PHA EUROPE POLICY BRIEF AND CALL TO ACTION LAUNCH EVENT IN THE EUROPEAN PARLIAMENT

Brussels, June 6 2012
Dear friends,

I am delighted to introduce the Summer 2012 edition of Mariposa News, PHA Europe’s official journal. The past six months have been very busy for PHA Europe and its members with many new and interesting developments.

On Rare Disease Day, FEBRUARY 29, PHA Europe successfully launched its second pan-European “Breathtaking” awareness campaign, under the patronage of EURORDIS, with events organized by the members associations in 17 European countries. The Spring edition of Mariposa contains detailed reports and photos of the PHA Europe campaign.

On MAY 5 the first ever PULMONARY HYPERTENSION WORLD DAY was celebrated in Madrid. This date will mark a very important milestone for the PH community worldwide. The PH World Day received the endorsement of PH associations from five continents, of patient organizations related to PH as well as rare disease federations and scientific societies and organizations worldwide. The event had an outstanding scientific programme and a very prestigious panel of speakers. All the activities were lead and organized by the Spanish Pulmonary Hypertension Association ANHP in the course of many months of hard work on a national and international basis.

On JUNE 6 PHA Europe officially presented its POLICY BRIEF and CALL TO ACTION to the EUROPEAN PARLIAMENT in Brussels. The very successful launch event was hosted MEP Karin Kadenbach with the support of MEP Hannes Swoboda. The Call to action is the result of almost a year’s work of a Roundtable of PH Experts representing clinical, patient, research, and industry interests. Speakers at the event included Roundtable members Nazzareno Galiè (University of Bologna, Italy), Simon Gibbs (Imperial College, London), Giuseppe Nicola (University of Bologna, Italy), Jean-Luc Vachiery (Erasme, ULB, Belgium), Paola Testori Coggi, Director General of DG SANCO (Health and Consumers) and Laurent Nicod, Scientific Committee Chair at the European Respiratory Society.

On JUNE 22-24, PHA organized its 10TH INTERNATIONAL PH CONFERENCE in Orlando. The Conference featured the Second World Leaders’ Summit as well as many new programs for global patients, PH organization leaders and medical professionals. Over 1,500 patients, caregivers and medical professionals from five continents attended, including a number of PHA Europe members. In the past months PHA Europe representatives have taken part in other important events such as the EUROPEAN CONFERENCE ON RARE DISEASES and EURORDIS ANNUAL MEETING in Brussels on MAY 23 to 25 and the 6th INTERNATIONAL NURSE WORKSHOP in Barcelona on JUNE 8 AND 9. Reports of these meetings are included in the section of this journal which follows the international PH association-led events.

PHA Europe’s member associations have also been very active at national level and I would like to thank them for sharing their achievements with us and providing precious input for the National Activities section of Mariposa News. Looking ahead to the coming months, PHA Europe will be present as an exhibitor at the Annual Congresses of the EUROPEAN SOCIETY OF CARDIOLOGY (Munich, AUGUST 25-29) and the EUROPEAN RESPIRATORY SOCIETY (Vienna, SEPTEMBER 1-5).

Preparations are well under way for our GENERAL ANNUAL MEETING which is taking place in Barcelona SEPTEMBER 12-16. Over 40 PH patient leaders from 22 countries have already confirmed their attendance. The meeting has a very wide ranging program with interactive sessions about issues of common interest. Our medical guest speaker this year is Prof. Simon Gibbs, one of the world’s leading PH specialists. Another important event coming up later on in the year is the ESC EDUCATIONAL COURSE ON PULMONARY HYPERTENSION which will be held in Sophia Antipolis, France, OCTOBER 18-19. It is a great honour for me to have been invited to present the patient perspective within the context of the Annual “G6” which brings together the main stakeholders involved in PH patient care: physicians, nurses, industry, regulatory authorities, policy makers and patients.

Thank you all again for your support and collaboration. It has been a very successful year so far and I look forward to seeing you in Barcelona to share our achievements and discuss future projects.

Gerald Fischer
President PHA Europe
Karin Kadenbach
Member of the European Parliament

Karin Kadenbach MEP opened the event and reminded participants of its objectives, i.e. to introduce the Pulmonary Hypertension Policy Brief and Call to Action, to raise awareness of the urgent need for health policy, health services, and research to take account of pulmonary hypertension and to explore the possibility for EU and national level action. Mrs Kadenbach stated that she was struck by the fact that, despite the obviously serious nature of the disease, awareness is very low. She expressed her hope that this meeting would contribute to increased awareness and more suitable policy and research at EU as well as national levels. One of her reasons for hosting the event is that pulmonary hypertension (PH) is a forgotten disease. However, a variety of initiatives on the current EU health agenda can help bring about a greater focus on PH; Mrs Kadenbach MEP and co-host Hannes Swoboda MEP can be helpful in this respect.

Jean-Luc Vachiéry
Cliniques Universitaires de Bruxelles

The first speaker, Jean-Luc Vachiéry, addressed the issues in relation to pulmonary hypertension. High blood pressure in the lungs, i.e. pulmonary hypertension, kills silently, rapidly, and surely. The right side of the heart (connected to the pulmonary arteries) has to pump two to five times more than the left side (attached to the aorta). There are five PH subtypes, some of which are rare, i.e. pulmonary arterial hypertension. The disease is unknown to the public and barely known by many health care professionals. In terms of diagnosis and prognosis, it takes around two years between the onset of the symptoms and diagnosis, largely because there are no specific symptoms. However, the consequences of a delayed diagnosis can be dramatic. Fifty percent of patients die if the disease is left untreated. Furthermore, beyond the symptoms and the disability, PH completely changes patient’s lives.

In terms of therapy, PH is still an incurable disease. The only current cure is lung transplantation. Therapies can improve symptoms, quality of life and extend life by several years. However, the price to pay for efficacy lies in side effects and complexity.

Prof. Vachiéry concluded by underlining that PH is a time bomb. Time matters: every moment counts to detect the disease in time, to provide appropriate therapy and care, to decide when therapy should be changed, and to determine when transplantation is needed.

Simon Gibbs
Imperial College of London

The second speaker, Simon Gibbs, emphasised that too many PH patients receive poor care. Clinical experience and vigilant patient care can mean a longer and better life for patients and their families. The best way to provide this
A CALL TO ACTION ON PULMONARY HYPERTENSION

PAOLA TESTORI COGGI
European Commission, Director General DG SANCO

Paola Testori Coggi explained that the EU health remit is very limited; Member States are responsible in this area. However, the Commission takes many initiatives that complement Member States’ activities and priorities, such as:

1. Chronic diseases feature high on the current Commission agenda, and this is supported by the European Parliament. In general, Member States do not invest enough in promotion and prevention.
2. The Commission also stimulates awareness-raising and knowledge dissemination through information campaigns; it helps to identify the health needs of the EU population.
3. Serious cuts in health budgets are occurring across the EU to keep health systems sustainable. The Commission works with health ministers to ensure greater efficiency in health care provision rather than just reducing spending. Making systems more efficient would also help reduce costs. For instance, more care delivered in home settings, better data analysis, and better use of E-Health would be part of the solution.
4. Making better use of the health workforce is another issue; it is already known that within the next 10 years, there will be a shortage of some 100,000 health staff. Providing the right services will be key, and training for these services will be essential.
5. The new research programme, HORIZON 2020, will have a substantially larger budget, and research on health will be part of this programme. This will focus on information and data collection on the prevalence and incidence of diseases and conditions, including pulmonary disease.
6. Paola Testori Coggi also recalled that the EU has been quite successful in the area of rare diseases. For instance, there are registries for rare diseases which are supported financially by the EU. In relation to orphan drugs, many drugs have now been authorised (70 drugs treating 60 rare diseases).

Key words in all of the Commission’s health activities are “disease prevention” and “health promotion”.

PISANA FERRARI
Vice President PHA Europe

In her presentation, Pisana Ferrari outlined four main challenges still facing PH patients:

1. Improved awareness and screening
2. Medical challenges: There are delays in diagnosis or misdiagnosis as well as inequalities in access to treatments across the EU. Reimbursement for treatment can be problematic. There is a lack of expert centres and surgery facilities (FEA, lung transplant), and waiting lists for transplants are long. Despite progress and better medicines, the prognosis for PH is still very poor. Ms Ferrari underscored that much remains to be done and that patient associations are the stakeholders best positioned to understand the needs and priorities of patients. Finding a cure for PH would benefit not only the PH patient, but society as a whole.

GERALD FISCHER
President PHA Europe

Gerald Fischer briefly introduced PHA Europe as an umbrella organization of 25 PH patient organisations located in 22 European countries. He then formally launched the Call to Action and provided a flavour of its content. The Call to Action has involved all key PH stakeholders: medical professionals, nurses, researchers, representatives of the health industry, and leaders of PH patient groups around the world. It addresses the five main unmet needs of PH patients, i.e.:
1. Improved awareness and screening
   Awareness is needed to make the patient conscious of the fact that breathlessness can be a sign of a severe condition. Primary care physicians have to be informed about specialised centres for PH. Screening programmes are needed to identify and diagnose at-risk patients to improve patient outcomes; physicians must know about the different subtypes of PH.
2. Centres of expertise
   As PH is difficult to diagnose for non-specialists and as approved treatments are limited, expert centres with specialised physicians are essential for proper diagnosis and appropriate care. E-Health can help to establish important links between the centre and the nearby doctor. Treatments prescribed by centres of expertise must be available at affordable costs for every patient. Improved donor legislation will ensure easier access to transplantation.
3. Psychosocial support
   Many patients feel isolated and alone after a PH diagnosis. Some treatments are complex to manage. Some patients suffer from depression and consider suicide. Therefore, there is a need for integrated care systems which include psychosocial, nutritional, and rehabilitative support.
4. Empowered patient groups
   Patient groups provide a wide range of supportive services and are the voice of patients and carers. Most patient groups are run by people living with or affected by PH. The patient perspective should be integrated by involving patient groups in health policy debates and decision-making. Financial support for patient groups should be encouraged.
5. Clinical research and innovation
   There are approved drug treatments only for PAH,
none for other forms of PH. Diagnosis is invasive and stressful. Endpoints are still very vague and clinical trials are the only option for some patients. Therefore, until a cure is found, patients need clinical trials in PH subtype other than PAH, more paediatric studies, and non-invasive diagnostic tests. Finding a cure is the most important need.

Mr Fischer concluded by saying that the recommendations in the call to action do not ask for increased financial support, but rather a reallocation of existing resources.

LAURENT P. NICOD

Scientific Committee Chair, European Respiratory Society

The next speaker, Prof. Laurent P. Nicod (Scientific Committee Chair, European Respiratory Society) briefly introduced ERS as the leading professional respiratory medicine organisation in Europe, which aims to alleviate suffering from respiratory diseases and promote lung health through research, sharing of knowledge, and medical and public education. The organisation launched a EUROPEAN RESPIRATORY ROADMAP in 2011, which includes some key recommendations for policy makers for the future of respiratory medicine under four headings: prevention, clinical research, sharing of knowledge, and medical and public education. Prof. Nicod stated that one of the most important issues is the need for public awareness, and whether there is an underlying condition (e.g. COPD).

Likewise, continued support for clinical research and innovation was vital. There is also a need to optimise care to improve patient outcomes. Research efforts need to be another indicator. Others named increased and sufficient funding for patient groups; the EU could perhaps play a role in this respect. However, it was pointed out that funding of national patient organisations is a national responsibility rather than an EU one.

How do we raise awareness of PH?

The EU has limited powers within the field of health. Nevertheless, the Parliament can help to raise PH awareness by means of health-related topics on the EU policy agenda, e.g. the TRANSPARENCY DIRECTIVE, the HEALTH FOR GROWTH PROGRAMME, the CHRONIC CONDITIONS REFLECTION PROCESS, and ACTION ON RARE DISEASES. All these initiatives will need to be scrutinised for the effect on patients and health care. MEPs can table amendments that can render PH more visible, even if they are not adopted in the final text. Today’s event is important as it has given the condition a face. The Call to Action is a useful tool as it comes forward with concrete recommendations. It gives guidance to policy-makers.

There is a need to implement existing programmes. The EU has taken many initiatives in the field of rare diseases. The National rare disease plans are in place in most of the EU member states. However, these are not being implemented in many cases, largely due to the current economic crisis. It would be useful for the EU to exert pressure on member states to implement these plans. Yet again, this boils down to national competence. Changing governments and short-term policy aims often stand in the way of progress.

There are huge differences between the various Member States; there are countries less advanced in medicines, States; there are countries less advanced in medicines, where we do not know as much as is needed to understand current activities. The EU could help to bring these countries into a larger network where exchange of experience and good (care and research) practice could be facilitated. The EU COMMISSION has a clear role to play here.

Medicines development

While it is true that progress has been made in the field of drug development for rare diseases (with 70 authorised drugs for 60 diseases), there are some 8,000 different rare diseases. The industry’s success rate in developing effective drugs is about 10 percent. Something needs to be done to improve the outcome of research: 90 percent of PH remains untreated and PAH medicines address only one subtype. There are immense inequalities in terms of access to drugs between and within member states. A huge task lies ahead for member states to improve access to treatments. In this respect, the EU has made some small steps forward, e.g., the DIRECTIVE ON CROSS-BORDER HEALTH CARE. This affords the patient the legal certainty that they will receive treatment.

DRUG AUTHORISATION AND CLINICAL TRIALS

The precautionary principle is applied very strictly in this field; certification and verification are key words. While this principle may be applied too stringently on occasion, the safety of drugs remains paramount. There is no single or simple answer. Even if drug approval is centralised, reimbursement is the remit of the Member States. Access to treatment should follow central authorisation. However, while access to medicines should be improved, so should training of health professionals.

Karin Kadenbach MEP closed the meeting by thanking all speakers, participants, and organisers and once again offered her support in putting PH on the EU policy agenda.

Christine Marking
**Official celebration of World Pulmonary Hypertension Day**

*Madrid, 5th of May 2012*

The first PH World Day was celebrated in Madrid on the 5th of May 2012. This date will mark a very important milestone for the PH community worldwide. The PH World Day received the endorsement of PH associations from five continents, of patient organizations related to PH (e.g. scleroderma) as well as rare disease federations (EUROKIDS, ORPHANET, NORD) and scientific societies and organizations worldwide (including the EUROPEAN RESPIRATORY SOCIETY, the AMERICAN THORACIC SOCIETY, the EUROPEAN LUNG FOUNDATION). The event had an outstanding scientific programme and a very prestigious panel of speakers. All the activities were lead and organized by the Spanish Pulmonary Hypertension Association ANHP in the course of many months of hard work on a national and international basis.

The Official Opening Ceremony on the 5th of May featured presentations from some of the most distinguished international experts in the pulmonary hypertension field, who offered a summary of the main historical and scientific developments in this field.

