

# REVIEW OF ACTIVITIES AND ACHIEVEMENTS

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# 2015





# IPIC2017

## INTERNATIONAL PRIMARY IMMUNODEFICIENCIES CONGRESS

an IPOPI event

8/10 NOVEMBER  
2017 // DUBAI

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



## A MESSAGE FROM THE CHAIRPERSON



Jose Drabwell, IPOPI Chairperson

Dear Friends and Colleagues,

2015 was filled with milestone activities and achievements for IPOPI and is to date our most successful year in terms of the number of programmes that were implemented as well as financially speaking. IPOPI was involved on many different fronts including policy, advocacy, regulatory, NMO development and awareness raising activities as well as in the organisation of our second landmark conference, the International Primary Immunodeficiencies (IPIC) congress in Budapest, Hungary.

With 700 delegates coming from 75 countries, attendance at IPIC2015 increased by 75% compared to 2013 (400 delegates) testifying to the successful model of the conference which focuses primarily on the clinical management of primary immunodeficiencies. Based on the CME evaluation forms that were collected during the event an impressive 80% of delegates indicated that the congress programme would change their clinical practise. This piece of statistics reinforces IPOPI's belief that a clinically-focused international conference such as IPIC was much awaited by the medical community. With Dubai as our 2017 destination for the congress, we are already working on further strengthening this strategic direction and ensuring regions such as the Middle East and Asia can benefit from such an educational event.

Besides its involvement in international developments, IPOPI also capitalized on its increasingly successful regional and national programmes. In Asia, IPOPI joined the Care-for-Rare Foundation to co-organize the first South East Asian PID expert meeting in Bangkok that culminated with the launch of the South East Asian Primary Immunodeficiencies Network (SEAPID) which brings together experts from countries such as Thailand, Malaysia, Vietnam, Singapore and Indonesia. A few months later the Asian Pacific Society for Immunodeficiency (APSID) was also launched and both groups are working to cover a region which represents almost 60% of the world's population to optimize access to PID care in the region working hand in hand with IPOPI and its NMOs. IPOPI organised two launch meetings in the region in 2015 in Jakarta (Indonesia) and Hanoi (Vietnam) and is now proud to have welcomed new NMOs in these countries, joining Japan, India, Thailand, Malaysia, Hong Kong, Singapore and soon additional countries in the region. IPOPI was also active in Africa and Latin America organizing regional patients' meetings in collaboration with regional societies (ASID and LASID), both of which were greatly attended events and the starting point of several new collaborations.

On the advocacy front, IPOPI held its successful 6th IPOPI PID Forum at the European Parliament in Brussels to launch the PID principles of care, IPOPI's most recent key publication elaborated with the assistance of a network of 30 regional key opinion leaders in the field. In addition IPOPI continued its SCID newborn screening campaigns, notably with a policy event held in the Italian Senate in Rome to request the implementation of SCID newborn screening in the country.

I hope you will enjoy reading this annual report which summarizes many of the other activities and achievements of IPOPI in 2015.

A handwritten signature in white ink that reads "Jose Drabwell". The signature is fluid and cursive.

Yours sincerely,  
Jose Drabwell

# EXECUTIVE COMMITTEE, OFFICERS AND STAFF

## EXECUTIVE COMMITTEE

**Jose Drabwell**, Chair (United Kingdom)  
**Martine Pergent**, Vice Chair (France)  
**Christine Jeffery**, Treasurer (Australia)  
**Marcia Boyle** (USA)  
**Roberta Pena** (Argentina)  
**Dragana Koruga** (Serbia)  
**Adrian Górecki** (Poland)  
**Violetta Kozhereva** (Russia)  
**Bruce Lim** (Malaysia)

## STAFF

**Johan Prevot**, Executive Director  
**Leire Solis**, Health Policy and Advocacy Manager  
**Magda Lourenço**, IPOPI Communications and NMO Programming Proj. Manager  
**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. Andrew Cant** (United Kingdom)

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

**Prof. Amos Etzioni** (Israel)

**Dr. Jose Franco** (Colombia)

**Dr. António Condino Neto** (Brazil)

**Prof. Luigi Notarangelo** (USA/Italy)

**Prof. Surjit Singh** (India)

**Dr. Joanne Smart** (Australia)

**Prof. Martin Van Hagen** (The Netherlands)

**Prof. Klaus Warnatz** (Germany)

### GENERAL PRACTITIONER ADVISER

**Dr. Lotte Vosmar Denning**



Dr. Nizar Mahlaoui,  
IPOPI MAP Vice Chair



Leire Solis, IPOPI Health  
Policy and Advocacy  
Manager



**Back row**, left to right: Johan Prevot - IPOPI Executive Director, Bruce Lim - Malaysia, Jose Drabwell – IPOPI Chairperson, Roberta Pena – Argentina, Prof Bobby Gaspar – IPOPI MAP President, Magda Lourenço – IPOPI Communications and NMO Programming, Carol Tavener – IPOPI Bookkeeping and accounting.  
**Front row**, left to right: Violetta Kozhereva – Russia, Dragana Koruga – Serbia, Christine Jeffery – Australia, Marcia Boyle – USA, Carla Morgado – IPOPI Executive assistant, Martine Pergent – IPOPI Vice Chair.

