International Pompe Association
IPA secretariat
c/o VSN, Luitenant Generaal van Heutszlaan 6
3743 JN Baarn
The Netherlands

+31 (35) 6029612
www.WorldPompe.org

Annual Report 2024

Introduction

Exciting advancements continue to evolve within the Pompe community and the IPA remains committed to monitoring these advances and to cultivate relationships with all parties involved in the development of treatments or interventions for Pompe. In addition, we work closely with the Medical/Scientific Community to improve our mutual understanding of Pompe disease and the unmet needs of the Pompe Community. As you read the 2024 Annual Report, it is the hope of the IPA Board that the scientific advances and treatment options that are coming, as well as the IPA's planned collaborations for 2025 (and beyond), will fill you with the same excitement for the future that we have.

Chairman's statement 2024

October 22, 2024 marked the 25th Anniversary of the founding of the IPA. The beginnings of the IPA were a small group of national organization leaders that saw the value in working together to represent the global Pompe Community. When the first meeting was held in 1999, the founders of the IPA set the following mission statement: 1) Campaign for early diagnosis and effective, affordable and safe therapies for Pompe Disease; 2) Strive to provide information and support to all patients, their families and others with interests in Pompe disease.

Specific goals/objectives that we set for ourselves were:

- Stimulate research into the causes, treatment and prevention of Pompe disease
- Stimulate the rapid application of research into the causes, treatment and prevention of Pompe disease
- Promote early and accurate diagnosis and screening programs for Pompe disease
- Support and encourage national organizations to obtain approval and reimbursement for therapies from government bodies and health providers
- Encourage other organizations or individuals to establish a mechanism for all patients to gain access to therapies irrespective of their personal financial status
- Encourage the formation and support the continued development of national Pompe organizations
- Establish and maintain a key position with industry, researchers and individuals with interests in Pompe disease
- Provide educational and informative publications through electronic and other media

Looking back over the last twenty-five (25) years, I am proud to say that the IPA has remained true to our mission statement, and continue to pursue the goals and objectives that we set for ourselves. But it takes all of us working together to achieve progress. Industry alone cannot bring a treatment to market. Neither can patient groups (national or international), or clinicians/scientists. But when we all work together, listen and learn from each other, we can achieve great things.

As we mentioned in the Chair Report last year, it is incredibly rare for a rare disease to have a treatment, let alone treatment options. However, our work is not done. Treatment options are not available globally yet, and better options are still needed. To that end, once again we are fortunate to have scientists that are dedicated to understanding our disease at the most basic levels—that are pushing the envelope of science to find novel and better ways to treat the entire spectrum of Pompe. We are also fortunate to have clinicians who have dedicated their entire careers years to understanding how Pompe affects each of us, and working to improve treatment and management of our disease.

In May 2024, the global Pompe Community came together in San Antonio, Texas for the 2024 AMDA/IPA Pompe Patient and Scientific Conference. At this meeting, the depth and degree of the dedication our expert community has towards Pompe was on full display. From understanding the new natural history of Pompe on treatment, to the effects of newborn screening on patient outcomes (both IOPD and LOPD), to advances in treatments and potential gene therapies, it was a very full weekend of presentations and reports. It was very clear by the end of the Conference that, as a Community, we are not ready to stop looking for better choices, better treatment management, and a better understanding of Pompe. And we are fortunate to be surrounded with clinicians and researchers who feel the same.

In the rest of this report, you will read about the activities that have occurred over the last year, and activities that we believe will be vital to the future of Pompe treatment, knowledge, and disease management. #Together We Are Strong!

2024 International Pompe Day—Every Move Counts!

April 15, 2024 was our 11th Annual International Pompe Day. As we look forward to new treatments like gene therapy, substrate reduction therapy, and more, it is important for patients to stay as healthy as possible while these treatments are being developed. For 2023 International Pompe Day, the IPA focused on staying healthy and active through a campaign called: Every Move Counts. For the 10th International Pompe Day, the IPA Board issued a challenge to the community: show us how "Every Move Counts!"

