



IPOPI 13<sup>th</sup> PID Forum - Newborn screening for rare diseases. A PID perspective, held at the European Parliament, Brussel, Belgium, December 4, 2019.

# REVIEW OF ACTIVITIES AND ACHIEVEMENTS 2019

an IPOPI event



# IPIIC2021

INTERNATIONAL PRIMARY  
IMMUNODEFICIENCIES  
CONGRESS

BERLIN-GERMANY

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**DIAGNOSIS  
AND CLINICAL CARE**

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**BERLIN,  
3-5 NOVEMBER 2021**

# A MESSAGE FROM IPOPI'S PRESIDENT AND EXECUTIVE DIRECTOR

2019: the new frontier



**MARTINE PERGENT**  
President



**JOHAN PRÉVOT**  
Executive Director

430 – this is the number of Human Inborn Errors of Immunity identified in the latest classification published by the International Union of Immunological Societies (IUIS). In the past 10 years the number of known primary immunodeficiencies (PIDs) has more than doubled. Whilst the field is developing at a fast pace the entire patient community is on the move as well. IPOPI is proud to work and represent a growing number of 69 national member organisations (NMOs) who all share our common vision to improve PID patient lives. When working in a field that is expanding as rapidly as primary immunodeficiencies and especially when this work involves representing the interests of the patient community, it is of the utmost importance to stay up to date with the latest research, treatment and diagnosis advances. This also ensures that

patient centredness, the theme of the 2019 World PI Week campaign, can successfully underpin the processes by which these advances are made. Awareness and education were therefore prioritised on the IPOPI agenda in 2019!

The 4th edition of our International Primary Immunodeficiencies Congress (**IPIC2019**) was held in Madrid, Spain. This was a fantastic meeting and the best attended to date with 750 delegates from 70 countries and every region of the world. Clinicians, patient group leaders and representatives, nurses, industry stakeholders and other healthcare professionals met around a scientific programme fully dedicated to advancing knowledge on the latest clinical management and diagnosis developments in the field. The congress was a great example of stakeholder collaboration and received the valuable support of 37 supporting scientific and professional organisations as well as 21 corporate partners. Interaction underlined the overall congress approach and the programme featured many successful flash poster presentation sessions enabling young researchers to present and discuss their work with the experts and delegates.

IPOPI has strengthened its educational programme through the organisation of successful Clinical Care Webinars and the publication of new patient information leaflets. Skills-building meetings targeting our regional patient group leaders were also organised.

IPOPI continued to work with its partner organisations and coordinated two successful regional PID patient meetings in Africa (Senegal, collaborating with the African Society for Immunodeficiencies) and in Latin American (Mexico, in collaboration with the Latin American Society for Immunodeficiencies).

Advocacy will always remain at the core of IPOPI's mission to improve access to early diagnosis and optimal treatments for PID patients worldwide. A significant step forward in 2019 and the culmination of a campaign led by IPOPI, in collaboration with IUIS and several other PID stakeholder organisations, was the inclusion of PID diagnostic tests into the World Health Organisation (WHO)'s List of Essential In-Vitro Diagnostic Tests. Since diagnosis is the first step to timely access to treatment; this represented a great advance and IPOPI continues to be committed to work with the WHO as the EDL evolves in the future. This will ensure people living with a PID can be spared the diagnostic odyssey they too often have to face and its disastrous consequences.

IPOPI also worked on advocacy programmes and campaigns in different world regions. These included our active participation in the Asia Pacific Economic Cooperation (APEC) Rare Disease Policy Workshop in Chile as well as at the United Nations' High-Level Event of the NGO Committee for Rare Diseases in New York, USA. Our very successful 13th PID Forum at the European Parliament which saw the launch of a Call to Action on newborn screening for rare diseases supported by 30 Members of the European Parliament. This came as an additional important step in IPOPI's campaign to ensure babies born in the EU with rare diseases can have equal access to newborn screening, a life-saving tool for a condition such as Severe Combined Immunodeficiency (SCID). Additionally, IPOPI continue to provide policy and advocacy support to several of our national member organisations in Europe, Africa and Latin America.

It is both with pride and gratitude to all our NMOs and stakeholders for their invaluable support and collaboration that we look back at 2019 as a year where new great milestones were reached. For this, we thank you and look forward to more great achievements in the upcoming decade!

