



INTERNATIONAL  
PATIENT ORGANISATION  
FOR PRIMARY IMMUNODEFICIENCIES

REVIEW  
OF **ACTIVITIES**  
**AND ACHIEVEMENTS**  
**2016**







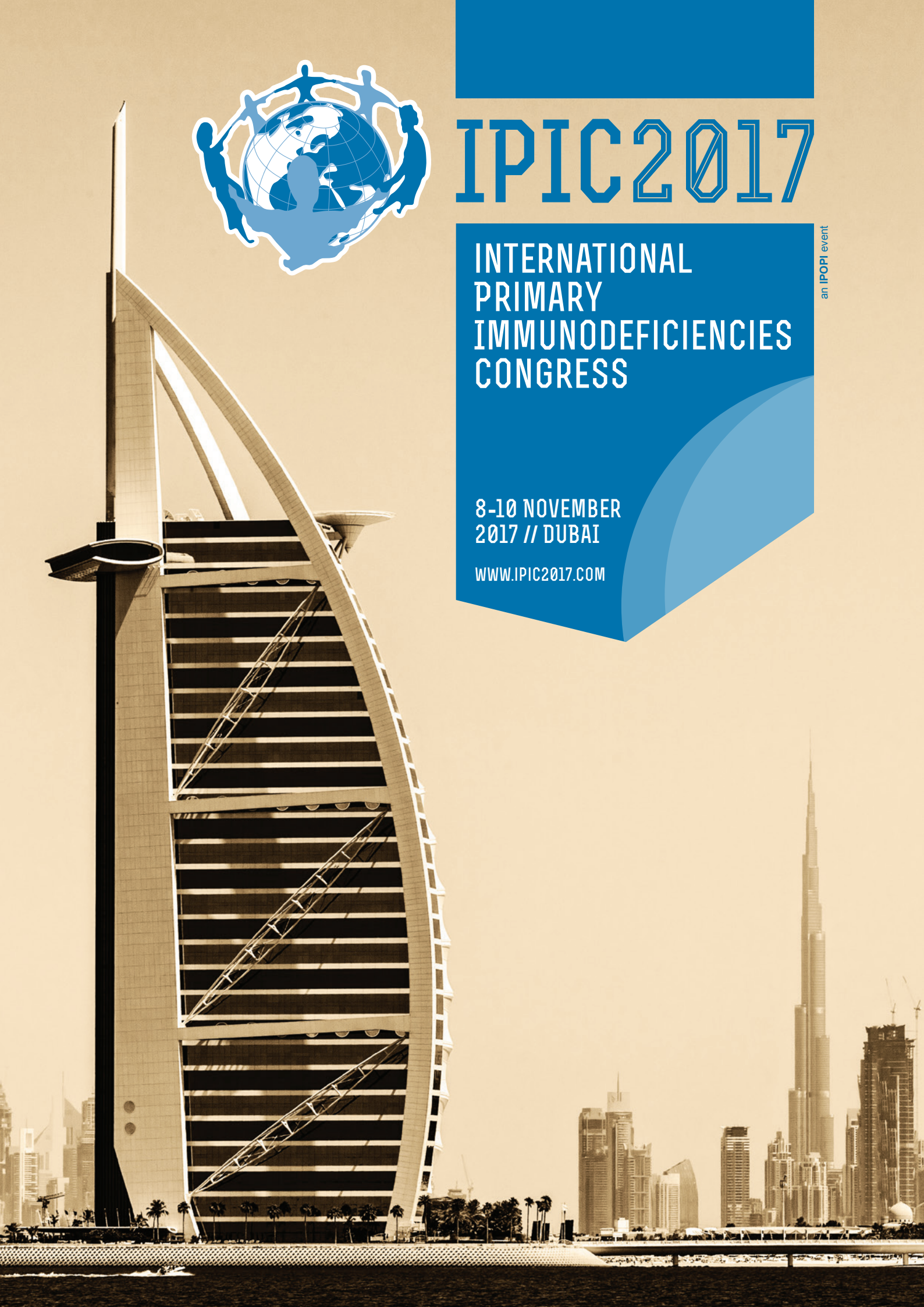
# IPIC2017

INTERNATIONAL  
PRIMARY  
IMMUNODEFICIENCIES  
CONGRESS

an IPOPI event

8-10 NOVEMBER  
2017 // DUBAI

[WWW.IPIC2017.COM](http://WWW.IPIC2017.COM)

