



IPOPI'S NATIONAL MEMBER ORGANISATIONS AT THE GLOBAL PATIENTS MEETING IN LISBON, PORTUGAL, 2018

REVIEW OF ACTIVITIES AND ACHIEVEMENTS 2018



an IPOPI event



IPI2019

INTERNATIONAL PRIMARY
IMMUNODEFICIENCIES
CONGRESS

DIAGNOSIS AND
CLINICAL CARE

6-8 NOVEMBER
MADRID, SPAIN

A MESSAGE FROM IPOPI'S CHAIRPERSON AND EXECUTIVE DIRECTOR

2018: on the education and advocacy front



MARTINE PERGENT
President



JOHAN PRÉVOT
Executive Director

Supporting national PID patients' communities and reinforcing their expertise, raising awareness, advocating to make decision makers aware of the specificities of PIDs and how society benefits when PID patients are diagnosed early and properly treated, cooperating with a growing number of stakeholders, allowing and promoting a patient holistic approach... This underpins the actions of IPOPI year after year! 2018 was no exception... to the contrary!

A major IPOPI event in 2018, our **Global Patient Meeting** gathered the IPOPI global family during 4 intensive and friendly days in Lisbon, in collaboration with ESID and INGID, our partner organisations. It was with great satisfaction that we welcomed over 100 attendees,

our highest attendance to date, including new patient representatives from IPOPI's newest national member organisations. Nothing makes us happier than seeing our global patients' family growing and strengthening the ties between our members. Of course, we did work hard: great lectures were given by the committed physicians of our MAP and other key stakeholders, followed by skills building workshops to better serve the PID cause, to share experiences and get inspired. In addition to this, there was also time for emotions and fun especially during the IPOPI PID Champions Awards ceremony. Two very special ladies, one starting a promising patient advocate career and another who has pioneered PID patient advocacy worldwide, were recognized for their outstanding contributions. Each of them, in their own way, showed us a path to follow: hats off to Madison Shaw and Marcia Boyle! Speaking of paths and acknowledgement, our Global Patients Meeting also marked a change in IPOPI's governance. We want to pay tribute to Jose Drabwell who, as IPOPI's chairperson, led our organisation for 10 successful years. Our heartfelt thanks go to Jose for this, including for her continued leadership, during most of 2018!

Information, education and advocacy all fit together, and need to be led on many fronts. 2018 looked a little bit like a pivot year: science has never been so successful in improving our knowledge of the conditions and their underlying factors and in improving and personalising treatment. At the same time, we have never been more aware of all the people in the world who still don't have access to diagnosis and treatment, and continue to face growing difficulties ranging from health care systems struggling financially and looking for cost savings, to supply tensions or shortages of essential medicines, in countries where this was not previously an issue, or dealing with new advocacy movements that actually play against the interest and lives of patients, such as, the anti-vaccination movement.

IPOPI used information, education and advocacy in both proactive and reactive ways throughout the year. This included enriching our collection of leaflets for both patients and physicians, initiating global webinars, organising joint or back to back meetings with patients and physician groups on a regional or national basis and, of course, actively preparing for IPIC2019. Advocacy efforts were deployed in several ways with, among many others: successful PID Forums held at the EU Parliament (despite the complex background of Brexit and upcoming EU Parliament elections), a reinforced SCID newborn screening campaign with our allies, our active participation in APEC and the WHO "Health for all" campaigns to ensure they are inclusive of people with PIDs, who represent a significant portion of the rare diseases community. IPOPI also addressed several national health authorities in support of our national member organisations requests.

We can certainly be proud of our past actions and, looking at the future, build on them to continue to be the PID patients' global voice. PIDs represent today close to 400 conditions and we hope that the way our community confronts its challenges can be seen as a model for many issues that affect the rare diseases community.

Before you take a look at our annual report, we would like to thank all our stakeholders, each of whom in their own way contributed to these activities and results and tell them how much this cooperation matters to IPOPI and people with PIDs!

Happy reading!

Martine Pergent
IPOPI President

Johan Prévot
IPOPI Executive Director

EXECUTIVE COMMITTEE, OFFICERS AND STAFF

EXECUTIVE COMMITTEE	MEDICAL ADVISORY PANEL
Martine Pergent President (from October 2018) <i>Vice-President (until October 2018)</i> Christine Jeffery Vice-President (from October 2018) <i>Treasurer (until October 2018)</i> Bruce Lim Treasurer (from October 2018) Jose Drabwell President (<i>until October 2008</i>) John Seymour Roberta Pena Dragana Koruga (ended mandate in October 2018) Violetta Kozhereva Andrea Gressani Annie Pienaar	Dr Nizar Mahlaoui , President – France Prof Martin Van Hagen , Vice-President - Netherlands PANEL MEMBERS Dr Adli Ali – Malaysia Prof Tadej Avcin – Slovenia Prof Aziz Bousfiha – Morocco Prof Charlotte Cunningham-Rundles – US Prof Monika Esser – South Africa Prof Alain Fischer – France Dr Jose Franco – Colombia Prof Isabelle Meyts – Belgium Dr Olaf Neth – Spain/Germany Dr Antônio Condino Neto – Brazil Prof Surjit Singh – India Dr Joanne Smart – Australia Prof Klaus Warnatz – Germany GENERAL PRACTITIONER ADVISER Dr Lotte Denning – Denmark SPECIAL ADVISER Prof Bobby Gaspar – UK
STAFF	
Johan Prevot: Executive Director Clare Glynn: Accounting and Administration Manager Magda Lourenço: Communications Manager Carla Morgado: Executive Assistant Julia Nordin: NMO Programmes Officer Leire Solis: Senior Health Policy and Advocacy Manager Carol Tavener: Administration	

IPOPI STRATEGIC PLAN

MISSION STATEMENT

The Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide through global collaboration.

IPOPI is the leading advocate for primary immunodeficiency patients worldwide. We work in collaboration with patients, doctors, politicians, regulators, pharmaceutical industry and other relevant stakeholders.

STRATEGIC OBJECTIVES 2016 - 2020

IPOPI's various projects and programmes are geared towards the 4 following strategic objectives:

- 1 -To promote early diagnosis & ensure optimal access to care
- 2 -To develop, strengthen and support National Member Organisations
- 3 -To raise PID awareness globally
- 4 -To stimulate stakeholder collaboration



WHAT ARE PIDS?