**OPENING SPEECH**

**IRENE DELGADO**

President ANHP, Spain

Good afternoon and thank you for being here with us to share this celebration that is so important for the PH community. Today is a very special day. For the first time we gather with the clear objective and intention to advocate at global level in favor of persons living with PH. In order to do it, patients and health professionals have joined hands to take a step forward in achieving that millions of persons have hope and a quality of life that today are denied to them or are jeopardized.

When I speak about patients I want it to be understood that I am speaking about all dimensions of the person, not only their physical affliction but also the social, familial, political, economical and emotional aspects as well as all those things that turn our lives into an experience full of emotions, tears, projects, achievements, disappointments and struggles to reach goals that have more or less impact but are important for each individual.

When I speak about health professionals, I am not only speaking about medical doctors, researchers, nursing staff, physiotherapists, psychologists, etc. I am also including politicians who decide with their policies whether we can access or not the treatments that save our lives or condemn us to disabilities and early death. I am also speaking about technicians who work in the regulatory bodies and all health agents regardless of their profession or specialty who contribute to making our life not only possible but also to offer us a dignified quality of life.

Although Pulmonary Hypertension has been known for many years and we have had an impressive development in the last 10 to 15 years, we are in the very first steps and we have only started the fight in a long battle where unfortunately nowadays in the world there are more victims than survivors.

But we have hope and we trust that we will grow stronger each day. Some of us had the privilege to be born in countries where access to health and better quality of life is easy because pharmacological treatments are not only marketed but also reimbursed. However, most PH patients literally suffocate, strive for each breath and die without knowing what has taken their future away.

This must be and actually is on its way to be changed. Patients come together into local and national organizations that in turn group in international organizations. We, the patients, have grown enough to support health professionals, promote research, facilitate communication and dialogue, and negotiate with health authorities the access to structural improvements that will offer to us the possibility to maintain us for some more years as active members of society, free of dependency and with strength to continue and work on a national and international basis.

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The Official Opening Ceremony on the 5th of May featured presentations from some of the most distinguished international experts in the pulmonary hypertension field, who offered a summary of the main historical and scientific developments in this field.

The inaugural speech was made by Irene Delgado, President of the Spanish Pulmonary Hypertension Association ANHP. The scientific speakers were introduced by the Leaders of the main world PH patient associations: the President of the European federation PHA Europe (Gerald Fischer), of the US association, PHA (Rino Aldrighetti) of PHA South Africa (Denneys Niemandt) and the Vice President of the Latin American federation SLHP (Cecilia Martinez).

The scientific symposium was declared of “National Health Interest” by the Spanish Ministry of Health, Social Services and Equity and by the Lain Entralgo Agency and was inaugurated by Dona Mercedes Vinuesa, Director General of Public Health, Quality and Innovation.
By pathologists, true pioneers for the identification of the disease. The German pathologist Ernst von Romberg was the first to describe pulmonary arterial sclerosis in 1891. He discovered atherosclerosis in the pulmonary artery and described the plaques. Von Romberg wrote about his findings in an article published in 1891. In this text von Romberg refers to previous studies by Corrado Tommasi Crudeli who was quoted to have described pulmonary atherosclerosis in 1868. I researched this and at the University Library in Bologna found the article by Tommasi Crudeli that it was published in The Rivista Clinica in 1868. It so happens that Tommasi Crudeli was an Italian scholar, from the University of Bologna!

Other pioneers for PH include Werner Theodor Otto Forsmann (1904-1979), an urologist, who performed the first right heart catheterization, on himself, in 1929, by using a urological catheterer. For his findings he won the Nobel Prize in 1956. Almost concurrently other discoveries related to PAH were made. In 1951, David Dresdale, an American cardiologist, first reported hemodynamic variables in cases of pulmonary hypertension without evident etiology, and coined the name “primary pulmonary hypertension” (now called idiopathic PAH). In 1958, Paul Wood, a UK cardiologist, hypothesized that there may be a vasoconstrictive factor in some cases of primary pulmonary hypertension. Prostacyclin was discovered by a Japanese researcher in 1988. Probably Dr. Salvador Moncada made both discoveries (he was a Fellow in both the studies). The endothelin pathway was discovered in 1982 and endothelin was first synthesized by a Japanese researcher in 1988.

World Congresses on PAH: The very first time PAH came to the attention of the general public was in the 70’s as the result of an outbreak of PAH due to the use of anorexiens. The first WHO (World Health Organization) Congress on PAH was held in 1973 and on this occasion the first classification was proposed. Three types of PAH were identified: plexiform, thrombotic and veno-occlusive. This time also marked the start of the NIH (National Institute of Health, US) Registry. The second WHO Congress was held in 1998 in Evian, then in Venice in 2003 and the last one in Dana Point in 2008. At each congress new things were developed and proposed: the five diagnostic classes in Evian, the treatment algorithm in Venice, the treat to goal management strategy in Dana Point. The classification adopted in Dana Point is still not used in the WHO.

WHO classifies PAH as “one of pulmonary diseases” under the current ICD 10 coding. This is now going to change. There has been a call to respond to go to ICD 11 and we have suggested that PH have its own code with the five diagnostic classes. We have recently received an official confirmation from WHO that the proposal has been taken on board as well as the five diagnostic classes. So when ICD 11 will be officially adopted PAH will have the correct classification. The next (5th) PH World Symposium is scheduled for Nice from the 27th of February to the 1st of March 2013. Twelve task forces have been set up. These include 145 experts who are already at work on 45 topics related to PH in advance of the symposium.

The future of Clinical Research: We have shown that PAH mediators are important outcome! This is (should be) the main endpoint. A metanalysis we have conducted in recent years has shown very clearly that PAH drugs can reduce mortality. One study, which has recently been completed, is, for the first time ever, based on long-term outcome (it is event driven, i.e. related to time to clinical worsening) and has had a positive result. Macienttan, the drug being tested, has shown a 45% reduction on clinical events. Outcome-based studies are the future of clinical research.

ORGANIZATION OF HEALTHCARE IN PH

MIGUEL ANGEL GOMEZ-SANCHEZ
Hospital, 12 de Octubre, Madrid

Why May the 5th for PH World Day? In Spain, in 1981, there was a dramatic epidemic of toxic oil-induced PH. On the 1st of May of that year an eight year old boy was the first person to die from intoxication. The epidemic, which was due to the use of adulterated colza oil, made over 20,000 victims. At that time the first PH unit was set up in my hospital. In 1990 we learned to use IV epoprostenol. PH is a disease which requires to be treated in expert referral centres. Treatments are complex, there is need to combine the treatments and reassess periodicaly, devices are hard to use, doctors need to be trained in hemodynamics, transplant and pulmonary endarterectomy are difficult and risky operations. It is a disease with low prevalence and high mortality. The cost is high, there is need to have access to multicenter...
COOPERATION BETWEEN PATIENT ASSOCIATIONS AND SCIENTIFIC SOCIETIES

Lewis Rubin
Emeritus Professor, UCSD

Now that there are many patient organizations all over the world the collaboration with scientific societies is all the more important. It is time to use the “power of people” to effect the necessary changes. This is important because despite progress there are still many unmet needs and the causes of PH are still not known.

Research grant support: in collaboration for research grants between patient associations and established scientific societies, last but is shared, which is very important. Ultimately the identification of pathways which will lead to a cure will depend on scientific investigation.

Scientific session presentations: the best way to generate interest on scientific work is to ensure that it gets a good spot at the key congresses. This is still a battle, we do not want to be relegated in a corner. Scientific societies should use leverage to get visibility at these meetings.

Education for scientists and practitioners: Many people around the world cannot get treatment and this is dramatic. But what also upsets me is that in so many countries diagnosis is still late. Physicians do not consider PH a high priority. Here again, patient associations and scientific societies can provide better techniques of education to physicians through:
- conferences and symposia;
- regional meetings;
- specialty meetings;
- consensus documents.

Local/global dialogue and accessibility: We now have numbers that allow us to influence governments and health authorities. There are still vast areas where patients have no access to care. Collectively we can influence industry and government to at least minimize inequalities. We need to work together to make this happen. The tremendous growth of interest groups in PH gives us the opportunity to progress together.

PULMONARY HYPERTENSION IN DEVELOPING COUNTRIES

Gerald Simonneau

National PH reference Centre, Antone Béclère Hospital

I am delighted to start my presentation by saying that there are many positive and exciting developments in this area.

- Economic growth is fast, especially in China, India and Latin America and reducing the burden of PH may become a realistic objective in the near future.
- Over the last ten years a number of referral centres have been set up (high-quality daily care, good basic and clinical research, high rate of inclusion in international RCTs).
- The World Health Organization Global Alliance Against Chronic Respiratory Diseases (WHO/GARD) has recently proposed a WHO Collaborative Center for Pulmonary Hypertension Education, Research and Care for a global and coordinated effort to fight chronic respiratory disease.
- There has been increasing participation of developing countries in multicenter randomized controlled clinical trials. Between 2000 and 2008 over 90% of patients recruited were either from the USA or Western Europe. In 2012, the largest multicenter trial to date (study on Macintenran), which involved over 700 patients, had a large percentage of patients from developing countries: 33% recruited in US and western Europe, 67% recruited in Asia, Eastern Europe and Latin America.
- We now have a lot of epidemiological data from developing countries (PH registries in Latin America, Eastern EU, Saudi Arabia, China). The most impressive one is China. A multicenter Chinese PAH Registry has included 966 adult patients between May 2008 and July 2011. On this subject, it is interesting to compare the characteristics of the populations studied. For example the differences in male-female sex ratio eg. there is a higher number of females in France than in China. There were also some differences with respect to hemodynamics (China being more severe) and distribution of etiology (idiopathic more frequent in US, congenital heart disease more frequent in China, US or Europe, familial and portal more frequent in France vs US and China).
- The role of expert centres will become more important as there are potentially more patients. The number of patients with pulmonary hypertension in developing countries is probably larger than in the developed world, due to some underlying causes that are not present in many developed countries.
- PAH associated with Shistosomiasis (parasite) For shistosomiasis it is estimated that 200 million people are infected worldwide, 120 million people present symptoms, 10 million have peripheral fibrosis and among these 10% could develop PH. Schistosomiasis is a leading cause of pulmonary hypertension in the infected areas (Brazil, Egypt…).
- PAH associated with Sickle cell disease Sickle cell disease is a common inherited hemoglobin disorder. It is characterized by chronic hemolytic anaemia and vaso-occlusion. Sickle cell disease affects 60,000 black people in the US, 10,000 French people (French Antilles). In sub-Saharan Africa the prevalence is as high as 30%. Worldwide, WHO has estimated that around 800,000 babies with severe forms of this disease are born each year.
- PAH associated with High Altitude 140 million people are living at high altitude (>2,500m). Chronic Hypoxia can cause increase in pulmonary pressure. High altitude pulmonary hypertension is a public health problem in the high-altitude areas of the world (Asia, South America). The precise prevalence is unknown. There is a wide variation in the susceptibility to hypoxia-induced pulmonary hypertension between different ethnic groups has been noted. Inhabitants of the Peruvian Andes are less susceptible than recent immigrants. The least susceptible populations are the Tibetans who have lived at high altitude for thousands of years.

The last year has witnessed major improvement in the awareness, diagnosis and management of Pulmonary Hypertension in the developing world, mainly due to the development of expert centers. Unfortunately, because of the high cost of novel PAH therapies (especially prostanooids and ERA), few patients can benefit from these drugs in emerging countries. The effectiveness of specific PAH therapies in patients suffering from Shistosomiasis, Sickle cell disease and high altitude PH is unclear and need further investigation.

Pulmonary Hypertension

Editorial Board.

For this publication, we would like to acknowledge the support of Medisimos, Roche, Actelion, United Therapeutics and Bayer.
Wednesday evening. This was a new networking opportunity that allowed international PH association leaders to connect and learn from each other alongside PHA’s Board of Trustees. The Second International Leaders’ Summit followed the next day, allowing the global PH community to share best practices in developing PH associations and building relationships. Panel discussions included: Patient Resources, Building Relationships in the PH Community, PH Advocacy and Awareness and Association Management.

REGIONAL INTERNATIONAL MEETINGS were held on Friday, bringing leaders and PH patients together to discuss topics important in their geographic regions. PHA Canada held a “Meet and Greet” for PH patients, families and medical professionals visiting from Canada, and the Sociedad Latina de HP hosted a networking event for the PH community visiting from Latin America. Friday’s global programming concluded with the International Reception for international PH association leaders and global PH physicians. This reception honored Dr. Julio Sandoval from Mexico City, the recipient of the first-ever International Physician Award. This prestigious award recognizes the accomplishments of a PH physician living outside of the U.S. who has made a difference for his/her patients and in the advancement of the PH field. Past Physician of the Year Award winner Dr. Michael McGoon from the Mayo Clinic presented the award.

With more awareness events happening around the world each year, the INTERNATIONAL STRATEGY MEETING provided a chance for the global PH community to discuss how to best leverage the awareness-raising efforts of PH associations around the world. The group convened on Saturday to review the existing awareness initiatives happening worldwide, and discuss possible areas of collaboration or mutual support. Chris McGrath from Queensland, Australia said, “The Conference has dramatically influenced my focus on the disease and shown me what we can be doing to raise awareness. I will be contacting those in the PH community regularly to get and share ideas”.

In addition to the other global initiatives taking place throughout the Conference, the Exhibit Hall featured an International Exhibit, highlighting PH resources available worldwide and providing valuable opportunities for connection.

HIGHLIGHTS FROM PHA’S CONFERENCE

With over 1,500 in attendance, PHA hosted more patients, caregivers, doctors, nurses and representatives from industry than ever before. Mary Fueston from Ohio noted that her favorite part of Conference was, “… seeing so many PH patients in one place and doing well. It was very uplifting to see patients who have out-lived their original diagnosis timeline and doing better than they were originally”. Take a look at some other Conference highlights: When people registered, they were given buttons with zebra stripes and began to see elements of Conference that...
For Medical Professionals:
PHA Fundamentals and Scientific Sessions will be available on PHA Online University, the premier resource for medical professionals to learn about screening, diagnosis and treatment of pulmonary hypertension, including FREE CME/CEU accredited courses, issues of Advances in Pulmonary Hypertension, PHA's quarterly medical journal, and regular webinars.

A TRANSITION...

At PHA's 2002 International PH Conference, 80 patients, caregivers and medical professionals from around the world came together to discuss how to more rapidly build the global PH community. At that time, two messages became clear. First, we needed to share best practices and, second, we needed staff help to make that happen. By 2004 PHA had raised the funds for a staff person who would collect and distribute information around the world. Allison Marian, Angie Zielinski (now Krotsch), Christine Diekler and, most recently, Meghan Tammaro held that position. These bright young people each made their own contribution to the development of our community and we are sorry to say that Meghan left us in late June for another opportunity. We will miss her.

