

Review of 2008 - 2009



IPOPI

INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES





Review of 2008 - 2009

*The association of national patient organisations dedicated to improving awareness,
access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide.*

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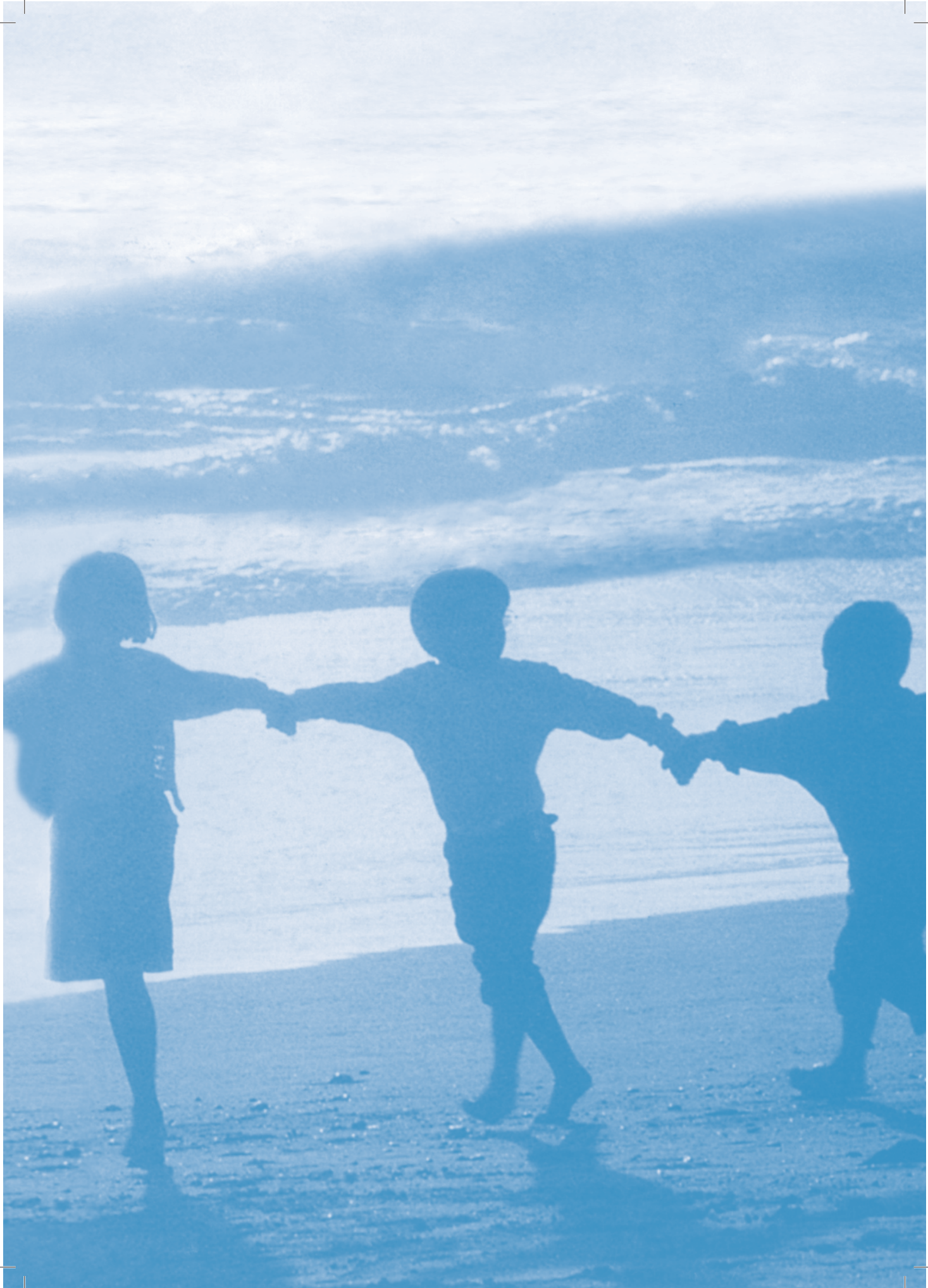
The Chairperson writes: ...

One of the greatest – and most exciting! - professional challenges I have faced is that of being the Chairperson of IPOPI. It is a privilege to follow Bianca Pizzera who did so much to set IPOPI on course to being an even more credible and serious global association of national patient organisations. It has also been a privilege to serve as Chairperson of a Board that truly represents the needs of people with PID from around the world. Our role is to serve the needs of our national member organisations (NMOs); improve the quality of life for people with PID worldwide and to assist their patient groups in whatever ways possible. To have spent time in different regions around the world has been truly amazing and we look forward to expanding our network of NMOs as a result.