Primary immunodeficiencies (PIDs) are a large and growing group of close to 400 different disorders caused when some components of the immune system (mainly cells and proteins) do not work properly.

Whilst **PIDs** are generally recognised as rare disorders, some are more common than others and, taken as a whole, they represent an important group of people whose lives are profoundly impacted by their condition.

PIDs are caused by genetic defects of the immune system which are hereditary. The immune system normally helps the body fight off infections by germs (or 'micro-organisms') such as bacteria, viruses, fungi and protozoa. Because their immune systems do not work properly, people with **PIDs** are more prone than others to infections. When **PIDs** are left underdiagnosed or are misdiagnosed, the immune system remains defective, often leading to illness, disability, permanent organ damage or even death.

DIAGNOSIS

Whilst it is estimated that around 60% of PIDs can be easily diagnosed, many PIDs remain underdiagnosed on a global scale. Hopefully, as the technical ability to identify gene defects improves, more and more genetic causes of PID will be identified. Several PID diagnostic tests have recently been included in the World Health Organisation (WHO) as Essential Diagnostics List (EDL) in a dedicated PID section recognizing them as essential health coverage means.

Medicine is advancing very rapidly and novel therapies that target the specific cause of the disease are becoming available. These may have significant advantages for PID patients' and it is important that patients throughout the world have access to these treatment possibilities.

Screening for some of the most severe forms of immunodeficiency, such as Severe Combined Immunodeficiencies (SCID), will also diagnose more individuals and improve treatment outcomes.

TREATMENT

With appropriate access to the different and very effective therapies which can only happen if early and correct diagnosis is made, many sequelae will be avoided, and lives of patients will be saved.

People with PIDs are normally treated by doctors who are specialized in diseases of the immune system. How a patient is treated depends on which PID they have and on many other factors. Treatments for PIDs include: immunoglobulin (IG) therapies, Hematopoietic Stem Cell Transplantation (BMT), gene therapy, Granulocyte-colony stimulating factor (G-CSF), Gamma interferon, PEG adenosine deaminase, prophylactic anti-biotics, among others.

In the case of IG therapies it is important to know that different IG products are not interchangeable or mixable. IG can be administered intravenously or subcutaneously. They are considered as Essential Medicines for PIDs by the WHO. Intravenous immunoglobulin (IVIG) infusion usually takes 2-4 hours allowing high doses to be given and only needs to be given every 3-4 weeks which may suit some patients better. Subcutaneous immunoglobulin (SCIG) only takes 1-2 hours and can often be given at home by patients themselves, parents or careers. It may therefore be more convenient for some patients. However only small doses can be given by SC infusion, meaning they are given more frequently than IV infusions, usually once a week. Facilitated subcutaneous immunoglobulin (FSCIG), a type of SC infusion containing recombinant human hyaluronidase, allows patients to have SC infusions every 3-4 weeks. IG therapy should be chosen on an individualized basis according to patient needs and preferences.

Tremendous advances have also been made with gene therapy treatments including the availability of a first-ever licensed gene therapy treatment for SCID ADA.

CLASSIFICATION

PIDs are currently classified into nine groups: predominantly antibody deficiencies; combined immunodeficiencies; combined immunodeficiencies with associated or syndromic features; diseases of immune regulation; congenital defects of phagocyte number or function, or both; defects of innate immunity; auto-inflammatory disorders; complement deficiencies, based on the main immunological defects; and phenocopies of Primary Immunodeficiencies.

For more details on the classification of PID read the IPOPI leaflet 'How are primary immunodeficiencies classified?'

IPOPI is committed to continuing to disseminate information to patients, their relatives and doctors and to work towards better access to early diagnosis and treatment for all patients living with a PID.

For more information about PIDs and how they are treated and diagnosed, and to get advice on how to stay healthy please refer to the IPOPI publications page on our website **www.ipopi.org/publications**



IPOPI'S KEY ACHIEVEMENTS AND ACTIVITIES, 2018

STRATEGIC OBJECTIVE 1 – TO PROMOTE EARLY DIAGNOSIS AND ENSURE OPTIMAL ACCESS TO CARE

IPOPI strives to ensure that patients' interest is part of policy decision-making through advocacy at national, regional and international level. Our actions promote the importance of access, choice and supply of treatment for patients.

11TH IPOPI EU PID FORUM – TOWARDS A REVISION OF PAEDIATRIC REGULATION: THE CASE OF PIDS

IPOPI organised the 11th EU PID Forum, hosted by MEP Mr José Inácio Faria (EPP Portugal), MEP Tomáš Zdechovský (EPP, Czech Republic) and MEP Elena Gentile (S&D Italy) on the topic of “**Access to paediatric medicines: the case of PIDs – Towards the revision of Paediatric Regulation**”. The Forum took place in June 2018 and aimed at gathering the views of stakeholders in the primary immunodeficiencies (PIDs) field, such as patients, physicians, researchers, regulators, and pharmaceutical industry. They discussed the priorities of the community and found consensus in view of a potential revision of the European legislation on paediatric medicines, to ensure the needs of paediatric PID patients are considered.



Speakers at the 11th IPOPI EU PID Forum



Participants at the Forum

Key recommendations emanating from the meeting include the need for harmonization of treatment guidelines, the need for a holistic approach to paediatric care, the need for broadening the regulation to include societal aspects as well as medical devices, the importance of safe human clinical trials, and the importance of informing and involving patients in the process of developing new medicines.

IPOPI'S 12TH PID FORUM ON PID PRIORITIES FOR 2019-2024

Ahead of the EU Parliament 2019 elections, IPOPI held its 12th PID Forum on the topic “Rare Disease priorities in the European Parliament 2019-2024: the voice of PID patients” at the European Parliament (Brussels, Belgium) in November. This Forum was chaired by Mr José Inácio Faria MEP (EPP, Portugal) and featured interventions from Members of the European Parliament representing five political groups: Dr Anna Zaborska (EPP, Slovakia), Mrs Sirpa Pietikainen (EPP; Finland), Demetris Papadakis (S&D, Cyprus), Norica Nicolai (ALDE, Romania), Ms Tilly Metz (Greens/EFA, Luxembourg), Mr Takis Hadjigeorgiou (GUE/NGL, Cyprus).



MEP Faria opening the 12th IPOPI EU PID Forum

The Forum brought the key challenges for the PID patient community to the European Parliament's attention. A document containing the **political priorities for the next EU elections** was developed and can be found in www.IPOPI.org.

