



# PULMONARY HYPERTENSION MariposaNews

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SUMMER ISSUE OF THE NEWSLETTER OF PHA EUROPE,  
EUROPEAN PULMONARY HYPERTENSION ASSOCIATION



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

*Brussels, June 6 2012*



# Editors memo

## Summer edition 2012

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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, UK), Jean-Luc Vachiéry (Erasmus, 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

## REPRESENTING PHA EUROPE, JANUARY-JULY 2012

In the first half of 2012 PHA Europe's President and Vice President represented and/or spoke on behalf of PHA Europe at a number of meetings, conferences, symposia:

February 9-10-11, Paris	PAH Patient and Care Survey Steering Committee meeting
February 29, Brussels	Rare Disease Day awareness campaign launch event and press conference
March 3-4, Rome	7th Bayer PH Symposium
March 15-16, Munich	PAH Patient and Carer Survey meeting
March 17, Fort Myers	1st Support group meeting for South West Florida (in cooperation with PHA)
March 22-23-24, London	John Vane Memorial PH Symposium and United Therapeutics manager meeting
March 27-28, Prague	Novartis manager meeting
May 5, Madrid	PH World Day official celebration
May 8-9, Brussels	Meetings with MEP's and other EU stakeholders
May 23-25, Brussels	European Conference on Rare Diseases
June 6, Brussels	PH Policy brief and Call to action launch event in European Parliament
June 8-9 June, Barcelona	6th Bayer International Workshop
June 13, Vienna	Worldwide Actelion General Manager meeting with J.P. and Martine Clozel
June 15-16, Berlin	10 years of Oral Therapy, Actelion
July 5-6, London	Communiqué Awards Ceremony
July 12, Basel	PAH Patient and Care Survey meeting
July 25-26, London	3rd European Orphan Drug Summit

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# A Call to action on pulmonary hypertension: the European Parliament launch event

Bruxelles, 6th of June 2012

PHA Europe decided in 2011 to convene a pulmonary hypertension roundtable to provide a forum for the sharing of PH knowledge and expectations across diverse stakeholders and geographic boundaries. At a meeting in Amsterdam in September 2011, the roundtable members, who reflected a wide range of clinical, patient, research, and industry interests, discussed the need for all PH patients to have access to optimal screening and diagnosis, appropriate treatment, psychosocial support, and integrated care through centres of expertise. Members subsequently agreed upon the framework for a call to action to enhance public awareness about the personal and societal burdens imposed by PH, and to improve the care of persons living with the disease. The PHA Europe Call to action was presented at the European Parliament on June 6 2012. The launch event was a resounding success. MEP Karin Kadenbach served as host, with support from MEP Hannes Swoboda. Speakers included Roundtable members Gerald Fischer (PHA Europe President), Pisana Ferrari (PHA Europe Vice President), Nazzareno Galiè (University of Bologna), Simon Gibbs (Imperial College, UK), and Jean-Luc Vachiéry (Erasmus, ULB, Belgium). Paola Testori Coggi, Director General of DG SANCO (Health and Consumers), addressed assembled guests about EU action in cardiovascular disease and pulmonary illness and Laurent Nicod, Scientific Committee Chair at the European Respiratory Society, lent his support to our initiative. Guests included Members of European Parliament and representatives from DG Research, DG SANCO, the European Patients' Forum, and the Standing Committee of European Doctors, among others.



## 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



From left: Gerald Fischer and Hannes Swoboda



From left: Karin Kadenbach and Gerald Fischer



From left: Malcolm Allison, Pisana Ferrari, Gerald Fischer, Jutta Ulbrich and Thomas Bertschik



From left: Laurent Nicod, Simon Gibbs, Karin Kadenbach, Hannes Swoboda and Gerald Fischer



care is by means of expert centres:

- High patient volume units can achieve better patient satisfaction, fewer complications, and shorter hospital stays than general cardiology or respiratory medicine departments.
- Care delivered in expert centres also results in lower costs.
- Multi-professional clinical teams can make an accurate diagnosis, provide the best treatment and monitor progress, and intervene proactively whenever there are signs of deterioration.
- Vigilant long-term care is also required to ensure that drug treatments are used to their best advantage and the timing of lung transplantation is optimal.

A multi-professional team can support individuals throughout their patient journey, which affects the physical, psychological, social, and spiritual aspects of their lives. Furthermore, expert centres are the right place to conduct clinical trials of new and potentially better medicines. In short, expert centres provide the opportunity for the best, most timely, and cost-effective care for PH patients. No one model of expert centres will fit all European countries. Instead, a common set of principles of best clinical practice might drive a network of centres sharing their experience across borders. A European network of centres might be established as has been described for other rare diseases.

#### 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:

*Physical challenges:* PH patients are more at risk of infections. Some drugs are complex, invasive, and painful and many have side effects such as syncope, arrhythmia, hemoptysis and dysphonia. There may be sleeping difficulties and problems concentrating and reading; there can be changes in physical appearance, self-perception, and problems with intimacy. Co-morbidities, impaired physical capacity, and deconditioning are frequent.

*Emotional challenges:* At the moment of diagnosis there will be shock, fear, and disbelief. There is an immediate need to rethink priorities and reorganize one's life around the disease. A difficult patient journey follows the diagnosis.



From left: Hall Skarra, Juan Fuertes and Luc Matthysen



Adam Heathfield



From left: Gerald Fischer, Pisana Ferrari and Keith Allen

There is a sense of isolation, of not being understood, and fear and uncertainty about the future. Anxiety and depression are common.

*Organisational challenges:* PH leads to decreased capacity to work or even loss of work which can result in financial problems for the patient and family. Patients experience difficulties in undertaking household tasks; in many cases home adaptations are required. Blood tests, check-ups, and drug management all need to be planned. Bureaucratic issues inherent in access to drugs, surgery, insurance, and pensions need to be faced. In other words, social, family, and work life are all severely disrupted.

*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 (PEA, 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 specialized 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 care 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,



From left: Grigorij Kogan and Nazzareno Galie



From left: Morgan Long and Thomas Bertshik



Sylvie St. Laurent



From left: Florence Csabo Perrin and JP Maréchal



From left: Martina Schmidt and Gerald Fischer





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 subtypes 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 care, research, and education. Prof. Nicod underlined the serious nature of PH, which, if left untreated, can lead to heart failure. The course of the disease depends on how quickly PH is diagnosed, how advanced the symptoms are and whether there is an underlying condition (e.g. COPD). New treatment approaches for pulmonary hypertension and links with industry have created positive synergies. National and international registries have been established. Patient groups are an invaluable support. Despite this progress, more needs to be done. There is a need for improved awareness and screening, as early diagnosis and detection is essential. Likewise, continued support for clinical research and innovation is vital. There is also a need to optimise care to improve patient outcomes. Research efforts need to be stepped up, as well as a strategic coordination amongst disease areas (as chronic diseases often occur together, particularly in older people). Turning biological knowledge

into clinical progress will require new resources and support systems. The EU research and innovation programme, HORIZON 2020, proposes a total of €8 billion for health research. Prof. Nicod called on the European Parliament to defend and perhaps increase the health research budget.