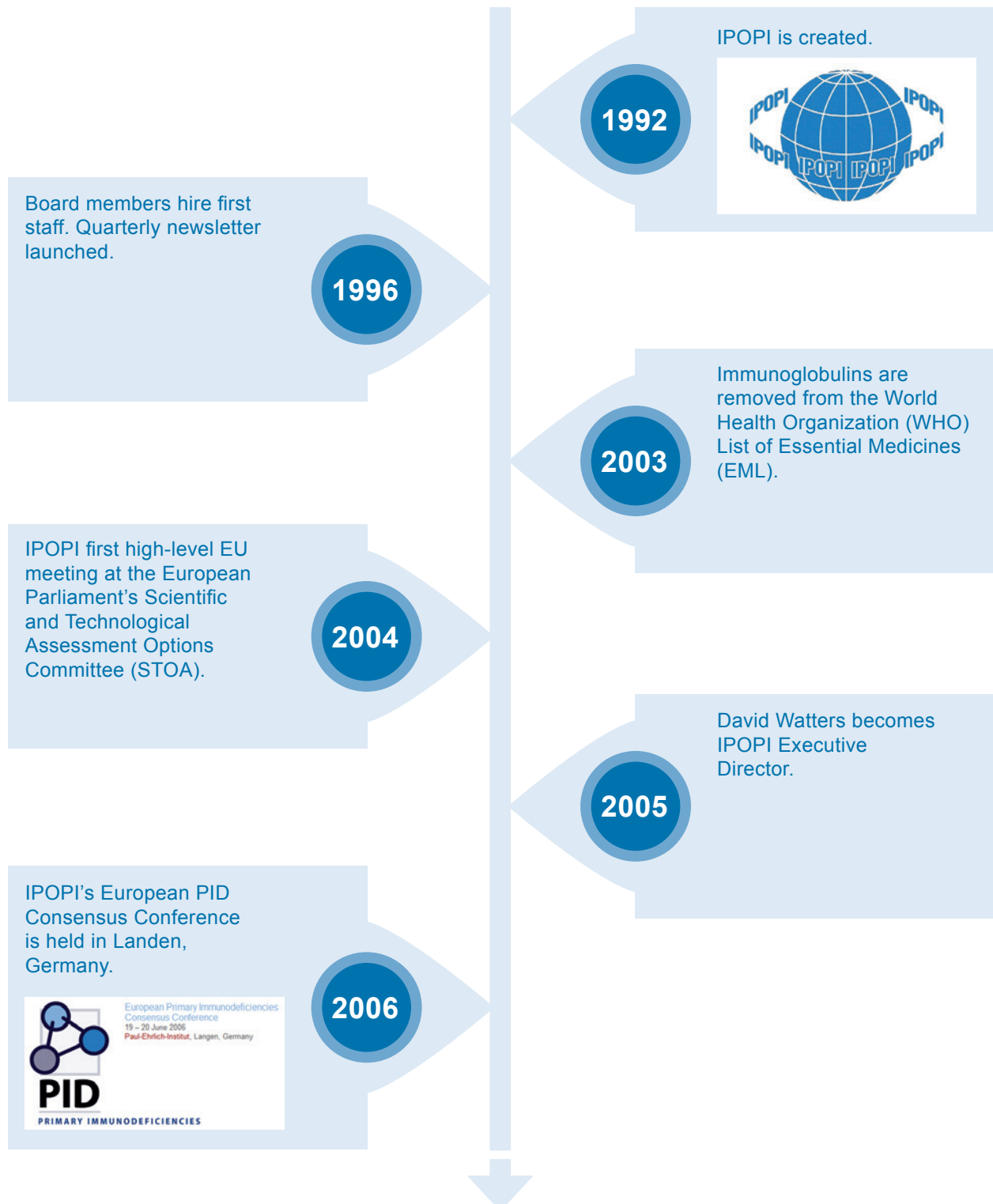


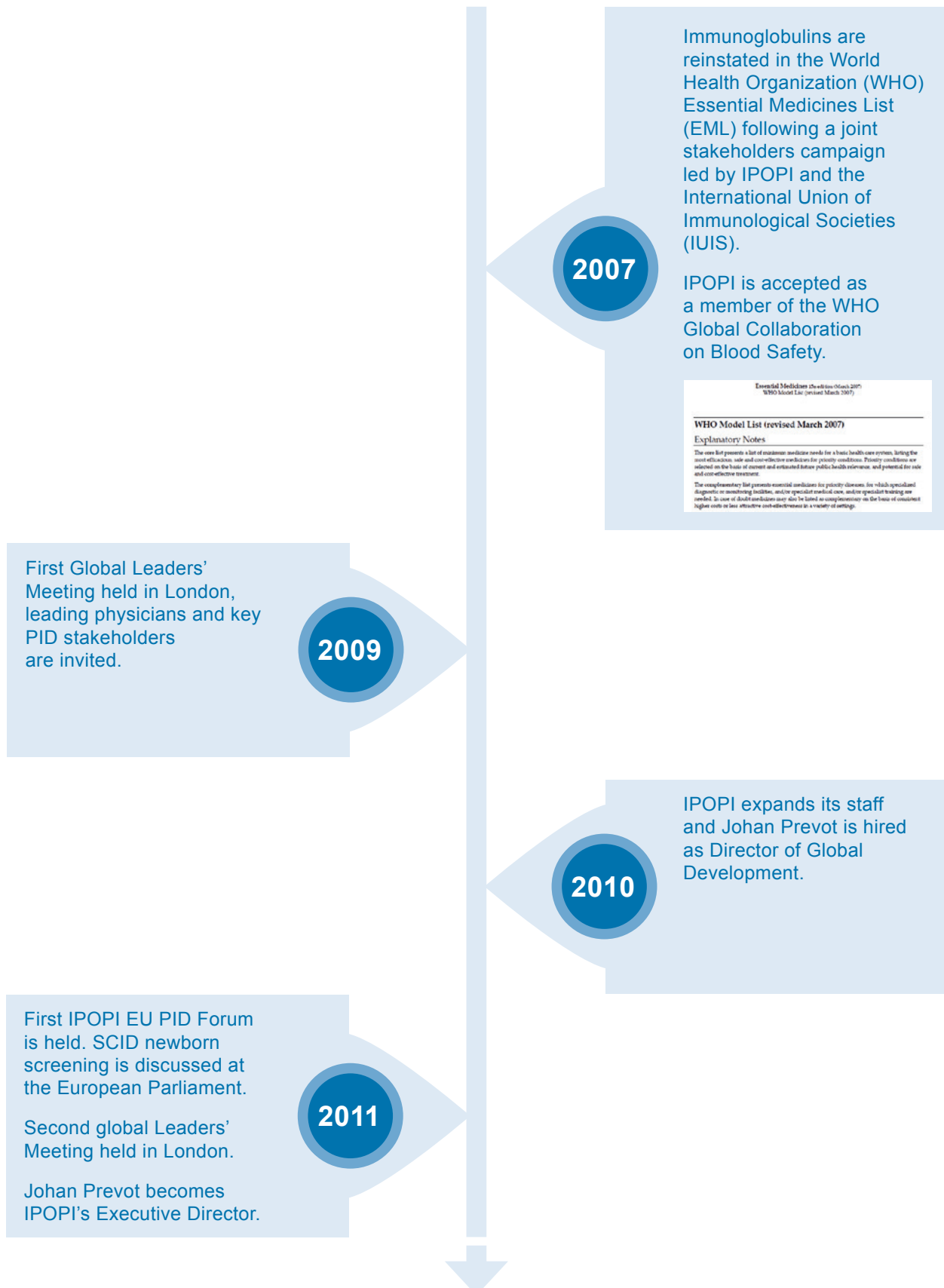
# IPOPI 25<sup>TH</sup> ANNIVERSARY

Celebrating our achievements and preparing the steps ahead.

## TIMELINE

1992 / 2006





IPOPI's Asian development plan is created thanks to a generous grant from IPOPI's Honorary President Bob LeBien.



2012

Publication of the Principles of Care for Primary Immunodeficiencies paper on Frontiers of Immunology, led by IPOPI.



2014

First International Primary Immunodeficiencies Congress - IPIC2013 - is held in Estoril, Portugal, meeting a need for a global PID clinical care congress.



2013

IPOPI reaches 60 National Member Organisations.



2016

## IN THE PIPELINE:

- . IPIC2017 in Dubai
- . IPOPI XV Biennial Meeting in Lisbon
- . Increased PID awareness in remote regions of the world
- . Launch of validated Burden of Treatment PID Questionnaire



## A MESSAGE FROM THE CHAIRPERSON



Jose Drabwell, IPOPI Chairperson

Dear Friends and Colleagues,

At the time of writing these lines, IPOPI is celebrating its 25th Anniversary and it has been a remarkable privilege to witness and be part of the growth of IPOPI. On the occasion of this major milestone, IPOPI is grateful to many people and many organisations who have been part of IPOPI's success story. In particular, we would like to thank Bob LeBien who had the vision and global thinking to create IPOPI which represents this fantastic community that is the extended primary immunodeficiencies family. Since its inception, IPOPI grew tremendously. IPOPI represents 61 patient groups from across the world and keeps growing. A lot has been accomplished but there is still a lot to be done, not only for the patients but with their involvement.

In 2016 IPOPI achieved so many milestones that it is hard to believe it all happened in one year! IPOPI has been extremely busy in all regions of the world, greatly contributing to increased awareness of primary immunodeficiencies globally.

With a new Board of Directors and a larger staff IPOPI is more than ever committed to building an improved global framework of care for our patients' community.

IPOPI was delighted to work hand in hand with ESID (European Society for Immunodeficiencies) and INGID (International Nursing Group for Immunodeficiencies) at the occasion of our Biennial Meeting, held in the beautiful city of Barcelona, Spain. IPOPI's XIVth Biennial Meeting was a great success with 100 patient organisation representatives attending and sharing experiences.

