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Annual Report 2022

Introduction

Exciting advancements continue to evolve within the Pompe community. The IPA continues to monitor, liaise and develop 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 better understand Pompe and the unmet needs of the Pompe Community. As you read the 2022 Annual Report, it is the hope of the IPA Board that the exciting advances that are coming, as well as the IPA's planned collaborations for 2022-2023 (and beyond), will fill you with the same hope for the future that we have.

Chairman's statement 2022

The IPA was started in 1999 when a handful of patient organization leaders from around the world met in the Netherlands with a shared purpose. We believed that, together, we could accomplish more for the Pompe patient community than any single group could alone. We believed that "Together We Are Strong," stronger than we are alone. We understood that there are common needs and desires for patients and their families all over the world, and we believed that by sharing our efforts, and by working together we would be in the best position possible to fulfil our mission statement: Campaign for early diagnosis and effective, affordable and safe therapies. Strive to provide information and support to all patients, their families and others with interests in Pompe disease.

With every year that passes, we learn more about Pompe disease. We learn more about the emerging phenotypes in Infantile-Onset Pompe (IOPD) and Late-Onset Pompe (LOPD) now that treatment is available. We are starting to learn about why patients respond differently to treatment. We learn more about how to best support patients and their families throughout the process of diagnosis, treatment, and disease management. But the most important thing to note is, we do all of this together with the Medical/Scientific Community, Industry, and Regulators. It is essential that the patient voice is at the forefront when determining the unmet needs of your community, the desires of our community, and there must be patient input on future clinical trial designs and study endpoints.

Therefore, we continue to forge and strengthen relationships between the patient community, the medical/scientific community, industry, and regulators. These relationships are vital to our ability to be a voice for patients in research, in drug development, and for equal access to treatment and care. The IPA recently had the opportunity to attend the Steps Forward in Pompe Meeting (SFPD) hosted by Sanofi. At this meeting, our Board Members had the first opportunity since before COVID to meet, talk and share with our partners in the Medical and Scientific Community in person. It was a wonderful opportunity, and led to many fruitful conversations, as well as inspired plans for next year.

We now have one next generation Enzyme Replacement Therapy (ERT) that is in the process of becoming commercially available around the world, another next generation ERT has an anticipated regulatory decision date for next year; multiple gene therapies under clinical development, with trials already underway; and, substrate reduction therapies (SRTs) under clinical development.

With new treatment options becoming more accessible, the IPA is working with the expert Medical/Scientific Community to provide information to patients and their families on treatment expectations, and when trying new options may be in the patients' best interest. These conversations are in very early stages, but this will be a significant focus of the IPA in the coming year.

In addition to the clinical developments in our field, we have also seen increased interest in incorporating patient perspectives. This has been seen in an increased activity of the IPA's Community Advisory Board (CAB), as well as increased interest in Patient Reported Outcomes (like the IPA/Erasmus Patient Survey). The IPA's Position Paper on Patient Reported outcomes can be found on the IPA website at: https://www.worldpompe.org/images/pdfs/IPA_Position_Paper_-_Patient_Reported_Outcomes.pdf

In the rest of this report, you will read about the activities that have occurred over the last year, and how we have sought to stay true to our mission. Together We Are Strong!

2022 International Pompe Day

April 15, 2022 was our 9th Annual International Pompe Day. Despite the challenges we have faced, it is an exciting time for the Pompe Community worldwide with new studies and treatments on the horizon, and Hope is what drives the patient community, and what inspires the medical/scientific community, and industry to keep working.

The IPA believes that raising awareness about Pompe Disease is key, and the theme selected for 2022 was **International Pompe Voices.** For International Pompe Day on April 15, 2022 we asked people in the global Pompe community to contribute a video illustrating how strength and resilience is gained through friendship. All recordings can be watched in the IPD 2022 playlist on the IPA YouTube channel, on the International Pompe Day blog and on the IPA website. There are 12 wonderful videos and conversations that really showcase where patients get their strength. We also had several experts participate and share how they are inspired by the Patient Community and what encourages them to continue their work. The IPA Board encourages everyone to take a few minutes to view these videos and share them with their friends and family. #TogetherWeAre Strong!