# IPOPI STRATEGIC PLAN

## MISSION STATEMENT

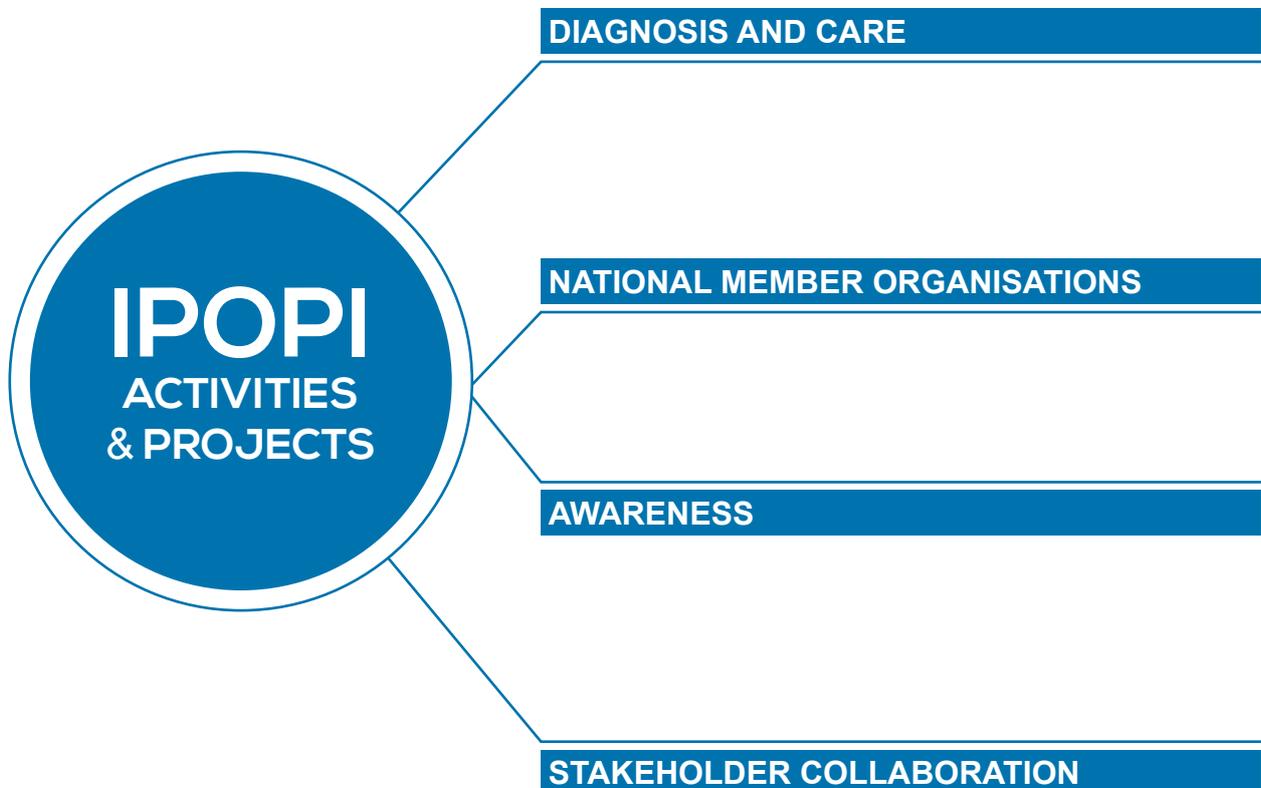
The Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide through global collaboration.

IPOPI is the leading advocate for primary immunodeficiencies patients worldwide. We work in collaboration with patients, doctors, politicians, regulators, pharmaceutical industry and many other 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 **280 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 misdiagnosed, the immune system remains defective, often leading to illness, disability, permanent organ damage or even death.

**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.

It is estimated that around 60% of PIDs can be easily diagnosed with a simple and inexpensive blood test, however **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. Screening for some of the most severe forms of immunodeficiency will also diagnose more individuals and improve treatment outcomes. 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.

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 Organization. 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. A new type of SC infusion containing recombinant human hyaluronidase, known as facilitated subcutaneous immunoglobulin (FSCIG) 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**.

IPOPI is committed to continue 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 please check [www.ipopi.org](http://www.ipopi.org)

# KEY ACHIEVEMENTS AND ACTIVITIES, 2015

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

### **IPIC2015 – International Primary Immunodeficiencies Congress**

IPIC2015 was a great success with 700 participants from over 70 countries in attendance!