The meeting skills building programme, designed to cater to our National Member Organisations' (NMOs) needs, included workshops on fundraising strategies, best practices for awareness raising campaigns, and medical lectures tailored for patients.

IPOPI XIVth Biennial Meeting was truly inspiring and the PID Champions Awards Ceremony made it even more so. IPOPI and its NMOs awarded Dr Teresa Espanol (Spain) as the winner of the LeBien Award and Mr Geoffrey Yu (Hong Kong) winner of the Luciano Vassalli Award. You can read about it in on page 12.

2016 was a turning point year for PID awareness in Asia, greatly because of IPOPI's Bob LeBien Asian Development Plan and continuous awareness efforts. IPOPI organised the two first-ever National PID Launch Meetings, in Taiwan and in the Phillipines, bringing together doctors and patients. These featured clinical workshops catering for the local doctors as well as patients' meetings to foster the launch of new patient groups in the region.

On the advocacy front, IPOPI held its 7th and 8th IPOPI EU PID Forums at the European Parliament in Brussels. The 7th Forum addressed the need to tackle care disparities for PID patients in Europe and the 8th PID Forum addressed the impact of Brexit on Rare Diseases patients, with a special focus on the experience of the PID community. As a result, a set of *Recommendations on PID care following Brexit for UK and EU policy makers* was launched and has become a reference document in discussions around Brexit and rare disorders care.

In addition, IPOPI continued its SCID newborn screening campaign with much progress in Spain. Together with the National PID patient group and the leading PID medical experts, and after several meetings, two of the main political parties offered their support to the inclusion of SCID in the national newborn screening programme and Catalunya announced it will start screening for SCID in 2017. Read more about it on page 11.

Of course IPOPI has also been working hard in the preparation of the 3rd International Primary Immunodeficiencies Congress - IPIC2017 - which will be held in the vibrant city of Dubai on 8-10 November 2017. The programme is quite exciting featuring world-renowned PID specialists. Among them are Prof Helen Chapel (UK), Prof Alain Fischer (France), Prof Bobby Gaspar (UK), Prof Isabelle Meyts (Belgium), Prof Steve Holland (US), Dr Despina Moshous (France), Prof Andrew Cant (UK), Prof J. L. Casanova (France), Prof Saleh Al Muhsen and ethicist James Taylor.

I hope you will enjoy reading this annual report and I look forward to seeing you all very soon in Dubai!

**Yours sincerely,  
Jose Drabwell**

A handwritten signature of Jose Drabwell in white ink, written in a cursive style.

# EXECUTIVE COMMITTEE, OFFICERS AND STAFF

## EXECUTIVE COMMITTEE

**Jose Drabwell**, Chair (United Kingdom)  
**Martine Pergent**, Vice Chair (France)  
**Christine Jeffery**, Treasurer (Australia)  
**Andrea Gressani**, (Italy)  
**Dragana Koruga**, (Serbia)  
**Violetta Kozhereva**, (Russia)  
**Bruce Lim**, (Malaysia)  
**Roberta Pena**, (Argentina)  
**Annie Pienaar**, (South Africa)  
**John Seymour**, (US)

## STAFF

**Johan Prevot**, Executive Director  
**Leire Solis**, Health Policy and Advocacy Manager  
**Magda Lourenço**, Communications Project Manager  
**Saara Kiema**, NMO Programmes Officer  
**Carla Morgado**, Executive Assistant  
**Carol Tavener**, Bookkeeping & Administration  
**Clare Glynn**, Financial Consultant

## MEDICAL ADVISORY PANEL

### CHAIRMAN

**Prof. Bobby Gaspar**, (United Kingdom)

### VICE CHAIRMAN

**Dr. Nizar Mahlaoui**, (France)

### PANEL MEMBERS

**Dr. Adli Ali**, (Malaysia)

**Dr. Tadej Avcin**, (Slovenia)

**Prof. Aziz Bousfiha**, (Morocco)

**Prof. Charlotte Cunningham-Rundles**, (US)

**Dr. Monika Esser**, (South Africa)

**Dr. Jose Franco**, (Colombia)

**Prof. Isabelle Meyts**, (Belgium)

**Dr. Olaf Neth**, (Spain/Germany)

**Dr. António Condino Neto**, (Brazil)

**Prof. Surjit Singh**, (India)

**Dr. Joanne Smart**, (Australia)

**Prof. Martin VanHagen**, (The Netherlands)

**Prof. Klaus Warnatz**, (Germany)

### GENERAL PRACTITIONER ADVISER

**Dr. Lotte Denning**



Prof Bobby Gaspar, IPOPI Medical Advisor President



Dr Nizar Mahlaoui, IPOPI Medical Advisor Vice-President



**Back to front:** Johan Prevot (IPOPI Executive Director) and Board Members Roberta Pena (Argentina), Christine Jeffery, IPOPI Treasurer (Australia), Dragana Koruga (Serbia), Andrea Gressani (Italy), Martine Pergent, IPOPI Vice Chair (France), John Seymour (US), Jose Drabwell, IPOPI Chair (UK), Violetta Kozhereva (Russia) and Bruce Lim (Malaysia). Annie Pienaar (South Africa) is also a Board Member but was not present at the time of the picture.

# IPOPI STRATEGIC PLAN

## MISSION STATEMENT

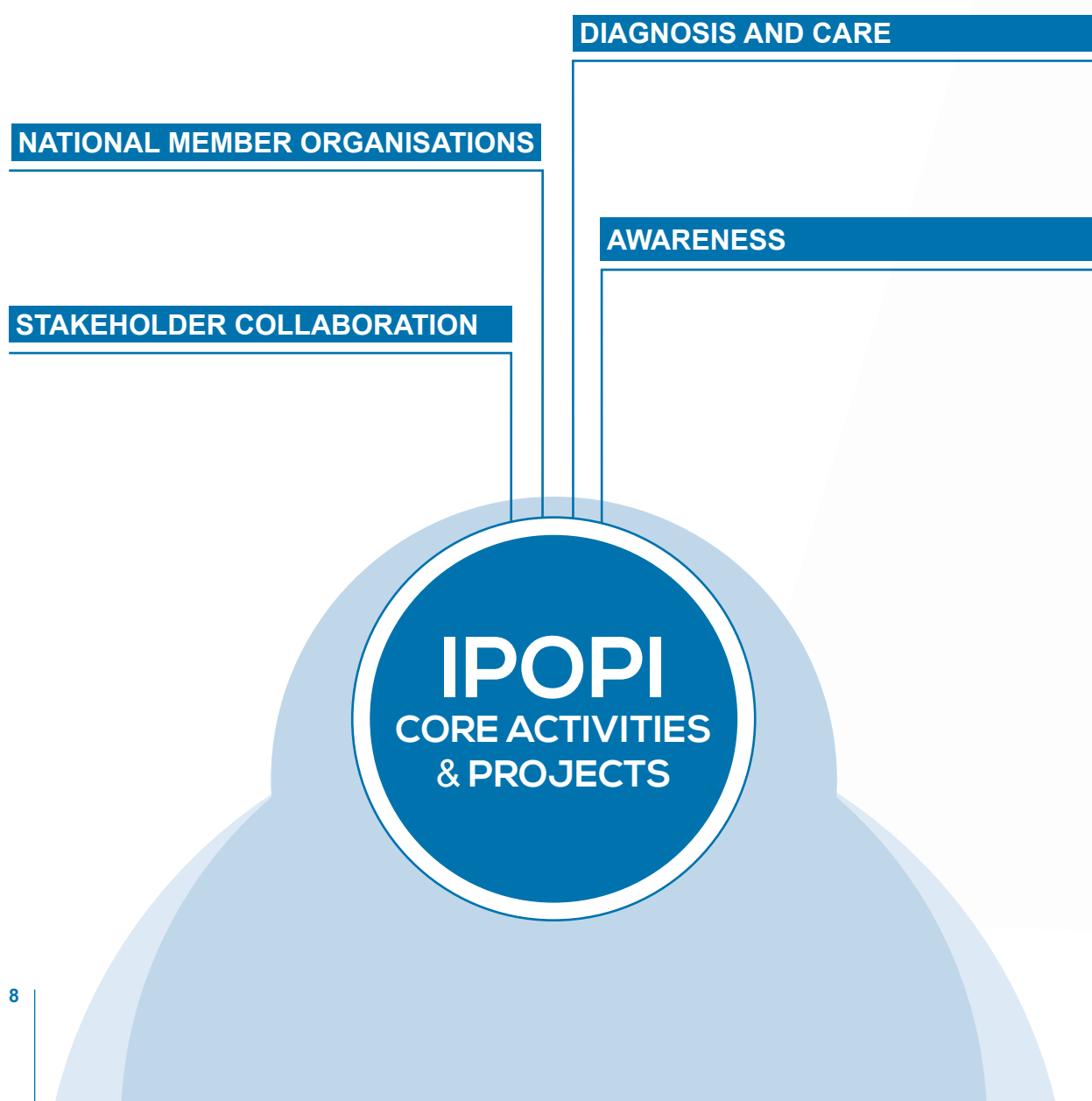
The Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide through global collaboration.