#### NAZZARENO GALIÈ

##### ISTITUTO DI CARDIOLOGIA UNIVERSITÀ DI BOLOGNA

The final speaker was Prof. Nazzareno Galiè, who articulated some recent successes in the field of PH. For example, there is now an organized international and multidisciplinary group of scientists and clinicians, patients associations are far more active, and G5 PH annual (stakeholder) meetings are being organized. The new PH classification was adopted by WHO, and eight drugs are approved, with more in the pipeline. Morbidity and mortality values have improved, medical practice guidelines are in place, and the pharmaceutical industry is far more involved. For the 2013 WORLD PH SYMPOSIUM ON PH, 145 experts are already at work in 12 thematic working groups. Despite progress, Prof. Galiè outlined some of the remaining issues, which include the need for:

- Funding for independent research and innovation;
- Public awareness and fundraising for patient associations;
- Educational initiatives from scientific societies;
- Better care facilities;
- Most importantly, a cure for PH and PAH.

#### AUDIENCE DEBATE

In the debate following the presentations the following issues were raised:

- *How will we define the success of the Call to Action in a year's time?*  
Participants stated that some of the indicators for success could be scientific progress and investment in research, with uniform cross-country PH care and disease management. Increased media attention would 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



From left: Hall Skaara, Marion Delcroix, Laurent Nicod



From left: Gerald Fischer and Nazzareno Galiè



Paola Testori Coggi

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, and 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.

- *How do we bring less advanced countries on board?*

There are huge differences between the various Member 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 the 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 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. Equal access to treatment should follow central authorisation. However, while access to medicines should be improved, so should training of health professionals. If drugs are not being prescribed and managed appropriately, patient will suffer.

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

Full text of Policy brief and Executive summary can be downloaded from the PHA Europe website at: [www.phaeurope.org](http://www.phaeurope.org)  
More photos can be found on PHAE Europe's Facebook page: <http://www.facebook.com/media/set/?set=a.433128056708596.81883145.141060385915366&type=3>



Jutta Ulbrich and Adam Heathfield



Jean-Luc Vachiéry



Simon Gibbs



Marion Delcroix and Laurent Nicod



From left: Luana Coccia, Laurene Souchet and Sarada Das



Juan Fuertes and Milan Luknar





# Official celebration of World Pulmonary Hypertension Day

Madrid, 5th of May 2012



From left: M.A. Gomez Sanchez, Gerald Simonneau, Cecilia Martinez, Nazzareno Galiè, Lewis Rubin, Irene Delgado, Jaime Morales, Rino Aldrighetti, Juan Fuertes  
Front row from left: Gerald Fischer and Denneys Niemandt

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 (eg scleroderma) as well as rare disease federations (EURORDIS, 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.

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.

## 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, fears, 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 contribute to historical, social and economical development. Today I have the great honor and the limitless joy to welcome you to the first celebration of the WORLD PULMONARY HYPERTENSION DAY.



From left: Jutta Ulbrich, Noriko Murakami and Cecilia Martinez



From left: Gerald Fischer and Sebastian Bonnet



From left: Pisana Ferrari, Peter and Yveta Makovnik at PHA Europe booth



From left: Miguel Angel Gomez Sanchez and Gerald Simonneau



I want to transmit a message: open your minds and your hearts because what we are going to listen to during this celebration must push forward all those things that will offer the chance to live to millions of people all over the world and to find a cure in a future that draws nearer and nearer.

I thank from the bottom of my heart the Members of the Scientific and Honor Committees for accepting our invitation and for placing their trust in what seemed to be a dream. Thanks to the speakers and moderators who answered our call and shared their wisdom with us. Thanks to the pharmaceutical industry because this would have not been possible without your support. Thanks to the Presidents of patients' organizations in the world who are here today to share this great celebration with us. Thanks to the long time members of the Spanish National PH Association who supported us from the very beginning to achieve what ANHP is today and thanks to the new members who have placed their trust in us although they don't know us well yet. I want to give special thanks to my comrade in this struggle, Juan Fuertes.

### PAH: AN UNCONVENTIONAL JOURNEY

#### PROF. NAZZARENO GALIÈ

Professor at the University di Bologna

The Pulmonary Circulation was formally described by William Harvey's in 1628 in his *Exercitatio Anatomica de Motu Cordis et Sanguinis*. By observation of the blood flow in vessels and through dissection, Harvey established that the blood circulates from the heart to the tissues via the arteries and then back to the heart via the veins and through the lungs. The "discovery" of PH was first made 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 catheter. 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 British researcher John Vane, (who received the Nobel Prize for Medicine in 1982 for this), Nitric Oxide in 1998. Probably Dr. Salvador Moncada made both discoveries (he was a Fellow in both the studies). The endothelin pathway was discovered in 1985 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 anorexigens. 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 treatment 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 medical treatment can improve outcome! This is (should be) the main endpoint. A metaanalysis 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. Macintentan, 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 periodically, 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



Ray Pezzani and Gerald Fischer



Cecilia Martinez with Dr. Jaime Morales



Pisana Ferrari and Gerald Simonneau



Jutta Ulbrich and Silke Gerlach with one of the guests at the conference



From left: Lewis Rubin, Gerald Fischer, Nazzareno Galie and Gerald Simonneau



trials. Fortunately, many steps forward have been taken in the last 15-20 years, and above all, survival is improving.

The ESC /ERS guidelines set out some criteria for referral centres:

- At least 50 patients treated
- Two new referrals per month
- Multidisciplinary teams
- Links to surgery facilities (TX, PEA)
- Audit with survival analysis
- Should take part in clinical trials
- Should have relations with national and European patient associations

If you look at the situation in Europe there are many very different “models” for referral centres.

In France there is one reference centre and there are different regional/local centres with minimum requirements. There is great interaction between the centres as well as close links with research centres. France is a great model for PH, with a lot of experience.

In the United Kingdom there are eight referral centres, seven for adults and one for children. PH Patients can only be treated in the official centres.

In Spain the situation is very different. We have a population of 47 million and the country is divided into 17 regions, each one with its own Minister for Health. There is no “official” PH centre. Units have been created “spontaneously”, they have acquired experience, share data and collaborate. It is a sort of “non-official” network. To prove that it works suffice it to say that a national registry has been set up. Data on prevalence, survival etc. are available. The European guidelines provide criteria for referral centres, but these are adapted to each country. Spain has its own standards (set by the Pulmonology and Cardiology Societies). In many cases it is difficult to follow the guidelines strictly, there are many gaps. We have more freedom and flexibility to work with the drugs, for example. Criteria are set for the units: doctors interested in setting up must have proven experience, the nurses as well. It would be interesting of course to have more TX and PEA facilities, but this is not considered vital as patients can be referred to other centres. External audit is requested to make sure we keep up to standards. It is essential to collect and share data as this is the only way to really evaluate the results. It is also important to take part in clinical trials and in the national registry. There should be specific areas to hospitalize patients and in particular for functional class IV, with specialty nurses. The coordinator and all structures should collaborate. Interaction with the local clinics is also vital. These should have referral criteria to send to other centres if necessary. To conclude, the collaboration of multidisciplinary teams with nurses is extremely important. Hopefully one day it will also be possible to provide support at home.