Martine Pergent  
IPOPI President

Johan Prévot  
IPOPI Executive Director

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# IPOPI STRATEGIC PLAN

## MISSION STATEMENT

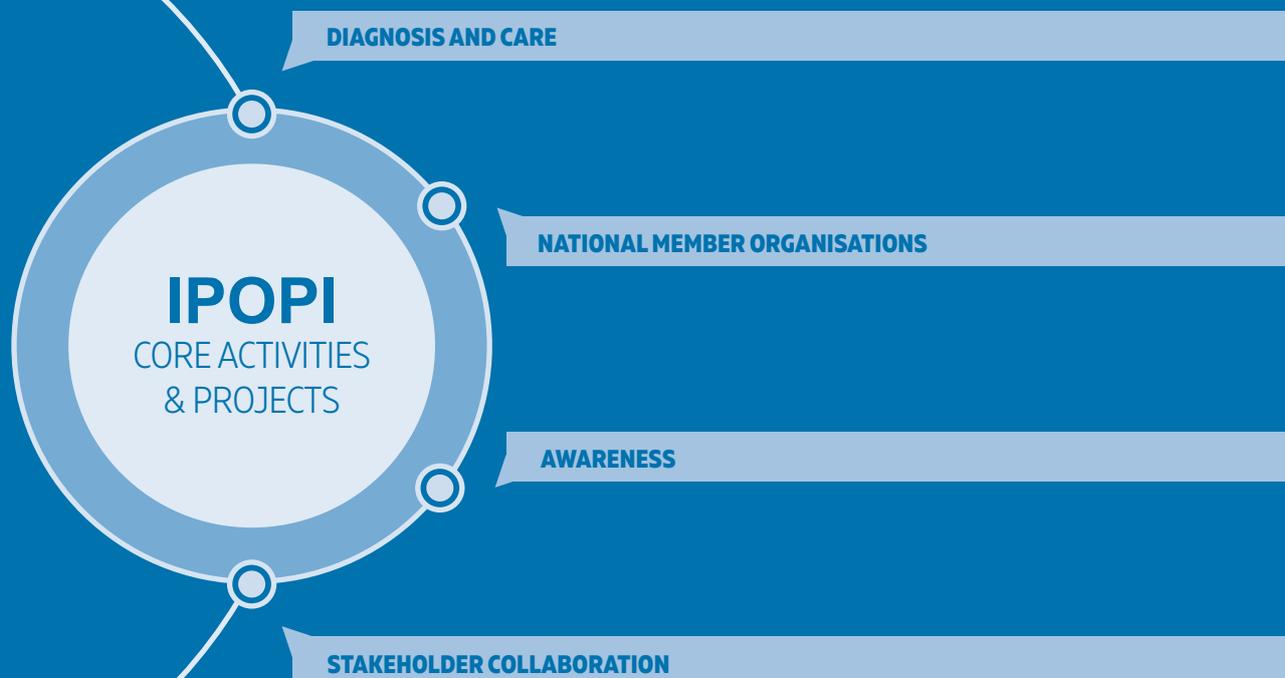
The Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide through global collaboration.

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 over 430 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 now considered to be human inborn errors of immunity 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 been included in the World Health Organization (WHO) as Essential Diagnostics List (EDL) in a dedicated PID section recognising 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 specialised 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 antibiotics, 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 caregivers. 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 individualised basis according to patient needs and preferences.

Tremendous advances have also been made with the development of new gene therapy treatments.

During difficult health access periods, such as **the COVID-19 pandemic** we are living, it is crucial that national and regional authorities prioritise PID patients' access to IGs in case of any supply tensions or shortages.

## CLASSIFICATION

PIDs are currently classified into ten groups: immunodeficiencies affecting cellular and humoral immunity; CID with associated or syndromic features; predominantly antibody deficiencies; diseases of immune dysregulation; congenital defects of phagocyte; defects in Intrinsic and Innate immunity; auto-inflammatory disorders; complement deficiencies; bone marrow failure; phenocopies of PID.

**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 leaflets page on our website <https://ipopi.org/publications/leaflets/>



## IPOPI'S KEY ACHIEVEMENTS AND ACTIVITIES, 2019

### 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.

#### IPIC2019 IN MADRID

IPOPI welcomed 750 participants interested in learning more about diagnosis and clinical care of primary immunodeficiencies (PIDs) at IPIC2019, the fourth International Primary Immunodeficiencies Congress, that took place in Madrid, Spain, at the Hotel Marriott Auditorium, on November 6-8 2019.

The congress was designed to advance PID clinical care by putting the patients' interest at the centre of all discussions. The scientific programme featured world-renowned experts in the field of PID as well as other key stakeholders and addressed pertinent topics, such as, Management of respiratory issues in PID, Novel PID diagnostics, Management of complex PID cases, Regional clinical priorities, Gene therapy developments, Managing PID in adulthood and old age, Ethical issues around PID care and diagnosis, Perspectives on future care and diagnosis developments.

**Dr Enrique Terol**, the European Commission representative for European Reference Networks (ERNs), was invited to give the Welcome Address, an interesting overview of the idea behind the creation of ERNs – tackling complex and rare conditions requiring specialised healthcare, knowledge and resources.

IPIC2019 was **endorsed by 37 medical and professional societies** and was granted 12 Continuing Medical Education (CME) credits by the European Union of Medical Experts.

**213 abstracts for posters presentation** were approved for the congress, many of which by young physicians with an interest in PID. The authors of the three best posters were distinguished with a monetary grant and given the opportunity to present in a dedicated congress session.

**Martine Pergent**, IPOPI President, stated that IPOPI was ‘excited to be back in Europe for the 2019 edition of IPIC building on previous events.’ She also mentioned that IPIC2019 delivered a diverse and stimulating scientific programme focused on the state of the art in PID diagnosis and clinical care.’

**IPIC2021 will be organised in Berlin, Germany**, on 3-5 November 2021. IPOPI expects excellent attendance with the participation of key international PID experts in the programme.