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

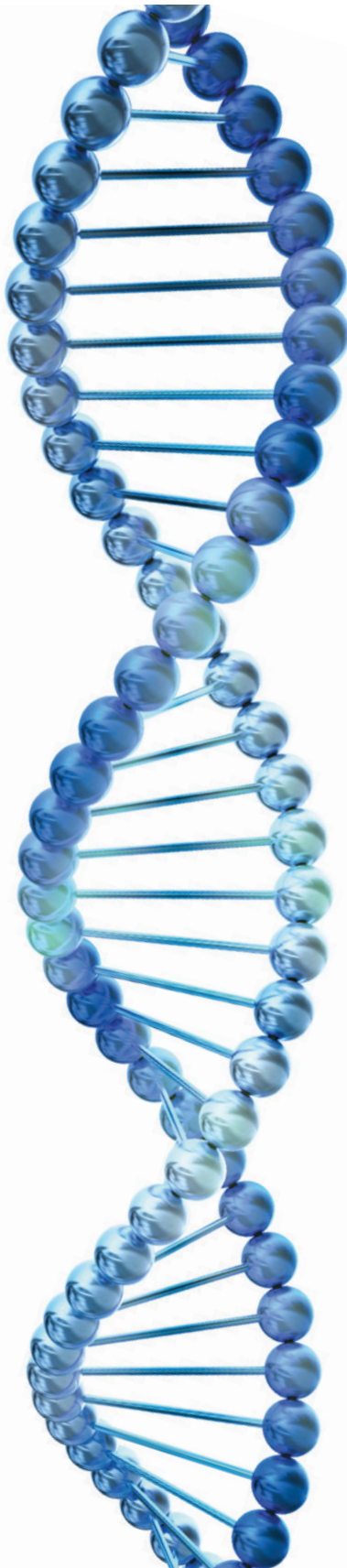
IPOPI's various projects and programmes are geared towards the 4 following strategic objectives:

- 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 300 different disorders caused when some components of the immune system (mainly cells and proteins) do not work properly. Whilst PIDs are generally recognised as rare disorders, some are more common than others and taken as a whole they represent an important group of people whose lives are profoundly impacted by their condition.

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

## Diagnosis

Whilst it is estimated that around 60% of PIDs can be easily diagnosed 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 carers. 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 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, 2016

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

IPOPI strives to ensure that the patient interest drives health policy decision through advocacy at national, regional and international level.

Our actions promote the importance of access, choice and supply of treatment for patients.

#### IPOPI European Parliament PID Forums

IPOPI organised two IPOPI EU PID Forums in 2016 with the support of several Members of the European Parliament (MEPs) and the participation of patient representatives, policy makers and industry representatives. The 7th IPOPI PID Forum on 'PID care disparities in the European Union' was held in October and the 8th IPOPI PID Forum on the effects of Brexit on Rare Diseases Patients had to be postponed to 2017 and took place in January 2017.

The **7th IPOPI EU PID Forum** on 'PID care disparities in the European Union' was chaired by MEP Dr Anna Záborská (Slovakia), Dr Elena Gentile (Italy) and Ms Karin Kadenbach (Austria). The Forum overviewed PID care across the EU and reinforced the need to commonly implement the **Primary Immunodeficiencies Principles of Care** across all countries.

Participants discussed the current challenges faced by new and old EU Member States from both the patients' and physicians' perspective. The meeting placed an emphasis on the importance of early and accurate diagnosis, the possibility of choosing between different treatment options, and the screening programmes and projects for Severe Combined Immunodeficiencies (SCID) taking place in several European countries.



7th IPOPI EU PID Forum – Supporting MEPs, IPOPI representatives and speakers

The **8<sup>th</sup> IPOPI EU PID Forum** was chaired by MEPs Mr Carlos Zorrinho (Portugal) and Mr Seb Dance (UK) and officially supported by MEPs Ms Linda McAvan, Ms Catherine Bearder, Ms Mairead McGuinness, Ms Marian Harkin and Ms Glenis Willmott.

Under the motto 'Don't Brexit on Rare Diseases Patients – the case of primary immunodeficiencies' participants analysed the impact of Brexit on Rare Diseases and PID patients with the aim to protect them in the Brexit process.

Brexit might have a negative impact on European patients and on scientific research. Rare diseases patients have had the possibility to be referred for treatment in the member state with the best treatment for their needs through the European Directive on Patients' Rights in Cross Border Healthcare. On the research front, British healthcare centres have been among the leading forces for scientific research, new treatments and multicentre clinical trials for PIDs.

The meeting resulted in the launch of a set of 'Recommendations on PID care following Brexit for UK and EU policy makers'. The paper calls upon the UK government and EU negotiators to ensure that even though the negotiations prioritise issues of trade, the health of all European citizens and rare diseases patients such as PIDs, is on the agenda of negotiations and a positive collaborative future is sought. The paper has been used on numerous occasions since the meeting by policy makers at EU level.



8<sup>th</sup> IPOPI EU PID Forum – Participants during MEP Mr Seb Dance speech.

#### **IPOPI SCID Newborn Screening Campaign – national campaign in Spain**

IPOPI joined efforts with AEDIP, the Spanish Association for Primary Immunodeficiencies, on a campaign to implement newborn screening (NBS) for Severe Combined Immunodeficiencies (SCID) nationally.