The objective was to provide a manifesto that would help profile the PID patient community's priorities on the agenda of the new Parliament and gather new supporters, as well as to support its EU-based National Member Organisations (NMOs) with a tool to use in their advocacy efforts, including in their interactions with their national politicians during the campaign.



Some of the speakers at the meeting

FIRST WEBINAR ON PID CLINICAL CARE

IPOPI organised its first webinar on primary immunodeficiencies' (PIDs) clinical care on November 14, on the topic of '**Advances in Management and Treatment of CVID**'. The half-hour webinar was moderated by IPOPI's President, Mrs Martine Pergent, and the invited speaker was **Prof Klaus Warnatz** from Freiburg University Hospital in Germany.



The webinar attracted 150 registrations from 43 different countries including patients and patient advocates, doctors, nurses and industry representatives.

The webinar on 'Advances in Management and Treatment of CVID' can be viewed on IPOPI TV.

SCID

IPOPI ADVOCATES FOR SCID NEWBORN SCREENING

IPOPI continues to advocate for the implementation of SCID newborn screening in European countries and beyond and is currently involved in several campaigns.

We have strengthened our base of supporters at the European Parliament and at the European Commission and have been supporting national PID groups in their advocacy efforts. In doing so, IPOPI has led the formation of a task force consisting of medical experts, newborn screening experts and patient representatives. The task force has been a driving force to gear the campaign and ensure a multi-stakeholder approach.

IPOPI has been supporting several campaigns at national level, be it by providing advice and logistical support in a sustained manner or by giving punctual advice and support when requested. In **Spain**, IPOPI is providing logistical and advisory support to its member organisation, AEDIP, in partnership with some medical experts to advocate for SCID newborn screening at national level. In the **United Kingdom**, at the request of our member, PID UK, we reached out to the Secretary of State for Health and Social Care, Matt Hancock, to ensure a swift kick-off of the planned pilot project.

Ms Leire Solís, Senior Health Policy and Advocacy Manager at IPOPI, was invited to present IPOPI's perspective on newborn screening for SCID at the International Society for Neonatal Screening (**ISNS**) regional meeting in October, in Bratislava, Slovakia, in a dedicated session, which shows that more countries are considering, studying or already screening for SCID, either at national level or regionally.

OTHER ADVOCACY CAMPAIGNS

IPOPI PROVIDES THE PATIENTS' VOICE AT APEC BLOOD POLICY FORUM

IPOPI's treasurer Mr Bruce Lim actively participated in the 5th APEC Blood Safety Policy Forum held in Taipei, Taiwan which focused on "Raising and Converging Standards through Centres of Excellence" with a presentation in the session "**Re-examining self-sufficiency in APEC economies – the patient perspective**". Mr Lim highlighted the importance of ensuring national blood policies, or future amendments to existing national blood policy should have the patient needs as the primary focus. IPOPI has been a regular participant in the APEC Blood Policy Forum meetings and has provided input in the preparation of the APEC recommendations on enhancing access to safe therapies for persons living with immunodeficiencies and bleeding disorders.

APEC has outlined a number of specific goals as part of the APEC Blood Supply Chain 2020 Roadmap, to establish quality systems and implement good manufacturing practices to **optimize the safety of the blood supply**. The Policy Forum helped identify ways in which centres of excellence can help accelerate GMP certification in APEC economies and open up new opportunities to converge the quality and safety standards for blood products across the Asia-Pacific region.

IPOPI remains committed to partnering with APEC to provide the patient perspectives in these important policy discussions which have the potential to significantly improve access to life-saving plasma derived medicinal products for patients living in the region.

MEDICAL ADVISORY PANEL

The MAP offers advice to the Board of Directors in all scientific and clinical matters. They provide invaluable medical input towards IPOPI PID information leaflets and the organisation of IPIC (International Primary Immunodeficiencies Congress), help with contacts from patients from all over the world and with emerging situations, such as SCID Newborn Screening, gene therapy and supply issues.

IPOPI is grateful for all the Panel members' commitment but wishes to specifically express its thanks to Dr Nizar Mahlaoui (Chairman) and to Prof Martin van Hagen (Vice-Chairman) for their kind contribution and dedication.

STRATEGIC OBJECTIVE 2 - TO DEVELOP, STRENGTHEN AND SUPPORT NATIONAL MEMBER ORGANISATIONS

IPOPI assists the establishment of new national member organisations and promotes the development of existing National Member Organisations.

XV IPOPI GLOBAL PID PATIENTS MEETING IN LISBON

The XV IPOPI Global PID Patients Meeting took place on 24-27 October 2018, in Lisbon, Portugal, back to back with the European Society for Immunodeficiencies (ESID) Biennial Meeting. IPOPI welcomed the leaders of its National Member Organisations (NMOs) who took part in a stimulating conference designed to inform, facilitate interaction and experience sharing among its membership. The meeting brought together over 100 people and was a great success.





One of the workshops

IPOPI organised several skills-building and interactive sessions designed to help NMOs better manage their groups. Several medical lectures provided participants with the latest primary immunodeficiencies' (PIDs) developments such as, new means of diagnosis by Prof Martin van Hagen (the Netherlands) and Dr Melanie Wong (Australia), management of adult PID patients by Dr David Lowe (UK) and novel curative treatments by Prof Alain Fischer (France). Dr Nizar Mahlaoui and Dr Tadej Avcin along with other MAP members provided participants with a very good overview of the highlights of ESID's scientific lectures.

Pharmaceutical industry sessions covered immunoglobulin access, quality management and safety and got very active participation from IPOPI members and other guests during the discussion.

IPOPI hosted its traditional PID Champions Awards Ceremony in downtown Lisbon. The LeBien Award was attributed to Marcia Boyle, mother of a PID patient and founder of IDF, the US national PID patient organisation, and the Luciano Vassalli Award was handed to Ms Madison Shaw, US PID patient who became an advocate for her condition at the early age of 12.

IPOPI's AGM took place on October 27 and resulted in the re-election of Mrs Martine Pergent from France and of Mrs Christine Jeffery from Australia. In the Board Meeting that followed they were also elected by their fellow Board Members as IPOPI President and Vice President respectively, Mr Bruce Lim, from Malaysia, as Treasurer and Mrs Roberta Anido de Pena was co-opted.

