



# PULMONARY HYPERTENSION MariposaNews

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

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Switzerland

Turkey



PHA EUROPE 



Dear friends,  
welcome to the Winter 2011 edition of Mariposa News. The timing of this issue coincides with the end of my first term as President of PHA Europe (2010-2012). It has been a great honour and pleasure for me to chair the association in the past two years and work in close collaboration with the Board and Members to further the PH cause in Europe. I would like to take this opportunity to thank the members once again for the appreciation and trust they have granted me by renewing this mandate for another two years (2012-2014).

I am proud to say that we have come a long way in the eight years since the foundation of PHA Europe in terms of creating a STRONG EUROPEAN PH COMMUNITY, of contributing to raising awareness on the disease and making the voice and concerns of the PH patients heard across Europe. Through our work we have earned RECOGNITION AS A KEY STAKEHOLDER for PH in Europe along with the nursing, medical and scientific community, the pharmaceutical industry and health policy decision-making bodies.

Over the years PHA Europe has also developed close and constructive relationships with non-European PH communities around the world who are, much as ourselves, actively working to improve the conditions of PH patients in their own countries or regions and are providing an invaluable contribution in this respect. Whilst recognizing the importance of international collaboration PHA Europe has always made it a point of stating that it is very keen to maintain its INDEPENDENCE and a strong EUROPEAN IDENTITY. Our aim is to ensure that every European country has a PH patient association and to provide the instruments and support for empowering them. We are not part of any other umbrella organization dealing specifically with PH and do have any plans to do so in the future. We also believe that the time is not ripe for the setting up of a broader, centralized PH world organization.

Looking back to the year which has just passed I would like to briefly summarize the main achievements. Our community has been growing steadily and it was wonderful to formally welcome this year as new members the PH associations of Bulgaria, Latvia, Netherlands, Norway, Sweden, Slovenia, Slovakia and Russia. Our GENERAL ANNUAL MEETING in Barcelona in September was a big success, with a rich agenda, prestigious international speakers and an exceptional turnout: 46 patient

leaders from 22 European countries. Sharing experiences and strengthening bonds between members across borders is very important. It is also nice to exchange visits and PHA Europe's President and Vice President were kindly invited to take part in meetings organized by the national PH associations of Ireland, Germany, Hungary and Slovakia.

In the course of 2011 PHA Europe was invited to provide the PH patient perspective at a number of prestigious scientific and industry events (Lisbon, Brussels, Vienna, Dresden, Nice, Bologna etc.) and also attended the European Society of Cardiology and European Society of Respiratory Medicine Annual Congresses.

Awareness-raising is one of our top priorities and in February PHA Europe launched its first PAN-EUROPEAN AWARENESS CAMPAIGN involving 12 countries. PHA Europe was subsequently invited by EURORDIS, the European Rare Disease Organization, to present the campaign at a workshop of the Council of European Federations as a case study of a successful event organized around Rare Disease Day 2011.

All of this was possible thanks to the invaluable support and collaboration of our members and sponsors whom I wish to thank once again. Best wishes to all the PH community for a very Happy New Year.

*Gerald Fischer, President PHA Europe*

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Elections for the Board of PHA Europe were held in September 2011 during the General Annual Assembly in Barcelona, September 8. PHA Europe wishes to thank the members of the outgoing Board for the great work carried out in the last two years and is delighted to introduce the new Board of Members, which will serve 2012-2014, composed of extremely qualified and dedicated individuals who are very active at national level in providing invaluable support to Pulmonary Hypertension patients and their families.



## **PRESIDENT** **Gerald Fischer**

University studies in world trade. Lives in Vienna where he works in tourism and, since 2001, manages the family liquor and schnaps business. Married and father of a 24 year old son and a 16 year old girl with PH. Founding Member and President of the Austrian PH association.



## **1ST VICE PRESIDENT** **Pisana Ferrari**

Political Science graduate. Has worked for EU institutions and consultancy firms and then in event management. Lives in Milan. Has a 22 year old daughter. Was diagnosed with PH in 1988, during pregnancy. Underwent double lung transplant surgery in 2002. Founding Member and President of AIPI, Italian PH patient association.



## **2ND VICE PRESIDENT** **Mélanie Gallant-Dewawrin**

Studied Law and legal management in Lille. 10 years teaching Law at the University and in a business school. Vice President of HTAP France 1998-2000, President 2000-2007, Managing Director since 2007. Married, one adopted daughter, Hannah, born 2006. Diagnosed with PH at the age of 6 in 1976. Double lung transplantation in 2009. Lives in Beaune, Bourgogne.



## **SECRETARY** **Luisa Sciacca della Scala**

University studies in Languages and Foreign Literature. Lives in Rome, is married and mother of three grown up children. President of AMIP Onlus, Italy's non-profit PH Patients' Association, since 2008. Founding member of the association in 2000. Has been involved in PH since 1998, when her close friend Maria Pia Proia was diagnosed with the illness and decided to found an association to facilitate other patients to overcome her same difficulties.



## **VICE SECRETARY** **Gergely Meszaros**

Graduated economist, tax advisor and lawyer. Most recently engaged by the Hungarian branch office of a global financial institution as Legal Counsel. Married, has a two year old daughter. Lives in Vác, near the capital of Hungary (Budapest). Gergely and his wife are volunteers for PHA Hungary. Gergely became involved in PH through friendship with Eszter Csabuda, founding member and President of the Hungarian association.



## **TREASURER** **Luc Matthysen**

Professional experience as Engineer in construction in Middle-East countries and Medical PVC with Solvay. Luc is married to Rosie Matthysen, a PH patient, and they have two married boys, Ben and Lionel. Luc and Rosie founded the French speaking PH association HTAP Belgique asbl (Wallonia, Brussels and German speaking part of Belgium) of which Luc is the current President. Young retiree (early retirement).



## **DEPUTY TREASURER** **Hall Skaara**

University degree in Computer Science from Ohio State University. Is also a certified project leader. Worked as a manager for more than 10 years for a large international computer company. Is now retired 50 % from his job in 2005 due to PH. Is married and has two boys, 24 and 19 years old. Founding member of the Norwegian PH Association.



## **AUDITOR I** **Hendrik Ramaker**

Had PH from birth but was diagnosed in 1998. Underwent double lung transplant surgery in 2001. Has a full time job, is married and has three children. Was President of the PH-VZW Belgium from 2001 till 2008. Is currently Vice President.



## **AUDITOR II** **Juan Fuertes**

Bachelor of Arts, worked for over 14 years in several countries with the International Committee of the Red Cross, specializing in humanitarian interventions related to armed conflict. Coordinator of the Spanish PHA (Asociación Nacional de Hipertensión Pulmonar) since January 2011.

FEBRUARY

**Vienna and Brussels, February 28**

## **“BREATHTAKING” CAMPAIGN FOR RARE DISEASE DAY**

PHA Europe’s Breathtaking campaign featured launch events in Brussels and Vienna and awareness raising activities in ten other European countries. The campaign received the patronage of EURORDIS.

PHAE EVENT



MARCH



**Lisbon, March 12-13**

## **6TH BAYER SYMPOSIUM ON PH**

PHA Europe’s Vice President was invited to speak about “The relevance of patient organizations to patients” at the 6th edition of the Bayer PH Symposium. 360 persons from 37 countries attended this meeting.

APRIL

**Dublin, April 9-10**

## **MEETING OF PHA IRELAND**

PHA Europe’s Vice President was invited to attend PHA Ireland’s meeting and talk about the activities of PHA Europe and its affiliates. Over one hundred persons attended.



MAY



**Vienna, May 6-7**

## **ACTELION EXPERT FORUM ON CONGENITAL HEART DISEASES AND PAH**

PHA Europe’s President was invited to speak about “The importance of a strong European PH association” at this meeting the main focus of which was PAH associated with congenital heart disease. Over one hundred persons attended.

JUNE

**Dresden, June 17-18**

## **SECOND BAYER INTERNATIONAL NURSE WORKSHOP**

PHA Europe’s Vice President was invited to speak about “Living with new lungs” at this important meeting bringing together around 130 nurses from 14 countries.



**Bologna, June 23-25**

## **UPDATE ON PULMONARY HYPERTENSION, ELI LILLY**

PHA Europe’s Vice President was invited to address the audience of this conference which saw the participation of over 200 medical professionals from around the world.

**Paris, June 30-July 1**

## **EURORDIS 4TH WORKSHOP OF THE COUNCIL OF EUROPEAN FEDERATIONS**

PHA Europe was invited by EURORDIS to present the “Breathtaking” awareness campaign to the Council of European Federations for rare diseases a case study of a successful campaign conducted around Rare Disease Day .

AUGUST



**Paris, August 27-31**

## **EUROPEAN SOCIETY OF CARDIOLOGY ANNUAL CONGRESS**

PHA Europe was present with a booth at the ESC Annual Congress and was represented by PHA Europe’s Vice President, Pisana Ferrari, Mélanie Gallant Dewavrin, Member of the PHA Europe Board and Managing Director of HTAP France, and Marzia Predieri from AIPI Italy.

SEPTEMBER

**Barcelona, September 8-11**

## **PHA EUROPE GENERAL ANNUAL MEETING**

PHAE EVENT

Forty-six PH patient leaders from 22 European countries attended the 2011 edition of PHA Europe’s General Annual Meeting. The meeting has a wide ranging agenda and prestigious speakers from the nursing and medical professions, from EURORDIS and the pharmaceutical industry.



## **LAUNCH OF THE WEB CAMPAIGN “TIME MATTERS”**

PHAE EVENT

This web campaign is designed to highlight the importance of “time” in PH (time to diagnosis and treatment, time for fulfilling one’s hopes and dreams...) through photos and stories posted by patients and family members around the world. The campaign will carry on through 2012.

SEPTEMBER

**Amsterdam, September 24-28****EUROPEAN SOCIETY FOR RESPIRATORY MEDICINE ANNUAL CONGRESS**

PHA Europe was present with a booth at the ERS Annual Congress and was represented by PHA Europe's Vice President Pisana Ferrari, Ferdinand Bolsius, President of the Dutch PH association and Marzia Predieri from AIPI Italy.

**Amsterdam, September 25****ERS TASK FORCE and EUROPEAN LUNG FOUNDATION MEETINGS**

PHA Europe is involved in the ERS Task Force "GP's meet rare lung disorders", which aims at increasing knowledge about rare lung disorders (including PAH) among primary care physicians. PHA Europe was also invited to participate in the first meeting of the Patient Advisory Committee of the European Lung Foundation.

**Amsterdam, September 26****EXPERT ROUNDTABLE ON PH - "WHITE PAPER"**

PHAE EVENT

PHA Europe's meeting brought together the key stakeholders involved in PH management - physicians, nurses, patients, regulatory authorities and pharmaceutical industry - with the aim of producing a common position paper on PH.

OCTOBER

**Sophia Antipolis, October 14-15****ESC EDUCATIONAL COURSE ON PH,**

PHA Europe's Vice President was invited to speak about "What the PH patients want" at the annual ESC educational course on PH. Over one hundred medical professionals from around the world attended the course.

**Bratislava, October 22-23****SLOVAKIAN PATIENT ASSOCIATION'S ANNUAL MEETING**

PHA Europe's Vice President was invited to attend the annual meeting of the Slovakian PH patient association and speak about the activities of PHA Europe.

**Frankfurt, October 28****GERMAN PH ASSOCIATION'S ANNUAL MEETING**

PHA Europe's President was invited to attend and address the audience at the 15-year anniversary celebrations of the foundation of the German PH patient association. Rino Aldrighetti, President of PHA USA was also present.

NOVEMBER

**Vienna, November 17-18****BRIDGES FOR CHILD, PEDIATRIC RARE DISEASES CONFERENCE**

PHA Europe's President was invited to speak about "How to obtain drugs for children with rare diseases" at a conference organized by "Bridges for CHILD" (Combat Health Inequalities in Life-threatening Diseases).

**Bologna, November 25-26****PULMONARY HYPERTENSION 2011 ACTELION/ELI LILLY**

PHA Europe's Vice President was invited to address the audience of this conference which saw the participation of over 250 medical professionals from 22 countries around the world.

**Miami, November 28****PH LATIN DAY**

PHA Europe's President was invited to attend the PH Latin Day held organized by the PH Latin Society in Miami.



DECEMBER

**Vienna, December 11****PHA EUROPE LEADERS MEETING**

PHAE EVENT

PH Patient leaders from 14 European countries met in Vienna to discuss future common activities including the "Breathtaking" campaign for Rare Disease Day 2012, next steps to take with respect to the position paper on PH, the new website and the agenda of the 2012 General Annual Meeting.



It looks like 2012 is going to be another pretty busy year for PHA Europe with lots of activities and new projects lined up:

### Awareness raising campaigns:

- Preparations are well under way for PHA Europe's **"BREATH-TAKING"** campaign for Rare Disease 2012. The concept and visuals were sent to the members for comments and in early January ten countries submitted projects about how to implement the campaign in their own country. The launch event will be in Brussels on February 29 and will include a press conference with Members of the PHA Europe Board and key medical opinion leaders such as Prof. Jean-Luc Vachiéry and Prof. Marion Delcroix who have already confirmed their support and attendance.
- **"TIME MATTERS"**, PHA Europe's web campaign was launched in September 2011 with the objective of highlighting the importance of "time" when you have PH (time to diagnosis, time to treatment). This campaign, which is currently under way and has over a hundred entries, will continue collecting patient photos and posts from around the world throughout 2012. The website, which was originally set up in English, is now online also in 14 other languages. We warmly invite all the members of the PH community to take part and help us make this campaign a great success!



### New websites

- The relaunch of the **PHA Europe website**, with a new graphic layout and more advanced functions is currently being finalized and is due to go live in at the beginning of January 2012. In addition to the home page which will be in English, the website will contain sub-domains with a common text in different languages and direct links to the national association websites, to the Time matters campaign and to PH discuss (see below). A geo-locator will facilitate the search for expert medical centres across Europe.
- **PH Discuss** is a new website that will enable our members to download & upload important documents and files in an easy manner. The platform also acts as a simple Forum to have organized discussions throughout all PHA Europe countries.

### Advocating for patient rights

- Further to the very successful Expert Roundtable meeting held in Amsterdam on September 26 2011 with key medical opinion leaders and patient associations from Europe, USA and Latin America, a first draft of a **Position Paper on PH** has now been sent out the Roundtable Members and PHA Europe affiliates for comments. The issue-based paper is addressed to policy makers at national and EU level.
- The reviewed and final paper will be presented in Brussels during the Rare Disease Day celebrations on 29 February 2012.

### PHA Europe presence at international events

- PHA Europe will be present as in past years at both the European Society of Cardiology Annual Congress (Munich, Germany, August 25-29) and European Society for Respiratory Medicine (Vienna, September 1-5).
- PHA Europe has already received invitations to speak on behalf of the European PH patients at several important scientific events such as the 2nd Annual Orphan Drug Congress 2012 (June 7-8, Barcelona) and the ESC educational course on PH (Fall, Sophia Antipolis).
- PHA Europe has received an invitation to give its patronage and attend the 5th World PH Symposium (February 27-28 - March 1 2013, Nice).

### Expanding PHA Europe membership

- Contacts will be made with patients, family members or health care providers in countries where there are currently no PH patient associations in order to promote and support the setting up of new groups.
- We look forward to welcoming as a full member the newly founded Finnish association at our next GAM.
- Patient associations in Ireland and Romania are currently undergoing internal re-organization and we hope in the near future that they will join PHA Europe.

### Empowering the national patient associations - the "White Spots" program

- A PHA Europe seed grant program to help "new" PH associations in starting up and to provide support to "young" associations is currently being defined. Details will be notified to the members in January.

### International online rare disease communities

- PHA Europe has joined the EURORDIS/NORD RARE-CONNECT project and will be happy to contribute to the creation of an international online PH community.

### General Annual Meeting 2012

- The date and venue have now been finalized: Castelldefels, Barcelona, September 13-16. A draft agenda will be sent out to the members in January.

Pisana Ferrari

## AUSTRIA

**First PH Children meeting  
Vienna, October 12**

PHA Austria held its first PH Children meeting on the 12th of October in the "Gartenhotel Altmannsdorf" in Vienna. It was a big pleasure to welcome Prof. Dr. Ina Michel-Behnke, PH pediatric specialist at the AKH Vienna General University Hospital.

**German Patient Association Meeting  
Frankfurt, October 28-30**

from left: Gerald Fischer, Bruno Kopp, President of ph e.v. and Rino Aldrighetti, President of PHA

Our President, Gerald Fischer, was kindly invited to attend and speak at the meeting of the German PH association. Gerald's daughter Maleen, a young patient, was filmed for the video which won this year's Journalistic Award at the meeting (see page 11).

**"Bridges for CHILD" Pediatric Conference  
Vienna, November 17-18**

Gerald Fischer with Prof. Ulrike Salzer-Muhar, AKH Vienna



Pediatric health-care differs in different regions of Europe. The life-expectancy of children with life-threatening diseases is actually affected by their birthplace. The goal of CHILD is to Combat Health Inequalities in Life-threatening Diseases by creating an EU-wide platform that fosters exchange, dialogue and partnering essential for the intended subsequent projects. The two-day conference had about one hundred participants, mostly stakeholders, involved in the care of very sick children with major organ disease, roughly evenly divided

between medical health care professionals, representatives from health care authorities, insurance companies, industry and NGOs, policy makers and delegates from parents organisations. The focus was on conditions such as congenital heart disease, kidney, liver and lung disease but included conditions such as leukaemia and other severe diseases requiring high-end care up to and including transplantation. Our President, Gerald Fischer, was invited to attend and speak on the topic "How to obtain drugs for children with rare diseases".