The Spanish SCID NBS campaign was kick started in March 2016 benefiting from an already existing pilot project showing the feasibility of SCID newborn screening in Spain. Despite 2016 was a very atypical year in Spanish politics with the political agenda centred around the creation of a new government following the December 2015 elections, very good progress was made.

IPOPI and AEDIP involved key medical experts in the country in creating a consensus document on SCID newborn screening which got the support of the main scientific societies in Spain.

Several meetings with party leaders and health authorities took place throughout 2016 and, early in 2017, the campaign got the support of two of the main political parties of the country and non-legislative proposals were tabled, forging the way for SCID NBS implementation.

#### **Medical Advisory Panel**

IPOPI's Medical Advisory Panel 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 and Zika Virus occurrence.

IPOPI is grateful for all the Panel members' commitment. Special thanks to Prof Bobby Gaspar and Dr Nizar Mahlaoui (President and Vice-President) for their leadership of the MAP, and to Prof Martin Van Hagen for his outstanding efforts and support to IPOPI in Asia.



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

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

### IPOPI's XIVth Biennial Meeting

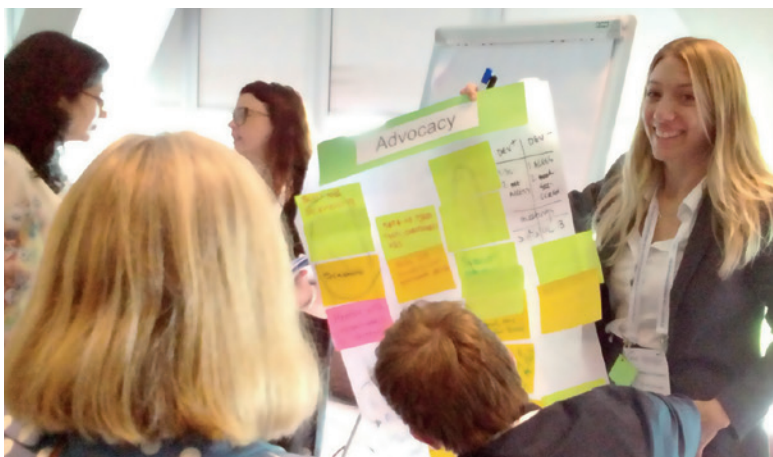
IPOPI held its XIVth Biennial meeting on 21-24 September, in Barcelona, in conjunction with the ESID and INGID Congresses, with 100 patient representatives attending.

IPOPI Biennial Meetings are unique events that provide our National Member Organisations (NMOs) with great opportunities for interaction and learning and the 2016 Biennial was no exception!

The skills building programme, tailored specifically for the needs of our NMOs, featured workshops on how to improve their strategic plans and organic development, lectures on strategies for fundraising, examples of best awareness campaigns as well as medical lectures designed for patients and updates on the immunoglobulin replacement therapy.



PID patients in action at the XIV IPOPI Biennial Meeting, Barcelona



Ideas worth spreading workshop



Pharmaceutical industry session

At the Annual General Assembly, IPOPI welcomed 6 new National Member Organisations as full members: Bolivia (FIDEP), Brazil (ANPIC), Slovakia (Association of Patients with Primary Immunodeficiency), Thailand (ThaiPOPI), Ukraine (RID) and Vietnam (VietPIPs).

Mr Andrea Gressani (Italy), Mr Bruce Lim (Malaysia), Ms Violetta Kozhereva (Russia) and Dr John Seymour (USA) were elected to the IPOPI Board of Directors and will be serving 4 year mandates.

The LeBien Award, which recognises an adult who has greatly contributed to the PID community at the international level, was presented to Dr Teresa Español and the Luciano Vassalli Award, which recognises great efforts in improving the lives of patients with PIDs achieved by a young adult, to Mr Geoffrey Yu, Vice-President of Primary Immunodeficiency League (Hong-Kong).



### IPOPI NMO launch meetings

IPOPI organised two very successful NMO Launch Meetings bringing patients and doctors together in Taipei (Taiwan) and Manila (Philippines) in October and November respectively.

Both events brought to light national challenges and prepared the ground for the launch of a national patient organisation in the country to further spread awareness of primary immunodeficiencies (PIDs) and help patients in Asia. The events' programmes featured clinical meetings and patients' workshops.

### IPOPI Launch Meeting in Taiwan

The Taiwan meeting brought together 60 patients and doctors allowing for the identification of key national challenges. Taiwanese patients can access treatment but misdiagnosis is very common due to the lack of medical awareness of PIDs. Participants agreed that there is an insufficient number of PID specialists and that pursue of immunology related studies should be fostered.



A scientific meeting for doctors and PHD students took place after the patients meeting. Prof Martin Van Hagen (Netherlands), Dr Adli Ali (Malaysia/UK), Dr Kuo-Wei Yeh and Dr Jaing Tang Her assessed the situation in South East Asia and provided informative presentations on how to suspect, diagnose and treat PIDs.

### IPOPI Launch Meeting in the Philippines

The Filipino meeting featured a two day programme split between a CME Scientific Meeting on the first day and a Patients' Workshop on the second. The event was very well attended, bringing together over 180 doctors on the first day and 80 patients and stakeholders on the second.

The guest medical speakers provided presentations on an array of topics tailored for the national doctors ranging from "How to diagnose PIDs with inexpensive tests" by Dr Teresa Español to "Novel insights into



Philippines consists of 7640 islands but there are only three PID treating hospitals.



PID: new genetic defects” by Prof Martin Van Hagen. Dr Teresa Español also highlighted some case study examples from Spanish patients and Dr Florecita R. Padua spoke about PID management in the Philippines. On the second day of the meeting participants got together to come up with a collaborative approach towards PID management.

Participants reached a unanimous agreement that there was a need to launch a national patient organisation in the country, which happened in the next few months. PhilPOPI is now a member of the IPOPI family!

## World Primary Immunodeficiencies Week (WPIW)

### WPIW IPOPI European Parliament Event

IPOPI celebrated WPIW at the European Parliament in Brussels, Belgium, on April 26 with a policy event supported by Members of the European Parliament (MEPs) Mr José Inácio Faria (Portugal), Mr Carlos Zorrinho (Portugal) and Mr Boleslaw Piecha (Romania). They joined patient representatives from 10 countries, doctors, stakeholders, industry representatives and IPOPI to commemorate the importance of raising awareness on PIDs around the world under the motto “Test.Diagnose.Treat”.

The event featured one session on international developments and another one on European policies that could support PID patients and medical specialists.

Participants signed the *WPIW Call for Action* showing their support to this great worldwide event.



WPIW “Test.Diagnose.Treat” campaign taken to the European Parliament

### Asian Regional Patients Meeting in Hong Kong

During WPIW, on April 27-28 IPOPI organised the first-ever PID Patients Asian Regional Meeting in Hong Kong. The event was a great success with 32 attendees representing patients, parents, NMO leaders, physicians and nurses from Malaysia, Singapore, Hong Kong, Laos, Indonesia, Vietnam, India, Japan, Thailand and the Philippines.



Workshop on PID Principles of Care: analysing their top priorities and tailor future actions



The two-day event started off with medical lectures with background information and new developments on PIDs and two specially designed workshops followed. The first workshop looked at the region key successes and challenges and the second focused on the PID Principles of Care, inviting attendees to analyse their top national priorities to better tailor future actions.



