



REVIEW  
OF ACTIVITIES  
AND ACHIEVEMENTS  
**2013**



# IPIC2015

## INTERNATIONAL PRIMARY IMMUNODEFICIENCIES CONGRESS

FOCUS ON CLINICAL CARE  
AND DIAGNOSIS



## A MESSAGE FROM THE CHAIRPERSON



Jose Drabwell, IPOPI Chairperson

Dear Friends and Colleagues,

2013 was a historical year for IPOPI and the global PID community can be proud of its achievements. Great initiatives took place during the year in all regions of the world to enhance access to early diagnosis and care for PID patients and raise awareness of Primary Immunodeficiencies. This was only possible because of global stakeholder collaboration.

The launch of the First International Primary Immunodeficiencies Congress (IPIC), IPOPI's first scientific congress, was a resounding success. With 400 participants from 52 countries, featuring a programme including great speakers and most importantly the very positive feedback received

at and since the conference has confirmed the need for an international congress with a focus on clinical care and diagnosis of PID. At the time of writing these lines, we have started to prepare for IPIC 2015 which will be held on 5-6 November 2015 in Budapest.

Excellent outcomes were also achieved in collaboration with stakeholders such as the ASID-IPOPI congress in South Africa, the LASID-IPOPI congress in Chile, the World Primary Immunodeficiencies Week, the launch of IPOPI's Bob LeBien Asian development plan and so many other advocacy and awareness campaigns. IPOPI has continued to expand its staff and 2013 was also the most successful year to date in terms of fundraising.

The year was also marked by changes within the IPOPI's Medical Advisory Panel (MAP). Dr Teresa Español who had led our MAP for so many years retired from her position as Chairperson and we are forever thankful to her for her commitment to IPOPI and the PID global community. Dr Español was awarded an Honorary Vice-President position within the MAP in recognition of her dedication. It is with great pleasure that IPOPI welcomed Professor Bobby Gaspar as its new MAP Chairman and Dr Nizar Mahlaoui as Vice-Chairman. I am certain that their advice and support to IPOPI's activities will enable us to achieve yet many more successes in the future.

New national member organisations also joined IPOPI and IPOPI's network of NMOs has now risen to 51.

All of our work is focused on ensuring that we optimize the lives of people living with Primary Immunodeficiencies. It is with this clear remit that we carry out our projects, programmes and activities. We are thrilled by the successful outcomes of our work in 2013 and it is with great inspiration, enthusiasm and renewed energy that IPOPI will forge its path forward in 2014.

I would like to thank all of our Board and Staff members, NMOs, MAP members, stakeholders and our sponsors for believing in IPOPI and helping us make it the organisation it is today. I hope you will enjoy reading our 2013 review of activities.

A handwritten signature in white ink that reads "Jose Drabwell". The signature is fluid and cursive, with a large loop at the end.

Jose Drabwell

## EXECUTIVE COMMITTEE, OFFICERS AND STAFF

### EXECUTIVE COMMITTEE

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**Martine Pergent**, Vice Chair (France)  
**Sven Fandrup**, Treasurer (Denmark)  
**Marcia Boyle** (USA)  
**Dragana Koruga** (Serbia)  
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**Johan Prevot**, Executive Director  
**Leire Solis**, Health Policy and Advocacy Manager  
**Magda Lourenço**, Communications and NMO  
Programmes Officer  
**Carla Morgado**, Executive Assistant  
**Carol Tavener**, Bookkeeping & Administration  
**Clare Glynn**, Financial consultant  
**David Watters**, External Consultant

### MEDICAL ADVISORY PANEL

**Prof Bobby Gaspar**, Chair (UK)  
**Dr Nizar Mahlaoui**, Vice Chair (France)  
**Prof Ewa Bernatowska** (Poland)  
**Prof Andrew Cant** (UK)  
**Prof Esther de Vries** (Netherlands)  
**Dr Monika Esser** (South Africa)  
**Prof Amos Etzioni** (Israel)  
**Prof Alain Fischer** (France)  
**Dr Jose Franco** (Colombia)  
**Prof Luigi Notarangelo** (Italy/ USA)  
**Prof Alessandro Plebani** (Italy)  
**Prof Surjit Singh** (India)  
**Prof Klaus Warnatz** (Germany)