She has been replaced by Julia Friederich. Julia has recently received a Masters degree in Medical Anthropology. She has international living experience, working in social programs in Ecuador and studying at Tsinghua University in China. Her language skills include Spanish, French and Chinese. In the late 1990s we were aware of three PH associations in the world. Today there are over 60. We welcome Julia to PHA and look forward to her service and contributions to the further strengthening of our community.

Conference Recordings Available Later this Year!
Missed PHA’s 10th International PH Conference and Scientific Sessions? Enjoyed a session at conference and want to share it with a friend or family member who wasn’t able to attend? For Patients and Caregivers:
Medically led and Patient and Family led sessions will be available on PHA Classroom, a vibrant e-learning center for patients and families to learn about PH through live e-learning events.

For Patients and Caregivers:
PHA’s new Early Diagnosis Campaign.

Check out PHA's Conference Highlights video to hear different perspectives of Conference from the PH community. As Desiree Rivera from Missouri puts it, the 10th International PH Conference and Scientific Sessions was truly, indescribable, "There are no words to describe the warmth I got from everyone. This will stay with me forever..." PHA looks forward to hosting the 11th International PH Conference and Scientific Sessions in Indianapolis, Indiana, June 20-22nd, 2014. For more information, visit www.PHAssociation.org. You can also find more Conference stories and links at PHA's blogs: Table Talk Wired and Empowered by Hope.

SPEAKER HIGHLIGHTS included Dr. Greg Elliott telling the medical history of PH and its evolving solutions...Colleen Brunetti in simple eloquence sharing the story of her journey as a patient with PH...13-year-old Matt Moniz (a National Geographic Adventurer of the Year in 2010) telling of climbing the nation's and the world's highest mountains to raise awareness and to honor his young friend, PH patient Ian Hess...Dr. Ray Beres (Allegheny General Hospital) relating his work and service to his family's immigrant roots...and, of course, the conversations in the halls and exhibit areas with so many patient, caregiver and medical heroes. All made the theme, The Power of One, very real. See: http://www.youtube.com/watch?v=j5A0dreSdcs

PHA hosted its largest Research Room to date, with over 225 participants. The Research Room, held at each biennial Conference, is dedicated to helping researchers further their studies by allowing for the collection of data, including biological specimens (cheek swabs and blood samples) from PH patients. This event gives researchers the rare opportunity to collect data from the largest gathering of pulmonary hypertension patients in the world at any given time. All nine research teams reported reaching their collection goals, with many exceeding their goals to happen. By 2004 PHA had raised the funds for a staff person who would collect and distribute information around the world. Allison Marian, Angie Zielinski (now Krotsch), Christine Diekler and, most recently, Meghan Tammaro held that position. These bright young people each made their own contribution to the development of our community and we are sorry to say that Meghan left us in late June for another opportunity. We will miss her.

She has been replaced by Julia Friederich. Julia has recently received a Masters degree in Medical Anthropology. She has international living experience, working in social programs in Ecuador and studying at Tsinghua University in China. Her language skills include Spanish, French and Chinese. In the late 1990s we were aware of three PH associations in the world. Today there are over 60. We welcome Julia to PHA and look forward to her service and contributions to the further strengthening of our community.
In his opening speech, John Dalli, European Commissioner for Health and Consumer Policy, highlighted the EU’s continuing engagement to improve the lives of the over 30 million rare disease patients in Europe. He believes that European co-operation on rare diseases can really make a difference, the difference between marginalisation and proper care. The fragmentation of knowledge about rare diseases and the small numbers of patients affected by a single disease makes it indispensable to work across borders. The European Commission’s work is to help bring together the scarce knowledge and resources fragmented across individual European Union countries and maximise synergies and results. This is what has driven the Commission over the last years and which has led to many different initiatives in the area of rare diseases:

- the 2008 Commission Communication on Rare Diseases puts forward a co-operation system between the Commission, Member States and various stakeholders.
- the Council Recommendation on Rare Diseases in 2009, by which Member States have committed themselves to adopting national plans or strategies for rare diseases by the end of 2013. The Commission developed technical assistance to help Member States create these national plans: the EUPLAN project and the 2012 Joint Action provide common guidelines.
- the Creation in 2010 of the EU Committee of Experts on Rare Diseases (EUCRED).
- the 2010 Council Directive on patients’ rights in cross-border healthcare provides a legal basis for establishing a system of European Reference Networks on rare diseases. Such Networks will comprise already existing centres with a high level of expertise, which would need to fulfil specific criteria to ensure quality and safety of the care provided. The Commission is currently working with the Member States in developing criteria, with a view to adopting them in 2013.

The EU has also supported rare diseases research for over twenty years:
- within the 7th Framework Programme for Research, the Commission allocated over 300 million euros to research on rare diseases. The Commission supports the development of Rare Diseases Registers, some of which are part of the reference networks.
- in December 2011, when the Commission adopted the Health Programme Work Plan for 2012, it set aside 4.5 million euros to help finance additional networks and rare diseases registries. Proposals are currently being evaluated.
- the EU is also part of the International Rare Disease Research Consortium, launched in 2011 (EU, United States and other countries) with the aim of delivering 200 new therapies for rare diseases and diagnostic tools for most rare diseases by 2020.

Rare diseases feature prominently also in the European Commission proposal for the new Health Programme and the new Research Programme for 2014 onwards. The EU Commission has proposed that the future Health Programme finances action on rare diseases, in particular exchange of information, rare diseases registries and European Reference Networks. These proposals are currently being debated in the European Parliament and the Council. As regards medicines, the EU has created specific incentives for pharmaceutical companies to develop new medicines for rare diseases in the framework of the Regulation on Orphan Medicines (2001).

Commissioner Dalli concluded by saying that much has already been achieved but more still needs to be done. “It is clear that, if we are to make inroads, we will have to work in tandem and maximise the value added of our actions”.

**EURODIS ACHIEVEMENTS AND PLANS FOR FUTURE**

It was very impressive to hear from EURODIS President Terkel Anderson about how active and successful the association has been in past years in terms of significant interventions in the field of patient advocacy, information and networking and support to rare disease patient associations. EURODIS membership has increased enormously and currently stands at over 500 members in 48 countries, with new alliances being set up in Russia. EURODIS staff of 23 supported by 59 volunteers. Mr Anderson stressed how action is all the more necessary in times of economic crisis in order to maintain solidarity. 2011 was a year of political concerns with some negative signals coming through and EURODIS must contribute to safeguarding the European social agenda. Yann Le Camn, EURODIS CEO, provided an overview of the association’s broad and ambitious program for 2012 which covers many different areas of action. With regard to advocacy, EURODIS will be involved in all the main EU health policy related issues currently on the table including, inter alia:

- Advocating for rare diseases within the context of the 3rd Health for Growth program, the 7th EU Research Framework Program and the new Horizon 2020.
- Participating actively in EUCRED, the European Committee of Experts on Rare Diseases (7 Eurodis members currently sit on this committee) to advocate for improving access to orphan drugs, for European Reference Networks and the setting up of Rare Disease Registries.
- Supporting the implementation of the EUCRED Joint Action (EJA), the aim of which is to work in partnership with EU member states and the European Commission to improve delivery of care to patients suffering from rare diseases.
- Promoting National Plans on RD: 20 new national conferences are planned for 2012-13 in the EU, five in additional countries - Russia, Ukraine, Serbia, Switzerland and Croatia.
- Advocating for better access to orphan drugs through CAVOD, Working Party for European collaboration on the scientific assessment of
the Clinical Added Value of Orphan Drugs and MoCa, the MECHANISM OF COORDINATED ACCESS (a platform for access to medicines involving doctors, industry, patient associations from 14 countries and the EU Commission) and initiatives on conditional pricing.

- Supporting the INTERNATIONAL RARE DISEASE RESEARCH CONSORTIUM (EURORDIS is a member of the Executive and Scientific committee on Therapies).

With respect to support to patient associations there will be action in the following areas:

- Organization of Rare Disease Day 2013 and of the first “Transatlantic” Forum on RD in collaboration with NORD, the US ORGANIZATION FOR RARE DISEASES and the Canadian RD group (Croatia, May 2013)
- Expansion of support programmes (seed money) and creation of a EURORDIS fellowship program.
- Expansion of RareCONNECT, the rare disease online community, of which pulmonary hypertension is also a part, with new features.
- Eurordis News will replace the previous newsletter, Russian has been added as 7th language and work has started on a Russian website.
- New initiatives to improve access to quality information through, for example, the relaunch of the EU helplines network (20 have signed up) and the application for 116 as EU-wide unique number.

PATIENT ADVOCATE CAPACITY BUILDING WORKSHOPS

The objective of the EURORDIS capacity building workshops is to give patient advocates the knowledge and tools to effectively work together in working groups at a national level. Six workshops were running in parallel and it was a difficult choice because all the topics were interesting. I attended the workshop “What are the recommended quality criteria for Centres of Expertise?”

Developing Centres of Expertise (CEs) and European Reference Networks in the field of rare diseases have been proposed in the COUNCIL RECOMMENDATION ON AN ACTION IN THE FIELD OF RARE DISEASES (2009) and more recently in the CROSS-BORDER HEALTHCARE DIRECTIVE (2010), as a means of organising care for the thousands of different rare diseases affecting patient populations across Europe. The CROSS-BORDER HEALTHCARE DIRECTIVE actually now gives the ERNs a legal basis (Recommendations are not legally binding for the Members States, Directives are).

EURORDIS first decided to address the issue of CE’s at its Membership meeting in 2006; in 2008 it submitted a position paper on the topic for the Commission and it has been advocating to impose the concept on the EU since then in a number of ways (eg, Polka project). EURORDIS, the European Expert Committee on Rare Diseases, in October 2011 adopted a set of Recommendations. The EURORDIS-recommended guidelines seek to introduce harmonious standards of quality practices by elaborating criteria for the Member States to incorporate in their process to designate Centres of Expertise (full text is available at www.eucerd.eu).

Kate Bushby, Vice President of EURORDIS provided a very comprehensive and interesting overview of the key points.

Centres of expertise (CEs)

- Mission and scope:
  - Definition and coverage: CEs are expert structures for the management and care of RD patients in a defined area, preferably national, and at international level if necessary. They will vary from country to country and will depend on size of territory.
  - Patient focus: CEs to collaborate with patient associations to bring the patient perspective, CEs to respond to the needs of patients from different cultures.
  - Core competences: Multidisciplinarity, including social services, palliative care, rehabilitation. CEs to contribute to building healthcare pathways from primary care and to have links to specialised labs and other facilities.
  - Role in spreading information: CEs to contribute to the elaboration of good practice guidelines, to provide education and training of healthcare professionals, to provide information and collaborate with other CEs, with RD Authorities, Orphanet etc.
  - Research: CEs to contribute to research, to improve the understanding of the disease and to optimise diagnosis, care and treatment, including the clinical evaluation of long-term effects of new treatments.
  - Criteria to designate centres covers:
    - Leadership and credibility: high level of expertise required which is measured, inter alia, by n° of patients, peer-reviewed publications, grants, positions, teaching and training activities, participation in clinical trials.
    - Muscular, inclusive: it will be necessary to demonstrate multidisciplinary approach and continuity of care.
    - Capacity: to improve delivery of care, to shorten time to diagnosis, to adhere to good practice guidelines, to ensure quality management.
    - Links and collaboration: at national, European and international level, collaboration with patient associations, referrals from other Member States.

Process of designation:

The Member States will establish the procedure and define the criteria. The designation will not only be valid for a defined period of time in order to allow for re-evaluation (quality based review).

The European dimension is important and it will be vital to network with Members States who have established centres of expertise in order to share experiences and quality indicators. Designated centres at Member State level are the key players in the European Reference Centers.

Where are we now? At the moment only France has designated centres within a national Plan on Rare Diseases. Others are designated outside a national plan.

Christel Nourissier, EURORDIS General Secretary and founding member of the FRENCH NATIONAL ALLIANCE FOR RARE DISEASES (Alliance Maladies Rares) spoke about the French situation.

France adopted a first National Plan for Rare Diseases which ran from 2005 to 2008. A second Plan is now under way (2011-2014). The French experience provides a very good example of the very influential role that patient advocates can play. The project for the 1st National Plan was managed by a Steering Committee of experts which included all stakeholders: the relevant Ministries (Health, Research, Social affairs), physicians, scientific agencies, insurance, industry. There was an interdepartmental coordinator whose role was to support a better articulation between health and social care. Patients were involved in working groups, in the Steering Committee and in the evaluation process of the 1st plan.

The 1st plan had some very important achievements: from 2005 to 2007 131 national CEs were designated at national level and 507 centres “of competence” at regional level, all connected to the CE’s. The designation was for five years. A working group was set up to establish the first evaluation process. It was decided that the centres should do a self-evaluation at three years and that there would be an external evaluation at five years by the Haute Autorité de Santé (HT agency).

The development of relations with patient associations was part of both evaluation processes (internal and external) and all the relevant patient associations were invited to participate by the experts at HAS for the external audit.

The process turned to be extremely successful in terms of patient involvement and empowerment. The centres of expertise were strongly encouraged to take the patient view and develop relations with the associations (they "had" to work in harmony but actually worked out well in practice.

Some centres even supported the establishment of patient organizations where there were none. The patient associations on the other hand did their “homework”: they had to collect the experiences of the patients (through phone calls, website, surveys, discussions). They made the best use of the information collected: they invited representatives of the centres to their meetings, included them in their Scientific committees and in turn we were invited to attend Board meetings at the hospitals.

Most patient groups are small and run by volunteers so it is difficult for them to be able to develop advocacy activities except through umbrella organizations. But they can interact with their centres of expertise.

The 1st French action plan had successes but also limitations. It carried a heavy workload and was very time consuming both for the centres and for the Haute Autorité de Santé. The outcomes of the evaluation were not used by the Ministry to reconsider designation and there was an 18-month delay between the 1st and 2nd plan. Therefore a working group has been set up to establish a less heavy evaluation process which will have more limited criteria and new terms of reference. One of the seven new indicators is integration with patient associations. The evaluation will be done annually and online, nothing has been decided yet for the external evaluation. A survey has been carried out on the results of the 1st action plan and it indicates that care has improved as a result of the establishment of centres of expertise (EUCERD website, national section at www.eucerd.eu).
The second presentation was about already established elsewhere in Europe. The case-to-case basis and may subsidize out of the regional authorities which can decide on a campaign did not change things unfortunately and Merck with the government Department for Health. The campaign was about access to KUVAN for PKU (Phenylketonuria) patients in Sweden and was presented by Mrs Anders Lundberg, from the Swedish PKU Association. PKU is metabolic disorder (there are different types) which, if left untreated, can cause severe brain damage. If discovered early there can be normal development. It is estimated that there are 200 PKU patients in Sweden. Since the 60’s PKU patients have been put on a very strict and burdensome low-protein diet to take Kuvan. Kuvan is a co-enzyme discovered in the late 90’s by a Japanese scientist. It obtained orphan drug status and was launched in 2008. In Sweden all drugs are subsidized by the state through the state authority TLV. TLV did not authorize Kuvan claiming it was too expensive in relation to the advantages it brings compared to the diet. They did not take into account the fact that the diet only “compensates” whereas the drug actually corrects the enzyme dysfunction. Merck, the company producing Kuvan, took TLV to court in 1st and 2nd instance. Both court decisions were negative and upheld TLV. Merck had excellent lawyers and did everything they could but to no avail. The Swedish PKU association (which is small and has a tiny budget) decided to get involved but did not have a standing in court as a party. It therefore decided to take action through a big awareness campaign which involved media outreach, calls to medical professionals, complaints with Ombudsman for Justice and for Children and meetings with the government Department for Health. The campaign did not change things unfortunately and Merck has also given up. The current situation is that there is no reimbursement. It is possible for patients to apply individually to the regional authorities which can decide on a case-to-case basis and may subsidize out of the regional budget. There is no chance of lowering the price as it already established elsewhere in Europe.