PID awareness magazine from Slovakia

### IPOPI NMO Support Programme 2016

Throughout 2016 IPOPI was pleased to be able to fund several NMO programmes with its NMO Supporting Programme.

- . BOPPI PID Spinning Marathon 2016 in Belgium
- . Medical PID Awareness Meeting for physicians in Bolivia
- . Nordic Meeting for PID Patient Organisations in Finland
- . PID Family Day 2016 in Malaysia
- . Printing and postage of IPOPI patient information leaflets in the UK
- . Awareness magazine in Slovakia



Medical PID Awareness Meeting for physicians in Bolivia

## STRATEGIC OBJECTIVE 3 – TO RAISE PID AWARENESS GLOBALLY

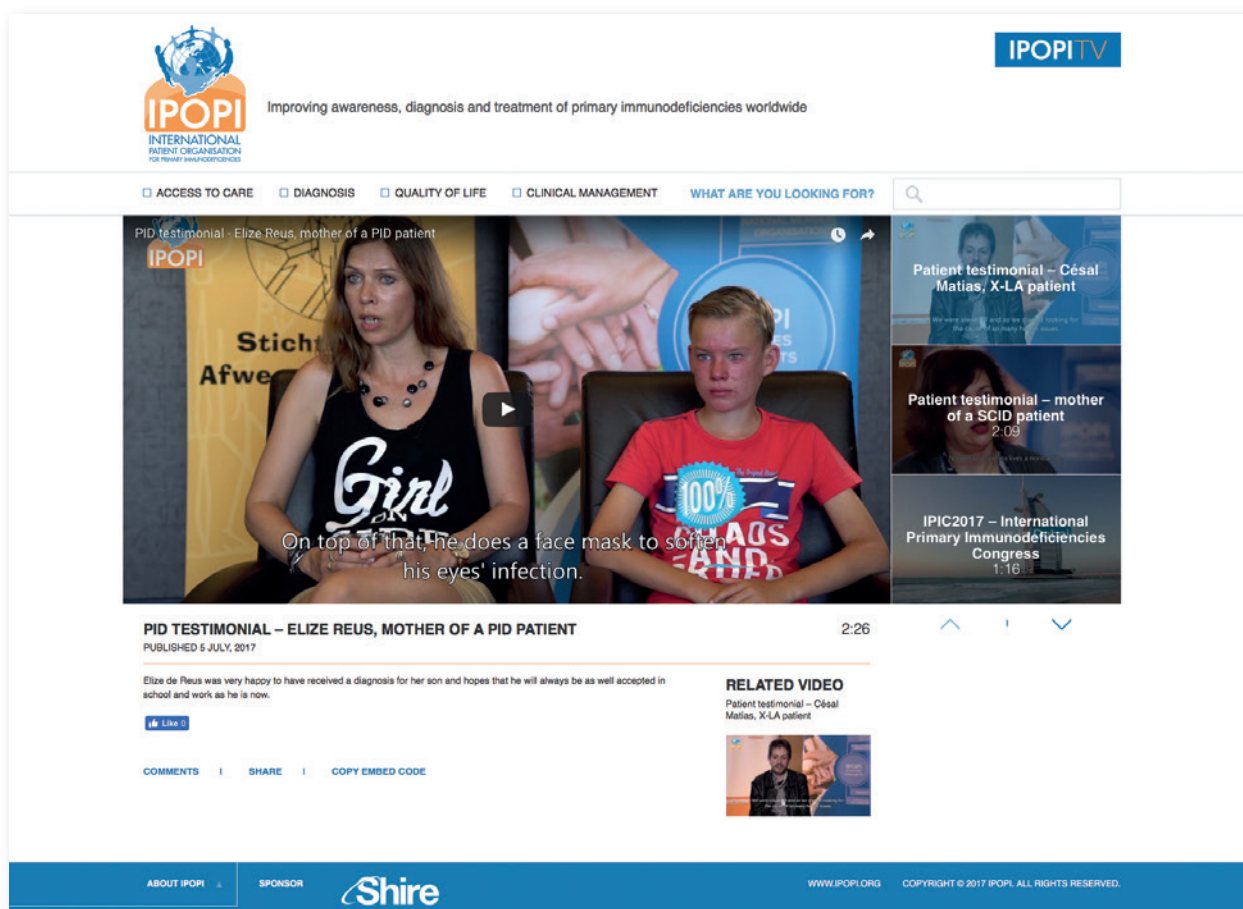
IPOPI uses communication tools strategically to spread awareness of PID and promotes the collection of key PID data.

### IPOPI TV

Video materials are increasingly important for online communication and IPOPI is at the forefront of the trend with the creation of the IPOPI TV!

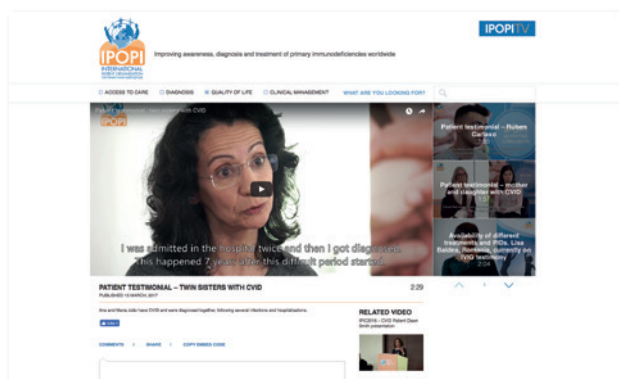
IPOPI TV features PID patient testimonials in five languages, physician interviews and clinical management lectures on primary immunodeficiencies.

The videos are divided into four categories: Access to care, Diagnosis, Quality of life and Clinical Management. The website is responsive making it as easy to navigate on a mobile device as on a personal computer. You can comment and like the videos directly on the website or embed them onto other online platforms. Please visit [tv.ipopi.org](http://tv.ipopi.org)



### New series of PID patient video testimonials

In 2016 IPOPI embarked on a second patient video testimonials' project! In 2014 the first series of videos was produced in English. For this new project IPOPI visited six different countries (Germany, Spain, Portugal, Netherlands, Poland and Sweden) to produce videos in six languages, with subtitles in English, broadening possibilities for awareness raising of primary immunodeficiencies (PIDs), access to diagnosis, treatments, burden of treatment and PID quality of life.



All videos can be watched on [tv.ipopi.org](http://tv.ipopi.org)

## Social Media

IPOPI is active on Facebook, Twitter and LinkedIn and our social media presence has grown significantly in the past year. By strategically using social media pages we have been making our actions and the PID community priorities known to thousands of people throughout the world. IPOPI does not resort to social media advertisement to boost reach and impressions, the attention our pages get is linked to the interest generated by the contents shared, the events we have organised and the good relationships developed with our partners and the objectives we are geared towards.

Check and follow our social media pages!