### 13<sup>TH</sup> IPOPI EU PID FORUM – LAUNCHING A NBS FOR RARE DISEASES CALL TO ACTION

IPOPI's 13<sup>th</sup> EU PID Forum was dedicated to Newborn screening for rare diseases. A PID perspective took place on December 4, 2019 at the European Parliament (Brussels, Belgium).

We are glad to have had 4 Members of the European Parliament (MEPs) co-chairing, namely, **Dr Manuel Pizarro** (Social-Democrats, Portugal), **Ms Tilly Metz** (Greens, Luxembourg), **Ms Irena Joveva** (Renew Europe, Slovenia) and **Ms Sirpa Pietikainen** (European People's Party, Finland). **Dr Tudor Ciuhodaru** MEP (Social-Democrats, Romania) actively participated in the meeting.

The meeting set up the scene for a strong political debate on newborn screening for severe combined immunodeficiencies as well as other severe forms of PIDs (such as complete Di George syndrome) and launched a Call to action, highly supported by the MEPs present and by the Forum participants.

The need for the development and implementation of overarching guidelines in the field of newborn screening for rare diseases and the creation of a European newborn screening standing committee was observed. This would facilitate the exchange of best practices and recommendations on newborn screening and allow national decision-makers to better access information and solid evidence from other Member States.



## IPOPI PID CLINICAL CARE WEBINARS

### ESSENTIALS ON PID DIAGNOSTICS WITH DR KATHLEEN SULLIVAN

IPOPI organised the 2<sup>nd</sup> webinar on PIDs' Clinical Care on February 6, on the topic of **Essentials on PID Diagnostics**. The half-hour webinar was moderated by IPOPI's President, Mrs Martine Pergent, and the invited speaker was **Dr Kathleen Sullivan** from Children's Hospital Philadelphia, USA.

The webinar attracted 120 registrations from 38 different countries including medical professionals and biologists as well as patients, patient advocates and industry representatives.

### IMMUNOGLOBULIN THERAPIES: FROM GOOD TO BEST PRACTICE WITH PROF TROY TORGERSON

IPOPI held the 3<sup>rd</sup> **Clinical Care Webinar on 26 September**, on **Immunoglobulin therapies: From good to best practice** with **Professor Troy Torgerson** from University of Washington and Seattle Children's Hospital, US. The event gathered 100 participants from 30 countries.

Prof Torgerson demonstrated how personalised care is very important for PID patients in need of immunoglobulin therapies and that a large range of immunoglobulin therapies are necessary to ensure that the patients receive the best possible care.

The worldwide audience of both events was able to send written questions which were answered by the speakers and by Dr Nizar Mahlaoui, the President of IPOPI Medical Advisory Panel (MAP) during and after the webinar. Both webinars were clinically oriented but suitable for a non-medical audience as well.

All IPOPI Clinical Care webinars can be viewed on [TV.IPOPI.org](http://TV.IPOPI.org).



Dr Kathleen Sullivan



Webinar 'IG Therapies: From good to best practices

## OTHER ADVOCACY CAMPAIGNS

### RARE DISEASES' EVENTS IN NEW YORK CITY, US

IPOPI actively participated in several rare diseases' events held in February, in NYC, US. **Johan Prevot**, IPOPI Executive Director, and **Roberta Anido de Pena**, IPOPI Board member, responsible for the Latin American region, actively represented IPOPI at the events.

At the 5<sup>th</sup> Rare Diseases International (RDI) Annual Meeting held at the Microsoft Global Headquarters, Johan Prevot, shared IPOPI's experience with WPIW, suggesting this may be a good model for RDI, to support local campaigns, in particular, in developing countries, during rare diseases' day, in order to ensure national implementation. IPOPI also attended the launch of the Global Commission to End the Diagnostic Odyssey of Children with a Rare Disease digital roadmap.

At the 2<sup>nd</sup> High-Level Event of the NGO Committee for Rare Diseases, held at the United Nations headquarters, the UN theme of 'Leave no one behind' clearly underpinned the dialogues of the round table discussion and was used to strengthen the message supported by all attending stakeholders that rare diseases should be seen as a global public health priority within the United Nations. A call for action was launched for the integration of rare diseases into the upcoming landmark UN political declaration on universal health coverage (UHC) and a UN resolution on rare diseases.



## IPOPI AT THE APEC RARE DISEASE POLICY WORKSHOP

IPOPI's Board Member **Roberta Anido de Pena** attended the Asia-Pacific Economic Cooperation (APEC)'s meeting on Implementing the APEC Action Plan on Rare Diseases in Latin America on July 10-11, in Santiago, Chile. APEC members Chile, Mexico and Peru help set the scene for the discussion by sharing the state of care for rare diseases in these countries. The meeting highlighted how it is necessary to think globally when it comes to rare diseases, establishing partnerships with various stakeholders. Newborn screening, diagnosis and management of rare diseases remain uneven across LATAM and it was agreed by Workshop participants that patient involvement and empowerment is pivotal to ensure the rights of patients are taken into account in policy making.