IPOPI's Board Members, Executive Director and Medical Advisory Panel Chairperson

# IPOPI STRATEGIC PLAN

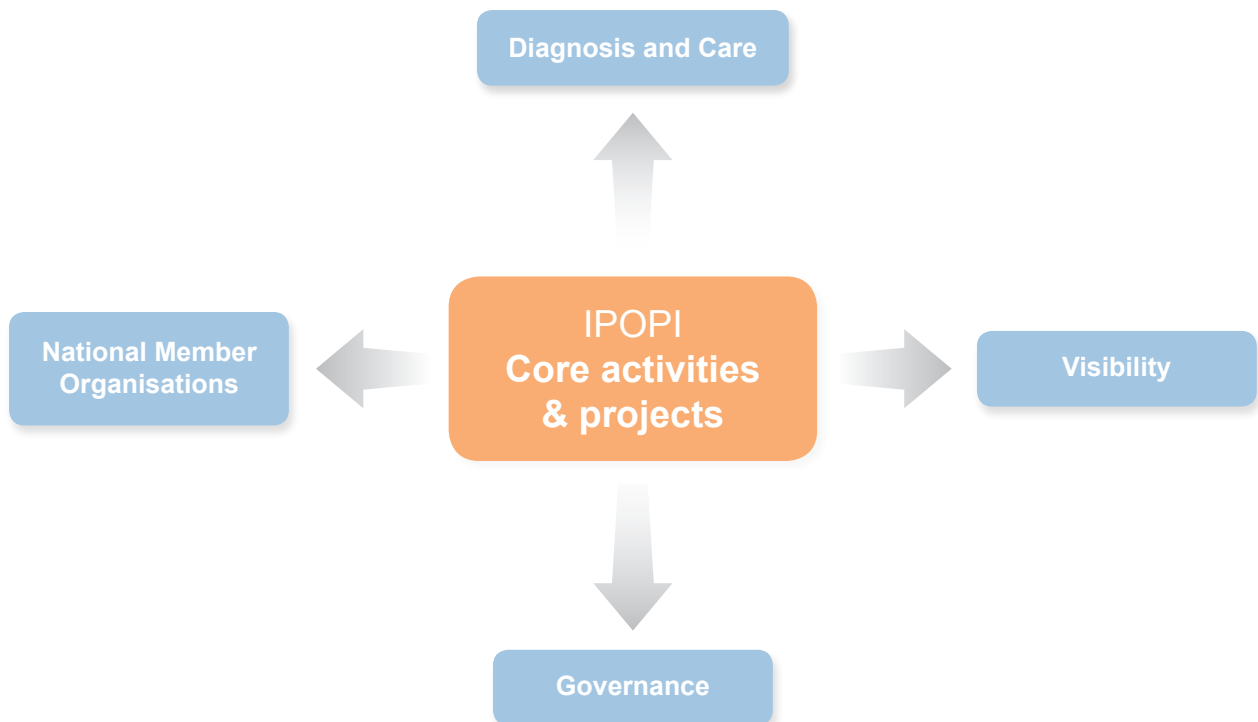
## MISSION STATEMENT

IPOPI is the association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency (PID) patients worldwide.

### Strategic Objectives

IPOPI's current 4 year strategic plan to 2015 builds on IPOPI's significant growth over the last couple of years as well as on lessons and outcomes learnt through the course of our various activities. In designing this plan, IPOPI streamlined its strategy around four key strategic objectives with the aim to guide IPOPI's actions and activities in the 4 years to come, but with a clear vision to the long term future:

1. To ensure optimal access to early diagnosis & care
2. To develop, strengthen and support National Member Organisations (NMOs)
3. To enhance IPOPI and PID visibility globally
4. To increase efficient governance of a well resourced developing organisation



## PRIMARY IMMUNODEFICIENCIES SOME KEY BACKGROUND INFORMATION

Primary Immunodeficiencies (PIDs) are a large and growing group of over 250 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 other people to infections. When PIDs are left under-diagnosed or mis-diagnosed, 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.

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. 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 (such as Immunoglobulin replacement therapy and Bone Marrow Transplantation among others) which can only happen if early and correct diagnosis is made, many sequelae will be avoided and lives of patients will be saved.

This is why 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.



## KEY ACHIEVEMENTS AND ACTIVITIES, 2013

### STRATEGIC OBJECTIVE 1 – ENSURE OPTIMAL ACCESS TO DIAGNOSIS & CARE



400 participants at the first edition of IPIC



Jose Drabwell in the opening address

#### International Primary Immunodeficiencies Congress – IPIC 2013

An impressive number of 400 participants attended the 1st International Primary Immunodeficiencies Congress (IPIC) organised by IPOPI and held on 7 and 8 November 2013 in Estoril (Lisbon), Portugal.

The congress will take place every two years in the future. The 2nd edition of IPIC will be held on 5-6 November 2015 in Budapest, Hungary.

The main aim of IPIC is to propose a unique, multi-disciplinary international forum focusing on clinical care developments in the PID field. Several key topics including PID treatments, complications management, genetic studies and counselling, PID pathogenesis and SCID newborn screening were presented and discussed at this first event.

The programme featured an array of world renowned clinical and scientific experts in the field including Prof Amos Etzioni, Prof Alain Fischer, Prof Bobby Gaspar, Prof Ricardo Sorensen, Prof Troy Torgerson as well as Mrs Marcia Boyle, Mrs Jose Drabwell and Mr Johan Prevot among many others. Key presentations given during the meeting can be viewed on the congress website [www.ipic2013.com](http://www.ipic2013.com) as well as the abstracts of the posters presented during the two Poster Walks.

Most of the participants at the meeting were physicians, however IPOPI's national member organisations were also very well represented and highly participative. IPOPI has received very positive feedback on the outcomes of this milestone event especially due to its clinical focus and international approach. The full Report of the congress is also available online with more detailed information.

#### 4th IPOPI EU PID Forum on Cross-border Healthcare

IPOPI held its 4th PID Forum on Cross-border Healthcare and PIDs in the European Parliament in Brussels, Belgium. The Forum looked into the potential benefits the [Directive 24/2011/EU](#) on the Application of patients' rights in cross-border healthcare could bring to patients with PIDs and was hosted by Member of the European Parliament Emer Costello (S&D, Ireland).

Experts representing patients, academics, physicians and industry gathered with Members of the European Parliament and the European Commission to discuss the need for Member States to fully implement the legislative text and the benefits for PID patients.

After careful discussion, several recommendations were made, while some relate to Rare Diseases as a generality, others refer specifically to PIDs:

- The European Union must continue its work in supporting its citizens that suffer from rare diseases including primary immunodeficiencies (PIDs) an important subgroup of 250 rare disorders.
- The Cross-border healthcare Directive lays the ground for improving patients' rights and access to diagnosis, treatment and care.
- Patient organisations and healthcare professionals should be involved in the process.
- Member States can improve diagnosis rates by improving patients' access to medical expertise abroad. Bureaucratic requirements should not hinder access to diagnosis and care.
- The EU should encourage Member States to provide timely access to lifesaving treatments such as bone marrow transplants and immunoglobulin therapies for patients with PIDs.
- Access to diagnosis/treatment/care should not be limited by the cost of the treatment (pay up-front).
- The availability of specialist treatments in another Member State should not incentivise Member State of affiliation to discontinue or not increase its level of care.
- Cooperation on HTA between Member States and with key stakeholders should focus on establishing an appropriate approach for rare diseases therapies (i.e. immunoglobulin replacement therapy) to ensure enhanced access to care for PID patients.
- Member States that have not yet implemented the Cross-border healthcare Directive should do as soon as possible, with the "spirit" of the Directive in mind.



