

International Patient Organisation for Primary Immunodeficiencies



STRATEGIC PLAN 2010 – 2015

July 2009

IPOPI, Firside, Main Road, Donderry, PL11 3LE, UK.
Tel & Fax : (+44) 01503 250 668- Mobile (+44) 077 36 36 6137
Contact : David Watters, Executive Director, info@ipopi.org
Website : www.ipopi.org

IPOPI is a charity registered in the UK – Registered Charity No 1058005



Introduction

Planning plays a key role in the development and success of any organization. It helps to achieve results, monitor progress, maintain control, give people responsibilities, strengthen accountability and build greater teamwork through a sense of involvement.

An organization may have a utopian vision or an unrealistic dream of what it would like to achieve but without a step-by-step plan on how to reach these goals, this vision would certainly remain just a dream.

In 2004 IPOPI commenced a new phase in its planning by asking all stakeholders to contribute to an electronically distributed questionnaire. Results were analysed and at a meeting of the Board in 2005, a brand new Strategic Plan was implemented. Taking into account the ever increasing pressures on the world of primary immuno-deficiencies, it was decided to review the Strategic Plan on an annual basis. These reviews took place in 2006, 2007 and 2008. This year, 2009, the board decided that it was time to consult our stakeholders again. The response was immense and I would like to express my thanks to everyone, National Member Organisations (NMOs), doctors, nurses and industry, who took the time to let us have their many ideas and suggestions.

This Strategic Plan covers the period from 2010 - 2015 and naturally will be updated on a regular basis, especially as new and challenging opportunities present themselves. It will continue to be a struggle to overcome differences in national, regional and cultural aspects, but the world of primary immunodeficiencies is becoming smaller as the number of patients who are diagnosed increases, and patient organisations multiply across the world.

We hope that with all your support, enthusiasm and commitment, the Strategic Plan 2010 - 2015 and its objectives will be achievable, and this in turn will benefit all people with a primary immunodeficiency across the world.

Jose Drabwell
Chairperson

The context of primary immunodeficiency

The conditions and under-diagnosis

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 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 antibody (immunoglobulin) replacement, cytokines, bone marrow or stem cell transplantation and, for the most severe, gene therapy.

The majority of symptomatic and treatable primary immunodeficiencies - collectively affecting an estimated at 1:10,000 - are classified as rare diseases. However experts* estimate that 70 - 90% of Primary immunodeficiencies remain undiagnosed, and incidence could be as high as 1:500** [**Prof. L. Notorangelo and **Prof. L. Hammarstrom presenting to European Parliament Scientific and Technological Assessment Unit, March 2004*]. If current scientific evidence is correct about the estimated prevalence of primary immunodeficiencies, early and accurate diagnosis of those conditions is essential. US studies from 2007 suggest prevalence rates of 1:1200 of the population. [*J Clin Immunol (2007) 27:297-502 - J M Boyle - R H Buckley*]

In undiagnosed or misdiagnosed sufferers, treatments are focused on the frequent and long term use of high dose antibiotics. Improved diagnosis of primary immunodeficiencies could therefore have a positive impact on the reduction in use of antibiotics, better use of health facilities and an improvement in the quality of life for the individual.

Immunoglobulin supply

It is important that primary immunodeficiencies are recognised as a public health issue so that there is greater awareness of the causes of the presenting symptoms and thereby diagnosis of the underlying conditions rather than the simple treatment of the symptoms themselves. With this in view IPOPI sponsored a successful EU Consensus Conference in 2006 with financial support from the European Commission. The outcomes are available at www.eupidconference.com.

Several therapies are available for the treatment of primary immunodeficiencies. The vast majority of diagnosed conditions are treatable with plasma products - especially immunoglobulins or C1-INH. It should be noted that those therapies neither cure the conditions nor do they reverse the damage brought about by late diagnosis.

The plasma product market place has been fraught with problems in the past - for example hepatitis C in PID and HIV in haemophilia were transmitted through plasma products - but there has been no recent history of such problems due to the exhaustive viral control measures implemented during the manufacturing process of plasma derivatives.

Although efficient removal of prions (responsible for vCJD) during the manufacturing processes of plasma derivatives has been well documented, concerns remain about the active presence of vCJD in UK blood products in the 1980s and any implications that might have for those communities around the world who received UK products. We are also sensitive to potential issues arising from the H1N1 pandemic and the threat this could pose to our immunologically compromised patient population.