For more info: <http://www.bridgesforchild.eu/>

**PH Latin Day  
Miami, November 28**

Our President, Gerald Fischer, was very kindly invited to attend the first PH Latin Day which was organized by the PH Latin Society in Miami (see also page 26).



from left: Migdalia Denis, President of the PH Latin Society, her husband, PHAE President Gerald Fischer, Juan Fuertes and Irene Delgado (ANHP, Spain)

**Patient meetings**

Local PH patient meetings at the Achensee, near Innsbruck (above) and in Vienna (right)



Two regional patient meetings were organized in Vienna (October 25) and at the Achensee, near Innsbruck (October 3).

**2011 Self-help Group Day  
Vienna, October 1**

PHA Austria attended the national "Tag der Selbsthilfe 2011" (self-help day 2011) in the Vienna City Hall and had a booth.

Eva Grassmugg, Selbsthilfegruppe Lungenhochdruck

### BELGIUM - HTAP BELGIQUE

#### HTAP France Patient Day

HTAP Belgique received a very kind invitation from HTAP France to participate in the Patients Day of North-Pas-de-Calais on October 1. This was already the second time we attended this wonderful, well-organized, interesting, friendly



event. We had the opportunity to listen to Prof. Eric Hachulla and cheer Mélanie and Mr Jacques Fertin.

#### LUSS Workshop

On October 7th, HTAP Belgique participated in a workshop organized by LUSS (Belgium Patients Platform). The National Institute for Health and Disability is working on a model of statute

for "Chronic illness". All rare diseases and orphan diseases will be covered. We will contribute actively to realize this project.

#### Fondation Roi Baudouin



On October 28, we participated in a discussion session organized by the "Fondation Roi Baudouin" on "Better Participation of Patients". This discussion centered on new possibilities for recognition and financing of patient associations and health care users groups.



left: Luc Matthysen at the HTAP France meeting with Mélanie Gallant Dewavrin  
above: Meeting of the Fondation Roi Baudouin

#### LUSS Conference

On November 24 LUSS, the Belgian Patients Platform, organized a conference dealing with "The place of the patients in an e-Health platform". The presentation was given by Maître Herweg from the Center of Research-Information, Law and Society.

*Luc Matthysen, HTAP Belgique*

### BELGIUM - VZW

#### 10th anniversary celebrations

The Belgian association Patiëntenvereniging Pulmonale Hypertensie vzw celebrated this very important anniversary on the 26th of November.

Lots of patients and family members joined us for an academic meeting which was held at the hospital of Leuven.

Many patients spoke about their life with pulmonary hypertension and their relation with our organisation. National and internal speakers spoke about the important role of

patient organisations.

It was very touching for all to remember together friends lost to PH over the past years. Prof. Marion



Delcroix updated us on new developments in pulmonary hypertension.

There were also speeches about the familial and hereditary forms of PH. Prof. Jean-Jacques Cassiman from the

*above: Ferdinand Bolsius, President of PHA Netherlands, was one of the guests at the meeting (centre, with red tie)*



Belgian organization for rare diseases [Radiorg.be](http://Radiorg.be), explained the Belgian national plan for rare diseases. We can say we had a very interesting and inspiring day and we are ready to go for the next ten years!

*Roland Loose,  
Patiëntenvereniging Pulmonale  
Hypertensie vzw België*



[www.ph-vzw.be](http://www.ph-vzw.be)

## CZECH REPUBLIC

### Reconditioning Stay, Podebrady,

In the summer of 2011 the Czech PH association held a one week rehabilitation-reconditioning stay (July 31-August 7)



*Photos of the reconditioning stay in Podebrady, August 2011.*

for patients with PH in Podebrady, a beautiful spa resort about 50 km west of Prague. We come back every year to Podebrady as it has turned out to be an ideal venue. We hold this event thanks to the support of the Government of the Czech Republic, in the form of a grant from the Ministry of Health. The program included light workout under the supervision of physiotherapists, short walks, medical lectures and discussion evenings.

### PHA Europe GAM

Our association took part in PHA Europe's General Annual Meeting in Barcelona and was represented by

*Dr. Regina Votavová at the PHA  
Europe GAM in Barcelona with  
Gerry Fischer and Maaike Atres  
(Netherlands)*



Dr. Regina Votavová. SPPH will try as much as possible in the future to work with PHA Europe and attend events at the international level. Currently we are looking to cover a position for someone who would be able to communicate at a high level in English. We also would like to actively participate in the "Time Matters" awareness campaign.

### Meeting of the Slovak PH Patient association

From October 21 to 23 the Slovak PH patient association organized a reconditioning-educational stay in Trenčianske Teplice. The program was very varied and interesting: medical lectures, lectures on coping with mental stress, on nutrition, physiotherapy demonstrations, cultural events. We are grateful to the Slovak association for the invitation to attend. At the end of Saturday's program I was very happy to make a presentation on the activities of the Czech Association.



*Katerina Novaková at the  
Slovakian patient meeting*

### Bulletin 2011

Last year we published the first issue of our association's journal, Bulletin. The 2011 edition is almost ready and it should be issued by the end of the year. We hope that our members will find it useful and interesting.

*Katerina Novaková, Sdružení Pacientů s Plicní Hypertenzí*

[www.plicni-hypertenze.cz](http://www.plicni-hypertenze.cz)

## FRANCE

The second half of this year has been very active, with many projects being completed. First of all, we held two patients meetings, one in Aurillac (Cantal, Auvergne, September 17), the other in Lille (North of France, October 1). We were very happy to have Luc Matthysen, President of



HTAP Belgique, as our guest in Lille. At each of these meetings we had about 50-70 patients and family members. Secondly, we have some projects in partnership with

pharmaceutical firms, and we would like to tell you about the newest ones. Eli Lilly has been quite involved in supporting a brochure about psychological care for PH patients written by Dr Arnaud Bourdin (pneumologist) and Dr Laurence Halimi (psychologist), both from the competence center in Montpellier.

We also have produced some videos to post on our website, and these were published in a press release including a wish list for PH patient care on the 16th of December. Go and have a look at them [http://htapfrance.com/je-m-informe/videos\\_htap.asp](http://htapfrance.com/je-m-informe/videos_htap.asp) ! This is an awareness project, but patient information at the same time. The videos include interviews with Sylvain, our President, who speaks of the role of the association, with myself, to summarize our main

activities, with a PH patient describing everyday life with PH, and a caregiver talking about his wife (who has PH). On top of this we are very proud to broadcast an interview with Prof. Gérald Simonneau about PH and one with Prof. Marc Humbert about PH

research. We hope this will be a positive communication experience and that we will get good feedback from the press and new patients. Another big project was the patient

study HTAP France has been working on since 2009, in collaboration with Pfizer. The study, which was related to patient care experience and patient needs, is now completed. We received over 350 answers to the questionnaires used in Phase 2. The results have been analyzed and will soon be published. I can already announce some of the preliminary results. The objectives of this study were to better understand the reality of the PH patient journey, with regard to medical care, medical information, social and psychological aspects: It also aimed at identifying the



*A group of PH patients, including Mélanie and Ludivine, visiting the brand new hospital in Dijon, where the competence centre is located and meeting the physicians*

needs of the patients and evaluating the adequacy and effectiveness of our activity as a patient association. Patients responding to the study declared themselves mostly as being in functional class III (48%) and II (32%). They were diagnosed on average 1.5 years after the first symptoms appeared. Two thirds are treated in a competence center and one third in a reference center. Only very few go to non-PH centers. Patients have a very positive perception of the care they get in PH centers, which they rate 8.7 on a 10 point scale. Regarding treatments, half of the patients are on two specific PH medications. They are relatively satisfied with the distribution of the drugs, but would prefer to be able to get them from a local pharmacy or, of course, directly at home (hospital pharmacies can be a long distance away). One in 5 patients says he lacks psychological support. It appears also that patients would like more information about transplantation: one out of four declare they lack this information. Only a minority has benefitted from a therapeutic education program.

Role of HTAP France: 6 out of 10 patients know HTAP France, and it is particularly true in the idiopathic PAH patients' population. It was very interesting to read that it is mostly the physicians and members of the PH team that give info about our association. This means that it is very productive for us to work with the PH specialists. The level of satisfaction about the association is very high (8.1





out of 10). Expectations of patients with regard to HTAPFrance include the promotion of research (69%), giving info about PH (59%), providing everyday life tips and counselling (46%), contacts with other patients, helplines (45%) and representation of patients and their interests (45%).

We will be very pleased to share the results of the study in greater detail at the next GAM and to explain how to proceed if you should envisage implementing such a study

in your country. The results are going to be really helpful not only for us as an association but also for the practitioners in order to better adapt their practice to the patients' needs. Looking to next year, as HTAP France is now 15 years old, we are planning some special celebrations around our 2012 General Assembly, which will take place on the 10th of March.

*Mélanie Gallant Dewavrin, HTAP France*

[www.htapfrance.com](http://www.htapfrance.com)

## GERMANY

### Double anniversary celebrations for pulmonale hypertonie e.v. Germany

Around 220 participants took part in the German PH Patients annual meeting held in Frankfurt October 28-30. This year there were two very special reasons to celebrate: the 15 year anniversary of the patient self help group and 10 year anniversary for the René Baumgart Foundation! Professor Ekkehard Gruenig, head of the Pulmonary Hypertension Center at the University Hospital of Heidelberg, publicly praised the commitment and dedication of both these organizations. The patient association was founded in 1996. The number of members has been rising steadily since then and now stands at around 1,300. The René Baumgart Foundation was created in 2001 to encourage scientific work on the origin and treatment of pulmonary hypertension.

One of the highlights of this year's meeting was the ceremony awarding the prize of €3,000 for outstanding journalistic contributions on the theme of "pulmonary hypertension" (print medium, radio, internet or TV). This year's prize was given to Dr. Edith Bachkönig for her TV documentary, "Forever sick - people living with rare diseases". This film combines testimonies of the daily lives of sufferers and their impaired lives with in-depth information from experts.



top: Bruno Kopp and Prof. Marius Hoper presenting the Journalist award to Dr. Edith Bachkönig  
middle: Gerald Fischer, President of PHA Europe with Bruno Kopp, President of ph e.v. and Rino Aldrighetti, President of PHA

Rigorous research, targeted diagnosis and therapy as well as strong advocacy in order to increase the chances in the fight against pulmonary hyper-

tension were identified as the key factors for the management of PH in future years. National and international networks are also very important. ph e.v. was set up under the auspices of the former Baden-Wuerttemberg Minister for Social Affairs Erwin Vetter and is part of national and interna-





## GERMANY



has come from Germany. He also stated that ph e.v. has played an important role in this respect and is to be thanked. The association has, among other things, contributed significantly to the creation of evidence in medicine, with regard to the positive effects of moderate exercise training.



tional networks such as the umbrella organizations BAG, LAG, ACHSE, PHA Europe and EURORDIS.

Gerald Fischer, President of PHA Europe, was invited to the meeting and presented new PHA Europe projects such as the "White Paper", a common policy statement to be presented to policy makers, and the awareness web campaign "Time Matters in PH".

The President and CEO of PHA USA, Rino Adrighetti, emphasized the value of mutual support and networking. PHA USA was founded by four women around a kitchen table in 1996 and today there are more than 235 PH support groups located throughout the United States.

As speaker of the ph e.v. Scientific Advisory Board, Prof. Werner Seeger, Medical Director of the University Hospital Giessen and Marburg, recalled that in the 1990s, no specific treatments for PH had been developed. He summarized the progresses of recent times and ventured a look into the future of PH therapy. It will be important not only to optimize the use of already approved drugs (eg. improve inhalation techniques) but also to explore new pathways and in this respect ongoing research is promising.

Professor Horst Olschewski, head of the Department of Pulmonary Diseases, Medical University of Graz said that in recent years all significant findings of basic research as well as almost all the impetus for the development of new drugs

### PH Research award

René Baumgart was my nephew. He was diagnosed at age 19 and died of PH at 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 in 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 2012 award must be submitted before the 31st of December 2011. The winner will be announced in the course of a ceremony at the 53rd Congress of the German Society for Pulmonology and Respiratory Medicine in 2012 in Nuernberg.

*Bruno Kopp, pulmonale hypertonie e.v.  
Photos © KENNEPOHL*



## HUNGARY

**National PH patient-physician meeting**

Tüdőér Egylet, the Hungarian PH Organization, held a national patient-physician meeting on October 15. The theme of the event was "Collaboration in the hope of a cure!" All Hungarian PH patients were invited, irrespective

of whether they were a member of the association or not, with the aim of getting to know each other, provide hope and share experiences and advice on how to cope with the burden of the disease in every day life. We were very happy that over one hundred persons accepted our invitation and were present.



Kristof Karlocai MD, Vice President, speaking at the meeting. Next to him on the podium is Eszter Csabuda, President

In the introduction to the event we informed the attendees that we (the association) intend to fight to have access in Hungary to all treatments and drugs used and authorized across Europe. We consider it as an important task of the association to help in the social and medical treatment of the patients and inform both the general public and physicians about this rare and very serious disease.

We paid special attention to the presentation of Dr. Judit Bidló, Head of the price subsidization department of the National Health-Insurance Fund. She informed us that the cost of one month's worth of PH drugs is millions of forints (at least € 3,500). We were assured that the support will be maintained, because of the life-threatening nature of the disease. The aim of the National Health Insurance Fund is to attain optimal disease management both in terms of physical wellbeing and funding. According to Dr. Bidló the fact that the handful of PH patients in Hungary are receiving the drugs is undeniably the merit of the physicians.

Dr. Kristof Karlocai from the Department of Pulmonology of the Semmelweis University spoke of current PH treatments, his colleague, Tamas Forster provided the audience with pieces of advice on lifestyle issues, Hajnalka Bálint and András Temesvári - cardiologists - made a presentation on PH resulting from congenital disease.

The atmosphere of the three-hour meeting was friendly and relaxed. Everyone found the presentations interesting and useful. As a result of the meeting the membership of Tüdőér Egylet, Hungarian PH association, almost doubled (!) and we have since received many letters thanking us for the meeting. The Board of the association has decided that if possible it would like to make this an annual event and to hold the national Hungarian PH patient-physician day around every mid October.

*Eszter Csabuda, Tüdőér Egylet*





### ISRAEL

#### Special Patient Session Organized at International PH Conference

In mid-November, Dr. Yochai Adir, Director of the Pulmonary Institute at the Carmel Medical Center in Haifa convened for the second time an international conference on pulmonary hypertension in which leading specialists throughout the world participated. The conference took place at the Daniel Hotel in Herzliya with hundred of physicians from throughout Israel and elsewhere in attendance. At the conference, a special session was designated for the participation of patients where a forum of three international specialists along with Dr. Adir considered numerous queries posed by patients concerning issues they encounter in their daily lives. The specialists responded attentively to the many questions.

The Israel PH Association will summarize the questions

and answers and forward them to patients who, due to their medical condition, were unable to attend the conference.

#### Medicine-Specific Patient Meetings

The Israel PH Association has organized for the first time meetings of patients treated with the same medicine. These gatherings aim at providing drug-specific information, for example, method of use, importance of regularly taking the medicine, when and how to suspend use of the medicine, how to cope with side effects, and related issues. To the best of our knowledge, this is the first time that such an initiative has been undertaken anywhere and our hope is that the model will be applicable in other countries.

*Aryeh Copperman, PH Israel*

[www.phisrael.org.il](http://www.phisrael.org.il)

### ITALY - AIPI

#### AIPI at the Slovakian annual patient meeting

It was a great honour for me to be invited to attend and speak about PHA Europe and AIPI activities at the annual



meeting of the Slovakian patient association. This meeting was held in a beautiful spa resort not far from Bratislava on October 21-23. The program was very interesting

with lectures about medical aspects and life style issues such as physical exercise, nutrition and mental health. I had brought small gifts for the participants and it was fun to see how some of the guests showed their appreciation by putting our "branded" AIPI cotton bags around their neck!

#### PH patient stories

The AIPI quarterly newsletter has been publishing patient stories ever since it was first started in 2003. A new publication containing excerpts from the over 70 stories collected so far is now being finalized and is

due to go to print shortly. The aim of this booklet is to share experiences and insights about coping with everyday life with PH.

#### Social activities

About fifteen patients and family members took part in an outing organized by our association around the "Eurochocolate" Fair held on October 14 and 15 in Perugia. AIPI Vice President Leonardo Radicchi (see below at right), who is from Perugia himself was the group leader. The two-day program included a visit of the city, lots of chocolate-tasting at the Fair, and a nice get-together over dinner in a typical local restaurant.



*Pisana Ferrari, AIPI Italy*



left: Giusy Lobaido, Rita Pellegrini, Leonardo Radicchi, Mary Giordano and Elisabetta Cordi - middle: Elisabetta Cordi and Roberta Masciolini - right: Leonardo Radicchi

[www.aiipiitalia.it](http://www.aiipiitalia.it)



## ITALY - AMIP

**Viareggio Health Festival**

A new opportunity to spread awareness on PH was provided to us this year: the presentation to the media earlier on in the year of our documentary "Living with PH", gave us a good amount of visibility, and led to an invitation to attend the Viareggio Health Festival (September 28th-October the 2nd). This event is the largest in Italy in the field of health and it is intended for the general public. It was visited by over 100.000 citizens. AMIP had a free booth where we were able to show our documentary and distribute material about PH and the Association. The AMIP newsletter, "Insieme", and "Mariposa News", the newsletter of PHA Europe, were a great success and we had so many requests that we had to keep them only for the people that seemed more interested.