The replies were inspiring and heart-warming. It was so clear that how each of us works to make sure we keep moving is as unique and diverse as our community. To properly honor these submissions, and share the inspiration that we felt, the IPA had a video made. We hope you are inspired to keep moving—because Every Move Counts!
https://worldpompe.org/international-pompe-day/2024-every-move-counts/

Patient Affiliates

We currently have 67 contacts with patient organisations and individuals, representing approximately 58 countries around the world.

Community Advisory Board

The International Pompe Association (IPA) established its own Community Advisory Board (CAB) for Pompe disease in 2019 as a patient-led initiative, uniquely organized and operated by the Pompe patient community, rather than being industry-driven.

Traditionally, a CAB is a neutral group formed and run by patient advocates to discuss and advise on the latest developments and challenges related to medical research and procedures, facilitating dialogue with research sponsors. CAB members—typically patients or advocates—bring expertise that guides sponsors in clinical program development, individual clinical trials, and other areas beyond research. Importantly, CABs ensure that clinical studies are designed to address the real needs of patients, contributing to higher-quality, patient-focused research.

The IPA CAB currently includes 29 members from multiple countries across three continents, representing a broad spectrum of Pompe disease perspectives. Beginning with 19 members in 2019, the CAB has since expanded with new members joining from the US, the Netherlands, Germany, Hong Kong, Japan, Greece, France, and Australia.

The inaugural IPA CAB meeting took place on October 28, 2019, in San Antonio, Texas, as a multi-company roundtable attended by industry representatives from Spark, Sanofi, Amicus, Audentes, and AskBio. Following the impact of the COVID-19 pandemic, CAB meetings moved online, with one-on-one virtual CAB meetings held with various industry partners throughout 2020 to 2023, including:

- AskBio/Bayer (July 2020 and 2022)
- Sanofi Genzyme (September 2020, June 2021, December 2021, June 2023, and two additional meetings in 2023)
- Avrobio (two meetings in September 2021)
- Maze Therapeutics (2022)

These virtual sessions have proven highly effective and have expanded industry engagement. In 2023, we initiated preparations for more in-person CAB meetings, aiming to continue this approach at the 2024 AMDA/IPA International Conference.

In 2024, CAB members engaged in significant activities, both in person and virtually. During the 2024 AMDA/IPA International Conference in San Antonio, two in-person, one-on-one CAB meetings were conducted—one with Sanofi and another with Amicus—and both were moderated by the IPA. Additionally, CAB members actively participated in the Conference, enhancing their engagement with the broader Pompe community and industry stakeholders.

Beyond the AMDA/IPA Conference, the CAB held two virtual one-on-one meetings, one with Shionogi and another with AskBio. The IPA CAB also welcomed two new members following the Conference, further expanding our representation.

Due to the online format of the virtual meetings, where only a subset of CAB members attends each session, the IPA is seeking additional CAB members, preferably from organizations, to ensure representation across the full spectrum of Pompe disease.

Prospective CAB members should:

- Be 18 years old or older
- Possess strong English language skills
- Be able to commit to a minimum of two meetings per year
- Sign a non-disclosure/confidentiality agreement

Specific training will be provided for new members. Interested individuals should submit an application letter outlining their relevant skills, experience, and interest to fabiodipietro@worldpompe.org.

Through these efforts, the IPA CAB remains committed to ensuring patient needs are at the forefront and enhancing the quality of Pompe disease research and development.

Current Commercially Approved Treatments

There are currently three commercially-approved treatments for Pompe disease. These treatments are not available in all countries, but efforts are underway by companies involved to continue seeking approval and reimbursement in additional countries.

Next Generation Enzyme Replacement Therapies

Amicus Therapeutics

As of November 2024, Amicus has received approval for Pombiliti + Opfolda in the European Union, the United Kingdom and the United States of America and they continue to work with other regulators and reimbursing authorities to provide commercial access in more markets.

In addition, Amicus currently has four (4) active clinical trials. These range from observational studies to pediatric trials. For more information, please visit www.clinicaltrials.gov/

Sanofi

Sanofi has two commercially approved enzyme replacement therapies on the market.

The first, Myozyme/Lumizyme was approved in 2006 in the European Union and United States, and continues to be used on a global scale.

