International Pompe Association

IPA year in review

A year of loss, resilience and renewal

For the global Pompe community, 2025 was one of the most difficult years in the history of the International Pompe Association. We mourn the passing of two extraordinary leaders: Maryze Schoneveld van der Linde and Tiffany House. Both shaped the IPA profoundly with their vision, tireless dedication, and unwavering commitment to patients worldwide. Tiffany once said about Maryze that “it is impossible to say how much she has done for the Pompe Community.” The same is equally true for Tiffany herself. They connected patients, researchers, clinicians, and industry; they inspired progress; and they fought for better futures for all Pompe families. Their loss leaves a deep void. But their legacy guides the path ahead.

Your IPA Board

Reorganization, new leadership and growth

Following these losses, the IPA undertook a structural reorganization. Alongside the restructuring of communications, the IPA elected new leadership. The new Chairman of the IPA is Brad Crittenden (Pompe Canada). Brad brings many years of international patient-advocacy experience and was already deeply involved in IPA communications. His leadership stands for continuity, transparency, and a renewed commitment to strengthening global collaboration across the Pompe community.

Newly elected to the Board of Directors were Andrea Faris, (AMDA, United States) photo left, Dimitri Kalaitzis (Stichting Ziekte van Pompe, The Netherlands), middle und Annic Kolbrück (Pompe Deutschland e.V., Germany), right.

Although turnover in a board is always happening, we have had a number of recent changes. As a global organization, it is important to us to really have global leadership, so we are proud to have representation from seven countries: Australia, Canada, Italy, Germany, the Netherlands, the United Kingdom and the United States.

The new board members were elected by the members of the IPA during the Annual General Meeting (AGM) held in November 2025.

A warm welcome to our newest member, META from the Czech Republic. The organization, represented by Katerina Uhlikova, supports patients with rare lysosomal storage disorders such as Gaucher disease, Fabry disease, and Pompe disease.

Progress despite adversity

1. International Pompe Day 2025 – "Every Move Counts!"

The Run–Walk–Roll campaign energized the global community and highlighted the importance of staying active while new therapies are being developed.

2. Expansion of the Community Advisory Board (CAB)

The CAB,Tiffany's vision, grew in its activities, held new meetings with industry partners, and continued to influence patient-centered clinical research. New members are being recruited to ensure broader global representation.

3. Advances in research and therapy pipelines

The report details the rapid global development of:

- next-generation ERTs,
- substrate-reduction therapies,
- and multiple gene- and cell-based approaches.

The IPA maintained active dialogue with Amicus, Sanofi, Aro Biotherapeutics, Shionogi, Astellas, AskBio, Crosswalk, Erasmus MC, and others.

4. Campaigns on dose flexibility and treatment expectations

The IPA continues advocating for flexible dosing options and supporting patients in navigating new treatment choices with realistic expectations.

5. Strengthening the IPA–Erasmus Pompe Survey

The long-running, patient-owned registry was updated, expanded to many languages, and remains crucial as more therapies enter the market. It prevents fragmentation of patient-reported outcomes across multiple commercial registries. In the coming month we will share the first ever annual report of the Pompe Survey. With this report we aim to give patients more insight into the information the survey provides. If you want to know more about the survey click here

6. Restructuring of IPA Communications

After Maryze's passing, IPA communications were reorganized under a new team led by Annic Kolbrück, with Andrea Faris and Brad Crittenden.

This includes:

- more consistent newsletters
- increased social-media presence
- stronger visibility for member organizations
- continued multilingual patient brochures ("Pompe Connections")

This shift marks a renewed commitment to dialogue, transparency, and community engagement. The IPA newsletter for example will be available to all interested parties starting next year. The link to subscribe can soon be found on the IPA website www.worldpompe.org

What 2025 taught us

This difficult year showed us: The IPA is vulnerable. But it is also incredibly strong. The strength of the organization comes from its people: volunteers, patient leaders, national associations, advocates, scientists, and families who contribute their time and voices. With new treatments approaching and multiple therapy options emerging, the global Pompe community needs a strong IPA more than ever. One that speaks with unity and purpose.

