



# REVIEW OF ACTIVITIES AND ACHIEVEMENTS **2017**







an IPOPI event



# IPI C2019

INTERNATIONAL PRIMARY  
IMMUNODEFICIENCIES  
CONGRESS

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DIAGNOSIS AND  
CLINICAL CARE

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6-8 NOVEMBER  
MADRID, SPAIN





# A Message from IPOPI's Chairperson and Executive Director

## 2017 – Reaching new frontiers

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Dear Friends and Colleagues,

It was in 2017 that IPOPI celebrated its 25th year of existence as an international patient organisation; a quarter of a century after it was launched in Lugano, Switzerland in 1992. Since then, IPOPI has come a long way from a small but ambitious volunteer-driven organisation fuelled by the vision of its founders to an efficient well-resourced international organisation led by a motivated Board and dedicated staff. Today IPOPI represents patients living with a primary immunodeficiencies in all regions of the world, in 63 countries and many more to come. These organisations are the backbone of IPOPI and will always be the most important stakeholders. We were delighted to be able to organise programmes and key events in most regions of the world to complement their great work and support the PIDs community to unite, advocate and raise awareness.

IPOPI was delighted to work jointly with numerous medical societies throughout the course of the year organising regional and national educational events for our national member organisation. A successful regional African PIDs patients meeting was held in cooperation with the African Society for Immunodeficiencies in Livingstone, Zambia, and similarly, IPOPI held an equally productive regional meeting in São Paulo back to back with the Latin American Society for Immunodeficiencies congress. These meetings provided a platform to strengthen IPOPI's regional networks, establish common priorities, launch calls for action and inspire new countries to join in. In Asia IPOPI continued to roll out its development programme with two key national meetings in Vietnam and Indonesia. IPOPI also co-organised an educational meeting for doctors in Thailand in collaboration with the Care for Rare Foundation. In addition, IPOPI actively took part in the drafting of the Asia Pacific Economic Cooperation (APEC) guidelines on blood products Good Manufacturing Practice (GMP) and access to safe plasma therapies.

A significant new frontier explored by IPOPI in 2017 was the organisation of the 3rd International Primary Immunodeficiencies Congress – IPIC2017 - in Dubai, the first time outside Europe and the first ever IPOPI event held in the Middle East. The event was hugely successful with a great level of attendance and received record-breaking support from 34 medical and scientific societies as well as from corporate supporters.

Frontiers are of course not only geographical, and the launch of PID Genius, the first companion app designed for PID patients by PID patients opened new horizons for PID patients by providing them with a virtual personal assistance in the management of their condition. A brand new IPOPI website was also launched, featuring IPOPI's news, publications and a map with relevant PID information from across the globe.

On the advocacy front new frontiers are being explored and IPOPI was delighted to see Norway being the first European country to fully implement SCID newborn screening in their country. IPOPI embarked on new campaigns in Spain and in the UK among others which should lead to more European countries implementing this life-saving public health tool in the future.

Sadly, the word frontiers throws up barriers as well, the fact that access to PID care and diagnosis remains very uneven between countries and sometimes even within regions. 2017 and the first months of 2018 will remain a very difficult period in the lives of the Romanian patients who had to face months without any access to their life saving immunoglobulin therapies which tragically ended with one patient passing away due to a severe infection. After months of battling for their rights, with the support of IPOPI and other stakeholders, it is with great pleasure to report that at the time of writing these lines, the situation has improved, and IG therapies are making their way back in the country. IPOPI want to salute the great work of the Romanian NMO ARPID during this crisis. This and the fact that numerous countries in the world still don't have proper access to PID diagnosis and care is what keeps IPOPI and its member organisations more motivated than ever to advocate for the patients and ensure they can live long and productive lives.

There were many other IPOPI activities and programmes some of which you can read about in this report. Do not hesitate to visit our website or contact us should you wish to receive more information.

We hope you will enjoy reading this annual report and look forward to seeing many of you at the XVth IPOPI Global PID Patients Meeting in Lisbon, Portugal.