The second is their next generation ERT, Nexviazyme/Nexviadyme, which was approved and commercially available in the US for late-onset Pompe in 2021. The European Union, the United Kingdom and Australia have now given their approval for the same treatment, which is known there as Nexviadyme and in Australia as Nexviazyme. However, work continues with reimbursers around the world to make this treatment commercially available.

In addition, Sanofi has several clinical trials under way to evaluate their next-generation treatment in various populations, including pediatric patients. For more information, please visit www.clinicaltrials.gov/

Research and Drug Development

The IPA always approaches all companies or investigators active in the Pompe field to discuss their treatment development programs; many are very willing to meet with us, either by teleconference, or face-to-face when we come together for an international meeting.

Below is a list of Pompe programmes we are currently aware of; visit https://clinicaltrials.gov/ for further details of clinical studies currently underway.

Next Generation Enzyme Replacement Therapies

Eleva Biologics

Eleva has previously announced that they are developing a moss-produced recombinant GAA; Repleva GAA/RPV-002 (glyco-improved Pompe-ERT). As of November 2024, it appears that their Pipeline is focused on Factor H and Fabry disease. However, they do still have links to publications regarding pre-clinical work in Pompe, so they will remain on our radar.

JCR Pharmaceuticals

According to JCR Pharmaceuticals website, as of November 2024, Pompe is no longer included as being part of their Pipeline.

M6P Therapeutics

M6P Therapeutics is working on a next generation ERT that is "naturally produced with the highest levels of M6P as compared to other rhGAA ERTs." According to their website, M021 "normalized glycogen and significantly improved muscle strength in Pompe mice in long-term studies" and "is substantially better than standard of care ERT glycogen for substrate clearance in muscles of Pompe mice." As of November 2024, their website states that the program is in Pre-Clinical Development and it still states that they anticipate applying for Investigational New Drug status (IND) in the second quarter of 2024.

Substrate Reduction Therapies

Substrate reduction therapies (SRTs) seek to affect the disease process by reducing the accumulation of glycogen in the muscles of Pompe patients by reducing the amount of glucose that is turned into glycogen. Below we have listed the two companies who are currently working on Substrate Reduction Therapies for Pompe.

ARO Biotherapeutics

Aro Biotherapeutics is a biotechnology company pioneering the development of tissue-targeted genetic medicines with a platform based on a proprietary protein technology called Centyrins. They recently completed a Phase 1 study in healthy volunteers, which showed that "ABX1100 was well tolerated . . . and it showed durable GYS1 mRNA knockdown in muscle biopsies, with effects lasting through at least 10 weeks following a single dose."

On October 29, 2024, Aro announced the initiation of a Phase 1(b) trial of ABX1100 in late-onset Pompe patients. According to their press release: "In the planned 1b study, investigators seek to enroll adults with LOPD to evaluate the safety and bioactivity of ABX1100. More information about the trial is available at ClinicalTrials.gov using the identifier NCT06109948."

Shionogi &Co., Ltd./Maze Therapeutics

Maze Therapeutics is developing MZE001, an investigational oral glycogen synthase (GYS1) inhibitor that aims to address Pompe disease by limiting disease-causing glycogen build-up. GYS1 is an enzyme responsible for glycogen production in human muscle cells, but not in human liver cells.

Maze completed a Phase 1 study in healthy individuals and announced positive results in February 2023.

In May 2023, Maze Therapeutics announced that it had entered into an exclusive worldwide license agreement with Sanofi for MZE001. However, this acquisition was stalled by the Federal Trade Commission (FTC), and Sanofi withdrew from the agreement in December 2023.

Following this, Shionogi & Co., Ltd. Acquired a worldwide license agreement for the rights to MZE001 on May 10, 2024. According to their press release, Shionogi is committed to advancing MZE001 and believe it has the potential to be used both as a monotherapy option and as an add-on therapy with enzyme replacement, the current standard of care, to enhance the treatment of patients with Pompe disease.

Cell and Gene Therapies (Regenerative medicine)

Regenerative medicine across rare disease has continued to gain interest. Below we have listed a number of different approaches that may each provide a solution for Pompe.