IPOPI PID REGIONAL EVENTS

IPOPI REGIONAL ADVOCACY AND MEDIA TRAINING WORKSHOP IN MALAYSIA

Primary immunodeficiency (PID) patient representatives and doctors from 5 South East Asian countries gathered in Kuala Lumpur, in Malaysia, for the **IPOPI Regional Advocacy and Media Training Workshop** in September, 2018. Participants identified key issues that affect their countries and trained for when they need to face a meeting with the Health Ministry and press conferences.



Group picture in Malaysia after the Workshop

The meeting included a joint open session in collaboration with the Malaysian Society for Asthma and Immunology (MSAI) and was honoured with the presence of Dr Nazrila Hairizan Nasir, Deputy Director Primary Health from the Family Health Development Division, from the Malay Ministry of Health. Speakers welcomed MSAI members and patient representatives to the meeting and then started discussing what advocacy meant and what were the keys for a successful campaign. The “**Primary Immunodeficiencies Principles of Care**” (PID PoC) were showcased as an advocacy tool that could provide patient representatives and doctors the fundamentals to start building their advocacy campaigns.



Role playing



Discussing pressing needs

Participants from Indonesia, Malaysia, the Philippines, Thailand and Vietnam discussed the challenges in their respective countries and then jointly identified the common challenges throughout the region. The workshop was very successful in equipping IPOPI's NMOs in the region with additional skills and tools to advocate on behalf of their communities.

IPOPI 2nd REGIONAL ASIAN PID PATIENTS AND DOCTORS MEETING

IPOPI organised its second Regional Patients and Doctors meeting in Asia, in November 2018, in Bangkok, Thailand. The two-day meeting was organised back to back with FIMSA (Federation of Immunological Societies of Asia-Oceania) Congress 2018. The meeting brought together 40 participants including patients, doctors and nurses from 7 countries (China and Hong Kong, Australia, India, Malaysia, Thailand, Vietnam and Indonesia).

The first day of the meeting was **geared towards doctors**, with lectures on clinical care, latest treatment and diagnostic advances by key immunology physicians from Asia and Europe. It was also the very first time IPOPI organised a panel discussion on difficult cases submitted by the participating doctors, to help solve complicated cases in terms of diagnosis or best treatment practices.

The second day was dedicated to **patient education**, with informative lectures on topics such as bone marrow transplantation (BMT), plasmapheresis and lessons learnt by the Haemophilia Association of Thailand. The afternoon awareness workshop had a focus on World PI Week, where patient representatives learnt about different World PI Week tools and resources and brainstormed on campaigning towards three different aspects: 1) *Public awareness*, 2) *Medical Awareness* and 3) *Political awareness*.



Second Regional Patients and Doctors meeting in Asia

IPOPI AT THE 2nd MEETING OF NORTH AFRICAN IG EXPERTS

The 2nd meeting of North African IG Experts was organised in conjunction with the 11th Moroccan annual PID congress in December at the Hassan II University in Casablanca. Prof Aziz Bousfiha and his team prepared the meeting and Prof Bousfiha had the chairing role. The joint meeting was very well attended by physicians from different Maghreb countries, from Sub-Saharan Africa, the Middle East, the US and Europe, and by several medical students. ASID President, Prof Tandakha Dieye, from Senegal, and ARAPID President, Prof Fethi Mel-louli, from Tunisia, were also in attendance.



Opening session



IPOPI President being awarded

IPOPI PRESIDENT HONOURED

As it is traditional in the region, the opening session is dedicated to someone the community wishes to honour. It was with great emotion and heartfelt gratitude that **Mrs Martine Pergent**, IPOPI's President, was given tribute for her action in the region by Moroccan actress and film director Ms Samia Akariou, godmother of Hajar (Moroccan Patient organisation), under the patronage of Prof Aziz Bousfiha and Mrs Maria Anebane, Vice-President of Hajar, and in the presence of the Dean of the faculty of medicine, PID patients and their families, and the medical community.

PID NATIONAL EVENTS

STRATEGIC WORKSHOP, PHILIPPINES

IPOPI organised a collaborative Strategic Workshop on September 27, 2018, with PhilIPOPI, its member organisation in the Philippines to discuss key strategic priorities for the NMO. The meeting was well attended by 25 participants including patient representatives, primary immunodeficiencies' (PID) specialists, IPOPI, and a representative from the **Philippine Department of Health, Dr Eric Tayag** who gave the keynote lecture on "The healthcare structure in the Philippines". The purpose of the meeting was to support the National Member Organisation (NMO) to reach a deeper understanding of the issues affecting PID patients in the Philippines, and to find solutions on how to address these issues.



Collaborative Strategic Workshop

Presentations from **IPOPI and MyPOPI** (IPOPI's NMO in Malaysia) on success stories of lobbying and working together followed, as well as testimonies from a doctor, a PID patient and a parent on key issues for the Philippine PID community.

The afternoon was dedicated to an **interactive workshop** where participants were able to brainstorm on the most pressing issues and find practical solutions for the NMO and their medical advisers. The NMO expanded their membership as a result of the meeting with new families joining their activities.

WORLD PI WEEK 2018 - RAISING AWARENESS OF PIDS AROUND THE GLOBE

The 2018 World Primary Immunodeficiencies Week (WPIW) was very successful in putting the spotlight on the importance of early recognition of PIDs, under the theme “**My future starts with early testing and diagnosis of Primary Immunodeficiencies**”. WPIW, celebrated each year between 22-29 of April, mobilised national primary immunodeficiencies organisations, patients, families, physicians, experts and partners in over 40 countries with campaigns, patient-specialist meetings, educational conferences, public lectures, TV and radio interviews.



The WPIW Steering Committee, of which IPOPI is one of the founding members, produced various materials to help participants reach the general public and the medical community. Infographics, a poster, a video and a scientific editorial helped patient organisations and individuals, raising awareness of PIDs in their national and local settings. Other activities included the photo contest “Picture Awareness” and the Campaign Stars 2018 initiative that engaged many people and helped creating a buzz on social media.