We talked to doctors that had never met a PH patient and others from the local hospital who had seen a few. We also gave out the ESC Guidelines on PH and suggested that they study them carefully and follow the suggestions they would find in them. I must really thank our member Luigi Lazzarotti (see photo) who spent many hours to help me and answered the many questions the visitors were asking. To be able to see PH patients both in person and in the documentary really made a difference and proved that there is really reason to HOPE for the patients of an illness that only a few years ago was considered fatal.



above: Luigi Lazzarotti  
at the AMIP booth at  
the Viareggio Health Festival



left: Luisa Sciacca della Scala,  
AMIP President, with  
Luigi Lazzarotti  
at the Viareggio Festival



The San Matteo (Pavia)  
PH medical team  
with the new ECHO

Vittorio Vivenzio  
receives a cheque for  
\$21.000 from Mr. Jim  
O'Gara, UPS country  
manager for Italy.  
far right: Dr. Stefano  
Ghio from the San  
Matteo Hospital



### **Echocardiograph equipment for the Pavia Hospital**

A UPS (United Parcel Service, Inc - NYSE) \$21.000 donation covered the entire 2nd installment of the echo equipment AMIP bought for the San Matteo Hospital PH Centre in Pavia! It is incredible how from a little seed something very big can come out. We trust Fate and aim high, but this time what happened was really beyond our remotest expectations. When AMIP organizes the soccer matches between Actors or Showmen teams and local volunteers, as we have done in past years, we sell the tickets for the match. A "campaign" among friends and friends of friends (... and friends of friends of friends) thus begins. In return for a very small sum of money we offer a nice afternoon to spend and the possibility to meet some famous people, so the job is not too difficult. Some time ago, through these matches, we had the chance to meet somebody that works for UPS; then, on the next occasion, this person spread the word with his colleagues and a big group of UPSers came to the *Derby del Cuore* that took place in Rome last June. News of their presence was reported in the UPS newsletter and it reached the top managers based in Milan. They suggested to us that we apply for a donation to the US Direction of their company if we felt we had a good and worthwhile project to present. We did! To gather all the necessary documents to satisfy the US Direction was long but not difficult (Statutes, Balance sheet, Non-profit registration, and many many others). All our documents are in perfect order so this was no problem. To make a long story short, on December the 13th Vittorio Vivenzio, Member of our Board, was in Milan to meet a top manager coming from US and receive a cheque for one of the biggest donations we have ever received!

Luisa Sciacca della Scala, AMIP Italy

### LATVIA

#### PHA Europe GAM

The founders of PHA Latvia, myself and Lolita Indriksone, had the pleasure of taking part in the PHA Europe General Annual Meeting in Barcelona, 8-11 September 2011.



The meeting was very useful and inspiring. We shared information and experiences between national PH associations and made contacts with patients from many European countries. We are very glad that PHA Latvia was formally admitted as member in the umbrella organization PHA Europe.

The meeting was very useful and inspiring. We shared infor-

#### Workshop on oxygen therapy

PHA Latvia organized a workshop and presentation about oxygen therapy in collaboration with the Head of "Medicusdata" Uldis Eglitis on October 7 in Riga. Four members took part the workshop and presentation.

#### Meeting with the Ministry of Health

On December the 8th I had the opportunity of meeting Antra Valdmene, Head of the Department of Health Quality system (Ministry of Health). During the meeting we talked about the need to ensure legal rights of PH patients in our country, about the work group for the Register of rare diseases, and social, rehabilitation and drug reimbursement issues.

Ieva Plume, PHA Latvia



above left: Lolita Indriksone, Pisana Ferrari and Ieva Plume at the PHA Europe General Annual Meeting in Barcelona  
left: Ieva Plume with Agnieszka Bartosiewicz (Poland)

### NETHERLANDS

#### Special day for patients with CTEPH

In the Netherlands we have two medical centres who are specialized in the treatment of patients with CTEPH, the chronic thrombo-embolic form of PH.



left: Dr. R.J. Snijder, lung specialist at the Antonius Hospital in Nieuwegein  
below: participants at the CTEPH meeting at the Antonius Hospital



One of the two hospitals, the Antonius Hospital in Nieuwegein (near Utrecht) invited our patients to come over for a presentation about this form of PH on September 14. It was a very successful initiative and many patients were present. There were presentations about what CTEPH is, how it develops, what to do about it, when/how can it be operated and the consequences of surgery. There was also a presentation from a patient who has been successfully operated. She is practically 100% cured but still has some minor emotional problems. Our association was very happy about this meeting because when you do a presentation for a certain group of PH patients you can provide them with more detailed information about their specific condition. We will try to organise a meeting like this at least once a year.

#### Re-launch of our website

At our latest Board meeting we decided to restyle our website in a more modern way. The layout of the site is not current any more. In the beginning of next year we hope to introduce our new website.

Ferdinand Bolsius, PHA Nederland

## NORWAY

### Norway's first patient meeting!

In September 2011 the Norwegian PH organization ran their first patient meeting. Fourteen PH patients and eight of their caregivers attended the conference.

"It was great to meet some of the other PH patients", expressed one of the participants. "I have had PH for many years, and I have never met another person with the same disease. It is wonderful to meet and talk to other people that understand me and my situation!" The Norwegian PH Association had made a comprehensive two-day

program with several prominent speakers. However, the greatest benefit of the conference was probably the opportunity to meet with other PH patients and caregivers. Valuable friendships were formed and a lot of important exchange of information took place.

During the formal part of the meeting, the leading Norwegian PH specialist gave a presentation about the disease and the treatment available in Norway. The patients in Norway are fortunate in that they do not have any restrictions as to which medications are available. The medications are, furthermore, free of charge due to the centralized public health care system. One method of medication that received particularly attention was the implanted pump. One of the participating patients has had this pump installed and demonstrated it in front of the group. The implanted pump is working very well for this patient, and it has enabled her to continue her work as a full time nurse at a clinic for people with lung and heart diseases!

In addition to the PH specialist, a leader of a rehabilitation clinic presented their rehabilitation program. Although patients with other heart and lung diseases primarily attend



left: Dr. Arne K. Andreassen  
middle: some of the participants -  
right: Dr. Khang Ngoc Nguyen  
middle row of photos from left:  
Nils Erling Myhr - right: PH patient  
Sølvi Molnes - lower photos from  
left: Myhr on the left and Molnes on  
the right - right: Hall Skaara



the program, they also had experience in helping PH patients.

Furthermore, in spring 2012 they will run a rehabilitation program for a group consisting only of PH patients!

Other presentations included:

- Psychological challenges of living with a chronic illness.
- A patient's private survival guide.
- PH medication of today and the future.
- Actelion's new web page and brochure for newly diagnosed patients and their caregivers.

Another goal for this first patient meeting was to create awareness for PH since it is estimated that there are the same number of undiagnosed cases as there are diagnosed cases in Norway. A local newspaper (36,000 subscribers) featured one of the patients in an article about PH. In addition, the leading PH specialist and myself were interviewed in a radio program that was aired nationwide.

The patient meeting was a great success and will be repeated next year. The meeting was made possible through a Tom Lantos grant plus a generous contribution from Actelion.

Hall Skaara, PHA Norway

left: patients and family  
members during the lunch  
middle: group photo  
with Hall Skaara, first  
from left with blue shirt  
right: view of the spa resort





### POLAND

#### General Assembly, Otwock, November 11

The Annual General Assembly of the Polish PH patient association was organized in Otwock, near Warsaw. The meeting was held at the European Health Centre. One of the items on our agenda was the renewal of the Board. Piotr Manikowski was elected as the new President of the Polish PH Association, Alicja Morze was elected Vice President and Treasurer, Kazimierz Kondek, Jacek Majewski and myself, are members of the Board.

The main speaker at our meeting was Prof. Adam Torbicki, Professor of Medicine and Head of the Department of Chest Medicine at the Institute of Tuberculosis and Lung Diseases, Warsaw. Prof. Torbicki's very interesting talk was about: "Pulmonary Hypertension: new challenges - new possibilities". Prof. Torbicki spoke about PH generally and also about the various surgery options (atrial septostomy and PCI surgery). He underlined the fact that Ministry of Health



far left: Prof. Adam Torbicki speaking at the Annual General Assembly of PHA Polska

should extend the PH Therapy Program in order to provide drugs and treatment for patients in functional class II. Access to PH drugs remains a very serious problem in our country. In future, the department of Cardiology of the European Health Centre will be treating PH. The PH centre from Warsaw will therefore move to Otwock.

#### Polish PH Therapy Program

Unfortunately the Ministry of Health has not modified its PH Therapy Program, which gives access to treatment only to patients in functional class III and IV, and currently only four PH drugs are covered by this program and are available: sildenafil, bosentan, iloprost and treprostinil.

#### Polish PH Association on TV

The Polish PH Association recently took part in well-known morning TV program called "Coffee or tea?" Two members of the association (Piotr Manikowski and Alicja Morze) as well as Prof. Adam Torbicki were invited to speak about PH. The presenters asked about treatment, diagnosis and life with PH. Piotr and Alicja spoke about their PH stories. Prof. Torbicki underlined the difficulties that PH patients still have to face and the fact that much remains to be done in this field.



The presenters asked about treatment, diagnosis and life with PH. Piotr and Alicja spoke about their PH stories. Prof. Torbicki underlined the difficulties that PH patients still have to face and the fact that much remains to be done in this field.

Agnieszka Bartosiewicz, PHA Polska

[www.tetniczenadciśnienieplucne.pl](http://www.tetniczenadciśnienieplucne.pl)

### PORTUGAL

#### XVII Jornadas de Cardiologia, Óbidos

The Department of Cardiology of the Hospital de Santarém promoted the organization of the XVII Conference on Cardiology, held in Óbidos November 4-5, attended by over 300 health professionals. The Portuguese PH Association participated in this Conference and in particular in the roundtable on "Pulmonary Hypertension - and now? Our talk was about the role of the patient associations. We started by describing the symptoms, the moment of diagnosis and the impact the disease has on the patient. We then looked at the impact of the disease at other levels: family, social, economic, professional. The disease changes life in many ways and affects the patient's perception of self. In a way, a "new" self emerges. We talked about how this "new persona" can interact with family, work, friends, the

medical team and also with the patient association. How can the Association help? This was the theme of the second part of our presentation. We spoke about aspects such as information sharing and patient education where we can provide information materials such as videos and brochures. We can foster the exchange of experiences and testimonies by organizing meetings with patients and physicians. We can also provide psychological and legal support, and can participate in European awareness campaigns (eg. Breathtaking campaign 2011, etc.) and so on. We also took the opportunity of this conference to present our national and international projects.

Maria João Saraiva,  
Associação Portuguesa de Hipertensão Pulmonar

[www.aphp-hp.org](http://www.aphp-hp.org)

## SLOVAKIA

**Educational-reconditioning stay and Annual General Assembly**

From the 21st to 23rd of the October the Slovakian PH patient association organized an educational-reconditioning meeting in Trenčianske Teplice, a very well known and beautiful spa resort in the mountains, North East of our capital, Bratislava. The meeting was attended by 35 members of the association.

We were very happy to greet as our guest speakers PHA Europe's Vice President Pisana Ferrari and the the President of the Czech Republic's PH association, Kateřina Nováková. In addition to the interesting agenda of the meeting patients and their family members had the opportunity of enjoying the spa treatments available at the hotel.

The meeting was opened by Eva Goncalvesová from the The National Institute of Cardiovascular Diseases who greeted the members.

Items on the agenda included lectures about PH, diagnosis, treatments, lung transplantation, as well as about lifestyle issues such as mental health, nutrition and physical exercise.

The General Assembly was held on the second day. During the assembly the annual budget was reviewed and approved and there were presentations about the different activities the association organized during the year made by some of the members. After that, Pisana spoke about PHA Europe and its activities as well as about those of the associations she chairs in Italy, AIPI. Pisana also brought us small gifts. Kateřina Nováková told us about PH related activities in the Czech Republic.

During the stay the patients were shown different types of rehabilitation exercises. They tried out posture exercises and breathing exercises. A cultural program had been organized as well and we enjoyed a guided tour of the famous historical 19th Century Turkish Hammam in the center of Trenčianske Teplice where we saw a belly dancer performance. During a short walk around the old town we tasted the local "Spawaffles" and our guide told us about the history of the spa resort, which dates back to the 13th century. In the evening there was a free discussion and patients were able to talk and share their experiences. The meeting was supported by the pharmaceutical companies Actelion and AOP Orphan.

**PHA Slovakia at the Slovak Society of Cardiology Congress**

For the first time the Slovakian PH association participated in the Slovak Society of Cardiology's Congress (August 6-10) with its own stand. We prepared and distributed a lot of materials for visitors, such as brochures about PH diagnostics, about psychological support for patients with PH, business cards and health cards for patients.

**Income tax advantages**

The Slovakian association has now been entered in what is called the "Register of notarial chambers" and thanks to this it will be able to receive 2% of income tax in 2012.

*Yveta Makovniková,  
Zdruzenie Pacientov s Plúcnou Hypertenziou*



*top left: Relaxation exercises - top centre: P. Mizerova, Iveta Makovniká and PhDr. Notová - top right: Kateřina Nováková, Pisana Ferrari and Yveta. Bottom centre: Yveta, Dr. Milan Luknar and Pisana Ferrari - bottom right: Kateřina Nováková with Vladimír and Peter Makovník*

### SPAIN

We will group the activities by categories as we did in the last Mariposa News for the sake of the reader.

#### Activities with the pharma industry

We attended two events organized by pharmaceutical companies. One of these events was hosted by Lilly where we had a short workshop with other patient organizations after some presentations about the drug process from the study of a new molecule to the marketing authorization. The other event was hosted by Pfizer. It was addressed mainly to health professionals and there were working groups to better understand the relationship between patient organizations and scientific societies.

#### Access to good quality PH treatment

This monitoring activity has taken a lot of effort in the past few months. Unfortunately, we have seen that several hospitals in Spain are giving generics of sildenafil in violation of the patent protection of Revatio as Orphan Drug that is valid until 2013. Worse than that, we have collected information leading us to believe that some hospitals are manufacturing their own versions of generics for sildenafil and possibly even bosentan without proper authorization, manufacturing control and pharmacovigilance by the Spanish Medicines Agency. We have alerted the authorities but no measures have been taken that we know of to investigate these allegations. We have published an alert for patients about these irregularities and we will keep working in cooperation with other patient organizations and the Pulmonology Scientific Society until the matter is solved. The ANHP has also received allegations of some disturbing manipulations of the original doses of iloprost. The ANHP is very concerned about all these irregularities and their possible impact on the clinical evolution of the patients.

#### Presence at events organized by PH Specialists

The ANHP has attended as participants to two important events organized by PH specialists. A very interesting conference on research about the use of stem cells in pediatrics hosted by La Paz Hospital (Madrid) and an international symposium organized by the multidisciplinary unit of the 12 de Octubre Hospital (Madrid). We have also taken an active role with the presentation of the patient perspective

at an event organized by Ramón & Cajal Hospital (Madrid) and the presentation of the ANHP during the opening of the workshop on nutrition organized by Clínic Hospital (Barcelona).

#### International support

The ANHP was present on September 6 at the European



above: Launch of the Respiratory Roadmap at the European Parliament in Brussels



left: Irene Delgado, ANHP President, and Migdalia Denis, President of the PH Latin Society, at the PH Latin Day in Miami on November 28

Parliament in Brussels for the launch of the Respiratory Roadmap to support the initiative of the European Respiratory Society and answering to the invitation issued by the European

Lung Foundation. The ANHP fully supports this type of initiative that aims to improve prevention and care of respiratory diseases.

The ANHP President and myself were also present and made presentations at the Latin PH Day celebrated on November 28 in Miami and organized by the HP Latin Society, the umbrella organization for Latin America. This was the central event of many other activities all over the continent and organized by the national societies. The ANHP is proud to endorse and support these celebrations that create awareness on PH and promote access to medical care and PH drugs for all patients. We sincerely congratulate the HP Latin Society for this important step.

We have also been working hard on collecting endorsement letters and support from PH Patient Organizations and Scientific Societies all around the world in order to declare



May 5 as the PH World Day. The ANHP is now starting with the preparations and special fundraising for the 2012 celebration in Madrid.

### Cooperation with Health Authorities

The ANHP is working with the health authorities in the autonomous region of Madrid on two main issues: the improvement of the rules and regulations that impact on the recognition of disabilities caused by PH and access to all available PH drugs at hospitals that wish to be recognized as PH reference centers.

### Publications

Pfizer has sponsored a new guide for patients living with PH written by Dr. Antonio Román Broto (and with an initial chapter on the view of the patient written by Irene Delgado). This new guide has the endorsement of the Pulmonology Scientific Society of Spain (SEPAR).



The ANHP has also started producing a newsletter to inform members about important subjects. We have produced issue number 1 about the problems with sildenafil, issue number 2 about the “Leadership and Planning for Results” Workshop. Issue number 3, about ANHP activities during 2011, is almost finished. We have also produced the new calendar 2012 funded by Bayer.

*above: new ANHP publication on PH and the first two newsletters issued by ANHP*

*right: Juan Fuertes at the Health Technology Assessment course in London, September 14-16*

### Training

It was very useful and inspiring for me to attend the course on Health Technology Assessment organized at the London School of Economics September 14-16, under the patronage of Bayer. We have also recently conducted a workshop for the Board of Directors on Planning for Results and Leadership funded by Praxair.