The second presentation was about MYELOMA PATIENTS FIGHT FOR REVLMID IN BELGIUM. Myeloma is a cancer of the plasma cells of the bone marrow. The speaker, Mrs Greette Goossens, is herself a patient and is part of the association CMP Vlaanderen (Belgian Flemish). Myeloma is a very serious disease, it is not curable but can be treated. In past years, since 2000, a number of novel agents have been introduced (Thalidomide, Velcade and Revlimid) leading to improved survival (from 24 months to several years). Revlimid was approved by national authorities in Belgium but with very strict and illogical criteria for access, not based on scientific evidence but only to save costs. In particular, the drug had to be discontinued if, after 4 cycles (4 months), the patient was not in remission (this time is too short for response which is known to be slow) and in any case had to be stopped after 8 cycles even if the patient was doing well! CMP, a small low budget patient group, decided to join forces with MyMu, the Belgian French-speaking Myeloma patient association. They started a joint advocacy and awareness campaign involving Belgian specialists (eg the Belgian hematological society), the European Myeloma Platform and Eurordis. They distributed letters, had articles published, organized meetings with key persons in the Belgian Ministries (Social Affairs and Reimbursement) and sought out Belgian politicians who had “equal health rights” on their agendas. Right from the beginning they were in communication with Gelgene, the company producing the drug, who eventually made some price adjustments. It took over a year but the campaign was a success and the Belgian state authority removed all the access criteria to the drug which is now freely available.

Marta Hanika Meutgeert, from VKS, the Dutch association for metabolic diseases spoke about the REIMBURSEMENT OF ENZYMES REPLACEMENT THERAPIES IN THE NETHERLANDS. The first ERT developed was for Gaucher (type I) in 1994, since then other ERTs have been developed for MPS I, Fabry, Pompe’s disease, MPS II and VI. ERTs are given by infusion (IV) once every week or two weeks. In most countries it is administered in hospital. Costs vary between 20,000 and 1,000,000 euros per year and the dose is weight-dependent (average of 170,000 euros per year). Insurance was privatized in the Netherlands in 2007 and it is compulsory to be insured. The relevant body, CVZ, decides what the appropriate care is for a given disease, according to state of the art and practice. If the treatment is judged to be of therapeutic value the insurance or government will pay. As we all know it is very difficult to evaluate efficacy with rare diseases: there are no metaanalyses and few randomized-controlled trials (the “gold standard”). Mostly drugs are approved on “exceptional circumstances”. All ERTs are re-evaluated after 4 years: in 2011 all treating physicians had to hand in their evaluations. Data on MPS I has been reviewed but there is no news yet for MPS II and VI, Fabry, Pompe etc. so there is total uncertainty. VKS is advocating for the development of a Dutch National Rare Disease Plan and are trying to access the right people in government and other institutes to share their view. A lot has been done but there is still more to do. The situation with respect to ERTs varies considerably across Europe with full reimbursement in some countries, reimbursement for only some metabolic diseases in others, and none at all in yet other countries.

Pisana Ferrari
The 3rd INTERNATIONAL PH NURSE WORKSHOP took place in Barcelona 8-9 May 2012. About 160 nurses and health care professionals from 20 countries were present at this two-day event. Discussions focused on three key areas: diagnosis, treatment and disease monitoring in PH, how to manage patients with different types of PH and the involvement of the various stakeholders in patient care (nurses, patients, industry, physicians). There were also presentations on country-specific aspects of patient management from Switzerland, the Netherlands and Turkey. It was a great honour for me to be invited to attend the event on behalf of PHA Europe and to be given the opportunity to speak about my personal experience as a PH patient. PHA Europe also had a booth where we distributed our brochures and the latest Mariposa News. From my own personal experience I know how vital a role the nurses can play in the management of PH. Persons living with a chronic and progressive disease require regular monitoring and frequent visits to the PH clinics. Over time relationships tend to build up with health care professionals. Of all the members of a patient’s health care team, it is usually the nurses who have the closest rapport with the patient. Nurses provide invaluable support in terms of patient education and training and this also has positive effects in terms of compliance to treatments and on outcome. Nurses and patient associations can also benefit greatly by collaborating and working together.

HOW WILL DISEASE MANAGEMENT CHANGE IN THE FUTURE?

J. CIFRIÁN

Great advances have been made in understanding the pathological mechanisms behind PAH. Drugs targeting three different therapeutic pathways have been developed as a result (endothelin, nitric oxide and prostacyclin) and survival has improved. There are clinical guidelines, a new diagnostic classification (2008) and a revised one coming up next year at the World PH Symposium in Nice. Thanks to treatments and better management there has been improvement in survival. Additionally, a number of promising drugs are now in the pipeline and could lead to new treatments being approved in the near future:

- Macitentan (endothelin receptor antagonist): has improved tissue penetration and access to ET1 receptors (SEAPHEN study now completed, it is a long-term, multicenter trial with a very large cohort).
- Selipixag: an elective IP receptor agonist (phase III, GRIFFON study).
- Oral treprostinil: an oral prostanooid with sustained release tablets (FREDOM-C study, in combination with ERA or PDE-5).
- Riociguat: a stimulator of soluble guanylate cyclase (sGC) with a dual mechanism of action. Is being investigated for CTEPH and other forms of PH (PATIENT and CHEST studies).
- Oral tadalafil: an oral PDE5 inhibitor currently being used in certain forms of leukemia. Has anti-proliferative and pro-apoptic effects on smooth muscle cells (IMPRES study).

With regards to surgery, currently only about 5% of persons undergoing lung transplant are suffering from PH, which is very low compared to cystic fibrosis, fibrosis etc. Survival is still not very impressive (50% at 5 years) but is improving. Transplant is still very underused with respect to its potentialities. It should be considered more often and earlier: PAH patients are generally being referred very late. Preventing graft rejection and increasing the availability of organs will be the real issues for the future. Genetics and cell therapy also represent a great promise. In order to make real progress in the field of research it is vital that clinical investigation be supported by the entire scientific community and not just rely on the industry.

PATIENT CARE MODEL OF CHRONIC DISEASES - TELEMEDICINE AS SUPPORTING TOOL

C. HERNANDEZ

Despite progress in diagnosis and treatment and an increased sensitivity on the part of health care professionals, there is still much room for improvement in PAH care. PAH is a complex disease (therapies, acute processes) and patients have complex needs which go well beyond PH itself (anxiety, depression…). Disease management must go alongside with patient management. It is fundamental to incorporate patients and work together for better patient care. A model for “integrated care” should include diagnosis, treatment, rehabilitation and health promotion. Integration is a means to improve service. New roles for professionals may emerge as nurses assume more responsibilities. In this context telemedicine can play an important role.

The Government of Catalunya, Spain, has elaborated a Master Plan to improve care for patients with respiratory diseases, an example of how to translate health politics into practical care. The program includes the establishment of a patient management unit with a case manager (specialist nurse) who works with primary care physicians, of a call center, home monitoring (for some patients), publications and education. Nurses have self-management and can remote control patients. Clear and standardized exclusion criteria are established for patients. All patients have a personal health folder. Four programs have been set up so far under the Catalunya Master Plan: one is for home hospitalization (treated by specialists) and involves 2,500 patients per year. Another relates to COPD patients, the aim being to reduce visits at the hospital. A third program involves patients on oxygen therapy. So far the results have been very positive: costs were reduced and the level of patient satisfaction was very good. The fourth program, for PH patients, started in June 2011 and involves 175 patients. The program includes not only services for patients (call center, telemedicine) but also support in decision making for professionals (from GPs to specialists), therapeutic education, answers to acute incidents, etc. Integrated care is equal to a multidisciplinary offer of personalized solutions.

HOW TO IMPROVE PATIENT SUPPORT?

WENDY GIN-SING

PH requires to be managed by a system with multidisciplinary teams which also takes into account holistic needs, education and support in managing treatment, social and spiritual aspects.

PH centres should provide:
- education about diseases and treatments;
- lifestyle information and advice;
- assessment of social and psychological needs;
- 24 hours specialist advice line;
- support for patients on IV treatment;
- medical reports and management plan;
- patient-held records.

Need for patient education: nurses are ideally placed for education and support. Nurses generally have a more holistic approach. Nurse are also generally the first to be contacted when patients have problems. Patients may feel more at ease discussing intimate and sensitive issues with nurses than with doctors. Therapeutic relationships tend to develop naturally between nurses and patients whilst training them to manage complex therapies.
Local health professional support should include:
- family doctor;
- local clinic;
- community nurses;
- psychological support and counselling;
- homecare company support for drug therapy;
- palliative end of life care support.

PH Community: this is a fantastic source of support:
- “buddy” programs;
- local support groups;
- national patient associations;
- social networking;
- conferences;
- open days at hospital.

Self-support: Proactive behaviourally-focussed self-management support can have a very positive impact. It should be designed to increase self-efficiency.

At Imperial College in the UK the nurses have developed a web application to facilitate self-management by:
- involving patients in decision making and problem solving;
- developing skills to enable patients to set care goals and plans;
- helping patients monitor symptoms and know when to take appropriate action;
- helping patients to manage social, emotional and psychological issues.

Patient goals:
- more therapy the only option? Quantity or diet?
- is it very important, in addition to receiving good medical care, to be treated with dignity and respect.

Developing future care requirements:
- designate expert centres;
- improve self-management;
- increased use of technology (tele-medicine, etc.);
- working together to set realistic plans and goals.

Examples of PH nurse management in different countries

It was extremely interesting to hear from nurses in the Netherlands, Switzerland and Turkey about the way in which PH nursing care is organized in these countries. It emerged from the presentations that the level of patient care in all of these countries is very high, with great attention being placed on individual situations and needs.

Netherlands: According to Dutch regulations PH is managed in university centres, with pulmonologists providing the specialist care, supported by multidisciplinary teams. All therapies are reimbursed by the national health service. There are 6 main PH clinics (population 7.5 million) who work in close contact with the smaller clinics, which concentrate mainly on oral therapies. There are 3 lung transplant centres and two for PEA.

In the hospital where the speaker works the PH nursing unit is composed of a clinical nurse specialist (Mrs van der Mark-Goebelje herself), a research nurse (in charge of clinical trials), a doctoral student (for research) and a secretary for the administration. The unit helps with paperwork on medical passports, requests for auxiliaries (wheel chairs, elevators) and referrals to the social worker or dietitian, who are not part of the team but collaborate. The unit organizes training for prostacyclin and runs a 24/7 helpline for patients on this treatment. District nurses provide initial help to change syringes and cassettes for prostacyclin patients who need it.

Dutch-Belgian network:
- One very interesting initiative is the nurse network group Netherlands-Belgium which was set up in 2004 and meets twice a year. It aims at:
  - standardising information;
  - optimising patient education;
  - advocating for patients;
  - performing scientific research in the nursing field.

Switzerland is a federation with four main language areas. There is a common health insurance system and there are very high standards of medical care. Treatment outside one’s canton, or region of residence, has to be authorized by the local doctor (difference with private insurance). Medical care is organized in three tiers: primary (GP), secondary (specialist near University hospitals) and tertiary (University hospitals). There are five University hospitals in the country (population 8 million). All drugs are available and every hospital is authorized to take part in clinical trials. There are both heart and lung transplantation facilities.

SAPH is the Swiss Society of Pulmonary Hypertension. It was founded in Bern in 1998 by a group of pulmonologists, cardiologists, intensive care specialists, hematologists and other health care professionals. It has a very important role as a reference point for health care professionals and patients alike. Its aims include:
- disseminate information about PH to physicians and general population;
- draw up of guidelines for diagnosis and therapy;
- collect epidemiological data in order to update the Swiss Registry;
- provide a forum for discussion for patients and physicians;
- organize scientific meetings and conferences;
- support research.

Nurses can now also be part of the SAPH. In June 2012 the Swiss PH registry include 1,237 registered patients, 358 of which alive, 23 patients have died and 26 post PEA. PH Patient meetings are organized by the hospital. Since the year 2000, one very active patient set up a internet website and forum which has been very successful and recently has become a patient association (Bruno Bosshard).

In Switzerland PH nurses provide:
- technical and psychological support;
- home visits;
- telephone helplines.

PH Nurses in Switzerland receive no formal training by the hospital and are trained outside. In most cases they are taught by guest nurses coming from the Netherlands or Belgium. In Switzerland the nurses receive a certificate from the canton.

Developing future care requirements:
- support research.

The importance of psychological support

S. Wiesberger

The speaker is a psychologist who meets monthly with a group of PH patients. Coping with severe diseases depends on the one hand on the type and stage of the disease, and on the other, on individual features. Lazarus and Folkman (1984 study) define coping as “the cognitive and behavioral efforts made to master, tolerate, or reduce external and internal demands and conflicts among them.” A “functional” coping process is proactive, problem oriented, based on efficacy and confidence in doctors. A “dysfunctional” coping process is characterised by loss of hope and/or denial.

PH is almost unknown in the general population. This can lead to isolation, sense of helplessness, anxiety disorders, depression, panic attacks.

A study by Lowe et al (2004) showed that the incidence of mental disorders among 164 patients persons suffering from post-traumatic stress disorder (PTSD) is around 35%, much higher than the general population, where it is estimated to be about 7%.

PTSD patients suffer from flashback memories and there is generally avoidance of stimuli related to trauma; in PH patients there may be fear of death in disease and fear of therapeutic interventions.

Ways of coping:
- social contact;
- support groups (patients should be encouraged to join);
- physical exercise;
- well-structured daily routine;
- psychotherapy (exposure therapy, EMRD - rapid-eye movements);
- treatments (drugs).

Questionnaires can be useful to discover mental disorders.

