



# UPDATE

Winter 2001



**Kees Waas**

My colleagues on the Board of IPOPI join me in sending good wishes to our friends throughout the world. As we celebrate many different faith festivals at this time of the year, we add our word of good will for the peace and health of the world. May the year 2002 bring to you and your family and friends better health, better diagnosis and better treatment.

2001 has been a busy time for IPOPI. Following the Congress in Geneva we were able to launch our International Development Programme – and elsewhere in Update you will read how that has already helped to establish a national patient organisation in South Africa. Our wish is that many potential or existing national member organisations (NMOs) of IPOPI will think creatively about making applications for funds to help achieve new and better things for members across the world. Thanks to the work of Fred Modell and Tom Moran and the generosity of industry, we have those funds to help develop NMOs. Let's make

sure we spend every penny in the coming year.

We have also been working on improving the IPOPI web-site to make it even better. The site receives hundreds of visitors every day and it is important that we keep it alive with new information. If you have not visited it for a while, do it today – the address is elsewhere in this publication!

And we continue to look at ways of bringing immunoglobulins to crisis areas of the world. Once this was very easy when there were plentiful supplies of Ig available. This is no longer the case and we are having to look at more constructive ways of enabling countries to produce safe product on a national basis.

The Board had hoped to meet in relaxed surroundings in Italy in the autumn but due to the terrorist attacks in the USA we decided to postpone our meeting in line with the arrangements made for the parallel European meeting on PPTA and EPFA. We are grateful to our friends at Kedrion in Italy for their generous offer of hospitality at their centre in Tuscany for our own private conference and regret that we were unable to take advantage of it this time.

I cannot end without expressing our understanding, sympathy and love to our friends in the USA and most especially to Marcia Boyle and Vicki Modell and everyone associated with their patient organisations. There were so many expressions of support buzzing through the e-mail systems in the days and weeks following the tragic loss of so many – and our love and support is still there.

With good wishes from us all!

**Kees Waas**  
Chairperson



*I am delighted to introduce Teresa as the next Chairman of the Medical Advisory panel of IPOPI. She is an experienced paediatrician who was introduced to paediatric immunology and primary immunodeficiency when she was a research fellow at the Institute of Child Health in London in the 1970's. It was clear then that Teresa was an enthusiast for primary immunodeficiency and had made up her mind exactly how her career would go within immunology, even at that time.*

*I remember meeting Teresa in London with Roland Levinsky; we were all attending the usual high-spirited party that accompanied the British Society for Immunology meetings. (Dick Gatti was probably playing the piano and we were dancing like lunatics!). Since that time meetings with Teresa have continued to be fun and I am sure she will bring to the Medical Advisory Panel not only her tremendous knowledge about primary immunodeficiencies but also her great enthusiasm.*

*Teresa's immunological interests have continued to be impressive. She is interested in all of the primary immunodeficiencies and continues to publish extensively on a wide range of diseases and therapies. She supervises many post-graduate students as well as being involved in collaborative research across most of the countries of Europe. She is ideally placed to provide advice to the executives of IPOPI and I know she will do an excellent job.*

**Helen Chapel**

## IDF National Conference in Baltimore, USA

Between June 21 and June 23, 2001, more than 1000 individuals came to Baltimore, Maryland to participate in the inaugural National Conference of the Immune Deficiency Foundation. Patients, families, health care professionals and representatives of industry and allied health organizations gathered to learn more about the primary immune deficiency diseases, to interact with others who share the same concerns, and to make new friends. The conference included youth programmes, with both educational and recreational activities, including a trip to

see a baseball game with the Baltimore Orioles.

The conference began on Thursday, June 21, with nearly 300 patients and families visiting with their Senators and Congressmen on Capitol Hill in Washington, D.C. to discuss issues affecting care and research for primary immune deficiency diseases. On Friday, June 22, during the opening session, Tom Moran, the IDF President, and Dr.

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Richard Barr, Chairman of the Board of Trustees, greeted the participants. Marcia Boyle, the founder of IDF, gave an overview of the Foundation and its activities, and Carol Ann Demaret, the mother of David, "the boy in the bubble," gave a poignant keynote address on the impact of David's life. Jerry Winkelstein, M.D., the chairman of the IDF Medical Advisory Committee, presented the Scientific Keynote Address.