Patient Affiliates

We currently have 66 contacts with patient organisations and individuals, representing almost 61 countries around the World.

Community Advisory Board

The IPA established its own Community Advisory Board (CAB) for Pompe disease in 2019. The difference between industry-driven advisory boards and the IPA's CAB is that the IPA's is a patient-driven endeavour that is organised and owned by the Pompe patient community. Traditionally, a CAB is a group established and operated by patient advocates to facilitate discussions, in a neutral setting, on the latest developments and challenges related to medical research and procedures with the company or body conducting the research. A CAB is a group of patients or advocates who offer their expertise to sponsors of clinical research, on overall program development, single clinical trials, and other aspects beyond the research program. A CAB helps also ensure that clinical studies are designed to consider the real needs of patients, resulting in higher quality research. The IPA CAB is composed of 23 members from 10 countries spanning across 3 continents and representing the full spectrum of Pompe disease. The original composition of the CAB was 19 members in 2019, and we now have 23 members with new additions to the board from the US, the Netherlands, Hong Kong, Japan and, France in 2022.

The first IPA CAB meeting was held on October 28, 2019 in San Antonio, Texas, and was in the form of a multi-company round-table, with 15 CAB members attending in person and 5 sponsors attending as observers. While the intent was to follow this up with another in-person meeting in 2020, the COVID-19 pandemic made that impossible. Instead, the IPA offered interest parties the opportunity to host online CAB meetings, and has already facilitated many virtual one-on-one CAB meetings with several different industry partners throughout 2020, 2021, and 2022: AskBio (July 2020 and 2022); Sanofi Genzyme (September 2020, June 2021, and December 2021); Avrobio (2 CAB meetings in September 2021); and Maze Therapeutics (2022). Virtual one-on-one CAB meetings have proved to be very effective and have been appreciated by industry partners. Therefore, we are constantly working with all parties to continue this valuable program, and an increasing number of industry partners have expressed their interest in the IPA CAB. Further online CAB meetings are expected in the months to come. Due to the format of online virtual meetings, with a sub-section of the CAB attending each webinar, the IPA is looking for CAB members, preferably members of an organization, to represent the full spectrum of Pompe disease. Potential CAB members should be 18 years old or older, with good English skills, able to commit to a minimum of 2 meetings a year and sign a non-disclosure/confidentiality agreement. Specific training will be provided. Application letters stating relevant skills, experience and interest can be sent to fabiodipietro@worldpompe.org.

Research and Drug Development

The Pompe CAB will certainly have its work cut out for it if all of the proposed therapies under development engage with it. At the last estimate there are at least 14 new therapies under development for Pompe disease, with rumours of several more to come.

The IPA always approaches all companies or investigators active in the Pompe field to discuss their drug development programme; 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

There are currently two next-generation ERTs in clinical studies, and two more are being proposed using lower cost platforms.

Amicus Therapeutics

In February 2021, Amicus announced the initial results from its Phase 3 PROPEL Pivotal Trial for AT-GAA (cipaglucosidase alfa and miglustat) for Pompe—its investigational two-component therapy for the treatment of late-onset Pompe disease (LOPD) that has previously received Breakthrough Therapy Designation from the U.S. FDA and the Promising Innovative Medicine designation from the MHRA in the United Kingdom.

As of November 2022, Amicus has not yet received FDA approval in the US due to "the Agency's inability to complete the manufacturing facility inspection." "Due to restrictions on travel related to COVID-19, the FDA was unable to conduct the required inspection of the WuXi Biologics manufacturing site in China during the review cycle. As a result, the FDA is deferring action on the application until the manufacturing site inspection is complete. The Company continues to expect the FDA to approve the two components of AT-GAA, including the BLA and New Drug Application (NDA) for miglustat, together." "In the European Union, where a pre-approval inspection is not required, the regulatory review is on track and the Committee for Medicinal Products for Human Use (CHMP) opinion is expected before year end." (per 10/28/2022 Amicus Press Release).