Much remains to be done and we are grateful to all our stakeholders for the very vital role they play in enabling us to move forward.

Jose Drabwell
Chairperson





Personnel

TRUSTEES AND OFFICERS

The Trustees are appointed by an open and democratic process. Nominations are invited in advance of the biennial meeting and an election is held on the basis of nominations received.

In 2008 in 's Hertogenbosch the following Trustees were elected or appointed alternate members (AM) under our new Rules of Procedure:



Jose Drabwell - UK
Chairperson



Dr. Teresa Espanol - Spain
Chair
Medical Advisory Panel

Stephen Baxter - *New Zealand*

Martine Pergent - *France*

Sven Fandrup - *Denmark*
Vice Chairperson / Honorary Treasurer

Joy Rosario - *South Africa*

Marcia Boyle - *IDF, USA*

Roberta Pena - *Argentina*

Vicki Modell - *JMF, USA*
(AM)

Dragana Koruga - *Serbia*
(AM)

Honorary Life President
Bob LeBien

Honorary Vice-Presidents:
Professor Helen Chapel
Bianca Pizzera

Past Chairpersons of IPOPI
Bob LeBien
Martin Gatehouse
Louise Carroll
Kees Waas
Bianca Pizzera

Executive Director
David Watters

Personal Assistant
Carol Tavener

Johan Prevot (from June 2010)
Director Global Development

Bankers
Bank of Scotland plc
London

Panel Members

Professor Alain Fischer - *France*

Professor Luigi Notarangelo - *USA/Italy*

Dr. Ewa Bernatowska - *Poland*

Professor Amos Etzioni - *Israel*

Dr. Matthew Helbert - *United Kingdom*

Professor Andrew Cant - *United Kingdom*

Professor Alessandro Plebani - *Italy*

Dr. Kathleen Sullivan - *USA*

Professor Ashgar Aghamohammadi - *IRAN*

Dr. Monika Esser - *South Africa*

Professor Surjit Singh - *India*

Dr. Rohan Ameratunga - *New Zealand*

Dr. Mathias Oleastro - *Argentina*

Nurse Nina Berger Hustad - *Norway*

By way of background information...

WHAT ARE PRIMARY IMMUNODEFICIENCIES

Primary immunodeficiencies (PIDs) are genetic diseases. They are a diverse group of more than 200 immune disorders, many of which result from multi or single gene defects. The defects may affect one or more components of the immune system and lead to a characteristic increased susceptibility to recurrent and persistent infections.

When primary immunodeficiencies are left undiagnosed or misdiagnosed, the immune system remains compromised, often leading to illness, disability, permanent organ damage or even death. PIDs are chronic diseases but with early diagnosis and adequate treatment most people with a primary immunodeficiency can live a near normal life. Primary immunodeficiencies can appear at any age. Some forms of primary immunodeficiency have a greater risk of developing certain types of cancer.

It should be recognised that primary immunodeficiencies do not refer to “one” disease, but cover a spectrum of over 200 recognised diseases which have immune deficiency as the common denominator (and more as new primary immunodeficiencies are officially defined each year). IPOPI is therefore concerned with a range of related diseases – and should not be regarded as narrow in scope, nor one-disease specific.

Some 60% of primary immunodeficiencies can be diagnosed with a simple and inexpensive blood test.

The remainder of the primary immunodeficiencies need to be diagnosed by specialists with profound knowledge of the disorders. Treatments include antibiotics, antibody (immunoglobulin) replacement, cytokines, and, for the most severe, bone marrow or stem cell transplantation and now gene therapy.

Diagnosis is a very real problem, especially with ‘late-onset’ conditions, which family doctors appear to find hard to recognise and diagnose. This is a major finding of our Consensus Conference – visit the website at www.eupidconference.com.



2008 - 2009 Years of Action

A SHORT HISTORY

IPOPI was formed following a meeting between Bob LeBien from the US and Dr Helen Chapel from the UK in a London hotel in 1988. In 1990 a meeting in Oxford, UK, of the then existing national patient organisations representing people with primary immunodeficiencies, decided that an international organisation could well benefit people with primary immunodeficiencies around the world. At that time, not so long ago, there were thirteen organisations. Since then, through meetings in Switzerland, Spain, Sweden, Greece, Germany, France, Hungary and The Netherlands, the number of member organisations has more than doubled.