Alexion/CANbridge Care

In October 2022, AstraZeneca (through its Alexion subsidiary) acquired LogicBio Therapeutics. CANbridge holds an exclusive global license from LogicBio Therapeutics, Inc ("LogicBio") to develop, manufacture and commercialize gene therapy candidates for the treatment of Fabry and Pompe diseases, based on LogicBio's AAV sL65 technology. The company is in very early stage of development in their Pompe programme, as they continue to make progress on their Fabry disease candidate.

Amicus Therapeutics.

Based on information from Amicus' third quarter 2024 earnings call with investors, it appears that, while they are still working on a gene therapy for Pompe, Fabry is their primary focus.

Astellas Gene Therapies

Astellas Gene Therapies continues with its phase I/II clinical trial for LOPD adults, FORTIS. Their Gene Therapy, AT845, utilizes a muscle-directed approach with an AAV8 capsid serotype that is being investigated to determine whether it can deliver a functional GAA gene to express GAA directly in tissues affected by the disease, including skeletal and cardiac muscle.

According to www.clinicaltrials.gov, the FORTIS study trial aims to recruit 18 patients at four locations (two in California, one in Utah, and one in Newcastle UK). The Primary completion date of this trial is now November 30, 2029.

Bayer/AskBio

AskBio, a Bayer company, has an active, but not recruiting, clinical trial listed on www.clinicaltrials.gov for adults (18+) with Late-Onset Pompe Disease (LOPD) to assess their gene therapy ACT-101.

ACT-101 is infused intravenously and designed to deliver a functioning copy of the GAA gene to the liver. The goal is to restore GAA production to a level sufficient to no longer require ERT.

Only one study centre is currently active in this trial, Duke University, NC, which has enrolled 7 patients.

Crosswalk/CODEXIS/Takeda

In July 2024, Crosswalk Therapeutics acquired the Pompe disease compounds from Codexis, Inc. The compounds were part of a collaboration between Codexis and Takeda that was abandoned after Takeda announced in April 2023 that it was moving away from early-stage gene therapies.

Crosswalk's mission is to develop functional cures for rare diseases, including Pompe disease. The company's name symbolizes the idea of parents guiding their children safely through the medical care system.

According to Crosswalk's website, it is seed-stage biotech company dedicated to relentlessly pursuing functional cures for rare disease patients and their families. With an initial focus on rare diseases, we aim to expand our scope to include genetically defined common diseases in the future.

Erasmus MC – Professor Pim Pijnappel

Research continues at the Erasmus MC to study several regenerative therapies for Pompe, including stem cell regenerative therapies and RNA Oligonucleotides, as well as lentiviral gene therapy. Dr Pijnappel made a presentation at the 2024 AMDA/IPA International Pompe Patient and Scientific Conference on the status of his work. A recording of his presentation is available on the AMDA website: https://amda-pompe.org/conferences

GeneCradle Therapeutics

GeneCradle is working on an AAV-mediated gene therapy for Infantile-Onset Pompe. According to ClinicalTrials.gov, there is a trial based out of China that is currently recruiting. It will include 6 patients with IOPD who are younger than 6 months of age. This study was first posted on October 5, 2022, and we are still waiting for more information to be released.

Regeneron
According to the August 2024, Regeneron Corporate Presentation, Regeneron has a Pre-Ind research program for Pompe in their pipeline that is exploring CRISPR/Cas9 + AAV Transgene Insertion. This Program is in a very early stage, so we will continue to keep an eye on it as the program develops further.

Lacerta Therapeutics
While Sarepta had a licensing agreement with Lacerta Therapeutics to develop AAV-9 gene therapy for Pompe disease, this agreement was terminated in 2023. Further, it appears that as of mid-2024 Lacerta has ceased operations.

Spark Therapeutics
Spark Therapeutics was developing SPK-3006, an investigational gene therapy for treatment of Pompe disease. However, as of July 2024 Spark announced that it has stopped its Pompe Program. According to the Community Update they provided: The decision to close our Pompe program was not related to any safety issues or concerns. Rather, the decision was part of a larger portfolio review and realignment of our strategy as an organization to ensure we're delivering impactful gene therapies to people as quickly as possible.

