



PULMONARY HYPERTENSION  
**Mariposa Journal**

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# Editor’s memo

## Winter 2012 edition

Dear members, supporters and friends,

it is my great pleasure to introduce the Winter 2012 edition of Mariposa, PHA Europe’s official journal. The year is soon coming to an end and I take this opportunity to summarize PHA Europe’s main achievements and to share some thoughts about the current and future role of PHA Europe.

I would like to start by thanking our member associations for all the hard work they are doing in their own countries, and for their active participation in the activities of PHA Europe. Their support has been a key element of the success of a number of important European events.

In February PHA Europe coordinated the 2012 edition of the “Breathtaking” campaign for Rare Disease Day, which was granted the patronage of EURORDIS, the European Organization for Rare Diseases. A launch event and press conference were held in Brussels and member associations from seventeen European countries organized very successful awareness raising national activities.

In June PHA Europe held the launch event of its PH Policy brief and Call to Action in the European Parliament in Brussels, hosted by MEP’s Hannes Swoboda (Chairman of the Socialist group of the EP) and Karin Kadenbach (EP Heart Group). Guest speakers included Dr. Paola Testori-Coggi, Director General of EU Commission DG SANCO (Health and Consumers) and Dr. Laurent Nicod, Scientific Committee Chair at the EUROPEAN RESPIRATORY SOCIETY as well as prestigious representatives of the international scientific community. The PH policy brief was the result of almost a year’s work of a roundtable of experts representing different stakeholders in PH, where the members provided significant input.

The increasing engagement of our member associations was also demonstrated by the very high attendance at the PHA Europe General Annual Meeting which took place in September 2012. It is gratifying to see how our community has gradually built up over the years. In 2012 alone three new countries have come on board and we now have a very broad and highly representative membership, with 28 patient associations from 24 countries.

The General Annual Meeting provided the opportunity to present the results of another important project which saw the active collaboration of a number of member associations across Europe: the International PAH Patient and Carer survey. The data resulting from the survey reinforced the arguments put forward in the PH Policy brief and Call to action with respect to the need for an integrated and multidisciplinary care for PAH patients that includes

the emotional, social and practical impact of the disease as well as the unmet information needs.

In the course of the year it has been very rewarding to see that PHA Europe is increasingly recognized as a key stakeholder in the field of Pulmonary Hypertension in Europe. A notable expression of it’s stature in the international scientific community was the request made of PHA Europe that it endorse the 4th World PH Symposium in Nice in 2013. It was also an honour to be invited to present the patient perspective at the EUROPEAN SOCIETY OF CARDIOLOGY’s annual Educational Course on PH during the G6, the annual PH stakeholder meeting, where I chaired one of the sessions. PHA Europe representatives were asked to speak at a number of other scientific events including the 2nd annual Drug Congress, the 7th PAH Bayer Symposium, the 6th PAH Bayer Nurse workshop and Actelion’s 9th Deutsches PH Forum. Networking and exchanges with other international PAH patient associations were also very lively during the year, and PHA Europe representatives were invited to attend and speak at the PHA’s International Leaders Summit in Orlando (USA), the PH Latin Day in San José (Costarica) and the Annual PH Patient Day in Budapest (Hungary).

Further, in 2012 PHA Europe took part in the work of a number of task forces (EUROPEAN RESPIRATORY SOCIETY task force on rare lung diseases) and advisory committees (EUROPEAN LUNG FOUNDATION patient advisory committee). Also, thanks to the productive ongoing collaboration with EURORDIS, in 2012 a PHA Europe representative joined that organization’s Drug Information Transparency and Access (DITA) task force.

As in past years, PHA Europe participated as an exhibitor in the annual Congresses of the EUROPEAN SOCIETY OF CARDIOLOGY (Munich, August 25-29, 2012) and EUROPEAN RESPIRATORY SOCIETY (Vienna, September 1-5, 2012). These congresses are key events which are consistently attended by thousands of healthcare professionals, industry and other interested parties. Our presence at these occasions enables us to distribute materials and present PHA Europe’s activities, to have access to the most up to date information on research and treatments and expand our contacts, an essential ingredient of networking, while also serving to raise awareness of PH among top medical and industry professionals. We were heartened in this respect by the large number of symposia, posters, abstract sessions, tutorials and other sessions specifically dedicated to Pulmonary Hypertension at the congresses of both the cardiology and respiratory societies.

This past year was extremely positive also with respect to research on new treatments. The recently announced results of Phase III trials on Macintentan and Riociguat may lead to new and innovative treatments, possibly in the foreseeable future; other promising drugs are also being investigated at this time. We look with great interest and hope at the work of the international scientific PH community and in particular at the recommendations which will emerge from the 4th World PH Symposium in Nice in February 2013.

PHA Europe has identified our central projects for the coming year. The following offers a brief overview:

- Community building and capacity building in national associations:** Regular communication and exchange between PHA Europe and its members is vital to heighten awareness and engagement. Language barriers can result in delays in the association’s activities. In order to build further capacity among member associations where knowledge of English is limited, we have instituted a Fellowship program which will be officially launched in January 2013. Thanks to this program member associations will benefit from an English-speaking part-time assistant who will act as the national association’s liaison person with PHA Europe and assist with the national association’s day to day work. We have just concluded accepting the applications for these positions, and look forward to begin implementation of this program shortly.
- PHA Europe will also continue to invest resources in its **White Spots program**, which aims to establish contacts and encourage patients, caregivers or physicians in the countries where there are no associations, to create one. We seek to offer assistance in setting up, providing institutional and legal support concerning statutes and registration of new associations, basic start up informational tools (website, Facebook) and educational materials and resources. Expanding the new association’s contacts and organizational activities will be achieved also through exchanges with representatives of established associations, and the participation of leaders of these new associations in PHA Europe’s General Annual Meeting.
- World PH Day, a new dimension of awareness:** PHA Europe plans to build on the great success of the first World PH Day, organized in 2012 by the Spanish PH association ANHP, to concentrate its annual awareness campaign around this date (May 5). The advantage with respect to holding activities on Rare Disease Day (as in the past two years) is that this day would be specific to PH and therefore potentially be able to attract more attention and media interest. The World PH Day has received a very wide consensus from PH associations around the world and events are expected to take place in all continents. A PH World Day website is currently being set up which will allow all the participants to present their projects, share results and disseminate information about the initiative. An international committee will be in charge of coordination.
- Communications and Information Technology (IT):** PHA Europe believes that IT and social media can play a vital role in awareness raising and in the course of the coming year we will be working on the unification of websites and facebook pages for our members in order to enhance the role of social media and be able to effectively conduct joint online campaigns.

- Advocacy and lobbying:** These activities will also be an important part in PHA Europe’s program during the coming year. Follow up action relating to the PH Policy brief and Call to action will be continued at the EU and national levels. Also, we will be closely monitoring any health and/or research related EU initiatives to identify opportunities to provide input and possibilities for participation.
- Implementation of the Patient and Carer survey:** The findings of this survey describe important implications concerning quality of life issues for patients, their families, clinicians and communities. PHA Europe will undertake close coordination with the national associations to optimize the distribution of survey materials to patients, carers and health care professionals as well as to implement its Calls to Action.
- 10th Anniversary:** we are very happy to celebrate our tenth anniversary next year (PHA Europe was founded in Vienna in 2003) and plan to organize an event during our General Annual Meeting.

PHA Europe looks forward to working with its members associations in successfully conducting this ambitious and vital program of activities during the upcoming year. I take the opportunity to thank our industry partners for their trust and generous support of our activities, which has allowed us to build up our association and to come this far. We trust in their renewed support and look forward to a long and mutually beneficial future collaboration. Many thanks again to our members.

Best wishes for a very Happy New Year

Gerald Fischer  
PHA Europe President

CONTENTS	
• PHA Europe activities in 2012	p. 04
• General Annual Meeting	p. 06
• Conferences and events June-December	p. 12
• PH Latin Day	p. 14
• News from European PH Associations: <i>Austria, Belgium, Bulgaria-PHA Bulgaria, Bulgaria-BSPPP, Czech Republic, France, Germany, Greece, Hungary, Israel, Italy-AIPI, Italy-AMIP, Latvia, Netherlands, Norway, Poland, Slovakia, Slovenia, Spain, Sweden, Switzerland-SPHV</i>	p. 16
• News from PH Associations around the world: <i>South Africa, USA</i>	p. 38
• International survey reveals the hidden but significant impact of PAH on patients and carers	p. 40
• Update on PH treatments and research: <i>Macintentan and Riociguat trials</i>	p. 43
• News from European institutions and NGO’s	p. 44
• Upcoming events in 2013	p. 47
• Interesting links and reading suggestions	p. 48
• Members of PHA Europe and contact details	p. 49



FEBRUARY

**Brussels, February 29**  
**“BREATH TAKING” CAMPAIGN FOR RARE DISEASE DAY** PHAE EVENT

PHA Europe’s Breathtaking campaign featured a launch event and press conference in Brussels and successful awareness raising activities in seventeen other European countries. The campaign received the patronage of EURORDIS, the EUROPEAN ORGANIZATION FOR RARE DISEASES.



MARCH

**March 3-4**  
**7th BAYER SYMPOSIUM ON PH**

Prof. Nazzareno Galiè (University of Bologna) and PHA Europe’s Vice President Pisana Ferrari enacted a “A PH patient and doctor dialogue” at the 7th edition of the Bayer PH Symposium. Over 300 medical professionals from around the world attended this meeting.



MAY

**Madrid, May 5**  
**1st PH WORLD DAY**

PHA Europe’s President Gerald Fischer was invited to attend and introduce part of the programme of the first World PH Day, initiated and organized by the Spanish PH association ANHP. The event, which received the endorsement of innumerable organizations worldwide, had an outstanding scientific programme and a very prestigious panel of speakers.



JUNE

**Brussels, June 6** PHAE EVENT  
**CALL TO ACTION LAUNCH AT EUROPEAN PARLIAMENT**

PHA Europe held the launch of its PH Policy brief and Call to Action in the European Parliament (EP) in Brussels during a very successful event hosted by MEP’s Hannes Swoboda (Chairman of the Socialist group of the EP) and Karin Kadenbach (EP Heart Group).



**Barcelona, June 8-9**  
**3rd BAYER INTERNATIONAL NURSE WORKSHOP**

PHA Europe’s Vice President Pisana Ferrari was invited to speak on the topic “Partnering with Nurses: the PAH experience” at this important meeting which brought together around 160 nurses from 20 countries worldwide.



**Orlando, June 22-25**  
**INTERNATIONAL LEADERS SUMMIT**

PHA Europe Board Member Juan Fuertes represented PHA Europe at the International Leaders Summit held during the 10th International conference organized by PHA. Over 1.500 patients, caregivers and medical professionals attended this important PH gathering.



**Berlin, June 15-16**  
**9th ACTELION DEUTSCHES P(A)H FORUM**

PHA Europe’s Vice President, Pisana Ferrari, and Guenther Thimm of the German PH Association ph e.v. were invited to speak at this scientific conference, organized for the 10 year anniversary of oral therapy for PAH, attended by over 200 medical professionals.



JULY

**London, July 6**  
**COMMUNIQUE AWARDS FOR “BEST PATIENT INITIATIVE”**

PHA Europe’s 2011 “Breathtaking” campaign, designed by the UK communications agency Ketchum Pleon, won 1st prize at the “Communiqué Awards” for “Best Patient Initiative” 2012. PHA Europe President Gerald Fischer attended the awards ceremony in London (second from right in photo)



AUGUST

**Munich, August 25-29**  
**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, and Marzia Predieri from the Italian PH Association AIPI.



SEPTEMBER

**Vienna, September 1-5**  
**EUROPEAN RESPIRATORY SOCIETY ANNUAL CONGRESS**

PHA Europe was present with a booth at the ERS Annual Congress and was represented by PHA Europe’s President Gerald Fischer, Vice President Pisana Ferrari, Board Member Juan Fuertes and Marzia Predieri from the Italian PH patient association AIPI.



**Vienna, September 1-5**  
**EUROPEAN RESPIRATORY SOCIETY and EUROPEAN LUNG FOUNDATION MEETINGS**

During the ERS annual congress PHA Europe Vice President Pisana Ferrari took part in the meeting of the ERS Task Force “GP’s meet rare lung disorders”, and Board Member Juan Fuertes took part in the meeting of the Patient Advisory Committee of the European Lung Foundation.

**Barcelona, September 12-16** PHAE EVENT  
**PHA EUROPE GENERAL ANNUAL MEETING**

Thirty-nine PH Patient Leaders from twenty countries participated in PHA Europe’s 2012 General Annual Meeting in Barcelona. The agenda included medical updates from distinguished members of the international scientific community and presentations from experts in advocacy, communication, market research, social media and online disease communities.



OCTOBER

**Sophia Antipolis, October 14-15**  
**EUROPEAN SOCIETY OF CARDIOLOGY EDUCATIONAL COURSE ON PH**

PHA Europe’s President Gerald Fischer was invited to speak about “The expectations of PH patients” at the G6, the PH stakeholder meeting which is held during the annual ESC educational course on PH. Over a hundred medical professionals from around the world attended the course.



**Barcelona, October 18**  
**2nd ANNUAL ORPHAN DRUG CONGRESS**

PHA Europe Board Member Juan Fuertes was invited to attend and address the audience on patients’ views on pricing of orphan drugs and on the importance of involving patient groups in early stage of orphan drug development.



NOVEMBER

**Budapest, November 17**  
**HUNGARIAN PATIENT ASSOCIATION’S ANNUAL MEETING**

PHA Europe’s President Gerald Fischer was invited to attend the annual meeting of the Hungarian PH patient association and speak about the activities of PHA Europe. The meeting was attended by over 140 patients and caregivers, PH physicians and other stakeholders.



**Lugano, November 24**  
**SWISS PATIENT ASSOCIATION MEETING**

PHA Europe Vice President Pisana Ferrari was invited to speak about her experience with PH and the role of patient associations at a meeting of the Swiss Italian PH patients support group.

**San José, Costa Rica, November 28**  
**PH LATIN DAY**

PHA Europe Board Member Juan Fuertes represented PHA Europe at the PH Latin Day, an initiative of the Latin PH Society that takes place each year on November 28. The celebrations were divided in two parts: a PH Leaders Summit for the patients and a Medical Symposium addressed to medical professionals.



DECEMBER

**Vienna, December 12-13** PHAE EVENT  
**PHA EUROPE BOARD AND TASK FORCE MEETING**

PHA Europe Board Members met in Vienna on December 12-13 to discuss future action and strategies for the coming year and the setting up of dedicated task forces which will work on specific projects.





# General Annual Meeting

Barcelona, September 12-16, 2012



The General Annual Meeting (GAM) represents one of PHA Europe's central activities. It provides PH patient association leaders with the opportunity for sharing information and experiences, mutual learning, networking, education, and skills development. The GAM also serves as a platform to showcase European and national initiatives and activities and discuss future strategies and projects. This year's meeting took place from September 12 to 16 in Castelldefels, near Barcelona (Spain). Thirty-nine PH patient association leaders participated, representing twenty countries across - and even outside - Europe: Austria, Belgium, Bulgaria, Czech Republic, Finland, France, Germany, Hungary, Ireland, Israel, Italy, Latvia, Norway, Netherlands, Poland, Slovenia, Slovakia, Spain, Sweden, Switzerland. Nearly sixty persons - patient representatives, speakers and guests - participated in this year's GAM.

## A VERY SUCCESSFUL YEAR 2012

The GAM opened with the institutional part of the program, the General Annual Assembly. During this session President Gerald Fischer presented the Annual Report of activities and the 2012 accounts. He described 2012 as being a very intense one, with many successful initiatives and projects accomplished. These include the pan-European "Breath-taking" awareness campaign for Rare Disease Day, the launch of the PH Policy brief and Call to Action in the European Parliament, the first World PH Day, the completion of the PAH Patient and Carer Survey, the participation of PHA Europe representatives as speakers at major European scientific symposia and industry events - key developments that significantly expanded PHA Europe's outreach. The President's report indicated that PHA Europe is increasingly being considered as a key stakeholder in European policy, medical and industry

forums. The President concluded this part of the meeting by thanking the representatives of the national associations for their hard work and enthusiastic collaboration, without which none of PHA Europe's achievements would have been possible.