This session was followed by concurrent scientific sessions given by members of the IDF Medical Advisory Committee and other professional leaders on IGIV Therapy, Genetic Testing, Bone Marrow Transplantation and Gene Therapy, followed by concurrent sessions on Specific Diagnosis. That evening, all participants participated in an exciting Extravaganza at the National Aquarium in Baltimore.

On Saturday, June 22, sessions included Alternative Methods of Delivery of Gamma

Globulin, a general session on new Health Legislation, followed by a series of concurrent Life Management Sessions. The conference culminated with the IDF 20th Anniversary Celebration awards banquet, during which Mary Ellen Conley, M.D. received the annual IDF Scientific Achievement Award, named in honour of the Boyle Family. Marcia Boyle, John Boyle and Jerry Winkelstein, M.D. were recognized by IDF with awards for founding IDF and for 20 years of dedication and service.

All who participated in the conference returned home energized and committed to increased involvement and advocacy on behalf of the primary immune deficiency diseases. We look forward to seeing even more people at our next national conference in 2003. IDF is most appreciative of the conference's Platinum, Gold and Silver sponsors, who helped make this special event a reality.

## International Meeting of Parents and Children with Immunodeficiencies in Berlin July 27th – 29th, 2001

After months of intensive preparation the first international meeting for parents and children with immunodeficiencies was held in Berlin at the end of July this year. The event took place in the Jugendheim Fuchsbau, a community centre for young people, in the Reinickendorf district of Berlin.

After registration, and receiving the conference material, all the attendees met informally in the garden of the centre enjoying the magnificent Berlin summer sunshine and delicious home made cakes! This was followed by a formal welcome from our Chairman Ulf Schmidt. Then the Mayor of Reinickendorf, Mrs Marlies Wanjura spoke to us and promised to support our interests in the future.

The children of most of the parents at the meeting are CGD-patients. Nevertheless, all the families from Poland, Netherlands, Sweden and Germany communicated with each other freely and easily. At the end of the day there was a lovely buffet supper at the Hotel Igel which was also attended by Dr Markus Schmitt, Prof. Dr Ulrich Wahn, Dr Wolfram Ebell and Dr Ilka Schulze. They answered all our questions with much patience and knowledge. All families were looking forward for the next day.

On Saturday we heard lectures about stem cell therapy, BMT and subcutaneous therapy given by the doctors we met the evening before. These lectures were very informative for the parents and we appreciate their interest and input very much indeed. At the

same time most of the children enjoyed themselves at a game of mini golf. Afterwards, we all had lunch in a local restaurant. Then we split up for sightseeing. Some went on a boat trip on the canals of Berlin; others went sightseeing by bus. On Sunday morning we had breakfast together at the Fuchsbau. After the meal most people left for the journey home.

Everybody was very satisfied with the event overall. Our children had a lot of fun together and all of us have got new friends now. It would be very nice if we could have this kind of meeting more often. Perhaps we can do so every second year, between the IPOPI meetings.

We must extend special thanks to Stephan Höhnke, who welcomed our guests from abroad, Margitta Woop who took special care of the younger children and helped them to enjoy themselves, and Mrs. Müller, who played with the older children and brought gifts for everybody. All the children were also given a nice little teddy bear donated by "Good Bears of the World" and other sponsors of the event were the Baxter Company and the Blattl Hotel, who were generous to provide discounted accommodation for our guests. To our friends and sponsors we say a very big 'Thank You' for making our inaugural meeting such a success.

*Carlotta Koss, Christiane Koss, Joachim Schulz, Stefanie Fibich and Gabriele Höhnke*

# Updates on Immunoglobulins Safety and Supply

## 1. Introduction

Coagulation factors, albumin and immunoglobulins have always represented the principal products derived by the fractionation of plasma. The pricing of immunoglobulins, globally, has relied on the shared cost of production of these products. This status is rapidly changing: with the introduction of recombinant factors for the treatment of haemophilia there is a decline in the use of plasma based coagulation factors, and albumin consumption in many countries has dropped markedly, following the publication of the Cochrane study on the side effects. Whilst, with an expanding demand of 20% a year, IVIG is becoming the driving product on the international market.

This means that the reduction in the other two components leads to a direct substantial increase in the cost of immunoglobulins.

## 2. Safety

Hepatitis B (HBV) was a major problem in the 70s, but the development of appropriate HBV screening assays has eliminated transmission of hepatitis B in immunoglobulins.