IPOPI representatives and other stakeholders at the 4th IPOPI PID Forum

#### IPOPI SCID Newborn Screening programme

IPOPI has been actively advocating for the implementation of SCID Newborn Screening in the European Union Member States for the past 3 years.



MEP Glenis Willmott, IPOPI's SCID campaign supporter with Jose Drabwell

In February 2013, with the support of MEP Glenis Willmott (S&D, UK), an Oral Question was submitted by the European Parliament ENVI Committee responsible for all Health matters (Environment, Public Health and Food Safety) following IPOPI's EU Advocacy campaign for SCID Newborn Screening.

Following the European Commission and the European Council responses to the Oral Question which pointed to the issue of national competence, IPOPI participated in May in a consultation launched by Eurordis aimed at re-tabling the topic of Newborn Screening for rare diseases in the discussions of the EUCERD (EU Committee of Experts on Rare Diseases) to emphasize the importance of establishing

European recommendations to guide member states. In July EUCERD released its adopted Opinion Paper on "Newborn Screening in Europe: Opinion of the EUCERD on Potential areas of European Collaboration" which included actions to improve the quality and the efficiency of the screening process, while respecting the values of the Member States. In IPOPI's opinion, a set of EU guidelines on Newborn Screening would be extremely helpful to help Member States collaborate, exchange information and consolidate approaches in an area where information and expertise are scarce. IPOPI has released a position statement (available on [www.IPOPI.org](http://www.IPOPI.org)) on this topic and is committed to keep advocating for SCID newborn screening at EU level.

IPOPI has also started working on national advocacy programmes specifically designed to fasten the implementation of SCID newborn screening in European member states. At the time of writing this report, work has started on such a programme in France. Several SCID NBS pilot projects are now also in place in Europe helping to save lives of children through timely screening and IPOPI is following their outcomes closely.

### Working with stakeholders

IPOPI has always appreciated the value and the benefits of working with stakeholders. In 2013, IPOPI intensified joint-stakeholder collaborations with key medical opinion leaders, other patient organisations, industry stakeholders, nurses and others. This includes work with the Plasma Users Platform (PLUS) which is made up of patient groups representing consumers of plasma products and of which IPOPI is a founder member. IPOPI participated in the 4th annual PLUS Consensus Meeting which was held in Estoril, Portugal, on 5-6 September 2013.

Specifically two sessions were organised during the meeting. The first one focused on Risk-Based Decision Making (RBDM). The Alliance of Blood Operators (ABO) RBDM project aimed at developing an integrated risk management strategy set the stage for the discussion. It was agreed that the project could be a significant first step to establish a framework for managing the interrelationship of risk tolerance, supply of blood and plasma derived medicinal products and economic considerations in the future. PLUS has since then been further engaging into the project and is organising a joint meeting with the ABO RBDM project in London in 2014. The second session focused on Treatment in 2020: Patient Views. Mr Johan Prevot and Dr Nizar Mahlaoui provided presentations on behalf of the PID community outlining the importance of the prioritization of key indications such as: PIDs on demand-management mechanisms in a context of increasing demand for IG therapies, the need for individualized IG therapies to best meet patients' needs and rebutting attempts to look upon immunoglobulin as a generic medicine. The need for an increased number of reference centres and qualified registries was also highlighted. The next PLUS consensus conference is scheduled for 15-16 January 2015.

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

- EMA PCWP meetings, all year
- Rare Diseases Day Meetings and Gala Dinner, February 2013
- 19th International Plasma Protein Congress, March 2013
- Wildbad Kreuth Policy Meeting on optimal use of clotting factors and immunoglobulins, Germany, April 2013
- IPFA-PEI International Workshop, Helsinki, April 2013
- Eurordis General Assembly meeting, Dubrovnic, May 2013
- European Haemophilia Consortium events and round tables
- Bioplasma conference, September 2013, Indonesia
- 64th WHO Expert Committee on biological standardization at WHO, October 2013



Dr Teresa Español, being awarded as Honorary Vice-President of IPOPI's MAP

### Medical Advisory Panel

2013 marked a change in the membership of IPOPI's Medical Advisory Panel, as Dr Teresa Español retired from her position as Chairperson during the 1st International Primary Immunodeficiencies Congress. A special ceremony was held during the congress reception to recognize Dr Español's outstanding commitment over the years to IPOPI and the PID patients community. Dr Español was awarded with an Honorary MAP Vice-President title. Prof Bobby Gaspar (United Kingdom), who has been increasingly working with IPOPI in recent years succeeded Dr Español as Chairman of IPOPI's MAP. In addition, Dr Nizar Mahlaoui (France), was appointed as Vice-Chairman. IPOPI is delighted and honoured to have them both on board.