## 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 important 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, lustre 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:

- conference 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 SIMMONEAU**

*National PH reference Centre, Antoine Bécère 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 Macintentan), 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 956 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 than 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 periportal 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 Antilla). In sub-Saharan Africa the prevalence is as high as 30%. Worldwide, WHO has estimated that around 300,000 babies with severe forms of this disease are born each year.
- *PH associated with High Altitude*  
140 million people are living at high altitude (>2,500m). Chronic Hypoxia can cause an 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 years have 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.

*Pisana Ferrari*



International PH leaders at conference lunch



Noriko Murakami, Peter Makovnik and Iveta Makovnikova





# 10th International PH Conference and Scientific Sessions PHA, USA

Orlando, 22-25 June 2012

On June 22-24, 2012, more than 1,500 patients, caregivers and medical professionals came together in central Florida for the largest PH gathering in the world-- the Pulmonary Hypertension Association's (PHA) 10th International PH Conference and Scientific Sessions. For three days, attendees had the opportunity to learn from, encourage, and inspire one another as they continue on in the fight against PH. The 10th International PH Conference and Scientific Sessions truly lived up to its theme: "The Power of One: From a Kitchen Table to Around the World." This year's Conference was not only the most highly attended in PHA's history, but also the most global. International leaders came from across the world to interact with patients, caregivers and medical professionals, and to find information about the advances in PH that could make a difference in their communities back home. Attendees came from Argentina, Australia, Brazil, Bulgaria, Canada, China, Ecuador, France, Iran, Israel, Japan, Mexico, Nepal, New Zealand, Nigeria, Norway, Saudi Arabia, Slovakia, South Africa, Spain, Turkey, United Kingdom, USA and Venezuela.



## INTERNATIONAL LEADERS' SUMMIT

The **INTERNATIONAL PH CONFERENCE AND SCIENTIFIC SESSIONS** draws in an increasing number of participants from around the world each time the event is held, and PHA is pleased to offer programming specifically for global attendees. In 2010, PHA hosted the First International Leaders' Summit for PH association leaders

from all over the world. The 10th International PH Conference and Scientific Sessions featured the Second International Leaders' Summit as well as many new programs for global patients, PH organization leaders and medical professionals.

The weekend kicked off with the **INTERNATIONAL LEADERS' DINNER** with the PHA Board of Trustees on

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





reflected the theme. Dr. Lynn Brown (University of Utah) explained what these meant on Friday when she described PHA's new Early Diagnosis Campaign.

The **SCIENTIFIC SESSIONS** have grown in both attendance and quality since the first was added to Conference in 2004. This year with over 500 medical professionals attending, learning and teaching, the comments from physicians and researchers proved that Dr. Todd Bull (University of Colorado) and his committee brought science on their chosen topic – The Genetics of Pulmonary Hypertension – at the highest level. Dr. Kazuyo Kegan, a researcher from Johns Hopkins University noted, “As a basic science researcher, I never had this level of opportunity to see and communicate with people who have PH and their families. This reminds me of our goal for the research (which we tend to forget in our daily life), and motivates us”.

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 the point of running out of supplies. Studies included a wide range of topics, including the psychological effects of having PH, genetics, biomarkers and restless leg syndrome.

The Conference Opening Session featured the new **HISTORY OF PHA VIDEO**. This video captured the heart of PHA's story, and it was wonderful to see people's reactions to the dramatic opening highlighting PHA's surviving founders. See <http://pharino.blogspot.it/2012/07/from-kitchen-table-to-around-world.html>

**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 Benza (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>

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.PHAAssociation.org](http://www.PHAAssociation.org). You can also find more Conference stories and links at PHA's blogs: Table Talk Wired and Empowered by Hope.

Haley Elmers  
Manager, Office of the President, PHA

**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 Medical Professionals:

PH 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 Slubowska (now Knott), Christine Dickler and, most recently, Meghan Tamaro 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 1990's 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.







# 6th European Conference on Rare diseases and Eurordis Annual Assembly

Bruxelles, 23-25 May 2012

The 6TH EUROPEAN CONFERENCE ON RARE DISEASES AND ORPHAN PRODUCTS took place in Brussels 24-25 May. It was the largest to date, with almost 700 delegates attending from 55 countries (Europe, USA, Canada, Latin America, Australia, Far East). Approximately one half of the attendees were patient representatives. Health care professionals, academics and policy-makers represented one third of the audience and the remaining participants represented the biotech and pharmaceutical industry. The event was entitled, "A better future for patients: Shaping together the Agenda 2020". There were 36 different sessions involving more than 100 speakers. Topics discussed include: National Plans for Rare Diseases, Centres of Expertise and European Reference Networks, Information and Public Health, Research, Access and Regulation of Orphan Drugs and Therapies, and Patient Empowerment. The presence of EU Commissioner for Health, John Dalli, highlighted the commitment of the EU to the field of rare diseases.

Prior to the Conference, on the 23rd of May, EURORDIS held its Annual General Assembly and this was followed by a selection of forums and capacity-building workshops designed to empower patients and patients' advocates and encourage learning from each other's experience. It was a pleasure and honour for me to attend the EURORDIS General Assembly and parts of the ECRD, representing PHA Europe, together with our Board members and colleagues Mélanie Gallant Dewavrin (HTAP France) and Luc Matthysen (HTAP Belgique). HTAP France's poster on the results of a recent patient survey conducted by the association was selected by the organizers to be part of a very broad and interesting poster session. All of the presentations from the ECRD are available on the Eurordis website. In this article I have included summaries of only a few selected presentations and workshop discussions.



## EU COMMITMENT IN RARE DISEASES

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 EUROPLAN project and the 2012 JOINT ACTION provide common guidelines.
- the Creation in 2010 of the EU COMMITTEE OF EXPERTS ON RARE DISEASES (EUCERD).
- 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 PROGRAMMES FOR RESEARCH alone 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 (2000).

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".

## EURORDIS ACHIEVEMENTS AND PLANS FOR FUTURE

It was very impressive to hear from EURORDIS 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. EURORDIS membership has increased enormously and currently stands at over 500 members in 48 countries, with new alliances being set up in Russia. EURORDIS 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 EURORDIS must contribute to safeguarding the European social agenda.

Yann Le Camm, EURORDIS 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, Eurordis 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 EUCERD, the EUROPEAN COMMITTEE OF EXPERTS ON RARE DISEASES (7 Eurordis 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 EUCERD 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, patients 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 actively participate 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? How can patients participate in the evaluation of Centres of Expertise?”

Developing Centres of Expertise (CE's) 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 ERN's a legal basis (Recommendations are not legally binding for the Member 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). EUCERD, the European Expert Committee on Rare Diseases, in October 2011) adopted a set of Recommendations. The EUCERD rec-

ommendations seek to introduce harmonious standards of quality practices by elaborating criteria for the Member States to incorporate into their process to designate Centres of Expertise (full text is available at [www.eucerd.eu](http://www.eucerd.eu)).