Yours sincerely,



Jose Drabwell  
IPOPI President



Johan Prévot  
IPOPI Executive Director



# Executive Committee, Officers and Staff

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# IPOPI Strategic Plan

## MISSION STATEMENT

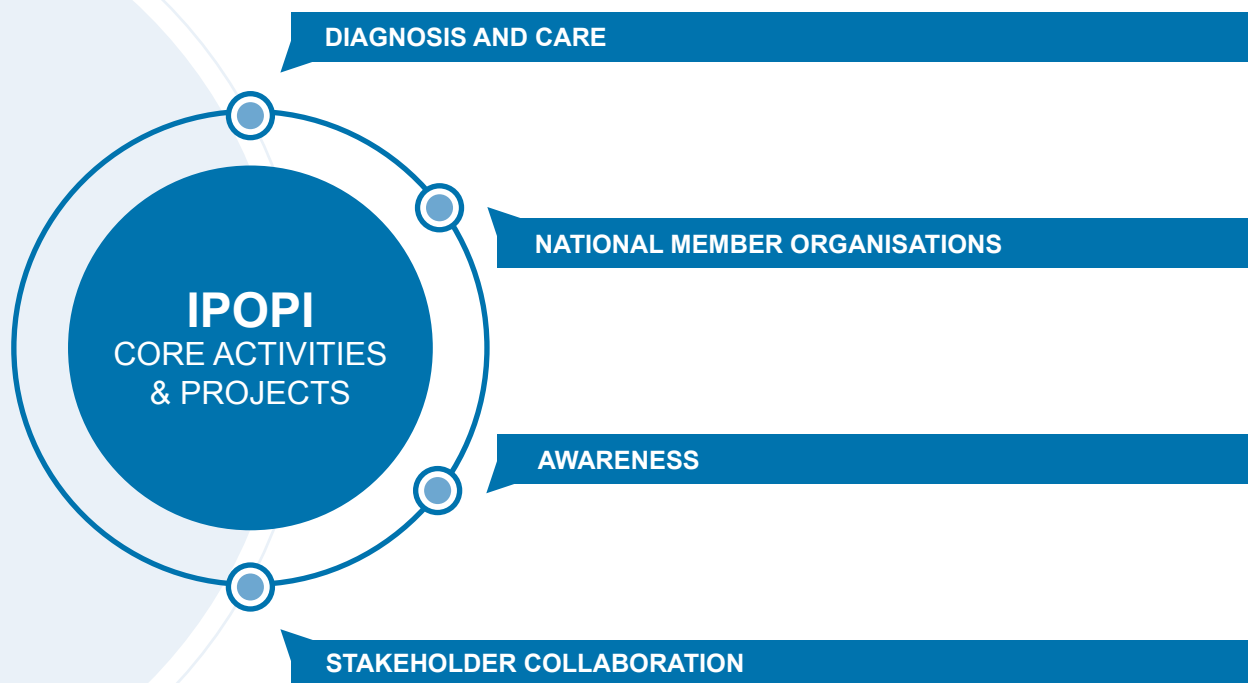
The Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide through global collaboration.

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

## STRATEGIC OBJECTIVES

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?

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Primary immunodeficiencies (PIDs) are a large and growing group of over 350 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 'microorganisms') 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 with a simple and inexpensive blood test, 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.

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 specialise 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 World Health Organisation. 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 individualised 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.



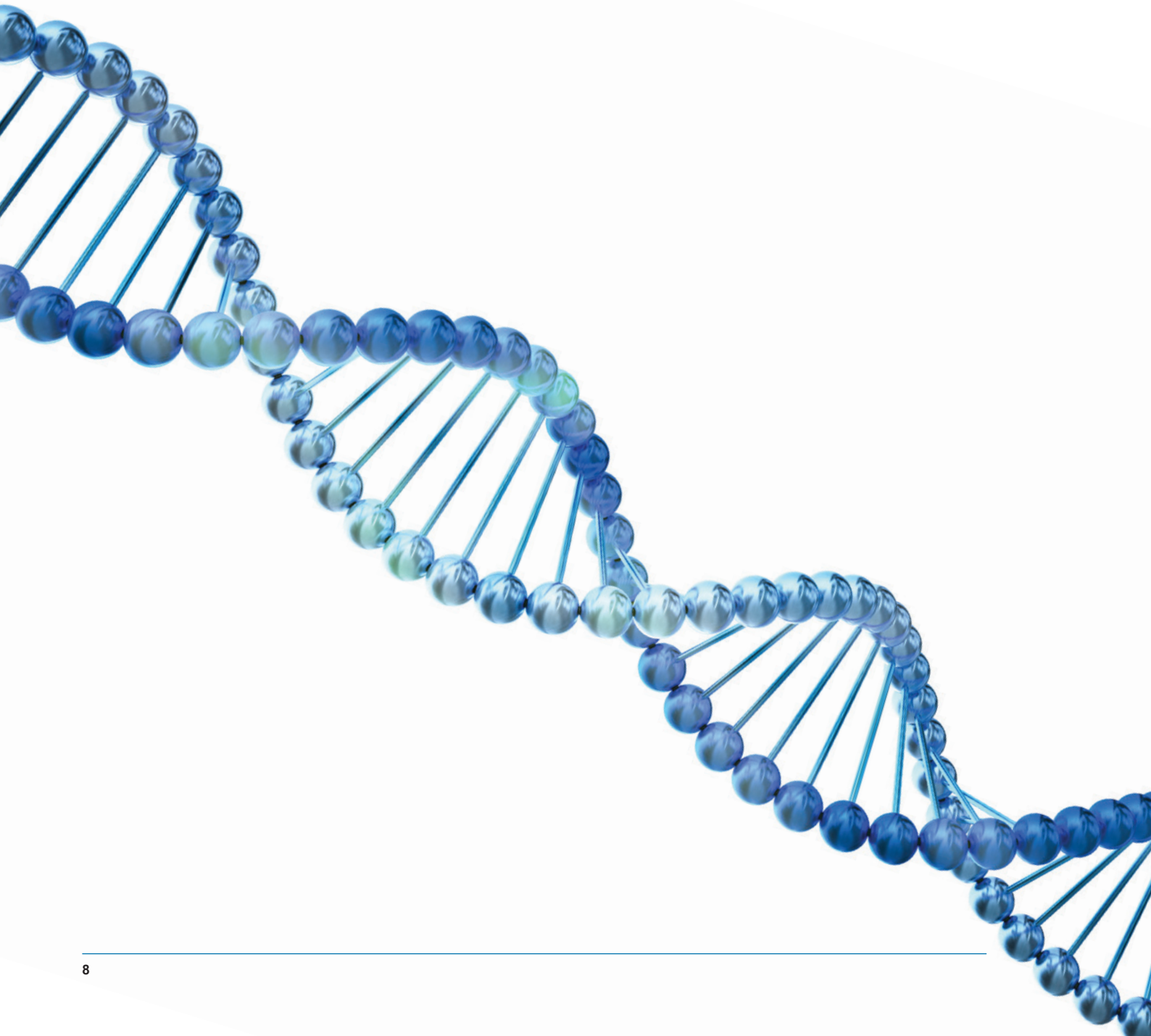
## Classification

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](http://www.ipopi.org/publications)**