The PHQ-2, a self-administered two-item questionnaire to test depression (it is part of a broader test, PHQ-9). The PHQ-2 enquires about the frequency of depressed mood and anhedonia (lack of pleasure in doing things) episodes over a period of two weeks. The purpose of the PHQ-2 is to establish a final diagnosis or to monitor depression severity, but rather to screen for depression in a “first step” approach.
Patients who screen positive should be further evaluated with the PHQ-9 to determine whether they meet criteria for a depressive disorder. The HADS (Hospital Anxiety and Depression scale) is accepted worldwide as a tool to screen for depression and anxiety. Last but not least, nurses need to take great care of their own health (risk of burn out, need for supervision within the team, interchange).

**PARTNERING WITH NURSES: THE PAH PATIENT EXPERIENCE**

**P. Ferrari**

My brief for the workshop was to speak about my experience as a patient and about how nurses and patients can partner for better PH care. In my presentation I underscored the fact that PH is a severe, chronic and very disabling disease which goes far beyond the physical symptoms and significantly affects family, social and work life. Despite progress in diagnosis and treatment, which has led to improved management and survival, patients still face many difficulties in a number of areas: physical, emotional, organizational, medical. The nursing profession plays a vital role in the management of PH.

Persons living with a chronic and progressive disease such as PH require regular monitoring and frequent visits to the PH clinic. Over time relationships tend to build up with her/his health care professionals. Of all the members of a patient's health care team, it is usually the nurses who have the closest rapport with the patient. Nurses can provide invaluable support and this also has positive effects in terms of compliance to treatments and on patients’ own health (risk of burn out, need for supervision within the team, interchange).

**THE ROLE OF THE NURSE IN MANAGEMENT OF CTEPH PATIENTS**

**A. Ponnabaram**

Approximately 25% of CTEPH patients have no previous history of pulmonary embolism. Pulmonary endarterectomy (PEA) is the treatment of choice for CTEPH patients who are eligible. There are three distinct phases in the process: pre-operative, peri-operative and post-operative.

Work up at the PH centre:

Patients travel to Papworth Hospital (London) for outpatient clinic. Many are on oxygen, some are on wheelchairs. They have to travel sometimes a long way and there can be financial issues.

The PEA Clinic is a nurse-led clinic which provides education, assessment of individual patient needs, a patient guide, contacts with a former patients and consultation with the surgeon. Once the patient has taken the decision to go for surgery there are tests to be taken - inferior vena cava filter and, if the patient is older than 50, coronary angiogram - and then the patient is put on a waiting list. During this time there will be regular calls, support to manage symptoms and to identify deterioration (24 h helpline) and liaison with the GP.

Coming for surgery:

When the patients come for surgery there is preparation where a number of things need to be organized including admission, accommodation, travel and family support. PEA Surgery

The patient is usually in surgery the entire day. In most cases he will be extubated the following day. In-patient stay is usually about two weeks. A PH nurse will be providing support, advice and liaison in collaboration with a specialist. Patients are gradually weaned off oxygen when saturation is over 90%. Most are weaned off before discharge.

Going home:

Sometimes patients are sent to a local hospital or alternatively go home and are referred to a community nurse. Discharge advice will be provided about wound care, exercise and mobility, anti-coagulation, fluid restriction and diuretics, targeted therapy, possible complications. Patients are trained to report immediately any abnormal symptoms. There may be need for a neurological assessment as sub-dural hematoma is common (much less so cerebral hemorrhage or other accidents).

Post discharge support will include a phone call every 1-2 weeks to identify issues and complications. There is a follow up at 8 weeks. After 3-4 months patients come back to the PEA surgery centre, at 6 months to the referral centre and at one year back to the PEA centre. Three Cs are critical in PEA: Coordination, Continuity, Communication.

**DIAGNOSIS AND TREATMENT OF CTEPH**

**D. Jenkins MD, Papworth Hospital, UK**

Pulmonary endarterectomy (PEA) can be defined as an operative treatment. In 2011 Papworth Hospital, UK was the center in the world that has performed the highest number of PEA.

There are still many more patients that could benefit from this surgery. The problem is that diagnosis is difficult on account of the symptoms being non-specific. Education is improving but is far from being sufficient.

There are forms of CTEPH other than pulmonary hypertension. Also, PAH patients can have blood clots in the pulmonary arteries and not have CTEPH.

There should be a network of PH centres where specialists are encouraged to refer. How to select for surgery? Screening includes: Chest Xray, VR scan, CT scan, angiogram, MRI angiogram. These tests are necessary not only to diagnose CTEPH but also to determine which patients can benefit from surgery.

**TREATMENT: LIFELONG ANTI-CAOGLUATION IS STANDARD PRACTICE**

There is little evidence at present of efficacy of any PAH-specific drugs and at the moment there is no CTEPH licensed drug. The real issue is surgery. If the clots are distal the patient may not benefit from the surgery.

The operation: the operation consists of the simple removal of obstructive material. The operation is performed with cardiopulmonary bypass and cooling to 20°C. When the operation is done the CT scan has been eliminated (cognitive function actually improves after surgery). Two thirds of the patients get back to normal hemodynamics.

There are three main papers on survival after PEA. The largest series available is from the San Diego group which has since 1990 performed 1,400 operations (Stuart W. Jamieson, UCSD, Annals of Thoracic Surgery Nov. 2003).

In this study the investigators examined in detail the results of their last 500 consecutive patients. The perioperative mortality rate has steadily declined since then and was 4.4% for the 500 patients operated on between 1998 and 2002. Long-term outcome was analysed in the study in 308 patients: survival was 75% at 6 years or more. 93% of these patients were in functional class I or II. In a more recent study from Papworth UK survival on the 314 patients analysed was 90.0% at 5 years.

Paola Ferrari
AUSTRIA

VIENNA ZOOonRUN
On the 21st of June the Austrian PH association organized the 1st Vienna Zoo Run. The project was the fruit of a collaboration between the Vienna Zoo (the oldest Zoo in Europe) and PHA AUSTRIA. We hired professional organizers of running events and convinced them to give us a special rate. The advertisements and PR activities were done by the Vienna Zoo. The purpose of the run was to renew the polar bear area and to help our Luftkinde (“Airkids”) by supporting research on a non-invasive way to measure cardiac output. The event was extremely successful with more than 1,400 runners and many sponsors including Goodyear, Coca-Cola, major banks and insurance companies. We were very happy to split the €40,000 we raised with the Vienna Zoo.

RENEWAL OF OUR MILESTONE SQUARE:
Unfortunately the fountain at our “Milestone square”, where we sell fundraising stones (these stones pave the square, which is in the Prater area), was broken and there were no funds to repair it. Luckily we obtained permission from the Vienna Municipality to turn it into a flower bed. The square now looks very attractive and we are very happily selling our stones again on a daily basis. Up to now we have over 15,000 stones on our PH square.

BLUE LIPS CELEBRITIES
Further to our campaign in February to raise awareness for PH on Rare Disease Day we are continuing to take pictures of Austrian and international celebrities, like Rose McGowan (Charmed) or Jonathan Banks, holding the blue lip lollipops. In the Mariposa spring 2012 edition we had not included photos of our Breathtaking posters and billboards and in particular of those with oxygen masks attached (300 all over Vienna) so here they are now (see below).

Gerald Fischer, Selbsthilfegruppe Lungenhochdruck
www.lungenhochdruck.at
GOODBYE ROSIE

Rosie Matthysen was the initiator and first President of HTAP Belgique, the Belgian francophone PH Association, and Founding Member of PHA Europe, the European PH association. She tragically passed away in May after a long and brave struggle against the disease. Her passing is a great loss for the entire PH community and she will be sadly missed. Rosie was diagnosed with PH when she was 58 years old but had been experiencing symptoms for a long time. As is often the case, she was initially told she had anxiety and depression. Rosie started a new oral treatment study, beraprost, but fell into a coma. She was put on subcutaneous 24/7 prostanoids and had to undergo an atrial septostomy. She woke up from her coma a month and a half latter. She was well for a number of years on treatment but recently deteriorated. When she died she was on a waiting list for a double lung transplant and full of hope for a better future. She had just turned grandmother for the third time: Isaline was born only a few days before her passing away. Despite her disease Rosie was very active in HTAP Belgique providing invaluable support and information to other patients. She also regularly attended the PH educational “Master classes” at the Erasme Hospital where she spoke of her experience of the disease and treatment management. At European level Rosie was invited to bring the PH patient perspective at several meetings of the European Medicines Agency working group on new drug approvals. Last year Rosie very generously accepted to be filmed and share her personal life with PH in the touching video “My Breathless Story” which won second prize in the Eurordis contest for Rare Disease Day 2011.

“Shock, sadness, emptiness, incomprehension and anger are the first words coming into our minds. But the nice memories of Rosie, her kindness, charm, helpfulness, loveliness and her smile chase those bad feelings away. That’s what Rosie is still doing for us. We will keep her in our hearts eternally. Our fondest thoughts go to her husband Luc, her two sons Lionel and Benjamin, and their families”.

Gerald Fischer
President PHA Europe

RARE DISEASE DAY 2012

RADIORG.BE, the Belgian organization of EUIORDIS, organized information stands in the main Belgium University Hospitals during the last week of February. The purpose was to give information to health professionals and also to the general public about rare diseases. HTAP Belgique joined RADIORG.BE at Erasme Hospital and CHU Liège.

PATIENTS ASSOCIATIONS DAY AT CHU MONT GODINNE HOSPITAL

This day, organized by LUSS, the BELGIAN PATIENTS PLATFORM, was a great success and more than 20 different associations were present. The stand of HTAP Belgique was just next of the one for lung transplant. Many visitors came to the booth and asked for information about PH.

GAM OF EUORDIS AND 6TH EUROPEAN CONFERENCE ON RARE DISEASES & ORPHAN PRODUCTS (ECRD 2012)

This 3-day conference was attended by more than 600 participants. PH was well represented by Pisa for PHA Europe, by Mélanie Gallant Dewavrin for HTAP France and by myself for HTAP Belgique. It is very important to be present at events such as this and to show how active the PH community is.

Luc Matthysen,
HTAP Belgique

CARDIOLOGY CONGRESS BRNO

SPPH participated in the XX Annual Congress of the CZEC REPUBLIC

From June 16 to 17 SPPH members met in Sè for the Annual General Assembly. Before the official opening of the Assembly an art workshop was organized where several participants learned how to decorate objects with cut-out paper napkins (see photo below). The meeting itself featured presentations by two very distinguished and interesting guests. JU Dr. Ondrej Dostal, a lawyer who is specialized in medical issues, spoke about insurance and patients’ rights in the Czech Republic, and Dr. Pavla Nítová, a psychologist from Slovakia, addressed the issue of how to help better manage the difficult situation of patients with severe chronic diseases. The meeting was very successful and the talks were found to be very helpful by all.

Katerina Nováková
Sdružení Pacientů s Plicní Hypertenzí
www.plicni-hypertenze.cz

RDD 2012 CAMPAIGN FOLLOW UP

The Czech patient association took part in the ”Breath-taking” campaign by issuing press releases to all the main media. Reports were posted in 12 media outlets and blogs, with the potential reach of 17 million people, especially on the internet. In addition Dr. Jansa, a leading PH physician in Prague, spoke on the radio show Contact and, together with Veronica Schächterova, from SPPH, appeared on TV Metropol. The ”Breath-taking” posters were also used at the Czech Congress of Cardiology in Brno and at the weekend meeting in Sè (see photo below).

NEWS FROM EUROPEAN PH ASSOCIATIONS

Czech Republic

WEEKEND MEETING MEMBERS SPPH

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Gerald Fischer
President PHA Europe
PHA CONFERENCE IN ORLANDO

HTAP France had not been to a PHA conference since 2008, when it was held in Houston. Thanks to a PHA grant, I was able to attend this year’s conference, and a grant from GSK made it possible to invite another patient representative, Laure, as well as a PH physician, Dr Emmanuel Bergot, from Caen, Normandy. It was a wonderful opportunity to meet 1,500 people from the PH world community! There were 26 nationalities represented, especially patients from all over the United States, Latin America, and Canada. From a medical point of view, we heard about a new issue: the possible link between obesity and PH. A different form of the disease? Otherwise, we were mostly aware of the other subjects being addressed. We had international meetings, where we could share experiences. This was really interesting and we did learn from others. In fact it was very similar to our annual meetings as PHA Europe when we share ideas about projects that have worked at national level. PHA Canada presented its awareness campaign, with the song "Let me breathe" that can be found on the internet and can be used by other associations. There were also two meetings on the theme “PH World Day” where questions were not really solved and still need to be: “Should there be a world PH Day?…” And, if yes, when?” Considering a number of countries already have well established awareness months, that is PH Latin Day, and that World PH day was initiated by Spain, deciding was not easy! Nevertheless, not enough countries were present to make a decision, so we will all have to vote later on in the year.

BOURSORAMA

Boursorama, a banking and financial services group, recently collaborated with HTAP France and collected used toys, baby-bottle warmers, coffee machines, books etc, amongst its staff to give to the Pediatric Cardiology ward at the Hôpital Necker (Paris), led by Prof. Damien Bonnet. Boursorama also sponsored France’s annual Family Weekend (Weef) and one of the regional meetings.

SPORTS EVENTS FOR PH IN FRANCE

It has been a few years now that since we started organizing awareness activities around sports events. People who can run do it for those who cannot: they make their lungs work for those who cannot breathe right!

Ultramarathon: Since 2005, a few runners run all the distance from Toulouse to Port la Nouvelle (205 km), in June. It was the 10th edition this year and we had the chance to have as our guest testimonial (“Parrain”) the athlete Christophe Lemaire. There were 200 participants running and cycling alongside to support the “ultramarathonians”. For the first time, the event was financially remunerative and we were able to post ads in radio, internet and press to raise awareness about PAH.

A nice anecdote is that a cardiologist for children, Dr. Dulac, from Toulouse, did a part of the way on a bicycle, followed by two of his patients (the track is very flat, fortunately and the distance was short!) PH race in Lyon: In Lyon, Sylvain, his family and friends organised for the 7th time a PH race at the Parc Gerland. About 180 runners were registered (even more actually ran), in 3 different races: 6 kms for adults, 1 km for children, and a family relay. Even though it was a rainy day, the atmosphere was very friendly and festive. It raised awareness in different ways, for instance 5,000 handouts were distributed in fastfood restaurants called "Quick"; there was a radio spot to announce the event, and people in the park (always numerous in Gerland on Sundays) came around and asked questions. The participants paid a fee, and the event raised over 6000€.

Mélanie Gallant-Dewavrin
HTAP France
www.htapfrance.com

From left: Rino Aldrighetti from PHA and Mélanie Gallant-Dewavrin
From left: Mélanie Gallant-Dewavrin, Dorothy Olsen, founder of PHA
PHYSICAL ACTIVITY AND SEDENTARY BEHAVIOR IN CHILDREN AND ADOLESCENTS

PHYSICAL ACTIVITY AND SEDENTARY BEHAVIOR

Physical activity is generally associated with a lower risk of chronic diseases, obesity, and premature death. On the other hand, sedentary behavior, such as sitting or lying down for long periods, has been linked to a higher risk of chronic diseases and premature death.