### European Medicines Agency (EMA)

As an active patient representative on the European Medicines Agency Working Party (PCWP), Jose Drabwell ensured that the views of the patients and the PID community were incorporated in the discussion and work of this important regulatory committee. Among other developments, in September, a joint meeting of the PCWP and the Healthcare Professionals' Working Party (HCPWP) was held to address patient input in medicine development and post-authorisation studies. Whilst patient representatives have participated in this process they are still excluded from key decisions on licensing. The participants stressed that patient involvement in the Pharmacovigilance Risk Assessment Committee (PRAC) will lead to increased public understanding of the risk-benefit concept and potentially increase trust in the regulatory system and improve knowledge on the safe and rational use of medicines. Discussions have started to take place on a pilot phase for a patient to be an observer at the Committee for Medicinal Products for Human Use (CHMP).

### IPOPI National Rare Diseases Plans Implementation Position Statement

IPOPI released a position statement outlining the need for EU member states to take action with regards to the implementation of national rare diseases plans as foreseen in the Council Recommendation 2009/C 151/02 on an action in the field of rare diseases from 8 June 2009. The council recommended that all member states elaborate and adopt a plan or strategy by the end of 2013 at the latest. However several member states have still not implemented any official plans and IPOPI called for a swift implementation.

As an important group of more than 250 rare but chronic diseases affecting both children and adults for which effective treatments exist, IPOPI recommended that Primary Immunodeficiencies be specifically addressed by national policy makers when national plans for rare diseases are developed and PID patients should be consulted on the implementation of the activities envisioned in the plans. The full statement can be accessed at the IPOPI website – [www.IPOPI.org](http://www.IPOPI.org)

As a follow up to IPOPI's statement a Parliamentary Question was tabled by MEP Nessa Childers.

## STRATEGIC OBJECTIVE 2 – TO DEVELOP, STRENGTHEN AND SUPPORT NMOs

### Bob LeBien Asian Development Plan

In 2012 at the occasion of IPOPI's 20th anniversary Bob LeBien, one of the Organisation's founders and Honorary President was awarded with the first IPOPI Bob LeBien award created in honour of Bob who together with Prof Helen Chapel had the vision to initiate the creation of IPOPI and the commitment to serve as the founding chairperson.

In his thank you speech Bob went over the history of IPOPI and stressed the importance of learning from the past: "I believe that every organisation should have knowledge of its past, because it gives a better sense of the present and a possible direction for the future". Bob also highlighted the fact that IPOPI's regional development was instrumental in improving PID patients' lives and ensuring patients are empowered. Specifically, Bob indicated that the challenge ahead for IPOPI is how to reach developing regions of the world, and primarily Asia. Since then Bob has personally supported the start of an IPOPI outreach campaign in the Asian region and several actions were developed in the region.



**Thailand** - The meeting brought together around 50 participants including patient representatives, doctors, nurses and other stakeholders.

In September, IPOPI presented at the Asian Bioplasma Conference advocating for patients' access to diagnosis and care and the importance of launching patient groups in Asia. In October, in collaboration with the Asia Pacific Association of Pediatric Allergy, Respiratory and Immunology (APAPARI 2013), IPOPI held the First Thai PID Patients Meeting in Bangkok, Thailand in conjunction with the congress. Later in the same month in collaboration with the "National Clinical Immunology Symposium (NACLIS) 2013 International Conference: Creating Awareness on Primary Immunodeficiencies (PID) in South East Asia (SEA)" the First IPOPI National Malaysian PID Patients Workshop was held in Kuala Lumpur, Malaysia. During December, a similar meeting was held in Hong-Kong at the Academy of Medicine with the support of the Department of Paediatrics and Adolescent Medicine, LKS Faculty of Medicine of the University of Hong Kong. As a direct result of these meetings a new patient group called MyPOPI has been created in Malaysia and at the time of writing these lines, Hong Kong and Thailand are actively working towards similar launches.



**Brazil** - Participants from two patient groups and two hospitals gathered for this meeting.

#### **IPOPI NMO Awareness Campaign Workshops – Brazil and Romania**

IPOPI's Awareness Campaign Workshops have been pivotal in helping several NMOs launch national awareness campaigns. In 2013 two workshops were held.

For the first time IPOPI organised an Awareness Workshop in Latin America. The event was held in São Paulo, Brazil. IPOPI's Brazilian NMO ABRI (Associação Brasileira de Imunodeficiência) and the other national patient group, ANPIC (Associação Nacional dos Portadores de Imunodeficiência Primária Congénita), both actively participated

along with key Brazilian medical stakeholders and a specialized public affairs agency.

A joint awareness campaign was agreed and an action plan was established centered around three key strategic areas: Treatment, Diagnosis and the Patient Associations cooperation. Subsequently to the campaign a joint response to the Brazilian Ministry of Health public consultation on Rare Diseases was submitted carving out specific recommendations for primary Immunodeficiencies and further steps for political outreach meetings with key political decision makers are being planned.

IPOPI held a second very successful Awareness Workshop in 2013 organised in cooperation with ARPID (the Romanian Association of Patients with PIDs) in the beautiful city of Sibiu.

The event brought together stakeholders from Romania including patient representatives, family members, leading Romanian doctors, industry representatives as well as IPOPI staff and health policy consultants. Several common priorities to focus on were identified, including among others the need for better treatment and treatment options for PID patients requiring IG replacement therapy and the discrepancies between adult and children care. Objectives pertaining to direct patient care and structural care issues were determined and a specific awareness campaign action plan was agreed.

Since the workshop ARPID has launched several awareness campaigns, participated in the activities of the Romanian rare diseases group and engaged with the Romanian Minister of Health.

