NEWSLETTER

MAY 2010

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In the May 2010 issue…

ABOUT EHC: The European Haemophilia Consortium (EHC) is a European patient group representing national member organisations from 43 countries in Europe. The EHC is working to reduce the burden of the disease on both the individual and on society. Its mission is to improve the quality of life of people with Haemophilia in Europe. For more information contact info@ehc.eu

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THE PRESIDENT'S REPORT

Dear Readers, Members and Friends,

I hope this Newsletter finds you well, full of energy and new ideas for future projects.

The 9th Round Table focused on Ageing and Haemophilia and took place in Brussels on February 23. It called for an enhanced multidisciplinary care approach with a comprehensive group of specialists available for ageing Haemophilia patients and enhanced social integration of ageing patients, including networking social groups for patients, and the promotion of physical activity.

The next EHC Round Table will take place in Brussels on the 2 June and will focus on "Prophylaxis for Adults". The programme has been finalized and it seems we will have a very interesting event. If you want to participate, please do not hesitate to send an e-mail to: info@ehc.eu. I would like to see many of you there!!

As usual the EHC team is going on with the monitoring of European regulations, building positive relationships with our stakeholders, fostering our external communication in order to increase EHC visibility and role in Europe.

Before wishing you a nice reading, let me remind you that the 23rd EHC Annual Congress will take place on 22-24 October 2010, Lisbon – Portugal. The Conference will consist of plenary lectures and presentations in which a broad spectrum of scientific and practical issues and problems in Haemophilia care will be discussed. It will be an excellent possibility to share ideas, knowledge and recent scientific achievements.

All the best

Ad Veldhuizen

President of EHC

EHC News

The European Haemophilia Consortium calls for better social support to ageing patients

The 9th Round Table focused on Ageing and Haemophilia

Participants of the 9th European Haemophilia Consortium (EHC) Round Table which took place on 23 February, called for a greater multidisciplinary approach to ageing Haemophilia patients, with a strong emphasis on their social and emotional needs. Stakeholders also advocated for an increased focus on pain management which stands to significantly impact the quality of life of Haemophilia suffers.

The debate focused on the growing population of ageing haemophilia patients, and the associated risk of co-morbidities. Stakeholders stressed that until the last decade, there was a minority of ageing patients, mainly due to the lack of early treatment and comprehensive care. As Haemophilia patients live longer, they present a range of new medical, economic and social challenges which need to be thoroughly addressed across the EU.

Dr. Mauser-Bunschoten presented the importance of a multidisciplinary care approach for ageing patients

Dr. Mauser-Bunschoten, from the University Medical Center of Utrecht, reiterated the fact that co-morbidities are a result of ageing and an irreversible process for the +40 years population. She also indicated that co-morbidity would soon become a bigger problem for Haemophilia patients than Haemophilia itself. For this reason, she advocated for an enhanced, integrated approach of Haemophilia care, which gathers an experienced team of various specialists who are close to the patient's

home.

The debate also focused on the impact of health-related Quality of Life (QoL) on elderly patients and the need to conduct questionnaires that combine different and tailored determinants of QoL. Dr. Mackensen from the Institute of Medical Psychology of Hamburg presented the results of a study conducted in Italy that revealed the greatest areas of impairment for Haemophilia patients to be sport, physical health and self-image. Dr. Mackensen also stressed that one of the main issues facing ageing Haemophilia patients is chronic pain, and she regretted that the issue had been missing from the debate on Haemophilia until now. Dr. Mackensen highlighted that ageing haemophilia patients greatest concerns are losing their autonomy, coupled with persistent chronic pain.

Dr. Santagostino talking on the cost of care of ageing Haemophilia

Dr. Santagostino from the Haemophilia and Thrombosis Centre of Milan drew attention to the cost of care for ageing patients with Haemophilia. She pointed out that there is at present no study available on the cost of care for this segment of the patient population. However, surveillance programmes have been recently launched in many European countries to collect epidemiological and clinical data on long-term Haemophilia-related co-morbidities and age-related diseases for elderly Haemophilia patients. Indeed, the management of cardio-vascular diseases and cancer represent new challenges for these ageing patients who are also often affected by disabling arthropathy, long-lasting HIV infection, or liver disease. These age-related co-morbidities have an enormous economic impact on the cost of care. This means there is an urgent need to identify adequate and viable strategies to guarantee healthy ageing for Haemophilia patients.

Ms. Cluzel representing the European Older People's Platform, AGE called on for greater social cohesion, mutual learning, exchange of good practice, and a desire to strive for intergenerational solidarity. She also stressed that she would welcome strengthened collaboration between the EHC and AGE.

The Round Table was also the occasion to debate the broader economic and fiscal consequences of ageing, looking at trends in the demography of Europe and the impact of ageing on national public spending in health. Mr. Bartosz Przywara from the European Commission DG Economic and Financial Affairs,

presented the key points and healthcare projections of the Commission 2009 Ageing Report. He stressed that prevention and improvement in health status would substantially offset ageing effects, limiting future spending, as ageing leads to increase long term care expenditure. Mr. Przywara also pointed out that new health technologies may increase health expenditure, but in the long term should lead to cost - efficiencies.

Dr. Bacher, Danish Haemophilia Society

Finally the Round Table gave the opportunity to National Members to present progress and challenges of haemophilia care for ageing patients in their respective countries. NMOs representatives stressed that the dialogue and social support seems to become a key aspect in ensuring well being of haemophilia patients through their advanced years. Consensus arose on the need for more social support to ageing patients with Haemophila, making sure they are not isolated, and giving them the opportunity to maintain a good quality of life.