### New services for patients

The ANHP is now providing psychological telesupport for PH patients. A psychologist regularly calls by phone patients that need emotional support to go through rough stages in order to provide guidance, monitoring and counseling.

### Media

Interviews have been given to regional TV and radio stations to contribute to the awareness of the general public on PH. We have also sent the alert on the irregularities observed on PH treatments to the media.

### Fundraising

As every year, members of the ANHP have been contributing to collect funds through selling participations to the Christmas National Lottery. This is one of the main sources of independent income of the organization.

### Research

The ANHP is contributing to the research of Worldnet21 on the development of a new application for mobile phones that will be used to monitor biometric data of PH patients and will facilitate the follow up by the specialists on the clinical evolution and functional class of their patients. Finally, the ANHP provided support to a study conducted by Actelion about quality of life through questionnaires filled by patients and caregivers.

*Juan Fuertes, Asociación Nacional Hipertensión Pulmonar*



### SWEDEN

The PHA Europe General Annual Meeting in Castelldefels in September 2011 was very inspiring and an eye opener for us as a new patient association. Our focus this fall has been to spread information that there is a patient organization for PH. We have established contact with the PH-nurses and specialists who on a daily basis get in contact with PH-patients, because if these people know about the patient association they can inform their patients.

Since 2007 nurses and specialists who work with the disease in Sweden are part of an organization called "The Swedish association for PH". The members of this association meet twice a year and in October our patient association was invited to their meeting. Hopefully this meeting will be the starting point for a fruitful cooperation.

We have also met representatives from the pharmaceutical industry and discussed potential projects in the future.

The Swedish patient organization is part of the much

larger association, the "Swedish Heart and Lung". Our organization is the only organization within Heart and Lung that is focused on a special disease and the knowledge about PH is very poor even within Heart and Lung. Our organization was invited to the GAM for Heart and Lung in September where we had the opportunity to inform about the disease and our patient organization.

The discussion board about PH that we started in the summer of 2011 is now very lively. Generally there are 3-4 new posts every day and we are glad that so many want to share their daily experiences with others. The numbers of members of our Facebook group are also growing. In November we updated our website and, via Google translate, it is now available in most languages, [www.pah-sverige.se](http://www.pah-sverige.se)

Best regards from Sweden

*Patrik Hassel, PAH Sverige*

[www.pah-sverige.se](http://www.pah-sverige.se)

### SWITZERLAND - HTAP REVIVRE

#### HTAP Revivre outing at the Servion Zoo, Canton de Vaud

May 28 was a sunny day with a cool fresh breeze blowing from the north. Our group met at 10:30 and we were treated to



*Photos from the HTAP Revivre outing at the Servion Zoo, Canton de Vaud*

*above right: Lydia Benallouch, President of the association, first from left, with some of the guests*



some delicious appetizers prepared by our Board members. It was nice to discover new recipes for savoury cakes and taste the first cherries of the season straight from René and Rachel's garden. We then proceeded to visit the Servion Zoo, a small and enchanting animal park. Lunch was served in the zoo's restaurant, just by the tiger's pen, and after this break we continued our visit to the zoo, chatting away and taking photos. At about five o'clock we



Some of the animals at the Servion Zoo,  
Canton de Vaud



Members of HTAP Revivre at the zoo outing

had a light snack and then we all headed back home, with lots of beautiful images in our minds and friendship in the heart. A beautiful day of sharing, laughing, friendship and the joy of being together.

Looking forward to our next meeting!

Monika Sorge Maître, HTAP Revivre

[www.saph.ch/SuisseRomande](http://www.saph.ch/SuisseRomande)

## SWITZERLAND - SCHWEIZER SPHV

### Regional meetings



Regional meeting in Zurich, August 16

This summer there were several new PH-regional meetings. Our goal is that patients and their families have the opportunity to meet several times a year in their own region and get together. People with rare diseases often feel isolated. What patients and their families go through in everyday life

is not easily understood. It is a painful experience and it is a great help to hear somebody say: "Yes, I can



Regional meeting in Brunnen, September 15

role in promoting such meetings and in providing information. These



Regional meeting in Basel, August 11

understand". Somebody to share experiences and advice on how to cope with the disease in their daily lives, to get support and information. The association has an important







Regional meeting  
in Khur,  
September 20



Regional meeting  
in Frankfurt,  
October 18-20



Regional meeting  
in Zuchwil,  
November 14



meeting can also serve as a platform for patients to voice their concerns and raise awareness on a more general level.

### Cooking event

On August 27 a group of parents of children with PH met in Zurich for a cooking event. The children had a cooking lesson and in the mean time the parents took a city tour around Zurich. When the parents came back from their visit the children were very proud to show the meal they had prepared: a little appetizer, a main course and dessert. In the afternoon the children went to play in the garden and the parents were able to talk to each other and share experiences.



### Swiss delegation at the PHA Europe GAM

Two members of HTAP Revivre and two from our own association represented Switzerland at the PHA Europe General Annual Assembly September 8-11: Franz Fischer and Daniela Isidoro-Silveira from SPHV and Lydia Benalouch and Monika Sorge Maître from HTAP Revivre.

### German PH Association meeting

Thanks to our sponsors we were able once again to afford to travel to Frankfurt for the German PH patient meeting October 28-30. Two of our members, Daniela Isidoro-Silveira and Franz Fischer represented our association at this meeting. There were many interesting workshops and lectures and lots of interesting information to take home. One of the events organized was about "How to deal with my doctor?" Christina Galfetti, social psychologist and patient, gave an exciting keynote speech. Participants were able to contribute to the discussion. The speaker gave some advice on how to bring one's concerns to one's treating physician. The doctor-patient relationship is a social interaction of two people where the communication is very important. The second part was facilitated group work. Patients and family members were separated into smaller groups and the important points on how to help make the next visit to one's doctor a "success" were collected.

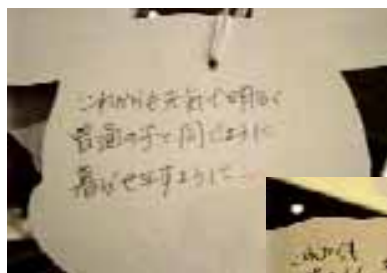
Bruno Bosshard, SPHV

from left: Lydia  
Benalouch,  
Daniela  
Isidoro-Silveira,  
Monika Sorge  
Maître and Franz  
Fischer at the PHA  
Europe General  
Annual Meeting  
in Barcelona



## JAPAN

On September the 25th, PHA Japan organized a “PH Day gathering” in Tokyo in collaboration with Bayer Yakuhin. The aim of the meeting was to promote the understanding of pulmonary hypertension and to deepen the exchange between doctors, patients and family members. Four doctors and 40 patients from three to 80 years attended the meeting. Together with the patient’s relatives the total number of participants was about 70 people. It was the first time that PHA Japan organized this kind of gathering



Messages written by patients: “I want to lead healthy & good life like other children” and “I want to live longer and help other people”



in Japan in collaboration with a PH pharmaceutical company. The focus of the event this time was on young patients.

The first part of the meeting was titled “Let’s study PH with a Quiz”. The quizzes covered various topics: “Clinical condition of PH” supported by Professor Shouzaburo Doi from Tokyo Dental and Medical University, “PH in connective tissue disease” by Professor Fumitaka Mizoguchi also from Tokyo Dental and Medical University, “PH in Respiratory Medicine” by Professor Yoshiteru Morio, from Juntendo University and “Current treatments for PH” by Professor Hiroyuki Fukushima from Keio University.

The participants scratched the answers from three options and the four doctors announced the correct answers and gave more details.



Individual counseling sessions with the four doctors then took place. In addition, an “art workshop” had been organized around the blue-purple awareness color “pansy”. Cards written by patients and their families were later displayed in the conference room (see photo on left). It was very important for patient to realize that although PH is a very serious disease which is still being diagnosed too late, treatments for PAH are now available. In order to improve the environment for the PH patients, we will have to continue to work together with healthcare professionals and pharmaceutical industry. We believe we can help many patients by firmly promoting this three-way co-operation.

At the end of conference, the room was full of smiling faces!

Noriko Murakami, PHA Japan





## LATIN AMERICA

### PH Latin Day, Miami



We are very pleased to announce that the celebration of the Latino Pulmonary Hypertension Day was a success. Over fifty persons attended the meeting, including eminent health care professionals, patients, friends and family members, with the aim of sharing experiences and knowledge about pulmonary hypertension. This success was made possible by the support of all.

The event was held as a tribute to Dr. Lewis Rubin who is a world-renowned specialist in Pulmonary Hypertension. It was coordinated by Dr. Andres Carmona and there was an interesting and wide ranging agenda which included the following topics:

- *Social networking, media, continuing education programs and network integration*, by Dr. Lourdes Denis, Venezuela - Pulmonary Hypertension Association, USA.
- *The Latin Vision for Rare Diseases and Orphan Drugs*, by Dr. Virginia A. Llera, Argentina, President of Geiser.
- *Advances in Pulmonary Hypertension in Latin America*, by

Dr. Jaimes Morales, Mexico, Section Chief, Pulmonary Circulation, SMNYCT.

- *The PPH Latin Day and its reach globally*, by Ms. Migdalia Denis, USA, President of the PH Latin Society.
- *Overview of pulmonary hypertension worldwide*, by Mr. Juan Fuertes, Spain, Coordinator, National Association for Pulmonary Hypertension (ANHP), Spain.
- *The World Pulmonary Hypertension Day: origin and significance*, by Ms. Irene Delgado, President of the National Association for Pulmonary Hypertension (ANHP), Spain.



above: from left, Juan Fuertes and Irene Delgado (ANHP, Spain), Cristina Nates (PH Latin Society) and Dr Lewis Rubin together with organizers and guests at the PH Latin Day in Miami



We were very happy to welcome our special guests representatives of PHA (USA), and Gerald Fischer, President of PHA Europe, the European Pulmonary Hypertension Association (Austria).

Our warmest thanks to all our speakers and guests on behalf of all pulmonary hypertension patients who ultimately are the ones who most need attention and solidarity.

Migdalia Denis, PH Latin Society



left: Irene Delgado (President ANHP, Spain) and Migdalia Denis (President PH Latin Society)  
right: Virginia A. Llera, Dr. Jaime Morales, Gerald Fischer, President PHA Europe, Migdalia Denis and Irene Delgado



## USA

### Tenth International PH Conference and Scientific Sessions

PHA's 10th International PH Conference and Scientific Sessions *The Power of One: From a Kitchen Table to Around the World* is an opportunity for the PH community to look back at the progress that has been made in the PH field over the years. PHA's first conference in 1994 brought together 100 patients and family members. The 10th anniversary Conference, taking place June 22-24, 2012 in Orlando, Florida, USA, will bring together almost 1,400 PH patients, caregivers and medical professionals for three days of education, support and networking.

PHA's conferences offer opportunities for every attendee: Scientific Sessions for medical professionals seeking to enhance their knowledge of the disease, Patient/Family Led and Medically Led Sessions for patients and their families, and Meet-Ups for the community. Our 10th International PH Conference will feature 15 sessions led by PH patients, 40 sessions led by PH physicians, and 15 Support Group meetings. In addition to enriching our regular programming, we've also increased the number of sessions offered in Spanish and sessions focused on pediatric PH.

PHA is also pleased to offer networking opportunities for our global audience. At PHA's last Conference, the First International Leader's Summit brought together PH association leaders from around the world. The Summit was intended to build relationships among the leaders and promote peer-to-peer learning. National PH association leaders from 20 nations were able to attend the Summit in 2010 and we hope that more leaders will join the global com-

munity for the Second International Leaders' Summit on Thursday, June 21, 2012. Additionally, during the afternoon of Friday, June 22, regional international meetings will take place where global guests can come together to meet patients and families from their own region. On that same day, we will be hosting an evening International Reception for all global guests.

Join us in Orlando, Florida, June 22-24, 2012 for PHA's 10th International PH Conference and Scientific Sessions and for the Second International Leaders' Summit on Thursday, June 21. Registration opens on January 11 and more information can be found online on our website: [www.PHAssociation.org/conference](http://www.PHAssociation.org/conference).

Any questions about the Second International Leader's Summit can be sent to Meghan Tammaro, International Services Manager ([MeghanT@PHAssociation.org](mailto:MeghanT@PHAssociation.org)).

Meghan Tammaro, PHA



[www.phassociation.org](http://www.phassociation.org)

### Next PHAE newsletter

The Spring 2012 issue of the PHA Europe newsletter is due out at the end of **April/early May**.

The first part of the new Mariposa News will feature a report on the PHA EUROPE BREATHTAKING AWARENESS CAMPAIGN which will be organized around International RARE DISEASE DAY, on the 29th of February. We will include reports and photos of events organized by PHA Europe members within the context of this campaign and would be very grateful if you could send your contributions by **the end of March at latest**.

The second part of the newsletter will contain any other materials relating to activities conducted by the members at a more general level and not connected to the campaign.

You may send any contributions for Mariposa News in your own language and we will provide for translation. The newsletter will be available in PDF format and in a printed version. The PDF will be e-mailed, and the printed copies will be sent by post to the national associations. Many thanks in advance!

## European PH patient leaders' annual meeting in Barcelona



*The meeting was great, and it was wonderful to see that this group of so many different people with different backgrounds is getting stronger and closer every year. It gives us all the energy we need to go on working for the good cause. Although there were heavy days with a full agenda there was fun and laughing as well and this made it feel like a little holiday :) It was such a great event that I forgot to be tired: all the positive energy gave me wings!*

**Maaïke Atres, Netherlands**

The 2011 PHA Europe annual meeting was the largest to date, with 46 PH patient leaders from 22 European states attending. For the second consecutive year, the meeting was held in a charming location, Castelldefels, a small town not far from Barcelona. We had the same hotel on the sea, all to ourselves, an essential factor in cementing relationships and creating what experts call “team building”. The atmosphere was warm, friendly and permeated with a great sense of solidarity. It was wonderful to meet up with old friends and welcome the new ones. Sharing experiences and information is a very important aspect of this event.

The four-day program began with the President's annual report. 2011 was another very busy year for PHA Europe. The association held its first pan-European awareness-raising campaign on Rare Disease day, involving 12 European countries (under the patronage of EURORDIS), it is steadily building up its membership (8 new countries since 2010 bringing the total to 22) and achieving increasing recognition as one of the key stakeholders in PH across Europe. PHA Europe representatives were invited in the course of the year to present the PH patients' perspective at a number of prestigious international scientific and industry events. Recent and on-going projects include a new web-campaign on the importance of the “TIME” factor in PH and the convening of an

expert Roundtable to draw up a common position statement addressed to policy makers.

Once the annual report and 2011 budget had been discussed elections were held for the new PHA Europe Board which will serve from 2012 to 2014 (see page 3 for the list and profiles of the new members).

The meeting had a very wide-ranging agenda which covered many different topics of common interest such as the problems that still need to be addressed despite the consid-



erable progress that has been made over the last 15-20 years in the field of PH:

- scarcity of information on the disease;
- delays in diagnosis;
- inaccessibility of drugs in some countries;
- lack of specialized centres;
- problems with transplantation and the existence of specialized structures, follow-up, etc.;
- need for psycho-social support.

In some countries, particularly in Eastern Europe, only one or two of the seven approved drugs are available and prostanoids are completely lacking. Patients from many of countries must go abroad for transplantation and other surgical procedures for PH. And it is very difficult to be reimbursed; for instance, in Russia there is no reimbursement for any PH drug. Empowering the national associations is vital in order for them to be able to face up to these challenges and a few sessions were devoted to discussing and exchanging experiences about:

- organizational and bureaucratic problems;
- building up membership;
- finding volunteers;
- establishing a fruitful and lasting collaboration with healthcare professionals;
- fundraising - necessary in order to ensure continuity;
- improving communication strategies;
- making the most of the new social media and the web;
- lobbying and advocacy;
- joining forces with other groups in order to reach critical mass.

On this last topic it was very interesting to hear about the great potential for online rare diseases patient communities from EURORDIS, the European Organization for Rare Diseases,

represented by Denis Costello. "Rare-connect" was born under the aegis of EURORDIS and its North American counterpart, NORD and PHA Europe has decided to be part of this exciting project ([www.rarediseasescommunities.org/](http://www.rarediseasescommunities.org/)). Very interesting and encouraging scientific and medical updates were given to us by top PH expert Prof. Jean Luc Vachiéry, Head of the Department of Pulmonary Vascular Diseases of Hôpital Erasme, Belgium, and PH Nurse Specialist Wendy Gin-Sing, from Imperial College, UK.

Prof. Vachiéry spoke about how the future perspectives for PH patients can be improved by more timely diagnosis and treatment, by optimising existing resources, for example through "upfront" versus "sequential" or add-on combination therapy (see also page 37 for his talk at the ERS 2011), and novel therapies targeting different pathways. Many are in the pipeline and appear to be promising. In Prof. Vachiéry's view, future clinical trials should be based not on the classic endpoints (6 minute walking test etc) but on "strategy", i.e. to improve management.

Wendy Gin-Sing's description of how PH is managed in the UK and in particular at the medical centre she works for was also extremely inspiring and provided a great example of how optimal management of PH should be organized.

Finally, it was a great pleasure to have as guest speakers at the meeting our main industry partners: Actelion (Dr. Alessandro Maresta, Head of Global Medical Affairs, Switzerland), Bayer (Dr. Jutta Ulbrich, Global Advocacy Manager and Dr. Silke Gerlach, Global Scientific Affairs, Germany), Glaxo-SmithKline (Dr. Mihaela Ianoşev, Medical Director, Critical Diseases, UK) and Pfizer (Dr. James Clayton, Director Critical Diseases, UK). Over the years these companies have given PHA Europe their invaluable support, helping us to grow stronger, and we are very grateful for this.