Eleva Biologics (previously known as Greenovation)

Eleva continues to work on it's moss-produced recombinant GAA. According to their website, as of November 2022 they are still in the Pre-Clinical phase. In addition, they claim that their approach shows superior uptake into muscular cells, thanks to the inherent glycan pattern of moss.

JCR Pharmaceuticals

According to JCR Pharmaceuticals website, as of November 2022, they are still in Pre-Clinical Development of an ERT that has the potential to cross the blood-brain barrier.

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." As of November 2022, their program is in Pre-Clinical Development. They anticipate applying for Investigational New Drug status (IND) in the second quarter of 2024.

Pharming Group NV

Pharming continues to develop a transgenic ERT for Pompe. According to their website as of November 2022, they are currently studying their alpha-glucosidase therapy in IND-enabling studies.

Sanofi-Genzyme

In July/August 2021, Sanofi received a positive opinion on avalglucosidase alfa, a long-term next-generation enzyme replacement therapy for the treatment of people with Pompe disease, from the U.S. Food and Drug Administration (FDA) and also from the European Medical Authority.The positive opinion is based on data from the Phase 3 COMET study,

which found that avalglucosidase alfa showed clinically meaningful improvements in respiratory function and movement endurance measures in people with late-onset Pompe disease.

Following those decisions, the treatment became commercially available in the US in third-quarter 2021 for late-onset Pompe. In Europe, the treatment has been approved, but is not yet commercially available as it must be approved on a country-by-country basis.

This is the first new treatment for Pompe to be approved since 2006.

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

According to their website: "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. The company is developing a wholly-owned pipeline of Centyrin-based therapeutic candidates and is working with industry partners to leverage Centyrins for tissue-specific targeting of therapeutics for a diverse set of diseases."

In August 2022, Aro Biotherapeutics was granted Orphan Drug Designation for their Pompe product ABX1100. ABX1100 targets the gene for glycogen synthase 1 (Gys1), an enzyme that is responsible for glycogen synthesis in muscle. Inhibition of Gys1 has been shown to reduce glycogen levels and thus represents a novel treatment approach for Pompe disease.

At the TIDES USA 2022 meeting, Aro Biotherapeutics presented data demonstrating that ABX1100 profoundly reduces Gys1 mRNA and GYS1 protein, leading to meaningful reductions in glycogen levels in the skeletal muscle in the Pompe mouse disease model. Aro Biotherapeutics anticipates entering clinical trials with ABX1100 in mid-2023.

Maze Therapeutics

Maze Therapeutics is working on a substrate reduction therapy (SRT) that has potential therapeutic benefits for Pompe. Their approach to treating Pompe disease is achieved by halting skeletal and respiratory muscle glycogen synthesis and its subsequent accumulation by inhibiting the action of the gene GYS1 through SRT.

Maze has completed a Phase 1 study in healthy individuals, and hopes to announce these results by the end of 2022. They also hope to start a Phase 2 trial in Pompe patients in the first half of 2023.

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.

Amicus Therapeutics.

Amicus is working with the Gene Therapy Program in the Perelman School of Medicine at the University of Pennsylvania (Penn) to pursue research and development of novel gene therapies for Pompe disease. This program remains at the Pre-clinical development stage as of November 2022.

Astellas Gene Therapies (previously known as Audentes Therapeutics)

According to their website, Astellas Gene Therapies is developing AT845, a novel gene replacement investigational therapy to address the recognized limitations of ERT by targeting the muscle tissues, the primary tissue affected in Pompe disease. 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 that is efficiently transduced to express GAA directly in tissues affected by the disease, including skeletal and cardiac muscle. At WORLD 2022, Astellas presented their initial results from the FORTIS clinical trial. However, in June 2022, Astellas announced "that the US Food and Drug Administration (FDA) has placed a clinical hold on the FORTIS Phase 1/2 trial following the occurrence of a serious adverse event (SAE) of

peripheral sensory neuropathy in one of the trial participants. FORTIS is a clinical trial evaluating AT845, an investigational adeno-associated virus (AAV) gene replacement therapy in adults with Late-Onset Pompe Disease." As of November 2022, the clinical trial is still on hold and we are waiting to see how things progress with the FDA.