Bigger concerns arose at the beginning of the 80s, with the transmission of HIV (human immunodeficiency viruses – AIDS) through blood transfusions. In the early 90s, a new very resistant virus, identified as hepatitis C virus (HCV), was responsible for heavy cases of contamination.

Except for HCV, that have infected, with transmission via immunoglobulins, thousands of patients with primary immunodeficiencies all over the world, the more infected by HBV and HIV were the haemophiliacs and the recipients of blood transfusions, whilst the manufacturing procedures of immunoglobulins and albumin, worked as a filter for HBV and HIV, and thus saving "fortunately" those patients from infection.

Currently available immunoglobulins preparations all include well-validated viral inactivation procedures and the new technologies and manufacturing processes are considered to have significant capacity to eliminate transmissible agents.

(There has been no transmission of HBV, HCV, or HIV by US licensed plasma derivatives since the introduction of effective virus-inactivation procedures –1996-).

Nowadays Ig is safe for all known viral infections, however it is important to keep in mind that standards of productions and quality assurance assays must be maintained: ALERT must always be high.

## 3. Availability

In the last few years we are experiencing an increasing use of IVIG for a growth in diagnosis of primary immunodeficiencies as well as an ever-growing number of other conditions for which high dosage is thought to be an appropriate treatment, but without any adequate scientific evidence of efficacy (off-label use).

Licensed indications for IVIG:

- primary immunodeficiencies
- ITP
- Kawasaki Syndrome
- Recent BMT in Adults
- Chronic B-Cell Lymphocytic leukaemia
- Paediatric HIV Infection

Major "Off-label" uses of IVIG :

- Neurological
- Post transfusion Purpura
- Guillan Barre Syndrome
- Asthma
- Bleeding Disorders

- Myasthenia Gravis
- Adult HIV Infection
- Chronic Fatigue Syndrome
- Fertility/Spontaneous Abortion
- Rheumatoid Arthritis

As a consequence the IVIG demand has sensibly increased, while the production response does not appear to be so fast.

We must also consider that there is a decline of raw plasma, the main cause being:

- blood donors are dropping internationally (about 10% only in the USA that supply 50% of European needs)
- withdrawals and losses due to controls and donors and pool screening
- precautionary measures against the potential risk posed by vCJD (FDA policy requiring withdrawals and wastages of product where a donor had a risk factor for classical CJD).

In the United States a severe shortage in IVIG supply was experienced over 1997-98 with dramatic consequences for patients with primary immunodeficiencies: (data from IDF)

- 80% of patients reported problems obtaining IVIG
- Of those patients, 56% reported adverse health effect including:
  - infections and malaise
  - adverse reactions to new brands
  - pneumonias, bronchitis, and lung infections
- Public Health Crisis
- Demand controlled – rationing protocols, prescribing practices
- Prices Increased
- Manufacturer's interest in new IVIG products, i.e. European products – Octapharma (Austria), Grifors (Spain) might obtain FDA license already in 2001 – this can be very dangerous for Europe supplies as the IVIG prices in US are much higher.

Security of IVIG supply is an issue which needs careful attention internationally.

## 4. New Variant CJD

Bovine Spongiform Encephalopathy (BSE) is a slowly progressive and ultimately fatal neurological disorder of adult cattle. Creutzfeldt-Jacob Disease (CJD) is the human spongiform encephalopathy. The cause of CJD is a transmitted protein called a prion. This particle, smaller than a virus, incorporates itself within the cells of the brain and spinal cord leading to their destruction. It does not generate an immune response.

Epidemiological surveillance of Creutzfeldt-Jacob Disease (CJD) was reinstated in the UK in 1990 to identify any changes in the occurrence of this disease after the epidemic of BSE in cattle. Similar Epidemiological studies of CJD have been carried out in France, Germany, Italy and the Netherlands between 1993 and 1995.

Ten cases of CJD had been identified in the UK in 1996 with a new neuropathological profile. The clinical course of disease in the ten patients was distinct from that usually seen in sporadic CJD. Other consistent features that are unusual include the young age of the cases, and the absence of electroencephalogram features typical for CJD.

On 20th March 1996 the UK Government announced a likely connection between BSE and a new variant CJD (vCJD).

The Lancet has recently published a research letter pertaining to BSE and blood. (The Lancet 2000, 356:999-1000) The research conducted indicated the possibility that CJD can be transmitted

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through blood transfusions. The researchers transfused blood from sheep infected with symptom less CJD into non-infected sheep. One of the nineteen sheep who received the transfusion showed signs of the disease after 610 days.

