

e-Update

July 2007



The global organisation working to improve the quality of life for people
with Primary Immunodeficiencies.

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

IPOPI – MAKING A DIFFERENCE!

We have been able to let you know that our efforts to have IM and IV immunoglobulins added to the WHO Essential Medicines List (EML) was successful. We hope that, especially in developing countries, this will help to secure supplies of immunoglobulin.

You should also know that IPOPI stands ready to help national member organizations achieve that end.

On another front we have been very happy to support Dr Helen Chapel who, on behalf of IUIS, has made an application to have subcutaneous immunoglobulin added to the paediatric EML. It is our earnest hope that, if this approach is successful, subcut will also be added to the main EML. We will keep you informed.

In the meantime know that IPOPI is about making a difference and we stand ready to give help and advice wherever possible – we are just an e-mail away!

Immunoglobulins re-instated on the WORLD HEALTH ORGANISATION Essential Medicines List (15th List , March 2007)



Published on WHO website:
www.who.int/medicines/publications/essentialmedicines/en/index.html
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**A registry of
immunoglobulins worldwide
supply is available on the
IPOPI website
www.ipopi.org**

**DR HELEN CHAPEL,
Honorary Vice- President
of IPOPI**

The Chairman and Board are delighted to announce that Dr Helen Chapel has honoured IPOPI by agreeing to become an Honorary Vice-President of IPOPI.

Dr Chapel was 'in at the beginning' of IPOPI, the first steps towards our formation being undertaken by Dr Chapel and Bob LeBien in 1990 in Oxford. Since then she has been a keen supporter of our work and chaired the Medical Advisory Panel for many years prior to the appointment of Dr Teresa Espanol.

We salute Helen for all she has achieved for people with primary immunodeficiencies around the world and are honoured that she now allows us the privilege of having her as our very first Vice President, alongside Bob, our Life President.

**GAINING ACCESS
TO RARE DISEASE
RESEARCH RESOURCES**

Jose Drabwell writes.....

Bianca and I attended a meeting in Paris on the 4th and 5th May organised by Eurordis.

The theme of the conference was "Gaining Access to Rare Disease Research Resources". Eurordis is a European Organisation for Rare Diseases, which is a patient driven alliance of organisations active in the field of rare diseases, dedicated to empowering people living with rare diseases and to improving their quality of life. In fact Eurordis has 274 members in 32 countries.

Their website is available in German, English, French, Italian, Portuguese and Spanish. – www.eurordis.org .

The EU commission has launched the Seventh Framework Programme for Research and it has a budget of 55 billion euros. This conference focused on the involvement of patient organisations in EU research activities by demonstrating the need for co-operation between small disease groups and the medical and scientific communities.



Sharing of basic databases, guidelines for clinical studies, medical protocol for rare diseases, classification of these diseases, drug regulation, creation of lists of experts both physicians and patients were just some of the topics discussed.

Research is so important especially for rare diseases. It is known that approximately 80% of these diseases are caused by defective genes and since the year 2000 more than 1500 of these genes have been identified. In 2001 EuroBioBank was established by 12 European Biobanks from 8 EU states and this network collects, processes, stores and distributes biological material for selected research projects, such as DNA, cells and human tissues – www.eurobank.org .

Examples were presented by patient organisations, which have been successful in receiving EU funding for specific research projects and there were many helpful suggestions from

representatives from DG Research, EU Commission. Ultimately the EU Commission wants to improve the health of its citizens. Increase competition of health related industries, address global health issues such as emerging epidemics and support research in rare diseases are part of its programme.

The conclusion of this very interesting and informative conference was the need for Eurordis to create a template for applying to the EU for funding, give support and advice for such an application, aid with scientific knowledge, provide training for involvement in the application and ability to translate documents into many European languages. There was also a request for the simplification of these applications and for quality of life studies.

As well as attending the two day conference we also had an opportunity to relax when all the participants were treated to cocktails and dinner at the Eiffel Tower, where a young mother who had lost her daughter to a rare condition last year, gave a very moving and beautiful violin concert.



FAMILY CAMP IN DENMARK

Sven Fandrup writes...

As is the tradition the Immunodeficiency Association, Denmark (IDA) held its yearly family camp from June 30 - July 7 in the northern part of Jutland.