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

### Centres of expertise (CE's)

#### Mission and scope:

- *Definition and coverage:* CE's 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:* CE's to collaborate with patient associations to bring the patient perspective, CE's to respond to the needs of patients from different cultures.
- *Core competencies:* Multidisciplinary, including social services, palliative care, rehabilitation. CE's to contribute to building healthcare pathways from primary care and to have links to specialised labs and other facilities.
- *Role in spreading information:* CE's to contribute to the elaboration of good practice guidelines, to provide education and training of healthcare professionals, to provide information and collaborate with other CE's, with PA's, Orphanet etc.
- *Research:* CE's 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 for the designation of 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.
- *Multidisciplinary, inclusiveness:* 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 definition criteria. The designation will 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 Member 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 CE's were designated at national level and 500 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 interviewed 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!) It actually worked out well in practice. Some centres even supported the establishment of patient organizations where there were none. The patient associ-



Marta Hanka Meutgeert



Luc Matthysen at the Eurordis capacity building workshop

ations on the other hand to do 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 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](http://www.eucerd.eu)).



Christel Nourissier



## PATIENT ADVOCATE FORUMS

EURORDIS forums are designed to offer patient advocates an opportunity to meet, discuss and learn from each other. In each session, a few patient representatives are invited to share their experience and this is followed by a 10 min Q&A. I attended the forum on “Advocacy actions for access to treatment & medicines”, which was moderated by Jonathan Ventura, RADIORG, Belgium (Belgian Rare Disease Organization). Three national advocacy experiences were described, the first one of which failed despite enormous efforts, one which was successful through joining forces with another group and the third is still pending a solution.

The first case study 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 intake diet. 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) decide 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 the 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 REVLIMIDIN IN BELGIUM**. Myeloma is a cancer of the plasma cells of the bone marrow. The speaker, Mrs Greetje 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 Hanka Meutgeert, from VKS, the Dutch association for metabolic diseases spoke about the **REIMBURSEMENT OF ENZYME REPLACEMENT THERAPIES IN THE NETHERLANDS**. The first ERT developed was for Gaucher (type I) in 1991, 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-dependant (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 metanalysis 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.

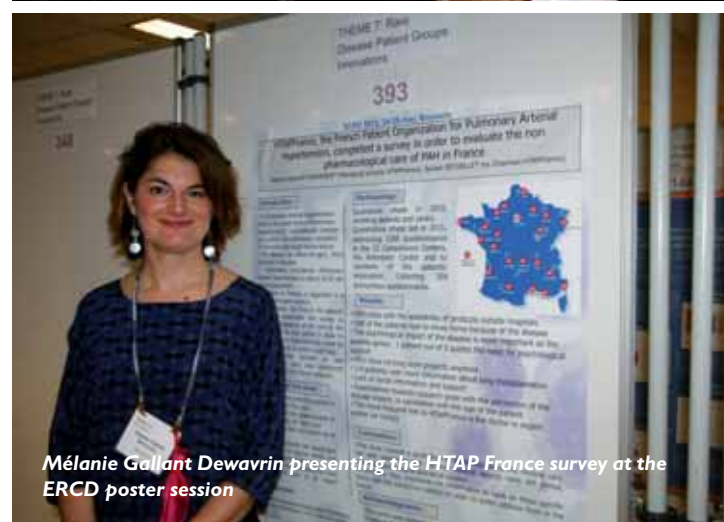
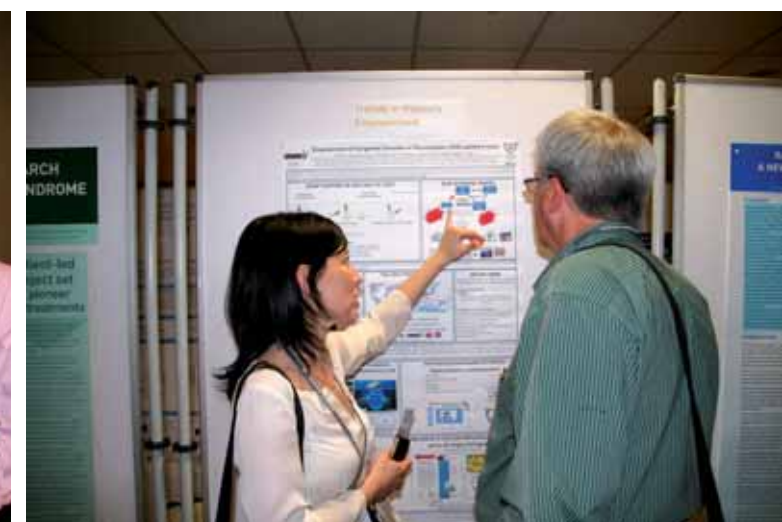
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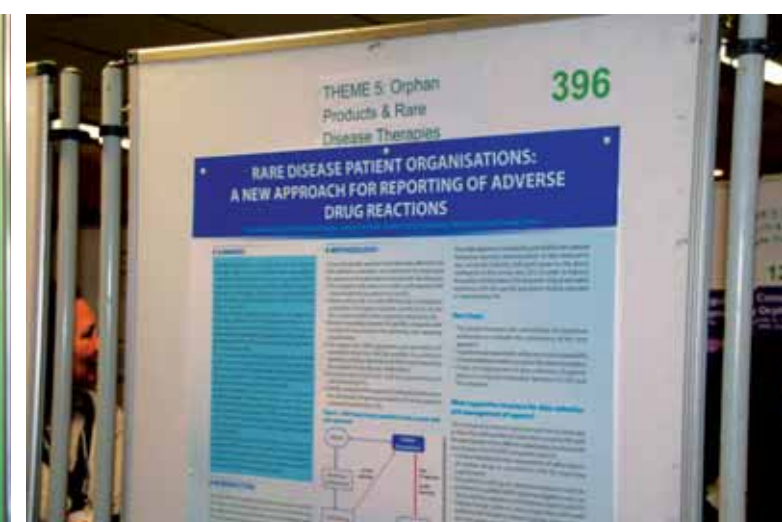
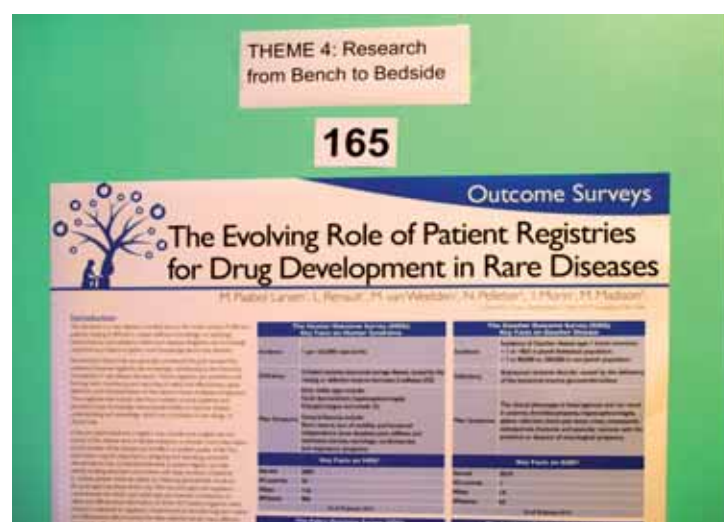
Poster session at the ECRD 2012



Domenica Tarascio (second from left), Head of the Rare Diseases Center of the Italian Ministry of Health, with fellow Italian researchers



Mélanie Gallant Dewavrin presenting the HTAP France survey at the ECRD poster session







## 3rd Bayer International PH Nurse Workshop

Barcelona, 8th of June 2012



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 FUTURE?