The congress took place at the Marriot Budapest Hotel on 5-6 November and featured invaluable perspectives on clinical aspects of primary immunodeficiencies (PIDs) from world renowned experts such as Prof Alain Fischer, Prof Amos Etzioni, Prof Jennifer Puck, Prof Bobby Gaspar among many others. It also included ethical and patient perspectives on several aspects of PID clinical care. Prof Bobby Gaspar, IPOPI's Medical Advisory Panel Chairman and IPIC2015's Scientific Committee President stressed that 'the inclusion of patient perspectives, ethical dilemmas and multi stakeholder involvement made this meeting a unique learning experience'. **Based on CME evaluation feedback forms 80% of doctors indicated that the contents of the congress programme would impact their clinical practice.**

The sessions and symposia covered a broad range of issues such as the management of gastro-intestinal complications, severe combined immunodeficiencies (SCID), the promises and pitfalls of new genetics, the importance of patient registries treatment options among others.

IPIC congresses have unique clinically orientated programmes prepared with the input from all the key PID involved parties: doctors, patients and nurses. The next IPIC will be held on 8-10 November 2017 in Dubai.



## PIDs Principles of Care - Implementation Package

IPOPI published in 2014 the “Primary Immunodeficiencies Principles of Care” in a combined effort with a worldwide multi-disciplinary team of 28 medical experts representing the five continents.

The *PIDs Principles of Care* cover the role of specialised centres, importance of registries, the need for international collaborations for scientific research, the role of patients groups, management and treatment options for PIDs, and management of PID diagnosis and care throughout the world.

IPOPI is committed to help its members better advocate for improved access to diagnosis and care. To help them prioritise and apply these six Principles we developed an Implementation Package with a dedicated guide and infographics. The Implementation package was first presented at the 6<sup>th</sup> IPOPI PID Forum dedicated to the PIDs Principles of Care and has since then been used in regional meetings. IPOPI intends to review the progress made on the Implementation in 2016/2017.



## IPOPI 6th PID Forum at the European Parliament

IPOPI's 6th Primary Immunodeficiency (PID) Forum was chaired by Members of the European Parliament Carlos Zorrinho (S&D, Portugal) and José Inácio Faria (ALDE, Portugal) and dedicated to the Principles of Care for Primary Immunodeficiencies (PID).

Participants stressed the need to implement the PID Principles of Care across the European Union member states and placed a specific emphasis on international collaboration in research, care and treatment. The meeting focused on the key role of patient organisations and the need for newborn screening, an essential tool to fasten access to curative treatment for severe combined immunodeficiencies (SCID).

The Forum allowed for an agreement on what the 'gold standard' treatment for PID patients should be and helped identify the European tools and policies that can be used to improve access to diagnosis and care for PID patients. The MEPs present offered their support for further actions and better policy-making with the collaboration of other EU institutions, patient organisations and healthcare professionals.



Prof Martin Van Hagen, Jose Drabwell, MEP Carlos Zorrinho, Johan Prevot and Prof Bobby Gaspar at the 6th IPOPI EU PID Forum.

### SCID Newborn Screening Meeting at Italian Senate

IPOPI in collaboration with AIP Onlus (IPOPI Italian NMO - Associazione Immunodeficienze Primitive) and Italian experts in the field held a meeting on SCID newborn screening (NBS) at the Italian Senate in Rome in September 2015. The meeting was chaired by Senator Paola Taverna with the support of Senator Laura Bianconi and aimed at promoting the need for implementation of SCID NBS in Italy.

Several experts and representatives from the Health authorities spoke at the event including among others Professor Chiara Azzari (Florence) who is heading a SCID newborn screening pilot programme in Tuscany, Prof Maria Cristina Pietrogrande (Milan), Prof Raffaele Bodolato (Brescia), Dr Serena Battilomo from the Ministry of Health. Gaetano Priolo (AIP) and Bianca Pizzera (IPOPI Honorary Vice President) provided the patients' viewpoint from a national and international perspective.

The Italian National Paediatrics Society and the Italian Rare Diseases Organisation (UNIAMO) were also in attendance and the meeting got the support of the Italian Undersecretary for Health.

The event marked an important milestone in IPOPI's SCID NBS advocacy campaign in Europe and helped identify the way forward in Italy.



Prof Maria Cristina Pietrogrande and Prof Chiara Azzari with Jose Drabwell and Bianca Pizzera.



Participants at the Italian Senate.

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

### Bob LeBien Asian Development Plan

Bob LeBien, IPOPI's Honorary President and first winner of the IPOPI Bob LeBien Award has been personally supporting the launch of an IPOPI outreach campaign in the South-East Asian region. The Bob LeBien Asian Development Plan allowed IPOPI to develop several actions in the region during 2015.

### First Care-for-Rare European-South East Asian PID Meeting” in Bangkok, Thailand

IPOPI in collaboration with the Care-for-Rare Foundation, co-organised the “First Care-for-Rare European-South East Asian PID Meeting – from Bench to Bedside” in Bangkok, Thailand in January 2015. The meeting was endorsed by Thailand's Minister for Health, His Excellency Mr Rajata Rajatanavin, and attended by over 100 PID doctors and stakeholders.