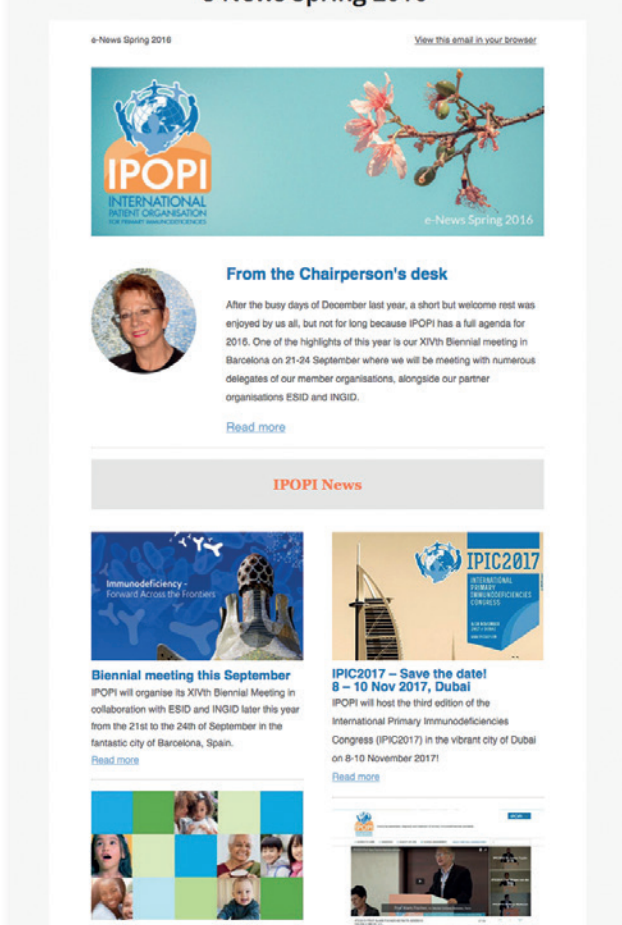
[twitter/ipopi\\_info](https://twitter.com/ipopi_info)

[facebook.com/ipopid](https://facebook.com/ipopid)

[www.linkedin.com/company-beta/2093945/](https://www.linkedin.com/company-beta/2093945/)



## e-News Spring 2016



## IPOPI e-News

IPOPI e-News is a quarterly electronic publication and remains a pivotal IPOPI publication. It boasts a responsive design, is easy to navigate on mobile devices and user friendly! To subscribe to e-News please visit [e-news.ipopi.org](https://e-news.ipopi.org)

Its design and navigation were updated in 2015 and, as a result, its popularity grew significantly in the last year.

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

## Burden of Treatment Study

IPOPI, with the University of Sheffield in the UK, launched a new PID Burden of Treatment Questionnaire, aimed at quantifying and measuring the impact of the different immunoglobulin treatments adults take to manage their PID.

The final goal of this study is to create a PID validated Burden of Treatment Questionnaire that will help PID stakeholders produce stronger and more focused data. This will be highly useful to raise awareness among policy makers and healthcare professionals about the burden of treatment for people living with PIDs. It is aimed to have the publication released by the end of 2017.



## STRATEGIC OBJECTIVE 4 – TO STIMULATE STAKEHOLDER COLLABORATION

IPOPI has always valued the benefits of working together with its stakeholders and 2016 has seen many partnerships reinforced and new ones being forged.



ERN RiTA at 3<sup>rd</sup> Conference on European Reference Networks

### European Reference Network for PIDs

IPOPI is currently collaborating with the European Reference Network (ERN) RiTA. RiTA stands for Rare Immunodeficiency, Autoinflammatory and Autoimmune 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 ERN 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. Johan Prevot is the PID patient representative on the RiTA Board.



### SCIDNET initiative

SCIDNET is an ambitious project, financially supported by the European Commission, aimed at developing genetic medicines for Severe Combined Immunodeficiency (SCID) to be licensed in the next four years.

The initiative, led by Prof Bobby Gaspar (University College of London and IPOPI's Medical Advisory Panel Chairman), benefits from the participation of numerous partners representing leading university hospitals, associations and Small and Medium Enterprises in Europe. IPOPI is participating to represent the views of patients with primary immunodeficiencies (PIDs).

More information can be found at [www.scidnet.eu](http://www.scidnet.eu)

### PLUS, the Platform of Plasma Protein Users

PLUS organised two stakeholder consensus meetings in 2016, both held in Portugal, to discuss the revision of the EU Blood Directive and the topic of Men having Sex with Men (MSM) in Blood Donations.

The meetings were attended by several patient organisations including IPOPI, the European Haemophilia Consortium (EHC), the World Federation of Haemophilia (WFH), International Patient Organization for C1 Inhibitor Deficiencies (HAEI), Alpha-1 Global, American Plasma Users Coalition (A-PLUS), and stakeholder organisations including the European Blood Alliance (EBA), International Federation of Blood Donor Organizations (IFBDO), Irish Blood Transfusion Service (IBTS), International Plasma Fractionation Association (IPFA), Plasma Protein Therapeutics Agency (PPTA) and European Plasma Collectors Committee (EPCC).

As a direct outcome of the meeting, a joint letter was submitted to the European Medicines Agency (EMA) on MSM blood donor deferral policies. The letter calls upon regulatory authorities to ensure all necessary measures are taken to avoid that diverging MSM deferral policies in different countries end up restricting access to Plasma Derived Medicinal Products (PDMPs) for patients.

**PLUS**  
PLATFORM OF PLASMA  
PROTEIN USERS



Johan Prevot, IPOPI Executive Director, speaking at the PPTA Conference

### International Plasma Protein Congress

IPOPI was among 300 representatives from patient groups, policy makers, regulators, physicians, scientists and industry leaders, participating in the IPPC congress held in March in Barcelona, Spain. The meeting focused at pointing solutions to improve access to care and helped emphasise the importance of cooperation between all plasma stakeholders.

Johan Prevot, IPOPI Executive Director was invited to speak in representation of PLUS – Platform of Plasma Protein Users – on a session dedicated to ‘Patient voice inclusion in the organisation of care: PLUS vision and Patient involvement in European Reference Networks’

### IPFA – International Plasma Fractionation Agency

IPOPI was pleased to participate in the annual International Workshop on “Surveillance and Screening of Blood Borne Pathogens” co-organised by the International Plasma Fractionation Association (IPFA) and the Paul-Ehrlich-Institut (PEI).

The 23rd edition of the Workshop was held in May, in Lisbon, and formally hosted by the Portuguese Institute for Blood and Transplants (IPST as in the acronym in Portuguese).

The workshop addressed a diverse range of issues, most especially detection, epidemiology, costs and regulation of new and old agents, that threaten the safety of blood components and plasma derivatives.



**EUROPEAN MEDICINES AGENCY**  
SCIENCE MEDICINES HEALTH

### EMA

Patients, as the end-users of medicines, are considered as key stakeholders by EMA. IPOPI President Jose Drabwell participates in European Medicines Agency (EMA) meetings as a member of the Patients’ and Consumers’ Organisations Working Party (PCWP). Jose continues to play a very important role helping to voice PID patients’ perspectives in pharmacovigilance, patient involvement in clinical trials, data transparency, among others. Leire Solis, Health Policy and Advocacy Manager, supports Jose in this active role.