Echoing the Round Table exchange, the EHC called for:

- An enhanced **multidisciplinary care approach** with a comprehensive group of specialists available for ageing Haemophilia patients
- Enhanced **social integration** of ageing patients, including networking social groups for patients, and the promotion of physical activity
- Robust strategies to ensure **cost-effective treatment** for ageing patients and to guaranty healthy ageing
- **Increased information** on access to physiotherapy and other services which contribute to the well-being of patients
- Adequate care that includes **chronic pain management** which today dominates ageing Haemophilia patient's concerns

For more information, please see: http://ehc.eu/index.php?id=115

Next EHC Roundtable of Stakeholders will focus on Prophylaxis for Adults

The 10th Round Table of Stakeholders organised by the European Haemophilia Consortium (EHC) will take place on 2nd June 2010 and will focus on prophylaxis for adults, placing particular emphasis on the cost benefits of prophylaxis, and the challenges of prophylactic treatment for adults.

Another important aspect to be debated during the Round Table is the challenges for patients with inhibitors.

Finally, the economic crisis, together with the growing ageing population is putting additional pressure on health systems, challenged to take measures which seek to lessen the economic and social burden of diseases on already-stretched health systems. The Round Table will look to provide the national perspective on the use of prophylaxis treatments including the views of the insurance community.

Round Table participants will try to answer more fundamental questions on prophylaxis including:

- What are the cost benefits of prophylaxis?
- What are the challenges and benefits of prophylaxis in adult patients?
- Is on demand treatment a preferred option for adults than prophylaxis?
- What is the optimal dosage and frequency of treatment?
- What are the treatment objectives for individual patients and how can clinicians involve the patient in the development of a patients care plan?
- How can health systems best accommodate prophylaxis and what is the ideal patient pathway for Haemophilia patients. The challenges and differences in approach to prophylaxis.
- What is the likely impact of today's economic crisis on prophylaxis treatment in overstretched healthcare? Will there be an increased desire to delay costs?
- What can be done to advocate for regulatory conditions that favour prophylaxis?
- What is the patients role in this process?

The EHC Round Table entitled "Prophylaxis for Adults" will give clinicians, patient organisations, EU health stakeholders and representatives from the pharmaceutical industry the opportunity to address the different issues surrounding the topic of prophylaxis for adults.

For more information please contact: info@ehc.eu

A Survey on Prophylaxis versus On Demand Therapy in Young Adults with Haemophilia in 4 European Countries.

Prophylaxis is widely accepted as the optimum standard of care for children with severe haemophilia. However, there is still a lot of discussion and debate on the necessity of prophylaxis continuing into adulthood. Prophylaxis has been the standard of care for children and adults in Sweden for over 25 years. Therefore young adults in Sweden with severe haemophilia would, in the vast majority of cases, have been on prophylaxis since a very early age and this would have continued into adulthood. In contrast young adults with Haemophilia in countries such as Ireland, UK and France would generally be treated with on demand therapy although some would be on prophylaxis. However in these countries prophylaxis would have been introduced sometime in the last 10 to 15 years and therefore these young adults would not have grown up with the benefits of prophylaxis and would have started their lives with on demand therapy.

A survey was undertaken by Brian O Mahony and Declan Noone of the Irish Haemophilia Society. The survey gathered data on young adults with severe haemophilia in 4 European countries – Sweden, France, UK and Ireland with the co-operation of the Haemophilia Societies in Sweden, France and the UK. A total of 58 young men with haemophilia between the ages of 20 and 35 were interviewed by phone. They were asked questions in relation to how long they had been on prophylaxis, about target joints, major bleeds and mobility problems. They were also asked to answer a very simple quality of life questionnaire with five questions (EQ5D). The EQ5D questionnaire is based on questions that related to mobility, self care, ability to carry out usual activities, levels of pain and discomfort and levels of anxiety and depression The questionnaire allowed us to extrapolate a utility value for the quality of life on a scale from zero to one – zero would correspond to death and one would correspond to a perfect quality of life. The results of the survey are crystal clear. The benefits to the adults in Sweden of having been treated prophylactically from an early age and continuing into adulthood were obvious.

People with severe haemophilia in Sweden reported less target joints, less major bleeds and significantly lower mobility problems when compared to people with haemophilia in the other three countries. (Table 1) The average number of bleeds per year in Sweden was just over 3 whereas, in the other 3 countries it varied from 16 to 20 bleeds per year. Only 25% of those in Sweden had target joints, whereas 94% to 100% of those in the other 3 countries had target joints.

	Average Bleeds per Year	Target Joints	Major Bleeds
Ireland	16.5	94%	68%
UK	17.5	100%	44%
France	20.1	100%	80%
Sweden	3.2	25%	20%

Table 1 : Bleeding pattern

There was a very clear differential in relation to the number of days missed from work or college. (Table 2) In Sweden the average number of days missed per person per year was 0.5, in Ireland it was 5, in the UK it was 6.6 and in France it was 15.

The figure in France is very high because the French persons with haemophilia questioned for the survey included two young men with haemophilia who had recently undergone joint replacement surgery and therefore they were missing a significant amount of college or work. This is not unusual as the requirement for orthopaedic surgery and joint replacement surgery will be there for individuals who don't have access to prophylactic therapy.

	Average days missed/person/year
Sweden	0.46
Ireland	5
UK	6.6
France	15

Table 2: Days missed from Work or College Annually

There is also a dramatic difference between Sweden and the other 3 countries when it came to the respondents views of their quality of life as measured by the EQ5D questionnaire. The average quality of life in the UK, Ireland and France

varied from 0.68 to 0.74, whereas the average quality of life in Sweden was 0.93.

This Swedish score is very close to a perfect quality of life (1.0) whereas those in the other 3 countries have the perception that their quality of life is about 25% lower than those in Sweden.

When we looked at the data in relation to the treatment regime it was very clear that the differences were not due to nationality or other factors. They were due to the fact that the individuals in Sweden had been on prophylaxis from a very early age and this was continuing into adulthood. This study is a small scale study with a relatively small numb of participants. It would be interesting to gather more data from more countries and in particular it would be interesting to look at data from countries which use different prophylactic regimes. However the benefits of long-term prophylaxis continuing into adulthood are clear from this survey. The improvement in quality of life, in ability to take part in society, in ability to attend college or work and not be limited by bleeding episodes or joint damage in Sweden is startling. Out of the 58 patients surveyed only 16 had no target joints. 15 of the 16 are living in Sweden. We hope to extend this survey to other countries and to continue this work in the future.