## PHA EUROPE MEMBERSHIP

Three requests for membership were reviewed and formally accepted during the General Assembly: we were pleased to welcome the Bulgarian Society for Patients with Pulmonary Hypertension (BSPPH), PHA Finland, and PHA Ireland. PHA Europe is proud of its increasingly broad and highly representative membership which now stands at twenty-eight patient associations from twenty-four countries. Despite this considerable achievement we are mindful that many countries in Europe still have no patient association and it is for this

reason that our "White spots" program will continue with the aim of ensuring that all patients in Europe will one day benefit from the invaluable support of such groups.

## PHA EUROPE'S "HORIZON 2013"

In order to encourage conference participants to take a more active part in the sessions it was decided at this year's GAM to change the physical set up of the meeting room. In past years the sessions had been conducted with tables organized in a U-shape. This year the participants were assigned to smaller groups in round tables, where it would be easier to have a discussion. Each table was equipped with flip charts and a spokesperson was designated to report back to the general discussion. Seating assignments were changed every day to allow everyone to get a chance to meet everybody else. Key discussion points were determined in advance of all the sessions to guide ensuing conversations. One particularly lively session was devoted to the role of PHA Europe, its key challenges and future priorities for action. Issues raised and recommendations aired included the importance of reasonable pricing and access to drugs, which must remain a key priority for any future action. It was agreed that PHA Europe organize workshops on advocacy, lobbying and media relations, geared to activists in the national associations, with the aim of empowering them in these skills. A consensus was reached that particular attention, support and guidance should be given to the smaller associations, including funding and resource provision for start ups. Similar consensus was reached concerning the organization and promotion of educational programmes at national level for such target groups as general medical practitioners, other clinicians, nurses and medical students. It was suggested that despite the existence of scientific society guidelines (European Society of Cardiology/European Respiratory Society) much remains to be done about ensuring their application in real life situations and PHA Europe may have a role to play in this as well. Other, more practical, suggestions put forward included the creation of a European PH passport and setting up of a secure website for sharing information between associations. These ideas emerged during a very useful and constructive first session. The patient leaders gave a favourable opinion of the work carried out to date by PHA Europe and expressed their expectations for the future, a sure sign of the success of this session and their increasing engagement.

## "MEET THE EUROPEAN PH LEADERS"

PHA Europe has traditionally invited its pharmaceutical industry partners to speak at the annual meetings. During the 2012 GAM a one day session, called "Meet the European PH Leaders" was scheduled, during which the national patient associations were given the opportunity to present their activities and current and future issues related to PH management in their countries. We were delighted to have as our guests for this session representatives of Actelion, Bayer, Glaxo-SmithKline, Pfizer and United Therapeutics (with its Spanish distributor Ferrer). In the course of the (twenty) national presentations and discussions that followed, what emerged very



Denis Costello from Eurordis, the European Organization for Rare Diseases



Gerald Fischer, PHA Europe President



Prof. Simon Gibbs



Christine Marking



Prof. Horst Olschewski



Middle row, from left: Luisa Sciacca della Scala, Franz Fischer, Hall Skaara, Patrick Hassel, Marion Blouin, Ferdinand Bolsius, Regina Prenderville, Vania Toteva, Tuulia Naepi, Leny van der Steen, Agnieszka Bartosiewicz, Barbara Salobir, Marzia Predieri, Natalia Maeva, Klara Klanar, Marta Tomczyk, Jana Guranova, Yveta Makovnikova, Dominika Tepla, Ieva Plume, Eszter Csabuda, Mélanie Gallant Dewavrin, Irene Delgado, Juan Fuertes, Melinda Lackovski, Hilde Thimm, Lyubomir Dimitrov. Front row: Mihaela Raykova, Vittorio Vivenzio, Gerald Fischer, Pisana Ferrari. Back row, from left: Patrick Fischer, Anna Galvina, Luc Matthysen, Guenther Thimm, Veronika Jelinkova



From left: Juan Fuertes, Irene Delgado, Jutta Ulbrich, Yveta Makovnikova, Jana Guranova, Gerald Fischer, Klara Klanar, Barbara Salobir, Ieva Plume, Anna Galvina, Agnieszka Bartosiewicz



clearly is that the situation is extremely heterogeneous across Europe, with many countries, for example several in Eastern Europe, experiencing severe difficulties with availability and access to drugs, reimbursement, lack of expert centres and surgical facilities (which are needed for pulmonary endarterectomy and lung transplantation) and of psycho-social support. In some countries, including those where PH care is better organized, reimbursement for drugs and social support is dependant on functional class (FC III-IV only) and/or not all EU approved drugs (ERAs, PDE-5 inhibitors or prostanoids) are available. Reimbursement of triple combination therapy is also increasingly becoming an issue. The member associations play a vital role in advocating for the rights of patients and have been extremely active in fighting for optimal treatment for their members, but are now contending also with the challenge represented by health budget cuts resulting from the general economic crisis in Europe. Much remains to be done in the fight against health inequalities in Europe and this will require empowerment of the patient associations to stand up for the rights of patients nationally, and a concerted European strategy for advocacy and lobbying.

#### MEDICAL UPDATES

Education has always been a very important part of the GAM. It is vital for the patient leaders to have the most up to date information on novel treatment strategies and ongoing and future research. Over the past years a number of prestigious members of the international scientific community have honoured us with their presence, including Prof. Nazzareno Galiè (University of Bologna, Italy), Prof. Jean-Luc Vachiéry (Erasme-ULB, Belgium) and Prof. Andrea Olschewski (Ludwig Boltzmann Institute, Austria). This year it was a great privilege to have as our distinguished guests Prof. Simon Gibbs (Imperial College, UK), and Prof. Horst Olschewski (University of Graz, Austria). Prof. Gibbs made an invaluable contribution to the drafting of the PHA Europe Call to Action and played a leading role at the European Parliament launch event in June 2012, where he addressed the audience on the importance of expert centres for the optimal treatment of PH (see also Mariposa n.8/2012). Prof. Gibbs revisited this very important issue during the first part of his presentation at the 2012 GAM. What is expected of designated PH centres? Prof. Gibbs related that these centres should be able to provide accurate and complete diagnosis, an

individualised plan of clinical management, prescription and initiation of disease-targeted therapy for PAH, follow-up and adjustment of therapy as appropriate. These centres should adhere to established standards of care and guidelines, subject themselves to audit and exhibit accountability. They should participate in clinical trials and educational programs and provide lifestyle counselling and psycho-social support. Prof. Gibbs referred to data from a recent survey carried out by PHA UK which shows that PH patient satisfaction in the UK is highest in designated centres and this is where the future of PH care lies. The UK system is well established and successful, which unfortunately is not the case in many European countries. The establishment and designation of such centres should be replicated elsewhere.

The second topic of Simon Gibbs' talk was future clinical trials and their challenges. Traditionally the six minute walking distance (6MWD) criterion has been used as the main primary endpoint to indicate reduction in disease status. It has been accepted by regulatory authorities for evaluating PAH treatment effectiveness and multiple therapies have been approved using the 6MWD. Over the years, with the increase in knowledge of PAH and the availability of more data about clinical trials (including metanalysis), the limitations of the 6MWD have become more apparent, especially regarding its correlation with outcome. It is increasingly recognized that a composite clinical outcome endpoint that includes morbidity/mortality should in future be used as the primary endpoint in phase III trials. The prospective endpoint should include all-time mortality, non-elective hospitalizations, clinical worsening (increase in symptoms, decrease in exercise capacity, need for more drugs) and failure to improve. A recommendation about future endpoints is expected to result from the proceedings of the 4th PH World Symposium being held in Nice in February 2013. The third part of Prof. Gibbs' talk concerned research. He briefly described the new drugs currently in the pipeline: Macintentan, Riociguat, oral Treprostinil and Sildenafil. These are all in different stages of development and data about the first two have recently been disclosed (see page 43). Later in the conference Dr. Horst Olschewski, Professor of Medicine and Director of the Department of Pulmonology at the Medical University of Graz (Austria), updated the participants on the research work currently being conducted at the Ludwig Boltzmann Institute (Austria), where he serves as Director. Among the subjects he discussed were non-invasive



PHA Europe Board, from left: Juan Fuertes, Hall Skaara, Mélanie Gallant Dewavrin, Gerald Fischer, Pisana Ferrari, Luc Matthysen and Luisa Sciacca della Scala



Hans-Dieter Kulla leading one of the workshops



ways to measure cardiac output and to diagnose PH, highly significant developments in the care of PH. Right heart catheterisation is still today considered the golden standard for an accurate diagnosis of PH and is also widely used both in establishing patients' baseline evaluation and in follow-up, in particular when making decisions about treatment changes or escalation. Right heart catheterization is an invasive procedure which poses some risks, especially in children, who have to undergo the procedure under general anesthetics. Accordingly, any developments which could lead to a non-invasive means to assess hemodynamic parameters would represent a major breakthrough. Research that is ongoing at the Ludwig Boltzmann Institute appears to have great potential and may open up new and very interesting possibilities in the not too distant future. The diagnostic tools described by Dr. Olschewski included measuring cardiac output through inert gas rebreathing, gauged by a small device that is cost effective, user-friendly and provides fast results. The procedure is very promising but requires further investigation as it is very sensitive to breathing manoeuvres. Another technique under investigation at the Ludwig Boltzmann Institute is cardiac output assessment with dynamic contrast-enhanced computerized tomography (CT). The correlation of this instrument with right heart catheterization is still being perfected and researchers are optimistic about its potential. This CT technique is also sensitive to small increases in the pulmonary mean pressure. Finally, 3D magnetic resonance imaging (MRI) with contrast has shown promise to detect PH and is currently being evaluated.

#### ADVOCACY AND LOBBYING TRAINING

Skills development is another important focus of the GAM. This year the agenda included a session on "Effective advocacy at European and national levels" lead by Christine Marking, a respected EU public affairs consultant based in Brussels. The session was designed to motivate and empower members in

advocating issues of importance to persons living with PH, for example, in communicating the key messages in PHA Europe's PH Policy Brief and Call to Action (launched in June 2012 in the European Parliament) and in the PAH International Patient and Carer Survey (see below). Christine Marking began her presentation by providing an overview of the main EU institutions and their roles in the EU decision making process. She explained that the EU has a limited mandate in the health area. Nonetheless, the number of EU level health activities is on the increase. There is a growing recognition of the need for Member States to cooperate, exchange experience, and learn from one another with respect to health care. The EU uses "soft" influence on Member States to direct national policies. In a growing number of fields, the Commission issues guidelines and recommendations for national action plans and centres of excellence and puts in place formal policy exchange concerning such matters as organ donation, rare diseases, health inequalities, patient safety. Research is also an area where the EU has been very active and has allocated considerable funding. Seen in this light advocacy at EU level is highly relevant, in parallel with national level.

The second part of Christine Marking's presentation emphasized practical advice and concrete examples that were insightful even for those of our members who have not previously had experience in this area. She stressed the importance of defining clear and realistic goals, assessing existing realities and identifying the key players. She described how defining action steps and a timeline can be used effectively in advocacy campaigns.

#### PAH PATIENT AND CARER SURVEY

The International PAH patient and carer survey was conducted across five European countries from 2010 to 2012 (France, Germany, Italy, Spain and the UK) and involved 455 patients and caregivers. PHA Europe was honoured to be part



From left: Mélanie Gallant Dewavrin, Hilde Thimm and Barbara Salobir



Mihaela Raykova



From left: Yosef Gotlieb and Luc Matthysen



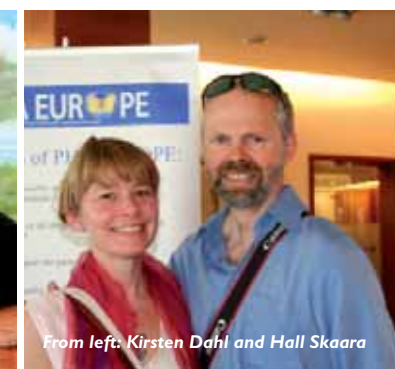
Ferdinand Bolius



From left: Juan Fuertes and Irene Delgado



From left: Luisa Sciacca della Scala and Vittorio Vianzo



From left: Kirsten Dahl and Hall Skaara



From left: Klara Klančar and Melinda Lackovski



of the Steering Committee along with other patient representatives (PHA and PHA UK) and distinguished members of the medical and nursing professions. The patient and carer survey was completed earlier in the year and the findings were condensed in a report which was officially presented to the members at a dedicated session of the GAM.

We were very glad to welcome to the session representatives of the market research and communications agencies who played a key role in the conduction of the survey: Jane Barrett, from Insight Research UK, Anna Gibbins, from Packer Forbes Communications UK, and Gurmit Sandhu, a Swiss-based consultant. Jane and I shared the task of presenting the survey report and key findings (see also article on page 40). Anna Gibbins then gave a very interesting presentation about how to promote the survey findings and the Call to action, based on these findings, to the media. She also distributed a very comprehensive and useful media tool kit. Gurmit Sandhu facilitated four workshops during which the draft output materials from the survey were reviewed by the members. These materials will be amended to incorporate the comments made and distributed later on in the year to be used as part of the follow up action. Needless to say the survey generated great interest and it is expected that many associations will organize local launch events to promote the survey findings and Call to action.

#### FRENCH SURVEY

Mélanie Gallant Dewavrin presented the results of a very interesting survey conducted by the French PH Patient association HTAP France in 2010-2011 involving 372 patients. The aim of the survey was to better understand the main issues involved in everyday life with the disease and to identify the key unmet needs on which the association should define priorities for future action.

#### THE FUTURE ROLE OF SOCIAL MEDIA

Social media play an increasingly important role in disseminating information and raising awareness. With continuing advances in information technology their future potential may prove even greater. PHA Europe has always recognized this as being a strategic and important area and it is high on its list of priorities. During the GAM one-to-one sessions were held between the PH Leaders and Patrick Fischer, of Limeforce Social Media, who is an expert in the field. Patrick conducted a general session involving an engaging presentation on PHA

Europe's online platform for PHA Europe, which will cross-link the Facebook pages of its constituent associations. Linked sites would not only give the patient organizations a more professional look but it would also allow them to drive more traffic towards announcements of recent or upcoming events. Using this network news would be updated automatically on all national sites, allowing them to focus on country-specific content and at the same time making this content (posts, video, audio) available to the entire PH community. This would spur the growth of the online community and therefore enhance social media "marketing" of PH. The idea behind the unified Facebook pages is to have a clearly recognizable identity ("brand"), a key objective of social marketing. Most associations have a Facebook page or group, Patrick will be working with them over the next few months to bring them into the unified network, with the goal of implementing a joint online campaign for next World PH Day, May 5, 2013.

#### RARE DISEASE ONLINE COMMUNITIES

We were very pleased to host Denis Costello and Robert Pleticha who manage the online rare disease community "RareConnect", a joint project of the European and US organizations for rare diseases, EURORDIS and NORD, and presented this important communication resource. The aim of RareConnect is to promote conversation and connections between patients, families, and caregivers as a means to improve the lives of people with rare diseases. RareConnect currently has 33 rare disease online communities, including the pulmonary hypertension community, which was launched in March 2012.

The PH community has over 40 international associations subscribed that share videos, patient stories, event updates and links, as well as a discussion forum which applies human translation service in five languages. It is still however underused with respect to its great potential and it would be very important for all the member associations to promote it at national level. In his presentation Robert Pleticha spoke about how an effective use of RareConnect can complement social media strategy. He encouraged the members to share the link to RareConnect on their websites and social media and to join in the conversation on the forum by posting blogs and stories. RareConnect is continuously improving its services and plans to provide users with free full text access to articles in selected scientific journals, a unique educational opportunity.



From left: Franz Fischer and Jana Guranova



From left: Phana Ferrari, Jana Guranova, Yveta Makovnikova, Veronika Jellinkova and Dominika Tepla



From left: Regina Prenderville, Marion Blouin and Guenther Thimm

#### BREATHTAKING 2012 AND PH WORLD DAY 2013

The 2012 "Breathtaking" PH awareness campaign coinciding with Rare Disease Day was a great success in all of the fifteen countries which organized dedicated and unique events. Two additional associations participated in events organized by other associations in their country.