WPIW in Puerto Rico



Ukrainian PID Advocacy campaign

IPOPI NMO SUPPORT PROGRAMME 2018

Throughout 2018 IPOPI was pleased to be able to support and fund several NMO programmes with its NMO Supporting Programme, including:

- Austrian National PID meeting and family day
- Indonesian PID patients awareness campaign
- PID diagnosis pilot project in Paraguay
- PID educational meetings and campaign in Ecuador
- Ukrainian PID Advocacy campaign
- MyPOPI (Malaysian group) Family Day and leaflets in Malaysia
- Support towards translation and distribution of IPOPI patient information leaflets in Argentina, Estonia, Spain, Vietnam and in the UK
- Support towards educational patients and doctors' meetings and initiatives in Bolivia, Uruguay, Venezuela, Colombia, Suriname, Puerto Rico, Morocco, China, Philippines, Indonesia, Vietnam, Thailand, Nepal, Tunisia, Morocco, Uganda, Sudan, Iran, Israel, Finland, Ukraine, Kazakhstan, India, Ghana

STRATEGIC OBJECTIVE 3 - TO RAISE PID AWARENESS GLOBALLY

IPOPI uses communication tools and strategies to spread awareness of PID, share information and promote the collection of key PID data.

NEW SERIES OF IPOPI PID INFORMATION LEAFLETS

IPOPI has since 2011 been producing primary immunodeficiencies (PID) information leaflets and a new series was published in 2018. The series comprises 3 leaflets dedicated to 'CVID Management', 'PIDs and HSCT' and 'A Guide for Internal Medicine Specialists' with very helpful information and guidance for patients, their families and extended community.



'CVID Management' explains what Common Variable Immune Deficiency is and how it is diagnosed and treated. 'PIDs and HSCT' goes over how haematopoietic stem cell transplantation can help some people with PIDs. The 'A Guide for Internal Medicine Specialists' introduces the complex presentation of adult patients with PIDs who are often referred for internal medicine services with multiple, sometimes non-specific symptoms. The leaflets were initially made available in English but since their publication translations into Estonian and Spanish have been produced with the invaluable help of IPOPI's national member organisations and additional translations are expected.

IPOPI E-NEWS

IPOPI e-News is our most important publication, sent directly to thousands of readers and also shared on social media. e-News is released every March, June, September and December, and features highlights of IPOPI's and its members most important achievements.

e-News has a dedicated website, is mobile responsive and very easy to read on-the-go!

To subscribe to e-News please visit e-news.ipopi.org

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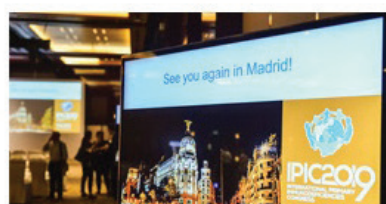


From the Chairperson's desk

The XVth PID Patients Global Meeting is upon us. This ever-growing global event will bring together around 100 PID patient group leaders from around the world and will be organised in parallel with the conferences organised by ESID and INGID. This year the event will also be an opportunity to celebrate IPOPI's anniversary with our membership and over a quarter of a century of global PID patient advocacy.

[Read more](#)

IPOPI News

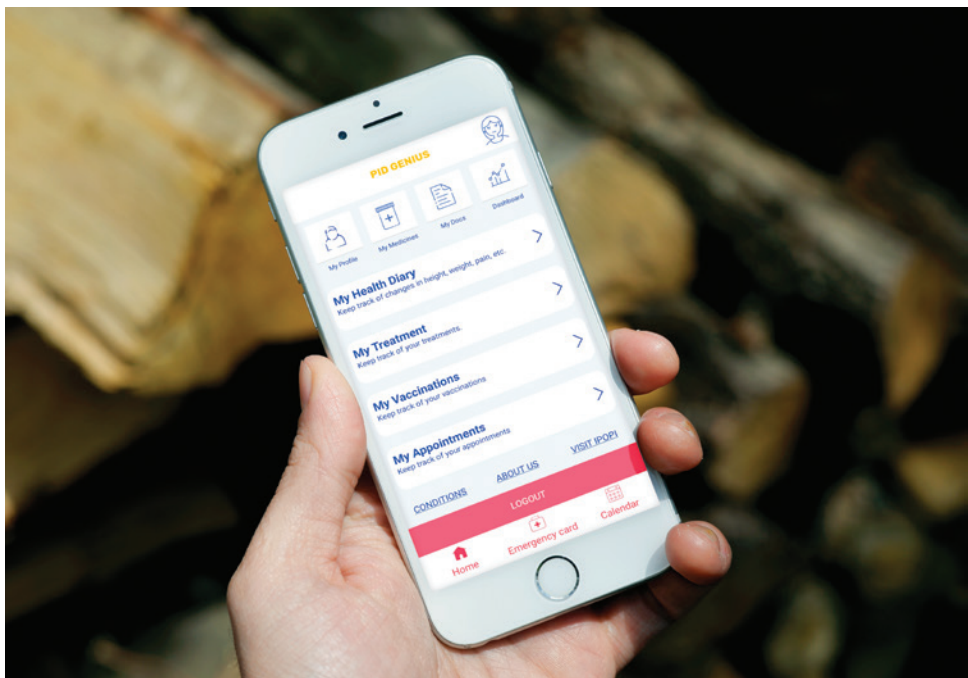


PID GENIUS COMPANION APP

IPOPI has developed PID GENIUS to support people living with primary immunodeficiencies manage their health information. PID GENIUS is a dynamic app playing the role of a personal assistant that helps keeping track of your treatments, day-to-day symptoms and most important documents with ease. It helps reduce paperwork and facilitates the discussion with your doctor.

An updated version with five additional languages was launched in October 2018.

PID Genius is available for download at the Apple App Store and on Google Play.



STRATEGIC OBJECTIVE 4 - TO STIMULATE STAKEHOLDER COLLABORATION



IPOPI values the benefits of working together with partners and stakeholders and 2018 has made us closer with old and new friends.

EUROPEAN REFERENCE NETWORK FOR PIDS

IPOPI continued its collaboration with the European Reference Network RITA and is committed to giving voice to the needs of patients with PIDs.

RITA stands for Rare Immunodeficiency, Autoinflammatory and Autoimmune Disorders and unites specialised centres in Europe dedicated to primary immunodeficiencies and other autoinflammatory and autoimmune disorders.

The European Commission launched an initiative to create European Reference Networks, virtual networks involving healthcare providers in support of patients living with rare disorders. Since 2018 clinicians have at their disposal the Clinical Patient Management System (CPMS), a secure web-based application to support all ERNs in the diagnosis and treatment of rare and complex disorders across national borders.

RECOMB & SCIDNET PROJECTS

IPOPI is a contributor to both of these ground-breaking projects and provides the patient perspective on key aspects of the project, contributing to communications' and awareness activities with a wide range of stakeholders.