One week of information, networking, relaxation and social get together.

This year the family camp had 20 families on board. Broken down into more precise figures it was: 41 adults, 38 kids, 7 young adults and 5 grandmas. With this diverse group of participants it was necessary to run three different programmes: One for parents (Adults), one for the kids and one for the young adults. And, as a new item this year a lecture for grandparents on the immune defence system and immunodeficiencies.

For the kids the activities comprised bon-bon production, physical activity (ball games etc.) and a first aid course for PID-patients. One of the good events for the kids was a "home treatment course" (SCIG) during which the kids could choose to sting an orange or their father! And - not to forget - a session with the "Teddy Bear - doctor", where the kids could bring their most loved Teddy Bear to the doctor and have him help it. More Teddy Bears suffered from PID!

The young adults had lectures on the immune system, a doctor talking about immunodeficiencies (diagnosis, treatment and care) and a morning discussing "how is my life with my PID and how will my future be". But they also went to a cinema to see "Transformers", played bowling, went bathing in the marvellous "water - land".

The parents went through a number of lectures with doctors, social workers and family advisor. But they also had time to sit together exchanging worries, solution to problems and common gossip.

The whole family camp ended Friday evening with a gala dinner during which the kids and the young adults entertained with a very, very fine cabaret.

Great fun for all! And lots of good information for the families!

THE LUCIANO VASSALLI

AWARD

Instituted by

The International Patient Organisation for

Primary Immunodeficiency - IPOPI



1. This award has been established in memory of Luciano Vassalli, who had such a great impact on the primary immunodeficiency world in his short life.
2. The award comprises a diploma and a commemorative coin. It will be given to a young person who has made a difference to the world of primary immunodeficiency for such acts as, for example, supporting a sibling, parent, people with a primary immunodeficiency or for making an impact on the PID world.
3. The maximum age limit for receiving this award should be 25 years.
4. Nominations will be accepted from NMOs only and should be received by the IPOPI board before the last day of January in the year of a biennial meeting.
5. The IPOPI board of Trustees will make the final selection.
6. The recipient will be given the award at the AGM of IPOPI at a biennial meeting by the Chairperson of the Board of IPOPI.



If the recipient is not able to attend the IPOPI biennial, the award will be given to a representative of the National Member Organisation.

A REPORT FROM INDIA

**SO YOU THINK YOUR NMO IS BUSY?
The Board of IPOPI were very
impressed by this short report of
activities from Mrs Rubby Chawla of
IPSPI – our NMO in India!**

IPSPI Activities Report

1) Presentation on PID by Mrs. Rubby Chawla, President, IPSPI on behalf of Medical Sciences, Raipur, Chattisgarh at Hotel Aditya under the Chairmanship of Dr. Arvind Neral, MD Pathology & President Dr. Ramesh Kr. Sahu MD Radiology. There was a gathering of 100 Post Graduate Doctors, mostly Paediatricians and Physicians. After Presentation distributed the posters & pamphlets of 10 warning signs and 4 stages of Immunologic testing. Mrs. Rubby Chawla was felicitated by presentation of bouquet & memento by the Chairman, Association of Medical Sciences, Raipur, Chattisgarh.

2) Presentation in Orissa – Pedicon – 2006 (IAP – Indian Academy of Pediatrics) by Mrs. Rubby Chawla President, IPSPI on behalf of IPSPI. There was a gathering of 350 Paediatricians of Orissa & delegates invitees from other States of India. After Presentation distributed the posters and pamphlets of 10 warning signs & 4 stages of Immunologic Testing. Mrs Rubby Chawla was felicitated by Presentation of bouquet, Certificate & memento by Chairman of the Organising Committee, IAP, Orissa.

3) Awareness to Mr B.N. Singh, Managing Director, Rourkela Steel Plant by appraising personally and presenting Posters of 10 Warning Signs and 4 Stages of Immunologic Testing & article. Request for addressing RSP employees on PID.

4) Meeting with Director Dr. P.C.Sharma of JLN (Jawahar Lal Nehru) SAIL Hospital, Bhilai for Presentation in JLN Hospital.

5) Presentation on PID among students and ex-teachers of SSSX (Senior Secondary School X) in Bhi lai Hotel in Nostalgia '81.