At this particular time, when budgets are coming under threat, when treatment regimes are been questioned, when high cost but high value treatment regimes such as prophylaxis are under threat in some countries it is timely to have this reminder of the transformation of quality of life which can be brought about by giving a person with severe haemophilia optimum therapy on an ongoing basis. Parents of children with haemophilia in UK, France and Ireland whose children are treated using prophylaxis are also perhaps getting a glimpse into the future with this survey. If your child complies with his therapy and takes his prophylaxis regularly, as a young adult there is every reason to believe that his quality of life should be near perfect and he should be able to fully participate in college, in employment and in the everyday normal activities of life with no impediment.

Brian O Mahony

EHC Steering Committee Member

NMO News

Order of Smile for Bogdan Gajewski, President of the Polish Haemophilia Society

Bogdan Gajewski, when receiving the Award

During a press conference on 18 May 2010 referring to the Haemophilia Day, Bogdan Gajewski, President of the Polish Haemophilia Society, was awarded the Order of Smile.

The Order of Smile is the only order in the world that is awarded to adults by children – for kindness, friendship, goodness and patience shown to children. The order has been designed by children and it can be awarded only on their own initiative.

Bogdan Gajewski was nominated by children and youths with haemophilia, who wrote a letter of recommendation to the Chapter of the Order. Thanks to efforts of the Polish Haemophilia Society, in 2008 Polish children with haemophilia until the age of 18 started receiving prophylactic treatment, which considerably improved quality of their lives. Thanks to the prophylaxis, they avoid most of complications connected with haemophilia that have been the everyday reality of adult pwh in Poland: painful bleeds into joints and muscles as well as their irrevocable destruction.

"It is thanks to Mr. Bogdan Gajewski that we are on prophylaxis. I have to inject myself fairly often but thus I can play football and ride a bicycle. And my Ma doesn't worry that I might be hurt. I forget that I have haemophilia and I feel like my friends – I feel I'm healthy. . . . Bogdan Gajewski is a person I look up to. I'd like to become someone like him." This is what ten-year-old Konstanty wrote in his letter.

Before being knighted each candidate has to drink a tumbler full of pure lemon juice – and smile.

Bogdan Gajewski has been active in the Polish Haemophilia Society since its very foundation in 1988. Since 2007 he has been the Society's President. Still before being elected President he started a web discussion group for pwh in Poland which now has more than 600 registered members, and also a similar web discussion group for East and Central Europe. He runs a web site (www.hemofilia.of.pl), and translates and edits books and DVDs on haemophilia. Those educational publications are aimed at children and their parents as well as adult patients. So far more than 20 books and DVDs have been published, and there are other waiting. The extraordinary effort of the Society including writing letters and petitions to the Ministry of Health and individual MPs has contributed to a considerable improvement of the standard of haemophilia treatment in Poland.

The Order of Smile is a special order, having nothing to do with any social or political organization. It is awarded without any regard to the nationality, religion or colour of skin. So far more than 900 people have been distinguished in that way. Among the Knights of the Order you can find Pope John Paul II, the Dalai Lama, Mother Theresa of Calcutta, Steven Spielberg, Oprah Winfrey.

Among the Knights of the Order there are also two other members of the Polish Haemophilia Society: Zbigniew Sendulka (a former president) and Zdzislaw Grzelak (current vice-president). This is a sign of appreciation for their work and engagement to improve the treatment for children with haemophilia.

"I'm very glad because the Order of Smile has been granted to me by children with haemophilia. I know what they feel, what kind of problems they face because I have been through it myself. Thanks to prophylactic treatment they can live without suffering, without crutches, all that has been the everyday reality for adult pwh in Poland," says Gajewski. "Not everything is OK yet. We shall struggle to extend prophylaxis to cover young pwh under 25, and in future all adults that need it. We want also that Poland should use the safest clotting factors, those that aren't produced from blood – recombinants, and that people with inhibitors should obtain proper treatment.

Thank-You Poster: the efforts of the Polish Ministry of Health

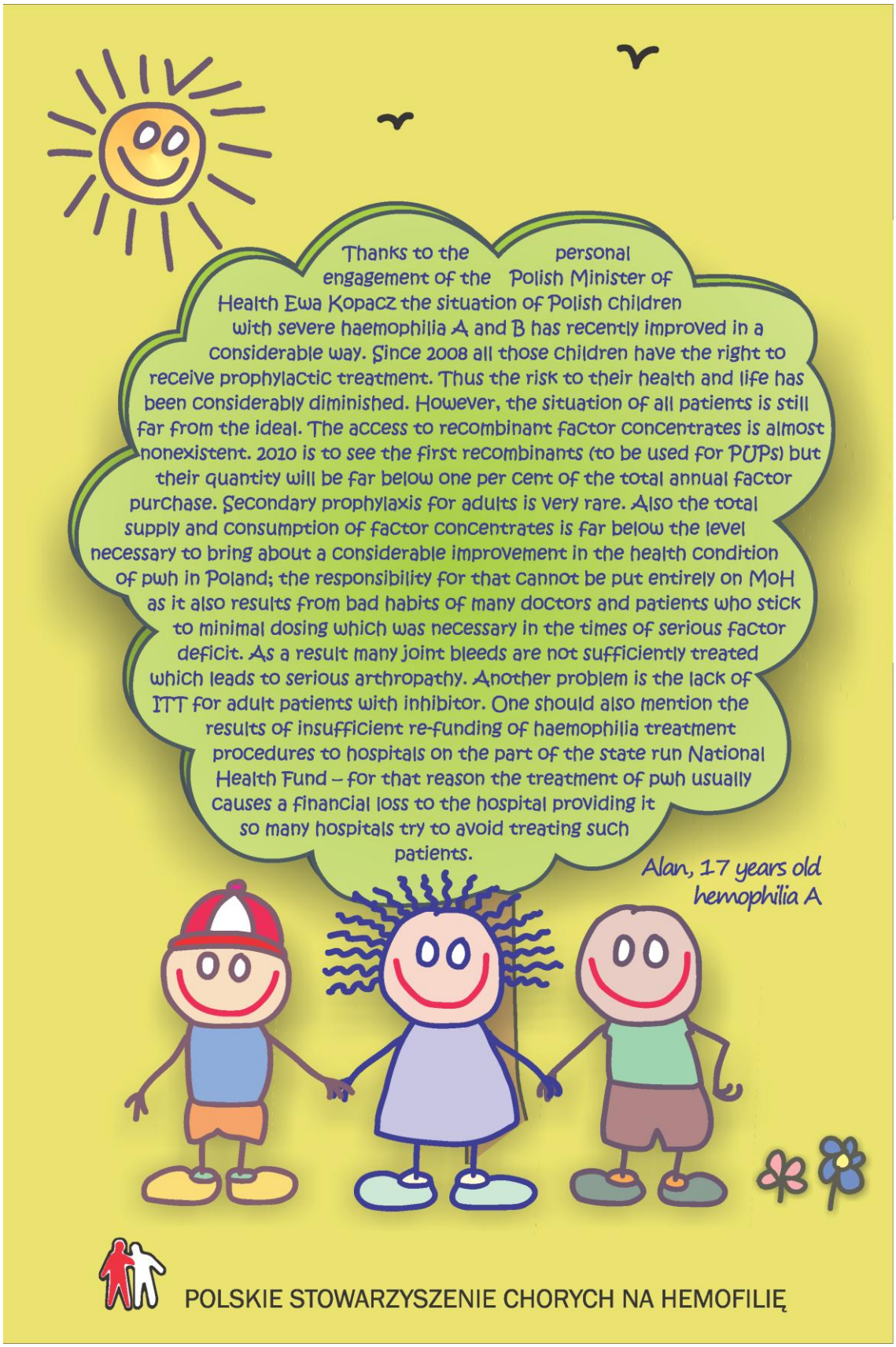
The poster that you can find on the next page was conceived by Alan Gorski, Poland.