Everything that IPOPI does is driven by its Strategic Plan. This plan is based on solid research with all stakeholders – national member organisations, clinicians, nursing staff, industry, regulators and funders, and is revised on a regular basis.

In 2004 IPOPI enjoyed the privilege of addressing issues concerning primary immunodeficiency at the Scientific Technology Options Assessment Committee of the European Parliament. This



raised the profile of the primary immunodeficiencies within the European political framework and provided a stepping stone that helped to secure European Commission funding for a European Consensus Conference on the diagnosis and management of primary immunodeficiencies held at the Paul Ehrlich Institute in Langen, Germany, in June 2006. The outcomes can be viewed on line at www.eupidconference.com. The Consensus Conference produced outcomes that are replicable in many countries throughout the world and provide a model for similar activities in other regions and nations.

In 2006 IPOPI spearheaded a successful action with the International Union of Immunological Societies (IUIS) that saw immunoglobulins (Ig) restored to the World Health Organisations Essential Medicines List (WHO EML). This is the only known restoration of a medication to the EML.



ACTIVITIES

In 2008 IPOPI met in 's Hertogenbosch in the Netherlands for the regular biennial meeting. At this meeting Bianca Pizzera (Italy) stood down as Chairperson and Jose Drabwell (UK) was elected in her place. As a reward for her outstanding service as Chairperson, Mrs Pizzera was appointed as an Honorary Vice President of IPOPI. At the formal AGM we were delighted to welcome Cyprus as a new national member organisation.

In 2008 we re-introduced the NMO Support Programme which allowed national member organisations with initiatives to help their members, or to increase awareness, to apply to IPOPI for a grant to enable the idea to become a reality. Grants have been made to Argentina, Cyprus, Morocco and Serbia.

We also introduced a new Twinning Programme and so far we have a new twinning agreement between France and Morocco.

There can be no doubt that the biggest single event of the years 2008 and 2009 was the Global

Leaders Meeting held in London in late October 2009. This meeting brought together 52 people, representing leading immunologists, nurses, those representing the 'for profit' and 'not for profit' sectors of the plasma industry; The European Medicines Agency; US Food and Drug Agency; the World Health Organisation and patients, to address issues of major concern. This led to the production of a set of conclusions and actions which will be developed over the coming two years. This was the first time such an international meeting had been held and we hope to repeat the event in 2011. IPOPI is indebted to Baxter Healthcare for generous funding that made this event possible.



The safety and affordability of plasma products are kept under constant review by our Plasma Products Supply, Safety and Affordability Committee (PPSSAC). While attention was paid to the incidence of vCJD and Influenza A – (H1-N1) (swine flu) - there were no major threats to the supply, safety or affordability of immunoglobulins during this period. Looking ahead we do see potential threats arising from new uses in managing conditions such as Alzheimer's Disease and we remain concerned that those who promote this research should remain aware of the problems in scaling up supply to meet demand.



While we have concerns for many of our NMOs – especially in the emerging countries - India currently gives a high level of concern. The cost of immunoglobulin has escalated at an unbelievable rate although at the time of writing there appears to be more affordable Ig coming onto the Indian market. Through the generosity and thoughtfulness of a member of the PiA in the UK we were able to provide a small quantity of subcutaneous Ig from the UK with a doctor in India. However, as our global knowledge increases, so to does our global concerns.