The first "World PH Day", initiated and organized by the Spanish PH Association (ANHP), was held in Madrid on May 5, 2012. World PH Day received the endorsement of PH associations and other organizations worldwide and, given the notable success of the event, PHA Europe has decided to launch its 2013 PH awareness campaigns on May 5, World PH Day, instead of Rare Disease Day, since the former serves to spotlight PH specifically rather than it be one of many rare diseases. A dedicated day would have a higher potential to attract the attention of the general public and generate media interest. As part of the campaign, PHA Europe would coordinate activities in the different European countries, including press conferences, events, ads, billboards, involving celebrities, effective online campaigns and much more. Events could be organized across several continents with the possibility of setting up an international coordinating committee and website.

#### AWARDS CEREMONY

One of the highlights of the GAM was the welcome dinner and awards ceremony. PHA Europe President Gerald Fischer thanked all the member associations for their dedication in implementing the 2012 "Breathtaking" awareness campaign and announced the winners of the Best Campaign for 2012. The first prize (3.000 €) for the best and most cost-effective campaign was awarded to the Slovenian PH association, a very "young" and dynamic association (founded in 2011) which succeeded in generating great media interest (TV, radio and press) around its activities, and in involving some of the country's main celebrities. The second prize (2.000 €) was given to the Latvian association and the third prize (1.000 €) to the Slovakian association, both of which are very new (PHA Latvia was founded in 2011, PHA Slovakia in 2010) and conducted extremely successful campaigns. The Spanish PH association ANHP received a Special Award for its outstanding contribution to the international PH cause through the initiation and organization of the First World PH Day on May 5, 2012.

#### SOCIAL PROGRAM

On the afternoon of the final day we travelled by bus to Montserrat, Spain's first National Park, a renowned religious and art centre, set high in the mountains amid a spectacular

natural scenery and well known for its Benedictine abbey, Santa Maria de Montserrat. It is the site of the Virgin of Montserrat sanctuary, identified by some as the location of the Holy Grail in Arthurian myth. "Montserrat", "jagged mountain" in Catalan, is prominent by its peculiar rock formations which are visible from a great distance. After the visit we drove to Barcelona where we enjoyed a memorable farewell dinner in wonderful a restaurant overlooking the city.

#### CONCLUSIONS

This year, for the first time, evaluation forms were distributed to all the participants at the end of the meeting. It was very rewarding to see the high level of appreciation not only for the speakers and topics covered but also for the organizational aspects. It was clear that the GAM is a key driver for patient-oriented activity and engagement and plays a crucial role in inspiring and motivating those in attendance. Warmest thanks again to all our members for helping us to make this meeting such a great success!

Pisana Ferrari

Following the meeting some very touching comments and expressions of gratitude from the participants were posted on our internal discussion forum, PH Discuss. For reasons of space it would be difficult to quote them all, so we have chosen to publish one which, in our view, sums up perfectly what this meeting has come to mean for us all.

*"The conference experience has remained close to me since I left. We have built a community that I am proud to be part of, and I value the ties between us. Between the lectures by top medical specialists, healthcare professionals and organizers and our deliberations, I was able to catch up with the lives of people I have come to know from our previous meetings and who have become dear friends. Over meals or during outings, I also encountered new members of our association who either as patients, family members, or advocates have made common cause to improve the lives of PH sufferers and dare to dream of a cure for the disease, which I know from my own bout with it can take your very breath away. It has also killed people I have known and cared about. Although we come from many different traditions and lands and sometimes struggle to overcome the challenges posed by the assorted languages we speak, those who gather for the European PH meeting are dedicated to each other and to improving the lives of patients and their families. Each of us are involved in maintaining PH associations in our countries and the decisions we make and the information we receive at the international gathering spread out over a widening circle to ameliorate the lives of those who have been affected by the disease. This congregation of caring and giving spreads hope and goodness. Aside from the impact we hope in alleviating the lives of others who live with the disease, our efforts extend even further. It is an example of what I imagine the prophets of light of all faiths and convictions had in mind in hoping for the betterment of humanity. It resonates. I would say, something like peace. Let's go from strength to strength during the coming year and make serious inroads in creating a PH-free world."*

Yosef Gotlieb, PH Israel



From left: Tuulia Naeppi, Patrik Hassel and Marzia Predieri



From left: Hans-Dieter Kulla and Eszter Csabuda



From left: Anna Galvina and Ieva Pume



Lyubomir Dimitrov, Agnieszka Bartosiewicz and Natalia Maeva



## Conferences and events June-December



### LAUNCH OF CTEPH ASSOCIATION, NICE ESC EDUCATIONAL COURSE AND G6, SOPHIA ANTIPOLIS

Sophia Antipolis was another big step for PHA Europe to become not only an important member of the G6 (annual stakeholder meeting), but also a real part of the PH scientific community and family. It was already nice to fly to Nice together with Prof. Irene Lang and to have all this time to talk about the CTEPH meeting, which preceded the G6. This meeting was the official kick off of the CTEPH association, started by Prof. Gérald Simmoneau, Prof. Irene Lang and Prof. Marion Delcroix. Among the participants there were representatives of the industry, patients and doctors, including specialist surgeons Eckhard Mayer from Germany and Nick Kim from San

Diego. The presentation of the association's work and its business plan was done very professionally by Rita Locher and I wish a big success for the new association.

Our hotel was in Juan les Pins, which was very emotional for me. When I was 19 years old, I drove my VW beetle from Vienna to Juan les Pins to the famous jazz festival with Ray Charles & Ella Fitzgerald and after the concert my friend and I could listen to them very privately in the bar from Hotel Eden Roc until sunrise. The very charming small village of Juan les Pins did not change since then and some of the bars and the famous discos are still there.

The ESC educational course and G6 in Sophia Antipolis had a very high standard, with extremely interesting presentations from specialists around the world. The PHA Europe presentation had a big success and we could come another step closer to specialists, nurses and regulatory authorities. It was a very big honour for me to be asked to chair, together with Prof. Gérald Simmoneau, the morning session on the Saturday (G2 - Gérald & Gerald!). He let me introduce the speakers and ask the first questions. I am not a doctor, but felt flattered by receiving 12 external points :-)



### EUROPEAN CONFERENCE ON HEALTH INEQUALITIES IN THE NEW EU MEMBER STATES

On September the 20th, I was invited to attend the FIRST CONFERENCE ON HEALTH INEQUALITIES in the new EU member States. This was an extremely well organized meeting by the EUROPEAN PATIENTS' FORUM (EPF) and its partners in Sofia, Bulgaria. The interest of the local media was enormous and I was happy that the first of the only three statements from the audience in the opening part with the Bulgarian minister of Health and the Bulgarian MEP in charge for Health issues, as well as representatives from EU Commission DG Sanco, came from PHA Europe and made a very good impact. I must say that it was very nice to see such an initiative kick off and there must always be a start, but it showed me that we (patients) are still extremely helpless. In my opinion it will take a very long time to be able to say that every patient in Europe can have affordable access to the best possible therapy. We, PHA Europe, will continue to focus in each country individually to make a change for PH patients with our Call to action and with the professional work of our partners.

*Gerald Fischer*

### ESC AND ERS ANNUAL CONGRESSES 2012

It was a pleasure to represent PHA Europe at the ESC annual congress with my colleague Marzia Predieri of the Italian patient association AIPI, and at the ERS Congress, where Marzia and I were joined by PHA Europe's President, Gerald Fischer, and Board Member Juan Fuertes. The annual congresses of the EUROPEAN SOCIETY OF CARDIOLOGY and EUROPEAN RESPIRATORY SOCIETY are major scientific events which are consistently attended by thousands of healthcare professionals, industry and other interested parties. Being present enables us to distribute materials, present our activ-



ities and expand our contacts, an essential ingredient of networking, while also serving to raise awareness of PH among top medical and industry professionals. Most importantly, the ESC and ERS congresses give us access to the most up to date information on novel treatment strategies and ongoing and future research. It was very encouraging to see the large number of sessions, expert forums, symposia, posters, abstract sessions, oral presentations, tutorials, specifically dedicated to PAH at the congresses of both the cardiology and respiratory societies. These were led by world renowned specialists and topics for discussion included new endpoints for clinical trials, contributing factors to PAH, future trends in treatment management, outcomes in lung transplantation, the role of exercise training for PAH and many more still. The preliminary results of the Phase III study on Macintentan, a novel molecule for the treatment of PAH, were presented at both congresses (more data on this study and of the Phase III clinical trials on Riociguat were later released in October CHEST, the annual meeting of the AMERICAN COLLEGE OF CHEST PHYSICIANS in Atlanta). These are very positive developments that we hope will lead to new drugs being approved in the near future.

*Pisana Ferrari*



### 2nd ORPHAN DRUG CONGRESS

There are many different international forums where PHA Europe can raise PH visibility. One of these was the 2ND ORPHAN DRUGS CONGRESS held on 17th and 18th October in Barcelona, where in my lecture I spoke of:

- Prices of orphan drugs - the patients' view.
- The importance of involving patient groups in early stage of orphan drug development and clinical study programs.
- The impact of patient organisations on awareness about rare diseases at national level.
- The influence that patient organisations can have on healthcare authorities to gain full reimbursement.

At this Congress I was also on the panel for the interactive discussion on: "Creating an International Multilingual Online Patient Community".



During the congress the representatives of the pharmaceutical companies discussed orphan drug market and pricing policies in a very interesting string of presentations. Dr. Segolene Aymé, from the EU COMMITTEE OF EXPERTS ON RARE DISEASES (EUCERD) presented the INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM (IRDiRC) that has been resourceful in accelerating protocol development, approval and implementation as well as managing and analyzing data in clinical research.

There were very interesting presentations about new instruments that will have an enormous influence in pricing at European level. The main one will be the CAVOMP-IF (CLINICAL ADDED VALUE OF ORPHAN MEDICINAL PRODUCTS - INFORMATION FLOW), which is a process for the exchange of knowledge between Member States and European authorities to help improve informed decisions based on the clinical added value of orphan medicinal products. This concept of added value is one of the most important elements that is taken into account when it comes to pricing of new treatments at the Health Technology Assessment Agencies.

### PATIENT ADVISORY COMMITTEE OF EUROPEAN LUNG FOUNDATION

Another important forum for PHA Europe is the participation in the PATIENT ADVISORY COMMITTEE meetings organized by the EUROPEAN LUNG FOUNDATION (ELF) throughout the year. These coordination meetings reached a turning point at the EUROPEAN RESPIRATORY SOCIETY annual congress in Vienna because the Foundation has succeeded in increasing the participation and profile of patients' representatives at these scientific events. The ELF's mission is to bring people with lung diseases and the general public closer to science by providing opportunities to influence respiratory research agendas at the European level. It aims to work with patient organizations across the continent to involve people with lung diseases at every level of research and listen to their needs to ensure their voice can be heard by health professionals and politicians.

*Juan Fuertes*





# PH Latin Day

San José (Costarica), November 2, 2012



## Introduction

November is PH Awareness Month in the Americas. The Latin PH Society (SLHP is the acronym in Spanish) traditionally organizes several important activities that are concentrated around November 28, which is the Latin PH Day. SLHP has over the past year identified the need to have a Board that could be more implicated in the day-to-day work and have the capacity to help in the foundation and development of patient organizations in Latin American countries. Leaders of the strongest Spanish and Portuguese speaking countries now form the SLHP Board (Brasil, Spain, Venezuela, Argentina, Colombia and the Dominican Republic). In my double capacity as representative of PHA Europe and of the Spanish PH Association, I have helped the Board to draw the statutes and functions of the different SLHP bodies as an advisor for the SLHP.

## PH Latin Day celebrations

Last year the Latin PH Day was held in Miami and the experience has led to a very successful celebration in 2012 that exceeded all expectations. This year's choice of venue was San

José, capital of Costa Rica. Celebrations consisted of two main events: the PH Leaders Summit and the Medical Symposium. The idea behind the choice of Costa Rica was to help create a patients' organization in this Central American country. PH doctors in Costa Rica provided information to their patients so that they could attend the Leaders Summit on November 24 in San José.

## PH Leaders Summit

During the Leaders Summit, Costarican patients had the opportunity to learn and mingle with leaders from Spanish and Portuguese speaking countries. The experience gave a very important push to the creation of their own organization and helped raise their hopes of being able to exercise influence to achieve early diagnosis and access to medication. The program for the patients had a list of speakers that shared their knowledge in a way that reinforced the institutional capacities of existing PH organizations and gave the Costarican audience an invaluable base of know-how to build a strong organization from scratch.

In the morning sessions, Migdalia Denis, SLHP's President,

explained the experience of being a patient in a country without support and the success story of her survival and progress from functional class IV and oxygen dependent to functional class I, enjoying an excellent quality of life, thanks to adequate medical and pharmacological care and personal determination. Her presentation about the SLHP gave the audience important insights on the value of joining forces across borders. After Migdalia's inspiring opening, her sister, Lourdes Denis, promoted the SLHP newsletter, which has important contents for the PH community in Latin America. Ending the morning session and on behalf of PHA Europe's President, Gerald Fischer, I spoke about the PH Policy brief and Call to Action, presented the dissemination tools, and explained the use of framework agreements. The solidarity of the big associations (mainly PHA and PHA Europe) in sharing their campaigns and materials has become a global trend thanks to the celebration of the first World PH Day and the creation of a library where PH associations around the world can share their materials so that smaller organizations can retrieve the concept and the artwork for their own use saving effort and financial investment.

The afternoon session was no less powerful and inspiring. Irene Delgado presented the experience of the Spanish PH association ANHP as an example of how to create and develop a strong patient organization. Other members of the SLHP spoke about how much can be achieved in Latin America in spite of the difficulties imposed by the financial situation and the relative "youth" of their organizations. The PHA intervention by Debbie Castro also reinforced the importance of the dissemination of materials and the coherence of the message campaigns at global level in harmonizing communication strategies for common goals. The afternoon program included information on the medical aspects of PH by Dr.s Balloira and Matsuno, members of the SLHP Scientific Advisory Committee.

The day ended with a very motivating talk about leadership that left the audience full of energy in spite of a long day packed with information and emotions.

## Medical Symposium

On November 28, the Latin PH Day, Dr. Carlos Salazar and his team of Costarican PH specialists, organized a one day symposium on PH addressed to medical professionals. Lewis Rubin, Professor Emeritus at UCLA and one of the leading world PH specialists, was present at the meeting. The symposium was dedicated to Prof. Nazzareno Galiè, from the University of Bologna (Italy) for his exceptional contribution to PH in the international arena. The medical session was also attended by two SLHP Board Members and myself as representative of PHA Europe.

## Conclusion

Although the organization of two separate events for patients and physicians was somehow complicated during the preparation process because of an initial lack of communication, the Latin PH Day events led to a communion between medical doctors and patient organizations that created new bonds, an enormous respect and admiration for each other's work and new cooperation paths closing the gap between both communities. All in all, the week dedicated to PH in Costa Rica was a great success and there is great hope in that country for the seed that has been planted. PHA Europe is proud to have witnessed how patients and doctors have gone through a process of growth that will greatly change the future of PH in Central America. SLHP and Doctor Salazar's team have made a huge step forward in the progress of the patient-doctor pairing that is conducive to a tenfold development of a country's capacity to progress in the PH field.

Juan Fuertes





# AUSTRIA

## PATIENT MEETING “GAM STYLE”

This year we decided to try out a new way of organizing the annual patient meeting, inspired by PHA Europe's Leaders' Meeting in Barcelona in September. We had tables of eight and



on each one of these tables there was a volunteer moderator. Prof. Walter Klepetko, from the Vienna General Hospital spoke about lung transplant, Prof. Irene Lang about future medications, and a representative of the Austrian government about social support. Before every speech, we had ten minutes of brainstorming about possible questions to put to the speaker. After the presentations, the moderator asked the remaining open questions and the discussion became very lively and



productive. The introduction was done by myself and it was about the work of PHA Europe. The dinner after the meeting was a big family dinner with a very nice singer.

## PH CHILDREN'S MEETING



At the end of August PHA Austria held its annual PH children meeting in Vienna Gartenhotel Altmannsdorf. It was a big success for the children, because the toy manufacturer LEGO who sponsored the meeting offered very nice Lego toys and we also had a super nanny :-). My daughter Maleen said she felt like a small child again playing Lego and did not want to stop. For the parents, we had nice talks on the garden terrace with our local pediatric specialists from cardiology, pulmonology and transplant.



The press printed a nice article in the very popular journal “NEWS” featuring a double page with a photo of Maleen, Anna & Johannes holding up blue lollipops.