The PPTA has issued a statement in which it notes that although the results are preliminary they should be considered seriously. However, it is important to recognise that there is still no evidence of vCJD transmission through bloods between humans.

Important data that shows the effectiveness of the fractionation process in removing TSE agents was presented at the EMEA workshop in May 2000.

Because of the epidemic of BSE in the UK and more recently in France, Germany, Italy and other countries in Europe, severe preventive measures have been taken:

- In UK, since March 1998, there is a ban on the use of national blood for production of plasma concentrates, including immunoglobulins
- In 1999, a ban on blood transfusions from donors who have spent cumulatively six months in the UK between 1980 and 1996 was imposed in the United States, Canada, New Zealand, followed, in 2000, by Australia, France, Germany, Switzerland and Italy. Thus reducing the availability of plasma for manufacture of immunoglobulins.
- The risk of transmission vCJD from blood products is universally considered "theoretical" and the severe measured adopted "precautionary".

The gravity and poor knowledge of vCJD justify any doubts, but there is the need to make a balance between a "theoretical risk" (safety) and a "certain harm" (insufficient supply) of blood and derived products. What is more dangerous for patients who depend on immunoglobulins for their life?

## 5. Summary and Conclusions.

At present level of understanding Ig is safe for all known viral infections, though being biological sourced material, the zero risk

does not exist.

Since 1996 there have been no new problems of infections thanks to the universal introduction of virus inactivating procedures, however product safety is an important issue, over which IPOPI and all Patient Organisations for Primary Immunodeficiencies must remain vigilant.

Currently, the greatest concerns involve the supply of immunoglobulins. It has been estimated that demand for immunoglobulins will increase by 20% per year, while supply will increase by only 10%.

The major factors which affect the availability of product are:

- Availability of plasma
- Demand for immunoglobulins
- Pricing of immunoglobulins

## 6. Recommendations (for IPOPI)

Appoint a sub-committee, including some MAP members, to work on the main problems such as :

- To dialogue with Regulatory and Government Agencies, Health Departments and Product Manufacturers
- To advocate effective political contacts making
- To encourage increased plasma availability
- To monitor product safety and security of supply
- To encourage clinical trials
- To discourage the use of blood components in therapy unless the benefit is clear
- To encourage partnership between manufacturers patient's representatives and prescribers to improve consistency of information and data evidence relating to risk/benefit analysis for gaining informed consent
- To dialogue with other Groups of Blood users (Haemophilia, Thalassemia, etc.)
- To dialogue with Private and Public Industry Associations (PPTA, EPFA, ABRA etc.)
- To keep a close exchange of information and collaboration among IPOPI NMOs and contact countries.

# A national diagnostic and therapeutic protocol for XLA – news from Italy

A national diagnostic and therapeutic protocol for XLA has begun in Italy. The aim of the project is to give each child with a clinical and immunological phenotype of XLA a definitive diagnosis and to provide all Italian regional hospitals treating these patients a therapeutical protocol based on internationally agreed guidelines. This project has been co-ordinated by the Paediatric Department of the University of Brescia in collaboration with the Italian Association of Paediatric Haematology and Oncology (AIEOP).

Why did we decide to undertake this project nationally? Firstly, because we wanted to give all children with a XLA phenotype the opportunity to have their diagnosis confirmed at the molecular level. Whilst the diagnosis of XLA does not require any further laboratory investigation in the case of a male with hypogammaglobulinemia, absence of circulating B cells and a history

of affected males in other generations, for sporadic cases (which account for as many as 70% of all patients) a definitive diagnosis of XLA can only be made by BTK mutation analysis since mutations in autosomal genes can also cause an XLA-like phenotype. Reaching a definitive diagnosis is essential for genetic counselling, including carrier protection and pre-natal diagnosis. Two qualified laboratories with the necessary skill for BTK gene molecular analysis have been identified, one in Northern and the other in central Italy (Brescia and Rome respectively). Each doctor who wishes to receive molecular confirmation for children with a presumed diagnosis of XLA can send blood samples to referral laboratories through a swift postal delivery system.

The second aim of the proposed protocol is to standardise nationally immunoglobulin substitution therapy, which is the treatment of choice for these patients. This should help

to eliminate the differences observed between separate hospitals regarding dosages and intervals of immunoglobulin substitution therapy and allow patients to achieve protective IgG serum levels. The protocol will also provide practical indications on how to manage the problems associated with immunoglobulin administration, such as prevention and treatment of adverse effects, risk of transmission of viral infections, how to infuse immunoglobulin and the characteristics of commercially available products.