In children and adolescents, physical activity is important for bone and muscle development, as well as for weight management. Sedentary behavior, on the other hand, may contribute to weight gain and obesity, which can increase the risk of developing chronic diseases later in life.

Research has shown that children and adolescents who engage in more physical activity and less sedentary behavior have better health outcomes than those who do not. However, many children and adolescents do not meet the recommended levels of physical activity, and there is aneed to increase physical activity and decrease sedentary behavior in this population.

One potential strategy to increase physical activity and decrease sedentary behavior in children and adolescents is to create a more active environment. This could include providing more opportunities for physical activity in schools and communities, as well as encouraging parents and caregivers to support their children and adolescents in being active.

Overall, there is a need for more research on the relationship between physical activity and sedentary behavior in children and adolescents, as well as for more targeted interventions to increase physical activity and decrease sedentary behavior in this population.
SHIRA FAIR
On May 28th, PHA Israel sponsored the second “Shira Fair,” in memory of Shira Dinur, who died at the age of 26 from pulmonary hypertension. Her family and friends decided to commemorate her through a fair to be held each year. Last year, the event took the form of a “happening” in a park with booths offering various items for sale. This year, the gathering took a completely different form – an exclusive evening cocktail party with five hundred participants! Music, a magician and various performances accompanied the event. Funds were gathered for the translation into Hebrew, Israel’s national language of a guide published by the US PH association. The book is in the final stages of translation and is being proofread prior to printing.

WEEK END FOR PH COUPLES
On April 29th, a workshop for couples took place for the first time. Many people at various stages of life suffer from stress and pressure and discover what is dearest to them, their relationship with their spouse, has been harmed as a result. Chronic illness afflicting one spouse results in significant pressures and threats to a couple’s relationship and upsets the natural balance between the partners. The illness erodes the resources of the healthy partner and creates pangs of conscience, feelings of guilt, irritability and anger.

The workshop was conducted by Dr. Suzy Cagen, a therapist specializing in individual and family therapy and an international lecturer and researcher on spousal relationships and behaviors that lead to the failure of relationships due to illness.

SELF COACHING TRAINING SESSION
On May 15 PHA Israel sponsored for the first time a support group on “self-coaching” lead by a professional personal trainer from the “Tut” company who spoke on the importance of thinking in ways different from those to which we are accustomed, how to change one’s approach to achieve better results, and the three principles for success in various fields of life. A similar support group will take place in the coming month in the north of the country.

Prepared by Aryeh Cooperman
Exec. Director, Israel PH Association
Translated by Dr. Yosef Gotlieb
PHA Israel Board Member
www.phisrael.org.il

ANNUAL GENERAL ASSEMBLY
AIPI Italy held its 2012 Annual General Assembly in Bologna on the 1st of April 2012. Over 150 patients and family members from all over Italy attended the one-day event. Topics on the agenda of the morning session included the annual report of activities 2011, updates in the medical and research areas, pension and insurance issues and the importance of psychological support. For the first time we also organized a presentation about how patient associations can make the most of the new social media. A nice lunch had been organized in order to allow guests to meet and socialize. In the course of the afternoon session Alessandro Serra, who is a very famous Italian comedian, entertained us with a very funny show and one of our members, who is a singer, treated us to some wonderful Frank Sinatra oldies. The afternoon ended with our traditional lottery. The first prize was an Apple IPad.

NEW BOOKLET
The AIPI quarterly newsletter has been publishing patient stories since 2003. A new publication containing excerpts from the over 70 stories collected so far was officially presented at the Annual General Assembly. The aim of this booklet is to share experiences and insights about coping with everyday life with PH.

Pisana Ferrari, AIPI Italy
www.aipiitalia.it
ITALY-AMIP

These last months were, as always, full of activities for AMIP Italy: contacts with patients and doctors, the organization of fundraising events and the Annual Assembly, during which a new President was elected (Vittorio Vivenza), a new Scientific Committee was formalized, new projects were decided upon and much, much more. And finally, at the end of the month of June, here in Rome, we held our Annual Patients’ Meeting. But today I don’t feel like writing a long report about our activities. I’d rather focus on a beautiful and very inspiring story. This story began exactly one year ago, on June the 14th, when I received a phone call to inform me that our young member Giorgia Onorati was in hospital undergoing at last her long-awaited heart and lung transplant. My first reaction was to pray for her, but, thinking that perhaps my prayers were not powerful enough, I immediately put my friends to the task: “Pray very hard, Giorgia is now in the operation room for her transplant!”

Three days later, during last year’s AMIP’s annual Patients’ Meeting, Dr. Sara Alfieri, one of the doctors who were taking care of Giorgia, was able to give us the good news: Giorgia was reacting well to her surgery! Let’s come to this year’s Patients’ Meeting that took place on June 22-24: Giorgia was well enough to be with us! She immediately put my friends to the task: “Pray very hard, that perhaps my prayers were not powerful enough, I want to talk about my disease, but rather about my passion for photography…”

I take this opportunity to thank Dr. Sara Alfieri who offered the following brief explanation about Giorgia’s complex condition: “Giorgia received a heart-lung transplant one year ago. At birth she presented left diaphragmatic hernia which was successfully treated with interposition of a rigid plaque. The long term follow-up has been complicated by pulmonary hypertension, scoliosis, gastro-esophageal reflux and finally by heart bi-ventricular failure and atrial fibrillation. Her medical schedule foresees: heart-lung transplantation, gastro-esophageal plastic surgery and scoliosis correction. The heart-lung transplantation was complicated by an acute left colon occlusion that required an immediate position of a temporary ileostomy: the occluded loop was imprinted in the correction of the diaphragmatic hernia. Some months later the ileostomy was removed and fundoplication for correction of gastroesophagal reflux was done (Nissen operation). Currently the patient is trying to improve her nutritional status and the respiratory function in order to achieve a clinical condition which will allow us to complete her planned surgical schedule (scoliosis correction)”.

Luisa Sciaccia della Scala, AMIP Italy

www.assoamip.net

LATVIA

INDUSTRY CONTACTS

Since February this year PHA Latvia has established contacts with representatives of international pharmacy companies in Latvia and acquired financial support from Bayer, GlaxoSmithKline, United Therapeutics and Pfizer.

MEETINGS WITH HEALTH AUTHORITIES

During March, April and May PHA Latvia held meetings with officials from Ministry of Health and The National Health Service to discuss healthcare for PAH patients in our country.

NATIONAL PLAN FOR RARE DISEASES

On the 10th of May in the “House of the European Union” PHA Latvia held a public discussion about “The National Plan for Rare Diseases in Latvia years 2012 – 2015.” There were participants from several non-governmental organizations, representatives of Ministry of Health, The National Health Service, the Centre for Disease Prevention and Control, doctors and representatives of pharmacy industry.

SUPPORT FOR OXYGEN THERAPY

Starting from April 2012 PHA partially covers costs of oxygen equipment rental for those PAH patients for whom a cardiologist or PH specialist has recommended supportive oxygen therapy at home. Our country does not take into consideration these costs.

SUPPORT TO PATIENTS AND ADVOCACY

We are working on two important levels. PHA Latvia provides practical assistance for its members and other PAH patients and also works on representing our interests in our country’s decision-making process - we prepare resolutions about legislative projects for healthcare and social services and have started to advocate to our PHA Latvia, represented by myself (President), in the relevant task forces of Ministry of Health.

RELATIONS WITH OTHER ASSOCIATIONS

Since Spring 2012 we have started a strong partnership and cooperation with other associations for rare disease patients: the “Latvia Hemophilia Society”, the Rare Disease Association “Caladrius”, the association “Motus Vita” and the “Group of Rare Tumors”. We are working on founding an Alliance of Rare Diseases, because we have come to conclusion that the majority of our problems and issues of social and health care are the same. So we have decided that it is necessary to gather our powers.

Ieva Plume, PHA Latvia

www.phalatvia.lv/en/
Our patients is how to manage with oxygen during flights and when they are abroad. In reply to this problem we are trying to rent a very small concentrator at a reasonable price. We are currently negotiating with a company who delivers these concentrators.

NEW WEBSITE
In April we introduced our new website which has many new features and a nice colourful graphic layout.

FUNDRAISING FOR PH
In Spring there were several fundraising activities organized by patients. We raised of €15.000, which was a very successful result.

ANNUAL PATIENT MEETING
PHA Netherlands held its yearly meeting in April 2012. Every year one of the leading Dutch PH specialists comes to our meeting to provide an update of the latest developments in PH treatments and research. This year we had the pleasure of welcoming Dr. Anco Boonstra from the VU MC in Amsterdam. He showed us, inter alia, a new pump system for Remodulin which is currently being tested. The tiny pump can be implanted under the skin in the abdomen and as a result patients do not have pain when they change the infusion site. After a nice lunch we had three workshops; one about CTEPH, one about PH and problems on holidays and the third about scleroderma and PH. There was a lot of interest in the workshop, in particular the one about holidays. The main problem for

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**RARE DISEASE DAY IN POLAND**

Members of the Polish association took part in the national initiatives for Rare Disease Day. A big event was organized in a beautiful city park in Warsaw, Lazienki. To underline solidarity with the families affected by rare diseases, this year’s celebrations were held under the slogan “Together we are strong!”.

Speakers at the event include Bartosz Andriowicz, Minister for Health, Beata Czaplicka, Member of Parliament, Jacek Gralinski, Chairman of Committee on Rare Disease in Ministry of Health, and Anna Dymna, a very famous actress who has her own foundation, “Against the Odds”. This association helps many ill and disabled people in Poland.

The conference was combined with an exhibition of photos entitled “Photogenic” and a screening of the film “Welcome to the world’s Rare”. A photo of one of our members from Polish PH Association was also on the exhibition.

**WORKSHOP FOR NURSES**

The workshop “Care of patients with PAH”, was held on 15-16 June in Józefów. The workshop was for nurses who are involved in PAH patient care.

The first talk was by a psychologist, Karolina Tolińska, who presented a report about her work with PAH patients. She spoke about contacts with patients and support provided directly or through the phone specifically dedicated for patients with PAH. The conclusion was clear: help is not only necessary but also indispensable.

In the second presentation, the Polish Association had the opportunity to present their activities. Piotr Manikowski talked about the history of the association, who can be a member and how to sign up, goals, informational and educational activities, website and forum. Each participant received a folder with information about the association and campaign materials from 2011 and 2012.

Finally, Professor Marcin Kurzyrna presented the role of nurses in patient care. This role is much wider than it appears as it is generally the nurses who have the most direct contact with each patient.

**NEWS FROM EUROPEAN PH ASSOCIATIONS**

**PH WEBSITE**

The most important news of last month is the creation of a new website on pulmonary hypertension. We thought at length about style and contents and our three main guiding principles were that despite the seriousness of the disease it should have a positive approach, that it should be very informative but also easy to use. The website is now online at: www.ph-sos.com

On the website patients can find information on the disease, on drugs, treatment strategies, doctors. It is interactive and it is possible to ask a question to a doctor and a lawyer and have replies.

We have also created a forum on which patients can communicate with each other: http://forum.ph-sos.com

In order to fully understand the importance of this forum, it is necessary to know Russia. In our country there was previously no website, no forum. Knowledge of doctors and medical centres is very low. Patients with a diagnosis of pulmonary hypertension can count only on themselves. There is nobody to turn to for replies to their questions and doubts. In the forum they can communicate, exchange experiences, give advice. A moderator ensures that the answers are correct, this is very important. At the end of this year we plan to open this website in English.

**ACCESS TO DRUGS**

In Russia Tracleer, Ventavis and Revatio are available but none of these drugs is free for the patients. We managed to considerably reduce the cost of Tracleer for our patients through negotiations with Actelion. We had to go to court and involve lawyers for Revatio. Some of our cases managed to be won and patients now receive Revatio at the expense of the state.

**REGISTRIES**

We have also been working on setting up a register of patients with PAH. Its aim is to understand how many persons in Russia have the disease. Currently 82 patients are included in our register. This is not enough data for such a big country, but we are working actively to increase the number.

**FUNDRAISING**

Over the last few months we have been very active in fundraising activities in order to purchase drugs for our patients. In these actions there is also active participation of the patients themselves.

Eugeniy Komarov, Natasha Foundation
www.ph-sos.com
ESTABLISHMENT OF A PH CENTER
We were very pleased on the 9th of November 2011 (ZS928 - 2011 - CTM) to receive an announcement about the decision of the MINISTRY OF HEALTH, that at the DEPARTMENT OF HEART FAILURE and CARDIAC TRANSPLANTATION of the NATIONAL INSTITUTE OF CARDIOVASCULAR DISEASES, the first center for pulmonary arterial hypertension has officially been established.

TAX INCENTIVES FOR PH
On the 19th of February 2012 we published information about our association on the website of the newspaper Pravda. Its purpose was to emphasize our association in order to obtain 2% from tax payers to the non-profit organizations.

RARE DISEASE DAY AND FOLLOW UP
On the 29th of February 2012 we were involved in the breathtaking campaign. The campaign was launched in the morning with press conference in the shopping center called Eurovea in Bratislava. It was attended by dozens of journalists, the Slovak Radio and Television Bratislava. The campaign continued in the business center Eurovea familiarizing visitors with the PH disease. We also attended the first congress of the SLOVAK DAY FOR RARE DISEASES, which was held under the auspices of the MINISTER OF HEALTH and EURORDIS. On the 1st March a business dinner was held in the restaurant Reduta and its purpose was to improve cooperation between doctors and patients. The dinner was attended by cardiologists and pulmonologists. After dinner, the campaign continued in the Reduta area for visitors of the Slovak PHilarmonic concert. Simultaneously the campaign ran in another Slovak town in Liptovský Mikuláš. Overall we distributed over 1,500 flyers and brochures, 1,000 lollipops and 150 brochures on the diagnosis of PH. The campaign has been documented by videos, articles and many photographs. In my capacity as President of the PH association, I had interviews about my experience as a PH patient and various articles came out in the magazine Pokrok, Šarmantná žena, Zdravie, Plus jeden deň and on the show presented by one of the most popular TV’s in Slovakia called TV Markíza. As part of the follow up activity, on the 23rd March, Jana Gúľašová presented about our PH association activities in Liptovský Mikuláš in the shopping center called Stop Shop.

TEAMING UP WITH SLOVAKIAN RARE DISEASE ASSOCIATION
We teamed up with similar civic associations in Slovakia (“The gift of life”, “Slovak patient”) and we have become a member of the Slovak Alliance of rare diseases. On the 30th and 31st March, I participated in the 2ND NATIONAL CONFERENCE OF PATIENT ORGANIZATIONS in Piešťany. In the course of the two days there were several lectures about patients’ rights.