**J. CIFRIÁN**

Great advances have been made in understanding the pathobiological 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 (SERAPHIN study now completed, it is a long term, multicenter trial with a very large cohort).
- Selixipag: an elective IP receptor agonist (phase III, GRIPHON study).
- Oral treprostinil: an oral prostanoid with sustained release tablets (FREEDOM-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 (PATENT and CHEST studies).
- Imatinib: an oral PDGF inhibitor currently being used in certain forms of leukemia. Has anti-proliferative and pro-apoptotic 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 GP's to specialists), therapeutic education, answers to acute incidents, etc. Integrated care is equal to a multidisciplinary offer of personalised 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, for example, intimacy 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: is more therapy the only option? Quantity or quality of life? It is up to patients to decide. For the majority of patients quality of life is the single most important factor when choosing a treatment. For patients it is 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 17 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-Gobielje 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, pediatricians 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, 558 of which alive, 25 post TX 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 SAPH doctors (there is some provided by industry) and there is no real job description or definition of competences. This is a problem which needs to be further addressed. Despite this the nurses are highly motivated and work with enthusiasm.

## THE ROLE OF RESPIRATORY AND MENTAL TRAINING

N. EHLKEN

Dispnoea, as measured by the 6 mwd test, is related not only to the disease but also to fear of exercise, weakening of the muscles, deconditioning, breathing pace. Patients tend to breathe fast when they are tired from the physical effort and this is not good. Some simple breathing techniques can be of great help. Pursed lip breathing, for example, has the effect of getting all the CO<sub>2</sub> out of the lungs. On exertion or stress it also helps to calm down. It can be used in combination with mental imagery. It can be done with an arm moving out from the center to the back, there is an increase in activation of circulation. It can be used in stress situations also for cardiac patients. Physical rehabilitation is another key issue. A training program was conducted by the Heidelberg PH team in 2003. There was a baseline examination and reassessment after 3 weeks of training, which showed benefits for supervised physical exercise in PH patients. A more recent analysis, on 183 severely affected PH patients, has shown a change in distance walked at the 6 mwd of 85 metres (the average with medication as per data of a recent meta-analysis is 35 metres!) There was improvement also in quality of life, particularly in the more affected patients. PH patients have an impaired physical capacity but can be helped with exercise and mental training. Self assessment of the level of exertion is crucial and they should be guided in this.



## 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, panick 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 dispnoea 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 (Patient Health Questionnaire-2) is 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 not 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 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 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 can provide invaluable support and this also has positive effects in terms of compliance to treatments and on outcome. Nurses and patient associations can benefit greatly by collaborating and working together.

The physical burden of PAH is quite challenging as PAH-patients, in addition to breathlessness and fatigue, may experience syncope, hemoptysis, arrhythmia, dysphonia. They are also generally more prone to infections and there may co-morbidities. Most drugs have side effects and some are very complex to manage, invasive and/or painful. Information and training can help patients better understand their condition and prevent serious deconditioning and unhealthy life styles.

PH patients live in fear and uncertainty and life can really be an emotional rollercoaster. By interacting with patients on a regular basis nurses are ideally placed to detect signs of anxiety and depression (which in turn can affect prognosis and QOL) and refer for counselling and psychological/psychiatric support. In some cases they may decide to refer to a social worker.

PH is quite a challenge also in terms of treatment and follow up management. Nurses and patients can develop logs, patient diaries, checklists.

Finally, tele-medicine has great potential as a future supportive tool and will require close future collaboration between nurses and patients.

### PARTNERING WITH INDUSTRY IN ADVANCING INNOVATIVE PH PATIENT MONITORING

**A. DUSEN**

The pharmaceutical industry can play a very important role in advancing PH care through partnership with the different stakeholders.

*Patient associations:*

If you take the example of HIV, in the early days, patient activism was antagonistic to industry, but eventually ended up in partnership. In PH the industry can partner with patient associations (eg. EURORDIS, PHA, PHA Europe) and there are many examples of successful projects undertaken together.

*Other companies:*

Industry can also partner with each other as in the AMBITION STUDY which studies monotherapy versus double "upfront" combination therapy in PAH (Lilly, Glaxo-SmithKline and Gilead). The issue of sequential versus upfront combination therapy was also part of the HIV debate: in the mid 80's HIV patients were on monotherapy, in recent years the trend has been to start with the most aggressive therapies right from the start and as a result there has been better response and HIV has practically become a chronic disease. For PH we are now in the "sequential" stage and there is limited evidence on combination therapy. Part of the problem is the heterogeneity of the population, which leads to empiric drug sequencing, not specific to the different phenotypes. There are also gaps in diagnostic procedures and bio-markers. Industry could help by partnering for research into bio-markers (novel diagnostics). Early intervention could change the course of the disease and regular monitoring would ensure timing for changes is right.

*Nurses:*

Industry can also partner with nurses, for example, in relation to observational data bases, registries, investigating the results of regular monitoring in terms of patient feedback and compliance. A study could involve nurse home support and its efficacy. It would also be very interesting to develop a model of a data-collecting device which the patients could use at home. Possible remote devices could measure blood pressure, heart rate, cardiac output and other parameters to measure exercise capacity, etc. The device could feed the information back to the clinic. It would also be extremely important to engage the patients to see how much they understand, how well they are able to track their parameters. They could be encouraged to self-monitor through the use of logs, etc. The way forward is tele-medicine and novel technologies and industry can make a contribution in these and many other respects.

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

**A. PONNABERANAM**

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 medication, 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, education and liaison in collaboration with a specialist. Patients are gradually weaned off oxygen when saturation is over 90%. Most are weaned off before dismissal.

*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 to have performed the highest number of PEA's.

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-coagulation 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. With shorter cardiac arrest periods cerebral impairment 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 over the years 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.

Pisana Ferrari





# AUSTRIA

## VIENNA ZOO RUN

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](http://www.lungenhochdruck.at)





## BELGIUM



### RARE DISEASE DAY 2012

RADiORG.BE, the Belgian organization of EURORDIS, 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 to the one for lung transplant. Many visitors came to the booth and asked for information about PH.



### GAM OF EURORDIS 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 Pisana 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



### 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 50 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 later. 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 second 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 third 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

## CZECH REPUBLIC

### 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 Seč (see photo below).

### CARDIOLOGY CONGRESS BRNO

SPPH participated in the XX Annual Congress of the CZECH SOCIETY OF CARDIOLOGY May 13 to 16 with a booth. It presented the results of the "Breath-taking" campaign and informed professionals about pulmonary hypertension. Visitors at the booth were invited to test their knowledge of the disease. This year's event was attended by 3,300 participants.

### WEEKEND MEETING MEMBERS SPPH

From June 16 to 17 SPPH members met in Seč 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 Nôtová, 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.

Kateřina Nováková  
Sdružení Pacientů s Plicní Hypertenzí  
[www.plicni-hypertenze.cz](http://www.plicni-hypertenze.cz)







## FRANCE



From left: Rino Aldrighetti from PHA and Mélanie Gallant Dewavrin



From left: Mélanie Gallant Dewavrin with Dorothy Olsen, founder of PHA

### 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 there 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](http://www.htapfrance.com)







## GERMANY

### RENE BAUMGART RESEARCH AWARD

René Baumgart was Bruno Kopp's nephew. He was diagnosed with PH at age 19 and died at the age of 23. The René Baumgart Foundation, founded in 2001 to commemorate him, grants a yearly award of €5,000 for the promotion of medical research in the area of PH (children and adults). The award is addressed to cardiologists, pulmonologists, pediatric cardiologists, cardio-thoracic surgeons and basic scientists. Important evaluation criteria for the scientific papers submitted are originality, innovation and clinical relevance of the work. Applications for the 2013 award are now

open and proposals must be submitted before the 31st of December 2012. The winner will be announced in the course of a ceremony at the 54th CONGRESS OF THE GERMAN SOCIETY FOR PULMONOLOGY AND RESPIRATORY MEDICINE in 2012 in Hannover, March 20 to 23.