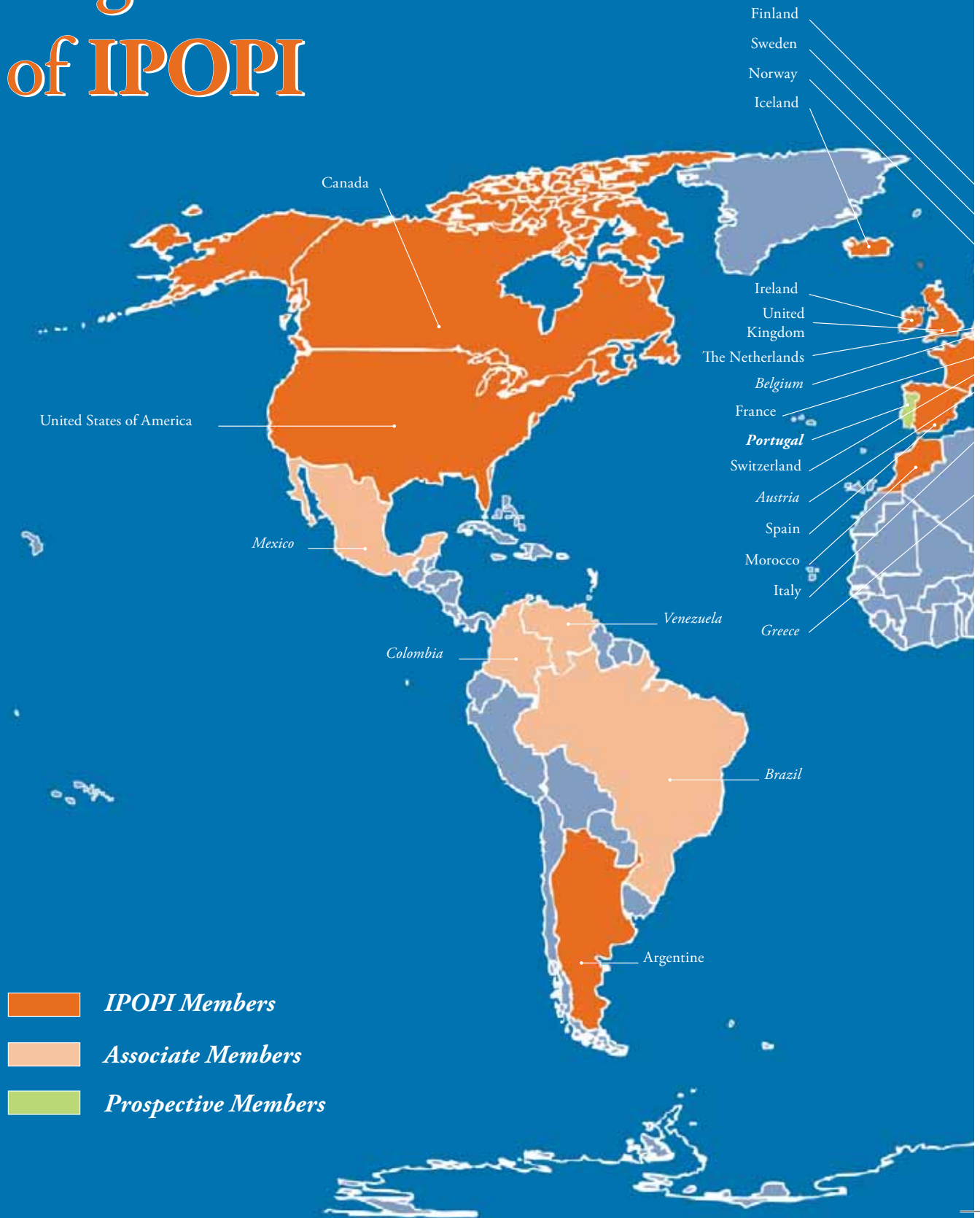
When we meet in Conference in Istanbul in October 2010 we will welcome Austria, Belgium, Brazil, Colombia, Greece, Japan, Mexico and potentially more new members, bringing our total membership to at least 35 countries.

GLOBAL ACTIVITIES

IPOPI continued as a committed member of the World Health Organisation's Global Collaboration on Blood Safety and as a member of the European Medicines Agency's Patient and Consumer Working Party. As appropriate, representations were made concerning the safety and availability of plasma products at European and global levels. As part of our commitment we were founding members of PLUS – The Plasma Users Platform -where many organisations representing people who rely on plasma products meet and campaign as necessary on issues of mutual concern. One of our most effective outreach tools is our website – www.ipopi.org – which is currently achieving over 250,000 hits a month from all the corners of the earth. It is well worth a visit!