RECOMB aims at developing and testing a gene therapy for children affected by a specific type of severe combined immunodeficiency (SCID): Recombinase Activating Genes (RAG) deficient SCID. This project is led by Prof Frank Staal, from Leiden University Medical Center, and is being funded by the European Commission through the EU Horizon 2020 research budget to develop this.

The SCIDNET consortium, also an EU Horizon 2020 funded programme, is led By Prof Bobby Gaspar, IPOPI's Medical Advisory Panel former Chairman. SCIDNET is focused on gene therapy for SCID-ADA.

www.recomb.eu

www.scidnet.eu

PLASMA SUPPLY DEVELOPMENTS

IPPC2018

IPOPI took part in the International Plasma Protein Congress (IPPC) organised by the Plasma Protein Therapeutics Association (PPTA), in Budapest, Hungary on March 13-14. The IPOPI Board of Directors and staff was in attendance and, similarly to previous years, IPOPI held its Board Meeting back to back with the Congress. IPOPI also attended the PPTA Stakeholders Meeting that took place on March 12 and the Platform of Plasma Protein Users (PLUS) members' meeting, held on March 14.



IPOPI Executive Director presenting at IPPC in Budapest

The main topics discussed at the conference were related to plasma supply issues and potential solutions, awareness of rare diseases in the world and national challenges of plasma collections. IPOPI's Executive Director, Mr Johan Prevot, was invited to give an overview of "Awareness of PID in the world", providing the global perspective of primary immunodeficiencies' (PID) patients to the industry audience.

PLUS CONSENSUS MEETING TO DISCUSS PLASMA SUPPLY

The Plasma Protein Users Platform (PLUS) and relevant stakeholders gathered for their Annual Consensus Meeting, held this year in Dublin, Ireland on January 11-12, 2018, to mark the 50th anniversary of the Irish Haemophilia Society. The meeting was attended by PLUS member organisations, associations representing the pharmaceutical industry, blood and plasma donor organisations and Marketing Research Bureau. The meeting was chaired by Dr Jacqueline Kerr from Paul Ehrlich Institute and coordinated by IPOPI.



The programme featured sessions on **plasma-derived medicinal products (PDMP) supply developments** and access. These sessions provided the background for the discussion on strategies to encourage blood and plasma donations in Europe.

IPOPI AT IPFA/PEI 25th INTERNATIONAL WORKSHOP IN ATHENS

IPOPI participated in the 25th Workshop on "Surveillance and Screening of Blood-Borne Pathogens" organised by the International Plasma Fractionation Association and the Paul Ehrlich Institute in May in Athens, Greece. This very specialised two-day meeting offered the opportunity to review state of art developments in the field.

The different presentations demonstrated the various steps taken and new technologies used by stakeholders to fight pathogens, such as, Hepatitis E Virus (HEV), which often is asymptomatic, Chikungunya and Zika virus and new emerging ones like Usutu virus.

From a viral safety standpoint, it is also important to point out that plasma-derived medicinal products such as immunoglobulins undergo viral inactivation/removal steps which make them very safe medicinal products with no known viral transmission since the mid-1990s.



EMA

The European Medicines Agency consider patients, as the end-users of medicines, as stakeholders. IPOPI's Board Member and former President, Mrs Jose Drabwell (UK) is a member of the Patient's and Consumers' Working Party and continues to develop an important role voicing patient's needs and perspectives in pharmacovigilance, involvement in clinical trials, data transparency and more. Ms Leire Solis, Senior Health Policy and Advocacy Manager supports Mrs Drabwell in this role.

WHO

In 2018 IPOPI coordinated a joint application, together with IUIS (International Union of Immunological Societies), to include primary immunodeficiencies diagnostics tests in the second World Health Organization (WHO) Model List of Essential In Vitro Diagnostics, also called **Essential Diagnostics List** (EDL). This list recognizes that in vitro diagnostics are essential for advancing universal health coverage and is a great advocacy tool especially for developing countries.



The WHO Strategic Advisory Group on In Vitro Diagnostics (SAGE IVD) considered several diagnostic tests submitted by IPOPI and IUIS as important, including, white blood cell count, HIV 1/2 antibody (anti HIV Ab), immunoglobulin plasma levels (IgG, IgA and IgM) and Lymphocyte Subtype enumeration.

As diagnosis is the first step to timely access to treatment, a great advance has been made. IPOPI is committed to continue to work with the WHO as the EDL develops over the years, to ensure people living with a PID can be spared the diagnostic odyssey they too often have to face and its disastrous consequences. The more visibility we give to PIDs, the more we will diagnose and the more we will treat or even cure!

RELATED MEETINGS

IPOPI actively participated in the following events among others:

- Health First Europe & TO-REACH Round Table on Innovation and Health Systems, Brussels, January 2018
- Rare Diseases Day Events, Brussels, February 2018
- Accelerating and Improving Diagnosis for Rare Diseases Patients, ERN-BOND Meeting, EU Parliament, Brussels, February 2018
- Rare Lives Photographs Exhibition, EU Parliament, Brussels, February 2018
- EHC Round Table on 'Economics and Access, Health Care Systems and Novel Therapies', Brussels, February 2018
- Stepping Into a Digitalised Future: Integrating eHealth Into Public Healthcare Systems Conference, Brussels, February 2018
- ERNs and Centres of Excellence: a challenge beyond borders Meeting, EU Parliament, Brussels, February 2018
- 44th EBMT Congress, Lisbon, Portugal
- IPFA Asia Pacific Workshop on Plasma Quality and Supply, Kuala Lumpur, Malaysia, March 2018
- 1st African and Arab PID expert meeting, Morocco, March 2018
- IPIA Meeting, Dublin, April 2018
- BPIDG Belgian PID Experts Meeting, Ghent, April 2018
- European Congress on Rare Diseases (ECRD), Vienna, May 2018
- ePAG Workshop, Vienna, May 2018
- 4th Rare Diseases International Annual Meeting, Vienna, May 2018
- 2nd Scientific Congress of the Asia Pacific Society for Immunodeficiencies (APSID), Chongqing, China, May 2018
- IPFA/PEI 25th International Workshop, Athens, May 2018
- Autoimmunity 2018 Congress, Lisbon, May 2018
- HTA Conference Towards a HTA Harmonized Framework, Brussels, May 2018

- World Federation of Haemophilia Congress, Glasgow, May 2018
- NHS meeting on the Use of Immunoglobulins “Putting Policy into Practice”, May 2018
- EP Interest Group on Innovation in Health and Social Care, “Shaping the future of public health towards screening and early diagnosis”, Brussels, June 2018
- SCIDNET meeting, Leiden, June 2018
- FIMSA conference, Bangkok, October 2018
- ISNS Newborn Screening Meeting, Bratislava, October 2018
- European Society for Immune Deficiencies (ESID) & INGRID conferences, Lisbon, October 2018
- ERNs in Action Meeting, Brussels, November 2018
- EU Health Summit, Brussels, November 2018
- EFCNI Workshop, EU Parliament, Brussels, November 2018
- APEC Blood Policy Forum, Taipei, Taiwan, December 2018
- EU Parliament Interest Group Innovation Health & Social Care Meetings, Brussels, 2018, all year

LOOKING AHEAD

IPOPI looks forward to continuing supporting the PIDs community through international awareness, advocacy work and medical education.