### **IPOPI Regional Meetings – South Africa & Chile**

IPOPI held a fruitful and productive regional patient meeting during the ASID (African Society for Immunodeficiencies) congress held in Sun City, South Africa from 5-9 June 2013 which was organised for the first time jointly with the meetings of the Allergy Society of South Africa (ALLSA) and the Thoracic Society of South Africa (SATS) making it one of the largest medical congresses on the African continent in 2013. The meeting brought together around 30 participants including patient representatives, doctors, nurses and stakeholders from South Africa, Morocco, Uganda, Senegal and Zambia. After the presentations an interactive discussion allowed the participants to identify key issues and recommendations among others:

- the need for clinical and diagnosis guidelines in the region
- the need for increased medical awareness
- the need to work with other active regional patient and advocacy groups
- the need for more patient support
- the need for more data on prevalence and epidemiology
- the need for an integrated approach to screening and diagnosis



**South Africa** – All the stakeholders together at the meeting

The beautiful city of Santiago in Chile hosted the 3rd LASID (Latin American Society for Primary Immunodeficiencies) congress from the 17-10 October 2013 attended by 460 participants and IPOPI seized the opportunity to organise a Regional LATAM Patients Meeting. 10 countries participated (Argentina, Brazil, Colombia, Chile, Costa Rica, Mexico, Peru, Puerto Rico, Uruguay and Venezuela).

After two medical lectures Jose Drabwell and Roberta Pena (responsible for Latin America) gave examples of several IPOPI advocacy campaigns and spoke about early diagnosis and access to treatment in the region. Concrete actions and steps to be taken were identified to increase awareness around PID in the region. All participants agreed to work together regionally in the future in order to address common problems that affect all countries in the region.

## World Primary Immunodeficiency Week (WPIW)

IPOPI is a founding member of WPIW a worldwide awareness campaign which aims at stimulating efforts to improve the recognition, diagnosis, treatment and quality of life of people with Primary Immunodeficiencies worldwide.



# World PI Week

Test. Diagnose. Treat.

Since the first edition of WPIW, IPOPI has been able to financially support 31 national awareness campaigns spread over the 5 continents. These included media outreach campaigns, medical lectures, family days, political events, online campaigns, among others.

WPIW is a great opportunity for national PID Patient organisations to emphasise the number of PID patients in their country and IPOPI strongly encourages them to be involved in each annual campaign

## News from the National Member Organisations

The following list does not intend to be fully comprehensive but only to provide some examples of NMO campaigns carried out in 2013:

- **Brazil:** ABRI and the other local PID patient group, ANPIC, took part in an IPOPI Awareness Campaign Workshop. As a result a national political awareness campaign was started
- **Finland:** IMPPU organised a Career and Education Event for physicians, patients and pharmaceutical industry
- **Germany:** DSAI, found a patron, an actress called Michaela Schaffrath, who was very helpful in getting media coverage for DSAI's events and activities
- **Greece:** Armonia, translated 6 IPOPI leaflets into Greek
- **India:** IPSPI from India have organised several awareness events for physicians
- **Morocco:** Hajar's WPIW 2013 campaign included a media campaign, the 1st PID Spring medical school, Hajar's Family Day events and a gala dinner as well as targeted actions on blood donor's day.
- **New Zealand:** IDF NZ celebrated its 25th anniversary with many PID awareness campaigns and with the production of new handover materials
- **Poland:** Immunoprotect conducted a political awareness campaign in 2013 which led to Subcutaneous IG coverage and availability for adults in early 2014.
- **Romania:** ARPID launched two major communication tools: a new website built with IPOPI Web ToolKit and translated the first series of IPOPI Patient Information Leaflets into Romanian
- **Russia:** Our Russian NMO obtained coverage for immunoglobulin therapy for more than 40 patients in different Russian regions and keeps advocating for better access to treatment
- **South Africa:** PiNSA provided Sub-cut training to 9 doctors with the help of PiNSA's Medical Advisory Panel Doctors and a qualified nursing sister.
- **Spain:** AEDIP increased its political PID awareness activities by meeting with the Spanish MoH and with regional health authorities as a follow up to 2012 IPOPI Awareness Campaign Workshop in Barcelona
- **UK:** PID UK Increased PID awareness through their new website and with a national media outreach campaign





French Parliament PID Event – representatives from IPOPI, IRIS, CEREDITH and Members of the French Parliament

### National Parliamentary events

IPOPI has started working on a National Parliamentary Event Programme in 2012 which is being used to increase the visibility of PIDs at national level and politically position PID patients priorities within the national health policy system.

The first National Parliamentary Event was held in Paris, France, in December 2013 focusing on the topic 'Improve access to diagnosis and treatment for PID Patients'. Patricia Schillinger and Dominique Orliac, Members of the French Parliament chaired the event attended by members of IPOPI, IRIS (our French NMO) and CEREDITH (Reference centre for PIDs).

A set of 14 recommendations was proposed by IRIS, IPOPI and CEREDITH and shared with the meeting participants: healthcare authorities, physicians, patient representatives and other stakeholders.

At the end of this fruitful meeting Patricia Schillinger called for a national campaign for SCID newborn screening and Dominique Orliac emphasised the importance of a safe, continuous and diversified supply of immunoglobulin.

### IPOPI NMO Support Programme 2013

Through its core funding, IPOPI was able to support the following national member organisation programmes in 2013:

- Advocacy campaign Ireland, Q4 2013 including liaison with Irish Minister of Health
- Educational videos about PIDs produced by Immunoprotect (Poland)
- South African Patients Meeting during the ASID congress
- PID Nordic Patients conference in Iceland
- Communications Campaign in Argentina
- Communications Campaign in Spain
- Advocacy and awareness campaign in Brazil
- Advocacy and awareness campaign in Romania
- Awareness campaign in Morocco
- Awareness campaign in Portugal
- Support to attend awareness workshop to Bosnia and Herzegovina

## STRATEGIC OBJECTIVE 3 - TO ENHANCE IPOPI AND PID VISIBILITY GLOBALLY

### IPOPI Global Patients Survey

A manuscript outlining the data and findings from the IPOPI survey on Patient Needs and Outlooks whose final report was released in 2012 was prepared and submitted for publication in 2013. At the time of writing these lines, the manuscript has been accepted for publication in the Journal of Patient Preference and Adherence, an international peer-reviewed open access journal. Patient satisfaction, acceptability, quality of life, compliance, persistence and their role in developing new therapeutic modalities and compounds to optimise clinical outcomes for existing disease states are key areas of interest for the journal. The main objective of the manuscript is to gain understanding of how existing PID therapies affect patient lives and to identify desired improvements to immunoglobulin treatments.