*Pisana Ferrari*





## European PH patient leaders' annual meeting in Barcelona

- Dear all, thank you first Pisana and Gerry for all your efforts and thanks to all of you so much that PHA Europe GAM was so full of positive energy and hope for all patients with PAH. We wish you all, all the best, to be a beacon of peace wherever you are.

*Klara and Barbara (Slovenia)*



*Maleen Fischer (Austria) and  
Maria Patey (Ketchum Pleon, UK)*

- Hello everyone, greetings from Madrid where I am spending a few days of vacation before returning to Israel. I left Barcelona with the belief that our community is growing every day stronger. Pisana and Gerry deserve much credit for creating the surroundings so that this could take place. Ultimately, we all contributed in our own ways and I look forward to our continuing success. Keep in touch. Blessings to you all.

*Yosef (Israel)*

- Dear friends, it has been great to share this time with you. As always, we came back to Madrid with new ideas and full of energy to carry them on. Irene was pulling my leg saying that I write long messages, so I will make this one short: THANK YOU ALL because you are a source of energy and inspiration, I admire your strength and your courage and I hope that we will contribute to your growth in the same measure with our own work and initiatives. Hugs to all of you and special thanks to the organizers who always give us such a great support.

*Juan (Spain)*



*Günther and Hilde Thimm  
(Germany)*

- Hello everybody, it was a great time in Barcelona! We would like to thank you for a very wonderful and inspiring meeting. We came back to Poland with new ideas and full of energy to work. We also want to thank Pisana and Gerry for their hard work in the organization of the meeting. We wish you all the best and a big hug for everyone.

*Agnieszka and Piotr (Poland)*



*Gergely Meszaros (Hungary),  
Pompiliu Paiu (Romania)  
and Eszter Csabuda (Hungary)*



*Agnieszka Bartosiewicz (Poland)  
and Jaana Rajahalmi (Finland)*



*Luc Matthysen (Belgium) and  
Regina Votavova (Czech Republic)*



*Mihaela Raykova  
and Todor Mangarov (Bulgaria)*

- Hello everybody, greetings from Latvia! It was an amazing time in Barcelona! We would like to thank everyone for very inspiring meeting. We came back to Latvia with new ideas and full of energy to work. Big thank to Pisana and Gerry for their hard work! Warmest regards and kisses.

*Ieva and Lolita (Latvia)*

- Dear all, 4 days hard work of 2 fantastic people make PHA Europe 2 times bigger and irreplaceable for our organisations. Thanks Gerry and Pisana.

*Hendrik (Belgium)*

- Hallo everybody, I hope you had a nice flight home. Thanks to the cooperation of all, Gerry and Pisana have done a HUGE WORK and made this meeting possible. The results can't be ignored or underestimated. It has been extremely important and interesting and I think that we are looking forward (among other goals) to the White Paper. Thank you Gerry and thank you Pisana so so much for what you have done and for what you will do for PHA Europe. While I am back in hospital among "my" patients, I wish you all the best and a very big GOOD LUCK in your mission! Arrivederci

*Marzia (Italy)*

- Hello friends, thank you all for the big surprise in Barcelona! I was truly impressed to learn about your serious and deep involvement in intense activities meant to ultimately improve the lives of so many people, your commitment, and also your result oriented efforts and accomplishments. Gerry and Pisana, congratulations for an interesting, exemplary organized and pleasant meeting! You have all been an inspiration for me, and examples to follow! Greetings from Romania.

*Radu (Romania)*



*Ieva Plume and Lolita Indriksone (Latvia)*



*Juan Fertes and Irene Delgado (Spain)*



*Hall and Carol Skåra (Norway) with Ümit Atli (Turkey)*



*Gerald Fischer (Austria), Regina Votavova (Czech Republic), Maaïke Atres (Netherlands)*



*Kirsten Dahl (Sweden) and Barbara Salobir (Slovenia)*



*Franz Fischer (Switzerland) and Hendrik Ramaker (Belgium)*

## European PH patient leaders' annual meeting in Barcelona



Guenther Thimm (Germany)  
and Jaana Rajahalma (Finland)

- Dear Pisana, thank you and “Bravo!”, as we say in French, for this very well prepared and very interesting meeting! I was really sorry not to be able to stay until the end. I hope you managed to rest a bit after the impressive amount of work you put into our meeting. Many thanks to you, Gerry, Luisa and all those who contributed to making it such a success and to all those who attended.

*Monika (Switzerland)*

- Hi everybody, back in the good old rainy and cold Sweden. Though its autumn and I have caught a cold I am filled with energy from our meeting in Barcelona. One year ago I felt pretty alone searching for information about the disease and trying to get in contact with others with experiences about PAH. These days in Barcelona have meant very, very much for me personally and for the work of creating a sustainable PHA Sweden. I feel so lucky that I got the opportunity to meet all you fantastic people in Barcelona!

*Love from Patrik (Sweden)*



Maaïke Atres and Ferdinand  
Bolsius (Netherlands)

- Dear all, first of all I would like to apologize because my English is not fluent enough, I'm so shy and self conscious when it comes to trying to speak it that I could not speak with you as I would have liked. It has been very gratifying and motivating to see how PHA Europe has grown. In my first PHA Europe GAM in 2005 we were only 9 countries, meeting the leaders of 22 countries a few years later and seeing that we have become a big family is the best news ever because there is strength in numbers and working together will help us to get a better future. I would like to thank the veterans who have been fighting for years for the rights of the patients in their countries and I would like to tell the younger associations that you should not be scared, do not shy away before the tasks that look impossible because they can be done. For sure, it takes a lot of work and endurance but growth comes with serious work and sense of responsibility. My first meeting to found the Spanish PHA was in the room of a hospital with 7 other patients and look what we have done in six years publishing, attending and preparing events, getting recognition... I hope to see you again in Madrid on May 5th and we will celebrate together the first PH World Day. Allow me to congratulate Gerry and Pisana for their hard work in the organization of the GAM. As we say in Spanish, besos (kisses) to all of you.

*Irene (Spain)*



Ümit Atli (Turkey), Klara Klancar and  
Barbara Salobir (Slovenia)



Radu Toma (Romania)



Gerald Fischer (Austria), Wendy Gin-Sing  
(Imperial College, UK) and Jean-Luc Vachiéry  
(Erasmus Hospital, Belgium)



Mihaela Ianoşev (GSK, UK), Gerry Fischer  
and Alessandro Maresta (Actelion, Switzerland)



- Hey! I just want to thank you all wonderful people I met in Barcelona. My English is not so good (at least I think so), and I'm very shy to speak it, but hopefully you will understand what I'm writing here :-). Meeting was so useful and inspiring, we have so much to do here in Finland, and I'm full of ideas because of everything I've heard and learned during the time in Barcelona. And the place was just paradise, I miss the beach and warm weather already! Great big hugs and special thanks for Pisana and Gerry, who made an enormous job to organize this event, and of course everyone else too, it was great to meet you all nice, amazing people from all over the Europe. Hopefully we will meet again, let's keep in touch, best wishes and hugs from rainy Finland.

*Tuulia (Finland)*

- Greetings from the cold north! I wish you all could see what we saw when leaving the meeting to catch the plane: You were all waving to us and you all had a great smile on your faces! It was really touching. We truly have become a great PH-family with no country borders! The disease connects us all and we probably feel unified in a way most people will never be lucky enough to experience... Thanks for a great conference, and thanks to Gerry and Pisana for all their hard work! I will send a link to some of the photos I took at a later point.

*Hall (Norway)*

- Hello everybody, it was great to seeing you and I would like to thank all of you for the friendly atmosphere, it was wonderful. It was so nice and I'm sure our meeting was for all of as very interesting and we went back home with new ideas and full of energy. Many thanks to Pisana and Gerry for organizing the meeting. I hope all of you are well and wish you all the best.

*Lydia (Switzerland)*

- Dear friends, we had a good flight back to Germany and want to thank Pisana, Gerry and all the others who organized the meeting for the excellent job they have done. It was great to see the PH-family growing and working together for what we want to achieve. We are looking forward to seeing you, those who were not able to come, and more new members next year. Warm regards.

*Hilde und Günther (Germany)*



*Iris Tal (Israel)*



*Mihaela Ianosev (GSK, UK)*



*Jaana Rajahalme and Tuulia Nappi (Finland)*



*Luc Matthysen (Belgium)  
and Marzia Predieri (Italy)*



*Patrik Hassel (Sweden)  
and Monika Sorge Maître (Switzerland)*



*Pisana Ferrari (Italy), Gerald Fischer (Austria)  
and Rosie Matthysen (Belgium)*

## European PH patient leaders' annual meeting in Barcelona



*Patrick Fischer (Austria)*



*James Clayton from Pfizer (UK)*



*Marzia Predieri (Italy) and  
Lydia Benallouch (Switzerland)*



*Evgeniy Komarov (Russia)  
with his interpreter*



*Vladimir and Peter Makovnikov  
(Slovakia)*



*Juan and Kirsten  
dancing...*



*... also Rosie and Irene!!!*



*... Pisana with Juan...*



*Agnieszka Bartosiewicz and  
Piotr Manikowski (Poland)*





*Denis Costello presenting the 3rd prize on behalf of EURORDIS for the 2011 photo and video contest to Rosie Matthysen (Belgium)*



*Guenther Thimm (Germany), Yosef Gotlieb (Israel) and Pisana Ferrari (Italy)*



*Sylvain Redeyllet and Mélanie Gallant-Devawrin (France)*



*Jutta Ulbrich from (Bayer, Germany) and James Clayton (Pfizer, UK)*



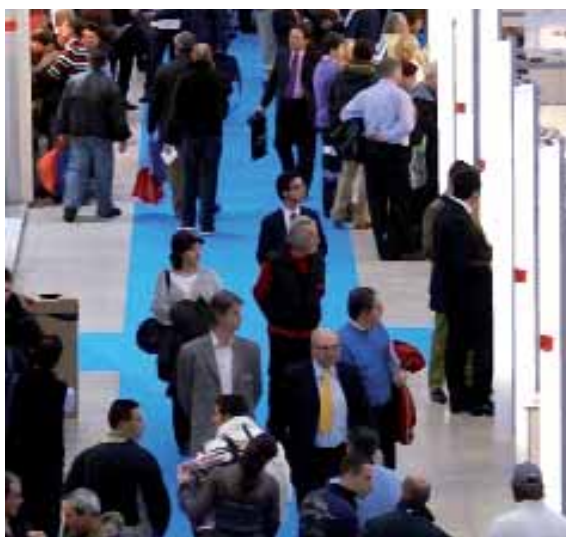
*Klara Klancar (Slovenia) and Daniela Isidoro-Silveira (Switzerland)*



*Wendy Gin-Sing, Nurse Specialist (Imperial College, UK)*



*Rosie Matthysen (Belgium), Marzia Predieri and Luisa Sciacca della Scala (Italy)*



The EUROPEAN SOCIETY OF CARDIOLOGY (ESC) and EUROPEAN SOCIETY FOR RESPIRATORY MEDICINE (ERS) Annual Congresses are key international scientific events consistently drawing tens of thousands of participants from around the world. The 2011 editions figures are impressive: 30.000 participants for the ESC and 20.000 for the ERS. PHA Europe has been present at the ESC and ERS with a booth and representatives of the association ever since it was first founded, in 2003, in Vienna. The number of scientific meetings, symposia, focus-on, poster and abstract sessions dedicated to pulmonary hypertension within these Congresses has been increasing over the past few years at an incredible rate and these are always extremely well attended. This reflects on the one hand the level of commitment and dedication of the international scientific community involved in the field of PH and their capacity to bring the disease to the forefront of world attention. On the other hand, the topic has gained interest also thanks to the considerable advances in research and development of new drugs which have opened up new, more encouraging, perspectives for patients in terms of survival and quality of life.

Taking part in major international scientific events such as the ESC and ERS provides PHA Europe with a unique opportunity to present its activities, distribute its publications and meet with key medical opinion leaders, industry representatives and members of international organizations. The scientific sessions also have a very important educational role as PHA Europe representatives have the possibility of hearing about the latest developments in research and new treatment strategies which they can then report back about to its affiliates. The 2011 edition of the ESC Congress was held in Paris August 27-31 and the PHA Europe delegation consisted of myself, Mélanie Gallant Dewavrin, Managing Director of the French PH patient association HTAP France and Marzia Predieri, Member of the Board of AIPI, Italy. At the ERS in Amsterdam, September 24-28, Ferdinand Bolsius, President of PHA Netherlands, joined Marzia and myself to man the PHA Europe booth and attend some of the sessions.

### **“Treat to target” PAH management strategy**

Prof. Marc Humbert (PH national reference center, Clamart, France) presented the most recent data on survival - 60-70% at three years - drawn from the French Registry. This represents a great improvement with respect to the past but is still unsatisfactory. “Treat to functional class II” represents a very good treatment strategy but more can be done to achieve earlier diagnosis. Early detection of the disease remains difficult for idiopathic patients without risk factors but can be applied to patients with co-morbidities. Better outcomes have been shown from screening of populations at risk such as scleroderma patients and Prof. Humbert gave the example of a 8-year follow up study carried out at Clamart. A strong case for the Treat to target approach was also made by Vallerie Mc Laughlin (University of Michigan, USA) in her analysis of the Reveal Registry, a 55 center, 3,000+ patient observational study on PAH patients diagnosed through right heart catheterization. The survival at 24 months in patients who had improved from functional class III to II was significantly better (86%) than that of patients who



left: Marzia Predieri distributing brochures at the ESC 2011 Congress, Paris  
below: Marzia Predieri, Mélanie Gallant Dewavrin and Pisana Ferrari at the PHA Europe booth





had stayed in functional class III (78%). Prof. Nazzareno Galiè (University of Bologna, Italy) in his presentation analyzed data related to survival of 831 patients in the period 1999-2010. In idiopathic PAH the mortality was 7,5% annually. This was not a homogenous group, some patients were on monotherapy, others on double or triple therapy. If you break this data down you see that in 117 patients with idiopathic PAH on double combination therapy the mortality is lower, 12%. If you further break this down, the mortality is lowest in the patients in functional class II (4%) than those in functional class III (22%). Taking another group of 32 patients on triple combination therapy with epoprostenol you have a mortality of 20%, BUT, if you subdivide this further, the mortality of those in functional class II is much lower (9%) than that of functional class III (45%). Functional class correlates significantly to prognosis. In his conclusions, Prof. Galiè says that functional class is both an achievable and a desirable target in PAH treatment.

### Combination therapy: escalating to prostanoids?

This was a very interesting debate with Prof. Simon Gibbs (Imperial College, UK) arguing for a gradual escalation to prostanoids and Prof. Jean-Luc Vachiéry (Hôpital Erasme, Belgium) in favour of a more aggressive upfront approach. Simon Gibbs said that there is currently no evidence of the advantage of upfront add-on of prostanoids whereas there are excellent results from oral combination therapy. Before escalating to prostanoids one should add a PDE-5 to an ERA drug. Prostanoids have risks (infection) and drawbacks (self-image problem, timetable of pump changes) and are considerably more difficult to manage. The patient choice is also important when making this kind of life-changing decision. There is always time to assess at a later stage and then escalate to prostanoids. In his reply Prof. Jean-Luc Vachiéry stressed that the disease is progressive: quality of life is important but physicians must address the issue of survival! Doctors have a huge responsibility in this respect. And there is scientific evidence to corroborate the upfront approach: of all the clinical trials on combination therapy conducted in the past only two have met the primary endpoint (6 minute walking test increase) and both relate to a combination with a prostanoid (PACES and TRIUMPH1). Patients on prostacyclin are the ones who do better and this appears clearly in clinical experience. The current dogma of approved trials is based on a two-drug regimen with sequential combination therapy (you start with one and add another when things get worse). An alternative approach could envisage upfront combination therapy, based not on classic endpoints but on "strategy", ie to improve management.

A question and answer session followed with interesting points being made about one approach versus the other:

- If you start a patient on prostanoids it is not possible to



above: Ferdinand Bolsius, Pisana Ferrari and Marzia Predieri at the PHA Europe booth at the ERS 2011 Congress, Amsterdam

withdraw and one will never know whether he would have worsened without. Once on it they stay on it...

- Upfront combination therapy has worked well in other diseases (heart failure, HIV).
- Oral prostanoids would considerably decrease the complexity of management.

### Endothelial dysfunction: topical versus systemic?

Another interesting discussion centered around the issue of whether the endothelial dysfunction (narrowing of the blood vessels, cell proliferation) that is associated with PAH is topical (i.e. related only to the pulmonary circulation) or systemic. Prof. Martin Wilkins (Imperial College, UK) in his presentation claimed that although there may be some evidence of systemic endothelial dysfunction, vascular remodeling (lesions in the blood vessels) should remain the main target. Mortality is still high despite recent advances and in his view it is totally premature to think of systemic endothelial dysfunction. Prof. Ardeshtir H. Ghofrani (University Clinic, Giessen, Germany), on the other hand, suggests that patients with peripheral endothelial dysfunction do worse than others and this should be considered. Patients should be viewed in a holistic way, taking into account all the different aspects: there is more to it than just the disease. Evidence for peripheral endothelial dysfunction involves exercise impairment, cerebral perfusion, which can damage the cognitive function, erectile dysfunction associated with PH and systemic hypertension. With reference to the latter, it is important to remember that approved drugs using the three pathways address systemic hypertension. sildenafil and ERA's were originally researched for anti-hypertensive use and for heart failure. In the discussion which followed Prof. Gérald Simmoneau raised the issue of whether vascular remodelling is a cause or a consequence of PAH. Prof. Ghofrani says that data collected from the pediatric field shows that the endothelial dysfunction may actually precede the vascular remodelling.