Alan said: "The poster was created spontaneously. It is a symbolic response to the activities of the Polish Ministry of Health introducing the break-through prophylactic treatment for children and youths under 18 with haemophilia in Poland. It is also a kind of thank-you to the Health Minister Ewa Kopacz for her personal engagement. What is more, it is also meant as an encouragement to further decisions so that this age barrier might stop being the end of our happy times.

I wanted to show that illness does not have to mean sadness, pain, isolation, that sometimes it is enough to notice another person, to show him some kindness in understanding his needs, thus changing his life forever.

If you try to define frankly what decides about happiness in our lives and what is our lives' true aim, then you could say that basically we don't need anything but the warmth of the sun and birds singing when we are among friends on whom we can rely.

I hope that with the help of a few simple symbols I've managed to show all that."


Thanks to the personal engagement of the Polish Minister of Health Ewa Kopacz the situation of Polish children with severe haemophilia A and B has recently improved in a considerable way. Since 2008 all those children have the right to receive prophylactic treatment. Thus the risk to their health and life has been considerably diminished. However, the situation of all patients is still far from the ideal. The access to recombinant factor concentrates is almost nonexistent. 2010 is to see the first recombinants (to be used for PUPs) but their quantity will be far below one per cent of the total annual factor purchase. Secondary prophylaxis for adults is very rare. Also the total supply and consumption of factor concentrates is far below the level necessary to bring about a considerable improvement in the health condition of pwh in Poland; the responsibility for that cannot be put entirely on MoH as it also results from bad habits of many doctors and patients who stick to minimal dosing which was necessary in the times of serious factor deficit. As a result many joint bleeds are not sufficiently treated which leads to serious arthropathy. Another problem is the lack of ITT for adult patients with inhibitor. One should also mention the results of insufficient re-funding of haemophilia treatment procedures to hospitals on the part of the state run National Health Fund – for that reason the treatment of pwh usually causes a financial loss to the hospital providing it so many hospitals try to avoid treating such patients.
Alan, 17 years old
hemophilia A
POLSKIE STOWARZYSZENIE CHORYCH NA HEMOFILIĘ

Irish Haemophilia Society

Agreement on vital issues relating to Blood and Plasma:

The Dublin Consensus Statement

There are many contentious issues in relation to blood and plasma which divide the key stakeholders globally. These issues include: the question of paid and unpaid donors for blood and plasma, donor deferral measures and the relative rights of donors and patients. Over the past several years, it had become increasingly clear to me that the views of many of the key stakeholders were fixed on these issues and there was very little real dialogue taking place. From a patient organisation perspective, it was also very clear that a distinction needed to be made between the collection of blood and the collection of plasma for fractionation and subsequent manufacture into plasma derived medicinal products. The amount of plasma which is required globally to make plasma derived factor concentrates; immunoglobulins, albumin and other products manufactured from plasma, greatly exceeds the amount of plasma which can be recovered from whole blood donations. Clearly therefore there is a need for, on the one hand, plasma to be collected as recovered plasma from whole blood donations but also a very significant amount of plasma to be collected by plasmapheresis (called source plasma). In the vast majority of countries, blood donors are voluntary and non-remunerated whereas plasmapheresis donors who donate more frequently are voluntary remunerated donors. The plasma from both sectors is urgently required but, our concern as patient organisations was that there were often calls for banning of plasma from paid donors and that many of the opinions expressed were not based on science or in the best interests of patients. Under the auspices therefore of the group of patient organisations who constitute the Plasma Users Coalition (PLUS), which includes the EHC and WFH, I organised a Consensus Conference for the key stakeholders which took place in Dunboyne Castle, Ireland in January 2010. I was greatly assisted in preparing all the materials for the conference by Alison Turner the Chief Executive of the National Blood Authority in Australia. The conference was attended by 15 key opinion leaders from a broad range of stakeholders. This included representatives from patient organisations (PLUS, WFH and Alfa Europe), National Blood Authorities (Australia, Canada and Ireland), the industry sector (PPTA), the not for profit sector (IPFA and EBA), the International Society for Blood Transfusion (ISBT) and the donor organisation (IFBDO). The World health Organisation attended as observers. The participants engaged in two days of constructive dialogue and discussion which resulted in an agreed consensus statement called the Dublin Consensus Statement. The Statement contains an introduction setting out broad principles and it then contains four sections relating to patients, donors, sector relationships and global utilisation of

donated blood and plasma. There is a recognition in the introduction that national blood transfusion systems can be based on voluntary non remunerated donors but also that an adequate supply of plasma products requires both recovered and source plasma to meet patients needs on a global level.