AVROBIO

AVR-RD-03, the Lentiviral gene therapy platform used by AvroBio modifies the patient's own stem cells taken from the bone marrow. As of November 2022, this program is still in pre-clinical research. According to their website: "AVROBIO has a preclinical research program for a gene therapy for Pompe disease. We have shown a favourable preclinical safety and efficacy profile in a mouse model of Pompe disease."

Bayer/AskBio

In October 2020, Bayer acquired AskBio, and its gene therapy platform. The intent is to help to bring their therapies to commercial market. In regards to Pompe disease, AskBio continues to enrol patients with Late-Onset Pompe Disease (LOPD) to assess multiple doses of its gene therapy ACT-101 (also known as ACTUS-101). ACT-101 is infused intravenously and designed to deliver a functioning copy of the GAA gene (malfunctioning in Pompe disease) to the liver. The goal is to restore GAA production to a level sufficient to no longer require ERT. The primary objective of this study is to assess the safety of ACT-101 for the treatment of LOPD in adults, as well as assess the impact of this treatment on patient health as measured by changes in exercise capacity (6-minute walk), pulmonary function, and other factors including quality of life. The first patient was dosed in January 2019, and trial is on-going.

CODEXIS/Takeda

Through a partnership between CODEXIS and Takeda, early research into a potential gene therapy for Pompe disease is taking place. As of November 2022, their website states that this Program is in the research development phase, so not much is known yet about this approach.

Erasmus MC – Pim Pijnappel, Associate Professor Molecular Stem Cell Biology

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.

GeneCradle Therapeutics

GeneCradle is working on an AAV-mediated gene therapy for Infantile-Onset Pompe. According to ClinicalTrials.gov, there is 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, so we are still waiting for more information to be released.

LogicBio Therapeutics/CANbridge Care

LogicBio'sGeneRide technology is a program to watch as a second-generation gene therapy. They claim that their technology will be suitable for infants and will not require re-dosing. They are looking across the range of LSDs and GSDs, but say that applying their technology to Pompe disease is more challenging. As of November 2022, this program remains in the Research Phase of development.

Regeneron

According to the June 2022, Annual Shareholder Meeting Report, Regeneron has a Pre-Ind research program for Pompe in their pipline 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.

Sarepta Therapeutics/Lacerta Therapeutics

Sarepta has an agreement with Lacerta Therapeutics to develop AAV-9 gene therapy for Pompe disease. As of November 2022, this approach remains in the Research Phase of Development.

Spark Therapeutics

Spark Therapeutics is developing SPK-3006, an investigational gene therapy for treatment of Pompe disease. Their Phase II trial, Resolute, is still ongoing. The purpose of the RESOLUTE clinical trial is to study the effects of gene therapy

SPK-3006 (investigational study drug). Spark announced in February 2021 that the first patient had been dosed in the Phase 1/2 trial.

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. Therefore, in 2023 it is our intention to start a working group with leading experts in the Medical Community to develop Treatment Transition and Expectation Guidelines for our Community. We believe this is a vital topic, and one that deserves our full commitment.

IPA-Erasmus COVID-19 Survey

The IPA would like to thank everyone who participated in the COVID-19 one-time Survey, as part of the overall IPA/Erasmus Survey. The purpose of this survey was to look at both the effects of the pandemic on the life-style of Pompe patients, as well as the effects of infections with COVID-19 on Pompe patients. We believe that it is critical to gather this data so that we can properly and timely document these effects.