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

- . EMA PCWP meetings, London, all year
- . European Parliament MEP liaison Programme, all year
- . Eurordis General Assembly, Edinburgh, UK, May 2016
- . European Rare Disease Conference (ECRD), Edinburgh, UK, May 2016
- . Rare Diseases Day Events, Brussels, Belgium, February 2016
- . Health First Europe (HFE) Meetings, Brussels, Belgium, all year
- . EU Parliament Interest Group Innovation Health & Social Care Meetings, May & November 2016
- . 15<sup>th</sup> Biennial Convention of the Philippine Society for Asthma, Allergology and Immunology, September 2016
- . European Haemophilia Round Table Meeting, Brussels, Belgium, February 2016
- . Health First Europe (HFE) General Assembly, Brussels, Belgium, June 2016
- . 3<sup>rd</sup> APEC (Asia Pacific Economic Cooperation), Hanoi, Vietnam, December 2016
- . Eurordis DITA Task Force Meeting, Paris, France, September 2016
- . 11<sup>th</sup> ICORD (International Conference on Rare Diseases & Orphan Drugs), Cape Town, South Africa, October 2016
- . XIth International Conference on Rare Diseases and Orphan Drugs (ICORD) meeting, South Africa, October 2016
- . Meeting with Philippines Paediatrics Society, October 2016
- . Meeting with Taiwanese Immunology Society, October 2016
- . UKPIN Conference, London, UK, November 2016

### Looking Ahead – 2017 and beyond

IPOPI looks forward to continuing supporting 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.

The third International Primary Immunodeficiencies Congress - IPIC2017 - in the vibrant city of Dubai is just around the corner! More details [www.IPIC2017.com](http://www.IPIC2017.com)



TO BE HELD IN EUROPE

Venue will be announced at IPIC2017



# FINANCIAL REPORT 2016

2016 marks a year of good financial performance for IPOPI, its second most successful to date in terms of income/expenditure and most successful year for a non-congress year.

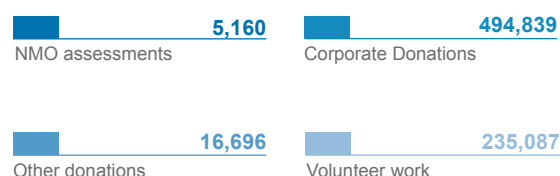
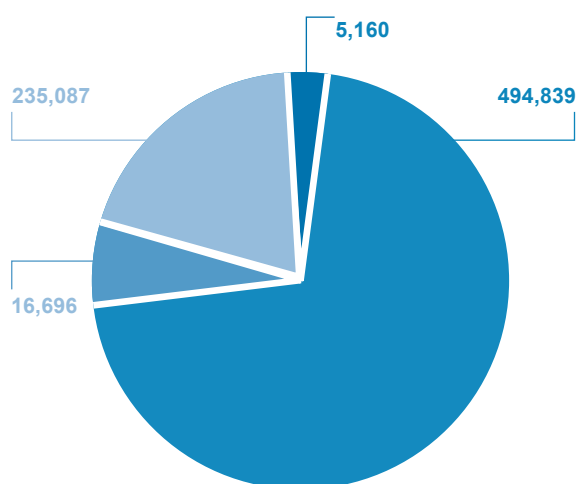
IPOPI focused its efforts and used its resources to enhance its advocacy activities, drive awareness campaigns as well as strengthen and increase its work with NMOs.

Thanks to a successful congress in 2015 (IPIC2015 in Budapest, Hungary) IPOPI was able to dedicate substantial financial support towards its Biennial patients meeting enabling close to 100 patient representatives to participate.

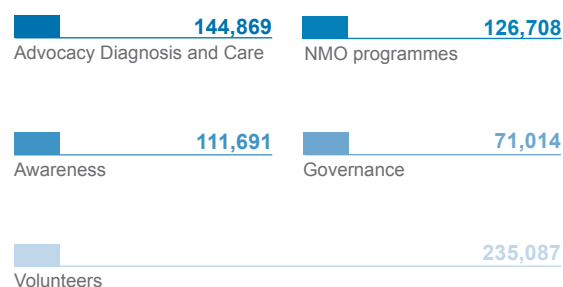
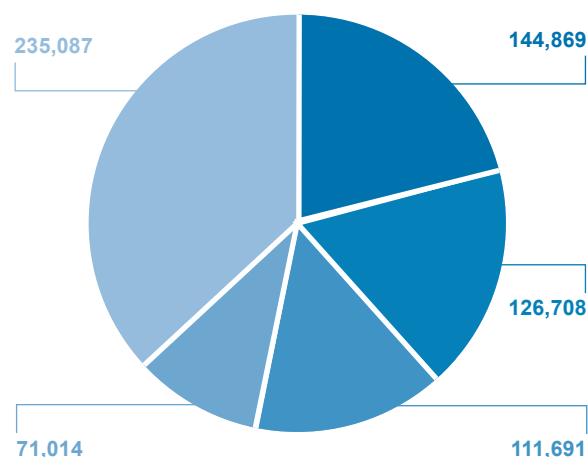
IPOPI continued to ensure it has a reserve policy in line with the UK charity commission recommendations.

IPOPI is a charity registered in the United Kingdom and under UK Charity Law. As such, we are required to have our accounts audited every year by qualified accountants and submitted to the Charity Commission for approval. The following charts provide a summary of key aspects of our financial accounts in 2016. Our full audited accounts are available on the Charity Commission website and on request. Importantly 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 2016 — 751,782 GBP



## EXPENSES 2016 — 689,369 GBP



## Sources of Funding 2016 - Percentages

Industry funding	66%
NMO membership fees	1%
Other	2%
Congress	0%
Volunteers	31%
<b>Total</b>	<b>100%</b>

No contribution received from a single company reached the majority of total income. The highest single contribution by a company represented 28% of the total income.

## Income and expenses 2016 (GBP)

<b>INCOME</b>	<b>2016</b>
NMO assessment fees	5,160
Corporate donations	494,839
Other donations	16,696
Volunteers	235,087
<b>Total</b>	<b>751,782</b>

<b>RESOURCES EXPENDED</b>	<b>2016</b>
Advocacy, Diagnosis and Care	144,869
NMO programmes	126,708
Awareness	111,691
Governance	71,014
Volunteers	235,087
<b>Total</b>	<b>689,369</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. We also express our gratitude to our 61 National Member Organisations whose important activities and efforts in their respective countries are vital to improve the conditions for people living with a PID. Our NMOs are key stakeholders who contribute towards the global mission of IPOPI.

### Corporate Partners

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

#### PLATINUM



#### GOLD



#### SILVER



#### BENEFACTOR

Biotest / Pfizer / BPL  
Sigma Tau

### Project sponsors

In 2016, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities

Baxalta/Shire  
Biotest

CSL Behring  
GSK

Kedrion  
LFB

Perkin Elmer  
Octapharma

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 2016 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 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, Sociedad Española de Inmología (SEI), SEAPID (South East Asia Primary Immunodeficiencies Network), Sociedade Portuguesa Alergologia Imunologia Clínica, Sociedade Portuguesa de Pediatria- infecciologia pediática, Thai Immunology Society (AAIAT), Vietnamese Paediatrics Association, World Health Organization (WHO), World PI Week and its member organisations.





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**INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES**  
IPOPI is a charity registered in the UK. Registration No. 1058005.

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