Gerald Fischer, Selbsthilfegruppe Lungenhochdruck  
[www.lungenhochdruck.at](http://www.lungenhochdruck.at)  
<http://on.fb.me/RzdEcb>



# BELGIUM

## CURRENT STATUS OF REMODULIN® IN BELGIUM

Due to the current very low level of reimbursement from INAMI (National Institute for Health and Invalidity), the distributor Therabel SA had to discontinue the distribution of subcutaneous Remodulin® in Belgium. All the patients which were already cured by Remodulin® will keep their treatment. The pharmaceutical company United Therapeutics Corp. will deliver the Remodulin® following a “Medical need program” to Therabel SA. This company will distribute the drug to the two centers of expertise in Belgium; Hôpital Erasme ULB and the University Hospital of Gasthuisberg. New patients requiring prostacyclin will be put on Ventavis® by inhalation or on intravenous infusion Flolan®. There is no central authorization in Europe, each country has to validate separately. This means that there is an important disparity of market authorizations in Europe.

### OVERVIEW OF TYPES OF ADMINISTRATION

Administration form	Market Authorization
Subcutaneous infusion (SC)	Y
Intravenous infusion (IV)	Y
Oral inhalation	N
Oral	N



## PATIENTS ASSOCIATIONS DAY IN CHU AT LIÈGE HOSPITAL

During the week of “Quality of Care and Security of Patients” in the CHU Hospital of Liège and together with LUSS, the Belgium Patients Platform, thirteen associations were invited to have a stand in the main entrance hall. Our booth was visited by clinical professionals, students and patients who asked information about PH. This initiative will be repeated next year in other hospitals.

Luc Matthysen,  
HTAP Belgique





## BULGARIA-PHA BULGARIA



From left: Todor Mangarov and Vania Toteva



### NATIONAL CONFERENCE ON PH

On October 13 and 14 PHA Bulgaria organized its first National Conference on pulmonary hypertension. The conference brought together patients, family members, medical professionals and other stakeholders. Lecturers included some of the best specialists in Bulgaria - cardiologists and pulmonologists - from the three Bulgarian reference centers for PH. The presentations covered topics such as the classification of pulmonary hypertension, diagnosis, treatment options. Prior to the scientific lectures, a summary of the main results of the PH national treatment program, since its launch in March 2011, was presented. PHA Bulgaria's President, Todor Mangarov, spoke about the association's activities and its achievements and objectives for the future. Todor also spoke about issues of practical use for the patients: what are the forms of PH with available treatment in Bulgaria, what patients have to do in order to receive medication for PH, what changes are needed and in what direction PHA Bulgaria and its members should act to ensure more awareness and better treatment.

During the conference, a seminar for medical students was also held. This seminar was attended by students from six medical universities in the country. Our country's future doctors had the opportunity to hear about the importance of early diagnosis and of the prevention of rare diseases, with a focus on pulmonary hypertension.

Finally, a meeting was held for the patients and their families attending the conference, during which a PH community and a group for support and assistance were established. This meeting was attended by 37 patients (according to the association, the total number of patients in Bulgaria at the moment is 47). Attendees were told about PHA and the "Lantos" grant, thanks to which the meeting took place. A psychologist gave a very interesting presentation on "Diagnosis of Pulmonary Hypertension: How to communicate with family and friends" and "How to talk to your doctor?", after which there was a discussion with the patients. The meeting with the psychologist helped PH patients realize they are not alone in the difficult fight against the disease and that they have someone to rely on for moral and physical support. Patients and their relatives were able to talk about problems in their everyday lives and to share how they deal with them. At the end of the meeting anonymous evaluation forms were handed out. The feedback was very positive. Patients felt that it is useful and important to meet other patients. Many would appreciate in future meetings to have more time to share problems and to discuss issues like nutrition, physical exercise (when and how) and to strengthen psychological counseling and guidance.

Vania Toteva, PHA Bulgaria  
[www.apph-bg.org](http://www.apph-bg.org) - <http://on.fb.me/UDRowS>



## BULGARIA-BSPPH

### 13th CARDIOLOGY CONGRESS

The XIII Bulgarian National Congress of Cardiology took place in Sofia from the 4th to the 7th of October. About 1.500 cardiologists from the country and abroad took part. Among the participants there were also representatives of BSPPH. During the congress a scientific session specifically on Pulmonary Hypertension was held. Leading cardiologists and representatives of the patient organizations discussed problems related to the diagnosis and treatment of the disease and to the need for centers of expertise. Logically the doctors and the



representatives of the patient organizations will be united in ensuring that the status of such centers in Bulgaria will be formalized by the Ministry of Health as soon as possible.

### PHYOGA AWAKENING

BSPPH together with the Bulgarian Yoga Association are starting a joint project - PH Yoga Awakening. For three months, twice a week and absolutely for free, patients with pulmonary hypertension will learn the secrets of yoga. The method that will be used is an integral one, which deals with the person as a whole. There must be harmony between body, mind and emotions, and this is what yoga practices offer: yoga strengthens the physical body, calms the restless mind, and brings out the huge individual creative potential. The course is focused on the most widely diffused practices of yoga for good mental and physical health. It includes a variety of methods and techniques and will combine the *Asana* (physical postures) with *Pranayama* (breathing techniques), relaxation and meditation. *Ashtanga* is a stimulating practice that improves the strength and endurance of the body. Yoga breathing exercises open the chest and diaphragm, allow the breath to purify the cardiovascular system with deep inhalations and exhalations. Breathing exercises are aimed to train the internal muscles to pump powerful breaths and keep the pulmonary artery passages open. Qualified teachers trained in yoga in India's Bihar Yoga Bharati University, the first recognized Yoga University in the world, and in Yoga Academies in Australia, Europe and the U.S., will show us the secrets of yoga.



### CHARITY CALENDAR 2013

*"We are no different for the real friends"*

There is only one month to go to the end of 2012. We will welcome the New Year 2013 full of hope. We wish for health, joy and new opportunities. No person can choose when or what disease to suffer from. Some people have rare diseases, but that does not mean they do not have the right to live to the fullest. On the contrary, they fight twice as much as the others to achieve their aims. What does "being different" mean? Some people strive to be different all their lives, others are different all their lives. 10% of the population in Europe have something different in them, in other words, they suffer from one of the 8.000 rare diseases. Six Bulgarian women - successful professionals - who suffer from different rare diseases agreed to have their pictures taken by the photographers Yana Peneva and Stanislava Yamakova, during an amazing photo session, to be part of a charity calendar for 2013, entitled "We are no different for the real friends." These ladies are different, but together they are stronger. Each one of them knows that she is different inside, but their appearance does not distinguish them from other women. They need better care, in order to have normal physical capacity, in order to keep on contributing to society. Natalia Maeva, the chairperson of Bulgarian Society of Patients with Pulmonary Hypertension (BSPPH) is among the stars of the charity calendar. Thanks to the help of the International Women's Club (IWC), the calendars will be sold for charity during the IWC Annual Charity Bazaar 2012, which is due to be held, as it traditionally is, on the 2nd of December 2012 at the Inter Expo Center, Sofia. The funds raised will be used to buy medical equipment needed for Saint Marina Hospital, Varna.



### NATIONAL PATIENT ORGANISATION

In November BSPPH became a full member of the National Patient's Organization of Bulgaria. This organization has members from 20 national patient organizations and over 15 regional associations. This non governmental organisation is the biggest patients union in Bulgaria.

Natalia Maeva,  
 The Bulgarian Society of Patients with  
 Pulmonary Hypertension- BSPPH  
[www.bspbh.eu](http://www.bspbh.eu)  
<http://on.fb.me/TLJaPD>





## CZECH REPUBLIC

### PHA EUROPE GAM BARCELONA

This year, our physiotherapists Veronika and Dominika, had the pleasure of taking part in the General Annual Meeting of European PH associations in Barcelona, held from the 12th to 16th of September 2012. For both of them the meeting was really inspiring and very useful. They had the opportunity to meet patients and other representatives from many European countries and make contacts with them. As they said they returned with a lot of new ideas and plans for our association. In the future we would like to make Veronika our liaison person between PHA Europe and our association because of her nursing background and knowledge of English.

### RECONDITIONING STAY IN POD BRADY

From August 12 to 19 our association organized another reconditioning stay in a very beautiful spa resort in Pod brady, a town west of our capital. Every year we choose this venue because of its main advantage which is that the grounds are flat and this makes it easier for our patients to walk. The program was the same as last year's and included light workouts under the supervision of our two physiotherapists, short walks along the river Labe, discussion evenings and a medical lecture with our PH doctor. The workout consisted of breathing and posture exercises and twice some patients chose to do their exercises in the pool. As an innovation we



From left: Yveta Makovnikova, Jana Guranova, Dominika Tepla and Veronika Jelinkova

included a sightseeing outing to our program. The route of the train led through the beautiful surroundings of the city of Pod brady and along the river Labe and I must say that we really enjoyed the ride.

### PUBLICATION

We have just completed the second issue of our association's journal, *Bulletin*, where Veronika shared with us her experiences from the GAM in Barcelona, and this will be issued before the end of the year. This year we would also like to publish a brochure called "The PH patients guide", which is currently being written by Dr. Regina Votavova and is almost complete. This brochure will be published thanks to the financial support received from the Ministry of Health in the form of a grant.

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



## FRANCE

### LA DIAGONALE DES FOUS: RUNNING TO RAISE AWARENESS OF PAH



Alain Mialon (see photo) ran with HTAP France's logo on his back, for 170,7 kms, diagonally from South East to North West, of the *La Réunion* island, in the Indian Ocean, to raise awareness of PAH. The island has a lot of ups and downs (more than 10.000 meters difference in levels) and he ran through the mountains. His HTAP France tee shirt n° 297 was seen for 54 hours from 0 metres above sea level, to 3.000 metres of altitude high in Cilaos, one of the highest villages of the island. Alain is a volunteer for HTAP France, and organises every year since 2005 the ultramarathon Toulouse-Port la Nouvelle. See also:

<http://www.htapfrance.com/blog/default.asp?page=7>

Alain and his companion Danielle first heard of PAH in 2005. At that time they met Marie Seube, a patient from Toulouse, whose friend had the idea to run for her. Alain and Danielle have since then helped organize this event which now gathers more than 200 runners. But what Alain did in *La Réunion* was quite a crazy challenge: the race is called "The Diagonal of the Crazy". Only half the runners usually manage to finish the race, and for his second participation to this amazing event, Alain arrived after running 54 hours, which is very honourable for a "Senior 3" like him. By the way, I forgot to tell you, Alain is 63! Before the race and after the race, there were several TV presentations, with interviews of



Alain and Dr. Patrice Poubeau, who coordinates the PH competence center in Saint Pierre (*La Réunion*).

Thanks to Alain's great project some articles in the local press also mentioned HTAP France.

Before leaving the Island, Alain and Danielle together with Roselyne (Mélanie's mother) organized and attended a HTAP France patients meeting, with the doctors and nurses of the competence center.

We are thankful to Alain for his long time engagement. Next year we will celebrate the 10th Ultra-Marathon, and Alain says he will also run the GRR in *La Réunion* again... Let's follow him, running for us!

### REGIONAL MEETINGS

Two regional meetings took place this autumn, one in Nantes (South Brittany), and one in Rouen (North Normandy). These meetings are always organized on the same pattern: in the morning, the association HTAP France presents itself and there are workshops on practical subjects. Low salt diet, how to wear the pump and social support are some of the subjects proposed in these workshops. Then we all have a nice lunch and chat over coffee. In the afternoon doctors from the local competence center present the news on PAH and answers questions for the whole afternoon. We end with a drinks party. There were around 60 persons in both of the meetings.







### FAMILY WEEKEND

In October HTAP France organized its 5th WEEF, our "PAH parents and children week-end". It was in the middle of France, in the beautiful mountains of Cantal. Sixty persons shared these two days, intended to help families get to know each other and exchange tips and, why not, become friends. We spent a really nice time there and had the pleasure to welcome two new pediatric practitioners, who were very satisfied and surprised to learn many aspects of every day life that they did not even suspect. I think we gained two more fans within the doctors!

### PRESS

I have recently been asked to write an article about the role of HTAP France in a French medical journal called "Le con-

cours medical" (40.000 copies), for a special issue on Pulmonary Hypertension directed by Pr. Jean-François Cordier. We were all very happy with it as we think it is a good way to simultaneously raise awareness on PH and allow practitioners to read about patients preoccupations.

### FUTURE PROJECTS

In the next Mariposa Journal I will be able to tell you more about next year's main projects, and especially about our second Patients Conference. Save the date: 4-6th October, in Lyon. It will be in French.

I wish you all a very happy new year on behalf of HTAP France.

Mélanie Gallant-Dewavrin, HTAP France  
[www.htapfrance.com](http://www.htapfrance.com) - <http://on.fb.me/SDPDPU>



## GERMANY

### ANNUAL PATIENT MEETING

The 15th Annual Meeting of the German PH association ph e.v. took place from October 26 to 28 in Frankfurt am Main. Approximately 250 patients, their families and other interested parties attended. The meeting featured expert presentations and workshops and allowed for many opportunities to meet and share experiences. Guest speakers



included some of the most prestigious members of the international scientific community: Prof. Ardeshir Ghofrani (Giessen and Marburg), Professor Ekkehard Grunig (Heidelberg), Dr. Stephan Rosenkranz (Cologne), Professor Eckhard Mayer (Bad Nauheim), Prof. Horst Olschewski (Graz) and Dr. Hans Klose (Hamburg).



During the patient meeting the ph e.v. "Best PH journalist award" for 2012 was given to Dr. Thomas Kron for his article "Out of breath - but still life". Dr. Thomas Kron is a physician and freelance science journalist. His article was published in the widely known online medical portal [univadis.de](http://univadis.de). The new chairman of ph e.v., Hans-Dieter Kulla and his team presented the work of the association, which currently has around 1.300 members. During the event there was a moment dedicated to the memory of the association's founder and chairman Bruno Kopp, who prematurely and tragically passed away on the February 27, 2012.

Hans-Dieter Kulla, pulmonale hypertonie e.v.  
[www.phev.de](http://www.phev.de)



## GREECE

### A NEW PATIENT ASSOCIATION, PH GREECE!

My name is Ioanna Alisandratou, I will turn 40 on December 15. I have two children, Vaggelis, who is 11 years old, and Christos, 13 years old. I was diagnosed with PH in August 2010 but from my medical history and symptoms it is likely that I have been ill since 2008. I worked for twenty years as a financial planner but have retired since my diagnosis. I stopped working quite young so I have now a lot of time to be a volunteer to the PH cause. My clinical condition is very good and the only drug I take is Revatio to which I respond very well. I have the strength and mental disposition to help other patients. I regularly participate in all conferences in my



country. I will be soon in the national conference of PESPA, representing our patients. I would like to organize a conference in Greece on May 5 next year, for World PH Day, and I am already working on it. I am involved to all social media such as LinkedIn, Twitter, other blogs. My new Facebook page has made lot of people talk about us. Here is the link to our Facebook page, click "like" to help us get more friends!

Ioanna Alisandratou, PHA Greece  
<https://www.facebook.com/PulmonaryHypertensionGreece>



# HUNGARY

## ANNUAL PATIENT MEETING

“Cooperation in the hope of a cure” was the motto of the 2nd national patient meeting organized by Tüdőér Egylet (PH Hungary), attended by over 140 patients and caregivers, PH physicians and other stakeholders.

In her introductory speech Eszter Csabuda, President of PH Hungary, stressed the fact that Pulmonary Arterial Hypertension (PAH) is a rare and serious disease, sometimes claiming young lives. She stressed that most of the patients are only diagnosed and appropriately treated 2-3 years after the symptoms arise. This delay, with respect to treatment and the progress of the disease, is huge, because timely treatment is more effective. The reason of the delayed diagnosis is simple: there are no typical symptoms which could be directly linked to this disease. Eszter herself was only properly diagnosed after 8 years. Eszter told the audience that

the Hungarian PH association is a young organization, founded in 2008 by Kristof Karlocai, MD, a PH expert, and 40 patients. She emphasized that the goals of the association are complex: to give hope, inform about treatment options, help to deal with the disease in day to day life and fight for the availability in Hungary of those drugs which are already approved and used in other parts of Europe. It is also important to provide psycho-social support and to raise awareness of the disease, because patients not receiving treatment can pass away within 3-5 years. At present 170 patients are diagnosed and probably the same number of undiagnosed patients can be found in Hungary.