Pisana Ferrari



The EUROPEAN SOCIETY OF CARDIOLOGY (ESC) held the 2011 edition of the EDUCATIONAL COURSE ON PULMONARY HYPERTENSION (PH) on October 14-15 in Sophia Antipolis, France. This course is organized by Prof. Nazzareno Galiè (Italy) and Prof. Gérard Simonneau (France). Over a hundred cardiologists, pulmonologists and other specialists involved in PH care from all over the world attended as participants. This year the topic of the discussion was “Gaps of knowledge, gaps of evidence, gaps of organization” and the course was followed by the Annual G5 meeting”. The G5 meeting brings together the key stakeholders involved in PH: physicians, nurses, patients, industry and payers. PHA Europe has been invited at the ESC Courses since 2009 to give the patient perspective. It was a great privilege to represent the European patients at this prestigious educational event.

### GAPS OF KNOWLEDGE

#### Genetics

Genetics of PAH was addressed by Prof. Marc Humbert, (PH Reference Center, Clamart, France). Prof. Humbert explained that mutations of the gene coding for BMPR-II (BMPR2), a receptor expressed on pulmonary vessels wall cells, are the leading genetic cause of familial PAH. However, carrying a BMPR2 gene mutation is neither necessary nor sufficient to develop PAH: indeed you can have PAH without the mutated gene and you can have the mutated gene and not get PAH (the penetrance is around 10-20%). Even in familial PAH, patients can have PAH without identified BMPR2 gene mutation: the mutated gene is found in 80% of families with familial PAH history and also in 20% of apparently sporadic PAH who are in fact potentially heritable. Importantly, there can be reduced expression of the product of the BMPR2 gene in the pulmonary arteries of patients with PAH without identified gene mutation, highlighting the importance of this receptor in the pathomechanisms of PAH. Much still remains to be done in order to gain a full understanding of the mechanism behind the genetics of the disease.

#### Candidate therapies for the future

Regarding candidate therapies Prof. Humbert explained that with current therapies, which cover three pathophysiological pathways, survival had improved. Before the advent of epoprostenol and the new drugs the life expectancy was dramatic. It now stands at 60-70% at 3 years in incident “newly diagnosed” cohorts of patients. In order to better improve outcomes in PAH the following objectives are important:

- better assessment of PAH severity;
- better defined treatment goals;
- early detection and management;
- improvement in medical therapies (novel therapies, upfront or sequential combination of therapies);
- better integration of non-medical therapies such as atrioseptostomy and lung transplantation.

Despite recent progresses, PAH survival is still unsatisfactory and there is a need for better knowledge of the disease and development of novel therapies targeting other pathways. The pipeline for new drugs is actually quite a crowded environment, with the exception of CTEPH, for which the therapy of choice is surgical pulmonary endarterectomy. However, we still have around 50% of CTEPH patients who are inoperable and in need of medical treatment(s).

Some of the drugs in the pipeline cover existing pathways with novel approaches eg. FREEDOM-C, GRIPHON and SERAPHIN studies. FREEDOM-C tests oral treprostinil (a prostanoid). The results have been published recently and the study did not meet the primary endpoint (6 minute walking test). The big challenge for the company will be to reach a sufficient but at the same time well tolerated dose. Selixipag (GRIPHON study) is an elective IP receptor agonist currently tested in a Phase III study. Macitentan (SERAPHIN study) is a tissue-targeting endothelin receptor antagonist, currently in Phase III of the clinical trial.

A number of clinical trials target new therapeutic pathways and among these imatinib (IMPRES study) is the one in the most advanced stage of investigation, with results of the Phase III trial presented at the ERS 2011 last September. Imatinib is an orally-active tyrosine kinase inhibitor which has some anti-proliferative therapy at least in part because it targets a receptor of PDGF, an important growth factor for pulmonary vessels wall cells. Imatinib is already licensed for the treatment of chronic myeloid leukemia and has a few other oncology indications. The presented study showed improvement in walking capacity and hemodynamics, and a trend for more clinical worsening on imatinib versus placebo. Imatinib may provide an interesting new therapeutic option for selected PAH patients, symptomatic on two or more established PAH therapies but needs to be investigated further (side effects, possible cardiac toxicity).

Riociguat, a stimulator of soluble guanylate cyclase (sGC), is another tested treatment option currently in Phase III. The phase II trial was an open label one studying PAH and



CTEPH. It is currently being investigated in 2 Phase III trials, one in PAH (PATENT study), the other one in CTEPH (CHEST study).

Another compound is inhaled nitrite. This substance has vasodilator and anti-proliferative properties. In newborn lambs, an aerosol of inhaled nitrite has been found to reduce pulmonary blood pressure. This will need to be followed up with further human research.

There is potential for testing drugs interfering with the serotonin pathway, among which terguride, a serotonin receptor type 2 antagonist. This substance has other effects and it is already approved for the treatment of hyperprolactinemic pituitary adenoma, ovulation disorders due to hyperprolactinemia, galactorrhea and suppression of puerperal lactation. A proof-of-concept study is currently being undertaken to test the possible action on PAH.

To summarize, in the next five years we are likely to see many new agents: some relating to “old” and established therapeutic pathways (prostacyclin, endothelin and NO) as well as other on “new” pathways.

## GAPS IN EVIDENCE

### Goal oriented therapy

The afternoon session began with a presentation by Prof. Marius Hoeper (University of Hannover, Germany) about goal-oriented therapy. The European (ESC/ERS) guidelines on PH set out this approach which involves a baseline assessment of the patient, follow up assessments and review of therapy according to established treatment goals. In Prof. Hoeper's view this is a good strategy which applies to all patients: it “works” and there is now evidence to this:

- treatment goals are based on clinical variables of prognostic importance (functional class, 6mwd, bio-markers, hemodynamics, etc.);
- follow up assessment is at least as important as baseline assessment for prognosis;
- reaching treatment goals is associated with favourable prognosis, not reaching them is associated with poor prognosis and quality of life;
- nevertheless, treatment goals need to be individually adapted.

### Sequential or upfront combination therapy?

Prof. Olivier Sitbon (PH Reference Center, Clamart, France), addressed this very important topic. The majority of randomized controlled trials (RCT) conducted up to now have been related to monotherapy, there are a few on sequential combination therapy, and only one on upfront combination therapy. All trials on monotherapy have been conducted over a short period of time (12-16 weeks). The primary endpoint, that was the change in the 6-minute walk distance (6MWD), was met in all those studies. However, only i.v. epoprostenol has shown improved survival in a randomized controlled trial. Some other drugs also impacted outcome by delaying the time to clinical worsening

(TTCW). The TTCW is going to become the primary endpoint in current and future trials. A recently published meta-analysis (Galiè, et al) including all clinical studies conducted on PAH, covering over 3,000 patients, showed a reduction in mortality of 43 % with active drugs compared to control. However, the average time of this studies was quite short (14 weeks) and the impact of PAH drugs on long-term outcome cannot be drawn from these trials. This underlines the need to analyze long-term survival in large international multicenter cohorts and the importance of registries in this setting.

In other words, we can do much better. How? Basically, there are two ways. The first is to optimize what we already have (treat to target and sequential combination therapy or upfront combination therapy), the second to use new drugs. The ESC/ERS guidelines say that in case of inadequate response we have to consider sequential combination therapy. What is the evidence for this? There have been quite a few studies (STEP, PACES, PHIRST, TRIUMPH, FREEDOM-C) and both primary and secondary endpoints have been met only in one (PACES)! The alternative could be initial combination therapy. For functional class IV the guidelines already foresee this but the level of evidence and the degree of recommendation were quite low. The nature of PAH being malignant there is a rationale for a more aggressive approach right from the start. This has been successfully applied in other diseases (e.g. heart failure, HIV infection, cancers...). Today, only one RCT (the BREATHE-2 study) has tested this approach: epoprostenol monotherapy vs epoprostenol and bosentan combination therapy. However, there was no significant difference between the two treatment arms, even if there was a trend for a better hemodynamic improvement in the combination therapy group. Prof. Sitbon explained that in his centre, at Clamart (France) they are very confident with this approach, tried out successfully on 23 patients with upfront epoprostenol and bosentan combination therapy which were evaluated over a 30-month period. Data from Clamart is now available also on triple upfront combination therapy. Twelve newly diagnosed young patients (average age 41 year-old) were initiated on a combination of Flolan-Revatio-Tracleer. These were very severe patients with low cardiac index (CI <2 L/min) and very high pulmonary vascular resistance (PVR >1,700 dyn.s.cm<sup>-5</sup>). The results are very encouraging: after four months, 11 patients had dramatic hemodynamic improvement (one underwent lung transplantation) with a mean fall in PVR of 70%, normalization in CI and even decrease in mean pulmonary artery pressure by a mean of 20 mmHg. All were in class II and showed a marked improvement in the 6MWD. At 22-month mean follow up, all patients were alive and in functional class I or II. If the same approach were applied to less severe patients the results could be even better. Looking towards the future the next regimen for clinical trials would be “strategy” trials.

One ongoing study, AMBITION (352 patients planned), is investigating upfront combination therapy with two oral drugs, ambrisentan and tadalafil, not from the point of view of the drugs but the strategy (upfront combination therapy vs monotherapy). It is an event-driven trial with the primary endpoint being time to clinical worsening. It will end when 82 “events” (hospitalization, disease progression, unsatisfying long term response, death) will occur. Prof. Sitbon concluded his talk by saying that in future upfront combination therapy is likely to be the best approach but this will need to be validated by large randomized-controlled trials.

### Interventional therapy

Prof. Adam Torbicki (Institute of Tuberculosis and Lung Diseases, Warsaw, Poland) analyzed the existing gaps in evidence with regards to the main surgical options for PH.

- Ballon atrial septostomy (BAS) is a procedure which relieves the burden from the right side of the heart by shifting the blood to the left. This happens also in patients with Eisenmenger syndrome who are known to have a better prognosis than other PAH patients. BAS leads to a better systemic perfusion at the cost of an acceptable degree of desaturation. It works only if the pressure is higher in the right atrium than in the left. There is some evidence to show that patients on double combination therapy having undergone BAS have a better survival. In Prof. Torbicki's opinion BAS, which is now used as a “bridge” to transplant, could be used at an earlier stage of the disease. This hypothesis would need to be validated by a study, which, for obvious ethical reasons, could be randomized but not blinded. BAS is a high risk procedure and there are legal issues involved as well.
- Pott's shunt is another surgical procedure which creates a connection between a systemic artery and the pulmonary artery. This way there is shunting of blood to the lower part of the body. There is only a small case series and more reports would be needed to fully understand the benefits.
- Lung transplant: the big issue here is when to list? Randomized controlled trials are not ethical. Optimal timing is depending also on local organ donation availability. Recurrent bleedings (hemoptysis) are not common but mortality is very high and hemoptysis is emerging as a possible new indication for transplant (study conducted in Clamart 1998-2008). Dilatation of the proximal pulmonary artery can lead to dissection and death. How can these patients be screened for lung transplant? In some cases the risk of pulmonary artery rupture is high and there is much to do to protect patients, maybe lung transplant.
- Pulmonary arterectomy can be a very good option for CTEPH patients as a recent paper on CIRCULATION indicates (679 patients analyzed). The main problem is the fact that not all patients are operable.
- Balloon angioplasty in CTEPH is highly controversial as there is a risk of rupturing the artery and of edema. Perhaps

it could be an option for non-operable patients deteriorating despite treatment.

### GAPS IN ORGANIZED CARE

Prof. Marion Delcroix, University Hospitals Leuven (Belgium) presented the initial results of an online survey on PAH management in developed countries which is due to be published shortly. All therapeutic approaches were taken into account: first line drugs, prostacyclins, new drugs and surgery. Questionnaires comprising 50 items were sent to 21 national experts and out of these 17 participated and provided feedback. One of the topics addressed was referral centres. 47% of the countries which took part in the survey have designated centres but despite this the problem is that many patients are still being treated in small centres. The data on availability of drugs is another big issue as it varies greatly from country to country with sildenafil, bosentan and inhaled iloprost present in all 17 countries, treprostinil in 82% (IV treprostinil 53%), epoprostenol in 76%, tadalafil in 47%, ambrisentan in 16%. Many patients in developed countries are still dying without access to prostacyclins. It will be very interesting for us to see the published report.

### ANNUAL G5 MEETING OF STAKEHOLDERS

#### The vision of the industry

Alessandro Maresta, Head of Global Affairs at Actelion, Switzerland, presented the industry vision for the future of PAH. Dr Maresta's presentation focused on the great achievements in terms of diagnosis and treatment over the last 15-20 years. Many steps forward have been made, in particular with regard to improved survival. In the year 2000, a diagnosis of PAH was the equivalent of a death sentence, PAH today is a chronic and progressive disease, in future the great hope is that it will become part of life, just like many other chronic illnesses. This can be achieved by:

- better therapies, targeting current and new pathways;
- better designated randomized controlled trials;
- early detection;
- optimization of treatment.

This vision for the future needs to be shared and discussed with key opinion leaders in the field, including patients. It will be necessary to conduct long term studies with novel and robust morbidity-mortality primary endpoints such as the time to first event (worsening of the disease, initiation of intravenous prostanooids, BAS, lung transplant, death), with all the events judged by an independent adjudicating committee.

Early detection is another key issue. This can be achieved in part by screening populations potentially at risk. The DETECT study is currently evaluating ten screening tools that will be evaluated against the golden standard (right heart catheterization). The results of the first phase will be presented at ESC and ERS 2012. Dr. Maresta concluded his presentation by underscoring Actelion's continuing commitment to awareness, education and research and its desire to collaborate with all the shareholders to achieve optimal management of PH.



### The vision of the nurses

Wendy Gin Sing is Nursing Consultant from Hammersmith, UK. In her talk she presented the role of nurses involved in PAH in the UK, focusing on seven key issues:

- Patient education: info, training on infusion and inhaled therapies (videos), management of side effects, monitoring of drugs, strategies for managing heart failure, advice on emergency situations.
- Patient and caregiver support: lifestyle advice (travel, work, intimacy, nutrition, exercise), psycho-social support, liaison with local health care providers, working with the patient associations.
- Nurse education: ESC nursing meeting in 2012, PAH Virtual Clinic (see page 47), Master's degree at Edge Hill.
- Nursing research: local and national audit, quality of life measurement tools, prevention of sepsis, holistic approach to patient management (to include anxiety, depression etc.).
- Multidisciplinary teams: referral centres in the UK have a strict criteria and must have at least 2 medical consultants, 1 clinical nurse specialist, 1 radiologist and access to psycho-social support.
- Influencing national policies: steering committee with industry, NHS and advisory boards, input into national guidelines, lobbying (eg. free oxygen on airlines).
- Networking: International Nurse meeting (Dresden 2011), European nurse Forum.

Wendy Gin-Sing concluded her talk by saying that her objective for the future is to create a European PH nurse network. She stressed the importance of having a holistic view of patient care which can improve the patient experience.

### The vision of the Regulatory authorities

Dr. Amany El Gazayerly (EUROPEAN MEDICINES AGENCY, EMA), started her talk by reminding the audience that the regulatory authorities have nothing to do with "money" i.e. economic considerations, but only with safety and efficacy. How are drugs registered? Companies apply to the EUROPEAN MEDICINES AGENCY which grants approval for all of Europe (centralized procedure). Patients are involved in the process leading up to drug approval. Incentives and facilities are available for companies through the EU orphan drug regulation for treatments related to rare diseases.

Looking at treatments approved for PAH we see that not all have not been approved through the standard centralized EMA procedure: Flolan was registered through national authorities, Treprostinil was started by France and then recognized by "mutual recognition".

Clinical trials still speak of primary and secondary endpoints but we are moving to new terminology, such as, for example, quality of life. Of course this is important because it the patients who will be treated but regulators tend to prefer "hard" indicators. With regard to new products future studies will be moving more towards morbi-mortality endpoints and this is an interesting development.

Regarding pediatrics, Dr. Gazayerly briefly mentioned the

two drugs currently available in the field of PAH: sildenafil and bosentan. Sildenafil was tested on 250 patients and the assessment led to the registration of the pediatric indication. So this is not off-label use but a real indication which includes idiopathic and congenital heart disease. There is a warning about higher doses which needs to be taken into account. The situation with bosentan is different as the EMA does not have a complete program. In July 2010 EMA published a decision on the agreement of a paediatric investigation plan and on the granting of a waiver for bosentan for pediatric use.

Dr. El Gazayerly ended her talk by stating that the EMA understands the need to take into account new developments and in particular it has addressed the updating of the guidelines on Process Validation in a concept paper published in 2009 on the EMA website:

[http://www.emea.europa.eu/docs/en\\_GB/document\\_library/Scientific\\_guideline/2010/03/WC500075029.pdf](http://www.emea.europa.eu/docs/en_GB/document_library/Scientific_guideline/2010/03/WC500075029.pdf)

### The vision of the patients

The annual G5 stakeholder meeting provides an excellent opportunity for European PH patients to voice their concerns to the international medical community. PHA Europe has been invited to attend since 2009.