6) Presentation on PID in SSS Sec –X & among Teachers Ex – Teachers Students & Ex – Students. Distribution of Posters and Pamphlets to 200 Ex – Students, 30 Teachers.

7) Awareness of PID to Paediatricians Bhilai & Raipur, Chattisgarh, India. Distribution of Posters and Pamphlets to Paediatricians.

8) Awareness of PID to Honourable Governor of Chattisgarh, Sh. Narsimham. Governor was interested to set up a Lab. for Testing of PI Disorders at Government College +Hospital, Raipur (Capital of State Chattisgarh) as there is no diagnostic facility of PID in Chattisgarh State. Governor of C.G. has asked for the Project Cost at the earliest.

9) Awareness of PID to Collector of Indore. She assured to bring it in Govt. Agenda. She contacted Dr. Gagan Gupta, Paediatrician, Nephrologist, Indore & Board Member of Govt. Medical Association for the awareness campaign and PID inclusion in Govt. Agenda.

10) Awareness of PID to Dr. Gagan Gupta & Dr. Rachana Gupta, Paediatrician, Assistant Professor of MGM Medical College, Indore. Awareness to Paediatricians of Madhya Pradesh by distributing Posters and pamphlets of 10 Warning signs and 4 Stages of Immunologic Testing in Madhya Pradesh Pedicon - 2007 (IAP - Indian Academy of Pediatrics)

11) Awareness to General Public by Presentations & distribution of pamphlets of 10 Warning signs in different languages. India being a Country of 28 States & all the States having its own regional language. Till now, distribution of pamphlets has been possible in three languages - English, Hindi and Oriya.

12) Family Day was observed on 28th & 29th of April, 2007, World Immunology Day at PGI, Chandigarh. This was the first time that all the Patients Families had assembled in one place. We had a good Press & Media coverage. On 28th April 2007, Dr Surjit Singh (Hon. Advisor of IPSPI) made arrangements in the Conference Hall of Advanced Paediatric Centre, PGI Chandigarh for all the Reporters of National and Local Newspapers & Media Persons. They were briefed about PID by Dr. Surjit Singh. Mrs Rubby Chawla & Mr Rakesh Chawla briefed about IPSPI, the Personal suffering, Non-Awareness of PID, the challenges faced by Patients Families, the Awareness Campaigns by courtesy of WIN & JMF.

13) On 29th April 2007 Family Day was observed. The families of Patients with hypogammaglobulinemia met at the Advanced Paediatric Centre in PGIMER, Chandigarh. The arrangements were made in the Auditorium of Advanced Paediatric Centre, Chandigarh. We had an Interaction Session.

Each and every Parent shared their sufferings and recounted their unique experiences of facing this challenge over last few years. It was great to know each other. The atmosphere was as if everybody belonged to one family . We had also invited the Media persons for the coverage. Then there was a break for lunch.

14) Post Lunch we had invited Mr. K.D. Arora (AS. Retd.) of Kusum Trust , Ms. Suparna Malhotra, a Rotarian from Chandigarh & Mr. Shrivastav a journalist , Sales Executive of IVIG manufacturing companies. (Bharat Serum, Claris & Reliance) . They were all invited one by one to come on the dias & speak on support for IPSPI . Mr. K.D. Arora assured of his support to at least one patient.

We are thankful for his contribution as he has been supporting our children with PID for the last two years. Ms. Suparna Malhotra also committed on behalf of Rotary Club Chandigarh to support IVIG for one child . We are thankful for the contribution Mr. Shrivastava, journalist, talked on mobilisation of funds and support for IPSPI . Sales Executive of IVIG Manufacturing Companies committed to talk to their H .O. for reduction in prices of IVIG.

This was followed by Group Photos Session and everybody returned with promising memories in their heart .

THE FAMILY DAY WAS A GREAT SUCCESS!!

Kids gather to spread awareness

TIMES NEWS NETWORK

Chandigarh: The Chawlas from Rourkela, Orissa, had lost their sons at the tender ages of 9 and 6 years. They were fortunate to save the third one. Suffering from a rare deficiency, which is genetic, Rishi Chawla, the only surviving child of his parents, is a primary immunodeficient. On the occasion of the world immunodeficiency day, parents, with their suffering children, had come to PGI to spread awareness.