## IPOPI 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 the COVID-19 pandemic, plasma and immunoglobulin supply, SCID Newborn Screening, gene therapy, among others.

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

## IPOPI PID REGIONAL EVENTS

### IPOPI REGIONAL AFRICAN PATIENTS MEETING IN DAKAR, SENEGAL

IPOPI's African Regional Meeting was organised between April 11-12, back to back with the 6<sup>th</sup> ASID Biennial Congress, and brought together patient representatives from seven countries spread over the African continent. A great opportunity to exchange experiences and strengthen their national campaign efforts towards better care for PID patients in their countries and increase regional cooperation.

The meeting programme featured a joint opening ceremony with ASID, followed by a **two-day IPOPI meeting which combined educational lectures and patient workshops**. It was well-attended not only by patient representatives but also by physicians, nurses, other PID and Rare Disease organisations and company representatives from Africa and other parts of the world. During these two days, participants increased their knowledge about the status of PID diagnostics and treatments, as well as about future possibilities for the African region. This was combined with knowledge exchange amongst the NMOs and a workshop where they presented and discussed several interesting advocacy ideas, both for immediate action and longer-term commitments. The suggested advocacy ideas from the NMOs and the meeting in general gave great hope for the future of PID awareness, diagnosis, treatments and registries in Africa.



Participants at the meeting



Advocacy workshop

## IPOPI REGIONAL LATAM MEETING

IPOPI was glad to organise the Regional Latin American PID Patients' Meeting, held in parallel with the biennial Latin American Society for Immunodeficiencies (LASID) congress. The meeting took place on October 10-11 in Cancún, Mexico, and brought together patient representatives from a great majority of IPOPI's Latin American National Member Organisations (NMOs), including Argentina, Bolivia, Brasil, Ecuador, El Salvador, Mexico, Nicaragua, Paraguay, Puerto Rico and Venezuela.

During the two days of the meeting participants learnt about topics such as *The ABC of PIDs and autoimmunity, Vaccination and PIDs, Plasma collection, Immunoglobulin therapies* and many more. They were also given the chance to present the situation in their countries and the important work they are doing, providing the participants with the shortcut of learning from each other.

The meeting had an important **advocacy** scope, linking the international, regional and national environments. The meeting workshops challenged participants to simulate a meeting with the Ministry of Health, prepare and participate in a simulated press conference as well as develop a proposed strategic plan for their NMOs.



Simulated press conference



LATAM NMO patient representatives

## IPOPI PID NATIONAL EVENTS

### IPOPI'S 3<sup>RD</sup> VIETNAMESE DOCTORS AND PATIENTS MEETING

The 3rd IPOPI Vietnamese Doctors and Patients' Meeting, following two meetings in 2015 and 2017, took place on November 23-24, in Hotel Nikko Saigon, Ho Chi Minh, and was organised in close collaboration with Children's Hospital 1. It was a well-attended and successful meeting, hosting 100 doctors on the first meeting day as well as 40 PID patients and parents during the second day.



Doctors' meeting in Vietnam

The first day was focused towards **educating healthcare professionals and medical students** about primary immunodeficiencies (PIDs). This symposium featured both international and national speakers who provided various interesting lectures on key PID diagnosis and clinical management, with topics such as *The A to Z on PIDs*, *Molecular diagnosis and flow cytometry and Vaccination and PIDs*, among others. This day also featured a session where local doctors were introduced by international experts to present their case studies, creating a good interactive environment.

The second day was devoted to **patient education** and welcomed patients and parents from both south and north Vietnam, among them active members from IPOPI's Vietnamese national member organisation, VietPIPs. This day offered sessions on *What are PIDs, a comparison of IVIG and SCIG* as well as an enlightening Q&A session with IPOPI MAP Chair, Dr Nizar Mahlaoui, and Vice-Chair, Prof Martin van Hagen. During this session, patients and parents provided the experts with many interesting and difficult questions. This fruitful meeting day also offered national updates from Vietnam, Malaysia and Australia as well as an awareness workshop where the participants brainstormed about a campaign for World PI Week 2020.



### **WORLD PI WEEK 2019 - BRINGING ABOUT CHANGE FOR PID PATIENTS WORLDWIDE**

Guided by the theme “*Putting primary immunodeficiency patients at the center of their care*”, the 2019 campaign aimed at improving the recognition, diagnosis, treatment and quality of life for people with primary immunodeficiencies worldwide.



WPIW is a global campaign celebrated each year between 22-29 of April. The 2019 edition mobilised national PID patients, families, physicians, experts and other stakeholders in at least 48 countries, launching social media campaigns, family days, patient-specialist meetings, educational conferences, public lectures, TV and radio interviews and much more.

IPOPI works hard for WPIW to be implemented in as many countries as possible. We had the pleasure of supporting 31 of our national member organisations in their WPIW campaigns.

To facilitate participation, the WPIW Steering Committee, of which IPOPI is a founding member, provided a range of different materials aimed at helping participants reach out to both the public and the media, as well as to politicians and the medical community. Some of the available tools included a campaign package for social media with banners and logos, engagement toolkits, press releases, an infographic, photo contest and much more.