PH WORLD DAY
PHA Slovakia supported May 5 as the World Day of PH. We encouraged other Slovak institutions to approve it too - the SLOVAK SOCIETY OF CARDIOLOGY, Department of Pneumology and Phthisiology of the UNIVERSITY HOSPITAL in Bratislava and the SLOVAK ALLIANCE FOR RARE DISEASES. We also accepted with great pleasure the invitation of the Spanish PH Association to attend the Scientific Symposium and Official Ceremony of the first World PH Day which took place on May 4-5 in Madrid.

HEALTHY LIFESTYLE EXHIBITION
From the 10 to the 12th of May 2012 we attended the 14th annual exhibition called “Healthy lifestyle” in Trnava. During these three days we informed visitors about PH symptoms, diagnosis and treatment and handed out dozens of flyers and brochures. Jana Gúľašová and I also attended the first part of an educational course called “The patient and medicine”, organized by the SLOVAK MEDICAL UNIVERSITY in Bratislava. This training course is intended for patient advocates and will resume in November.

GENERAL ANNUAL ASSEMBLY AND RECONDITIONING STAY
From the 1st to the 3rd of June 2012 we held an educational-reconditioning meeting for PH patients in Banská Bystrica. It was attended by 36 people. The agenda consisted of lectures on various topics related to PH including disability and compensation benefits. On the second day of the meeting, patients learned how to relax under the guidance of a psychologist, Dr. Pavla Nótová, and we also had rehabilitation exercise sessions. During the General Assembly we held elections for the Board of PHA Slovakia. Members were informed about the recent activities of the association, supplemented by slides and photographic documentation and we presented the 2011 budget and plans for the future. I had the honour of being re-elected, Jana Gúľašová was elected Vice President. Dr. Milan Luknár was appointed Honorary Vice President. Our association has four new members.

PH EVENT AT EUROPEAN PARLIAMENT
Our Association took part in the Call to Action event at the European Parliament in Brussels on June 6. We appreciated this well-organized event very much. Although the Parliament member for Slovakia, Dr. Miroslav Mikulčík, was not able to attend the event due to his working duties, he expressed his support for early and appropriate diagnosis and management of people with pulmonary arterial hypertension as well as the need for the increase in the funding of research of PAH. All the materials were sent to him and we hope for further communication and future collaboration on behalf of PHA Slovakia.

CZECH PATIENT ASSOCIATION MEETING
Two of our members, Mária Jesenáková, and psychologist Dr. Pavla Nótová, attended a weekend meeting organized by the Czech PH patient association June 16-17. During these two days Dr. Nótová held two lectures. Our recent activities were presented to the Czech PH members and we gave them brochures on psychological support for PH.

PHA CONFERENCE IN ORLANDO
Our Association was also invited to the Pulmonary Hypertension Association (USA) meeting in Orlando, Florida, June 21-24. This conference included the Second International Leaders’ Summit attended by leaders of international patient associations. Several very interesting topics featured on the agenda, including management of support groups (Israel), psycho-social resources (Spain), patient databases and registries (France), addressing the medical community (Japan), relations with industry (South Africa), and governments (Ecuador), as well as patient relationships (USA) or new forms of addressing general public through media using patient stories (China). Prof. Breyer from the UNIVERSITY OF CENTRAL FLORIDA discussed several issues related to patient association management. We found his presentation especially useful as he suggested many tips on how to make our association more professional and efficient in acquiring resources and addressing general and targeted groups of interest. At the international Strategy meeting, we expressed strong support for World Pulmonary Hypertension Day along with other national and regional PH associations.

Iveta Makovníková
Zdravie Pacientov s Plúcnou Hypertenziou
www.hypertenziapluc.szm.com
NEWS FROM EUROPEAN PH ASSOCIATIONS

SPAIN

NETWORKING AND ADVOCACY
ANHP attended the winter congress of the Pulmonology and Thoracic Surgery Scientific Society of Spain (SEPAR) in Zaragoza on February 10-11. From June 8-11 we attended the SEPAR Congress in Madrid. As in past occasions, ANHP had a booth and was present throughout the congresses to promote the knowledge of PH amongst specialists and take advantage of the physical presence of the industry representatives to talk about common projects.

ACCESS TO DRUGS AND ILLEGAL GENERICS
Fearful of the consequences of the economic crisis, ANHP is also increasing its presence at national patients’ federations in order to protect access to PH drugs. ANHP is also heavily involved in fighting the use of illegal generics of sildenafil that might be causing serious damages to PH patients in Andalucía. The activities against the use of illegal drugs for PH have succeeded in the Canary Islands where the public hospitals have gone back to approved treatments. ANHP is now focusing on Andalucían hospitals. The efforts of ANHP in the field of drug safety and transparency brought pulmonologists and cardiologists to a meeting in Zaragoza hosted by Dr Nauffil, Head of the Vascular Department of SEPAR, who agreed to produce a Consensus Document on the safety of PH treatments.

RARE DISEASE DAY 2012 FOLLOW UP
ANHP actively took part in the “Breath-taking Campaign” on February 29 (see Mariposa spring 2012). Follow-up included a 45-second spot on the Tele5 live show Silicium (2 million viewers) conducted by Jorge Javier Vázquez, one of Spain’s best known TV showmen. He explained what PH is and ended the spot with the words in alentos, which is the Spanish translation for “breathtaking”

PSYCHOLOGICAL ASPECTS OF PH
In May ANHP held a workshop on the psychological aspects of PH at the La Paz Hospital in Madrid. The psychological support program is one of the traditional activities of ANHP. These workshops provide an excellent example of cooperation between physicians, ANHP and industry to provide patients with tools to cope with the difficulties of living with PH. ANHP has now also started a more proactive approach with a psychologist that regularly calls ANHP members to assist with their emotional needs. Depending on the specificities of each case, the psychologist decides on the frequency of the phone calls, when to discontinue calls and/or refer for a fellow psychologist’s or psychiatrist’s consultation.

NURSE TRAINING PROGRAM
In past years ANHP has been involved in a number of nurse training programs organized by the industry. ANHP’s President has been invited to bring her experience as a PH patient. The voice of a patient is very powerful when it comes to making health professionals understand the real issues involved in coping with the disease.

ANHP SPEAKING ENGAGEMENTS
ANHP was invited to participate and speak at the PHA International Leaders’ Summit in Orlando June 22-24 and at the 7TH ANNUAL PRICING, REIMBURSEMENT AND MARKET ACCESS IN PHARMA meeting on March 5-6 in Madrid. On behalf of ANHP I also attended the training day on IT RESOURCES MANAGEMENT FOR MARKETING ACTIONS OF THE PHARMACEUTICAL INDUSTRY in March 20 in Madrid where I was invited thanks to our presence on Twitter where we have reached a good positioning and down, to malnutrition, because of the one-sidedness of the diet, and to the loss of pleasure in eating, if many foods are forbidden. Mrs Imfeld also spoke of the fact that certain foods can interact with the drugs and therefore their effectiveness can be influenced. The consequences of iron deficiency and the relationship of vitamin K-containing vegetables and blood clotting were also addressed. During the second part of the meeting Ulla Treders, from the Pulmonary Hypertension University Hospital in Zurich, spoke about blood thinners and the important role they play in PH. Conference participants were asked to test their medical knowledge, by replaying to questions such as: Which is the safest painkiller when on anti-coagulation? Should INR be measured once a week or once a month? Which symptoms should be reported to the doctor or clinic? What is the effect of anticoagulants? After the presentation Mrs Treders answered the numerous questions from the audience. At the end of the presentations we warmly thanked the speakers and offered them a bouquet of flowers. We ended the day with a nice lunch which gave us the opportunity of getting to know each other better and of sharing experiences.

SWITZERLAND

2ND GENERAL ANNUAL MEETING
SPHV’s second Annual General Meeting took place on the 31st of March in Olten. Twenty two members attended. Elections were held and the following positions were covered: Daniela Isidoro (Treasurer), Hans Hug, Ursina Bosshard (2nd Auditor), Ursula Stauffer (Deputy Auditor). One of the items on the agenda was the approval of the 2011 budget. This year our annual accounts have a positive balance. The Executive Board of the Assembly proposed to nominate Bruno Bosshard as an Honorary Member, in recognition of all his work for PH over the last years. Without Bruno we would not have this association! Needless to say, the Assembly unanimously approved the proposal. Thus, our association has its first Honorary Member. The annual meeting was followed by a very nice lunch. This was made possible by our sponsors and I would like to take this opportunity to express my special thanks. The next Annual General Meeting is scheduled for March 23, 2013.

EVENT AT MEDISERVICE
On Thursday, May 10, 2012, we met at an event in the Medicare service in Zuchwil. This time we were a small group of 12 people. The speaker was Christina Calietti, a social psychologist.

SECOND REGIONAL MEETING
On May the 3rd we held our second regional meeting in a cosy bistro near the train station of Chur. This time I was able to welcome four people to the meeting. The hostess of the Cafe treated us to some wonderful cakes! Mmmmmhh! We discussed various topics including nutrition, medications, IV drugs, the daily life of PH patients and of the persons caring for them. With so much chatting the two and a half hours flew by! We decided that we will meet again in September.

SPHV MEETING
On the 2nd of June SPHV organized a patients meeting in Olten. There were about 50 people present. Mary Imfeld, a nutritionist from Zurich, provided very interesting insights into the principles of a healthy diet, including which foods can be combined for a more balanced and enjoyable meal. In her talk she took into account the different needs of patients including psychological, social, and aesthetic aspects. Diets are often one-sided and may result in difficulties with long-term compliance. This can lead to the so-called “yo-yo effect”, with weight going up and down, to malnutrition, because of the one-sidedness of the diet, and to the loss of pleasure in eating, if many foods are forbidden. Mrs Imfeld also spoke of the fact that certain foods can interact with the drugs and therefore their effectiveness can be influenced. The consequences of iron deficiency and the relationship of vitamin K-containing vegetables and blood clotting were also addressed. During the second part of the meeting Ulla Treders, from the Pulmonary Hypertension University Hospital in Zurich, spoke about blood thinners and the important role they play in PH. Conference participants were asked to test their medical knowledge, by replaying to questions such as: Which is the safest painkiller when on anti-coagulation? Should INR be measured once a week or once a month? Which symptoms should be reported to the doctor or clinic? What is the effect of anticoagulants? After the presentation Mrs Treders answered the numerous questions from the audience. At the end of the presentations we warmly thanked the speakers and offered them a bouquet of flowers. We ended the day with a nice lunch which gave us the opportunity of getting to know each other better and of sharing experiences.

Therese Oesch, SPHV
www.lungenhochdruck.ch
International PAH Patient and Carer Survey

PHA Europe is very pleased to share with you news of an exciting initiative that we have been involved in to help to make a difference to the lives of PAH patients and the people who care for them. The International PAH Patient and Carer Survey, sponsored by Actelion Pharmaceuticals Limited and conducted by an independent market research agency, was carried out to provide new insights into the wider impact of PAH on patients and carers beyond the clinical definition of the physical burden of the disease. This large, international survey is the first to explore the perspective of the carer as well as the patient on the physical, social, emotional and practical impact of the condition on their lives. It also looks at the information needs of PAH patients and carers and how these might change as the disease progresses.

Pisana Ferrari, Vice President, PHA Europe, was a member of an international Steering Committee consisting of PAH specialists, nurses and representatives from eight patient organizations that was formed specifically to ensure patient and carer information addressing challenges highlighted in the survey, that have been crafted specifically for your PAH patient and carer members. We will also outline the tools that have been developed to enable you to launch the survey report to the media in your country to generate widespread support for our cause, and greater understanding among the general public of the unseen effects of this debilitating disease.

Copies of the report will be available for download from our PHA EU website www.pha-europe.org and a summary of key findings will be also available on our Facebook page and PAH support forums from 14th September 2012.

Top-line survey results revealed:
- The ability to work and the impact on household income is of particular concern to patients and their carers.
- The world narrows for PAH patients and carers as they are increasingly confined to their home with less ability to travel and socialize.
- PAH affects a patient’s ability to be intimate with their partner mainly because of low self-esteem and being unable or afraid to exert themselves physically.
- PAH patients and carers found there were gaps in information about the emotional and financial aspects of living with PAH.
- PAH patient and carers experienced feelings of isolation, mainly as a result of little understanding of the disease among family and friends.

As a result of the survey, PHA Europe will make recommendations on how the management of PAH among patients, carers, healthcare professionals and patient organizations can be improved and we need the support of our member associations to help us to achieve this. At our Annual General Assembly on September 12-16 in Castelldefels, near Barcelona, Spain, we intend to share the full survey results and the new information resources, including patient and carer information addressing challenges highlighted in the survey, that have been crafted specifically for your PAH patient and carer members.

Rehab in Heidelberg, Germany

Exercise-related sessions are often featured at current PH conferences. This would not have been the case ten years ago. Since PH puts a strain on the heart of a PH patient, it was thought that physical activity or training may have a negative impact on patients by contributing to the evolution and progression of PH. However, a paper published in 2006 changed this belief. Professor Ekkehard Grünig, his assistant Nicola Elsken plus other colleagues, were the authors of this paper. They conducted a study to evaluate the effectiveness and safety of respiratory and physical exercise training in patients with symptomatic chronic PH. They concluded that low intensity exercise is well tolerated by PH patients and that they improved their six-minute-walk, on average, by almost 100 meters following a three week long program! A follow-up study in 2010 confirmed the results. Furthermore, training seemed to improve the patient’s quality of life and possibly influence survival rate.

At the start of the three-week stay at the rehab clinic, all patients undergo a thorough examination. Blood values and blood gas are collected and a spirometry test is performed. However, Prof. Grünig performs the most important test: while biking, an ultrasound of the heart is formed. However, Prof. Grünig performs the most important test: while biking, an ultrasound of the heart is performed and maximum O2 is measured, including O2 saturation and blood pressure. In this way, Prof. Grünig can see how the heart performs under strain and can prescribe an appropriate exercise level for the subsequent rehab stay. Patients stay at a beautifully situated rehab clinic outside Heidelberg. They start their day by weighing themselves and measuring their blood pressure and pulse. Daily training consists of an exercise program on stationary bicycles. The resistance is individually set and O2 saturation is measured while biking the 16-minute long interval training. Light weight training is also part of the daily routine. Breathing technique and walking training, which are individual sessions performed with a skilled therapist, are also a part of the program. Other sessions include massage, heat therapy, cooking classes, nutrition classes, etc. After three weeks, results are measured with an identical examination as at the start of the stay.

As a PH patient and leader of a national organization, I found the results achieved by Prof. Grünig and his team very interesting. I therefore applied to participate in the program, and was very impressed by the professionalism at the rehab clinic. I was also very impressed by the results I achieved by my three-week stay, even though the exercise routines seemed surprisingly easy. I have therefore no hesitation in highly recommending a stay at the Heidelberg clinic to all PH patients!