Ultimately this project should enable each patient to receive a final diagnosis and optimum treatment at a local hospital. The realisation of this protocol has been made possible by the active contribution of the Italian Association of Primary Immunodeficiencies (AIP).

# The Australian Primary Immunodeficiency Association

In 1994, Anne Daniel was sponsored by Westmead Hospital in Sydney to attend the International Patient Organisation for Primary Immunodeficiencies (IPOPI) conference in Sitges, Spain. The head of the Department of Immunology, Professor Graeme Stewart had met Dr Helen Chapel from London at another Immunology conference and heard from her of the good work that the Primary Immunodeficiency Association, the British patient support group, and IPOPI, the international group had accomplished. He set about persuading the Westmead Hospital charitable trust fund to sponsor a representative to attend.

As a scientist, and the mother of a newly diagnosed six year old with CVID, Anne was chosen to be that representative. This, then, was her introduction to patient organisations. On returning to Australia she admitted that she really did not have a clue what to do first, but soon found herself thrown in at the deep end! This came about after she handed her report of the IPOPI meeting to two clinical immunologists at Westmead Hospital, Professor Graeme Stewart and Dr Connie Katelaris. The most important item in the report was the information gleaned about the Hepatitis C contamination of various intravenous immunoglobulin (IVIG) products used overseas and what steps were being taken in response to that.

At this stage the committee consisted of Anne and Michael Daniels and Heather Stubing (Green) was the Treasurer. They joined forces with Louise Carroll from the New Zealand Group – Kids with Immunodeficiencies (KIDS) and began to lobby government representatives and interested immunologists. We gave a presentation outlining the consumer's case and requests for extra safety measures to be taken in the manufacture of IntragamI. Eventually an extra viral inactivation step was incorporated into the manufacture of IntragamI.

A flyer was addressed to prospective members, through all the clinical immunologists at the major Sydney hospitals. They were sent to the hospital day wards where the patients received their IntragamI infusions and also to some of the hospital pharmacies where some immunodeficiency patients that so not receive IntragamI obtain other medications. The response to this was slow, but we did receive a few replies. At about this time Anne received a phone call from Kathy Herder who was willing to help.

The first meeting was held at the Daniel's house in March 1995. The main items on the agenda apart from the Hepatitis C/IVIG issue were incorporation, registration, insurance, the constitution and draft application. Our biggest problem was the lack of funds. We decided that a newsletter was the best way of convincing other prospective members to join. Our first newsletter came out in June 1995. We distributed it in the same way as the flyer and gained just a few more members. Slowly, over the next few months our membership grew.

New members were generally newly diagnosed patients. In light of this, and with Dr Katelaris' support, we posted our next newsletter to every clinical immunologist in the country. Dr Katelaris was the president of the Australasian Society of Clinical Immunology and Allergy (ASCIA) and we were able to put a copy of our newsletter in with their own newsletter (a mailing list of over 200) without any extra postage cost. We published five newsletters by the end of 1996 and eventually we had members from all over Australia.

Kathy and Anne were invited to attend the annual ASCIA conference held in Sydney late in 1995. They were able to contribute to the discussion on treatments – giving the patients point of view when there is a choice of treatments, for example, home versus hospital, or subcutaneous infusion versus intravenous infusion. They also contributed to the discussion on safety of intravenous gammaglobulin preparation versus intramuscular gammaglobulin

preparations, with the knowledge gained from our involvement in the IntragamI meetings of the previous year.

We were represented at the next IPOPI conference in Gothenburg in June 1996. IPOPI sponsored the trip. In 1997 Kathy Herder took over as President. Her husband John also joined the committee as Treasurer. In 1997 and 1998 we tried to establish ourselves as a properly funded organisation and we also tried to set up a national constitution. Both these attempts diverted our extremely limited resources. In 1998 we tried to get to the IPOPI conference in Greece but could only afford to partially fund someone to represent us. Even though we got diverted in these years we were still getting involved with the Blood Bank and SCL and trying to get a more consistent supply of IntragamI. Our membership was continuing to grow. By 1999 we were on the point of giving up, but Mark and Kellie McNamara took over and they have kept things going. Hopefully the group will thrive in the years to come.