### **IPOPI NMO SUPPORT PROGRAMME 2019**

Throughout 2019 IPOPI was pleased to be able to fund several NMO projects and campaigns through its NMO Support Programme.

- Austrian National PID awareness and film materials
- Indonesian PID advocacy campaign and family day.
- Ecuador First National PID Meeting in Cuenca
- PID Consultative seminar and two seminars at Kabale in Uganda
- MyPOPI for their 3<sup>rd</sup> PID Family day
- Finnish PID video project
- Iceland Youth camp
- Malaysian 3<sup>rd</sup> Family day
- Nordic immunoglobulin workshop and Nordic PID meeting
- IPOPI leaflets for distributing among patients and nurses
- Awareness campaign in Kenya
- National conference in Romania
- Funding towards venue, materials and equipment in Puerto Rico
- IPOPI leaflets for IDFA National Conference in Australia
- IPOPI leaflets for the 4<sup>th</sup> Bolivian PID Conference

Among many campaigns!



Nordic immunoglobulin workshop and Nordic PID meeting

## STRATEGIC OBJECTIVE 3 - TO RAISE PID AWARENESS GLOBALLY

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

### NEW IPOPI PID INFORMATION LEAFLETS

IPOPI has since 2011 been producing primary immunodeficiencies (PID) information leaflets and a new series was published in 2018. In 2019 IPOPI published three leaflets dedicated to *CVID Management*, *PIDs and HSCT* and *A Guide for Internal Medicine Specialist* 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.

*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 in PDF and in eBook format, soon will be available in Portuguese and many other languages.



## 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 IPOPI's and its members most important achievements. Our newsletter's editorial, signed by IPOPI's President, is always one of the most read articles, as are the interviews with leading PID community members.

To subscribe to e-News please visit [e-news.ipopi.org](http://e-news.ipopi.org)



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**IPOPI**  
INTERNATIONAL  
PATENT ORGANISATION  
FOR PRIMARY IMMUNODEFICIENCIES

e-News Winter 2019

Kopaonik, SERBIA

**From the President's desk**

There is something about looking at another year, and even a new decade, that encourages reflection. On the past and on the future. A look back to the last quarter shows our growing activities on awareness and advocacy and among these, we organized IPIC2019, the 4<sup>th</sup> edition of the International Primary Immunodeficiencies Congress!

[Read more](#)

**IPOPI News**

## STRATEGIC OBJECTIVE 4 - TO STIMULATE STAKEHOLDER COLLABORATION

IPOPI values the benefits of working together with partners and stakeholders and 2019 was a shining example of new partnerships being built.

### WHO ACKNOWLEDGES ESSENTIAL DIAGNOSTICS FOR PIDS

PID diagnostic tests were included in the World Health Organization (WHO)'s Essential Diagnostics List (EDL) published by WHO on July 9, 2019.

The inclusion of PID diagnostics tests follows a successful joint application by **IPOPI and IUIS** (International Union of Immunological Societies) which was supported by numerous other organisations and societies in the field. This achievement is an important step in the international advocacy IPOPI leads to ensure awareness about PIDs, as this list provides evidence-based guidance and sets a reference for the development or update of national lists of essential in vitro diagnostic tests. IPOPI through its work on the list hopes to facilitate the advocacy

led by its national member organisations in collaboration with national experts to ensure that the diagnostics needed for PIDs are available at national level.

The **diagnostics now recognised as essential** for the diagnosis of PIDs by the WHO are the following: immunoglobulin plasma levels (IgA, IgM, IgG) to identify patients with low Ig levels and monitor replacement, lymphocyte subtype enumeration (CD4, CD8, CD20, NK (CD16/56)) to aid in the diagnosis of primary and secondary immunodeficiencies, HIV ½ antibody for the differential diagnosis of PIDs, complete blood count to detect a wide range of disorders amongst which PIDs.



The inclusion of PIDs in the WHO EDL efficiently complements the WHO Essential Medicines List where immunoglobulin therapies are already included for the treatment of PIDs, and will help bridge the gap between PID diagnosis and access to treatment.

#### **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 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.

RITA has grown a lot since its inception and currently has 24 full members.

## RECOMB & SCIDNET PROJECTS

IPOPI is a contributor to both of these ground-breaking projects and excited to bring in the voice of the patients with PID into the research and development process of these highly innovative therapies for SCID.

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.

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

Martine Pergent (IPOPI President), Johan Prevot (IPOPI Executive Director) and Leire Solis (Health Policy and Advocacy Senior Manager) attended the SCIDNET and RECOMB meetings organised from 8 to 10 April, in Leiden (the Netherlands).

The meetings, organised by the two research projects, brought together more than 50 participants representing most of the partners involved in the consortia to discuss the progress made by the different work groups, assess the timelines set in the proposal and jointly discuss the challenges and potential solutions encountered since the last meetings.

[www.recomb.eu](http://www.recomb.eu)

[www.scidnet.eu](http://www.scidnet.eu)

## PLASMA SUPPLY DEVELOPMENTS

### IPPC2019

The International Plasma Protein Congress (IPPC) took place in Amsterdam on 19 and 20 March, 2019. It focused on the challenges for patient access and the need for more plasma in Europe.