# Key achievements and activities, 2017

## 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.

### IPIC2017 in Dubai, UAE, brought together over 630 participants

IPOPI welcomed over 630 participants interested in learning more about diagnosis and clinical care of primary immunodeficiencies (PIDs) at its third International Primary Immunodeficiencies Congress - IPIC2017- in Dubai, United Arab Emirates.

Between 8 -10 November, **40 world renowned medical experts as well as patients, nurses and other stakeholders shared their expertise at IPIC2017**. The congress was designed to advance PID clinical care by putting the patients' interest at the centre of all discussions. Key PID topics such as early detection for optimal care, immune dysregulations, transplant advances, PID clinical priorities by world region, novel treatments, among many others were presented and discussed.

IPIC2017 was **endorsed by 34 medical and professional societies and granted 13 Continuing Medical Education** credits by the European Union of Medical Experts.

Over 170 posters for presentations were submitted to 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 it in a dedicated congress session.

**Mrs Jose Drabwell, IPOPI President**, stated that 'IPOPI is very proud of how well the congress is being received in the Middle East and could not have hoped for a better first edition outside Europe'.



IPIC2017 was well received in Dubai



IPIC congresses are organised every two years and attract physicians with an interest in PIDs, such as immunologists, paediatricians, general practitioners, among others. The uniqueness of IPIC comes from its clinically orientated programme prepared with the input from the key PID stakeholders: doctors, patients and nurses.

IPOPI is happy to announce that **IPIC2019 will take place in Madrid, Spain, on 6-8 November 2019!**  
**[www.ipic2019.com](http://www.ipic2019.com)**



## IPOPI European Parliament PID Forums

IPOPI organised two IPOPI EU PID Forums in 2017 with the support of several Members of the European Parliament (MEPs) and the participation of patient representatives, policy makers and industry representatives. The 9th IPOPI PID Forum on 'Growing older with a PID: transition of care and ageing' was held on 22 June 2017 and the 10<sup>th</sup> IPOPI PID Forum on 'Tackling the diagnostic odyssey in rare diseases through ERNs: the case of PIDs' on 22 November 2017.

The **9th IPOPI EU PID Forum on 'Growing older with a PID: transition of care and ageing'** was hosted by Members of the European Parliament (MEPs) Dr Elena Gentile (S&D Italy), supported by Mr Carlos Zorrinho (S&D Portugal) and counted with the participation of Ms Sirpa Pietikainen (EPP, Finland).

Forum participants agreed that **more attention to specific stages in life** and individual treatment are needed to ensure the well-being of the patient. The meeting discussions were bond into a set of **Recommendations on PID transition care and ageing** used subsequently to raise the issue of PIDs as lifelong diseases among policy makers.



Discussing transition care and ageing



Group picture of the 9<sup>th</sup> PID Forum speakers

The **10<sup>th</sup> IPOPI EU PID Forum** was Chaired by the Member of the European Parliament Mr José Inácio Faria (Portugal), and supported by MEP Mr Rory Palmer (UK) and MEP Ms Nessa Childers (Ireland). The meeting brought together representatives of the European Commission, Permanent Representations, PID patients, academics and healthcare professionals to discuss an issue of key importance across the rare disease field – that of diagnosis.

The 10<sup>th</sup> PID Forum investigated how the newly launched **European Reference Networks (ERNs)** can be used to optimise PID diagnosis in the EU and identify other potential mechanisms to improve PIDs diagnosis.

Key **recommendations** for the European Union were identified: development of harmonised access to a newborn screening in all Member States, especially important for SCID; need for uniform European-wide approach for genetic molecular diagnosis of PIDs; continued funding towards ERNs to improve diagnosis and outcomes of rare disease patients, including those of PID patients; ERN Coordinators should consider, where relevant, organising a working group on newborn screening to provide the European Commission with scientific proof from the Member States; Member States should ensure the use of advanced cross-border information technologies and raise further awareness on PID for better patient referral.



Participants at the meeting



MEP Rory Palmer talking about optimising PID diagnosis



# IPOPI SCID Newborn Campaign

National campaigns in the UK and in Spain

## UK

IPOPI, PID UK (UK PID patient group) and Prof Bobby Gaspar organised a Parliamentary Reception on newborn screening for Severe Combined Immunodeficiency (NBS for SCID) at the **Houses of Parliament, Westminster** on June 20, 2017. The meeting was hosted by Baroness Susan Masham and by Mr Jim Shannon, Member of Parliament (MP) and aimed at gathering political support for the inclusion of SCID in the United Kingdom (UK) national newborn screening programme. The participants of the event signed a **Call to Action** calling for the inclusion of SCID on the UK newborn screening programme that was sent to the UK National Screening Committee (UK NSC).