There was a very welcome recognition that the absolute focus of the blood and plasma sectors must be the patient and a clear statement that patients whose continued health is dependent on the use of plasma products have a right, through their representative organisations, to be consulted on any issue which may have an impact on the safety, efficacy or supply of treatment they receive. These statements might seem self-evident but in the past, in many of the divisive debates which have taken place on these vital issues, the interests and views of the patients were very much treated as a secondary consideration. There is also in the consensus statement a strong section on the rights of donors and the value placed on all of those who donate blood or plasma for the benefit of patients. However, this must not obscure the fact that the primary purpose of the collection of blood and plasma is for the benefit of patients and therefore patient organisations must be proactively involved in the decisions which may impact on their therapy. That is why it was vital, in my view, that this Consensus Conference was organised under the auspices of the patients organisations which come together under the plasma users coalition. The consensus statement was agreed by all the participants with the exception of the representative of the donor organisation who none the less very much appreciated being present at the conference and contributed to the discussions. The Statement has now been sent by each of the individuals present to their respective organisations to seek their formal endorsement. The statement has to date been formally endorsed by the EHC, the International patients organisation for Primary Immune deficiencies (IPOPI),hereditary Angiodema International (HAEI) and the Guillain Barre support association (GBS- CIDP).The statement is currently being considered by the other organisations whose representatives attended the conference. The Statement will be published by the prestigious Blood Transfusion Journal Vox Sanguinis in June and has been published online (A copy of the paper has been sent electronically to all EHC member organisations by the author.)

Of equal importance was the constructive nature of the dialogue that took place. Many of the participants at the Consensus Conference hold diametrically opposite views on several of the issues. During the discussions which took place, perceptions and views were altered over the course of the two day meeting. The participants clearly signalled that they wish to see this becoming an ongoing process and therefore a Consensus Conference will once again be held in early 2011 to continue this vital dialogue. Our aims as patients

organisations is to ensure that this dialogue and constructive engagement leads to the views of patients being front and centre in all of the major decisions taken in relation to blood and plasma in the future. In late March we discussed the outcome of the Conference with the EU Commission and PLUS will be proactively consulted when the EU Blood directive update reports are being prepared.

Please find below the full statement.

Dublin Consensus Statement

Principles to apply to the collection and manufacture of blood components and plasma products

Introduction

The three major priorities for the global community in providing patients with adequate and safe blood components and plasma products are to:

a) Provide safe and sufficient blood components in all countries through the development of national blood transfusion systems based on voluntary non – remunerated donors.

b) Maintain sufficient and sustainable supplies of blood components from established blood transfusion services, based on voluntary non – remunerated donors.

c) Provide an adequate supply of plasma products from recovered and source plasma to meet patient needs on a global level.

The blood and plasma sectors comprise

- Blood establishments whose principal objective is the collection of blood for the production of blood components and in some cases plasma for further fractionation, and
- The plasma sector which collects plasma for subsequent fractionation into plasma derived medicinal products. Plasma products made from both non-remunerated and remunerated donations are currently essential to meet global health needs.

The donation of blood or plasma and its transformation into products that save and enhance the lives of patients is an invaluable contribution to modern healthcare.

Respect for individuals, maintaining the health of blood and plasma donors, and providing safe blood and plasma products for patients, are of utmost importance.

Countries and regions are entitled to have policies and practices on blood and plasma which reflect their political, cultural, ethical and economic contexts.

The blood and plasma sectors must operate within stringent national, regional and international regulatory regimes that support the production of safe and effective products.

The following principles provide the foundation on which the blood and plasma sectors should build their operations.

Principles

1. **Patients**

The absolute focus of the blood and plasma sectors in health care must be the patient.

1.1 Meeting the health needs of patients through a sufficient supply of safe and effective blood components and plasma products is the principal goal of the blood and plasma sectors.

1.2 Patients are entitled to expect that all stakeholders in the blood and plasma sectors will support their need for access to safe and effective products.

1.3 Patients whose continued health is dependent on the use of blood or plasma products have a right, through their representative organizations, to be consulted on any issue which may have an impact on the safety, efficacy or supply of the treatment they receive. Health Authorities should ensure that robust mechanisms are in place to ensure that this happens.

1.4 The blood and plasma sectors must ensure that their actions do not compromise the health status of those that receive blood components or plasma products.

1.5 The blood and plasma sectors should take all reasonable steps to eliminate the possibility of adverse reactions and events including

transmission of pathogens. Risks vary from product to product and each product should be individually assessed.

2. Donors

2.1 The blood and plasma sectors must respect the intrinsic dignity of all people involved in the blood and plasma donation process.

2.2 The blood and plasma sectors and society in general should highly value all those who donate blood or plasma for the benefit of patients, recognize that donors perform a good action and treat donors with respect.

2.3 There is a limit to the capacity of the blood and plasma sectors to ensure the safety of blood and plasma products through testing and processing alone. It is therefore important that measures to defer donors are based on a precautionary approach and underpinned by evidence based assessment where feasible. Donors must have donor deferral policies clearly explained to them.

2.4 All people may offer blood or plasma to the community and their generosity is highly valued. However, the blood and plasma sectors have an obligation to only accept blood or plasma where the donor selection criteria are met.

2.5 All donors must give their free and informed consent prior to the donation.

2.6 All donors must be provided with clear and accessible information prior to their donation, which should include information on:

- The potential risks to them of donating blood or plasma,
- The intended use of their donation,
- who might benefit from their donation, including the health benefits for patients, benefits to the blood service and to any other party who facilitates the donation.