**Jose Drabwell**, IPOPI Board member, was a speaker during the first day and she called for joint stakeholder collaboration to tackle the current immunoglobulin and other plasma-derived medicinal products' supply tensions affecting people living with primary immunodeficiencies (PIDs). **Prof Martin van Hagen** (Erasmus University, Rotterdam), IPOPI's Vice-Chair of the Medical Advisory Panel, provided a clear overview of the reasons why immunoglobulins and biological products are not the same and how the specificities of the patient and of the disease should be taken into consideration before the administration of one particular biosimilar.



## PLUS CONSENSUS MEETING AGREES ON PRINCIPLES TO ENCOURAGE BLOOD AND PLASMA DONATIONS IN EUROPE

The Platform of Plasma Protein Users – PLUS – organised its annual meeting with relevant stakeholders in Estoril (Portugal) on January 24-25, 2019. The meeting was chaired by Dr Jacqueline Kerr, from the Paul Ehrlich Institute, and counted with the participation of PLUS member organisations, associations representing the pharmaceutical industry and the Marketing Research Bureau.

Participants to this meeting reached an agreement on **a set of principles to encourage blood and plasma donations in Europe**. This agreement is of relevance in times of increasing demand of plasma derived medicinal products, such as immunoglobulin replacement therapies, and the potential revision of European legislation on blood medicinal products.



PLUS members at the meeting

## IPOPI REPRESENTS PLUS AT EU COMMISSION ON BLOOD, TISSUES AND CELLS

Following the publication of the Evaluation of the EU legislation on Blood, Tissues and Cells, the European Commission organised a conference in Brussels on October 28, 2019 to present the findings and give stakeholders an opportunity to discuss them. Johan Prevot, IPOPI Executive Director and Steering Committee Member of the Platform of Plasma Protein Users (PLUS), took part in the session on *The challenge of achieving sufficiency and a sustainable supply to meet patient needs*. Mr Prevot spoke on behalf of PLUS to provide the perspective of patients relying on plasma derived medicinal products and stated that **safety of patients means global sufficiency based on regionally balanced plasma collection**.

**Mr Prevot** observed that the EU has and continues to rely on US compensated plasma donors as well as compensated plasma donors from a limited number of EU member states. He called for the EU to look at best practice examples from those member states who have embraced systems that have allowed for a co-existence of the two sectors (public and private) and asked whether the EU was ready to equally encourage compensated plasma donations and voluntary unpaid blood and plasma collection to remedy the current situation.



#### IPOPI AT IPFA/PEI 25<sup>TH</sup> INTERNATIONAL WORKSHOP KRAKOW

IPOPI participated in the 26<sup>th</sup> Workshop on “Surveillance and Screening of Blood-Borne Pathogens” organised by the International Plasma Fractionation Association and the Paul Ehrlich Institute held in Kraków, Poland, and attracted more than 200 delegates from more than 30 countries. **Ms Violetta Kozhereva**, IPOPI Board member, participated in the meeting on behalf of IPOPI.



#### EMA

The European Medicines Agency consider patients, as the end-users of medicines, as stakeholders. IPOPI's Board Member 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.

#### RELATED MEETINGS

IPOPI attended and participated in the following events among others:

- 3<sup>rd</sup> Eurordis multistakeholder symposium on improving patients' access to rare diseases therapies, Brussels, February 2019
- Indian CME meeting and patient congress, India, March 2019
- European Parliament Meeting “The next Generation's recommendations on EU Health Policy”, Brussels, April 2019
- European Health Parliament Think Tank Meeting, Brussels, April 2019
- 6<sup>th</sup> ASID Congress and IPOPI Regional African Patients Meeting, Dakar, April 2019
- All-Russian Patient meeting by Sunflower Foundation (OPPID), Sochi, April 2019