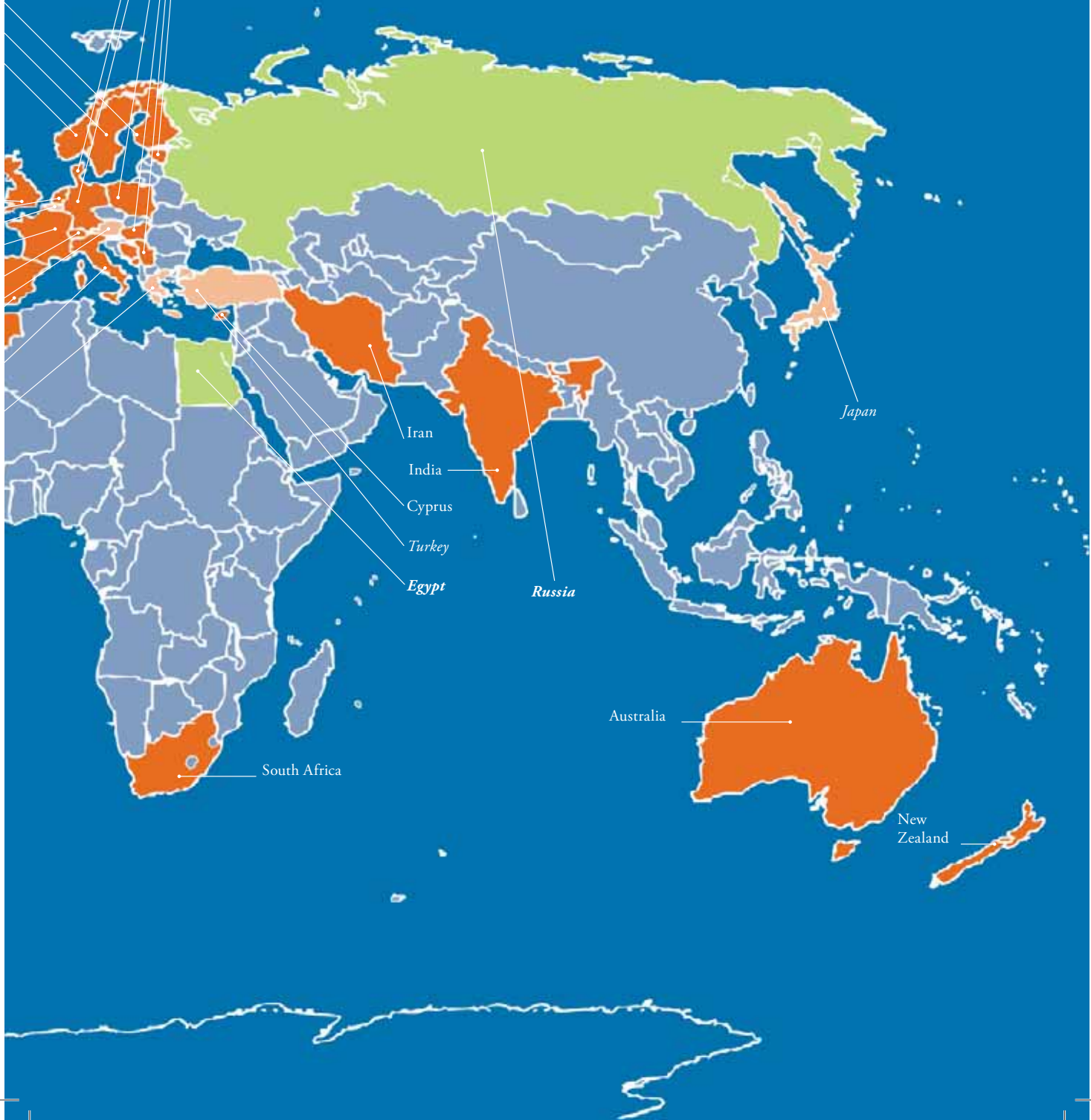
In May 2008 IPOPI carried out a multi-site visit to South Africa through the good offices of our NMO in Southern Africa and we are indebted to Dr Monika Esser and Joy Rosario for all their

National Member Organisations of IPOPI





Denmark
Germany
Poland
Hungary
Serbia - Montenegro
Estonia



Iran
India
Cyprus
Turkey
Egypt

Russia

Japan

Australia

South Africa

New Zealand

work and collaboration in setting up meetings with patients, consultants and registrars in Cape Town, Durban and Johannesburg.

In 2009 IPOPI visited Latin America and played a very full part in the LASID meeting, held in Colombia. We are grateful to Roberta Pena from our NMO in Argentina for all her work to organise things at a local level and acknowledge the valuable contribution of Dr Jose Franco. We already have potential new national member organisations in Latin America as a result of this visit.

Also in 2009, IPOPI participated in the Immune Deficiency Foundation's national meeting in Orlando, Florida. Stephen Baxter and David Watters made a joint presentation there.



We already benefit from a regional structure in the Pacific Rim through the good offices of our national member organisation (NMO) in New Zealand and Stephen Baxter in particular who have facilitated the development of our new member organisation in Australia. It is hoped that this work might be expanded into South East Asia where we have contacts in Japan and China.