Soon after, the UK NSC opened a public consultation on the inclusion of SCID NBS to which IPOPI contributed. In the following of UK NSC meeting it was decided that a pilot project for SCID NBS should be started in 2019.



Prof Gaspar and Baroness Masham



SCID families and meeting organisers

## SPAIN

IPOPI and AEDIP (the Spanish national patient organisation) have since 2016 joined efforts to push for the inclusion of SCID NBS. In 2017 a parliamentary event was held at the Spanish Congress on 23 October. The meeting was hosted by Dr Jesús María Fernández, Member of Parliament (MP), and had the support and presence of Dr Francisco Igea MP, Ms Isabel Cabezas MP and Ms Amparo Botejara MP.

These four MPs represented the four largest parties at the Spanish Congress and all of them supported the initiative of expanding SCID newborn screening to the whole country (currently only done in the autonomous community of Catalonia). It is uncommon for these four political parties to agree and support the same initiative, but as it was stated many times during the event, screening for SCID can help diagnosing the disease very early in time and facilitate access to curative treatment.

Given the support reached at the Spanish Congress and the many questions tabled to the Ministry by MPs, it is hoped the response will be positive.

Meanwhile, a Health Technology Assessment (HTA) was commissioned at national level and the Ministry of Health is assessing the administrative request made by AEDIP and supported by IPOPI amongst others.



Committed to expand SCID NBS to the whole country

## Other advocacy campaigns

Among other advocacy campaigns in support of, or in collaboration with, our national member organisations, IPOPI was pleased to continue its collaboration with the **Asian Pacific Economic Cooperation (APEC)** on their blood supply chain programme by providing the patient perspective in the preparation of two sets of APEC guidelines on Good Manufacturing Practices for Blood and Blood Products and the APEC Principles for Enhancing Access to Safe Therapy for Persons with Immunodeficiency and Bleeding Disorder.

## Medical Advisory Panel

2017 marked a change in the leadership of IPOPI's Medical Advisory Panel (MAP) as Prof Bobby Gaspar who had chaired the MAP since 2013 stepped down from his role. Dr Nizar Mahlaoui succeeded to Prof Gaspar as Chairman of the MAP and Prof Martin Van Hagen was appointed as Vice-Chairman of the MAP.

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, 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 express its thanks to Dr Nizar Mahlaoui (Chairman) and to Prof Martin Van Hagen (Vice-Chairman) for their kind contribution and dedication as well as to Professor Gaspar for his advice, support and productive chairmanship mandate.

### 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 national events

### INDONESIA

IPOPI held a two-day meeting in Jakarta, Indonesia, in collaboration with the Indonesian PID Patient Society (IPIPS), the Mangunkusumo Hospital in Jakarta and the South East Asian Network for Primary Immunodeficiencies (SEAPID) on 6-7 May 2017. The first day was dedicated to patients and the second day to physicians.

The **Indonesian Patients Meeting** was held on Saturday 6 and brought together Indonesian patients and their families to discuss national PID priorities. It motivated patients and parents to get involved in IPIPS activities and lead to the formation of a new patients' committee.

A **Clinical Care Meeting** held on Sunday 7 aimed at educating Indonesian MDs and medical students about PIDs and to extend medical knowledge beyond the capital region. Over 170 participants took part in the meeting including paediatricians, allergists, immunologists, nutritionists, infectiologists, parasitologists, dermatologists, pathologists, stem cell specialists and general practitioners as well as medical students and nurses from 4 different islands of Indonesia.



Patients' meeting in Indonesia



IPIPS members drafted next steps



## VIETNAM

IPOPI organised two meetings in Hanoi, Vietnam on 8-9 December 2017, in close collaboration with the National Children's Hospital and the Paediatric Association of Vietnam.

The first meeting took place in the National Children's Hospital in Hanoi and aimed at **educating healthcare professionals and medical students** about primary immunodeficiencies (PIDs). The second meeting was aimed at **PID patients from all over Vietnam** with a programme consisting of educational lectures and discussions.

The **National Patients & Doctors meeting** was a half day meeting and brought together more than 100 participants including patients and their family members, nurses and PID specialists from different regions of Vietnam. The meeting consisted of educational lectures about the immune system and plasma collection and manufacturing. The meeting empowered patients to volunteer in the IPOPI NMO VietPIPS and in identifying the most pressing issues for PID patients in the country.



PID care meeting for healthcare professionals and medical students in Vietnam



Participants glad to take part in the National Patients and Doctors meeting

## THAILAND

IPOPI collaborated once again with the Rotterdam Erasmus University Hospital, Chulalongkorn Hospital and the Care-for-Rare Foundation to co-organise the 2nd South East Asian "From Bench to Bedside" PID meeting in Bangkok, Thailand on 4 December 2017.



## IPOPI regional events

### PATIENTS MEETING AT THE ASID CONGRESS

IPOPI hosted a PIDs patients' meeting in Zambia on April 2, to discuss regional priorities in the African continent. The meeting took place in the city of Livingstone, and was organised back to back with the African Society for Primary Immunodeficiencies (ASID) 5<sup>th</sup> Biennial Congress. Patient representatives from several African countries gave their contribution to a step-by-step plan to improve PID care in the continent.

### LATIN AMERICAN PATIENTS MEETING AT THE LASID CONGRESS

IPOPI organised a meeting with its Latin American NMOs and other patient representatives from Latin America, back to back with the Latin American Society of Immunodeficiencies (LASID) Congress in São Paulo, on 5-6 October 2017.