- World PI Week Steering Committee meetings, all year
- World PI Week, 22-29 April, 2019 - "Putting primary immunodeficiency patients at the center of their care" events
- Nordic PID Meeting on immunoglobulins supply, Stockholm, May 2019
- EuropaBio Patients Bio-Forum, Brussels, May 2019
- European Rare Diseases Conference & Eurordis AGM, Bucharest, May 2019
- EFPIA EU Health Coalition Steering Committee meeting, Brussels, May 2019
- 72<sup>nd</sup> World Health Assembly, Geneva, May 2019
- Immune Deficiency Foundation's (IDF) National Conference, Washington DC, June 2019
- European Academy of Allergy and Clinical Immunology, Lisbon, June 2019
- Biotest Immunology Forum, London, June 2019
- PPTA Plasma Protein Forum, Reston, Virginia, June 2019
- 4<sup>th</sup> World Conference on Congenital Disorders of Glycosylation, Lisbon, July 2019
- Australasian Society of Clinical Immunology and Allergy ASCIA annual meeting, Perth, September 2019
- ESID Focused Meeting on PIDs & Malignancies, Brussels, September 2019
- International Conference on Immunology and Microbiology, Tokyo, September 2019
- 20<sup>th</sup> Cambridge Immunology Forum – Primary, Cambridge, September 2019
- 3<sup>rd</sup> Mahgreb Meeting on IG Therapy, Marrakesh, October 2019
- 12<sup>th</sup> Moroccan Conference on PIDs, Marrakesh, October 2019
- EU Commission Conference on Blood Tissues and Cells, Brussels, October 2019
- Enpr-EMA's annual meeting, Amsterdam, October 2019
- AABB Transfusion and Cellular Therapies annual meeting, San Antonio, October 2019
- Rare Diseases and Orphan Products Breakthrough Summit, Washington DC, October 2019
- IUIS - 17<sup>th</sup> International Congress of Immunology, Beijing, October 2019
- Rare 2030 Panel of experts' workshop, Brussels, November 2019
- European Patient Forum's meeting, Brussels, November 2019
- 14<sup>th</sup> International Conference on Allergy and Clinical, Indonesia, November 2019
- RESTORE Science Conference on Advanced Therapies, Berlin, November 2019
- British Society for Immunology Congress 2019, Liverpool, December 2019
- Hospital Necker's programme La Suite Necker, Paris, December 2019
- RITA-ERN working parties calls, all year
- Access to care / patient empowerment meetings and/or calls with doctors and patients in, Ecuador, Israel, Mexico, Nicaragua, Puerto Rico, Brazil, Colombia, Ghana, Kenya, Tunisia, Morocco, Uganda, Zambia, Senegal, Iran, China, Vietnam, Thailand, the Philippines, Indonesia, Hong Kong, Malaysia, Italy, Portugal, Romania, Slovakia, Serbia, Ukraine, Russia, Finland, Estonia, Sweden, Norway, the Netherlands, Belgium, France, USA, UK, Australia among others

## LOOKING AHEAD – 2020 AND BEYOND

IPOPI looks forward to continuous support of the PID community through international awareness, advocacy work and medical education.

Due to the COVID-19 pandemic several of our upcoming projects and meetings had to be readjusted and moved online. IPOPI is actively supporting the PID community during these difficult times including in the form of web chats, FAQs on COVID-19 and PID, as well as, several policy statements.

IPOPI and its Board Members keep monitoring this difficult worldwide situation in all regions of the world and adjusting our activities where needed.

New efforts 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 – is being launched!

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 IPIC2021, in Berlin, Germany, at Hotel Maritim, on November 3-5!



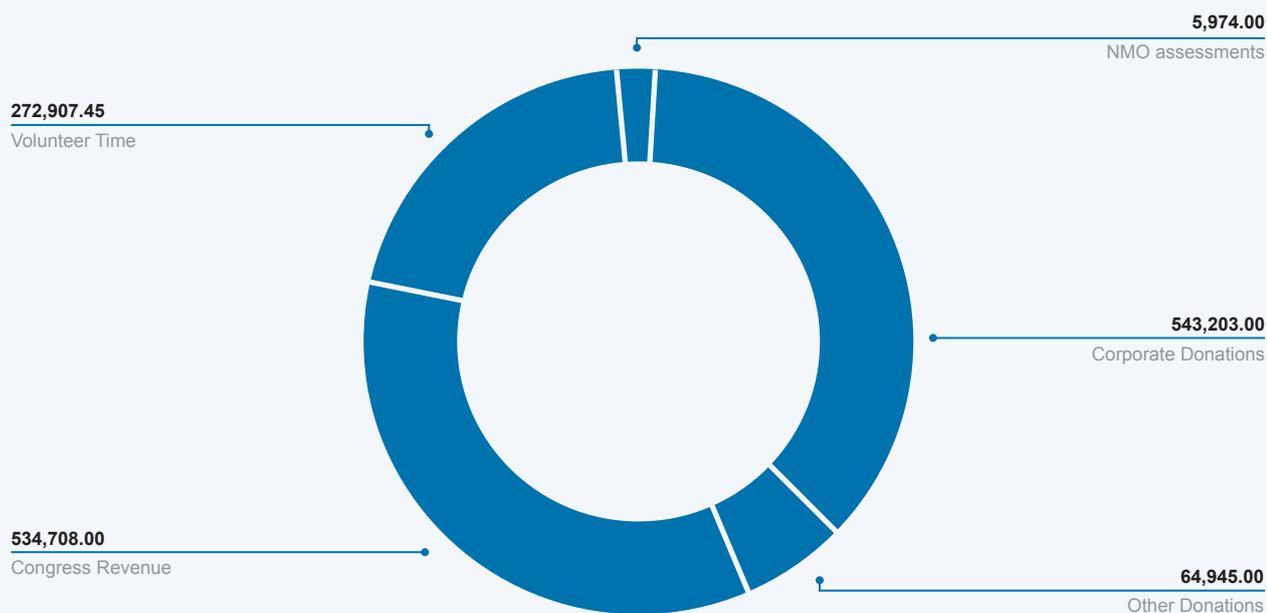


**FINANCIAL  
REPORT  
2019**

# FINANCIAL REPORT 2019

2019 was a good financial year for IPOPI. Besides a successful congress (IPIC2019) IPOPI organised patient and physician meetings and led policy and advocacy efforts to improve PID care across the globe. IPOPI continues to ensure a reserve policy is in place in line with the UK charity commission recommendations.