One of the key speakers was the representative of the National Health Insurance Fund, who informed the audience on the subsidization, availability and authentication of drugs. He also introduced the kind of protocols,



and gave a little insight into the indications followed by the National Health Insurance Fund.

In his presentation Kristof Karlocai, MD, Vice President of PH Hungary and physician at the Semmelweis University Lung Clinic, spoke about the changes of physiology resulting from the disease and the possible outcomes of the treatments. He underlined that as a result of the modern and state-subsidized drugs the quality of the patients life has improved significantly.

András Temesvári, MD, physician at the Gottsegen György Hungarian Institute of Cardiology, gave a presentation about the incidence of the disease and also provided proposals and hints about lifestyle issues and travelling.

László Abloncy, MD, pediatric cardiologist, treating 30 children with PH, stressed that it is harder to detect the symptoms of children and the progress of the disease differs. Timely treatment is crucial for children.

Last but not least, Gerry Fischer, President of PHA Europe, our guest at the meeting, gave a presentation about the European association, its objectives and activities, stressing the importance of international cooperation between associations. He greatly appraised the results, rapid growth and innovative projects of the Hungarian PH association.

Two Hungarian patients were transplanted in Vienna this year. On April 20, a mother of two children, 36, received new lungs. Her presence at the meeting, her heartfelt speech and her complete recovery prove that surgery is also a good option for PH patients.

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



From left: Gergely Meszaros, Gerald Fischer, Eszter Csabuda and Dr. Kristof Karlocai





## ISRAEL

### AN INNOVATIVE ANNUAL CONFERENCE

After much deliberation, the steering committee of the Israel Pulmonary Hypertension Association (IPHA) decided to try something different this year for our annual conference: holding it over two days at a hotel. Our aims were threefold: firstly, to allow patients and their families to get an update on various medical issues; secondly, to enable patients and caregivers to better interact and communicate with each other, and, thirdly, to provide patients and caregivers with a break from their usual hardships and let them enjoy themselves and recharge their batteries.

We decided to hold the conference in the countryside, in the Carmel mountain area, a short distance from the city of Haifa. Check-in at the hotel was at two in the afternoon and time was given for the patients to rest up from the journey before the start of the sessions. Our opening session started in the late afternoon with a panel of three medical professionals, two doctors and a nurse, who spoke about the difference between primary and secondary pulmonary hypertension, the importance of exercise as a treatment modality, and an explanation of echocardiographs and their importance to diagnosis and treatment. We then enjoyed a lovely meal which was followed by entertainment provided by a comedian.

The next morning was broken into two sessions, where participants could choose between a session on Chi Kong exercises especially adapted for PH patients and a session aimed at improving communication between couples where one of the partners suffers from PH.

The second session was geared toward having fun, with participants choosing between workshops on flower arranging and chocolate making.

The decision to have the conference at a hotel was decided with a lot of trepidation, mainly because it had not been done

previously in Israel. The results, though, proved to be a resounding success, with all three of the aims we had set out for the conference being realized: the participants went home having learnt a lot about their disease, they made friends and contacts, and a good time was had by one and all.

The question that we heard over and over again was when we could have the next get-away at a hotel. The message for other patient associations is that the financial investment and hard work was worth it as it was a truly memorable conference for all the participants.

### PROMOTING RESEARCH

Following their return from the PHA Europe conference in Barcelona last September, PHA Israel's representatives to the meeting, Aryeh Cooperman and Yosef Gotlieb realized the importance of integrating Israeli research and expertise in the global effort to improve diagnosis, treatment and to arrive at a better understanding of PH. Accordingly, efforts are underway to further international cooperation among Israeli expert physicians, research groups and companies in the European and international networks.

An approach letter will soon be sent to relevant experts and other relevant contacts offering PHA Israel's assistance in networking and facilitating collaboration, particularly with respect to the EU's initiatives for research cooperation (FP7 and, as of 2014, Horizons 2020). Briefings to discuss funding sources and procedural issues are under consideration. PHA Israel hopes to serve as a liaison between institutional bodies and Israeli specialists, researchers and industry group in furthering professional cooperation.

*Jonathan Berg and Yosef Gotlieb, PHA Israel*  
[www.phisrael.org.il](http://www.phisrael.org.il)



## ITALY-AIPI

### NEWSLETTER AND PUBLICATIONS

AIPI publishes a quarterly newsletter, the Fall edition of which has recently come out. Together with the newsletter we sent out a brochure we have produced which contains useful contacts and information for PH patients who are being followed by the Bologna S. Orsola -Malpighi hospital. Other recent publications include a booklet on Italian legislation for disabled persons which was realized in collaboration with another patient association, AMRER, (rheumatic diseases). This booklet offers information about parking permits, provisions for assisting disabled persons, possibility to take leaves from work etc. Our next project is a booklet with practical advice and tips for every day life with PH, due out early 2013.

### PALERMO EXCURSION

AIPI organizes one or two excursions each year. Last year it was Milan and Perugia, this year in June we organized a three day tour of Palermo, the capital of Sicily, a beautiful island just off the Southern coast of Italy. Marzia Predieri, the Treasurer of AIPI, accompanied the group, all PH patients and their family members. The program included many historical sites such as the Cathedral of Monreale with its extraordinary gold mosaic ceilings and the Zisa Castle (both from the 12th century), flea markets and antiques, picturesque food markets, an excursion to the old fishing village of Mondello and great local traditional food and wine tasting!

### CHARITY DINNER FOR PH

The Young Industrialists Group of the city of Modena holds an annual charity dinner. This year's event, which was held on December 12, was dedicated to pulmonary hypertension. Roberto Arletti (father of a young girl who had a double lung transplant), and Marzia Predieri, representing AIPI, were asked to talk about PH, the role of our association and our support activities for patients. After the presentation, many questions were asked by the audience. The event took place at the Museo Casa Enzo Ferrari in Modena, which is a huge modern museum hosting all the first models of Ferrari cars, all beautifully



shining and still working! The funds raised, for which we are very grateful to the Gruppo Giovani Imprenditori di Modena, will go into a special fund we allocate yearly to provide economic help to needy patients. Roberto and Marzia took the occasion to distribute AIPI booklets, brochures and newsletters. The participants seemed very interested in learning about the disease.

*Pisana Ferrari, AIPI Italy*

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

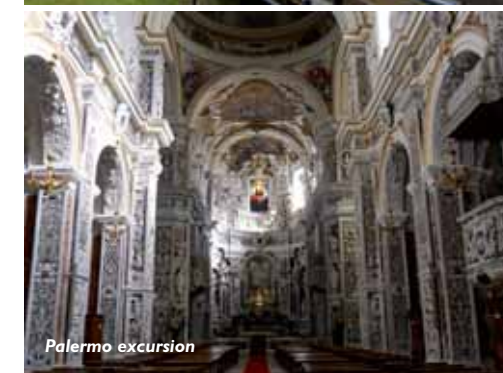
<http://www.facebook.com/AIPIItalia?ref=ts&fref=ts>



Palermo excursion



Charity event at Ferrari Museum



Palermo excursion



Charity event at Ferrari Museum



Palermo excursion



Charity event at Ferrari Museum





## ITALY-AMIP

## AN ENTHUSIASTIC START FOR THE AMIP SCIENTIFIC ADVISORY COMMITTEE

On September 29 at the hotel Villa Morgagni in Rome, on the occasion of the meeting “Multidisciplinary Approach to Pulmonary Hypertension”, organized by Prof. Dario Vizza, Head of the PH CENTER OF SAPIENZA UNIVERSITY OF ROME, we witnessed an event that for us at AMIP can only be described as historical. All six members of our Scientific Committee attended, each of them a director of an Italian PH Center in Italy, and decided to start a clinical collaboration which will undoubtedly bring the great results we have always wished for.

The day marked the birth of the “ITALIAN PULMONARY HYPERTENSION NETWORK”, or “IPHNET”, a network of Italian expert centers specializing in the research and treatment of PH, who will now share a common database. The database, web-based, has been designed with the contribution of all the members of the AMIP Scientific Committee and includes several clinical, hemodynamic, echocardiographic, neurohormonal variables. The implementation of graphs and tables will allow a simple way to follow-up the patients and to make therapeutic decision. Since the announcement of this project, other experts asked to join the network and, up to now, 11 Italian centers are involved. Each center will be asked to conform to a high clinical and scientific standard in order to ensure the good



The six members of our Scientific Committee that were present at the September 29th meeting: Dr. Massimiliano Mulé (Policlinico Vittorio Emanuele, Catania), Dr. Michele D'Alto (A.O. Monaldi, Napoli), Prof. Stefano Ghio (Policlinico S. Matteo, Pavia) besides him Dr. Scelsi, Dott. Francesco Parisi (Ospedale Pediatrico Bambino Gesù, Roma), Prof. Dario Carmine Vizza (Università la Sapienza, Roma), Dott. Patrizio Vitulo (ISMETT, Palermo)

quality of the data collected. Only in this way it is possible to make the IPHNET database a powerful clinical and research tool for improving the knowledge on the disease and the treatment of the patients.

The fact that our experts will be able to collaborate in such a well-organized and close manner is really exceptional and

should not be taken for granted. The fact also that the creation of the IPHNET has happened thanks to the perseverance of our association makes us really happy, AMIP was credited as having been a “very powerful engine” for the implementation of this project!

We are also grateful to Dr. Badagliacca, Dr. Poscia and Marco Gasbarrini who carried out this important project! The realization that our doctors will now work in full synergy is what we have always worked for. The patients will now feel safe no matter which Centre they go to for treatment. Our hope has always been that doctors SPEAK WITH ONE ANOTHER, and it looks like our dreams are becoming a reality in the most technically advanced manner we could ever imagine. We expect to see very positive results as soon as the IPHNET will be fully operational. Once again, we would like to emphasize the importance of such an agreement between the expert PH Centers. IPHNET will become a great resource not only for the patients who will benefit from the shared knowledge base, but also for the medical world who will gain in the type of information that will enable them to reach quick diagnosis but will especially enable them to collaborate with the Centers.

Luisa Sciacca della Scala, AMIP Italy

[www.assoamip.net](http://www.assoamip.net)

<http://www.facebook.com/AssociazioneMalatiDiIpertensionePolmonare?ref=ts&fref=ts>



From left: Vittorio Vivencio and Prof. Dario Vizza

Incontri di Ipertensione Polmonare:  
approccio multidisciplinare.

Roma, 29 Settembre 2012



CON IL PATROCINIO DI



## LATVIA

## HEALTH CAMP 2012 FOR PH PATIENTS

The first Health camp for PH patients in Latvia took place beside Lake Usma from July 13 to 15. There were 27 participants including 5 children. During these three days PHA Latvia organized meetings with a psychologist, relaxation sessions, yoga exercises, an untraditional sports game day called “Harmony of team”, and the general meeting of PHA Latvia members. There was a special program for developing plays, drawing, sports activities, and excursions in the nature around the area for the children. Patients and their relatives spent their free time boating, walking and enjoying the beautiful nature and silence at Lake of Usma.

## GAM 2012 IN CASTELLDEFELS



The PHA Latvia Chair Ieva Plume and Anna Galvina, a volunteer, and journalist, participated in the PHA Europe General Annual Meeting 2012 in Spain. The programme of GAM 2012 was very useful and inspirational

for our further work. We shared ideas, opinions and contacts with other PH organizations in Europe. A wonderful surprise awaited us there: the 2nd prize to PHA Latvia for the best Breathtaking campaigns for Rare Diseases Day 2012!

## WORKSHOP ON HEALTH CARE

On November 3, PHA Latvia organised a day-long workshop on health care for PH patients that took place in Riga. Experts of health care presented the ABC of oxygen therapy, a psychotherapist conducted a training session on depression, and the Chair of PHA Latvia Ieva Plume gave each PH patient a pulse oximeter, which was acquired through donations.

## OTHER NEWS...

- PHA Latvia continues to work on the National Plan for Rare Diseases and meet with officials from Ministry of Health and The National Health Service to discuss healthcare for PH patients in our country.
- Up to now we cover the costs of oxygen equipment rent for 13 PAH patients. The number of members has reached 30 but there are 55 diagnosed PH patients in Latvia.
- We have a new sponsor - Sanofi-Aventis - who joined the companies that support PHA Latvia.

Ieva Plume and Anna Galvina, PHA Latvia

[www.phalatvia.lv/en/](http://www.phalatvia.lv/en/)

<http://on.fb.me/Vx0udp>







## NETHERLANDS

### NEW AWARENESS MATERIAL

In the last few months we have been very busy developing new up-to-date materials. We have tried to make our new brochures more attractive by a nice graphic layout and more pictures. We hope to finish this project by the end of the year.

### CONCENTRATOR FOR RENT

Within a few weeks we will have finalised a contract with a dealer to rent the very small concentrators at a reasonable price.

Patients will be able to rent them when they go on vacation or when they need oxygen during a flight by plane. This will solve the problem of the very high costs to get oxygen in a plane.



Also the costs of oxygen abroad are not always paid by insurance companies. In this case a small concentrator is very helpful. We found a sponsor so that the price is only €5 per day.



### SPECIAL DAY TO "INDULGE" OUR PATIENTS!

In September we organised a day with all kinds of nice surprises for the patients. There was the possibility to have a facial massage, all kind of demonstrations and they could ride on a Segway. There was also a very nice lunch. The meeting was so popular so that we had to stop registrations. Because of this we intend to organise it again in Spring next year. These events are very useful to meet each other and to compare our experiences with PH and the consequences of the disease.

*Ferdinand Bolsius, PHA Nederland*

[www.pha-nl.nl](http://www.pha-nl.nl)

<http://on.fb.me/TFBsJT>



## NORWAY

### FALL ACTIVITIES AT PH NORWAY

Usually, PH Norway runs a patient meeting in the second half of the year. However, this time the decision was made to postpone the meeting to the first half of next year. This was done in order to hold the meeting at Feiringklinikken - the rehab center for PH patients. In this way, PH Norway will be able to form an even closer bond to the rehab center and they will also be able to utilize the human and non-human resources available there.

PH Norway was represented at the PH Germany annual patient meeting in October. PH Norway benefits by learning and getting ideas from this mature organization, especially on how to run successful patient meetings.

Two members (patients) of the PH board have recently received training from our "parent" organization (the Lung and Heart association - LHL) in patient support work. These two patients will make themselves available to the National Hospital Center where all newly diagnosed PH patients have their initial hospital stay. On request, doctors



can call the two support patients, and newly diagnosed patients will be able to have a face-to-face meeting with more experienced patients about living with the disease. The newly diagnosed patients will hopefully see that one can live with the disease and still have a very good quality of life. The two support patients will also be available for phone calls from all PH patients and their families. PH Norway invited to an informal gathering in the Fall. A few patients showed up and had a hike by the beautiful Oslo fjord. The walk was followed by a nice lunch at a nearby cafe.

*Hall Skåra, PH Norway*

[www.pha-no.com](http://www.pha-no.com) - <http://on.fb.me/TDzyKI>



## POLAND

### EDUCATION CAMPAIGN

Two members of the Polish PH Association - Alicja and Grzegorz Morze - took part in the "Harvest Festival" in August. They organized an information point for PAH. The stand attracted a lot of interest. Leaflets, posters and press releases presenting the activity of the association were distributed. These information materials were also at the medical center, pharmacy and shops throughout the community.



### TRAINING "THE INFORMED PATIENT"

On October 16 the Polish PH Association was invited to take part in the training program called "The informed patient - creation of social attitudes" by Bayer and by the "Association for Good Clinical Practice" in Poland. The training was aimed at people actively working for patients in associations, foundations and similar organizations. The aim of the training program was to present issues related to clinical trials. The venue for the session was the Polish Press Agency in Warsaw. See at: <http://bit.ly/VjR0AZ>



### SECOND CONFERENCE OF THE CARDIOVASCULAR PULMONARY SECTION OF THE POLISH SOCIETY OF CARDIOLOGY

The second Conference of the Cardiovascular Pulmonary Section of the POLISH SOCIETY OF CARDIOLOGY was held in Warsaw on November 16-17. There was talk about pulmonary embolism, chronic pulmonary hypertension and right ventricular function. The organizers summarized the effects of therapeutic programmes for pulmonary arterial hypertension. Participants discussed the challenges in relation to the fact that pulmonary arterial hypertension is becoming a more and more chronic disease. A special session for nurses also took place at the conference.