2.7 Donor information and samples will be kept private and confidential in accordance with relevant guidelines and legislation.

2.8 Donors should not be exploited by any individual or organization.

2.9 The blood and plasma sectors owe a professional duty to act in the best interests of those that donate and receive blood and plasma products.

2.10 The health of the donor should not be compromised by their donation.

2.11 Those seeking donations of blood and plasma may offer incentives for people to donate. Incentives offered will differ and reflect the social, economic, ethical and cultural environment in which the blood and plasma sectors operates. However, all incentives should be of a kind that

- pose no risk of harm,
- do not overwhelm the capacity of the donor to make an informed decision about whether or not to donate.

3. Sector relationships

The production of blood components and the manufacture of plasma products involve different manufacturing pathways, have access to different risk mitigation measures and the products are used to treat different diseases. The coexistence of two independent collection systems, one for blood and one for plasma, in the same region or country, could create a risk of shortage in the supply of blood components. Cooperation between the blood and plasma sectors is important to ensure that the best community outcomes are achieved including sufficiency of supply for patients.

3.1 Activities undertaken to support plasma collection should not compromise the ability of a nation or a region to collect adequate supplies of blood components to meet clinical needs.

3.2 Similarly, activities undertaken to collect or promote adequate supplies of blood products should take into account the ability of those who collect plasma for fractionation to meet the requirements of patients who rely on these therapies.

3.3 Organizations involved in whole blood and plasma collection should cooperate with the goal of ensuring the health of the donor and potential blood component and plasma product recipients.

3.4 The manufacture of blood components and plasma products to treat patients with very rare diseases should be welcomed and actively supported by all those that operate in the blood and plasma sectors.

3.5 All stakeholders in the blood and plasma sectors have the right to hold and express opinions and should treat each other with mutual respect.

4. Global utilization of donated blood and plasma

The products of the blood and plasma sectors are sometimes not needed to meet the blood and plasma product needs in that particular region. This is because a number of different products can be produced from a single fresh or plasma donation. Many regions lack the capacity to collect and produce all the blood products they need, so they are reliant on blood or plasma donated in another region. Donors expect their blood or plasma to be used to benefit patients who need blood and plasma products.

4.1 The needs of patients should determine the optimal collection of blood and plasma.

4.2 The Blood and plasma sectors have an obligation to donors to make their best endeavours to use that blood or plasma for the purposes for which it was donated.

4.3 Having satisfied the principal purpose for its collection, blood components, plasma and plasma intermediates not required for that purpose should be made available to meet the health needs of others and contribute to global health outcomes where feasible. Feasibility includes whether the costs of provision are able to be met and if the regulatory regime and healthcare systems in both regions supports availability.

4.4 Regulation of the collection and use of plasma for manufacture should be based on science and the precautionary principle, and facilitate global movement of products when safe and appropriate to do so.

About PLUS

PLUS is a coalition of 7 patient organisations, including EHC which was established in March 2009. The European Commission and other institutions indicated that they were more amenable to a regular consultation process with a coalition of organisations who represent constant or regular users of plasma products than they would be to such a process with each individual organisation. PLUS has succeeded in agreeing a regular consultation process with the EU Commission on vital issues relating to blood and plasma.

The Terms of Reference of PLUS are as follows:

- To facilitate the exchange of information towards the building of consensus views when possible among the organisations that represent regular users of plasma, plasma proteins or plasma derived therapies

- To ensure that the consensus views of the organisations are communicated to the EU Commission, MEP's, the Council of Europe and other relevant bodies and individuals.

- To ensure that the collective views of the organisations are proactively considered on a timely basis when Directives, Guidelines and recommendations are being framed in relevant areas

- The collective views expressed on behalf of the organisations in no way detract from each organisation's ability or right to express their own individual view on any particular issue. PLUS will express the collective view on an issue when agreed.

The Organisations who constitute PLUS are:

- European Haemophilia Consortium (EHC)
- World Federation of Hemophilia (WFH)
- International patients Organisation for Primary Immune Deficiency (IPOPI)
- Alpha 1 anti Trypsin Organisation (Alfa Europe)
- Guillain Barre organisation (GBS/CIDP Foundation)
- International Patient Organisation for C1 Inhibitor Deficiency (HAEI)
- Idiopathic Thrombocytopenic Purpura association (ITP)

Brian O Mahony
PLUS Steering Group
EHC Steering Committee

Baxter

Baxter Bioscience starts a panel of patients with inhibitors

As a leading manufacturer of recombinant and plasma based proteins to treat hemophilia and other bleeding disorders, Baxter aims to continuously improve the treatment of hemophilia patients.
For this reason Baxter aims to establish a sounding board for patients with inhibitors in Europe to understand the needs as well as the tools and services required for raising the standard of care.

> ***About inhibitors:*** *Factor VIII for hemophilia A and factor IX for hemophilia B are treatments temporarily replacing the clotting factor which is missing from the blood, thus helping to control bleeding. However, in some cases, the treatment does not work as it should because the person develops antibodies against the clotting factor, called inhibitors. Antibodies are a response of the immune system which attacks substances that the body perceives as foreign. For a person with hemophilia, the antibody may appear following treatment with factor VIII or IX because the body recognizes the factor as a foreign protein. These antibodies attach themselves to the factor and inhibit its ability to stop the bleeding. People with hemophilia having developed inhibitors will not necessarily bleed more often or for longer periods, but when a bleeding does happen it is more difficult to treat.*

In 2008, the 'Hemophilia Principles of Care' were published in Hemophilia. This document sets the principles for comprehensive hemophilia care in Europe. The document is organised as a ten point statement outlining the needs of people with hemophilia in Europe, followed by the explanation of each principle, with references to key supporting literature. The aim of these principles of care is to persuade European and national healthcare policy makers to take strong and decisive action to ensure that people with hemophilia have access to safe treatments and optimum care throughout Europe.

Despite the success of these principles of care, focus is still required to optimize therapy for patients with inhibitors.
By establishing a panel of patients with inhibitors and/or their caregivers, besides giving patients of various nationalities the opportunity to meet and share experiences, Baxter aims to understand the specific issues/needs for these patients and ensure that they are better represented under the Principles of Care.