According to Surjit Singh, additional professor, paediatric allergy and immunology, PGI, The human body needs the immune system to fight infection. In patients with immunodeficiency, there is a defect in this system. Immunodeficiencies can be either acquired or inherited. "Inherited immunodeficiencies are also known as primary immunodeficiencies (PIDs). Children born with an inherited immunodeficiency get recurrent infections in form of repeated pneumonias, diarrhoea, skin and soft-tissue infections. Such children remain continually unwell. In many cases, the child would die in infancy or early childhood, if the condition is not diagnosed in time and treated appropriately," adds Surjit.

The term "hypogammaglobulinemia" refers to a group of conditions in which patients are unable to produce gammaglobulins. These gammaglobulins are proteins which are required to fight infection. No individual can survive for long without gammaglobulins. If a child has hypogammaglobulinemia, he needs replacement therapy with gammaglobulin injections. These injections are given intravenously every 3 to 4 weeks and have to be administered for life.

RARE DISEASE

Children suffering from a rare genetic deficiency disease at PGI

The editorial team welcome reports from NMOs (along with pictures) for publication in e-Update - info@ipopipi.org

INTERNATIONAL PLASMA PROTEIN CONGRESS

VIENNA 6-7 MARCH



Beautiful Vienna was the city where the above Conference took place and there were more than 320 participants from 32 countries attending the two day meeting.

The theme of the congress this year was **“PUTTING PATIENTS FIRST”**. Therefore this meeting was of great importance to people with primary immunodeficiencies. The sessions were divided into the following categories:

1. Global Development
2. Accessing Care for Small Patient Populations
3. Regulatory Developments
4. Considerations in Collecting Plasma for Fractionation
5. Challenges of Contract Fractionation
6. Patient Access to Immunoglobulins

In the first sessions there were presentations from **the president of the WFH – Mark Skinner** who pointed out that their organisation are working with a figure of 70% not diagnosed and 75% not treated which will result in many people dying young or being disabled. Sharing knowledge, information and training of both NMOs and healthcare professionals are the tools they are developing successfully.

Professor Vladimir Gorodetskiy from Russia shared with the audience his experiences (in Russian) in the collections and use of fresh frozen plasma. Russia is constructing 3 new processing plants, which will begin production in 2009. At the moment home produced immunoglobulin accounts for 80-85% with the balance imported.

Dr. Surjit Singh from India spoke about health care developments in India where 15% of the annual budget goes to defence and less than 2% on health expenditure.

Child malnutrition, primary education and discrimination against a girl child have a large negative effect on healthcare as well as female literacy rate and lack of immunisation. Infant mortality rate is high and HIV infection is at an all time high of 0.8% of the population (higher than in South Africa). A pro-active campaign is taking place and a home produced anti-retroviral drug has been developed and is also being exported to South Africa and Latin America. Polio has not been eradicated and many national programmes in healthcare has been started, such as anti-malaria, leprosy eradication, TB control etc.

Jan Bult, President of the PPTA, presented the global developments from

1. patients perspective – such as quality of care, awareness and diagnosis, access to care, affordability
2. clinical perspective – desire for a permanent cure, awareness and diagnosis, budget constraints, evidence based medicine, freedom of choice
3. industry perspective – donors/collections, manufacturing challenges, harmonization, product portfolio, economics

Following this session presentations were made by **a patient from the Alpha One Deficiency Syndrome Foundation**, who described what it is like living with the condition, which can cause emphysema as well as liver damage. This patient is actually waiting for a lung transplant. **Larry Warren , the CEO of the Foundation** pointed out that the only way to detect this condition is by genetic testing. Apparently this disorder is quite common in people from Northern Europe and of Iberian descent.

Dr. Bodo Grimbacher showed the audience the benefits of the ESID registry as well as the sub-registers with disease specific data models. At the moment 206 different PIDs have been identified with CVID still representing the largest number of patients.

Dr. Manfred Haase from the Paul Ehrlich Institute demonstrated the problems with clinical trials for small patient populations and informed the congress that EMEA has now provided guidelines on this subject. The need for clinically relevant result should be more important than statistical evidence. Marketing Authorization can be given to a new medicine after just a very small study, although there will then be an ongoing assessment of this drug.