Speakers, medical students and guests at the meeting.



# 1<sup>ST</sup> CARE-FOR-RARE EUROPEAN-SOUTH-EAST ASIAN PID-MEETING: FROM BENCH TO BEDSIDE

**JANUARY 26, 2015, 9 AM – 6 PM  
CHULALONGKORN UNIVERSITY, BANGKOK, THAILAND**

This successful and historical event brought together for the first time PID experts from 6 countries in South East Asia including Indonesia, Malaysia, the Philippines, Singapore, Thailand and Vietnam. The meeting was held in Chulalongkorn University Hospital and included scientific lectures by Prof Christoph Klein (Founder of the Care-for-Rare Foundation) and Prof Martin Van Hagen (IPOPI Medical Advisory Panel).

The First Care-For-Rare European-South-East-Asian PID Meeting was pivotal in identifying key priorities for the region, launching a “PIDs in South East Asia” Call for Action, marking the birth of SEAPID (South East Asian Network of PID experts) and providing a stepping stone for more patient group launches in the region.

## First Indonesian PID Patients-Doctors Meeting

The first IPOPI Indonesian PID Patients-Doctors National Meeting took place in Jakarta, in May 2015 and brought together 55 participants from various regions in Indonesia including patients, family members, Indonesian doctors and guest lecturers from The Netherlands and Malaysia.



Lively discussion with the participants.



Speakers, guests and patient representatives from Indonesia.

The meeting was the first of its kind in Indonesia, providing both scientific lectures by Prof Martin Van Hagen, Dr Adli Ali and Dr Nia Kurniati and patients’ presentations.

There was a unanimous agreement between the patients and the doctors that a national patient organisation should be launched and that a network between the different PID centres should be created to share diagnosis facilities and exchange specimens.

Subsequently an Indonesian PID patient support group (PPIPI) was created and it is now an IPOPI member.

## First Vietnamese PID Patients-Doctors Meeting

The Vietnamese PID Patients-Doctors Meeting brought together 80 participants in December 2015 and culminated with the agreement to launch a patient group. The meeting took place in Hanoi and was organised in collaboration with the National Paediatrics Hospital and the Vietnamese Paediatrics Association.



Very engaged parents and patients in the audience.



Participants at the first Vietnamese PID meeting.

The meeting started with presentations from Prof Martin Van Hagen, Dr Narissara Suratannon and Johan Prevot and a lively discussion followed. Families of PID patients expressed their concerns about the future of their children and about the long distances some have to travel to get treatment.

Some of the participants indicated their willingness to take part in the patient organisation and the mother of a young boy was indicated as possible future chairperson.

## IPOPI's regional patient meetings

### IPOPI African Patients Meeting in Algiers, Algeria

IPOPI seized the opportunity of the IVth ASID (African Society for Immunodeficiencies) congress that took place in Algeria in May 2015 to organise a Regional Patients' Meeting giving African patient representatives from 9 countries in attendance (Algeria, Benin, Egypt, Morocco, Senegal, South Africa, Sudan, Uganda, and Zambia) the opportunity to consider necessary action plans and to align strategies.



From left to right: Keith Simbotwe (patient, Zambia), Mrs Djermouli, (patient, Algeria), Samir Kheddari, Algeria), Joy Rosario, (mother of patient, South Africa), Zahir Azzouz (patient, Algeria), Martine Pergent (IPOPI), Papa Ndiaye, (patient, Senegal), Winnie Manara (mother of patient, Uganda), Dr Maroufou Jules Alao (Benin), Prof Tandakha Dieye (Senegal), Paediatrician (Zambia), Mohamed Fdhil (Morocco).

Most African countries are faced with conflicting priorities in their public health authorities as other better known conditions are often prioritised over PIDs. Another important aspect is the high consanguinity rate and the importance of antenatal diagnosis which is not yet well implemented.

There was a general understanding that awareness efforts must be made so that PIDs are taken into consideration in public health policies and the meeting ended with the launch of an African PID Call to Action.

## IPOPI Latin American Patients Meeting in Buenos Aires, Argentina

IPOPI organised the 2015 edition of the Latin American Regional Patients Meeting back to back with the LASID Congress (Latin American Society for Immunodeficiencies Congress).



Roberta Pena and Jose Drabwell presenting.



Patients and speakers at the meeting.

This great event gave participants an opportunity to learn about sustainability tactics for the NMOs (National Member Organisations) in the region, how to implement locally the 6 Principles of Care for PIDs and the importance of SCID Newborn Screening.

The meeting took place on the 18th and 19th of November at the Sheraton Libertador Hotel in Buenos Aires Argentina and was well attended by 11 countries of the Latin American region that learnt from each other's experience and from IPOPI's specifically tailored Workshops.

## World Primary Immunodeficiency Week (WPIW) – an increasing success!

IPOPI is a founding partner of the WPIW initiative and continues to contribute to its growth. WPIW is celebrated each year from the 22 to 29 April and 2015 marked its fifth edition.