### New Patient Information Leaflets

In 2013 IPOPI issued the third series of its Patient Information Leaflets launched in 2011 that comprise helpful information and guidance for patients, their families and other interested stakeholders of the PID community.

The 2013 leaflets look at:

- Vaccines and Primary Immunodeficiencies
- Autoimmunity and Autoinflammation
- PIDs and Plasma derived therapies

The first leaflet provides general guidance on vaccinations that should be considered by PID patients, the second one contains information on autoimmune and autoinflammatory diseases in PID patients and the third one describes the plasma-derived therapies used to treat primary immunodeficiencies.

During the past year IPOPI was proud to collaborate in the production of several translated versions of the previously issued series of leaflets. All of them are available for download at [www.IPOPI.org](http://www.IPOPI.org)

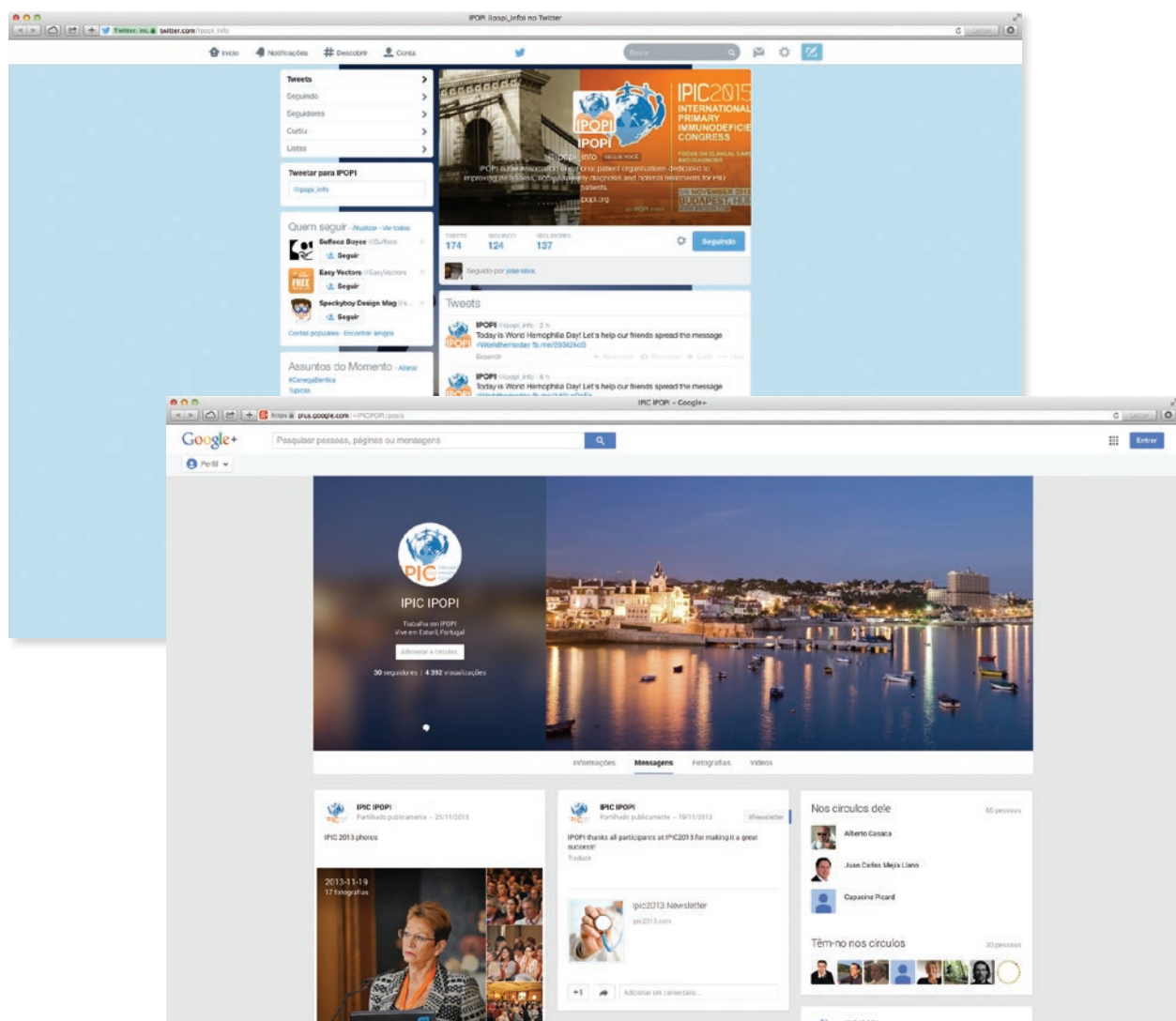


#### New IPOPI patient leaflets

- Primary immunodeficiencies — Autoimmunity and autoinflammation
- Primary immunodeficiencies — Plasma-derived therapies
- Primary immunodeficiencies — Vaccines and primary Immunodeficiencies

## New communication tools

In 2013 IPOPI expanded its social media tools to reach out to new audiences and release dedicated content in different platforms. In addition to Facebook, IPOPI became active on Twitter rapidly gaining attention and many active followers. In preparation for IPIC 2013 a G+ account dedicated to the congress news was created and widely promoted in the congress e-blasts and website.