Pulmonary hypertension patients have considerably benefited in terms of survival from the development of new drugs and treatment strategies, the setting up of expert centres and improved surgery options. There is still scope for optimization of current, approved drugs and research on drugs targeting "old" and "new" therapeutic pathways is promising and could further improve the perspectives of patients. But many issues still need to be addressed:

- PH remains an incurable, progressive and very debilitating disease with a poor prognosis and with severe implications on family, social and professional life.
- Psycho-social support is crucial in order to achieve a better quality of life. Patient associations can play a vital role in this respect and their work should be further promoted and medical professionals encouraged to collaborate (the ESC/ERS guidelines explicitly recommend this).
- There is need for more awareness both at the level of general population and among the medical community (primary care, medical curricula) in order to achieve earlier suspicion, diagnosis and treatment of PH.
- Patients need to be referred to expert centers for optimal disease management and this objective is still far from being reached.
- There are still considerable differences in access to approved drugs and surgery options across Europe.
- Much remains to be done with respect to raising awareness on organ donation and transplant: more favourable legislation alone could open new life-saving opportunities for patients at end-stage disease.

*Pisana Ferrari*



Over 250 medical professionals from 20 countries took part in the third edition of the yearly “Pulmonary Hypertension Update” organized by the University of Bologna (Italy) on November 25-26. It was a great honor to be invited to address the audience on behalf of the European PH patients. The two-day event featured distinguished members of the international medical and scientific community as guest speakers. The program covered a broad spectrum of PH-related issues, ranging from basic research to the more to clinical aspects such as diagnosis and treatment strategies. The management of PAH varies considerably from country to country and this very important topic was the object of one of the sessions. Top medical PH experts provided very interesting information and insights into the situation in their own countries. Despite considerable progress made in the last 15-20 years, there are still countries where PAH management is less-than-optimal and much remains to be done in general to increase awareness of the disease and to achieve earlier diagnosis and treatment.

The management of PAH varies widely from country to country, even within the European Union, and this emerged very clearly from the session devoted to this topic within the Bologna conference. Despite the fact that all the presentations were about “developed” countries, with organized PH centres and access to PAH-specific treatments, the situation is far from being optimal. Not all approved treatments are available everywhere and eprosteno, for example, is either not available or not used in quite a few countries. PAH drugs are expensive and this is a critical issue in those countries where there is no reimbursement at all. Many countries do not have transplant or pulmonary endoarterectomy facilities. Lack of knowledge among the medical class is another big problem which can lead to delays in diagnosis and patients starting treatment in advanced stages of the disease. Even geography can play an important role in how health care is organized. In vast countries such as Russia there are huge parts of the country which are far from the medical centres (problem of “rurality”); Slovakia is a small country but there are vast mountainous areas and the two medical centres are eccentrically located at the far ends of the country.

### Finland

Dr. Pentti Kornhnen is from the Tampere University Hospital in Helsinki. His country, Finland, has a population of 5.4 million. The health care system is publicly funded from tax revenue. All citizens have an annual contribution fixed at 600 euros after which the State covers expenses. The country is sparsely populated and there are long distances to cover and this has had an impact on care. There are 20 central district hospitals and 5 University Hospitals. The diagnosis is usually established at the University hospitals. For the most “demanding” treatments it is necessary to travel to Helsinki. Data from 2010 indicate a total of 110 diagnosed patients in the country. Of these, 52 have idiopathic PAH, 4 familial, 2 drug-induced, 49 associated forms of PAH, 3 pulmonary veno-occlusive. 70% of the patients are on monotherapy and this is too high. Of those who are on combination therapy, only 12% are on prostanoids (iloprost or treprostinil). Epoprostenol is not available, mostly for fear of complications and difficulties. As far as surgery is concerned, this is concen-

trated in the Helsinki centre where 16 lung transplants have been performed over the last 16 years, as well as one successful balloon atrial septostomy. Dr. Kornhonen concluded by saying that it is necessary to centralize the diagnosis and treatment with the University hospitals, leaving the most demanding cases to Helsinki. Ideally there should only be one center but this is difficult geographically speaking. There is definitely much that remains to be done in the field of prostanoids.

### Portugal

Portugal has a population of 10.5 million and the estimate prevalence for PAH is 500 patients. The cost to the State is around 9.5 to 33 million euros. All drugs are available, with the exception of eprosteno, but only in hospitals. There are no lung transplant or pulmonary endoarterectomy facilities in the country. The dilemma, says Dr. Abilio Reis, is that being a rare disease PAH has a low impact on public opinion. At the same time it is costly and penalizes the institutions. It is necessary to work on this to break the vicious circle and to elaborate a strategy for PAH which will address these weaknesses and concentrate the expertise. The idea is to build a national network. The first step will be to involve the national authorities and a suggestion has been put forward to set up a technical organization. The criteria for reference centres are currently being discussed. There are plans to set up a database for information and global management.

### Russia

Russia has a population of 142 million and only 20 PAH centres, which are clearly not sufficient, explains Dr. Natalia Goncharova. There is no national PH registry or data base. Dr. Goncharova is based at the V.A. Almazov Federal Centre of Blood, Heart and Endocrinology of St. Petersburg. This is the main reference centre for the North West part of the country and covers a population of about 12 million. Ninety-eight patients with either idiopathic PAH or inoperable CTEPH have been entered in the data base of the hospital since 2007. The mean age is 40 plus/minus 14 and 79% are under 50 years of age. The mean time to diagnosis stands very high at 42 months. A large majority of the patients, 69%, were in functional class III and IV at time of diagnosis. Access to



treatment is a big issue in Russia as there is no reimbursement by the State and no national program for PAH. Bosentan costs about 4,000 euros per month, iloprost 1,576 and even the cheapest drug, sildenafil, at 500 euros per month, is inaccessible to but very few patients. Ambrisentan is due to be introduced in 2012. At the present time patients who cannot afford drugs have to rely on charity funds and some funding from local municipal budget. The "Natasha Foundation" patient association - part of PHA Europe - also provides economic support to a few patients. Only 45 patients at the Almozov hospital are being treated.

Pulmonary endarterectomy is being performed at three centres and about 150 procedures have been carried out since 2004. Lung transplant surgery is lagging behind with only 4 performed in St. Petersburg of which two lung and two lung/heart. Dr. Goncharova ended her presentation by listing the main problems in Russia: lack of awareness, no registry, no data on prevalence, incidence etc, lack of choice and access to drugs for the majority of patients, no social support, absence of a well-organized transplant service.

### **Slovakia**

Dr. Milan Luknar from the National Institute of Cardio-Vascular disease, Heart Failure and Transplant Department in Bratislava, presented the situation in his country, which has a population of 5.4 million. The capital is eccentrically located and the country is mountainous so that there are 400 km between the expert centres of Bratislava and Kosice, the two designated expert centres for PH. In Slovakia health insurance is compulsory and for people in need it is paid for by the government. The concept of setting up a comprehensive program for the management for PAH started being developed in 2005 and has been adopted by the National Cardio-Vascular Institute in Bratislava. The central role is played by the cardiologist and the PH clinic. Management is based on the ESC/ERS guidelines, which have been translated into the Slovak language. All treatments are fully covered by the National Health Service. The following drugs are available: bosentan, sildenafil, ambrisentan, iloprost and treprostinil. Epoprostenol is not licensed and not available. Lung transplant for PH is managed in conjunction with the AKH University Hospital in Vienna. Patients are referred to Vienna for surgery and follow-up is conducted locally.

A retrospective study was conducted in 2005 by the Slovakian Society of Cardiology analyzing 129 patients diagnosed according to the European guidelines. The results indicate that patients are still being diagnosed very late and this highlights the need for more knowledge and awareness of the disease in the country. Slovak doctors have established a very good collaboration with the Slovak PH patient association.

### **Czech Republic**

The Czech Republic has a population of 10.4 million and three expert centres, two of which are in Prague and the third

in Olomuc. 70% of the patients are treated in the centre where Dr. Pavel Jansa is based, the General University Hospital. All approved drugs are available, including epoprostenol. Transplant and pulmonary arterectomy are covered by national programs. The number of patients treated at the present time is 350. The cost for the state is quite high and estimated at 72,000 euros per year per patient. A retrospective study on 271 patients shows that 80% were already in functional class III or IV at time of diagnosis with very poor hemodynamics. A screening program has been set up to identify PAH earlier in patients with systemic sclerosis.

Dr. Jansa concluded his talk by saying that the Czech Republic has a good, centralized and well-running program for the management of PH with all the key drugs available and national registry set up and running.

### **Saudi Arabia**

Dr. Majdy Idrees is a PH specialist at the Ryad Military Hospital. Saudi Arabia has a population of 30 million with six expert centres in Ryad and three in Jedda. At the Ryad Military Hospital a program for PAH was initiated in 2008 and 94 cases (59 of which female) have been examined in the past three years. This is the first registry of PAH patients set up in the area. All of the patients were diagnosed through right heart catheterization. Data collected shows that 57% of the patients were diagnosed in functional class III and 16% in class IV. Time to diagnosis was over three years for 40% of the patients and at least one year for 70%. Screening is needed for high risk populations and this issue still needs to be addressed. Basic access to treatments is guaranteed and all treatments are available. 30% of the patients are on monotherapy, 55% on double and 15% on triple.

### **United Emirates**

The United Emirates was founded in 1972 and is one of the countries with the highest population growth in the world. At the time of the first census, in 1975, the population was just over 500,000, it is now 8 million. Nationals represent only about 15% of the total, with the remaining 85% being immigrants. Dr. Ahraf Alzaabi, from the Zayed Military Hospital - national centre for PAH - explained that significant steps forward have been taken in setting up the PAH unit in his Hospital. Some progress has also been made with regard to health care costs: evaluation tests for PAH are now free. All treatments are available with the exception of epoprostenol (not enough infrastructure), but the big dilemma is that they are not reimbursed. At the Zayed Hospital 38 patients are currently being treated, most of which have PAH related to congenital heart disease. Much remains to be done in terms of unmet needs: increase the volume of patients, set up an audit system to monitor progress and compare with international centers and, above all, how to organize funds for the less privileged patients who cannot afford treatments.

*Pisana Ferrari*

“Health technology” is the application of scientific knowledge to health care. It can range from drugs to preventive care (vaccination campaigns or pre-implantation screening for genetic mutations) to use of helicopters to transport organs for transplantation.

## Health Technology Assessment (HTA)

The Health Technology Assessment (HTA) process deals with the economic evaluation of how a new drug changes the life of the patient. For example, pricing of new drugs and/or how the new drug has an added value for the patient, their caregivers and the society will determine whether the marketing authorization they get at the EUROPEAN MEDICINES AGENCY (EMA) level is implemented at national level.

What is the cost of a treatment and is it worthwhile for a country to pay for it? The impact at medical, social, economic and ethical levels that a new health technology will provide are the key elements that will be taken in to account by the national bodies in making their decision. Today HTA plays an important role in determining reimbursement decisions.

## Bodies involved in HTA

The health technology assessment is under the responsibility of HTA bodies at Member State level. Many organizations throughout the world assess healthcare technology. There is an evident need to cooperate and share information from different cultures and networks have been set up in Europe and world-wide. EUnetHTA is the EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT, established with the aim of uniting government-appointed organisations from EU Member States, EEA and EFTA countries and a large number of relevant regional agencies and non-for-profit organisations that produce or contribute to HTA.

<http://www.eunetha.eu/>

## EU policy in HTA

The European Commission is currently working on a “Joint Action” under the Health Programme together with the Member States in the field of HTA. In most Member States HTA plays a major part in evidence-based health decision-making. The Commission aims at enhancing the cooperation between the Member States in this field. The Joint Action, set up for 2010 to 2012, involves EU 24 Member States as well as the EFTA countries Norway and Switzerland and receives funding from the Public Health Programme.

[http://ec.europa.eu/health/technology\\_assessment/policy/index\\_en.htm](http://ec.europa.eu/health/technology_assessment/policy/index_en.htm)

## Why it is important for patients to be involved

In many countries, there is an “all for the patient but without the patient” policy and patients are not invited to give an input. Training in how HTA works will enable individuals to represent the best interest of the patient when it comes to introducing the new technology or rejecting its reimbursement or implementation by the health authorities.

We tend to use emotions to defend our right to access a new technology (for the purpose of this article we will consider as “new” any health technology that is not available in our health system disregarding how long ago the drug, the device or the procedure was approved for human use). However, emotions count very little and it is necessary to provide evidence-based data if patients want to be heard by the decision makers.

## Making the case for indirect cost savings in HTA

The approval for human use is based on safety and efficacy of the technology. HTA deals with economic aspects. But, economy means many things, not only financial costs. For example, does the new technology reduce hospital visits for the patient? Does it allow the caregiver additional time to have a professional life or a fulfilling personal life that delays the caregiver’s “burn-out”? These two questions are a good example because they entail different outputs; let us imagine that the new technology would indeed produce a reduced number of visits to the hospital for a patient that can still work:

1. It means a lesser cost for the health system.
  - If a simple consultation at the hospital costs an average of 100€ and attention at the emergency room costs, let’s say, 800€... it’s a simple math exercise.
  - Less use of hospital resources by a patient means an increase in the number of patients that can avail of the hospital services; therefore there is need of fewer hospitals for a given number of inhabitants.
2. It can also make the difference between employment and unemployment.
  - Absenteeism can be a reason to sever a person from their company.
  - Keeping the patient’s mind off the next medical follow up will increase productivity.
  - Keeping their job will bring tax revenue to the government.
3. It improves the capacity of the patient to enjoy life and make a difference in the lives of those around them (taking care of the children, lifting a burden off the caregiver’s shoulders, having a happier and healthier state of mind...) And so on and so forth...

## The contribution of EFNA

The EUROPEAN FEDERATION OF NEUROLOGICAL ASSOCIATIONS (EFNA) understands the importance of empowerment of patients and dedicates part of its efforts to build capacity in Health Technology Assessment. In this way, patients from all over Europe can work with their governments and HTA agencies so that their voices are taken into account when it comes to introducing a new health technology that will affect their quality of life or their life expectancy.

I personally attended to the course organized by the London School of Economics in cooperation with EFNA (London





left: Juan Fuertes at the course on HTA organized by the London School of Economics in cooperation with EFNA, London, September 14-16

below: Juan Fuertes at the EURORDIS Summer School for patient advocates, Barcelona, June 12-14



September 14-16). It was a very enlightening experience that I fully recommend to all patient associations that want to play a role in supporting access to health technologies for PH patients in their countries. The knowledge acquired in the course enables the participants to speak the same language as those who decide on our futures.

### The contribution of EURORDIS

EURORDIS, the European Organization of Rare Diseases, also firmly believes that it is vital for patients to understand HTA and be involved in its definition. Over the last few years it has allocated significant resources to build the capacity of its staff, volunteers and patient representatives in this area. HTA was the focus theme of the last EURORDIS summer school organized in June 2011 in Barcelona, Spain. More than 30 patient representatives from all over Europe were introduced to the main HTA assessment tools by academics, HTA agency and industry representatives and public health experts. In addition, patient representatives had an opportunity to present their HTA experiences in a panel discussion. EURORDIS is involved in many other projects related to HTA and is also part of the European network for Health Technology Assessment (see below) where it has been appointed as one of four patient representatives at the EUnetHTA Stakeholders Forum.

<http://www.eurordis.org/content/eurordis-and-health-technology-assessment>

### Recent European events in the field of HTA

EUnetHTA, the EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT, has recently organized a conference in Gdansk, Poland (December 8-9). The Conference was attended by 275 participants from HTA agencies, ministry representatives, and stakeholders representing patients' organizations, payers, health providers and industry.

The European Commissioner for Health and Consumer Protection, John Dalli, was present at the conference. In his talk he expressed the Commission's aims at enhancing the cooperation between the EU Member States in the field of HTA. He recognized the important role of HTA in health

decision making: "It will be a true challenge for companies, healthcare providers and policy makers to reconcile high prices with the growing demands of healthcare from an ageing population, and under a climate of economic and budgetary austerity. Health Technology Assessment (HTA) offers one key to addressing these issues. Indeed a large majority of Member States use HTA to support decisions on the use of new medicines, medical devices and new surgical procedures. This allows for a more transparent evidence-base for these decisions, which are often difficult to make". Mr Dalli stressed that it will be vital to keep in mind not only the unit costs in a hospital but also the broader benefits to society as a whole.

[http://ec.europa.eu/commission\\_2010-2014/dalli/docs/speech\\_08122011\\_en.pdf](http://ec.europa.eu/commission_2010-2014/dalli/docs/speech_08122011_en.pdf)

### Conclusions

Nobody knows better than the patients themselves what having a better quality of life or increased life expectancy really means. Patients can bring evidence-based data or reasoning that will influence the public opinion or the judgment of a decision by health policy makers.

Understanding HTA empowers the patients and patient organizations to intervene in the process of making a new technology available in their countries. Both patients and patient organizations should be involved in HTA and understand its mechanisms and its procedures because they will be the direct beneficiaries of the implementation at national level of new technologies.

Juan Fuertes, ANHP, Spain

### State of the Art of Rare Disease Activities in Europe 2011

This report was adopted by the EUROPEAN UNION COMMITTEE OF EXPERTS ON RARE DISEASES (EUCERD) July 2011. EUCERD was created in 2009 and has an enormous influence in the governments' attitudes and policies regarding rare diseases within the EU. It has 51 members with representatives from the 27 EU Member States and from all domains relevant to the fields of rare diseases and orphan drugs, including academia, government, the biopharmaceutical industry and patient organisations. The full report is divided in three parts and can be downloaded at <http://www.eucerd.eu/>. The report contains information on the political framework, expert services in Europe research and development, orphan drugs and therapies for rare diseases, patient organizations and information services. The activities around rare diseases are categorized in four sections related to public health, orphan drugs, research and transversal activities.