See at: <http://www.krazenieplucne.pl/> - <http://bit.ly/WYtteB>



### PARTICIPATION IN MORNING TV PROGRAM

Two representatives of the Polish association, Barbara Bieniasz and Kazimierz Kondek, took part in the recording of "Health Line", a short film recorded for the morning program "Coffee or Tea". This recording was used in the program on November 15. Grzegorz Kopec, Deputy Head of Centre for Rare Cardiovascular Diseases in Krakow, answered questions from the public about pulmonary hypertension and rare diseases.

See at: <http://bit.ly/W8GdcD>

*Agnieszka Bartosiewicz, PHA Polska*

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

<http://on.fb.me/RzeoOU>







## SLOVAKIA

### GENERAL ASSEMBLY OF PHA EUROPE, BARCELONA

Every September our association takes part in the General Assembly of PHA Europe. This year's meeting took place from the September 12 to 16 in Castelldefels, near Barcelona (Spain). Our association was represented by Iveta Makovníková and Jana Guráňová. The five-day program of the meeting was filled with valuable lectures, covering clinical studies, new drugs and research, PH centers patient surveys, media strategy... We very much appreciated the group discussions, where we could share our experiences and work on new programs in area of PH. The award we received for 3rd place in the "Best 2012 Breathtaking campaign" was very encouraging. Another positive thing was the deepening of personal and professional relationships between the members of PHA Europe.

### BUSINESS MEETINGS - BANSKA BYSTRICA, ŽILINA

In the month of September two important meetings were held: a meeting with the President of the association of patients with lung transplants, Mrs. Boháčiková, and a meeting with the President of the association of patients with heart transplants, Mgr. Vlasta Pagáčová. The aim of these meetings was the development of cooperation between our associations, as well as the sharing of work experience. President Iveta Makovníková also attended a three-day tourist-educational seminar with a rich sports and educational program for patients with heart transplants.

### PARTICIPATION IN THE XVII CONGRESS OF THE SLOVAK SOCIETY OF CARDIOLOGY

The XVIIth Congress of the Slovak Society of Cardiology was held on October 11-13 in Bratislava. As last year, we took



part in the SKS congress by being present with our own stand. We distributed leaflets and brochures about PH to visitors. We attended a symposium on PH that was supported by Actelion and which was led by the head doctor of the Slovakian Center for PAH, Dr. Eva Goncalvesová. Another interesting part of program was a presentation about the impact of PH on the every day life of patients, with short videos of PH patients. We were happy to see that were almost 160 health care professionals participating. The last day, after the symposium about cardio-physiotherapy, we took the first steps towards the successful realization of a rehabilitation stay for patients, something that we have been planning for a long time. Dr. Viliam Rus, Director of the Vyšné Ružbachy Spa, specialised in cardiovascular diseases, was very helpful with the preparations.

### UPCOMING EVENTS

- As our activities have grown this year and we have lots of news which we wish to share not only with our members but also with other interested parties, we have decided to issue a newsletter and hope to have the first number out by the end of the year.
- On November 23 two PH patients participated in the live TV broadcast program called Fokus on Slovak Television. The aim was to show the impact of PH on a patient's life and that of his family.
- On December 4 we are preparing a meeting for ZPPH members with representatives of partner pharmaceutical companies in order to present our work in the past year and to familiarize them with the plan for 2013.

Iveta Makovníková  
Združenie pacientov s pľúcnou hypertenziou  
[www.phaslovakia.org](http://www.phaslovakia.org)  
<http://on.fb.me/Ud1DUf>



## SLOVENIA

### NEWS FROM THE PAST YEAR

The Pulmonary Hypertension Association in Slovenia was established just over a year ago, in April 2011, on the initiative of Klara Klančar-Kavalič, a young mother with two daughters. A few years back Klara struggled with severe pulmonary arterial hypertension due to chronic thrombo-embolic disease, which was not recognized until very late and was also initially treated as idiopathic pulmonary hypertension. The disease progressed very fast and she was "near dead". Finally the disease was correctly diagnosed and Klara was one of the first two patients sent, four years ago, from Slovenia to the AKH, Vienna General Hospital, to Professor Walter Klepetko, who, together with his excellent team, performed a very successful pulmonary endarterectomy.

Klara was very happy and grateful. Her life had completely changed. Now she had again the normal life of a young woman. Her daughters had their mother back. Klara, with her positive attitude, decided she wanted to help other people from Slovenia who suffered from the same disease and that she would like to contribute to raising awareness about it so that her story would not repeat itself. She now knew that, thanks to

new treatment possibilities, this disease is no longer "so deadly". And in less than two years she has really succeeded in her aims, with help of all her friends, the nice people from the Pulmonary Hypertension Association in Slovenia, and the European Pulmonary Hypertension Association, especially 1st Vice President Pisana Ferrari and President Gerry Fisher. It was also very important to have the support of the University Clinical Center of Ljubljana, the main medical institution in Slovenia for PH, who had helped Klara to get a correct diagnosis and treatment.

The "Breathtaking" awareness campaign for RARE DISEASE DAY (RDD) 2012 in Slovenia began on February 22 with the participation on the national television in the prime time program *Good morning Slovenia*. Klara Klančar and assistant Professor Barbara Salobir were interviewed for this program. Dr. Salobir is a specialist of internal medicine and pulmonology dealing with patients with pulmonary hypertension and is a member of the Slovenian association. She helped provide support to the association throughout the campaign. The campaign followed with a one hour interview for *Radio Veseljak* on the day prior to RDD.

On the actual RDD day, February 29, at 9 am, the campaign started in the main hall of the University Clinical Center Ljubljana. Many other doctors from the University of Ljubljana as well as from other hospitals were present and of course Dr. Salobir. Some of the Slovenia's most prominent artists, media and sports personalities also attended and had their photos taken with blue lips and lollipops. Guests included singers, radio conductors, actress Tina Gorenjak, author Nastja Klevže, 18-time national motocross champion Sašo Kragelj and the 2012 World Cup female ski jumping team. At 12 o'clock, a press conference was held with many national and local journalists from press, from national and local TV and radio stations.

The "Breathtaking" campaign materials and lollipops were also distributed in shopping malls in Maribor, Capodistria and Celje. Advertising space on buses in Isola, Pirano and Ljubljana featured "Breathtaking" posters for whole week and in Capodistria for almost the whole month. Two young and talented journalists, Jan Vehar and Urška Krek, provided



invaluable support for the event.

The campaign was really big. There was a lot of talk about it in the newspapers, on national and private TV and on almost all the radio stations. All events were also reported on Facebook.

We are really grateful to all those who helped us and to the European Pulmonary Hypertension association, who recognized the success of our campaign and granted us the 1st prize for the "Best Breathtaking campaign" at the General Annual Meeting in Barcelona in September 2012!

Klara Klančar  
Društvo Za Pljučno Hipertenzijo Slovenije  
<http://www.facebook.com/PljucnaHipertenzija>



# SPAIN

## “WEEK OF THE HEART” ORGANIZED BY THE SPANISH SOCIETY OF CARDIOLOGY

As each year, ANHP had a booth at the celebrations for the “Week of the Heart”. The SPANISH SOCIETY OF CARDIOLOGY organizes a sort of Fair at Plaza de España, in the heart of Madrid, which is open to the general population in order to create awareness about prevention and healthy lifestyle. The ANHP one-day presence on Saturday September 29 is the event that gathers the highest PH patient participation aside from the General Assembly. We took advantage of the event to have a get together of patients from Madrid. Throughout the day, PH patients explained what PH is and how to cope with it to the persons who approached the booth and collected the information contained in the “Breath-taking campaign” brochures.

## PSYCHOLOGICAL SUPPORT

Despite the traditional position claiming that it is the patient who has to take the initiative of contacting a psychologist and pay for the consultation, the ANHP outreach project has yielded very positive results and patients are really satisfied with the proactive approach and the free-of-charge telephone support offered by ANHP. In September it was decided to continue with the project and to extend the services by holding patient workshops, traditional face-to-face consultations, studies on mood/satisfaction relationship and the setting of a wide patient base in order to prepare the grounds for a wider project proposal, as mentioned in the PHA Europe section, to gather evidence on morbidity and mortality related to depression and anxiety.

## COORDINATION WITH OTHER PATIENTS' ORGANIZATIONS

- ANHP attended the 1st working day on treatment compliance organized by the OBSERVATORY OF COMPLIANCE OF TREATMENTS and the GENERAL ALLIANCE OF PATIENTS, which took place on October 3.
- At a later stage, on October 23, we held a working meeting with the CEOs of the OBSERVATORY OF COMPLIANCE OF TREATMENTS and the GENERAL ALLIANCE OF PATIENTS. During the meeting we studied the needs of PH patients and synergies that can be created within these larger structures especially in regard of the need for earlier diagnosis and improvement of detection of PH.
- In the afternoon of October 23, we attended the Madrid Assembly of the SPANISH FEDERATION OF RARE DISEASES where we raised the need to strengthen the capacities of the patients' organizations through training on clinical trials and health technology assessment. We also highlighted the need to change the way we address the issue of access to orphan drugs. Patients usually stress how expensive

the treatments are, whereas the cost of orphan drugs is less than 3% of the national pharmacological expenditure despite the fact that 6 to 8 per cent of the general population is affected by a rare disease.

- ANHP also attended the training on fund raising for Southern European countries organized by the EUROPEAN PATIENTS FORUM in Lisbon, Portugal, on October 25-26. The excellent organization and contents of the training have conducted to a new strategic planning on fund raising for ANHP.
- In implementation of ANHP policies to support patients' organizations in Latin America, its president, Irene Delgado, has been elected to be Member of the Board of the PH Latin Society. She will attend the meeting of Latin Societies in Costa Rica on November 23 and 24 giving lectures conducive to institutional capacity building. She will also attend the scientific event on November 28 in celebration of the Latin PH Day.

## COORDINATION WITH HEALTH AUTHORITIES

- Spanish Ministry of Health  
ANHP had a meeting with the Director General on Disability to advocate in favor of better appraisal of disability for PH patients that will result in smoother access to social benefits. The Director General volunteered to send the PH Call to Action to the Disability Appraisal Centers reaching 3.000 evaluating doctors across the country.
- Regional Health Authorities  
The REGIONAL MINISTRY OF HEALTH in Madrid is preparing a mapping of “Centers of experience” where they will list the hospitals treating rare diseases. ANHP is contesting the criteria because the listing is not based on quality of care but on the mere clinical management of cases, which is misleading for patients that surf the net looking for centers of excellence where they can access better quality of life and life expectancy.

ANHP calls for the designation of Centers of Reference for PH and has initiated an appeal to the health authorities to include PH in the plan for centers of reference that has been approved within the framework of “2013 Spanish Year for Rare Diseases”.

## BOARD MEETING

ANHP's second Board Meeting for 2012 took place on October 27 and 28. There was a very positive evaluation of the growth and activities of the association and the Board made a decision to reinforce two aspects in its strategic planning for 2013: fund raising and communication. Throughout 2013 a strategic fund raising document will be produced, and its implementation will lead to activities conducive

to reinforcing the collection of funds by diversifying the sources so that core funding is guaranteed. Communication will be outsourced in order to have a professional approach and increase the visibility of the association and its activities, spreading the knowledge about PH amongst general practitioners and the population at large. The annual Christmas Lottery fund raising campaign started in October and will finish by the first week of December as usual.

## COOPERATION WITH PH SPECIALISTS

In accordance with ANHP policy of cooperating with medical doctors on the promotion of research in favor of PH patients, we have continued our support to the GENETIC RESEARCH PROJECT lead by 12 DE OCTUBRE HOSPITAL. We have also made some progress, however small, with the BURQOL-RD PROJECT that seeks to quantify the financial burden of PH for patients and caregivers in cooperation with the REGIONAL MINISTRY OF HEALTH of the Canary Islands and 12 DE OCTUBRE HOSPITAL that is working on the involvement of other medical centers with specialization on PH across the country.

- ANHP has also been invited to take part in an interview that will be published in the next Spanish issue of “Advances in Pulmonary Hypertension” edited by PHA.
- ANHP cooperated with the 12 OCTUBRE HOSPITAL on a study on physical and psychological improvement of PH patients receiving homecare by professional nurses compared to a control group. The DOMUS study proved that patients with additional support walked 14 meters more than the control group in the 6 minutes walking test and showed a better result in most aspects that determine quality of life. The results of the study were presented for the first time by Fernando Romero in Evora (Portugal) on October 6.
- Finally we have prepared the grounds for further cooperation with the medical community in new research projects for 2013 regarding pharmacovigilance, pharmacological good practices guidelines at public hospitals and new genetic research projects.

## ACTIVITIES WITH THE PHARMA INDUSTRY

Regular contacts with the pharmaceutical industry are held during the implementation of programs and projects. However, there are some additional activities that are either organized with our partners from the industry or that we attend. During the second semester of 2012, ANHP has been invited and attended the:

- Open Doors Working Day organized by Lilly
- Actelion PH Working Day

Special meetings for 2013 planning and follow up of 2012 activities have been arranged with: Bayer, Lilly, Actelion, VitalAire and GEBRO.

It is important to mention that the DOMUS study has been possible thanks to the special involvement of VitalAire, and ANHP wants to highlight the personal commitment of Mr. Javier González Cappa for his invaluable contribution to the success of the study.



## SPECIAL EVENTS

During the PHA Europe General Assembly, ANHP was awarded a Special Mention for the leadership and organization of the World Pulmonary Hypertension Day celebrated for the first time in Madrid this year. This award is reserved for member associations providing an outstanding service to the community. ANHP is very honored and we wish to take advantage of this activity report to publicly thank PHA Europe for this very special recognition of our work.

Irene Delgado and Juan Fuertes  
Asociación Nacional de Hipertensión Pulmonar  
[www.hipertensionpulmonar.es](http://www.hipertensionpulmonar.es)  
<http://www.facebook.com/hipertensionpulmonar>





## SWEDEN

Step by step our association is making progress. We have had a positive meeting with the Swedish PH Association (professionals working with PH). Hopefully in 2013 we will start working more closely together, both in order to raise awareness but also to generate interest among specialists. In Sweden some PH-centers have difficulties in attracting specialists. As you all know, it is difficult to find people who are willing to actively support the association but now we have appointed our first "PH-contacts". Information about our PH-contacts will be available at the PH medical centers. We are also establishing a co-operation with regional and local associations within the SWEDISH HEART-AND-LUNG ASSOCIATION (an organization we are member of). Heart-and-lung has a great local presence and this co-operation will allow our members to take part in their activities. Finally, we have started working on our new information

brochure, inspired by the materials we brought back from the PHA Europe General Annual Meeting in Barcelona. The tragic story of a family who lost their son to PAH has recently generated some awareness about the disease in Sweden. It all started in mid-November when public service television broadcast a program about the family and their fight with their insurance company. The insurance company argues that a congenital heart defect was the cause of pulmonary arterial hypertension (PAH), which is contradicted by his cardiologist and one of Sweden's most experienced pediatric cardiologists. If you have not clicked "like" on our Facebook page, please do so! Love from Sweden.

Patrik Hassel, PHA Sweden  
<http://pah-sverige.se/> - <http://on.fb.me/WcaOWZ>



## SWITZERLAND-SPHV

### PATIENT COACH PROGRAM

Members of SPHV were invited to participate in a two-part patient program offered by the important Swiss mail-order pharmacy MediService. Meetings were scheduled for the May 15 and November 27. Many of the members took advantage of this very interesting opportunity. The patient coach program was about "Language

and Pictures as a Strategic Means to Deal with the Disease".

### REGIONAL MEETINGS

This year our association organised several regional meetings: May 17 in Chur, August 14 in Zürich, August 16 in Bern and September 11 in Brunnen, which is in central Switzerland. These meetings normally consist of a relatively small circle of patients and accompanying persons, we reserve a table for the group in a restaurant and participants can make useful contacts, share experiences and have interesting discussions over a nice lunch.

### MEETING FOR CHILDREN

On August 25 2012 our association organised a meeting for families with children with PH in Windisch. This town is an interesting place for children because there are well-preserved ruins of an ancient Roman legionary camp. This historical environment provided the "theme" for the children's activities during the stay. The meeting started in the *fabrica*, which was the working room of the Romans. Since the weather was nice, the participants, 10 adults and 13 children, had a picnic under a big



sycamore tree in the garden of the Königsfelden Hospital, not far from the archeological site and the path of the Roman legionaries. After the meal, the meeting was continued under the leadership of our chairman, "Centurius Lucius Aurelius Pilates", who had organized a workshop. The kids learned how to prepare a nice perfumed oil from the herbs picked from the garden, how to make wall paintings and they also heard interesting stories about the time of the Romans in Windonissa (today Windisch).