IPOPI continues to support its Membership, especially newly created patient support groups to kick-start their online presence, thus reaching more people. POSPID (Serbia) launched their website through IPOPI's website start up ToolKit, IPiA (Ireland) and MyPOPI (Malaysia) have their new websites under construction.

IPOPI is continuously finding new ways to promote its activities and events and many additional promotional materials were created and used in 2013.

## STRATEGIC OBJECTIVE 4 - TO INCREASE EFFICIENT GOVERNANCE OF A WELL-RESOURCED DEVELOPING ORGANISATION

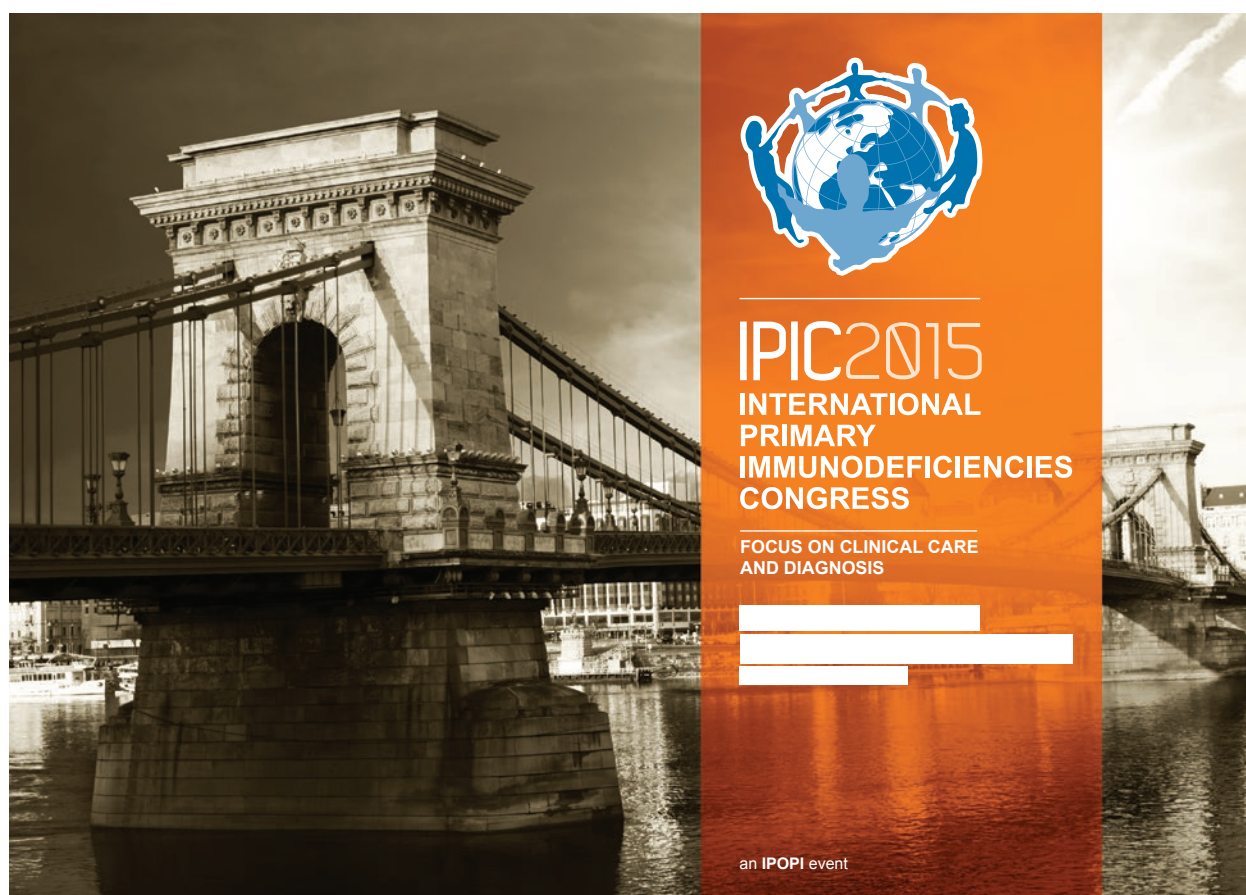
In 2013 IPOPI implemented its improved governance and diversified its level of funding by securing collaboration with new corporate players, notably through the organisation of the first International Primary Immunodeficiency Congress (IPIC). The recruitment of new staff together with a strategy focused approach to its core activities and ad-hoc projects enabled IPOPI to enhance its overall efficiency and make significant advances as reported above. The IPOPI registered offices remain in the UK.

### Looking Ahead – 2014 and beyond

There are many exciting projects ahead for IPOPI. 2014 is a Biennial Meeting year and it is with great excitement that IPOPI is preparing its 13th Biennial Patients Meeting to be held jointly with the meetings of the European Society for Immunodeficiencies (ESID) and the International Nursing Group for Immunodeficiencies (INGID). Two award ceremonies will also take place distinguishing an adult and a young person for making a difference in the PID community, the Luciano Vassalli and the Bob LeBien Awards.

IPOPI looks forward to continuing its advocacy work internationally. 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 undoubtedly strengthen PID patient organisations around the world.

And of course, in 2015 the second edition of the International Primary Immunodeficiencies Congress (IPIC2015) will take place in the beautiful city of Budapest!