The first meeting will take place in London, starting Friday June 11, 2010 and ending Sunday afternoon, June 13. The plan is to have two panel meetings every year.

Patients with inhibitors and caregivers interested in participating in this panel should contact Maureen Miller from Compass Healthcare Communications, at +1 877 377 9730 or by e-mail: mmiller@compasshc.com.

Wyeth is Now Part of Pfizer

On October 15, 2009, Pfizer completed its acquisition of Wyeth, and on October 16, 2009, the two companies began operating as one. The merger of local Wyeth and Pfizer entities may be pending in certain jurisdictions. In those instances, integration of our two businesses is subject to the completion of various local legal and regulatory obligations.

Pfizer Inc: Working Together for a Healthier World™

At Pfizer, we apply science and our global resources to improve health and well-being at every stage of life. We strive to set the standard for quality, safety and value in the discovery, development and manufacturing of medicines for people and animals. Our diversified global health care product portfolio includes biologic and small molecule medicines and vaccines, as well as nutritional products and many of the world's best known consumer health products.

Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. To learn more about our commitments, please visit us at www.pfizer.com.

Flexible, Patient-Centric Global Operating Structure

With the addition of Wyeth products, Pfizer now has a more extensive portfolio of biotechnology products, vaccines, and consumer health products. To better manage our expanded product portfolio, Pfizer has established patient-centric business units that are intended to match the speed and agility of small, focused enterprises with the benefits of a global organization's scale and resources. One of those business units is Specialty Care, which includes the hemophilia business built by Wyeth and started by Genetics Institute (In 1992, Wyeth acquired a majority interest in Genetics Institute.).

A Legacy in Hemophilia

At Pfizer, we are proud to have the opportunity to build on a long-standing history of leadership in the hemophilia community. This legacy in hemophilia spans decades: from the beginnings of our hemophilia business as Genetics Institute – where the work of pioneering and visionary scientists led to the discovery and development of the first recombinant antihemophilic factor for the treatment of hemophilia A – to many years of growth as Wyeth, and now, to a new chapter as Pfizer.*

Starting a New Chapter

Over the past several months, many Wyeth employees were involved in the integration planning process for the structure and operation of the combined company. This process allowed us at Pfizer to learn about Wyeth's long-standing history in hemophilia, and will help us to make sound decisions about our work within the hemophilia community. Like Wyeth, we at Pfizer share an incredibly strong passion for innovation and patient care, and are excited to extend that passion to the hemophilia community.

**Product was manufactured under a shared manufacturing agreement between Genetics Institute and Baxter Healthcare Corporation.*

EU HEALTH POLICY UPDATE

Commission delayed in the on the establishment of an EU Committee of Experts on Rare Diseases

The European Commission decided on 30 November, to establish a European Committee of Experts on Rare diseases (EUCERD) to assist the Commission in formulating and implementing the Community's activities in the field of rare diseases, and to replace the current European Union Rare Diseases Task Force.

The delays in setting up a new Commission, particularly due to the signing of the Lisbon Treaty has slow down the process of the adoption of a Commission Decision on the appointment of the members of the European Union Committee of Experts on Rare Diseases. As a consequence the first meeting of the EUCERD which was planned for May has now been cancelled until further notice.

For further information please see:

European Commission website, EU Committee of Experts on Rare Diseases:
http://ec.europa.eu/health/ph_threats/non_com/keydo_rare_diseases_en.htm

Call for expressions of interest for patient organisations:
http://ec.europa.eu/health/ph_threats/non_com/docs/call_patients.pdf

Calls for tender in the area of public health

The Executive Agency for Health and Consumers has published eight calls for tender in the area of public health.

The eight Calls for tenders are as follows.

- Multiple framework contracts with reopening of competition to support the **Health Information Strategy**.
- The identification and development of tools aiming at facilitating **recognition of prescriptions** from another Member State.
- **Training transplant donor coordinators** trainers.
- **Mental health systems** in European Union Member States, status of mental health in populations and benefits to be expected from investments into mental health
- The creation of a mechanism for the **exchange of knowledge** between Member States and European authorities on the scientific assessment of the **clinical added value for orphan medicines** : the aim is to:

- describe the regulatory process followed by an orphan medicine, from orphan designation at the European level to reimbursement in the Member State and examine to what extent the information produced by the authorities responsible for orphan designation and MA is directly useful for the medicine reimbursement decision process;
- describe the HTA expertise (focusing on relative efficacy and relative effectiveness) used at national level for this purpose and level of involvement of existing international Health Technology Assessment networks.
- describe what expertise is used when the medicine is prescribed to all the targeted population of patients affected by a certain rare disease.

- The preparation of a report on **health inequalities** in the EU Multiple framework contracts with reopening of competition on external assistance concerning activities in the area of heath security.
- The impact of **structural funds on health gains**.

To access all information about the calls please see: http://ec.europa.eu/eahc/health/tenders.html

The European Commission Work Programme for 2010

The European Commission adopted its work programme for 2010 on 31 March. The work programme focuses on addressing the challenges of the economic crisis and delivering policy that bring direct benefits to citizens.

The Commission has agreed a list of 34 strategic priorities that will be implemented before the end of the year. It has also agreed another 280 major proposals under consideration during 2010 and beyond. The Commission work programme sets the scene for the major policy commitments that the Commission will make in the coming years.

The new Commission focuses on four key areas:

- Tackling the crisis and sustaining Europe's social market economy
- Building a citizens' agenda which puts people at the heart of European action
- Developing an ambitious and coherent external agenda with global outreach
- Modernising EU instruments and ways of working

For further information please see:

The Commission Work Programme for 2010:
http://ec.europa.eu/atwork/programmes/docs/cwp2010_en.pdf

FDA and EMA agree on Single Orphan Drug Designation Annual Report

On 26 February 2010, in the context of the Rare Disease Day, the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) announced their agreement to submit a single annual report from sponsors of orphan products (drugs and biologics) designated for both the US and the EU.