Next was Dr. Jean-Marc Spieser who manages the activities of the biological standardisation programme dealt with the regulatory aspects of blood transfusion and organ transplantation as well as the conditions under which plasma should be kept.

Professor Albert Farrugia, who is a health regulator based in Australia, presented a lecture on whether regulation is hindering global availability of plasma protein. Of course delivery of health technologies is beset with other problems such as

1. inappropriate use
2. supply
3. quality
4. production
5. lack of resources
6. prohibitive drug prices
7. lack of research and development.

But regulation also opens up markets, provides insurance, support industry standards.

Dr Hartwig Gajek, medical director for Baxter showed that there could be an alternative to Randomized Clinical Trials. Maybe a patient reported outcome on the use of a drug in a small patient population could be much more useful. Unsuccessful trials are normally dismissed as negative, but it could be that a proportion of those tested reacted positively to the drug and therefore could have been beneficial.

Glenda Silvester, specialist in blood products with the EMEA, then spoke about international harmonization and co-operation with the United States and Japan. Furthermore the enlargement of the EU had to be positive as well as the exchange of information.

The second day of the conference dealt mostly with donors and plasma collections

Dr. Patrick Robert showed a five year outlook on plasma volume demand and concluded that supply will have to increase by collecting 3.6million litres of plasma or increase the yield of IVIG from 0.39 to 0.65 gram per litre.

Shinji Wada, President of Biomat, addressed the issue of balancing plasma manufacturing with global demand. Basically increased use of IVIG (Off and on label) against regulatory issues. So the conclusion is that an increase in fractionation capacity will be required to support the next phase of growth of the global demand.

Mrs. Ileana Cramer from Nabi Biopharmaceuticals gave examples of recruitment, incentives, recognition and most importantly retention of plasma donors.

Dr. Jurgen Wallner – University of Vienna – presented his ethics of donating plasma and the motivation for donating.

Ian Mumford of the Canadian Blood Services gave an in depth presentation of the industry in Canada. There are 20 collections sites as well as mobile plasma collections units. The plasma collected is non-renumerated and not for profit.

Collection of 190.000 litres of plasma per year goes for fractionation, which provides Canada with 40% of their requirements. The balance is imported from the United States. Off label use of IVIG has increased for use such as MS, Alzheimer. The Canadian Blood Service are committed to delivering a safe and secure supply of plasma protein products.

Uwe Jocham of CSL spoke about the challenges of Contract Fractionation and the ultimate aim of creating a mutually beneficial long term relationship with the customer.



Dr. Ana Padilla of the WHO referred to the international standards for quality assurance and safety of blood products in relation to the production, control and regulations of blood products as well as the good manufacturing practise.

Dr. Johann Kurz, Austrian Federal Ministry of Health, spoke about the EU commission Directives and the “import” and “distribution” of blood products and its components and the difficulties attache to the understanding of these protective measures.

Professor Janne Bjorkander, chair of the Swedish Physician Association for Primary Immunodeficiency, raised the question that IgG subclass is much more of a common immunodeficiency, maybe 1/250. He asked for a 2-3 year study to be done in a few European countries.

Dr. Thomas Szucs, a health economist from Zurich, used his statistics to show that one additional year of life expectancy results in a 4% increase in GDP.

Dr. Surjit Singh gave a presentation of 15 years of intravenous immunoglobulin from a developing country.

1970 – first used as substitute therapy

1980 – started to be used for immunodeficiencies, intramuscular only

1990 – IVIG more available, but very expensive.

There are now 4 Indian preparations being used as well as Biotest, Baxter and Novartis.

The cost of home grown IG is considerably cheaper, this product is made from plasma collection from Korea and China. There is no sub-cutaneous available.

David Watters informed everyone about IPOPI’s achievements and the challenges ahead.

The final speaker was **Dr Jacqueline Kerr** and she reported on the off-label use of IVIG and the possible new indications to be included, such as MS, Chronic Inflammatory Demyelinating Polyneuropathy, Multifocal Motomeuropathy, Myasthenia gravis, Polymyositis, Dematomayositis.

A most interesting meeting with less scientific presentations, therefore much easier to understand. More emphasis on patients, supply of IG and the acknowledgment that more IG needs to be produced.