Mark and Kellie became interested in the APIA when their newborn son Jai was diagnosed with X linked agammaglobulinemia. They didn't want to see the good work of the APIA subside so Mark became president in 1999 and Kellie was elected secretary. Michael Daniel continued his involvement taking the position of treasurer.

The struggle to expand continued with member numbers remaining fairly static. Newsletters continued to be distributed to keep the communication lines open. In 2000, the committee was successful in obtaining an authority to fundraise and the Triple O Charity was approached and agreed to provide the funds to sponsor the newsletter for the year 2001. The Triple O Charity consists of members of the Police, Ambulance and Fire Brigade who raise money through social functions and dance parties. This non-profit organisation distributes proceeds to various charities and we are grateful for their assistance. Because membership is relatively low and our members are spread throughout Australia traditional types of fundraising are difficult. The struggle remains each year to fund our organization.

In 2000 Mark McNamara, as APIA President, was chosen to carry the Olympic flame for the relay to the Sydney Olympics. (see photo) "I was honoured" he said "and it gave the APIA a small amount of much needed publicity". This year Mark and Kellie's son Jai (now five) was among several other patients of the New Children's Hospital in Sydney to launch the 'Bandaged Bear Day' promotion for the hospital. (see photo)

The APIA's aim is 'to provide support, guidance and assistance to sufferers of Primary Immunodeficiencies and their families throughout Australia'. It is only through the dedication of the volunteers committee that it keeps going and with further help from new members on the committee the APIA will continue to meet its aims.

In future we hope to continue to liaise with overseas organizations with the intention of attending conferences to maintain this bond.

***Editor: There are two patient groups in Australia and we learn that merger talks are taking place.***





**Tatu Kulmala**

## History

The Patient Organisation for Primary Immunodeficiencies in Finland was founded in 1986. The organisation started slowly as a small linking group between patients and doctors. During the first couple of years there were only a few members and the main concern of the organisation was to try to determine how many patients there were in Finland with a primary immunodeficiency.

In the beginning everything was difficult! Doctors treating primary immunodeficiency patients all over the country hardly knew each other and there weren't any standards for diagnosis. Gradually, a network of primary immunodeficiency patients developed and solid ground for the work of a patient organisation was established. As always the fruits of the work ripen slowly.

## Today

Now the organisation has about 120 members – comprised of patients and family members. There is an annual membership fee of 10 € and all the members receive regular quarterly edition of our magazine. We also have an Internet site for communication.

Our patient organisation arranges annual meetings and lectures concentrating on subjects that are important for the membership. The most difficult aspect of being a national organisation for patients with rare disorders is trying to represent all our members equally. There are not enough members to establish local groups. Therefore, when we are arranging a meeting with a specialist lecturer it's always hard to choose the location so that a maximum number of patients can participate. As we do not have enough

resources to organise very many activities each year some members suffer from not being able to participate.

## Administration

The Finnish patient organisation has one annual general meeting and it is usually in April. The annual meeting elects the board which consists of six people. The board is the hard working core of the organisation. Previously, there were only four people on the board and the work load was quite exhausting - now the situation is somewhat better!

The chairperson of the board is elected annually. Other members of the board are elected for two years periods. The board has meetings approximately six to seven times per year. Some of the meetings are conference calls so that members of the board don't have to leave their homes for one short meeting. Like the members of the organisation in general, members of the board are living throughout the country.

The organisation has neither an office of its own nor any employees. The day to day work is done by the board members as a 'hobby' and is done after usual working hours. Therefore this work can sometimes be quite demanding.

The patient organisation is member of a national "umbrella" organisation for rare diseases and small patient groups. Being a member of a bigger group is rewarding since the organisation is better able to participate in different kind of events, fairs, meetings etc. and in that way gets its voice heard.

The Finnish organisation also gets financial aid from the national lottery fund, which grants money to small organisations working in the field of social security and health. The funding received from the lottery is not very much but it is still the biggest income of the organisation and we are grateful for it. We are also grateful to numerous pharmaceutical companies who have sponsored a range of events and projects over the last few years.

It seems, though, that it is more difficult to get members involved in the work of the organisation than it is to raise money. Projects like printing a booklet about primary immunodeficiencies in Finnish can sometimes take many hours and all the work is done in the evenings and weekends. However, the situation is more or less the same with all small patient organisations.