### Recommendations on Centres of Expertise on Rare Diseases in Member States

This report was adopted by the EUROPEAN UNION COMMITTEE OF EXPERTS ON RARE DISEASES (EUCERD) in October 2011. It is the first set of recommendations adopted by this committee. Developing Centres of Expertise and European Reference Networks in the field of rare diseases has been proposed in the 2009 COUNCIL RECOMMENDATION ON AN ACTION IN THE FIELD OF RARE DISEASES and more recently in the CROSS-BORDER HEALTHCARE DIRECTIVE (adopted in October 2010) as a means of organising care for the thousands of heterogeneous rare conditions affecting scattered patient populations across Europe. In order to share knowledge and expertise more efficiently, the EUCERD recommendations 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.

<http://nestor.orpha.net/EUCERD/upload/file/EUCERDRecommendationCE.pdf>

### The Clinical Added Value for Orphan Drug (CAVOD) Study

This report, adopted in October 2011, has been funded by the EXECUTIVE AGENCY OF HEALTH AND CONSUMERS of the European Union. This agency is based in Luxembourg and is entrusted with ensuring the safety of products for human consumption in Europe. The report was undertaken in the framework of the European Union health programme and was carried out by Ernst and Young. It aims at providing insight on coordination at European level of something that is already familiar for the Pulmonary Hypertension Associations in Europe: the Orphan Drugs' Authorization Processes. The pathway of an Orphan Drug goes from the initial Orphan Drug Designation at the EUROPEAN MEDICINES AGENCY (EMA) to reimbursement by Member States. When the

new medicines are on the market they go through a process of assessment to identify their real value based on the actual experience in each country. The CAVOD study proposes a coordination mechanism that would allow Member States to share the information avoiding duplication and to make the most of available resources. The report has now been delivered to the European Commission and the recommendations and proposals it contains will be the subject of consultations with all involved actors and stakeholders.

The full CAVOD study can be found at <http://ec.europa.eu/eahc/news/news94.html>

### Rare Diseases' Registries: the EPIRARE PROJECT



EPIRARE (EUROPEAN PLATFORM FOR RARE DISEASE REGISTRIES) is a 3-year project co-founded by the European Commission within the EU PROGRAM OF COMMUNITY ACTION IN THE FIELD OF PUBLIC HEALTH. EPIRARE started officially on April 15, 2011. Collecting data is one of the very first steps in gathering information that will lead to research breakthroughs. But it does not help to have heaps of disorganized items, which is one of the challenges for researchers that try and extract useful information from different national registries. But first things first: is there a PH registry in our country? How does it work? In some European countries there is a PH registry, a database fed by PH specialists with data about their patients. These databases have rules about the criteria that allow the physicians to enter a case. The unification of criteria and gathered data provides material to extract statistical reports and identify trends. Giving researchers the possibility to access organized data from many countries enriches the departure point and gives solid foundation to their work. That's why it is important to develop a project in order to harmonize registries when dealing with a rare disease such as PAH.

The Epirare Project is seeking:

- to analyze the existing registries and identify good practices;
- to build consensus to address the regulatory, ethical and technical issues associated with rare disease patients' registries in Europe;
- to elaborate possible EU policies;
- to build the grounds for the creation of a future EU rare disease registry platform.



EURORDIS, the European Rare Disease Organization, is the second largest partner in this project, which brings the patients to be actively involved in the process.

<http://www.eurordis.org/content/epirare-project>

<http://www.epirare.eu/>

### Creation of the International Rare Diseases Research Consortium

One of the main threats we have to face as rare diseases patients is that the efforts in the research field can be lost due to the lack of coordination. If we think that there are up to 7,000 rare diseases, it is easy to understand the need to establish bodies that will ensure the care for each and every one of them in different aspects. One of these elements of interest is specially cherished by patients because it is the field that brings hope for a better future: research.

There is a growing consciousness at political level about the importance of rare diseases and the European Commission, together with the US National Institutes of Health and the Canadian Institutes for Health Research, initiated in April 2011 the INTERNATIONAL RARE DISEASE RESEARCH CONSORTIUM (IRDiRC) that has the task to maximize the impact of the scarce resources that are allocated to research in the rare disease fields.

[http://ec.europa.eu/research/health/medical-research/rare-diseases/irdirc\\_en.html](http://ec.europa.eu/research/health/medical-research/rare-diseases/irdirc_en.html).

What will the IRDiRC do?

In order to achieve its goals, the International RD Research Consortium will have to provide a frame of policies that will facilitate the efforts coordination. For instance, it will have to concentrate information that will be available worldwide in such a way that researchers will have access to:

- Harmonized data and samples.
- Molecular and clinical characterization of rare diseases.
- Transnational resources for preclinical and clinical research.

The success in these aspects would provide the basis to achieve the goals set by 2020; namely to deliver 200 new therapies for rare diseases and diagnostic tools for most rare diseases.

Do patients have a role to play at the IRDiRC? We most certainly do have a word to say about research. There are diverging objectives that need to be balanced. Whereas scientists would want to increase knowledge in very specific fields that have little or no relevance to impact the daily life of a patient, the patients push to find solutions to very practical issues that change quality of life and life expectancy.

There must be a balance and patient umbrella organizations for rare diseases have been invited to be part of each Scientific Committee whose members will be appointed by the Executive Committee of the IRDiRC.

What do the IRDiRC Scientific Committees do? There are three Scientific Committees formed by 15 members each: Diagnostics, Therapies and Horizontal Aspects. The members of the committees will be representatives of the academia,

patient organizations, diagnostics, pharmaceutical industry and regulatory bodies.

These committees shall:

- Act as scientific coordinating bodies
- Propose research priorities for consideration by the Executive Committee
- Propose policies and guidelines for adoption by the Executive Committee
- Assess progress made by the Working Groups (i.e. projects funded)
- Address arising issues of scientific nature
- Encourage exchange of protocols and best practices, and agree on standard operating procedures, quality standards, etc.

[http://ec.europa.eu/research/health/medical-research/pdf/draft-irdirc\\_en.pdf](http://ec.europa.eu/research/health/medical-research/pdf/draft-irdirc_en.pdf)

Call for nominations: PHA Europe has received information from EURORDIS about the importance of the IRDiRC and stressing the need to be present as patients in all three Scientific Committees. EURORDIS itself has presented one candidate to the Therapies Scientific Committee and has encouraged rare diseases umbrella organizations to nominate candidates to the Diagnostics and Horizontal Aspects Scientific Committees. EURORDIS has been very active in the IRDiRC from the beginning promoting the involvement of patients. The creation of the Consortium has been a great achievement for the rare diseases community worldwide and it is of the utmost importance that patients have been given a role to play at its highest working level.

### Nursing and PH



PHA Europe members will remember the very interesting presentation made at the GAM 2011 by Wendy Gin-Sing, Nurse Specialist at Imperial College- London, about the role of nurses in PH management in the UK. Thanks to her, we have learned to appreciate the importance of nurses for PH patients and patients' organizations. She is part of the faculty that will enable nurses to expand their competences in management of PH patients through an online program by San Lucas Medical and MDSNe.

<http://www.pahclinic.org/>

Juan Fuertes, ANHP, Spain

### NOVEL THERAPIES

The issue of candidate therapies for the future was discussed during the ESC Educational course on Pulmonary Hypertension organized in Sophia Antipolis on October 14-15. See page 38 for the interesting presentation by Prof. Marc Humbert (PH Reference Center in Clamart, France).

### NEW TREATMENT STRATEGIES

Another very interesting presentation at the ESC was related to the pros and cons of sequential (add-on) versus upfront combination therapy of currently approved drugs in the context of the optimization of current approved treatments: see page 39, Prof. Olivier Sitbon (PH Reference Center, Clamart, France).

### PHASE II AND III CLINICAL TRIALS

In the last six months the results of Phase II and III clinical trials on three new substances have been made public by the companies involved:

#### Imatinib

On September 25 Novartis announced new data from the pivotal Phase III IMPRES clinical trial showing that the investigational therapy QTI571 (imatinib) improved exercise capacity in patients with PAH after 24 weeks compared with placebo. The data were presented for the first time at the European Respiratory Society (ERS) Annual Congress in Amsterdam, the Netherlands. QTI571 targets a possible underlying cause of PAH by counteracting uncontrolled growth of arterial smooth muscle cells. IMPRES was a 24-week randomized placebo-controlled, double-blind, multicenter clinical trial evaluating the efficacy and safety of oral QTI571 as an add-on therapy in the treatment of patients with PAH. The study involved a total of 202 patients with elevated PVR despite treatment with at least two other specific PAH medications (i.e. endothelin receptor antagonists, PDE-5 inhibitors and/or prostacyclins).

The IMPRES study met its primary endpoint by demonstrating a significant improvement in the six-minute walk distance (6MWD) test in patients with elevated pulmonary vascular resistance (PVR) despite treatment with two or more specific PAH vasodilator therapies. The study's secondary endpoints showed that QTI571 produced statistically significant improvements compared to placebo in pulmonary arterial pressure, cardiac output and pulmonary vascular resistance. However, the most important clinical secondary end-point, the time to clinical worsening (i.e. death, hospitalization due to PAH, worsening of functional class, or  $\geq 15\%$  drop in 6MWD) was not different between imatinib and placebo. In addition serious adverse events and discontinuations due to serious adverse events were more frequent with imatinib

than placebo. Longer-term data are required to clarify the safety-to-efficacy ratio of imatinib in PAH patients.

Source:

<http://www.novartis.com/newsroom/media-releases/en/2011/1549437.shtml>

#### Oral treprostinil

On August 24 United Therapeutics announced the completion of its FREDOOM-C(2) Phase III trial of treprostinil diethanolamine (oral treprostinil), an investigational sustained release oral formulation of treprostinil, a stable synthetic form of prostacyclin, in patients with PAH. The study population consisted of 310 patients who were optimized on an endothelin receptor antagonist, a PDE-5 inhibitor, or both. In addition to one of these oral drugs, patients were administered oral treprostinil or placebo twice daily with the dose titrated (increased to tolerability) over the 16-week trial. Preliminary analysis demonstrates that the trial did not achieve statistical significance for the primary endpoint, six-minute walk distance (6MWD) at Week 16. Preliminary analysis of other secondary efficacy measures, including time to clinical worsening, change in combined 6MWD and Borg Dyspnea Score rating (shortness of breath test) and Dyspnea-Fatigue index, functional class, and PAH signs and symptoms, did not differ significantly between oral treprostinil and placebo. Further review and analysis of the FREDOOM-C(2) preliminary results are ongoing.

Source:

<http://www.biospace.com/News/united-therapeutics-corporation-oral-lung-drug/231057/Source=TopBreaking>

#### Riociguat

Bayer HealthCare announced on May 18 the findings of a Phase II, proof of concept study investigating the benefits of riociguat in the treatment of PH owing to COPD.

Riociguat works through the signaling pathway of nitric oxide (NO) as well as the phosphodiesterase type 5 inhibitors (sildenafil and tadalafil). NO relaxes the musculature in the blood-vessel walls, lowers the pulmonary blood pressure and relieves the heart by modulating the activity of the sGC enzyme, which generates cyclic guanosine monophosphate (cGMP). Riociguat has a dual mode of action: it sensitizes sGC to the body's own NO while also directly stimulating sGC independently of NO. This is thought to be important because the NO levels in the pulmonary circulation are decreased in patients with PH. Phase III trials in PAH and chronic thromboembolic pulmonary hypertension (CTEPH) are currently underway. Bayer is currently planning further Phase II studies for PH-ILD and PH-COPD. For PH owing to left ventricular dysfunction (PH-LVD), a global Phase II study was initiated in 2010, and is ongoing.

Source:

<http://www.press.bayer.com/baynews/baynews.nsf/id/Phase-II-study-riociguat-Bayer-shows-promising-results-patients-pulmonary-hypertension-owing-COPD?Open&ccm=001>





## **February 29, Brussels, Belgium** **INTERNATIONAL RARE DISEASE DAY**

PHA Europe is organizing a new PAN-EUROPEAN "BREATHTAKING" AWARENESS CAMPAIGN on Pulmonary Hypertension around RDD 2012. The main launch event is planned on February 29 in Brussels and activities will be organized by PHA Europe affiliates in numerous other European countries.

## **May 5, Madrid, Spain** **WORLD PULMONARY HYPERTENSION DAY**

The WORLD PH DAY, organized by the Asociación Nacional de Hipertensión Pulmonar (Spain), has received the endorsement of the international PH community, key medical opinion leaders and institutions.



## **May 23-25, Brussels, Belgium** **EUROPEAN CONFERENCE ON RARE DISEASES AND ORPHAN PRODUCTS**

The EUROPEAN CONFERENCE ON RARE DISEASES & ORPHAN PRODUCTS is a unique platform/forum for rare diseases in Europe, bringing together all the key stakeholders. It is an annual event, providing the state-of-the-art of the rare disease environment, monitoring and benchmarking initiatives.

## **June 22-24, Orlando, Florida, USA** **PHA USA INTERNATIONAL CONFERENCE**

PHA's 10th anniversary INTERNATIONAL CONFERENCE will bring together almost 1,400 PH patients, caregivers and medical professionals for three days of education, support and networking.



## **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 SOCIETY OF RESPIRATORY MEDICINE'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.

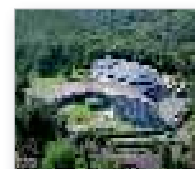


## **September 13-16, Barcelona, Spain** **PHA EUROPE GENERAL ANNUAL MEETING**

PHA Europe will be holding its 2012 GENERAL ANNUAL MEETING in Castelledefels (Barcelona) September 13-16 (the photo on the left is from 2011 meeting).

## **Fall 2012, Sophia Antipolis, France (date to be confirmed)** **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.



## **5th WORLD SYMPOSIUM ON PULMONARY HYPERTENSION** **Nice, February 27-28, - March 1 2013**

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 more cited in this scientific area.

## Interesting links and reading suggestions



### ... the EU institutions on Facebook!

A new, easier, “fun” way of keeping up-to-date on what is happening in the European Union political scene! The three main EU institutions - European Parliament, Council and Commission are now on Facebook. Look them up, click on “Like” and share with members of your national association to spread the info :-)

### European Parliament

The European Parliament appears to be the most popular of the three institutions on Facebook, with over 200,000 “likes” and 11,000 persons “talking about it”. The European Parliament is the only directly-elected EU body: members of the European Parliament (better known as “MEP”s) represent the citizens. The European Parliament is made up of 736 Members elected in the 27 Member States for a five-year period. The European Parliament is co-legislator for most of Europe's laws together with the Council (see below). These laws directly affect citizens and cover a wide range of topics including many which are of interest to PH patients: public health, consumer protection and research.

For more information see also: <http://www.europarl.europa.eu>

Find your country's MEPs at:

[www.europarl.europa.eu/members/public/geoSearch.do?language=EN](http://www.europarl.europa.eu/members/public/geoSearch.do?language=EN)



### The Council of the European Union

The Council of the European Union, sometimes just called the Council, is the institution in the legislature of the European Union representing the Member States, the other legislative body being the European Parliament. The Council is composed of 27 national ministers, one per state. The exact membership depends upon the topic; for example, when discussing agricultural policy the Council is formed by the twenty-seven national ministers whose portfolio includes this policy area. The Presidency of the Council rotates every six months between the governments of the 27 EU member states.

See also: <http://www.consilium.europa.eu/>

### European Commission

The European Commission is the EU's executive body and represents the interests of Europe as a whole (as opposed to the interests of individual countries). The term “Commission” refers to both the college of Commissioners and the institution itself. The Commission's headquarters is in Brussels but it also has offices known as “representations” in all EU member countries. The Commission prepares legislation for adoption by the Council - representing the member countries - and the Parliament -representing the citizens. It administers the budget and the policy programmes (Agriculture, Fisheries, Research etc.) in cooperation with authorities in the member countries. The EC also services the EU Commissioners. There are 27 EU Commissioners, one from each Member State. Each Commissioner is in charge of a specific sector of activity, eg. there is a Commissioner for Public Health, one for Competition, etc.

For general information see also: <http://ec.europa.eu/>

For the complete list of the 27 EU Commissioners 2010-2014: [http://ec.europa.eu/commission\\_2010-2014/index\\_en.htm](http://ec.europa.eu/commission_2010-2014/index_en.htm)



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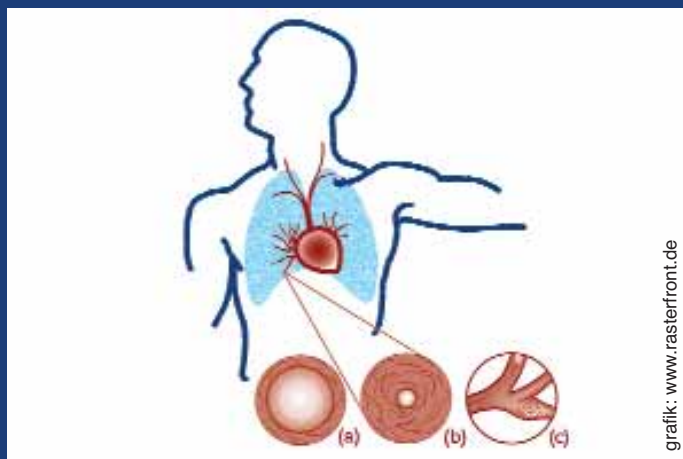


## AIMS OF PHA EUROPE

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.