In Northern Africa IPOPI has played a role in Morocco taking part in the first meeting of ASID, the African Society for Immunodeficiency and through that is also in close contact with doctors and patients, mainly along the Mediterranean coastline and in the French speaking countries. In this we have been greatly assisted by our NMO from France, IRIS and Board member Martine Pergent and we are grateful to Dr Aziz Bouzfiha and our national patient organisation, Hajar, who work tirelessly for people with PID in this continent.



In Europe and North America we have effective regional structures and within Europe we have a Nordic Regional organisation. So we will continue to develop those partnerships that we hope will benefit people who are yet undiagnosed with a primary immunodeficiency. IPOPI is also closely involved in Eastern European countries through collaboration with Professor Laszlo Marodi and the J-Project.



IPOPI actively participated in two policy meetings organized through PLUS at the European Parliament in Brussels, Belgium. As a result of these successful meetings, a European Union call for action focusing on patient access to plasma protein therapies was launched and it was recommended to create a European Parliament Interest Group on Rare Plasma Disorders.

IPOPI was also involved in an advocacy campaign on several key EU Health Policy dossiers pertaining to the rare diseases communication and recommendation papers, the pharma package (patient information), cross border healthcare proposed Directive and last but not least, the upcoming Blood Directive report on voluntary donations.

In 2008 we finalised production and began distribution of our new booklets on the following conditions - Common Variable Immunodeficiency; Wiskott Aldrich Syndrome; Severe Combined Immunodeficiency; Hyper IgM Disorder; X-linked agammaglobulinemia and Chronic Granulomatous Disorder. Those were published in English, French, German, Spanish, Portuguese and Russian and they have been distributed widely throughout the world at no cost to NMOs and individuals. IPOPI remains grateful to the Immune Deficiency Foundation for enabling the use

of the material in this manner and to CSL Behring whose funding enabled the printing and distribution of the booklets. Reports also inform us that the booklets have now been further translated into Greek and Arabic.

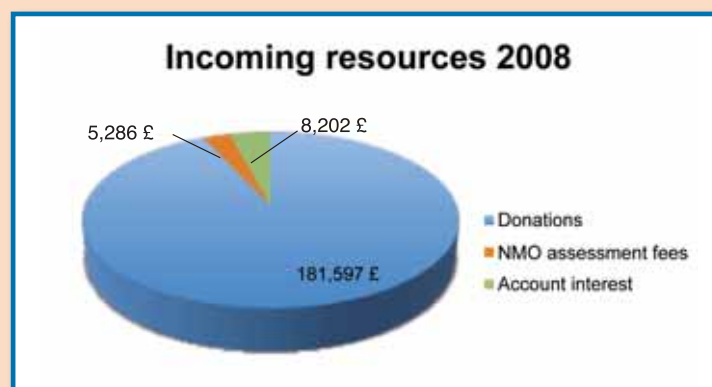
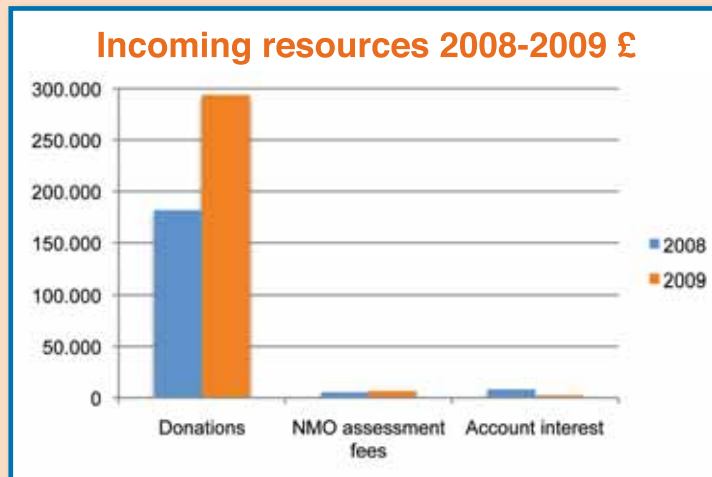
ISSUES

There can be no doubt that the biggest single issue facing IPOPI is the diagnosis and management of PID throughout the world. Without awareness in the medical community in particular, diagnosis will simply not be made. However, a close second is the availability of an adequate supply of safe and affordable immunoglobulins. The countries of the developed world are over-reliant on the US to collect plasma and fractionate into immunoglobulins (Ig) and other vital plasma derivatives. This situation is not helped by those who seek to immediately ban imported products to - as an example - Europe. Europe still relies on the US for some 70% of its plasma products. While there is a distant dream of self-sufficiency in those products - often caused by an inability to understand the difference between blood and blood components and plasma products - it should nonetheless be used as a spur to increase production through wise and prudent investment.