In the evening everybody had to take their sleeping bag and toothbrush into the legionaries' accommodation area. Centurius organized the evening activities and gave orders for several tasks to be undertaken: wood-chopping, carrying water from a fountain, starting a fire in order to cook for dinner as the Roman legionaries would have eaten in their times. After a very pleasant meal everybody had to do some physical exercise, including marching and fighting. At 10 pm the younger legionaries had to go to bed and the older ones spent a nice evening by the campfire telling pleasant stories and drinking Roman beer. On Sunday everybody had to get up at 8 am. After a legionary breakfast the camp had to be cleaned up. Before leaving the camp a lot of interesting stories and details about the Romans were presented so that everybody could return home trained as an experienced legionary!

### PHEV ANNUAL MEETING

A large delegation of SPHV members traveled to Frankfurt for the three-day patient meeting of the German association ph e.v. which took place October 26-28. The bus travel was sponsored by some pharmaceutical firms. This meeting was not only appreciated for the interesting and useful speeches, presentations and workshops, but also for the networking with other patients and with many specialists in the medical field of PH.

Therese Oesch, SPHV  
[www.lungenhochdruck.ch](http://www.lungenhochdruck.ch)







## SOUTH AFRICA

### SEMINAR “DEMYSTIFIES ORPHAN DISEASE”

“People with pulmonary hypertension (PH) generally have to visit an average of three doctors before they are diagnosed correctly with this chronic and terminal disease, for which there is no cure at this stage”. This is what Denneys Niemandt, Chairperson of the Pulmonary Hypertension Association of South Africa (PHASA) and himself a PH patient, said at the Pulmonary Hypertension Awareness Seminar that took place at the Wanderers Club in Johannesburg on the 3rd of November.

PHASA is a non-profit organisation that was started approximately three years ago. The organisation aims to

raise awareness among the general public, doctors and key role-players in the medical industry of this somewhat unfamiliar, but life-threatening, disease.

“PH was first described more than a hundred years ago,” said Prof. Mohammed Rafique Essop, one of the seminar speakers, who



Mr Denneys Niemandt



Prof. M.R. Essop



Dr. P. Williams, Prof. MR. Essop and their team from Millpark

talked about the diagnosis and evaluation of PH. “Yet the level of unawareness in this day and age is mind-boggling. With this seminar we want to take PH out of the closet; we want to demystify this orphan disease.”

Eastern Times incorporating Northern Times did a feature on PH earlier this year explaining that PH is, in layman’s terms, a disease affecting the arteries of the lungs. People who suffer of PH have to take life-long medication to prolong their lives as there is currently no cure for PH. Some of the other speakers included Dr. Paul G. Williams, a critical care specialist, who spoke on the management of PH in South Africa as well as Dr. Gunther Schleicher, who explained the different “levels” of PH, while Dinkey Cohen discussed the role physiotherapy can play in a PH patient’s life.

More photos are available at: <http://on.fb.me/ZCMNP2>

### FACEBOOK CAMPAIGNS

In recent months PHASA organized two Facebook activities. In November the association invited all PHfriends and PHfamily, all over the world to send their “Letters to God” and post them on the PHASA wall for the INTERNATIONAL PH AWARENESS MONTH (November).

See: <http://on.fb.me/TVVs8A>

PHASA also created an album to collect photos PH groups around the world with their initiatives for the Awareness Month: <http://on.fb.me/Zcuz7W>



Machelene Joubert, PHASA

Pulmonary Hypertension Association of South Africa

[www.phsa.co.za](http://www.phsa.co.za) - <http://on.fb.me/ULLbNc>



## USA

### AWARENESS MONTH AT PHA

Every year in November, people with a connection to PH across the U.S. make a special effort to raise awareness, funds, and support for PH-friendly legislation. The theme for the 2012 Awareness Month was “Sometimes it’s PH,” the early diagnosis campaign that uses zebras to represent the importance of remembering rare diseases when making a diagnosis. In speeches at most of these Awareness Month events, doctors and leaders spoke of the importance of early diagnosis in more effectively dealing with PH. The American Thoracic Society also declared a “Pulmonary Hypertension Week” to spread awareness of PH among doctors involved in thoracic medicine, which could help lead to earlier diagnosis for struggling patients.

On November 13, PHA hosted the 2012 Congressional Luncheon at the Capitol building in Washington, DC. Attendees had the opportunity to learn more about PH and visit their Members of Congress to lobby for more PH research and education. Patients came from twelve states; one group traveled for three days by train across the country! The patients lobbied for and sought co-sponsors for two bills - The Tom Lantos PH Research and Education Act and the Public Health Service Act. The PH Research and Education Act, named for a former Congressman whose granddaughter has PH, supports funding for research at the NATIONAL INSTITUTES OF HEALTH and educational campaigns under both Health Services and CENTERS FOR DISEASE CONTROL AND PREVENTION. The Public Health Service Act supports a National Pediatric Research Network that includes focus on pediatric rare diseases. The day also included moving speeches by PH patients, doctors, and PH Research and Education Act co-sponsor Senator Bob Casey (PA), at a lunch attended by 26 legislative staffers. Many of the lobbying patients reported that their senators and their aides seemed interested in further discussing the bill in the coming legislative year.

Individuals, families, and support groups across the nation also showed their ability to make change at a larger level by getting November officially proclaimed as Awareness Month in their towns and states. As of mid-November, 61 proclamations had been issued, with more than a third of them by one individual. Meanwhile, patients and PHfriends took every opportunity to gain awareness of the issue on television, radio, and newspapers in a media campaign called PHAware. In North Texas, the “Zebra PHfriends” support group made an appearance on the Good Morning Texas television program decked out in zebra regalia (including one full zebra costume!), while another group wore zebra gear and held signs in the background on Good Morning America. Articles about PH and patients’ stories appeared in local and wider-distribution newspapers, and



Group at Congressional Luncheon: A lobby group from Virginia visits Senator Warner's office November 13

people put up exhibits about PH and PHA everywhere from hospital tables to storefront windows.

Also important for those active in the online community, November 1 was declared “Online Awareness Day.” People changed their profile pictures on Facebook and posted about pulmonary hypertension on as many online forums as possible. One group created an event to post pictures of people in the PH community holding signs with their names and dates of diagnosis; participants then decided to make the pictures into a calendar.

Last but not least, people from Florida to California held creative fundraisers to raise money for PHA research and services. Members of the Baltimore support group, the Charm City Breathers, held a walk around the Baltimore American football stadium, a “Vision of Hope” gala (complete with an auction, everybody in their evening’s finest, and high-stepping dancers), and a tour of local bars - “Pulmonary Pints.” Other events included races, “brunches”, a “Zumbathon” exercise fundraiser, and even several “6 minute Marathons” which alone raised over \$10,000 for PHA!

Awareness Month is very important because it helps focus attention on a vastly under-diagnosed and under-exposed disease. It is also important because it unifies the community and gets people involved. As Charlotte McCabe of Washington says, “Because I decided to live and speak out about PH, my life regained its meaning and purpose. What a gift to be grateful for!”



Elsa Dasigo of Hawai'i gets an official Awareness Month proclamation from the Mayor of Honolulu



Diane at Congressional Luncheon: Diane Ramirez lobbies for PH-supportive bills at a Missouri Senate office

Julia Friederich, PHA

[www.phassociation.org](http://www.phassociation.org) - <http://on.fb.me/RIFUOC>





# International survey reveals the hidden but significant impact of PAH on patients and carers

## PHA EUROPE MEMBER ORGANIZATIONS CAN HELP TO IMPROVE THE STANDARD OF CARE RECEIVED

The first survey to explore the full impact of PAH on both the patient and their carers was launched at the PHA Europe General Assembly in Barcelona, Spain to our member organizations. The International Patient and Carer Survey was carried out to provide new insights into the wider impact of PAH on both patients and their carers, beyond the current definition of the burden of the disease. It addressed four areas that are currently not given sufficient attention when considering the effective management of the condition; the practical, the social and the emotional impact on the patient and carer and their information needs.

With your involvement we hope to use the International Patient and Carer Survey Report to help patients with PAH and their carers seek and receive the best standard of care. With the help of our members across Europe, we would like to unite to raise awareness of the report findings and use this evidence to address the current service gaps highlighted in the report.

The findings revealed the major limitations caused by PAH that affected both patients and their carers, including the ability of patients to carry out everyday activities, however small, such as household chores or shopping for food. Furthermore, it highlighted a significant financial impact due to restrictions on the ability of patients and carers to work, leading to many families directly affected by PAH living below average income levels. It was also particularly notable that there was poor understanding of the disease amongst colleagues, friends and even close family members, which led to feelings of social isolation amongst patients. The survey emphasized that a decline in patients' functional class (a classification of severity of the condition) was associated with a greater impact on many of the findings, beyond just the physical limitations of the condition, including the effect on emotional wellbeing, relationships, ability to work and to socialize.

At the General Assembly we put a call to action to our member organizations to launch the survey to the media in their own country, providing an opportunity to reach a wide range of target audiences with the findings from this unprecedented survey. Securing positive coverage around the survey results and report will help to raise awareness of the challenges of living with PAH for patients and carers and hopefully seed the calls to action with policy makers and service providers to work towards a united, standard of care for PAH.

## KEY FINDINGS

The key findings of the survey show that comprehensive care is essential to improve the practical, physical, emotional and social wellbeing of PAH patients and carers. More than half of carers surveyed said PAH had a 'very significant' impact on their daily life.

Employment/work and income emerged as areas of particular concern for both patients and carers as their household income was reduced by PAH, leaving many families with below average household incomes. Just over half of patients and one-third of carers reported feeling isolated, with a major reason being lack of understanding of the disease among family and friends, as well as among the general public. The emotional impact on patients of having PAH included feelings of frustration, feeling misunderstood, anger and low self-esteem.

Many patients reported that their sex lives were severely affected, as did carers who were partners of PAH patients.

Nearly one-quarter of carers felt stressed caring for their relative and trying to meet

their other responsibilities, which increased with decline in the patient's functional class.

Despite the major social, financial and emotional impact of PAH, patients and carers said they had been given little information on these aspects by healthcare professionals, with patient organizations emerging as the major sources of information and support.

Chart 1. How patients assess the impact of PAH on their daily lives

56% OF PATIENTS CONSIDER PAH TO HAVE A "VERY SIGNIFICANT IMPACT" ON DAILY LIFE

It appears that the impact of PAH on daily life is particularly important in FC1v patients (90% giving a score of 8-10 out of 10)

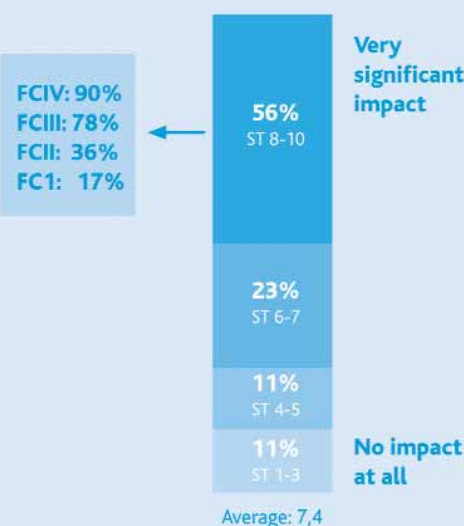
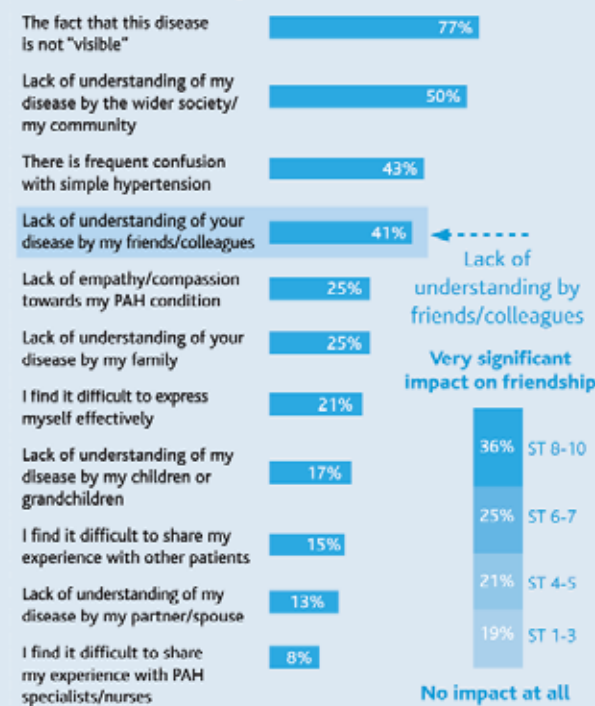


Chart 3. Causes for feelings of isolation as a result of PAH / impact on friendships

LACK OF VISIBILITY OF THE DISEASE APPEARS TO BE THE BIGGEST CAUSE OF THE SENSE OF ISOLATION

### Causes of isolation feeling



As a result calls to action have been put to healthcare professionals (HCPs), patient organizations, carers and patients to help address these issues, a summary of which is below:

- HCPs should extend assessment and care beyond the current defined symptoms of the disease to include the practical, physical, emotional and social impact showing understanding of the wider impact of PAH, in particular the financial impact it has on patients and carers;
- Patients and carers should be offered a comprehensive, coordinated care from a multidisciplinary team of HCPs and other specialists, including physicians, nurses, social workers, psychologists and patient organizations;
- The needs of the carer are currently underestimated and often overlooked; their needs should be addressed as part of the patient's multidisciplinary package of care;
- Patients under specialist care and their carers should be directed to patient groups to gain further support and access to the PAH community ensuring the timely provision of appropriate PAH information on a range of topics beyond only the physical impact, including patient/carers information resources on financial, emotional, social and physical impacts of PAH, symptom awareness campaigns and patient referral to appropriate patient support groups at specific times in disease progression.

## INFORMATIONAL NEEDS

The report highlighted that patient organizations have an important role to play in the provision of information to patient and carers and that earlier referral by HCPs to patient groups would be beneficial. PHA Europe is currently working to develop a range of materials to help address this gap in informational needs. At the recent launch meeting, our member organizations provided their advice and guidance into the development of these materials to ensure they were appropriate for their target audience. The materials will include:

- **Patient dialogue tool and checklist**

The dialogue tool and checklist enables the patient to "self-assess" how they may be affected by the wider impact of their disease beyond the current definitions of PAH. They

can then use this as a tool to initiate discussions with their HCP and seek further support;

- **Carer dialogue tool and checklist**

The dialogue tool and checklist enables the carer to "self-assess" how they may be affected by the wider impact of the disease when caring for a patient with PAH. They can then use this tool to support them with the care of someone living with PAH, to initiate discussions with both their partner and HCP and to seek further support;

- **Healthcare professional referral letter of patients to patient organisations**

The referral letter should be given to patients/carers diagnosed with PAH by their HCP to refer them to the

national patient organization, who can provide additional assistance and support i.e. financial and social support as well as addressing any further information needs.

The materials are currently being finalized and will be shared with you once complete. We hope you will find these useful to provide to your members to help support them with their PAH journey.