## Tasks and projects

The organisation maintains contact with its members through a regular newsletter, Internet sites and also via regular events. In addition to being a network of primary immunodeficiency patients, one of our priorities is to provide and distribute information about primary immunodeficiencies to the public and especially to doctors. Since the patient group is small there are many doctors who have never met a patient with a primary immunodeficiency. Naturally, the doctors are not to be blamed for that. However, it is important to try to spread awareness and information. In that way, for example, we can help diminish the time lapse between the beginning of the disease and its correct diagnosis. That time has luckily got shorter in Finland but in some cases we are still speaking about years, which, from the patients point of view, is far too long.

In order to achieve the above mentioned goal the organisation is starting a special project next autumn. The aim is to organise a lecture meeting twice a year in the biggest cities in Finland and gather general practitioners from all around that city to listen to the lectures. The subjects will relate to primary immunodeficiencies and its diagnosis. We hope to cover all the major cities in Finland in two years and that the project will be a success.

We have also published several booklets for our members. The first one, a general booklet about primary immunodeficiencies was published in 1998. It was posted to every hospital and communal health centre in Finland and the feedback was encouraging. Also the IPOPI travel guide was posted to all our members. Currently the patient organisation is printing a diary for primary immunodeficiency patients in which they can regularly chart the progress of their health, onset of infections, etc. So when a member is seeing a doctor it will be easier for them to tell how he or she has been and the follow up treatment is simple. Patients having IV treatment meet their doctor usually only once a month and it is important to gather information about the infections.

### International relations

The Finnish patient organisation has found it very important to keep contact with IPOPI and also with the other Scandinavian patient organisations. The Nordic organisations have been meeting together for over ten years and the next co-Nordic meeting will be held in Finland in spring 2002. Arranging the meeting will be a big challenge for the small organisation but it also gives good motivation to work a little bit harder.

Tatu Kulmala

## PiNSA

On 6 July 2001 a group of people met at the Red Cross Childrens' Hospital in Cape Town. These people came from varied backgrounds and were made up of medical people interested in the field of immunology, patients and family members affected by primary immunodeficiencies and also people from Natal Bio Products Institute, the producers of Polygam. Polygam is the local product used in the therapy of primary immunodeficiency.

The outcome of this meeting was the formation of a new association called PiNSA. There is a national steering committee made up of patient representatives from the three main centres i.e. Gauteng, KwaZulu Natal and the Western Cape and also a doctor representing the medical advisory panel.

This association has been formed for several reasons but mainly to create awareness of the existence of primary immunodeficiencies in South Africa. It is an insidious disease that manifests itself under the cloak of seemingly relatively benign, but repeated infections, which makes diagnosis easy to miss. Given our population of over 43 million, we should have in the region of 2000 patients diagnosed in South Africa whereas at the moment we only have a tenth of that figure. A booklet, flyer and poster is in the process of being produced for distribution amongst the medical fraternity which will highlight the existence of primary immunodeficiency and thus enable more accurate diagnoses.

Another reason the association has been formed is to give patient support. Primary immunodeficiency is quite rare, so newly diagnosed families find themselves isolated. The Internet has provided access to information and, to some extent, patient support through the work of organisations such as the PiA (Primary Immunodeficiency Association in the UK) and IPOPI (the International Patient Organisation of Primary Immunodeficiencies) but the large majority of our population do not have access to online communication. PiNSA will be providing this support through the formation of regional groups (and resultant face to face and telephonic support) and a printed newsletter. There will also be a website with a mailing list for those who can avail themselves of it.

The predominance of AIDS in South Africa has had a prejudicial effect on genetic diseases as has our unfortunate economic climate. There is simply not enough money for the amount of research primary immunodeficiency dictates and it is for this reason our medical fraternity needs the support of an association like PiNSA. It is hoped that we will have a symbiotic relationship and the foundation for this was established at the Red Cross meeting. The medical personnel who attended could not have been more enthusiastic and supportive, as was the NBI. Communication is going to be an essential aspect of the relationship and it was for this reason a medical advisory panel (MAP) was formed, headed by Professor David Beatty of Red Cross and represented by Dr Michael Loubser on the national committee itself.

Finally, international representation of the South African primary immunodeficiency community is essential. Little is known about this disease, relatively speaking, although findings are being made all the time. We need to be aware of these findings as does the international community need to be aware of developments in South Africa. We now have a properly constituted body and it is hoped that we will thus have a voice on issues such as availability and safety of blood products, new forms of therapy and the expression of concerns such as the side effects of long term therapy.