IPOPI was able to support 18 countries campaigns: Spain, France, Morocco, Serbia, Ukraine, Iceland, Romania, Finland, Netherlands, India, South Africa, Malaysia, Colombia, Portugal, Canada, Italy and Poland. We witnessed many exciting activities and awareness events, media outreach campaigns, medical education meetings, family gathering and sportive events.



For more information and to get involved please visit [www.worldpiweek.org](http://www.worldpiweek.org)

## IPOPI NMO Support Programme 2015

Through its core funding IPOPI was able to support the following national member organisation programmes in 2015.



'À toute jambes' from Krista Bracke, translated from Dutch to French by BOPPI (Belgium NMO) with IPOPI support

- . Board game about PIDs in Poland
- . Translation into French of a book by a CVID patient in Belgium
- . Printing of translated patient information leaflets in Argentina
- . Printing of translated patient information leaflets in Australia
- . Printing of translated patient information leaflets in UK
- . Printing of translated patient information leaflets in Ukraine

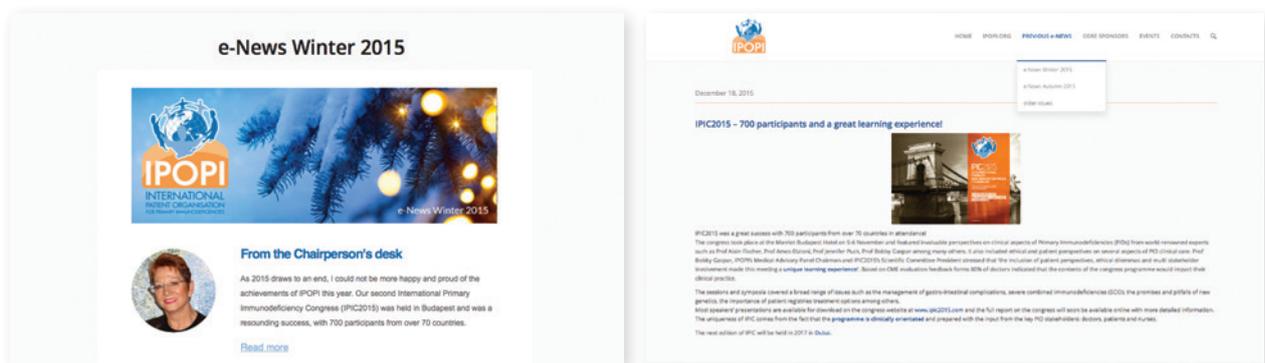
## STRATEGIC OBJECTIVE 3 – TO RAISE PID AWARENESS GLOBALLY

### IPOPI e-News – new format

Our quarterly electronic newsletter is released each year in March, June, September and December and remains our main periodic communications tool.

It comprises three specific sessions: **IPOPI's News**, **Around the world** and **NMO Focus**. IPOPI uses this important tool to report its actions, share statements, announce publications and promote upcoming meetings. Our NMOs are invited to submit articles on their own milestones for the Around the world section and in each issue there is an in-depth article on one NMO in a section called NMO Focus.

In 2015 IPOPI renewed and improved the format of e-News making it more visually appealing, easier to navigate and to share. The new format has its own web address and is well linked to IPOPI's website, agenda and sponsors page.



Please visit [e-news.ipopi.org](http://e-news.ipopi.org)

### Social Media

IPOPI's social media presence has grown significantly in 2015, boosted especially by IPOPI's events and publications. IPIC2015, the launch of the 2015 IPOPI patient information leaflets, our quarterly e-News, the EU PID Forums, etc, all provided great momentum for social media engagement and generated further awareness of PIDs. By strategically using its social media pages IPOPI has been making its actions known to thousands of people throughout the world.

Social media also provides a good platform for a less public type of interaction and experience sharing, especially valuable for rare diseases patients and parents. A Facebook private group called **IPOPI PID talks: Share, Learn and Get Inspired!** was created with this in mind. The IPOPI group has gained significant interest from the online PID community and while English is the most used language, other languages are also welcomed.

