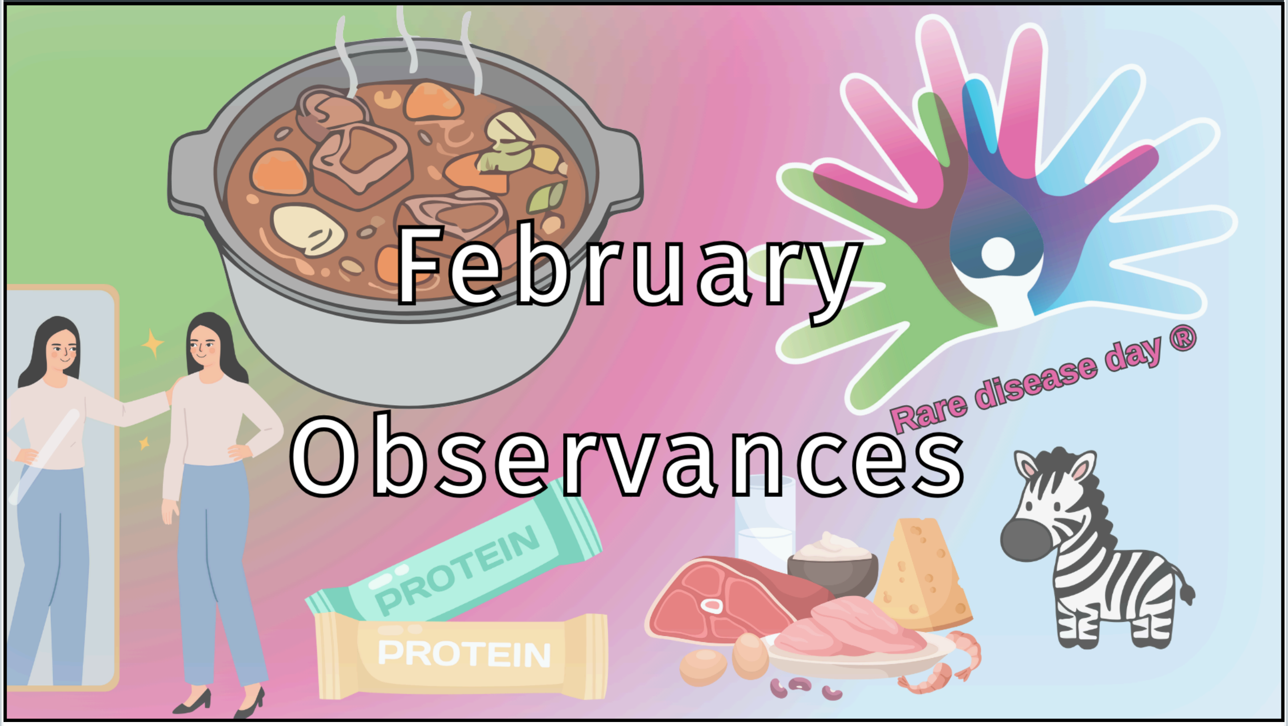
**Rare Impact Awards® categories are as follows:**

- Policy Changemaker
- Youth Leader
- Community Champion
- Scientific and Medical Trailblazer
- Abbey S. Meyers Leadership Award

Your nomination helps shine a spotlight on those whose work transforms lives and strengthens the rare disease community. We need your voice to ensure these changemakers receive the recognition they deserve."

Nominate your hero

## Observances



### Monthly Observances

Rare Disease Month: All month long, we pause to recognize the strength, resilience, and creativity of the rare disease community. It's a time to raise awareness, amplify patient voices, and remind one another that connection and advocacy can move progress forward.

### Daily Observances

National Homemade Soup Day | February 4: There's something grounding about a pot of soup simmering—simple, nourishing, and made with care. Today is a cozy reminder that comfort can be practical, too: a warm meal, a shared recipe, or a little extra kindness for yourself.

Self-Love Day | February 13: Self-love doesn't have to be big or dramatic; it can be small, steady, and quiet. Today is a gentle nudge to speak to yourself with compassion, honor your needs, and choose one thing that supports your well-being.

National Protein Day | February 27: Protein often shows up in everyday ways —meals, snacks, and choices that help support strength and energy. Today is a helpful prompt to check in on what fuels you and to talk with your care team about what "nourishment" looks like for you.

Rare Disease Day | February 28: Rare Disease Day is a global moment to shine a light on rare conditions and the people and families living with them. It's also a reminder that visibility matters, and that sharing a story, showing support, or taking one small action can help the entire community feel seen.

***Disclaimer:***
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**AMDA**

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