The IPOPI PID patients meeting was an intense two-day conference consisting of educational lectures and patient workshops, attended by **70 participants including representatives of 15 patient organisations** in Latin America.

IPOPI, LASID and INGID launched a **Call to Action** calling Governments in Latin America to improve the quality of life of PID patients through access to life-saving treatments and equal care across the region. This Call to Action has already been endorsed by many organisations in the PID and rare diseases field and has been signed by over 1000 individuals since its launch.



15 patient organisations from Latin America attended the IPOPI Patients' meeting in São Paulo



## World Primary Immunodeficiencies Week (WPIW)

### WPIW IPOPI EUROPEAN PARLIAMENT EVENT

IPOPI's 2017 WPIW Policy Event took place at the European Parliament on April 25 and focused on the availability and access to immunoglobulin replacement therapies.

The event brought together European, African, Asian and EU neighbouring countries patient organisations, healthcare professionals, researchers, and policy-makers who shared their views on the best solutions to address the problems of the PID community.

**Member of the European Parliament (MEP) Claudiu Ciprian Tănăsescu** hosted the event and started by pointing out that sharing of expertise is cornerstone in improving access to treatments. **Mrs Martine Pergent, IPOPI Vice-President**, highlighted the importance of individualised treatment and invited those present to share and learn from one another.

**Prof Dieye (Senegal) and Prof Meyts (Belgium)** agreed that the priorities of the PID community should be awareness raising, early diagnosis and the establishment of patient registries.

Participants agreed that there are many discrepancies in treatment of PID patients, which are posing a major obstacle to the quality of life of these patients. Mr Karl Petrovsky from the Plasma Proteins Therapeutics Association (PPTA) suggested that the **IPOPI PID Principles of Care** should be endorsed by the European Parliament or even by the European Directorate for Quality of Medicines (Council of Europe).



WPIW at the European Parliament



MEPs, PID specialists, patient representatives, industry representatives together for WPIW

## IPOPI NMO Support Programme 2017

Throughout 2017 IPOPI was pleased to be able to fund several NMO programmes with its NMO Supporting Programme.

- BOPPI PID Spinning Marathon 2017 in Belgium
- Second PID Meeting for Physicians in Bolivia
- Irish PID Family Meeting
- Family Day 2017 in Malaysia
- Polish video guide for PID patients
- Regional representatives' establishment and training in Ukraine



BOPPI PID Spinning Marathon, 2017



## STRATEGIC OBJECTIVE 3 – To raise PID awareness globally

IPOPI uses communication tools strategically to spread awareness of PID and promotes the collection of key PID data.

### New [www.IPOPI.org](http://www.IPOPI.org)

2017 marked IPOPI's 25<sup>th</sup> Anniversary and the highlight of the celebration was the launch of a new IPOPI.org website. The new website boasts a responsive design, is easy to navigate on mobile devices and user friendly. It includes all IPOPI news, publications (leaflets, ebooks, recommendations, papers, etc), events, campaigns and, more importantly, the IPOPI PID Map. With this new feature, you can find information on PID diagnosis, treatment and patient support from all over the world.