### Check and follow our social media pages!

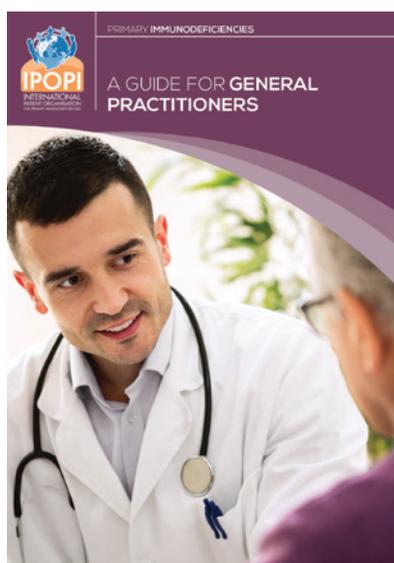
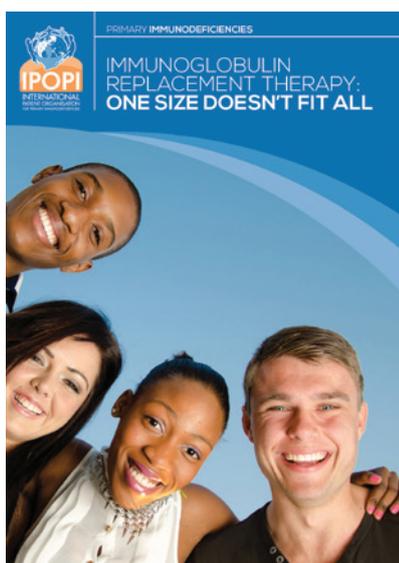
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- . [youtube.com/IPOPIPID](https://youtube.com/IPOPIPID)



## New Patient Information Leaflets

In 2015 IPOPI started developing a new series of patient information leaflets that were officially launched early 2016. The new leaflets provide helpful information and guidance for patients, their families and other interested stakeholders of the Primary Immunodeficiencies (PID) community.

The first leaflet, 'Immunoglobulin replacement therapy - One size does not fit all', explains the variety of factors that patients with PIDs should consider, and discuss with their physician, when an immunoglobulin replacement therapy is chosen. The second leaflet, 'A guide for general practitioners', demonstrates how general practitioners (GPs) can play a crucial role in identifying patients with PIDs and discusses key aspects of patient care while the third leaflet, 'How are primary immunodeficiencies classified?', explains the latest classification system for primary immunodeficiencies.



### IPOPI patient leaflets

- Immunoglobulin replacement therapy - One size does not fit all
- A guide for general practitioners
- How are primary immunodeficiencies classified?

To download please go to [www.ipopi.org](http://www.ipopi.org)

## Burden of Treatment Survey

IPOPI in collaboration with Sheffield University and Parexel, a specialised agency, embarked on the preparation of a new survey looking at Burden of Treatment of immunoglobulin therapies for adult PID patients. The survey, which was launched during World PI Week in 2016, will aim to quantify and measure the impact of the different immunoglobulin treatments adults take to manage their PID. IPOPI believes that in capturing this data, using a Burden of Treatment PID-specific validated instrument, the PID community will be equipped with data that can help better advocate for access to the choice of treatments needed to meet the individual needs of those living with PIDs.

## STRATEGIC OBJECTIVE 4 – TO STIMULATE STAKEHOLDER COLLABORATION

IPOPI has always valued the benefits of working together with its stakeholders and 2015 was no exception!



### SCIDNET

SCIDNET is a multi-stakeholder consortium aimed at developing licensed genetic medicines that can be used as a standard treatment for children with Severe Combined Immunodeficiency (SCID) worldwide in 4 years.

This project is coordinated by Professor Bobby Gaspar (IPOPI Medical Advisory Panel Chairman) at the University College of London and financially supported by the European Commission.

IPOPI is an official partner organisation in the project representing the PID patients' community. The role of IPOPI in this exciting partnership focuses on providing the PID patient viewpoint on the research and development process. IPOPI will advise on matters such as patient involvement in clinical design, trial protocols, patient/parent information, information dissemination to patient families, etc.

For more information on the project and IPOPI's role, please visit [www.scidnet.eu](http://www.scidnet.eu)



### European Reference Networks

The European Commission fosters the concentration of healthcare knowledge and resources necessary to provide highly and cost-efficient specialised care for rare medical conditions and has identified the development of European Reference Networks (ERNs) as one of its top health priorities for the next years.

As one of the first partners involved, IPOPI is committed to bringing the voice of patients with PIDs into its ERN. We are currently collaborating in the development of the Rare Immunodeficiency, Autoinflammatory and Autoimmune (RITA) Network, a European Reference Network uniting under the same umbrella, specialised centres in Europe dedicated to Primary Immunodeficiencies, autoinflammatory and autoimmune disorders.

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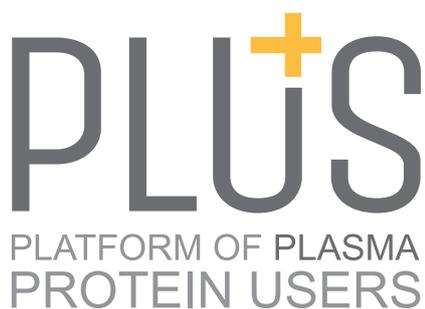


### **Johan Prevot joined Board of HFE (Health First Europe)**

The HFE (Health First Europe) board elections were held during the Annual General Meeting (AGM) in Brussels in June. Johan Prevot, IPOPI's Executive Director, who has been a member of HFE since 2014 was elected as a board member. John Bowis was re-elected as Honorary President and Albert van der Zeijden from the International Alliance of Patient Organisations (IAPO) and Bert van Caelenberg from European Federation of Public Service Employee (EUROFEDOP) were re-elected Vice Presidents.