New efforts under development, such as, IPOPI's PID Life Index, a data visualisation tool comparing the status of PID diagnosis and care between different countries promise to bring evidence of key areas in need of attention and development.

PIDetect, a PID diagnostics medical training for young physicians will also kick off following a successful pilot project involving a doctor from Vietnam and a PID reference center in The Netherlands.

European Parliament events, EU PID Forums and national advocacy campaigns all across the globe will ensure the PID patients' community is included in important policy-making decisions.

The launch of new projects and NMO programmes including national and regional meetings and workshops will continue to strengthen PID patient organisations around the world.

Don't miss IPIC2019, held in Madrid, Spain at the Marriot Auditorium Hotel, on November 6-8!



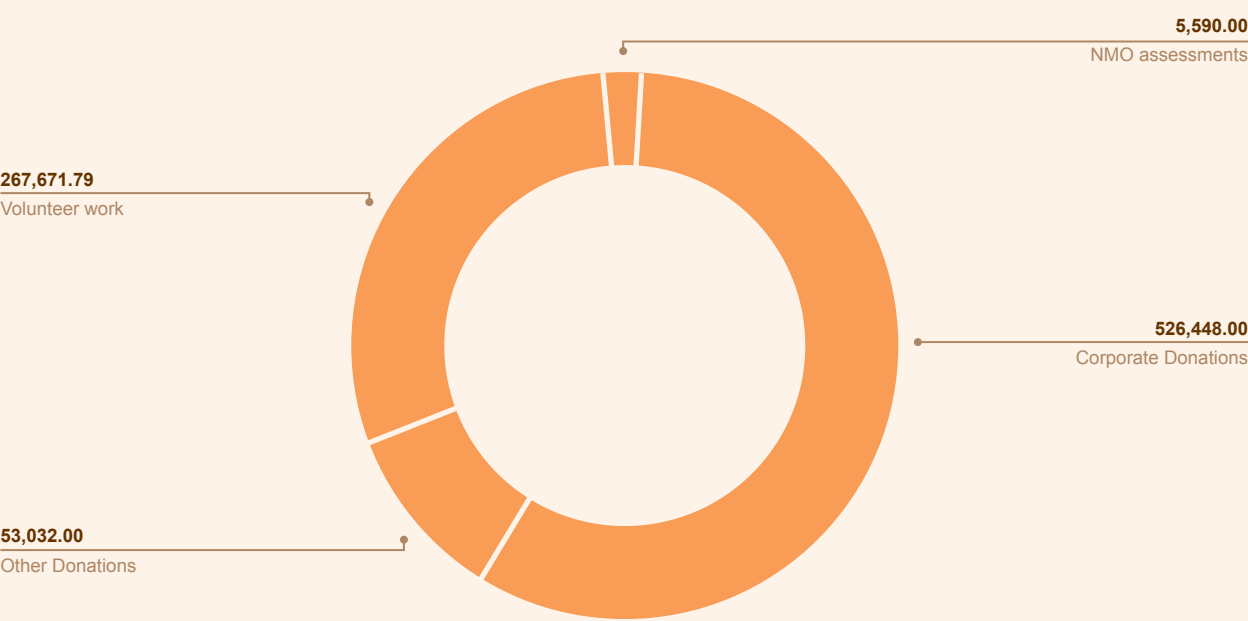


FINANCIAL REPORT 2018

FINANCIAL REPORT 2018

2018 was a good financial year for IPOPI with the highest annual income for a non-congress year. IPOPI was able to organise patient awareness and training programmes and to drive policy and advocacy efforts for better PID care across the globe.

INCOME 2018 (GBP)

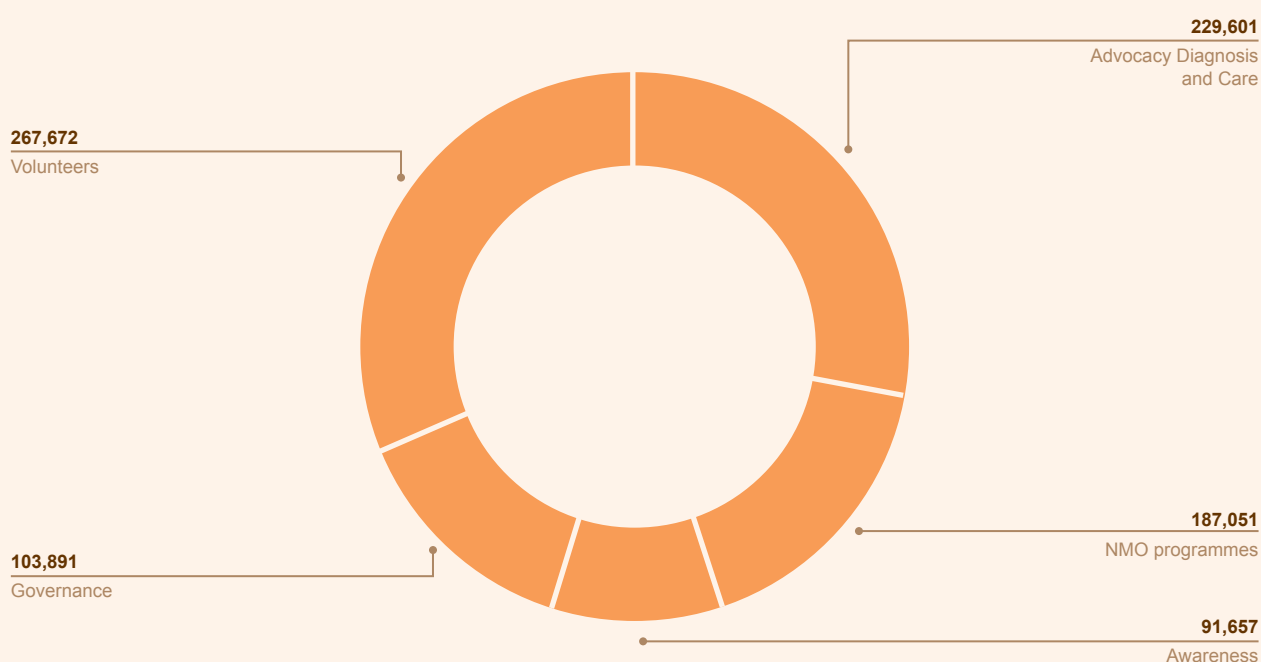


INCOME 2018 (GBP)