A call to all IPA member organizations

The challenges ahead are significant. But so are our opportunities.

The IPA Board issues a clear invitation and encouragement:

Become more active. Contribute. Participate. Engage.

We need member involvement in:

👉 the CAB

👉 international advisory and working groups

👉 the Erasmus Survey

👉 translation support

👉 data-registry initiatives

👉 sharing national activities for global visibility

👉 outreach and communications

👉 patient-education efforts

The losses of 2025 reminded us that leadership can change unexpectedly. But an engaged membership ensures long-term strength.

Looking ahead to 2026

With renewed leadership, a reorganized structure, and ambitious scientific developments on the horizon, the IPA enters 2026 with clarity and determination. Priorities include:

👉 advancing the global independent registry

👉 deepening collaboration with researchers and industry

👉 strengthening communication pathways

👉 expanding patient involvement

👉 supporting national organizations more proactively

The future of Pompe care is full of promise, but it requires all of us.

Global rare impact awards

At Sanofi's inaugural *Global Rare Impact Awards* in Vienna, three exceptional leaders were recognized for their lifelong dedication to rare disease advocacy, including two from the Pompe community:

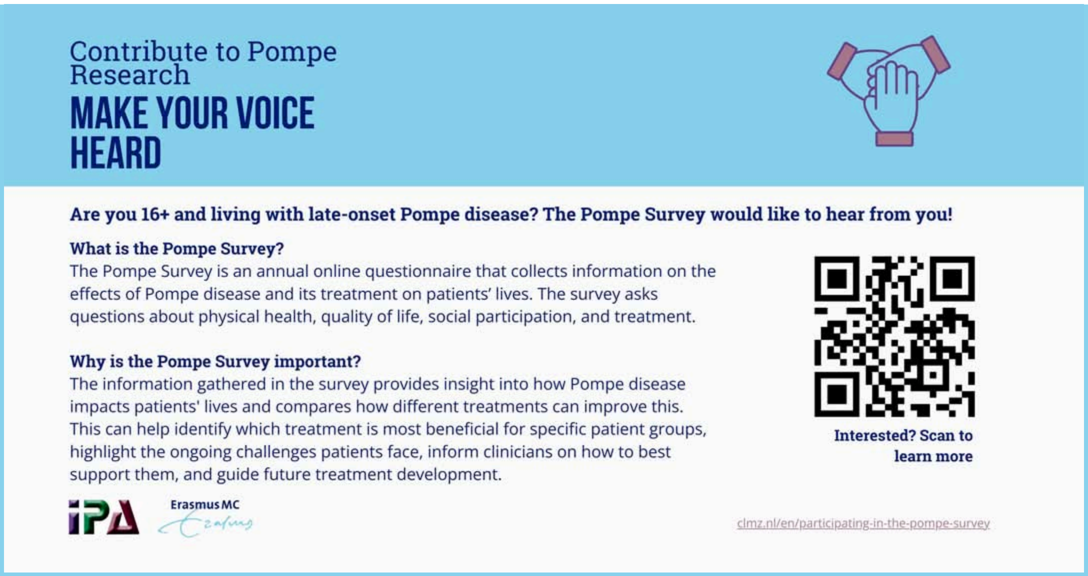
Tiffany House, the AMDA's late president, was celebrated as a visionary leader in patient advocacy whose compassion and dedication set a lasting example for collaborative leadership.

Maryze Schoneveld van der Linde, of the International Pompe Association, was honored for her tireless commitment to improving access to care and strengthening rare disease networks across developing countries.

Dr. I. C. Verma — Known as the "Father of Genetics" in India, Dr. Verma established two groundbreaking genetic centers and pioneered affordable genetic testing. His compassionate access program has helped treat over 300 patients with lysosomal storage disorders. (photo: Sanofi)

The entire board wishes you peaceful holidays and a happy New Year with lots of luck, happiness, and good health!

Together we are strong!



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

International Pompe Association - IPA

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