Health First Europe is an alliance of patients, healthcare workers, academics and healthcare experts and industry. HFE's mission is

to ensure that equitable access to modern, innovative and reliable medical technology and healthcare is regarded as a vital investment in the future of Europe. HFE and IPOPI share many common objectives including on patient-centeredness, patient involvement in HTAs, early diagnosis and prevention. IPOPI looks forward to a new chapter in the collaboration with HFE at the European level.



**PLUS, the Platform of Plasma Protein Users**, is formed by the following patient organisations: EHC, Alpha-1 Global, GBS/CIDP Foundation, HAEI, ITP Support Association, WFH, A-PLUS and IPOPI. PLUS meets every year to discuss pressing topics concerning plasma products and their impact on patients' health. The 2015 meeting was held in March, in Rome, Italy, and focused on the European legislation on blood and blood products and on the developments at the World Health Assembly regarding a Decision on "Blood and other medical products of human origin" which was also discussed at the World Health Organization (WHO) Executive Board earlier in 2015.

The PLUS Steering Committee elections being held during the meeting with Brian O'Mahony (EHC), Johan Prevot (IPOPI) and Henrik Boysen (HAEI) being elected.



### **Rare Diseases International**

IPOPI participated in the official Launch Meeting of Rare Diseases International (RDI), in May, in Madrid, Spain, together with around 60 other patient group representatives. The Participants adopted a Joint Declaration calling for rare diseases to become an international public health priority.

The RDI initiative is led by EURORDIS and national rare disease alliances from the United States (NORD), Canada (CORD), Japan (JPA), China (CORD), India (I-ORD), the Ibero-American pan-regional alliance (ALIBER) and the International Federation for Epidermolysis Bullosa (DEBRA International). Johan Prevot was nominated as member of the RDI Pre-Formation Steering Committee responsible for steering RDI until an elected Executive Committee is put into place.

### **International Plasma Protein Congress**

In March IPOPI attended the 21st edition of the International Plasma Protein Congress International Congress (IPPC) organised by the Plasma Proteins Therapeutics Association (PPTA) in Rome, Italy. The meeting programme focused on many interesting topics including: personalising treatments to better fit patients' needs, the challenges and opportunities patients are facing, access to care and health economics.

Jose Drabwell, IPOPI Chairperson, had the opportunity to provide an overview on the challenges and opportunities patients with PIDs are currently facing in their access to care. Jose went over IPOPI's activities including those supporting its national organisations in their advocacy efforts, awareness raising and campaigns to ensure an equal access to screening, diagnosis and care for PIDs around the world.

## EMA

Patients, as the end-users of the medicines, are considered as key stakeholders by EMA. IPOPI President Jose Drabwell participates in the European Medicines Agency (EMA) meeting as 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, IPOPI Health Policy and Advocacy Manager, supports Jose in this active role.

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

- . Eurordis council of federations Meeting, Paris
- . EMA PCWP meetings, London, all year
- . EMA Clinical Trials Meeting, London, December 2015
- . Eurordis General Assembly, Berlin, May 2015
- . Rare Diseases Day Meeting and Gala Dinner, Brussels, February 2015
- . European Parliament MEP liaison Programme, all year
- . European Parliament SCID NBS follow up campaign
- . European Haemophilia Consortium events and round table
- . European Reference Networks (ERNs) conference, Lisbon, Portugal, October 2015
- . IPFA-PEI International Workshops
- . EPPOSI workshops and meetings in Brussels
- . Health First Europe General Assembly Meeting, Brussels (May 2015)
- . 6th World Orphan Drugs Congress, November 2015

## Looking Ahead – 2016 and beyond

IPOPI looks forward to continuing supporting the PID 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.

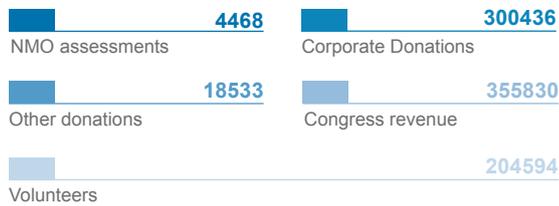
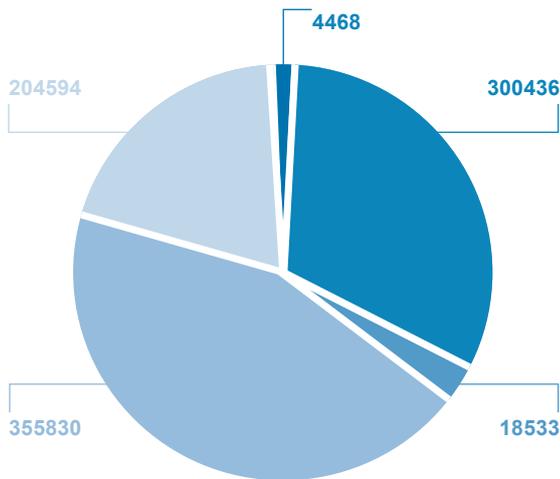
The third International Primary Immunodeficiencies Congress (IPIC2017) will be held on 8-10 November 2017 in the vibrant city of Dubai, stay tuned to [www.ipic2017.com](http://www.ipic2017.com) for updated information.