In order to reach as much of a global audience as possible, this Survey was conducted online. It was available in the following languages: English, Portuguese, Dutch, German, Italian, Spanish, French, and Chinese. The IPA Board would like to extend a special thank you to the following volunteer translators from our community: Olivier Cavallero, Lucy Golder, Birgit Wolf, Tony Wu, Ariadne Buenos Santos, and Daniel Saiz. Without their help, it would not have been possible to have the COVID Survey be the success that it was.

When the survey was closed on November 7, 2022, we have over 330 responses! The initial goal was to present the first results from this Survey at WORLD 2023. However, due to the need to extend the deadline of the Survey from October to November, it will not be possible to meet the deadlines for WORLD submissions. But we will be presenting this data as soon as possible in 2023.

IPA-Erasmus Survey

The IPA/Erasmus Survey (the "Pompe Survey") was first 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.

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 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 are doing throughout their disease and treatment experience. To that end, the IPA Board has been working very closely with Erasmus MC over 2021 and 2022 to update and streamline the existing Pompe Survey. In addition, to further the reach of the survey, we have worked with Erasmus to have it translated into additional languages. Once all translations are completed, the Survey will be available in the following languages: English, Dutch, Spanish, German, French, and Italian.

The IPA also continues to discuss the potential to develop a patient-owned registry of medical data that is connected with the IPA/Erasmus patient-reported questionnaire. This will continue to be an on-going exploration over the coming years.

Finally, the IPA Board continues to advocate for new modules that would allow for patients to track their own results. These meetings will continue in 2023.

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.

Communication Initiative

In 2021 the IPA Board hired a communication firm, ZUID, to assist us in better evaluating and meeting the needs of our members.

The first step of this Communication Initiative included a combination of written surveys and phone calls with some of our members to gather feedback on how the IPA can better serve our Members. Thank you to everyone who participated. Based on the feedback we received, it was determined that the next best step was to completely re-haul our website to make it more "user-friendly," and to better support the IPA's mission and our Members. We are very excited to say that the website will launch prior to our AGM on December 3, 2022. Please be patient with us as, over the coming weeks and months, we continue to add content to the new website.

As we continue to add content, we look forward to working with all of you on this in 2023, and welcome any thoughts you may have.

Communications

Maryze Schoneveld van der Linde prepares the IPA newsletters and also acts as a point of contact for international inquiries. Maryze is a great source of comfort to people who don't have the support they need in their own countries. In the past year she has helped Pompe families in 8 countries to get access to treatment: Kenya, India, United Arab Emirates, Pakistan, Gaza, Vietnam, Turkey and the Philippines. Additionally, she provided support to patients in need of diagnosis or equipment in 5 countries like Yemen, India, Iran, Philippines, United Arab Emirates. Maryze also spent countless hours working closely with Pompe families in Ukraine and contacts throughout Europe during the onset of the Ukrainian war with Russia to help them re-locate where that was the desire, or to secure uninterrupted access to therapy in Ukraine where they decided to stay. For more information on her work with the Ukrainian patients, please see the article on our website.

All relevant news and announcements (e. g. International Pompe Day talent contest) are published on 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: webmaster@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.

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, the Board focused extensively on updating all of the existing Pompe Connections, and Pompe Connections Treatment Edition Brochures.

We would 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

Due to the ongoing COVID-19 pandemic, many Conferences were moved to a virtual setting in 2022. Below please find short summaries of some of the Conferences that took place during 2022 that were attended by IPA Board members.

WORLD Symposium 2022

Several Board Members (and members of the Pompe Community) attended the WORLD Symposium in February 2022. Attendance was a combination of virtually 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.

Steps Forward in Pompe 2022

The IPA Board was invited to attend Sanofi's Steps Forward in Pompe. While we cannot discuss the specific presentations that were made due to Confidentiality Agreements, we can strongly say it was a very productive meeting. It was the first time since before the COVID-19 Pandemic that we were all able to meet in person. The networking, discussions, and sense of shared purpose were inspiring and the Board is very excited for new opportunities next year to continue working together for the best interest of the Pompe Community.

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 2022

The IPA's Annual General Meeting in 2022 will take place virtually on December 3, 2022.

Looking ahead

For 2023, 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 2022)