### NEW PRESIDENT

Phev has a new President, Mr Hans-Dieter Kulla.

*pulmonale hypertonie e.v.*  
[www.phev.de](http://www.phev.de)



### PHEV IS MOURNING FOR BRUNO KOPP

Bruno Kopp was the initiator, co-founder and the first president of the German Pulmonary Hypertension Association (ph e.v.) in Rheinstetten, Germany. He died on February 7<sup>th</sup>, 2012 due to Pulmonary Hypertension in the presence of his family in the Hospital of the University of Giessen.

Bruno Kopp was born in Aalen, Germany on August 22<sup>nd</sup>, 1954. After school he started training to become a mechanic. When he was diagnosed Pulmonary Hypertension (FPAH), he had to change profession and became an educator for handicapped children, and later teacher of special pedagogics.

In 1996 Bruno Kopp initiated and founded the German patients' support association Primary Pulmonary Hypertension - PPH e.V. - with

friends and relatives. Since then he had been President of the association, that changed its name to pulmonary hypertension e.v. in 2001. In 2001 ph e.v. founded the René-Baumgart-Foundation. Bruno Kopp was a member of the board and it's Secretary. In 2003 he initiated and organized, together with the Dutch PH friends, the first meeting of the European PH associations in Vienna. In the same year PHA Europe was founded in Brussels. He was one of the founding members and was working for PHA Europe as Vice-President and later as President from 2008 to 2009. For his outstanding work and his commitment for the people living with Pulmonary Hypertension Bruno Kopp was awarded the Order of Merit of the Federal Republic of Germany in 2007. In spite of the bereavement of Bruno Kopp for the association, the work of ph e.v. will go on in the spirit of Bruno Kopp.

Hans-Dieter Kulla  
President *pulmonale hypertonie e.v.*



## HUNGARY

### RARE DISEASE DAY FOLLOW UP

The Hungarian PH association was represented by a great number of members on Rare Disease Day (RRD) held in Castle of Vajdahunyad, Budapest on 25<sup>th</sup> of February. The event, organized by the Hungarian subsidiary of EURORDIS (RIROSZ), mainly focused on solidarity and current health care initiatives (in particular the NATIONAL HUNGARIAN SCHEME ON RARE DISEASES). We attended several presentations and as a patient organization had an opportunity to participate in roundtable negotiations with representatives of the NATIONAL HUMAN RESOURCES MINISTRY.



### RESPIRATORY SOCIETY ASSEMBLY

It has become a tradition that the HUNGARIAN RESPIRATORY SOCIETY invites the HUNGARIAN PH ASSOCIATION to make presentations at their annual scientific assembly. This year Dr. Kristof Karlocai, our Vice President, spoke of the difficulties in recognizing chronic pulmonary embolism and of drugs for PAH patients. I spoke about issues involved in running a patient association, including the legal and bureaucratic constraints, as well as about the

psychological aspects of the disease. The event closed with a Q&A section, during which PAH patient patients could share their stories with the audience. We had a good press coverages in printed magazines as well as in magazines. Our active appearance is resulting in continuous inquiries from potential patients asking assistance in their problems from our Board members.

### ANNUAL PATIENT MEETING

Tüdőér Egylet successfully raised its membership to 80 in the first half of 2012. The association held its regular Annual Meeting on 22<sup>th</sup> of May. As per current legislation for non-profit associations, I presented the annual statement and accounts, in my capacity as President, and these were approved by the general meeting. Following the more formal part of the meeting there was an opportunity to mingle and to get to know the new members of the association. The atmosphere was very pleasant and relaxed and we spent quite a few hours talking with each other.

### LUNG TRANSPLANT IN AUSTRIA

One of our members went through lung transplant surgery in Vienna at the AKH (ALLEGEMEINE KRANKENHAUS) and both Austrian - and after she was transported back to Hungary - Hungarian association members visited her in course of the recovery.

### WEBSITE

A lot of pictures and video recordings were taken on all the above events and are posted on the website of the association ([www.tudoer.hu](http://www.tudoer.hu)). Dr. Kristof Karlocai, Vice President of the association, also posted several medical articles on the recognition of PH symptoms as well as on the clinical care of the disease.

Eszter Csabuda, Tüdőér Egylet  
[www.tudoer.hu](http://www.tudoer.hu)







## ISRAEL

## 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](http://www.phisrael.org.il)*



## ITALY-AIPI

## 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.apiitalia.it](http://www.apiitalia.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 Vivenzio), 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 had brought with her a fantastic gift: a booklet edited on the occasion of her first Photo Show (now on display in the BG Hospital Hall). After the show, Giorgia's photos will be put up for sale at a charity auction on behalf of AMIP. Here are the opening words of George's booklet: "I'm Giorgia and I'm 15 years old. During these last five years the Pediatric Hospital Bambino Gesù has been my second home. In fact, because of pulmonary hypertension, I had a heart and lung transplant. Being obliged to spend most of my time closed within the walls of my house or the ones of the hospital, I've learnt to appreciate the outside world. For this reason I don't



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 imprisoned in the correction of the diaphragmatic hernia. Some months later the ileostomy was removed and fundoplication for correction of gastroesophageal 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 Sciacca della Scala, AMIP Italy  
[www.assoamip.net](http://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 in 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/](http://www.phalatvia.lv/en/)







## NETHERLANDS



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.

Ferdinand Bolsius, PHA Nederland  
[www.pha-nl.nl](http://www.pha-nl.nl)

## 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



## POLAND

## 1ST CARDIOVASCULAR CONFERENCE OF POLISH SOCIETY OF CARDIOLOGY

The First National Conference of the Cardiovascular Pulmonary Section of the POLISH SOCIETY OF CARDIOLOGY took place in Warsaw on 2-3 December 2011. Hundreds of doctors and invited guests attended the lectures on topics related to pulmonary hypertension. The Polish PH Association had its own booth, where representatives of the association informed interested visitors about its activities.

## WORKSHOPS FOR JOURNALISTS

Scientific and training workshops for journalists (*Quo vadis medicina*) took place on 2 December 2011 in Konstancin. The theme was "Innovations in the treatment of rare and common diseases." One of the subjects was pulmonary hypertension. We had two PH speeches - Prof. Adam Torbicki "Pulmonary hypertension - a war on many fronts," and Piotr Manikowski, President of the

Polish PH Association, on "The current situation of patients with PAH in Poland - the perspective of the patient".

## BREATH TAKING CAMPAIGN FOLLOW-UP

The Polish Association of People with Pulmonary Hypertension and Their Friends took part in the Breathing campaign for Rare Disease Day 2012 with many different activities, including: a big happening in Warsaw, distribution of leaflets and posters in main places in big cities and also in small cities in Poland, advertising in buses, interviews on radio, TV, Internet and newspapers.