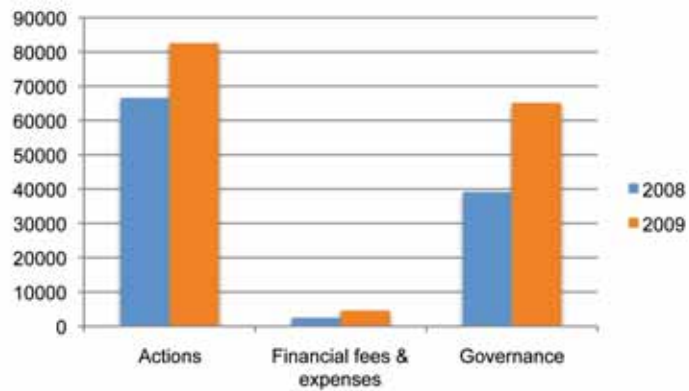
One fact is very certain: the demand for plasma-derived immunoglobulins is not going to decrease and, if anything, our small community is extremely vulnerable in the face of growing demand from other health sectors with a bigger and louder political voice. We need to be equipped with the facts that will enable us to secure future supplies of Ig for our people. We rely on PLUS and PPSSAC to maintain a vigilant watch. One of the very greatest threats arises from alternative uses for Ig which are, naturally, under constant exploration by industry: our hope is that they also have a contingency plan to match the increased demand with increased productivity.



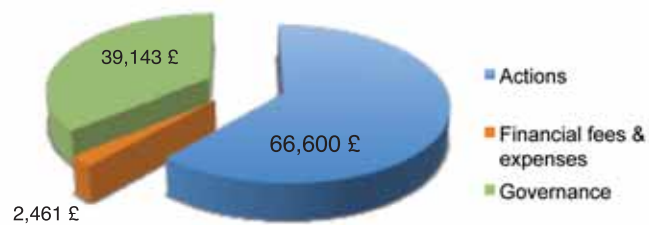
IPOPI is a charity registered in the United Kingdom and under UK charity law we are required to have our accounts audited annually by qualified accountants. Copies of the full accounts are available on request but here we represent graphically some aspects of our accounts:



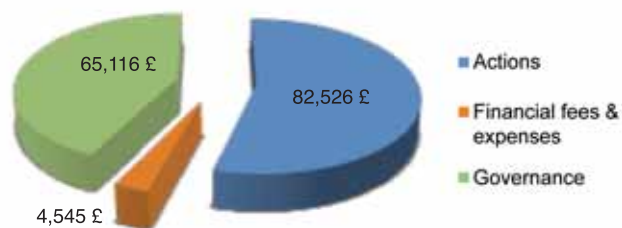
Resources expended 2008-2009 £



Ressources expended 2008



Ressources expended 2009



The Way Ahead

‘Where there is no vision, the people perish’ – so it is that IPOPI is an organisation of vision and an organisation where strategic long-term planning is paramount.

IPOPI has a Strategic Plan that helps us to budget and work within our limited resources. Our plan is not based on the vision of a few, but a vision explored and shared with all our stakeholders around the world. From this exploration it became very clear that the most vital issue is that of well equipped national member organisations which can effectively represent the patients in their country and ensure there is sufficient knowledge to achieve speedy diagnosis and the political impetus to allow spending on apparently expensive plasma derived products.

Our aim by whatever, and however many, means is to ensure that diagnosis and appropriate therapy is available to everyone in the world. This demands of all parties a creative and imaginative to raising awareness in medical schools, with family doctors, with consultants in the many medical specialities that approach the symptoms of primary immunodeficiency, with nurses, doctors and within families. We know of people who have been sent towards diagnosis by the description of PID inside the Christmas Card of a national member organisation; we know of people who were sent on the road to diagnosis by chats over a cup of coffee – but we need much more than that – we need the sensible infrastructure that leads doctors to think early that an underlying defect of the immune system might be the cause of illness that, for example, does not respond to repeat doses of antibiotics. From the IDF Conference in 2009 we learned the medical school phrase ‘where you hear hoof-beats, think zebra, not horses!’ – we need doctors to start thinking underlying causes and not simply treating the symptoms!

We want to see health authorities stopping the simple treatment of symptoms rather than the powerful – life-changing – knowledge that there are underlying causes of illness. Without that knowledge they will continue to effectively pour money into a drain and still have patients who are dependent, unproductive and very costly – contributing nothing to their national economy or family life.