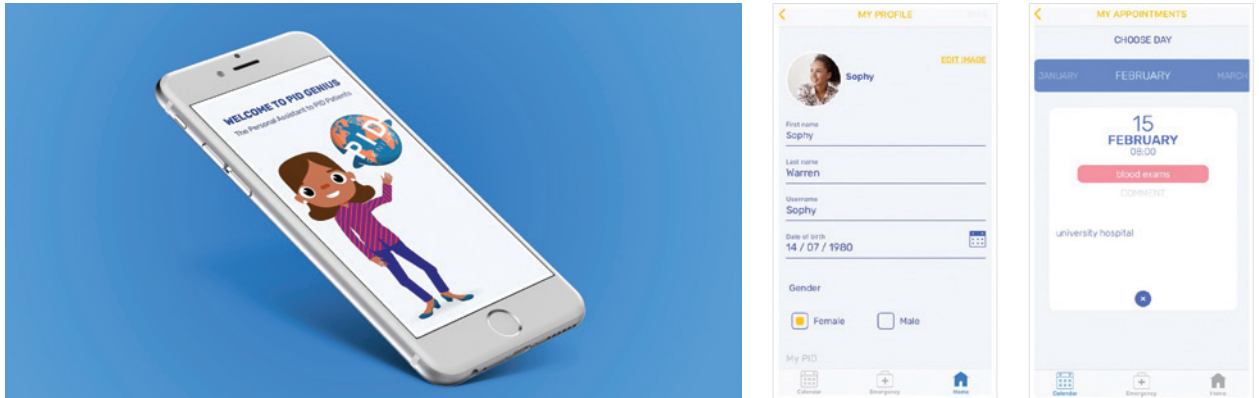


The screenshot shows the IPOPI website homepage. At the top left is the IPOPI logo, which features a globe and the text "IPOPI INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES". To the right of the logo is the tagline "IMPROVING PID PATIENTS' LIVES. WORLDWIDE." and a search bar labeled "Search IPOPI website". Below the tagline is a navigation menu with links: Home, About IPOPI, Our Work, PIDs, NMOs, Publications, IPOPI PID Map, Media, e-News, and IPOPI TV. The main content area features a large banner image of a young child's face. Overlaid on the banner is the text "NEWS" in a small box, followed by "NEW SERIES OF IPOPI PID INFORMATION LEAFLETS PUBLISHED" in large, bold, white capital letters. Below the banner are two smaller news items. The first item on the left shows a photo of Jose Drabwell and a woman, with the text "NEWS" in a small box, "Jose Drabwell Appointed as Officer in the Order of Orange-Nassau", and a sub-headline "Mrs Jose Drabwell IPOPI's President was appointed today as Officer of the Order of Orange-Nassau in the Netherlands, her country of birth." The second item on the right shows a photo of a staircase, with the text "NEWS" in a small box, "IPIC2017 – Congress report", and a sub-headline "FOCUS ON DIAGNOSIS AND CLINICAL CARE" with the website "WWW.IPIC2017.COM" and dates "8-10 NOVEMBER 2017 // DUBAI".

## PID Genius companion app

IPOPI launched PID GENIUS, the first **mobile app** developed by a patient organisation for people with primary immunodeficiencies (PIDs) for worldwide use, in November 2017. The app is available for download at the Apple App Store and on Google Play.

PID GENIUS is the personal assistant to PID patients in search of an easy and dynamic way of keeping track of their treatments, day-to-day symptoms, vaccinations, contacts and most important documents. PID GENIUS also offers the possibility of displaying stored information in dashboards, facilitating discussions between patients and their medical specialists.



## New series of IPOPI PID information leaflets

IPOPI has since 2011 been producing primary immunodeficiencies (PID) information and a new series was published in 2017. The series comprises 3 leaflets dedicated to 'PIDs and infections', 'PIDs and allergies' and 'PIDs and psychological management', all with very helpful information and guidance for patients, their families and extended community.





## IPOPI e-News

IPOPI e-News is a quarterly electronic publication and remains the most important IPOPI publication. It has a dedicated website, an updated look and is mobile responsive making it very easy to read on-the-go! To subscribe to e-News please visit [e-news.ipopi.org](http://e-news.ipopi.org)

IPOPI e-News issues have four different article categories. They comprise an editorial signed by the IPOPI Chairperson, a section with the IPOPI news featuring our most relevant events and campaigns, dedicated part for the NMOs' news featuring their national achievements and an NMO Focus article, an in-depth piece about one of our member organisations.



### STRATEGIC OBJECTIVE 4 – To stimulate stakeholder collaboration

IPOPI has always valued the benefits of working together with its stakeholders and 2017 has seen many partnerships reinforced and new ones being forged.

## European Reference Network for PIDs

IPOPI is currently collaborating with the European Reference Network RITA.

RITA stands for Rare Immunodeficiency, Autoinflammatory and Autoimmune Diseases and unites specialised centres in Europe dedicated to Primary Immunodeficiencies and other autoinflammatory and autoimmune disorders.

This collaboration between healthcare specialists responds to a European initiative launched by the European Commission to create European Reference Networks that can support patients living with rare disorders. As one of the first partners involved, IPOPI is committed to giving voice to the needs of patients with PIDs.



ERN RITA members at work

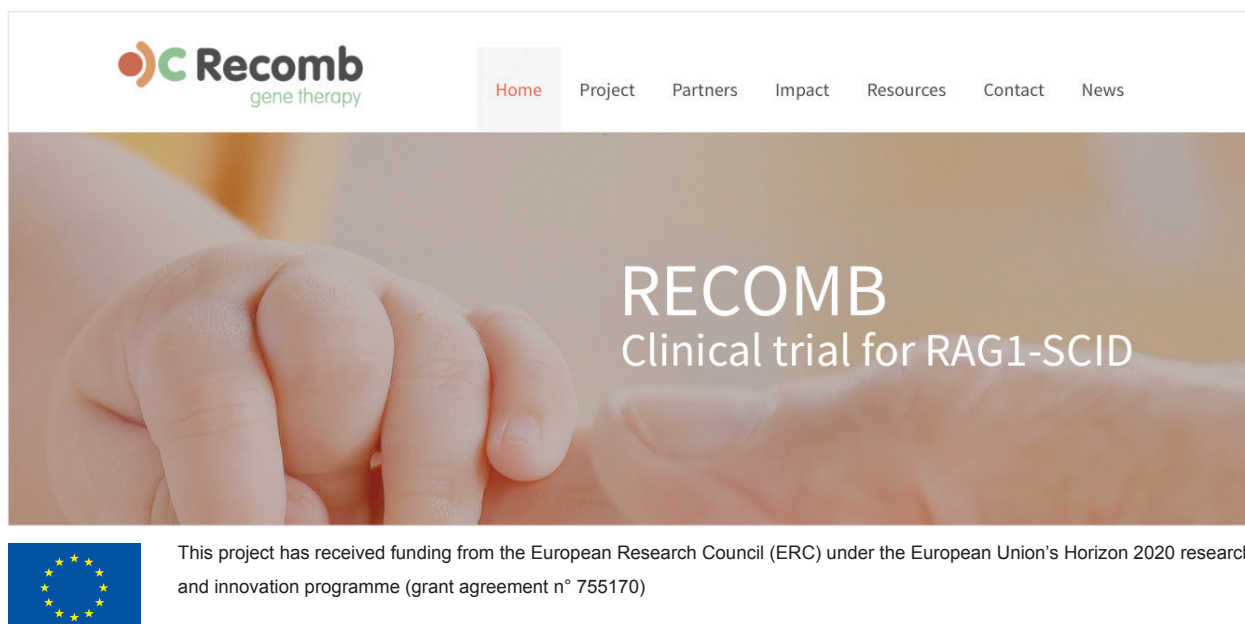
## RECOMB & SCIDNET projects

RECOMB is led by Prof Frank Staal, from Leiden University Medical Center, and aims at developing a gene therapy for children affected by a specific type of severe combined immunodeficiency (SCID): Recombinase Activating Genes (RAG) deficient SCID.