In 2007 IPOPI promoted the first International Day of Immunology.

We have received thrilling reports of activities in many countries - Denmark, India, Norway, Iran - and many more. We reproduce here the reports received from Cyprus and Germany

... from Cyprus

For the very first time on April 29th 2007 we took part in the 1st International Immunology Day in Cyprus. As David Watters had said, "the day gave us all a wonderful opportunity to promote awareness for Primary Immunodeficiencies".

As we all know, Cyprus does not yet have a patient association. Personally, when I read the e-mail which David Watters sent to us, I began to have different thoughts of how Cyprus can organize and share this lovely gala event.

I had the idea to send two e-mails, first to Dr David Webster at Royal Free Hospital and the second to Dr Alison Jones at Great Ormond Street for Sick Children, to invite them as guest speakers, and they accepted with excitement.

In four months we had a lot to organize. First, I contacted Dr Angastiniotis and Dr Maria Koliou, both pediatrics and invited them to my place to tell them my exciting news. I am so grateful to these doctors for their help, as I saw this as an enormous challenge and knew that it would not be easy to organize this event exactly as I wanted to, but I had the belief that I could do it!

Four months had passed and the big gala was finally near. It was April 26th and Dr Alison Jones and Dr David Webster had arrived in Cyprus. My brother and I went to welcome them at the airport. We had a lovely homemade cuisine prepared by my mother which was well enjoyed. So, it was down to scheduling the big event!

Friday, April 27th their first stop was a visit to our Makarios Hospital to see some patients with PID. It went very well it's always good to see how other medical practices work. It was Friday evening and you can't visit Cyprus without going to a traditional fish tavern - and so that is how we spent Friday night!

The big Gala Event...Saturday morning my father took David and Alison for sightseeing in Kyrenia Morfou and to our lovely Troodos Mountains. They really had a wonderful time.

You could imagine what I was like that day, so nervous and anxious about the whole event that was going to begin that afternoon.

In attendance was the Minister of Health, pediatricians and parents. In all there were 125 people attend the event.

And we were off to our first key guest speakers. Dr David Webster and Dr Alison Jones were introduced by Maria Koliou. She spent a few minutes introducing our current situation in Cyprus for our PID population.

Dr Webster spoke first about the Immune System and gave us a better understanding of how our immune system survives and works with people who have inherited PID.

Second to speak was Dr Alison Jones. She taught us how to recognize PID in everyday clinical practice.

This had successfully concluded our first session of the program.

To introduce our second session Dr Angastiniotis spoke a little about Psychological issues in Chronic Conditions like PID.

Dr David Webster began his second session speaking about Common Variable Immunodeficiency, a disease of older children and adults. He also provided advice on the diagnosis and management of this disease, alongside new persistent viruses. As you could imagine, it was very intense, but well explained and thorough for all to understand. Next Dr Alison Jones spoke about managing PID, immunoglobulin therapy and Bone Marrow transplantation for severe PID children.

The event was a HIT! Along with this being educational, I believe it was also a very big eye opener to our Medical Communities and parents in Cyprus and parents in Cyprus

For me, as you can only imagine, I was thrilled about the success, but I know that we still have a long way to go.

My next step to this dream for Cyprus is to build up a first PID Association this June 2007. I will be meeting with some parents to discuss this opportunity. Wish me luck! **Soon Cyprus will also be members of IPOPI!**

Maria Charalambous

.. and from Germany

To bring more awareness to the public and to inform about immunological diseases, symptoms and therapy options the German patient organisation Deutsche Selbsthilfe Angeborene Immundefekte e. V. (DSAI) organized several activities around the first **Worldwide Day of Immunology**.



DSAI was invited to presentations in some German cities - Munich, Duesseldorf, Hamburg, Marburg, Freiburg, Frankfurt, Kassel, Erlangen. Patients, specialists and politicians participated and were able to have discussions with each other.

In Berlin DSAI was co-organiser of a Scientific Symposium about Primary Immunodeficiencies with health politicians and immunologists. A patient spoke about her everyday life with this disorder.

The importance of this type of event is illustrated by the fact that the rate of diagnoses has doubled in the last two years.

*Eva Soergel
for DSAI, Germany*

Primary Immunodeficiencies:

Know them to fight them