NMO assessments	5,590.00
Corporate donations	526,448.00
Other donations	53,032.00
Volunteer work	267,671.79
TOTAL	852,741.79

IPOPI continues to ensure a reserve policy is in place in line with the UK charity commission recommendations. IPOPI is a non-profit charity registered in the United Kingdom and under UK Charity Law. As such, we are required to have our accounts audited every year by qualified accountants and submitted to the Charity Commission for approval. The following charts provide a summary of key aspects of our financial accounts in 2018. Our full audited accounts are available on the Charity Commission website and on request. IPOPI has included an economic valorisation of the work of its volunteers in the following charts based on the model developed by EURORDIS. This value has been independently reviewed by our chartered accountants.

RESOURCES EXPENDED 2018 (GBP)



EXPENDITURE 2018 (GBP)

Advocacy Diagnosis and Care	229,601
NMO programmes	187,051
Awareness	91,657
Governance	103,891
Volunteers	267,672
TOTAL EXPENDITURE	879,872

SOURCES OF FUNDING 2018 (%)

Membership fees	1,00%
Industry funding	62,00%
Other	6,00%
Volunteers	31,00%
TOTAL	100,00%

No contribution received from a single company reached the majority of total income. The highest single contribution by a company represented **22%** of the total income.

IPOPI would like to recognise the large number of people and stakeholder organisations who support our work through personal effort, shared objectives, alliances or financial support. These collaborations help IPOPI achieve more year after year for people living with a primary immunodeficiency worldwide.

We also express our gratitude to our 63 National Member Organisations whose important activities and efforts in their respective countries are vital to improve the conditions for people living with a PID. Our NMOs are key stakeholders who contribute towards the global mission of IPOPI.

CORPORATE PARTNERS

IPOPI would like to extend its sincere thanks to the following corporate partners whose contributions helped us implement our mission through raising awareness, advocacy/ policy efforts, representation in key events, communications activities, organisation of international and regional meetings as well as support to national patient programmes and campaigns.

PLATINUM



GOLD



SILVER



BENEFACTOR



PROJECT SPONSORS

In 2018, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities

- BlueBird Bio
- CSL Behring
- Grifols
- Horizon Pharma
- Kedrion
- NBI
- Octapharma
- Orchard Therapeutics
- Perkin Elmer
- Pfizer
- PPTA
- Shire/Takeda

IPOPI's corporate relations with the above-mentioned sponsors are subject to IPOPI's corporate relations guiding principles available on IPOPI's website.

KEY STAKEHOLDERS

IPOPI has strategically been growing its stakeholder cooperation and we would like to express our most sincere thanks to a number of stakeholder organisations for their partnership and collaboration in 2018 including but not limited to:

alpha-1 Global, African Society for Immunodeficiencies (ASID), Arab Society for Primary Immunodeficiencies (ARAPID), Asociación Española de Pediatría (AEP), Asia Pacific Economic Cooperation (APEC) Blood Policy Stakeholders Forum, Asia Pacific Society for Immunodeficiency (APSID), Australasian Society of Clinical Immunology and Allergy (ASCIA), Belgian Primary Immune Deficiency Group (BPIDG), British Society for Immunology (BSI), Care-for-Rare Foundation, Chulalongkorn University Hospital Bangkok, Clinical Immunology Society (CIS), Cipto Mangunkusumo Hospital Jakarta, Council of Europe, European Commission, European Federation of Immunology Societies (EFIS), European Foundation for the Care of Newborn Infants (EFCNI), European Health Coalition, European Medicines Agency (EMA), the European Haemophilia Consortium (EHC), European Network For Paediatric Research (Enpr-EMA), European Parliament, European Patients Forum (EPF), European Platform for Patient Organisations, Science and Industry (EPPOSI), Eurordis, European Reference Network RITA-ERN, European Society for Blood and Marrow Transplantation (EBMT), European Society for Immunodeficiencies (ESID), Faculty of Medicine Universitas Indonesia (FMUI), Federation of African Immunology Societies (FAIS), Federation of Clinical Immunology Societies (FOCIS), Federation of Immunological Societies of Asia-Oceania (FIMSA), Food and Drug Administration (FDA-US), Grupo Português de Imunodeficiências Primárias (GPIP), Hanoi National Hospital of Paediatrics, Health First Europe (HFE), Hungarian Paediatrics Society, Hungarian Society for Allergology and Immunology, Indian Academy of Pediatrics, Indian Society for Primary Immunodeficiency, International Alliance for Patient Organizations (IAPO), International Nursing Group for Immunodeficiencies (INGID), International Immunocompromised Host Society (ICHS), International Plasma Fractionation Association (IPFA), International Society for Blood Transfusion (ISBT), International Society for Neonatal Screening (ISNS), International Union of Immunological Societies (IUIS), Latin American Society for Immunodeficiencies (LASID), Le Centre de Référence Déficiences Immunitaires Héritables (CEREDIH), MSAI (Malaysian Society for Allergy and Immunology), Plasma Protein Therapeutics Association (PPTA), Platform of Plasma Protein Users (PLUS) and its member organisations, RECOMB H2020 consortium, SCIDNET H2020 consortium, Sociedad Española de Inmología (SEI), SEAPID (South East Asia Primary Immunodeficiencies Network), Sociedade Portuguesa de Alergologia Imunologia Clínica, Sociedade Portuguesa de Pediatria- infecciology pediatria, Thai Immunology Society (AAIAT), Vietnamese Paediatrics Association, World Federation of Haemophilia, World Health Organisation (WHO), World PI Week and its member organisations.

IPOPI is a non-profit charity registered in the UK, registration number 1058005
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www.ipopi.org



INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES
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