This is why early diagnosis and effective treatment of individuals with primary immunodeficiencies is imperative – this is why IPOPI is important and this is why our global mission compels us to continue our important work.



The Strategic Plan 2010 - 2015

Our vision

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

OBJECTIVE 1

WORK TO ACHIEVE OPTIMAL COOPERATION WITH ALL STAKEHOLDERS TO SECURE PATIENTS' INTERESTS IN DECISION MAKING

Action 1: Plan the next global leaders meeting in 2011 involving all stakeholders

Action 2: Promote and enable NMOs to benefit from the successful outcomes of previous conferences

Action 3: Compile global data on prevalence of Primary Immunodeficiencies by collection of relevant data from NMOs

Action 4: Update the Global Ig List of plasma derived immunoglobulins whenever new information occurs.

Action 5: Represent the interests of the primary immunodeficiency community internationally.

Action 6: Monitor the safety, supply and availability of plasma products used by PID patients.

Action 7: Monitor advances in therapies and care

Action 8: Monitor health threats to the PID community

OBJECTIVE 2

PROMOTE THE ESTABLISHMENT AND SUPPORT OF NMOs

Action 1: Establish NMOs

Action 2: Promote regional meetings to increase awareness of PIDs

Action 3: Provide assistance to NMOs and Associate Members

Action 4: Provide information to NMOs

Action 5: Encourage the exchange of information and experience.

OBJECTIVE 3

PROVIDE EFFICIENT GOVERNANCE TO MAINTAIN A STRONG, WELL RESOURCED, DEVELOPING ORGANISATION

Action 1: Monitor the operational procedures of IPOPI

Action 2: Provision of financial resources by fundraising

Action 3: Organise successful biennial conferences

Action 4: Provide resources and staff required

Action 5: Maintain and adapt the website

Action 6: Explore a mechanism for the more active involvement of NMOs



IPOPI express their gratitude to the following pharmaceutical companies for their support through unrestricted educational grants;

Baxter Healthcare SA

Biotest Pharmaceuticals

CSL Behring

Grifols

Octapharma

Talecris Biotherapeutics

We also wish to thank our national member organisations who all contribute so much to our work and especially to those who are able to pay their annual assessments.

Without the generous help of all those listed, the quality of life for patients with a primary immunodeficiency would be the poorer.

IPOPI is a Charity registered in the UK, registration number 1058005

www.tipolito-ala.it

GRIFOLS

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INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

Ipopi 2010 Budget

OBJECTIVE 1:

WORK TO ACHIEVE OPTIMAL COOPERATION WITH ALL STAKEHOLDERS TO SECURE PATIENTS' INTERESTS IN DECISION MAKING

€ 40,500

1:	Plan the next Global Leaders Meeting in 2011 involving all stakeholders	5,500
2:	Promote and enable NMOs to benefit from the successful outcomes of previous conferences	6,000
3:	Compile global data on prevalence of Primary Immunodeficiencies by collection of relevant data from NMOs	7,500
4:	Update the Global Ig List of plasma derived immunoglobulins whenever new information occurs	1,000
5:	Represent the interests of the primary immunodeficiency community internationally	16,500
6:	Monitor the safety, supply and availability of plasma products used by PID patients	2,000
7:	Monitor advances in therapies and care	2,000
8:	Monitor health threats to the PID community	-

OBJECTIVE 2:

PROMOTE THE ESTABLISHMENT AND SUPPORT OF NMOs

€149,500

1:	Establish NMOs	-
2:	Promote regional meetings to increase awareness of PIDs	€ 37,500
3:	Provide assistance to NMOs and Associate Members	81,000
4:	Provide information to NMOs	31,000
5:	Encourage the exchange of information and experience	-

OBJECTIVE 3:

PROVIDE EFFICIENT GOVERNANCE TO MAINTAIN A STRONG, WELL RESOURCED DEVELOPING ORGANIZATION

€209,500

1:	Monitor operational procedures of IPOPI	5,000
2:	Provision of financial resources by fundraising	5,000
3:	Organize successful biennial conferences	4,500
4:	Provide resources and staff required	190,000
5:	Maintain and adapt the website	5,000
6:	Explore a mechanism for the more active involvement of NMOs	-

TOTAL PROJECTED EXPENDITURE

€399,500

PROJECTED INCOME

Core partner funding	€360,000
Directed partner funding	30,000
Other income (not for profits etc)	10,000
Assessments from NMOs	7,000

TOTAL

€407,000