Campaigns

Dose Flexibility
The IPA board continues to have concerns over the inflexibility of ERT dosing for Pompe Disease. This extends beyond the current commercially-available treatment to future treatment options as well. Several treating physicians are interested in exploring higher doses, but cannot because of the prohibitive cost of additional drug. The IPA continues to raise this topic with clinicians and drug companies to explore ways forward. A major hurdle currently is the interaction between country labels for approved treatment and reimbursing authorities' ability to pay. Especially in light of the economic situation around the world, we anticipate that in the coming years it will be even more difficult to get treatments approved and reimbursed, let alone at higher doses. However, just because it is a difficult journey we face, the IPA is committed to advocating for patients around the world when it comes to equal access to optimal dosing.

Treatment Transition and Expectation Guidelines
One common question that Patient Organizations get from new patients is: "How will I respond to treatment?" Unfortunately, there is not a clear answer to this question. Patient response to treatment varies from patient to patient. In some cases, the degree of progression at time of treatment onset will affect response. In other cases, a high-sustained immune response will reduce efficacy. And in others, it is not clear why some patients respond better than others.

The only thing that *is* clear after over twenty years of experience with ERT is that patients WILL respond differently, and it is important for patients to have realistic treatment expectations. This is especially true now that there are more treatment options available to patients. Now, the questions are becoming: What criteria should patients use (in consultation with their physicians) to determine whether they should try a new treatment option? What should my expectations be if I switch treatments? How do I know if the new treatment I am trying is better for me?

These are important questions, and ones that the IPA Board is committed to helping to answer and we believe that developing an independent an International Pompe Data Collection Collaborative will be key to answering these questions (and new ones that come up) in the future.

IPA-Erasmus Survey
The IPA/Erasmus Survey (the "Pompe Survey") was started in 2002. It is a collaboration between the International Pompe Association (IPA) and Erasmus MC.

The goal at the time it was started was to better understand, from the patient perspective, the disease burden that patients with Pompe face. The timing of the initiation of the Survey was very intentional. There was an understanding at the time that it was imperative to begin collecting this information so that we would have a patient-owned, patient-reported questionnaire and data to capture the natural history of the disease in the early days, and then to capture how that may change over time with treatment.

The IPA-Erasmus Survey has been a phenomenal success over the last twenty-two (22) years. There have been numerous peer-reviewed articles written based on the collected data—articles that each and every Pompe patient contributed to and made possible.

Today, the large number of potential future therapies makes it even more imperative that we remain strong in our commitment to the Survey, and to expanding it as appropriate. This is because each new drug that is approved will likely come with a commitment to track its clinical outcomes and patient-reported outcomes. That could mean a separate registry for each drug unless an independent registry can be developed to hold all the data. This would, necessarily, result in patient reported data being spread throughout multiple registries depending on which treatment they are on at the time, and whether they choose (upon discussion with their treating physician) to switch between therapies.

Without an effort like the Erasmus Pompe Survey in existence, it will be impossible to truly track, on a global level, how patients feel that they are doing throughout their disease and treatment experience. To that end, the IPA Board has been working very closely with Erasmus MC to update and streamline the existing Pompe Survey. We are happy to announce that in 2023 current patients enrolled in the Survey were able to complete the updated survey. In addition, to further the reach of the survey, we have worked with Erasmus to have it translated into additional languages. The Survey is now available in the following languages: English, Dutch, Spanish, German, French, and Italian.

Finally, the IPA Board continues to advocate for new modules that would allow for patients to track their own results, and are beginning work on a new Survey that will be targeted at younger patients. These meetings will continue in 2025, and we hope to also incorporate an international board of advisors in the coming years.

For more information on the IPA/Erasmus Pompe Survey, please visit the updated webpage on the Erasmus University Website: https://www.erasmusmc.nl/en/research/project/ipa-erasmus-mc-pompe-survey.

This website also includes a partial list of publications that have come from the data so our Members and the Patient Community as a whole can see how their contribution has led to important understanding of Pompe disease.

Independent Global Pompe Patient Data Collection Collaboration: The Octopus Project

As of November 2024, there are multiple silos of Pompe patient data around the world. These include: the Sanofi Patient Registry, the IPA/Erasmus Survey Data (Patient Reported Outcomes), national data pools (ie Australia, France, Germany, the Netherlands, etc), and data collected at all of the expert centers around the world.