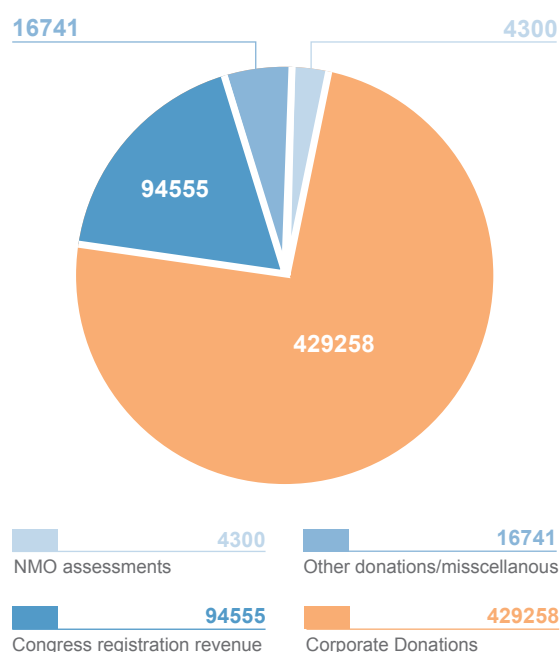


## FINANCIAL REPORT 2013

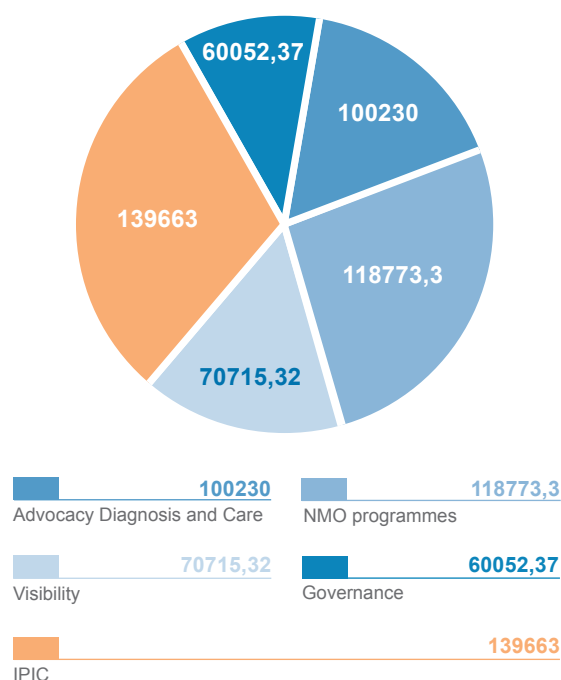
Financially 2013 was a successful year for IPOPI marked by a 31% increase in revenue compared to 2012. This was made possible thanks to additional funding generated by the first IPIC. IPOPI strengthened existing collaborations with its sponsors but also continued to diversify its sponsors base (for details see page 19). This has contributed to further secure IPOPI's reserve in line with UK Charity Commission recommendations as well as the ability to recruit new staff. IPOPI dedicated more resources towards its core activities and ad-hoc projects as well as support programmes to its NMOs which resulted in the highest number of projects and campaigns ever carried out within a calendar year as shown in this report. Expenditure therefore also increased by 36% compared to 2012.

IPOPI is a charity registered in the United Kingdom and under UK Charity Law. As such, IPOPI is required to have its accounts audited every year by qualified accountants and submitted to the Charity Commission for approval. These charts provide a summary of key aspects of the financial accounts in 2013. Full audited accounts are available on request.

Income 2013



Resources expended 2013 (GBP)



Income and expenses 2013 (GBP)

INCOME	2013
Corporate Donations	429,258
NMO assessment fees	4,300
Congress registration revenue	94,555
Other income	16,741
<b>Total</b>	<b>544,854</b>

RESOURCES EXPENDED	2013
Advocacy, Diagnosis & Care	100,230
NMO programmes	118,773.3
Visibility	70,715.32
IPIC	139,663
Governance	60,052.37
<b>Total</b>	<b>489,434</b>
Excess towards Reserve & IPIC	(55,420)

IPOPI is grateful to a 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 each year for people living with a primary immunodeficiency worldwide.

We also express our gratitude to our 51 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.

IPOPI expresses its gratitude to the following corporate sponsors whose contributions in 2013 helped implement IPOPI's mission and improve awareness, access to early diagnosis and optimal treatments for primary immunodeficiency (PID) patients worldwide:

#### Core sponsors

In 2013, the following corporate sponsors provided unrestricted grants towards IPOPI's core awareness, advocacy, communications and representational activities, meetings and projects:



#### Project sponsors

In 2013, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities

Baxter Healthcare Corporation  
Binding Site  
Biotest  
BPL  
CANE Spa  
CSL Behring  
Grifols  
Kedrion Biopharma  
IPFA (International Plasma Fractionation Association)  
LFB  
Octapharma  
Perkin Elmer

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

We started this review by mentioning the importance of stakeholder collaboration and it is therefore fitting to conclude this report by providing our most sincere thanks to a number of stakeholder organisations for their partnership and collaboration in 2013 including but not limited to: African Society for Immunodeficiencies (ASID), Asociación Española de Pediatría (AEP), British Society for Immunology (BSI), Clinical Immunology Society (CIS), 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), Food and Drug Administration (FDA-US), Grupo Português de Imunodeficiências Primárias (GPIP), 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 Union of Immunological Societies (IUIS), Latin American Society for Immunodeficiencies (LASID), Le Centre de Référence Déficits Immunitaires Héritaires (CEREDITH-FR), Plasma Protein Therapeutics Association (PPTA), Platform of Plasma Protein Users (PLUS) and its member organisations, Sociedad Española de Inmología (SEI), Sociedade Portuguesa Alergologia Imunologia Clínica, Sociedade Portuguesa de Pediatria, World Health Organisation (WHO), World PI Week and its member organisations.



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**INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES**

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