# FINANCIAL REPORT 2015

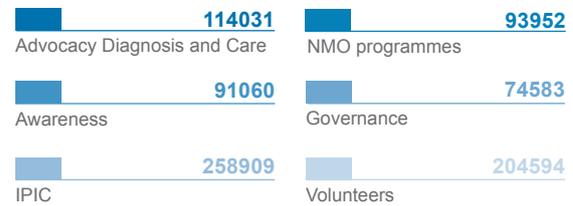
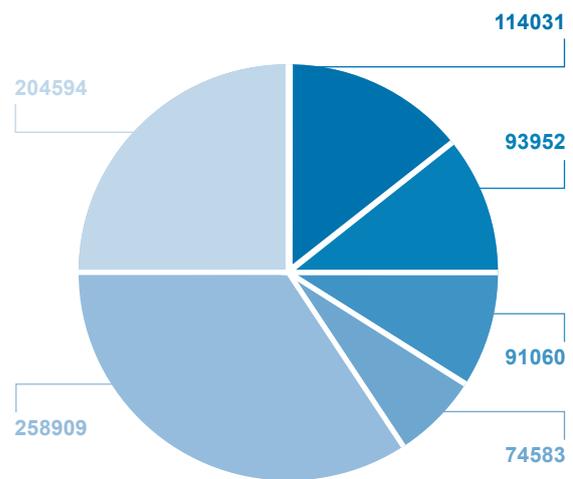
2015 has been a very good financial year for IPOPI, in fact its most successful to date. The increasing popularity of its International Primary Immunodeficiencies Congress played a key part in this success and enabled IPOPI to organise a hugely successful clinical conference praised by its attendees for its uniqueness and usefulness for PID clinicians. This also helped IPOPI deliver patient awareness and advocacy programmes, organise regional meetings and constitute extra funding for its 2016 Biennial meeting, thereby enabling to fund travel grants for patient representatives of its growing network of national member organisations. IPOPI continued to diversify its sponsors base (for details see page 19). IPOPI also ensured it has a reserve in line with UK Charity Commission recommendations. With an increased number of patient programmes, events and activities, 2015 also marks the highest yearly expenditure for IPOPI.

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 2015. 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 accountants.

### Income 2015 (GBP)



### Resources expended 2015 (GBP)



### Sources of Funding 2015 - Percentages

Industry funding	34%
NMO membership fees	1%
Congress	40%
Other (donations, bank interests)	2%
Volunteers	23%
<b>Total</b>	<b>100%</b>

No contribution received from a single company reached the majority of total funding received. The highest single contribution by a company represented 14% of the total funding, volunteer income included. With volunteer income excluded, the highest single contribution would represent 19%.

### Income and expenses 2015 (GBP)

INCOME	2015
Corporate Donations	300436
NMO assessment fees	4468
Congress registration revenue	355830
Other income	18533
Volunteers	204594
<b>Total</b>	<b>883861</b>

RESOURCES EXPENDED	2015
Advocacy, Diagnosis & Care	114031
NMO programmes	93952
Awareness	91060
Governance	74583
Congress	258909
Volunteers	204594
<b>Total</b>	<b>837129</b>
Excess towards Reserve & Patient meetings / programmes	(46732)

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 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.

The IPOPI Board of Directors and Staff also would like to thank Bob LeBien and his family for their generosity and continued support towards our Asian regional activities.

Lastly, IPOPI would like to extend its sincere thanks to the following corporate partners whose contributions in 2015 helped us implement our mission and improve awareness, access to early diagnosis and optimal treatments for primary immunodeficiency (PID) patients worldwide:

### Corporate Partners

In 2015, the following companies provided support under the IPOPI corporate partnership programme which is essential for IPOPI to efficiently manage and expand its wide range of activities including awareness, advocacy/ policy, representational, communication activities; organisation of international & regional meetings as well as support to national patient programmes and campaigns.

#### PLATINUM



#### SILVER



### Project sponsors

In 2015, the following companies provided sponsorship grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities

Baxalta

CSL Behring

LFB

RMS Medical Products

Biotest

Grifols

Octapharma

Sanquin

Cane SpA

Kedrion

Perkin Elmer

Sigma Tau

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

### Key Stakeholders

IPOPI has made stakeholder collaboration a specific objective in its new strategic plan 2016-2020 and we would like to express our most sincere thanks to a number of stakeholder organisations for their partnership and collaboration in 2015 including but not limited to: African Society for Immunodeficiencies (ASID), 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), 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, 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 Déficiés 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 pediatica, Thai Immunology Society (AAIAT), Vietnamese Paediatrics Association, World Health Organisation (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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