Hall Skåra
Leader Norwegian PHA
The Cyprus Presidency considers the issue of organ donation and transplantation as a pure act of altruism and social solidarity. At the same time, it recognizes the shortage in human organs intended for transplantation throughout Europe and aims to highlight the need for coordinated actions in the field, at community level. The European Commission, in addressing the challenges in the area of organ donation and transplantation in the EU, moves forward on the establishment of coordination and cooperation mechanism among Member States. For this reason, there relevant legislation has been adopted, as well as an ACTION PLAN FOR ORGAN DONATION AND TRANSPLANTATION for the period 2009-2015. The mid-term evaluation of the Action Plan is expected to be completed during the second half of 2012. In light of these, the Cyprus Presidency plans to adopt respective Council Conclusions, which will further invite the Member States, the European Commission and the other EU institutions for concrete actions in ensuring public awareness of the importance of organ donation and transplantation and securing EU funds for the development of respective programmes in this field.


EU HEALTH IN TIMES OF CRISIS

THE HEALTH FOR GROWTH AND CONSUMER PROGRAMMES

Crisis strickens hard on Europe and health systems are one of the services that politicians trim down to reduce the countries’ debt. Many chronic patients are scared that their access to proper healthcare and treatments will be jeopardized. It is important to monitor the situation and healthcare developments in our countries to lobby and try to prevent loss in medical and pharmacological services in life threatening sicknesses. It is also time to pave the way to recovery because this crisis will not last forever and the work we do now will shape the future to come. It is important to know what is in store at European level so that we can prepare and strengthen the relationship with health authorities by establishing a relationship where we don’t play the role of just beneficiaries but of active agents in development.

The two programmes mentioned in the headline form part of the EU priorities and should be approved by the end of 2013. They were announced by the European Commission in June and they should run from 2014 to 2020. In the current state of affairs, there might be need to protect those projects before they also suffer some budgetary cuts since 2012 and 2013 are not very promising financially speaking. The proposal made by the European Commission (as you remember the previous article, it is the EU body that drafts proposals for new European laws and manages the day-to-day business of implementing EU policies and spending EU funds) foresees a budget of €166 million for the HEALTH FOR GROWTH PROGRAMME and €197 million for the CONSUMER PROGRAMME.

Examples on which further action will be built:

• Co-operation on Health technology assessment (HTA), an EU-wide voluntary network of Member States’ HTA agencies to share information on the effectiveness of health technologies such as medicines, medical devices, and preventive measures, to support national decision-making on technology.

• Co-operation on rare diseases at European level to improve prevention, diagnosis and treatment for patients with rare diseases across the EU, including the EU portal for rare disease (www.orpha.net), the world reference database on rare diseases.

• Cancer prevention and control, through EU-wide screening guidelines to improve early detection so that the disease can be diagnosed at an early stage and lives can be saved, and through exchange of knowledge and best practice on cancer prevention, research and care.

What roles can PH organizations play?

The HEALTH FOR GROWTH PROGRAMME aims to support and complement the work of Member States to achieve the following four objectives:

• Developing innovative and sustainable health systems.

• Increasing access to better and safer healthcare for citizens.

• Promoting health and preventing disease.

• Protecting citizens from cross-border health threats.

The CONSUMER PROGRAMME will support EU consumer policy in the years to come. Its objective is to place consumers at the centre of the Single Market and empower them to participate actively in the market and make it work for them, particularly by:

• Enhancing product safety through effective market surveillance.

• Improving consumers’ information, education and awareness of their rights.

• Consolidating consumer rights and strengthening effective redress, especially through alternative dispute resolution.

• Strengthening enforcement of rights cross-border.

PH organizations can participate in training programmes that will enable them to understand the needs and language of healthcare authorities such as HEALTH TECHNOLOGY ASSESSMENT COORDINATORS and the EUROPEAN SUMMER SCHOOL, that enables patients to understand the processes that medicines and medical trials have to undergo before their approval. Professionalized patients are increasingly playing a supporting role. The trend is to increase the participation of patients in decisions that affect their lives. Awareness activities on PH, offer of services to Health Technology Assessment National Agencies, training of healthcare professionals on PH through seminars where patients encourage clinicians researchers and pharma industry to work on cost-efficient medical tests and treatments will draw the attention of the authorities and build a relationship where they will be called for consultation in some laws and rules making.

More details can be found at: http://ec.europa.eu/enterprise/sectors/healthcare/competitiveness/pricing-reimbursement/transparency/index_en.htm
PATIENT ADVOCACY AT EU LEVEL
DITE TASK FORCE AND BURQOL-RD

As most of the readers know, I am wearing two hats, one as coordinator of the Spanish National PH Association (ANHP) and one as member of the Board of PHA Europe. It is difficult sometimes to separate one from the other and this is one of these situations because both ANHP and PHA Europe are heavily involved in the subjects of this short article.

DITA stands for D RUG INFORMATION TRANSPARENCY AND ACCESS. The DITA TASK FORCE was created in 2009 to give support to the patient representative at the EUROPEAN MEDICINES AGENCY, holding a position at the Consumer Working Party. There are three other committees at the EUROPEAN MEDICINES AGENCY and the subsequent task forces at EC/EDQM, the EUROPEAN ORGANIZATION FOR RAPE DISEASES. I submitted my candidacy to be part of the DITA task force after consultation with the presidents of ANHP and PHA Europe, who agreed on the importance of keeping a high profile for PH in working groups and institutions that work on rare diseases. It is an honor to say that my candidacy has been accepted and I will be a member of the task force for a period of three years.

The collaboration and solidarity between PH organizations and rare disease patients’ organizations is a key element in our commitment to advance as a community, with the certainty that strong rare disease organizations are a platform and a voice for all those who need not to be alone, those who are most vulnerable because strength is in numbers and neither of us have a high prevalence.

The second subject I will tell you about is the BURQOL-RD project. BURQOL-RD is a 3 year project under the 2ND PRO GRAMME OF COMMUNITY ACTION IN THE FIELD OF PUBLIC HEALTH, that commenced in April 2010 and is promoted by the DG Sanco (Health and Consumers). The main aim of BURQOL-RD is to generate a model to quantify the socioeconomic costs and Health Related Quality of Life (HRQOL) of both patients and caregivers for rare diseases in different European countries (www.burqol-rd.com). There is a strong relation between the approval of reimbursement of new drugs and the benefit they bring. It is imperative to be able to balance the benefit of the new drug with its cost. The capacity to put into monetary terms that balance will allow us to lobby in favor of the reimbursement of PH orphan drugs in our countries. PHA Europe was very interested in this project and funded my attendance at a meeting in Brussels on May 22 to evaluate our capacity to be part of it. After the meeting we decided that ANHP would be the test case for PH. We will work closely with different hospitals in Spain in order to gather as much quality data as possible so that we can showcase as many different types of PH as possible in this analysis. We are very proud to be part of this project not only because of its importance for the PH community but also because it is funded by FP7 (FRAMEWORK PROGRAMME 7), which is the most important program of the EUROPEAN UNION to promote research and scientific advance in Europe.

Juan Fuertes
ANHP Coordinator and Member of PHA Europe Board

UPDATE ON PH TREATMENTS AND RESEARCH

MACINTENTAN TRIAL COMPLETED
PRELIMINARY RESULTS NOW PUBLIC

The results of the Phase III SERAPHIN outcome study on Macitentan in patients with pulmonary arterial hypertension were announced on April 30 by Actelion Pharmaceuticals.

SERAPHIN is a long-term, event-driven study involving 742 patients in 151 centers from 40 countries, treated for up to a half years with Macitentan, a new dual endothelin receptor antagonist. Patients were allowed to receive PAH background therapy throughout the study, either PDE-5 inhibitors or oral/inhaled prostanooids.

This study was the largest randomized, controlled study in pulmonary hypertension in PH. The study included nearly 500 patients randomized to Macitentan, and showed the following:

- Macitentan, at the 3 mg and 10 mg dose, decreased the risk of a morbidity/mortality event over the treatment period versus placebo. This risk was reduced by 45 percent in the 10 mg dose group. At 3 mg, the observed risk reduction was 30 percent.
- Treatment with Macitentan in the SERAPHIN study was well tolerated. Secondary efficacy endpoints, including change from baseline to month 6 in six-minute walk-distance, change from baseline to month 6 in WHO functional class and time over the whole treatment period to either death due to PAH or hospitalization due to PAH, also showed a dose-dependent effect. A trend in favor of 10 mg macitentan was observed on all cause mortality.

Full data from this study will be made available through scientific disclosure at upcoming congresses and publications.

PAH patients with a long-term treatment to include a clearly defined morbidity/mortality primary end-point. Macitentan, at both the 3 mg and 10 mg dose, decreased the risk of a morbidity/mortality event over the treatment period versus placebo. This risk was reduced by 45 percent in the 10 mg dose group. At 3 mg, the observed risk reduction was 30 percent. Treatment with Macitentan in the SERAPHIN study was well tolerated. Secondary efficacy endpoints, including change from baseline to month 6 in six-minute walk-distance, change from baseline to month 6 in WHO functional class and time over the whole treatment period to either death due to PAH or hospitalization due to PAH, also showed a dose-dependent effect. A trend in favor of 10 mg macitentan was observed on all cause mortality.

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Full data from this study will be made available through scientific disclosure at upcoming congresses and publications.

UPCOMING EVENTS

August 25-29, Munich, Germany
EUROPEAN SOCIETY OF CARDIOLOGY’S ANNUAL CONGRESS
PHA Europe will be present as an exhibitor at the EUROPEAN SOCIETY OF CARDIOLOGY’S ANNUAL CONGRESS 2012. This Congress is currently the largest cardiovascular medicine meeting in the world. It covers all disciplines from basic research to clinical practice.

September 1-5, Vienna, Austria
EUROPEAN RESPIRATORY SOCIETY’S ANNUAL CONGRESS
PHA Europe will also be present with a booth at the EUROPEAN SOCIETY FOR RESPIRATORY MEDICINE’S ANNUAL CONGRESS 2012. This Congress is the world’s broadest respiratory gathering. It involves not only leading experts in respiratory medicine, but also in the entire spectrum of research and practice.

September 12-16, Barcelona, Spain
PHA EUROPE GENERAL ANNUAL MEETING
PHA Europe will be holding its 2012 GENERAL ANNUAL MEETING in Castelledeta (Barcelona) September 13-16 (the photo on the right is from 2011 meeting). Over 40 PH Patient Leaders from 22 European countries have confirmed their attendance.

October 19-20, Sophia Antipolis, France
PH EDUCATIONAL COURSE
This yearly international course has as its target audience mainly cardiologists, pulmonologists and other specialists involved in PH care. PHA Europe has been invited to attend and to present the patient perspective in the context of the G6, a meeting which brings together the major stakeholders in PH.

November 28, San José, Costa Rica
PH LATIN DAY
The Día Latino de Hipertensión Pulmonar is organized by the Society of Pulmonary Hypertension of Costa Rica in collaboration with the PH Latin Society. PHA Europe has kindly been invited to attend and speak and will be represented by one of its Board Members.

Nice, February 27-28 / March 2013
5th WORLD SYMPOSIUM ON PULMONARY HYPERTENSION
PHA Europe has been invited to give its patronage and attend what will undoubtedly be the key PH event in 2013. Traditionally the PH World Symposia, started in Geneva in 1973 and held every 5 years after the Evan edition in 1998 (Venice 2003, Dana Point 2008), have marked the progresses in pulmonary hypertension science and have paved the way for further advancements. The Symposia constitute relevant publications which are among the most cited in this scientific area.
EU CROSS-BORDER HEALTH DIRECTIVE

euroPORD network, a collaboration of patient organisations, national associations and experts in cross-border healthcare, has been established to facilitate cooperation between health systems. This is especially important for rare disease patients who cannot find the right care locally or need to access a centre of expertise in another country. Member States have until October 2013 to transpose the Directive into national law and adopt appropriate measures.

For full text on the network “Read Q’s and A’s” at: http://www.eurocord.org/en/content/eurocord-releases-question-and-answers-help-patients-advocate-their-right-cross-border-healthcare

Next PHAE newsletter

The Winter 2012 issue of the PHA Europe newsletter is due out at the end of December. The first part will feature a report on the PHA Europe Annual General Assembly, which took place in Barcelona, Spain in September 2012. For the section on member associations, we will include reports and photos of events organized at national level in the second half of the year. We would be very grateful if the members could send their contributions by the end of November at latest. These may be sent in the national language, we will provide for translation. Any photos should have a minimum resolution of 1 MB. The newsletter will be available in PDF format and in a printed version. The PDF will be e-mailed and posted on the PHA Europe website. Printed copies will be sent by post to the national associations. Many thanks in advance!
AIMS OF PHA EUROPE

Pulmonary Arterial Hypertension causes breathlessness and is a rare, progressive and currently incurable lung disorder. PHA EUROPE, European Pulmonary Hypertension Association, was founded in 2003 and is registered in Vienna, Austria, as an international non-profit organization. PHA EUROPE is an umbrella organization bringing together Pulmonary Hypertension patient associations across Europe. The primary objective of PHA EUROPE is to establish a narrow cooperation between the members and the European institutions, international organizations and public institutions worldwide and work towards achieving the best possible standards of care for all European Pulmonary Hypertension patients.

WHAT IS PULMONARY ARTERIAL HYPERTENSION?

In patients with Pulmonary Arterial Hypertension characteristic changes occur within the pulmonary circulation, which include thickening of the linings and obstruction of the small pulmonary blood vessels. They are both structurally and functionally abnormal. In severe cases up to 80% of these very small blood vessels are rendered non-functional. As a result the pressure in the pulmonary circulation rises well above normal and this places strain on the right side of the heart. This strain can cause the heart to enlarge and the patient may develop heart failure. This is a disease that can affect all ages and is more commonly seen in females. Pulmonary Arterial Hypertension has an estimated prevalence of about 50 per million population.

TREATMENT OF PULMONARY ARTERIAL HYPERTENSION

Over the past decade a number of evolving therapies that either use complex delivery systems such as 24-hour intravenous or subcutaneous drug infusion, drug inhalation and, more recently, oral medications, have transformed the outlook for PAH patients. PAH is a condition that can be rapidly progressive and needs careful, ongoing expert care and management. The disease can be insidious in its onset, with unexplained breathlessness and tiredness as its two main symptoms. If undiagnosed and/or inappropriately treated the average life expectancy for these patients is estimated to be around 2-3 years. For those patients who fail to respond to medical therapies double lung, or in some cases, heart and lung transplantation may be appropriate.

THE FUTURE FOR PULMONARY ARTERIAL HYPERTENSION

While a cure for this aggressive and life threatening disease is still some way off, there is much to be optimistic about. There are an ever increasing number of therapies available for the effective treatment for Pulmonary Arterial Hypertension, which improve the quality and length of life. The aim for the present should be to ensure that all patients with Pulmonary Arterial Hypertension have access to centres of excellence in the diagnosis, management and ongoing treatment of this disease.