The Polish Association wanted to be very visible and reach out to as many people as possible, because in Poland, pulmonary hypertension is still little known disease. The campaign was a big success and many persons called the association after having read about the campaign in the press to get more information.



Piotr Manikowski



From left Basia Bieniasz, Prof. Marcin Kurzyński, Prof. Anna Fijałkowska, Prof. Adam Torbicki, Grzegorz Morze, Alicja Morze, Piotr Manikowski



## RARE DISEASE DAY IN POLAND

Members of the 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, Łazienki. 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 Arułowicz, Minister for Health, Beata Czaplicka, Member of Parliament, Jacek Graliński, Chairman of Committee on Rare Disease in Ministry of Health, Mirosław Zieliński, from the National Forum on Rare Disease Therapy and representatives from rare disease associations and Anna Dymna, a very famous actress who has 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.



From left: Piotr Manikowski, Anna Dymna and Agnieszka Bartosiewicz



Bartosz Arułowicz, Minister of Health

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 Kurzyński 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.

Agnieszka Bartosiewicz  
PHA Polska  
[www.tetniczenadciśnienieplucne.pl](http://www.tetniczenadciśnienieplucne.pl)



# RUSSIA

## PH WEBSITE

The most important news of last months 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](http://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/viewforum.php?f=2>  
<http://forum.ph-sos.com/viewforum.php?f=2>

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 site also 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 PH. 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.

Evgeniy Komarov, Natasha Foundation  
[www.ph-sos.com](http://www.ph-sos.com)





## SLOVAKIA

### ESTABLISHMENT OF A PH CENTER

We were very pleased on the 9th of November 2011 (Z5928 - 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 Philharmonic concert. Simultaneously the campaign ran in another Slovak town in Liptovský Mikuláš. Overall we distributed over 1,500 flyers and postcards, 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 magazines *Pokrok*, *Šarmantná žena*, *Zdravie*, *Plus jeden deň* and on the show presented by one of the most popular TVs in Slovakia called *TV Markíza*. As part of the follow up activity, on the 23rd March, Jana Guráňová 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 Phtiseology 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 EXIBITION

From the 10 to the 12th of May 2012 we attended the 14th annual exhibition called "Healthy lifestyle" in Trenčín. During these three days we informed visitors about PH symptoms, diagnosis and treatment and handed out dozens of flyers and brochures. Jana Guráňová 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 Nôtová, 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 Guráňová 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 Mikolášik, 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 with him. Dr. Milan Luknár attended the Brussels event on behalf of PHA Slovakia.

### CZECH PATIENT ASSOCIATION MEETING

Two of our members, Mária Jesenáková, and psychologist Dr. Pavla Nôtová, attended a weekend meeting organized by the Czech PH patient association June 16-17. During these two days Dr. Nôtová 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. Bryer 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  
Združenie Pacientov s Plúcnou Hypertenziou  
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## 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 Andalusian 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 Nauffal, 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 *Salvame* (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 *sin aliento*, which is the Spanish translation for "breath-taking"

### COOPERATION WITH SCIENTIFIC SOCIETIES

ANHP continued its cooperation with the Spanish Scientific Societies and events through its presence at the meeting in Lérida on March 6 hosted by SEPAR. This meeting worked on the multidisciplinary approach to Rare Respiratory Diseases (RRDs) within the framework of the SEPAR year for the RRDs. The association also included its publications and dissemination materials in the briefcases distributed at the First SEPAR and Latin American PH seminar that was held in Madrid on March 16.



### 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 AMHP members to assess 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 amongst health professionals. And, last but not least, I participated, on behalf of PHA Europe, to the DRUG INFORMATION ASSOCIATION (DIA) EUROMEETING in Copenhagen March 26-28, where the EUROPEAN PATIENTS ACADEMY (EUPATI) was presented for the first time.

Irene Delgado, Asociación Nacional de Hipertensión Pulmonar  
[www.hipertensionpulmonar.es](http://www.hipertensionpulmonar.es)



## 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 Calfetti, a social psychologist.

### SECOND REGIONAL MEETING

On May the 3rd we held our second regional meeting in a cozy 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! Mmmhhhh! 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



Terese Oesch and Bruno Bosshard

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 replying 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 Treder 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.

Terese Oesch, SPHV  
[www.lungenhochdruck.ch](http://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 agencies, 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 the survey would be relevant at both a medical and practical level. The Steering Committee was further consulted on the analysis of the survey results, to gain a thorough understanding of the personal perspectives of PAH patients as well as their carers and to help shape and endorse information resources to meet the current gaps that were identified by the survey.

Interviews were conducted initially with PAH patients and carers, recruited by some of our member organizations, to provide qualitative information on key survey themes. These survey themes were further explored in an online/postal questionnaire to provide quantitative data. A total of 326 PAH patients and 129 carers across five European countries (France, Germany, Italy, Spain and the UK), replied to questionnaires asking about four main areas: the physical and practical impact of PAH, the emotional impact, the social impact, and information needs and provision.

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. 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.phaeurope.org](http://www.phaeurope.org) and a summary of key findings will be also available on our Facebook page and PAH support forums from 14th September 2012.*



## 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 Ehlken 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 performed and maximum O<sub>2</sub> is measured, including O<sub>2</sub> 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 O<sub>2</sub> 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*



*Hall Skaara (second from right) at the rehab stay in Heidelberg*





## ORGAN DONATION AND TRANSPLANT A PRIORITY OF THE CYPRUS PRESIDENCY OF THE COUNCIL



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 midterm 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 on the importance of organ donation and transplantation and securing EU funds for the development of respective programmes in this field.

Source: Cyprus Presidency website  
<http://www.cy2012.eu/en/page/health>

## LIVE DONOR TRANSPLANT MEETING OF THE EU HEALTH MINISTERS IN NICOSIA

The Member States' Health Ministers held an informal meeting in Nicosia (Cyprus) on 10-11 July. Organ donation

was one of the key topics on the agenda. The ministers agreed to improve coordination of their actions to facilitate organ donations and transplants. They highlighted the importance of a well-organised transplant system and exchanged ideas on how to improve the respective programmes. At the initiative of the Cyprus Presidency, they focused in particular on ways of improving the situation of live donors. "It is worth considering the large number of patients who will benefit from compatible donor, the cost-effectiveness of transplant surgery and the large numbers of patients on waiting lists for a compatible donor, to understand the need for further coordinated actions to raise public awareness on this issue", commented Stavros Males, Cypriot Health Minister. Commissioner John Dalli (Health and Consumer policy), who also attended the meeting, urged Member States to include this issue in their health programmes. "Organ transplants can make the difference for thousands of Europeans. It is the only treatment currently available for advanced organ failure of the liver, lungs and heart", commented the commissioner. "Unfortunately, the number of transplants is limited due to donor shortages. Transplants from a live donor and cross border exchanges could thus help reduce shortages", he concluded. The discussions on live donor transplants explored in particular the donor's situation.

Source: Council press release 11 July 2012  
<http://www.cy2012.eu/en/policy-areas/employment-social-policy-health-consumer-affairs>

## EU HEALTH IN TIMES OF CRISIS THE HEALTH FOR GROWTH AND CONSUMER PROGRAMMES

Crisis has stricken 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 from 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 €446 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](http://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 COURSES and the EURORDIS 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 law and rules making.