## INCOME 2019 (GBP)



## INCOME 2019 (GBP)

NMO assessments	5,974.00
Corporate donations	543,203.00
Other donations	64,945.00
Congress Revenue	534,708.00
Volunteer Time	272,907.45
<b>TOTAL</b>	<b>1,421,737.45</b>

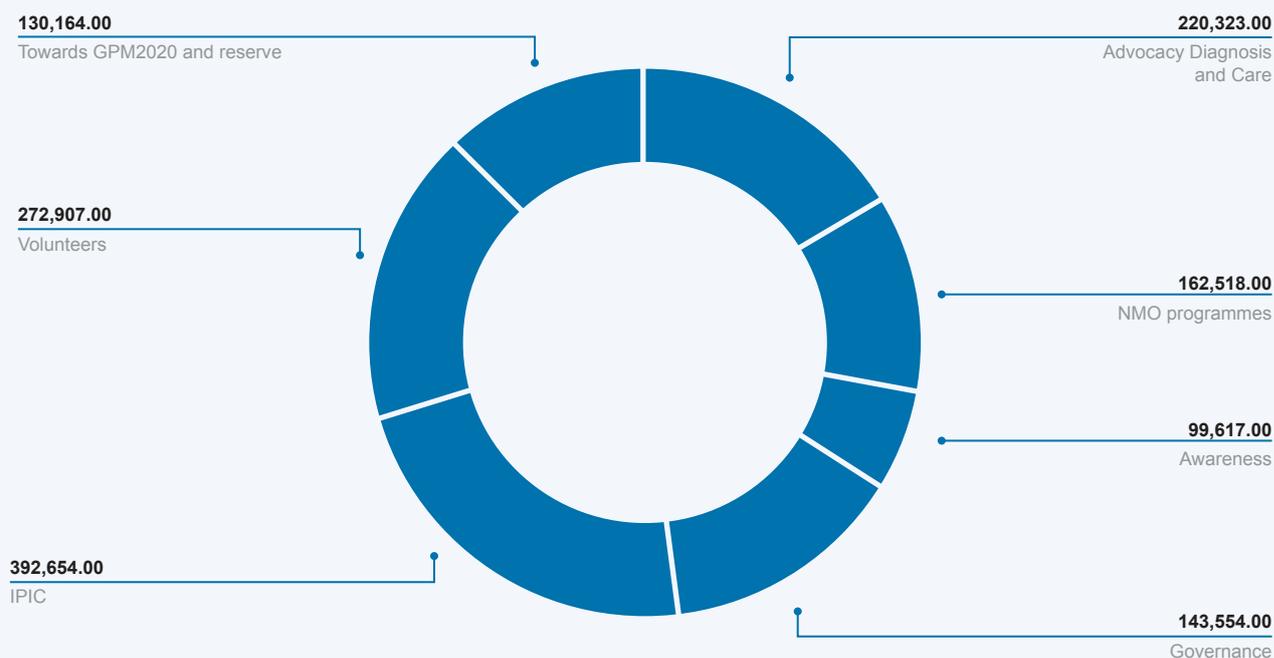
## INCOME 2019 (%)

NMO assessments	0.4%
Corporate donations	38.2%
Other donations	4.6%
Congress Revenue	37.6%
Volunteer Time	19.2%
<b>TOTAL</b>	<b>100.0%</b>

IPOPI is a non-profit charity registered in the United Kingdom and under UK Charity Law. 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 2019. 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 2019 (GBP)



## EXPENDITURE 2019 (GBP)

Advocacy Diagnosis and Care	220,323.00
NMO programmes	162,518.00
Awareness	99,617.00
Governance	143,554.00
IPIC	392,654.00
Volunteers	272,907.00
Towards GPM2020 and reserve	130,164.00
<b>TOTAL EXPENDITURE</b>	<b>1,421,737.00</b>

## SOURCES OF FUNDING 2019 (%)

Membership fees	0.4%
Industry funding	63.2%
Other	4.6%
IPIC	12.6%
Volunteers	19.2%
<b>TOTAL</b>	<b>100.00%</b>

No contribution received from a single company reached the majority of total income. The highest single contribution by a company represented **20%** 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 69 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 2019

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 2019, the following corporate sponsors provided support grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities.

- BlueBird Bio
- Binding Site
- Cane SpA
- Convatec
- Cytognos
- Epimune
- Health in Code
- ImmunoIVD
- Innovative Health Science
- Invitae
- Kedrion
- LFB
- NBI
- Octapharma
- Genethon
- Perkin Elmer
- PPT
- RMS/KORU

IPOPI's corporate relations with the above-mentioned sponsors are subjected 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 2019 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 (IAPPO), International Nursing Group for Immunodeficiencies (INGID), International Immunocompromised Host Society (IICHS), 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 Héritaires (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 e Imunologia Clínica, Sociedade Portuguesa de Pediatria-infecçologia pediátrica, Thai Immunology Society (AAIAT), Vietnamese Paediatrics Association, World Federation of Haemophilia, World Health Organization (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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**INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES**  
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