It is thanks to IPOPI that this association was formed at all, as it provided the seed money. It is my wish, as chair of the association for two years, to ensure that I hand over an organisation that is viable and dynamic and which goes a long way to reaching the goals as set out above. This is not going to be an easy task as we are 1) separated by distance 2) the committee members themselves experience the debilitating aspects of living with chronic disease (even if only once removed) and 3) we are all in full time employment. There is however a common commitment to the task at hand, and to each other, and I am thus positive that we will be successful.

Joy Rosario  
Chair PiNSA  
September 2001

## Bianca Pizzera

I am 54 years old this year and I am married with one son who is almost twenty.

During my childhood I never encountered any kind of severe or chronic disease. I did spend a night in hospital when I had my tonsils removed but the tears were soon forgotten by all the ice cream my sisters brought to me! It wasn't until many years later that my own family was diagnosed with XLA, a rare chronic disease, that I was faced with the truth and a challenge for which we were all unprepared.

In 1990 our immunologist informed us that during the ESID conference in Oxford she had met a group of patient representatives from different countries. I wanted to know more so I wrote to the patient organisations in the United States, in Sweden and in the UK. The response was encouraging and exciting, like a breath of fresh air.

Consequently, the Italian patient organisation of primary immunodeficiencies, the AIP, was launched.

I have attended all the IPOPI meetings since 1992 and in 1996 I was no longer 'just' learning but also passing on my experiences, advice and support with other IPOPI members and contact groups. Therefore, I felt that the time was right for me to come away from the sidelines and represent my organisation for IPOPI and contribute more fully to it's important work. I have now been a Board member for four years and I feel it is a great privilege and honour to serve IPOPI.

**Bianca Pizzera**  
**AIP, Italy**

Editor: We all have good reason to be grateful to Bianca for her valuable work as Acting Chair of IPOPI for almost eighteen months.



***A reminder to our readers that IPOPI has funds available to help national member organisations (NMOs) and potential NMOs to develop or develop projects that will benefit people with primary immunodeficiencies their countries.***

## IPOPI INTERNATIONAL DEVELOPMENT FUND

The IPOPI International Development Fund is solely dependent upon the generosity of individual pharmaceutical companies that make educational grants to IPOPI. Those companies have no say in how IPOPI allocates this fund although they will be informed of allocations made from the fund.

The purpose of the fund is to assist mainly new and emerging NMOs to become effective organisations helping people with primary immuno-deficiencies throughout the world. The fund is open to all NMOs as a resource to encourage better understanding and results for people with primary immunodeficiencies.

As the fund has been set up in 2001, there are currently no examples of the type of activity IPOPI have funded in the past. The Trustees do not want to restrict the thinking of NMOs to projects that have been funded in the past. NMOs should feel free to explore any avenue of activity that is appropriate to their national situation. No grant will be available for individual needs.

It will be appreciated that there will be limitations on grants in the same way that there will be limits on the funds available to IPOPI.

There is a pro-forma on which to make applications. Applicants will be asked for the following information

***Name of organisation***

***Is this an established NMO or an emerging group?***

***Who is the Chairperson or leader of the group?***

***Name of the person marking the application and their:***

***Address***

***Contact telephone/fax numbers***

***e-mail address***

Applicants will be asked for a brief description of the project – in which you let IPOPI know details of the project and the time scale involved – eg starting and finishing dates. If any publication is to be generated as a result of the grant, we require to see a copy of the text, preferably in English, before final approval can be given. If IPOPI

has to find a leading clinical immunologist to translate, this will delay your grant.

All applicants are required to provide a short budget statement with all amounts shown as UK pounds sterling.

Name and position of your main contact with IPOPI – so that we can find out more about your project and your group.

Please include a letter of support from one or more clinical immunologist in your country. By making an application you give IPOPI permission to approach leading immunologists in your country for a further reference.

If applicants are from emerging NMOs they must understand that they should have been in contact with IPOPI for some time and have established their intention of forming an NMO in their own country. Applications will not normally be considered from those approaching IPOPI for the first time.

At the end of the project IPOPI must receive copies of all receipts and proofs of expenditure involved in the project.

Applications will be considered throughout the year and there are no specific dates for receiving applications. Decisions can usually be made within four weeks for smaller grants. Larger grant applications may take longer to process.

This policy statement is subject to review during 2002.

For an application pack please contact:

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