PHA Europe is a forum where PH organizations can share experience and facilitate the exchange of information that will allow the transfer of know-how to build solid foundations and allow younger PH organizations to be able to host and obtain the funds for those types of events.

## FASTER ACCESS TO NEW DRUGS NEW EU LEGISLATION

The European Commission prepares policies and legal instruments that are submitted to the European Parliament for approval and affect the whole of the European Union. One of the problems in the access to new medicines is that the differences in the procedures at domestic level to agree on the price and reimbursement or not of a new drug often delays the access to newly approved treatments that can make a big difference for the patients.

The EU Commission has proposed a new directive reducing that delay to 120 days for innovative medicines and 30 days for generics. This means that the Member States still have the freedom to decide on the mechanisms used to determine price and reimbursement but the time limit is binding and the disrespect of the delay can bring penalties if the directive is approved.

This new directive should bring important savings to the national social security systems, improve the performance of health technology assessment agencies and promote the continuance of the industry's investment in research because it gives them access to the market and the ensuing profits in a shorter delay.

There is a mechanism conceived to protect the interest of producers and consumers: The TRANSPARENCY COMMITTEE. It is in charge of supervising the implementation of the Transparency Directive which lays down three major requirements for all Member States with respect to individual pricing and reimbursement decisions:

- Respect of the timeframe.
- Decisions must be communicated to the applicant (the company that markets the medicinal product) with reasons based on quantifiable and verifiable criteria to sustain them.
- Decisions can be challenged at judicial level.

More details can be found at:

[http://ec.europa.eu/enterprise/sectors/healthcare/competitiveness/pricing-reimbursement/transparency/index\\_en.htm](http://ec.europa.eu/enterprise/sectors/healthcare/competitiveness/pricing-reimbursement/transparency/index_en.htm)





## PATIENT ADVOCACY AT EU LEVEL DITA 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 DRUG 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 EURORDIS, the EUROPEAN ORGANIZATION FOR RARE 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 PROGRAMME 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 socio-economic costs and Health Related Quality of Life (HRQOL) of both patients and caregivers for rare diseases in different European countries ([www.burqol-rd.com](http://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 three and 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 prostanoids.

This study was the largest randomized, controlled study in

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.

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

2012

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 Castelledefels (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.



2013 AT A GLANCE



Nice, February 27-28 - 1 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 Evian 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 proceedings constitute relevant publications which are among the most cited in this scientific area.





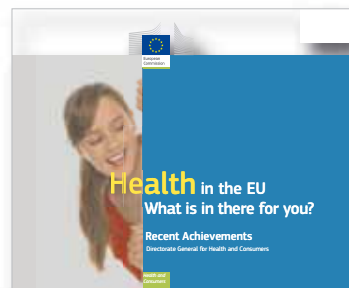
## REACHING OUT TO RARE DISEASE PATIENTS IN EUROPE

This recent document was produced by the Scientific Secretariat of the EUROPEAN UNION COMMITTEE OF EXPERTS ON RARE DISEASES (EUCERD). It aims to provide an overview of rare disease activities at European Union and Member State level in the field of rare diseases and orphan medicinal products up to the end of 2011. The information on each country is divided into a number of categories:

- Definition of a rare disease
- National plan/strategy for rare diseases and related actions
- Centres of expertise
- Pilot European Reference Networks
- Registries

The full text can be downloaded from the EUCERD website at: <http://www.eucerd.eu/>

## EU HEALTH - A REPORT OF RECENT ACHIEVEMENTS



In June 2012 the EUROPEAN COMMISSION Directorate General for Health and Consumers issued a publication called: "Health in the EU. What is in there for you?" which lists the EU's most recent achievements in the field of

Public Health. Topics covered include:

- Cross-border healthcare
- Cross-border health threats
- Safety and quality of medicines



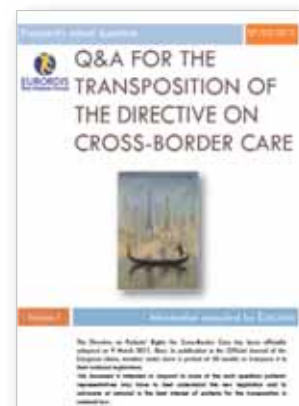
- Blood, tissues and cell donation
- 3rd Multi-Annual EU Health Programme (2014-2020)
- The risk factors of chronic diseases
- Global health

The full text can be downloaded from the EU Commission website at [http://ec.europa.eu/health/index\\_en.htm](http://ec.europa.eu/health/index_en.htm) in the section on "Highlights".

## EU CROSS-BORDER HEALTH DIRECTIVE

EURORDIS, the EUROPEAN ORGANIZATION FOR RARE DISEASES, has recently prepared a "Questions & Answers" document to help better understand the new EU legislation on cross-border healthcare. The DIRECTIVE ON PATIENTS' RIGHTS FOR CROSS-BORDER CARE was officially adopted in March 2011. It aims to help patients exercise their right to reimbursement for healthcare received in another EU country; provide assurance about safety and quality of cross-border healthcare and establish formal 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. The document presents the main aspects to look out for when seeking to apply patient's rights to cross-border healthcare including: the level of reimbursement, the need for upfront payment and the need for prior authorisation. The document answers frequently asked questions such as: "Can I seek healthcare abroad if the treatment is not available in my country?" "Where can we find information on care provided? Can we ask for travel and accommodation expenses to be also reimbursed?"

For full text click on the link "Read Q's and A's" at: <http://www.eurordis.org/en/content/eurordis-releases-qa-help-patients-advocate-their-right-cross-border-healthcare>



Pisana Ferrari

## 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 PHA EUROPE'S ANNUAL GENERAL ASSEMBLY taking place in Barcelona September 12-16. 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!

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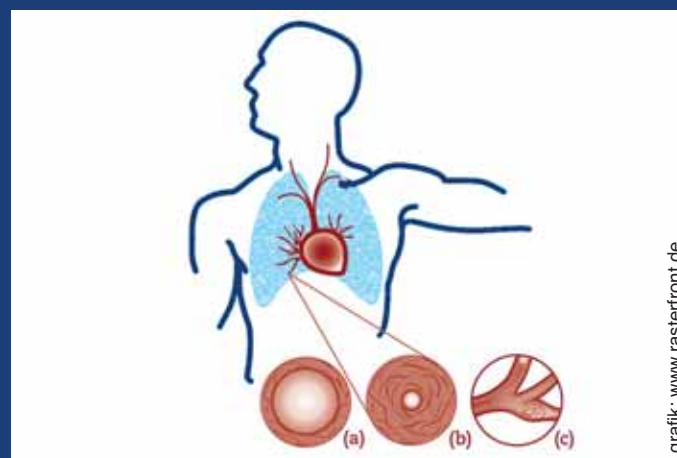


### AIMS OF PHA EUROPE

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 organisation. 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.



- a. Cross section of normal pulmonary artery*
- b. Reduced lumen of pulmonary vessels due to cell proliferation and remodelling in advanced Pulmonary Arterial Hypertension*
- c. Longitudinal section with pathological changes within the vessels (proliferation, deposition of blood clots, thickening) causing difficulties for the heart to pump blood through the lungs*

### 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.