The market is vulnerable however and the need to achieve a balanced supply of product to meet growing demand can lead to acute product shortages and at times this can achieve life-threatening proportions. It has to be appreciated that the costs involved in meeting the high standards in plasma collection and manufacturing, to ensure safe end products, are high but in the best interests of the public health. Standards are demanded by regulatory authorities as well as industry itself. It is also salutary to note that the blood product industry is the only industry that relies on the human body for its source product - i.e. human plasma - as opposed to the raw materials used in other areas of medicine.

In 2006/7 IPOPI and the International Union of Immunological Societies (IUIS) worked in collaboration to make sure that immunoglobulins were restored to the World Health Organisation's (WHO) Essential Medicines List (EML). In 2003 the EML Committee had decided to remove immunoglobulin and an unsuccessful approach had been made in 2005. With much hard work the 2007 meeting supported our view and our collaborative work led to a successful outcome. We see it as essential for poorer countries that immunoglobulin is listed in the EML as an incentive to make provision for those who will benefit from the therapy. IPOPI is also a member of the WHO Global Collaboration on Blood Safety.

In 2009 IPOPI is sponsoring a major 'summit' meeting of global leaders in the arena of primary immunodeficiencies with a view to achieving a better understanding of organisational goals and priorities. It is vital that we know what we all see as our role in the campaign to improve diagnosis and access to therapies.

In 2010 we will appoint a global Director of Development. This post will concentrate on opening up new national member organisations throughout the world and equipping the leadership of those organisations to understand health care systems and campaign for improved diagnosis of PID and access to the life-changing and life-saving therapies that have proven to be invaluable in so many countries.

IPOPI works closely with other plasma user groups and shares platforms with groups representing, for example, haemophilia, alpha-1 anti-trypsin, CIDP, etc and during 2008 became a founder member of the Plasma Users Platform - PLUS. Initial work with this group is Europe-based but the services are available on a global basis.

The way ahead

The way ahead is one paved with opportunity for closer co-operation and working between the various stakeholders involved with primary immunodeficiencies. As with many treatable long-term medical conditions there are close links between individual patients and their doctors and nurses. This is also the case with the local, regional and international bodies concerned with the management and diagnosis of the conditions and every two years the international patient group, the European clinical immunologists group and the international nurses group meet in conference together. Industry also plays a close role in listening to and offering unfettered support to the groups as it is able. IPOPI believes that it has a crucial role to play in managing future opportunities to develop a better global future for people with primary immunodeficiencies. This may be through evolving national centres of excellence, continuing political dialogue, encouraging international research and closer levels of co-operation between stakeholders at all levels involved with patient care.

IPOPI has been closely involved with developments in regions around the world. The European Union forms a unique focus for much of our work due to the disparate nature of the 'union' which is not a union at all levels. We have spent time in southern Africa; working with ASID - the African Society for Immune Deficiency - initially through their meeting in Morocco; we are putting resources into the development of new national member organisations in Latin America through our work with the Latin American Society for Immune Deficiency (LASID) as well as continuing our work of encouragement in India and working to opening new opportunities in Japan, China and South East Asia.

It is with all this in mind that IPOPI is continuously involved in developing a plan for the future which unfolds in the pages ahead. During the planning stages we were concerned to know how our stakeholders saw the future - how to best improve all aspects of life for people with

primary immunodeficiency throughout the world. It rapidly became clear that the route lay in the establishment of strong, effective, national patient organisations that were equipped for the task of communicating effectively within their own countries. Thus, IPOPI sees this as its main priority - helping to establish national patient organisations in viable countries around the globe that will be able to inform and advise those who guide health care in each country. In order to do this IPOPI itself must be a strong organisation that acts with one voice and is equipped to provide the tools that will be needed in different countries to convey the important message, that primary immunodeficiencies matter. Indeed our researches for this new edition of our Strategic Plan showed beyond doubt that the greatest need is that of resourcing our work so that we have the personnel available to implement our desire for a global improvement in the quality of life for people with primary immunodeficiencies around the world.

Unless the primary immunodeficiencies matter to the health authorities they will continue to spend vast amounts of money treating symptoms rather than underlying causes; they will continue to have sick and reliant members of society who could, with appropriate diagnosis and management, become productive members of society; and they will continue to see people die young from unexplained illnesses.

That is what primary immunodeficiencies are about; that is why primary immunodeficiencies are an important public health issue and that is why IPOPI has developed this strategic plan ***so that we have a united approach to the future.***

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.

Objective1:

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