The submission of a Single Report is made on a voluntary basis, and is only applicable to sponsors who have obtained an orphan designation status for their product both in the EU and US. Therefore, since 28 February 2010, sponsors have the option to send a single Orphan Drug Designation Annual Report to each Agency, instead of submitting two separate reports.

This new collaboration provide regulators with improved and shared information throughout the development process of an orphan product."We are very pleased with this collaboration on regulatory requirements and about sharing data that will help us understand the viability of the products," said Jordi Llinares, MD, MSc, Head of Orphan Medicines at the EMA. Professor Kerstin Westermark, Chair of the EMA Committee for Orphan Medicinal Products declared: "This new step in our collaboration provides each of our agencies with information in real time on any challenges arising during the development of products for rare diseases and will help identifying and acting on bottlenecks."

The benefits of submitting an annual report are also clear for sponsors, as it will help them reduce bureaucracy, and avoid work duplication.

For further information please see:

The EMA Press release:
http://www.ema.europa.eu/pdfs/human/comp/pr/12184610en.pdf

The tenth anniversary of the European Orphan Drug Regulation

Experts estimate that there are between 6000- 7000 rare diseases. These are diseases, that occur with a prevalence of less than 1 in 2000 inhabitants. Worldwide one in 5000 new born boys is suffering on a haemophilia. So the haemophilia belongs to the rare diseases. The number of affected people with a rare disease has a range between some hundred of thousand (like haemophilia) till some hundred or less. It is estimated that there is a therapy only for about 10 percent of the rare diseases. In Europe over 2,6 million people suffer on these diseases, many of them are children and newborn babies.

To improve the treatment options it is necessary to get the pharmaceutical industry interested in the research, development and production of drugs. These drugs are called "Orphan drugs". The industry will only invest money in new drugs if they see a market and the chance to make a profit. To support a better supply with "Orphan drugs" the European Commission passed the European Orphan drug regulation in 2000. The leadership in this project has the European Medicines Agency (EMA), which is located in London. The EMA is responsible for the European legislation of drugs.

Companies which are developing drugs for the treatment of rare diseases get financial support and technical advices. Companies can get a marketing authorisation for ten years for the developed drugs. This makes it easier for companies to calculate their financial options.

Over 120 participants from industry, research, government and patient groups celebrated the tenth anniversary of the European Orphan Drug Regulation earlier this month in a two-day event in London.

They were able to see a very good result of the last ten years. Over 1100 applications have been received, 720 orphan designations have been granted and 62 new Orphan drugs have been approved by the EMA for use in the EU. This gives a treatment option for 53 different rare diseases.

One can imagine that especially the performance of trials is a great challenge. The testing of a "normal drug" involves some hundred patients, but with a patient group of less than hundred, this way is not possible. Often there are only some patients in a country, so the international exchange of experience and knowledge is necessary. As in every normal drug efficacy and safety of Orphan drugs must be given for sure. The post marketing surveillance is very important in this way. A very important role plays patient registry in this context. Only this way you are able to get long time results and adverse affects of the new drug. Representatives of the European EMA and the American FDA underlined the

necessity of a close collaboration. One participant of the meeting descried it with the words, that "diseases even don't know borders".

In a workshop representatives from patient organisations discussed the experiences. In the board of the Committee for Orphan Medicinal Products (COMP) are 3 representatives of patient organisations. All agreed that patients must be involved in the process of making decisions on national and international level. This was a common position in all given lectures during the meeting. The main point discussed by the patient organisations was the access of patient to the drugs. Every development and application of a drug makes only sense if patient can get treated by them. A good example where problems still exist is the haemophilia. For this disease there is a good diagnosis and a very effective treatment with plasma derived and recombinant factors available. Nevertheless, only 20 –25% of all patient worldwide have access to this treatment. Even in rich Europe there are enough countries where the treatment is not performed at the state of the art.

All representatives of the patient organisations agreed, that even during this current financial crisis they should not be divided but act together.

To continue this very successful work of the last ten years on the field of Orphan drugs, further steps must be taken. The EU has called all member states to develop national action plans for rare diseases.

Dr. Uwe Schlenkrich

EHC Steering Committee Member

EHC Forthcoming Events

EHC Roundtable of Stakeholders

The next EHC Round table will take place in Brussels on the 2 June in Stanhope Hotel in Brussels and will focus on "Prophylaxis for Adults".

We look forward to seeing many of you there!

The 23nd EHC Annual Congress: 22-24 October 2010, Lisbon - Portugal

The 23nd Annual European Haemophilia Consortium conference will take place on 22-24 October 2010 at the Hotel Altis in Lisbon, Portugal.

The Conference will consist of plenary lectures and presentations in which a broad spectrum of scientific and practical issues and problems in Haemophilia care will be discussed. It will be an excellent possibility to share ideas, knowledge and recent scientific achievements.

Other Events of Interest

The fifth European Conference on Rare Diseases, 13-15 May 2010, Krakow, Poland

For more information on the Congress, please click here:
http://www.rare-diseases.eu/2010/index.php
http://img.rarediseaseday.org/polka/polka_brochure_final.pdf

EPF conference which will focus on Health Technology Assessment, 18th May, Brussels

European Patient Forum AGM will take place in Brussels on May 19 in conjunction with the EPF Health Technology Assessment Seminar on May 18. Participation upon invitation only.

For more information, please click here:
http://www.eu-patient.eu/Initatives-Policy/Events/Annual-General-Meeting-2010/

17th IPFA/PEI Workshop on "Surveillance and Screening of Blood Borne Pathogens", 26-27 May 2010, Zagreb, Croatia

This annual workshop, jointly organised by the International Plasma Fractionation Association (IPFA) and the Paul-Ehrlich-Institut (PEI) has become widely recognised as an authoritative international scientific meeting for discussion of developments concerning the microbiological safety of blood and blood products. The meeting attracts internationally recognised experts from blood services, regulatory agencies and industry.

In collaboration with the Institute of Immunology, Croatia.

For further information please visit: www.ipfa.nl, section "Events"