The project has been recently approved by the European Commission and is being funded through the EU Horizon 2020 research budget to develop this groundbreaking therapy.

IPOPI is actively contributing to the project, as well as to the SCIDNET consortium, another EU Horizon 2020 programme focusing on gene therapy for SCID-ADA, by providing the patient perspective on key aspects of the project and contributing to communications' activities with a wide range of stakeholders.

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



## PLUS, the Platform of Plasma Protein Users

IPOPI participated in the PLUS Consensus Conference in January 2017 in Estoril (Portugal). PLUS, the Platform of Plasma Protein Users, organised a meeting bringing together organisations representing people living with treatable rare plasma related disorders, plasma industry associations, as well as blood donors' organisations and blood establishments. Participants discussed and explored common grounds for understanding in view of the future revision of European blood legislation.

As a result of the Consensus Conference, a joint letter was sent to the European Commission blood legislation unit, with the objective of providing some shared concerns on the European blood legislation. The letter was signed by the participating patient associations, the Plasma Proteins Therapeutics Agency (PPTA), the International Plasma Fractionation Association (IPFA) and the European Plasma Alliance (EPA).





## International Plasma Protein Congress

IPOPI, together with 300 representatives from patient groups, policy makers, regulators, physicians, scientists and industry leaders, participated in the IPPC congress held in March in Prague, Czech Republic. The meeting focused on solutions to improve access to care and pressed the importance of cooperation between all plasma stakeholders.

**Mrs Martine Pergent, IPOPI's Vice-President** and representative of the French PID national PID patient association (IRIS) provided an interesting presentation on the plasma and blood policies and challenges in France during the session on 'How to ensure better national access to plasma protein therapies'.



Mrs Pergent on how to ensure better national access to plasma protein therapies

## IPFA – International Plasma Fractionation Agency

IPFA's 2<sup>nd</sup> Asia Workshop on Plasma Quality and Supply 2017 took place in Yogyakarta, Indonesia, March 2-3, 2017 and covered very important topics for policy makers, pharmaceutical industry representatives and patients from several countries in the Asia Pacific region.

Representatives from the Ministry of Health, Malaysia & Department of Health and from the National Blood Bank were among the attendees. One of the key conclusions of the meeting was that there is still work to be done before South-East Asia can fractionate plasma, both from the infrastructure and the business environment sides.

**Mr Bruce Lim, IPOPI Board member** and President of the national primary immunodeficiency (PID) patient group, MyPOPI, attended the meeting, increasing the PID community's visibility in the discussions.

## EMA

Patients, as the end-users of medicines, are considered as key stakeholders by EMA.

IPOPI President **Mrs Jose Drabwell** participates in the European Medicines Agency (EMA) meeting as a member of the Patients' and Consumers' Organisations Working Party (PCWP). Mrs Drabwell continues to play a very important role helping to voice PID patients' perspectives in pharmacovigilance, patient involvement in clinical trials, data transparency, among others. Ms Leire Solis, Health Policy and Advocacy Manager supports Mrs Drabwell in this active role.



## IPOPI participation in other stakeholders meetings

IPOPI also attended and participated in the following events among others:

- EMA PCWP meetings, London, all year
- European Parliament MEP liaison Programme, all year
- 2017Health First Europe (HFE) Meetings, Brussels, all year
- European Haemophilia Round Table Meetings (selected events), Brussels, 2017
- 3<sup>rd</sup> Immunology, Asthma and Allergy Research Institute (IAARI) February, vaccinations add in Tehran
- ERN Kick Off General Meeting, Vilnius, March 2017
- Rare Diseases Day Events, Brussels, March 2017
- ISPID meeting, Bangalore, India, March 2017
- Eurordis AGM and Membership Meeting, Budapest, May 2017
- World Health Organisation (WHO)'s World Health Assembly meeting, May 2017
- "Safeguarding Blood Safety for multi-transfused patients", organised by the Thalassaemia International Federation during the World Health Organisation (WHO)
- General Assembly, Geneva, May 2017
- Eurordis Membership Meeting, Budapest, May 2017
- Eurordis General Assembly, Budapest, May 2017
- EU Commission Meeting on Blood Legislation Evaluation, Brussels, June 2017
- Eurordis DITA Task Force Meetings, Paris, June 2017
- RITA ERN Board and Council Meeting, Amsterdam, November 2017



## Looking Ahead – 2018 and beyond

IPOPI looks forward to continue to support the PIDs community through international awareness and advocacy work. New and continuing programmes such as IPOPI's SCID National Parliamentary Events, national advocacy campaigns and the EU PID Forums 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. Aware that diagnosis remains a key baseline issue in the developing world countries, IPOPI is looking at launching a specific programme designed to tackle this specifically.