While data collection is imperative, it is just as important to be able to aggregate information across data silos to be able to best understand trends, and best disease management practices. This is especially vital now that patients are fortunate to have treatment options.

As a community, it is in our best interest to start collecting data in a uniform manner to allow patient data to be compared across data sets. This will allow our community to be able to collect enough data from around the world and across the disease spectrum to better understand how treatment options, disease management approaches, and combinations of therapy affect different subsets of patients across the Pompe spectrum.

In 2023, the IPA began working with a group of expert Pompe clinicians around the world to collaborate on developing an Independent, Global Pompe Patient Registry. In addition to regular meetings with the expert clinicians, the IPA Board has been collaborating with and learning from other key parties in the rare disease registry space. These include RareX and the International Niemann-Pick Disease Registry.

At the AMDA/IPA Conference in San Antonio, expert clinicians from around the world met with the IPA Board to direct our energies towards the puzzle of how to best collaborate on data collection. Key points of agreement included: Focus on a data collection collaboration, not a new Registry due to significant cost and time burden of registries; in the context of regulations and logistical challenges, find ways to share data, de-identified and with a patient identifier using a federated data mining approach; establish use of unique patient identifier numbers with all registries; consult rare disorder organizations about their experiences with registries, data collection strategies, and issues of data sharing to better understand best practices; agree on a core data set to be collected; amongst others.

It was decided to name this data collection collaboration the "Octopus Project." The idea with the IPA's Octopus project is that a specific research or clinical question could be posed and under an IPA governance process (with appropriate review board), reviewed for approval to proceed. Data would be extracted from various registries and data sets around the world. The IPA and advisors see this as a working group to share data to accelerate research, not a traditional registry.

Following this Round Table in the morning, the group met again in the afternoon with representatives from Industry and RareX. The meeting was called to discuss thoughts about a global registry and where the Pompe community wants to be in five, ten, and twenty years. It was acknowledged that there are existing industry registries and new ones on the horizon, national registries, patient organization registries, and academic medical center registries. Data are fragmented and inaccessible as a result. In the big picture and long term, patients want and need guidance on treatment options. The IPA is looking for the best ways to be more collaborative in combining data in order that patients can know how they are doing over time across therapies and to get a global picture of patient experience. The future holds more treatments with gene therapies, substrate reduction therapy, and other treatments generating more data.

It was understood that there is so much about Pompe that is not understood. Ultimately it is impossible to know trends in disease process without data. With multiple treatment options and increasing complexity, the time is now to share data. It was acknowledged at this meeting that questions about how to extract data from clinical records, who is the owner of the data, and how to protect privacy will have to be addressed. It was also noted that there is labor involved in data entry and there is a need to acknowledge the efforts of those who do this work. Clinics and centers have invested 30-40 years in data collection in some cases, so the new Octopus Project being created should not add to the workload. At the end of the day, the idea is that there is a need for a system where data speaks to each other—perhaps using an AI approach. Even more important, all this effort is about patients. There needs to be a better system to give data and its meaning back to the patients. Patients want to understand more and want their data back.

All parties agreed to continue working together and to meeting at least bi-annually. While there were a lot of unanswered questions, the commitment to working together and working **for** patients to better understand the nuances of Pompe was clear.

We look forward to continuing this important work in 2025 and providing updates as we progress on this Project.

Website Re-Design

Based on feedback from the community, the IPA completed a redesign of its website in 2023. We've made it easier to find historical information; stories and videos are available from our community; and, the updated Pompe Connections, a patient-focussed resource, are available and currently being updated into many languages. Please let us know if there are any other features that you'd find useful for us to include. Also, please be patient as we continue to update the pages in the coming months.

Communications

Maryze Schoneveld van der Linde prepares the IPA newsletters and also acts as a point of contact for international inquiries. Maryze is a valuable source of information to people who don't have support from patient organizations in their countries.

All relevant news and announcements (e. g. International Pompe Day, Conferences, Industry updates, etc) are published on the IPA's website www.worldpompe.org. Suggestions for articles are welcome and will be published if they are relevant to the global Pompe community (not of national relevance only). Please send your contribution to the Webmaster: info@worldpompe.org.