The fourth International Primary Immunodeficiencies Congress - IPIC2019 – will be held in Madrid, Spain at the Marriot Auditorium Hotel! See you there!



## Financial Report 2017

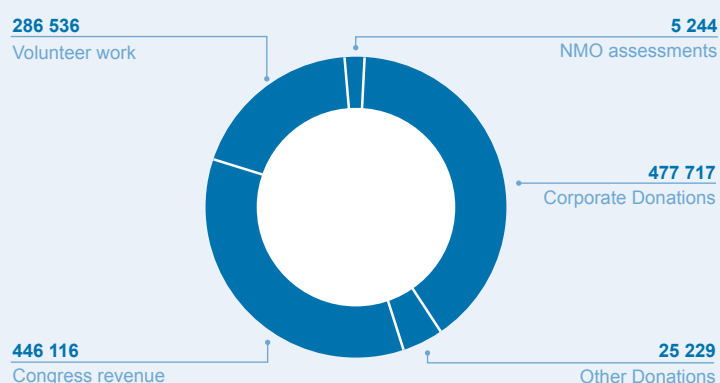
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2017 was a very good financial year for IPOPI with the highest annual income to date and a very successful 3<sup>rd</sup> International Primary Immunodeficiencies Congress - IPIC2017 - in Dubai. The congress financial gains enabled IPOPI to organise patient awareness programmes and are being used as well to fund the IPOPI XVth Global Patient Meeting taking place in October 2018, in Lisbon, Portugal. IPOPI continues to ensure a reserve policy is in place as well in line with the UK charity commission recommendations.

IPOPI is a non-profit charity registered in the United Kingdom under UK Charity Law. IPOPI is 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 2017. 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.

## Income 2017 (GBP)



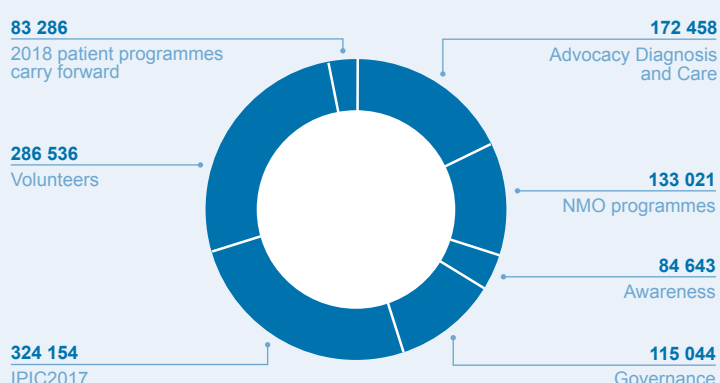
NMO assessments	5 244
Corporate donations	477 717
Other donations	25 229
Congress revenue	446 116
Volunteer work	286 536
<b>TOTAL</b>	<b>1 240 842</b>

## Sources of funding 2017 (%)

Membership fees	0,50%
Industry funding	38,50%
Other	2,00%
Congress	36,00%
Volunteers	23,00%
<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 **19.76%** of the total income.

## Resources expended 2017 (GBP)



## Expenditure 2017 (GBP)

Advocacy Diagnosis and Care	172 458
NMO programmes	133 021
Awareness	84 643
Governance	115 044
IPIC2017	324 154
Volunteers	286 536
2018 patient programmes carry forward	83 286
<b>TOTAL EXPENDITURE</b>	<b>1 199 142</b>



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.

IPOPI also expresses its 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



### SILVER



### BENEFACTOR



## PROJECT SPONSORS

In 2017, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities

- Baxalta/Shire
- Binding Site
- Biotest
- Cane SpA
- CSL Behring
- Genewerk
- Grifols
- GSK
- Kedrion
- LFB
- NBI
- Orchard
- Perkin Elmer
- Octapharma
- RMS Medical Products
- UCB

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

Other non-corporate partners who made a financial contribution included Genethon and the Dubai Convention Bureau. IPOPI would also like to thank Emirates airlines for their support towards IPIC2017 in Dubai.

## 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 2017 including but not limited to: Australasian Society of Clinical Immunology and Allergy (ASCIA), African Society for Immunodeficiencies (ASID), Arab Society for Primary Immunodeficiencies (ARAPid), Asociación Española de Pediatría (AEP), Asia Pacific Society for Immunodeficiency (APSID), British Society for Immunology (BSI), Care-for-Rare Foundation, Chulalongkorn University Hospital Bangkok, Clinical Immunology Society (CIS), Cipto Mangunkusumo Hospital Jakarta, European Federation of Immunology Societies (EFIS), Federation of African Immunology Societies (FAIS), Federation of Clinical Immunology Societies (FOCIS), European Medicines Agency (EMA), the European Haemophilia Consortium (EHC), European Platform for Patient Organisations, Science and Industry (EPPOSI), Eurordis, European Society for Immunodeficiencies (ESID), Faculty of Medicine Universitas Indonesia (FMUI), 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 Déficits 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, Sociedad Española de Inmología (SEI), SEAPID (South East Asia Primary Immunodeficiencies Network), Sociedade Portuguesa Alergologia Imunologia Clínica, Sociedade Portuguesa de Pediatria- infeccologia pediática, Thai Immunology Society (AAIAT), Vietnamese Paediatrics Association, 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](http://www.ipopi.org)





**INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES**  
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