On Facebook, there are two IPA pages:

IPA: www.facebook.com/International-Pompe-Association-IPA-119237914814204/) and **International Pompe Day**: www.facebook.com/InternationalPompeDay

For IPA Members only, a confidential (closed and publicly not visible) Facebook group is used for communication between the member organisations (https://www.facebook.com/groups/850602065054870/). If you want to join as a representative of your national patient organisation, please contact the IPA Board at info@worldpompe.org.

Andrea Faris has recently joined the IPA to increase our social media presence and to aid in bringing awareness to our work, and the resources we provide. We also want to share what is happening around the world on a local or national level. To that

end, she started an Instagram page (https://www.instagram.com/ipa_pompe/).

Newsletters and updates are sent out on an as-needed basis. Suggestions for new topics to be covered are always welcome.

Pompe Connections

Throughout 2022-23, the Board focused extensively on updating all of the existing Pompe Connections, and Pompe Connections Treatment Edition Brochures. Ultimately, the Pompe Connections brochures were updated to remove redundant links, product names and manufacturers names. Some revision of wording was undertaken to reflect multiple ERT types and multiple manufacturers. In addition, a number of Treatment Edition brochures were removed, as they could not be updated to fit the new requirements or in the case that the information contained in the brochure is available from other sites or is updated regularly.

We are happy to announce that the Pompe Connections are now updated and available in the following languages: Dutch, French, German, Italian, Japanese, Spanish, Turkish, Arabic, Korean, Russian, Greek, Czech, Hindi, Chinese, and Portuguese. We believe that this is a good resource for patients at all stages of their Pompe journey, and would encourage our members to share them with patients in their communities.

In addition, we would also like to ask our members, and the broader Pompe Community of patients, family members, and the medical community, to contact us at info@worldpompe.org if there are additional topics that you would like to see covered. In addition, please contact us if you would be willing to help with translations of current and future Brochures.

Meetings

In 2023 we saw the beginning of more in-person meetings, in addition to hybrid meetings that were in-person and virtual. This continued in 2024. Below please find short summaries of some of the Conferences that took place during 2024 that were attended by IPA Board members.

2024 AMDA/IPA International Pompe Patient and Scientific Conference

May 3rd-5th, 2024 saw the Pompe community returning to San Antonio, Texas for another international gathering of Pompe patients, caregivers, leading experts in research and disease management, and industry. In an effort to make the Conference more accessible for everyone around the world, for the first time a virtual attendance option was provided.

The IPA Board was in attendance, as were affiliate members from around the world. It was a wonderful weekend full of fun, education, knowledge-sharing, and relationship-forming. We would like that thank the entire Community for helping to make our 2024 AMDA/IPA Conference a HUGE success! With upwards of 225 in person attendees, and 80+ virtual attendees from over 13 countries, this was our largest, most successful conference yet!

If you were not able to attend, we have the following resources available for you on the AMDA website (http://www.amda-pompe.org)

The conference recordings are now up on our YouTube Channel, click here.
To view the official brochure of the 2024 AMDA/IPA Conference, click here!

WORLD Symposium 2024

Several Board Members (and members of the Pompe Community) attended the WORLD Symposium in February 2024. Attendance was a combination of virtually (following the conference) and in person. The WORLD symposium is an annual conference which brings together clinicians, scientists, industry and patients from all over the world to learn and share knowledge on all Lysosomal Diseases (LDs). Many presentations and posters highlight the current level of research activity for Pompe disease. We look forward to attending again next year.

Sanofi Rare Disease Registries Patient Council

The IPA Board has had a consistent presence on the Patient Council. The purpose of the Council is to provide the Patient Community's perspective on Sanofi's Pompe Registry.

IPA AGM 2024

The IPA's Annual General Meeting in 2024 will take place virtually on November 23, 2024.

Looking ahead

For 2025, the IPA welcomes ideas from the Pompe community for projects to raise global awareness, improve our support and engagement with national groups, and develop our relationships with the growing number of research and industry networks. We are a very close community and there is little doubt that Together we are Strong!

Thank you,

IPA Board
November 2024