## LAUNCHING THE SURVEY IN YOUR COUNTRY

To help support you in communicating these compelling findings to the media and start raising awareness of the challenges of living with PAH and to call for an improvement in the standard of care for PAH patients and carers, a media toolkit containing guidelines and template materials was provided to member organizations at the report launch

on the 14th September 2012 and is also available from our website at [www.phaeurope.org](http://www.phaeurope.org), or a copy can be requested by email at [p.ferrari@phaeurope.org](mailto:p.ferrari@phaeurope.org)

Key points for you to consider when planning your local survey launch include:

- **Be sure to convey the key messages from the survey**

These should include the aim of the survey, a summary of the findings and our call to action for better informational needs and a more comprehensive standard of care;

- **Target your audience**

It is important to identify the right people ("target audience") to communicate with through the media to ensure your key messages appear in the right place. Your





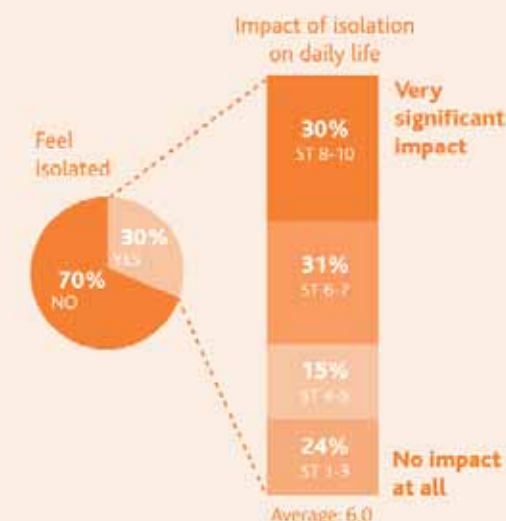
Chart 7. Changes to carers lives as a result of PAH

PAH LEADS TO MANY CHANGES TO CARERS' LIVES, WITH EXTRA TASKS, PLANNING AND RESULTANT EXHAUSTION



Chart 8. Feelings and impact of PAH on carers

1 IN 3 CARERS FEEL ISOLATED/EXCLUDED AS A RESULT OF CARING, WHICH IMPACTS ON THEIR DAILY LIFE



target audience may include other patient organizations, patients, carers and their families and friends, HCPs, politicians and healthcare policy makers;

- **Tailor your message**

There are different channels of media to use such as print, broadcast and online sectors. Your message should be tailored specifically to each sector and publication to ensure they receive the right information and are encouraged to cover your story;

- **Package your story**

To maximise your chance of coverage you can produce a press pack which can include a range of materials including a press release, fact sheet and case studies etc. Template of these materials are available in the toolkit;

- **Launch the story**

Once you have created your press materials you can “sell-in” your story to the media or hold a press conference. The toolkit includes tips on how to conduct a sell-in or press conference to ensure you get maximum coverage.

### SUPPORT FOR YOUR LOCAL LAUNCH

A grant is available from PHA Europe to assist you with your launch of the International Patient and Carer survey and for communicating the call to action. The grant can be used for costs towards translation, printing, press releases etc., if needed.

Proposals should be submitted to Pisana Ferrari by January 31, 2013 at [p.ferrari@phaeurope.org](mailto:p.ferrari@phaeurope.org)

### ACCESSING THE SURVEY REPORT

A copy of the survey report and calls to action can be downloaded from our website at [www.phaeurope.org](http://www.phaeurope.org)

A summary of key findings are also available on our Facebook page and PH support forums.

### SUPPORTING THE WHITE PAPER

PHA Europe will also be producing a white paper executive summary of the survey findings to support their White Paper report “Addressing the unmet need of persons living with Pulmonary Hypertension; a call to action”. This will also be available on our website shortly.

We look forward to continuing to work with you to raise awareness and understanding of PAH and its impact on the patient and carer. With your help we can aim for a more comprehensive and higher standard of care and improve both the patient and carer's experience of living with PAH.

*The survey and report were developed under the auspices of a steering committee of PAH specialists and representatives of PAH patient organisations supported by Actelion Pharmaceuticals Ltd, Switzerland. The market research agencies involved in the surveys were commissioned by Actelion.*

## MACINTENTAN SERAPHIN STUDY

The results of the SERAPHIN Phase III long-term outcome study with Macitentan (Opsumit®) in patients with pulmonary arterial hypertension (PAH) were presented by Lewis J. Rubin, M.D. on October 23 at the 2012 annual CHEST meeting of the American College of Chest Physicians (ACCP) in Atlanta, USA. Preliminary data had been presented at the European Society of Cardiology (ESC) and European Respiratory Society (ERS) annual congresses in August-September 2012. Macitentan (Opsumit®) is a novel dual endothelin receptor antagonist (ERA). SERAPHIN is a long-term, event-driven study involving 742 patients in 151 centers from 40 countries (North and Latin America, Europe, Asia-Pacific and Africa) treated for up to three and a half years with Macitentan. Patients were allowed to receive PAH background therapy throughout the study, either PDE-5 inhibitors or oral/inhaled prostanoids.

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

Macitentan (Opsumit®) submissions to healthcare authorities: *European Medicines Agency (EMA):*

On November 22, Actelion announced that the submission of the Marketing Authorisation Application (MAA) for Macitentan (Opsumit®) for the treatment of patients with PAH to the EMA has been accepted. The EMA will now start the formal review process.

*Food and Drug Administration (FDA):*

On October 22 Actelion announced that it had submitted a New Drug Application (NDA) to the FDA seeking approval for Macitentan (Opsumit®) in patients with PAH. On December 14 Actelion announced that the FDA had accepted the NDA for Macitentan. The review period is expected to last 12 months from the date of the submission.

## RIOCIGUAT PATENT-1 AND CHEST-1

Bayer HealthCare presented the results of two recent Phase III trials related to its investigational drug Riociguat on October 23 during a “Late Breaking Abstracts” session at the CHEST Congress 2012, the annual meeting of the American College of Chest Physicians (ACCP) in Atlanta.

Riociguat, discovered and developed at the Bayer research laboratories, is an oral soluble guanylate cyclase (sGC) stimulator. It works through the signaling pathway of nitric oxide (NO) 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 PAH.

PATENT-1 is a randomized, double-blind, placebo-controlled, multi-national program with active centers in 30 countries. In the study, 443 patients with symptomatic PAH were randomized and treated with either placebo or two different doses of Riociguat orally over a period of 12 weeks. The Riociguat dose was titrated over a period of eight weeks in doses of 0.5 mg increments from 1.0 mg up to 2.5 mg, three times daily. PATENT-1 evaluated Riociguat in both treatment-naïve patients and those pre-treated with an endothelin receptor antagonist or an oral, inhaled or subcutaneous prostanoid. The study met its primary endpoint by demonstrating a very significant improvement in the six-minute walking distance. Statistically significant improvements were also observed across secondary endpoints including pulmonary vascular resistance, N-terminal pro-hormone brain natriuretic peptide, WHO functional class, time to clinical worsening and Borg dyspnea score.

CHEST-1 is a trial evaluating Riociguat in patients with inoperable chronic thromboembolic pulmonary hypertension (CTEPH) or with persistent or recurrent pulmonary hypertension (PH) after surgery. Results showed that the CHEST-1 study met its primary endpoint by demonstrating a statistically significant improvement in the six-minute walking distance. Lead investigator, Professor Hossein Ardeschir Ghofrani, University Hospital Giessen and Marburg, Germany, said that “results from the Phase III development program suggest that Riociguat may be a potential new option in CTEPH”.



## HORIZON 2020 UPDATE OF DEVELOPMENTS



At the end of 2011, the European Commission published a proposal entitled 'Horizon2020', which is a comprehensive package of measures to boost research, innovation, and competitiveness in Europe. This will fund EU research on a wide range of topics and has a total budget of 80 billion Euro. The programme will run from 2014 to 2020. Horizon 2020 brings together all EU research and innovation funding under one single programme and will focus on a wide range of different research areas, including health, demographic ageing and well-being.

A first proposal for the overall framework of the Programme is currently being discussed in the Council of Ministers and the European Parliament with a view to adoption before the end of 2013. Next year, specific proposals for each of the research areas - including health - will be formulated. The European Parliament Committee on Industry and Research voted its draft report on the Programme on November 28.

## EUROPEAN REFERENCE NETWORKS (ERN) PUBLIC CONSULTATION ON IMPLEMENTATION

The Directive on the application of patients' rights in cross-border healthcare requires the European Commission to support Member States in the development of European Reference Networks between healthcare providers and centres of expertise. The main value of the European Reference Networks and the Centres of Excellence is to facilitate improvements in access to diagnosis and delivery of high-quality,

accessible and cost-effective healthcare in the case of patients who have a medical condition requiring a particular concentration of expertise or resources, particularly in medical domains where expertise is rare. European Reference Networks could also be focal points for medical training and research, information dissemination and evaluation, especially for rare diseases.

DG Health & Consumers is launching a public consultation targeted to stakeholders on the implementation of European Reference Networks (ERN). A concept paper and questionnaire have been prepared to guide the consultation.

Target groups for the consultation:

- Patient organizations, health professionals' organizations and healthcare provider's organizations.
- Healthcare providers and centres of excellence, academic and public health and healthcare specialized institutions.
- Public authorities and government-appointed bodies responsible or involved in the definition of criteria and the establishment and evaluation of centres of reference/excellence and reference networks of centres providing highly specialized healthcare.

The public consultation invites stakeholders' opinions and contributions based on evaluated experiences, regional or national models, technical and professional standards, criteria or recommendations which could provide inputs and facilitate the definition of technical and quality criteria (scope, general and disease specific elements).

Contributions to the consultations can be made by means of a questionnaire which is to be completed online. A preview/print version can help to prepare the response. A report will be provided after closure of the consultation. The deadline for submissions is January 24, 2013. View questionnaire at: <http://ec.europa.eu/yourvoice/ipm/forms/dispatch?form=ern&lang=en>

## SUPPORTING RARE DISEASES REGISTRIES EUROPEAN PLATFORM

The EU has recommended that Member States should consider supporting specific disease information networks as well as registries and databases. Patient registries and databases constitute key instruments to develop clinical research in the field of rare diseases, and support the improvement of patient care and healthcare planning. They are a useful way to pool data in order to achieve a sufficient sample size for epidemiological and/or clinical research and they are vital to assess the feasibility of clinical trials, to facilitate the planning of appropriate clinical trials and to support the enrolment of

patients as well as for the post-marketing surveillance of orphan medicinal products. The creation of a registry can be a powerful tool to create and structure networks of experts, whether they being European Reference Networks of Centres of Expertise or national expert networks for RD. In either case, the experts and centres of expertise involved are a primary source of data for registries.

The strategic objective of the European Commission is the creation of a European Platform on Rare Diseases Registration providing common services and tools for the existing (and future) rare diseases registries in the European Union. For an overview of existing registries in relation to rare diseases:

[http://ec.europa.eu/health/rare\\_diseases/policy/registries/index\\_en.htm](http://ec.europa.eu/health/rare_diseases/policy/registries/index_en.htm)

## STRENGTHENING PUBLIC HEALTH SERVICES AND CAPACITY WHO EUROPEAN ACTION PLAN



On November 9, on the occasion of the annual conference of the European Public Health Association (EUPHA), held in Malta, the WHO European Action Plan for Strengthening Public Health Services was launched. This outlines a way to strengthen public health in countries across the WHO European Region, and forms a key pillar of the implementation of 'Health 2020', the new European policy framework endorsed by the WHO Regional Committee for Europe in September 2012. Dr. Hans Kluge, Director of the Division of Health Systems and Public Health at WHO/Europe, presented the WHO's plans to modernize public health to address widening inequalities and the increasing challenges of the 21st century. With an ageing population and increasing chronic disease, countries must develop and strengthen

services for disease prevention and health promotion, while ensuring robust health protection services to address risks to health from pandemic influenza and climate change. Current spending on prevention across the WHO/Europe Region is estimated to average 3% of total spending only. However, spending on prevention is a cost-effective way to improve health outcomes. For example, prevention initiatives account for 50-74% of the decline in coronary heart disease morbidity in a range of high-income countries. The Action Plan contains 10 public health operations (EPHOs) that countries can adapt and work on together with WHO technical leadership and support, to assess and plan stronger public health services and capacity.

For the Action Plan see: <http://bit.ly/W5mslX>

## ORGAN TRANSPLANTATION DIRECTIVE ON BETTER INFORMATION

On October 9, the Commission adopted a Directive to encourage the cross-border exchange of information on organs and their donors. This new Directive establishes three types of procedures:

- Rules for the transmission of information on the organ and donor (type of organ and donor's age, sex and medical history).
- Provisions for the transmission of information to ensure the traceability of organs, in compliance with confidentiality and data security measures.
- Procedures to ensure the reporting of any serious adverse events or reactions.

The new Directive also establishes the obligation for national authorities to exchange and store information on cross-border organ exchanges and to provide a 24/7 service in case of serious adverse reactions or events. It will enter into force 18 months after its publication.

For more information see:

<http://bit.ly/Tu0kmY> and <http://bit.ly/RdBuZd>

## ORGAN DONATION JOURNALIST WORKSHOP

A third workshop on organ donation was held for journalists on October 9 in the Commission building, with the aim to raise awareness of some of the issues and challenges relating to this important health field. The Commission published some highly relevant background material as part of the





briefing for journalists, including a facts and figures brochure. Presentations were made by the Commission staff including:

- Stefaan van der Spiegel, Teamleader 'Substances of Human origin (DG SANCO), providing an introduction into organ donation and transplantation.
- Helene Le Borgne, Policy Officer for Organ Donation and Transplantation (DG SANCO), giving an overview of EU level activities in the field of organ donation.

Other high level Commission staff were involved as well, such as Andrej Rys (Director Public Health) and John Dalli (Health Commissioner)\*. The programme also included presentations on ethical consideration, family involvement and how to increase public awareness about the critical need for organ donations.

All papers and background documents can be found here: [http://ec.europa.eu/health/blood\\_tissues\\_organ/events/journalist\\_workshops\\_organ\\_en.htm#fragment0](http://ec.europa.eu/health/blood_tissues_organ/events/journalist_workshops_organ_en.htm#fragment0)

(\*) John Dalli recently resigned following an investigation by OLAF, the EU's anti-fraud office. OLAF was investigating a complaint made in May by Swedish Match, a tobacco firm, which alleged that a Maltese entrepreneur had tried to affect legislation.

Sources: Europa website, Euractiv, Europolitics, various NGO and academic sites.

Christine Marking

## NEW HEALTH COMMISSIONER TONIO BORG (MALTA)

Dr. Tonio Borg became Commissioner for Health and Consumer Policy on November 30, 2012, replacing John Dalli. This appointment comes after the official approval of Member States' representatives, on November 28 and the European Parliament's support on November 21.

Dr. Tonio Borg was Deputy Prime Minister and Minister for Foreign Policy in Malta, having worked in Malta his entire life and being in a Ministerial post since 1995, following a career in law. His mandate as Commissioner for Health and

Consumer Policy will be till the end of his Commission term, in 2014.

## E-RARE 5TH JOINT CALL FOR EUROPEAN RESEARCH ON RARE DISEASES



The aim of this call is to enable scientists in different countries to build an effective collaboration on a common interdisciplinary research project based on complementarities and sharing of expertise.

Topics:

1. Collaborative research using patient databases and corresponding collections of biological material that would generally not be possible at a national scale.
2. Research on rare diseases including genetic, epigenetic, and pathophysiological studies.
3. Research on development of applications for diagnosis and therapies for rare diseases.
4. Patient oriented research in the area of social and human sciences eg. psychological, psychosocial and behavioural research - as well as health services, health economics and health policy research and research on ethical, legal and social issues in the field of rare disorders.

More details available at:

<http://www.e-rare.eu/joint-call/5th-joint-call-european-research-projects-rare-diseases-jtc-2013>

Pisana Ferrari

### Next PHAE newsletter

The next Mariposa Journal will be issued in June 2013 (Summer edition). The main focus will be the celebrations for World PH Day on May 5. It will also feature reports from the national associations about activities in their countries, updates on research and treatments and any other interesting and relevant developments at European level.

We would be very grateful if the members could send their contributions by **the end of May at latest**. These may be sent in the national language and we will provide for translation. Any photos should have a minimum resolution of 1 MB. The newsletter will be available in PDF format and in a printed version. The PDF will be posted on the PHA Europe website. Printed copies will be sent by normal post to the national associations.

Many thanks in advance!

# 2013

Nice, February 27/28 - March 1

### 5th WORLD SYMPOSIUM ON PULMONARY HYPERTENSION

The PH World Symposia started in Geneva in 1973 and have been held every 5 years after the Evian edition in 1998. The symposia have marked the main progresses in pulmonary hypertension science and paved the way for further advancements. The Symposia proceedings constitute relevant publications which are among the most cited in this scientific area. PHA Europe has been invited give its patronage to the meeting and PHA Europe President and other representatives will be attending.



May 5

### WORLD PH DAY

World PH Day was initiated by the Spanish PH association ANHP with a landmark event in Madrid on May 5, 2012. PHA Europe and its member associations, PHA and other PH patient associations around the world will be uniting their efforts to celebrate World PH Day 2013, with national awareness raising activities and events that will be coordinated, shared and promoted via a common web platform.



May 30/31 - June 1, Dubrovnik, Croatia

### TRANSATLANTIC FORUM AND EURORDIS MEMBERSHIP MEETING

This FORUM of exchange, organized by EURORDIS and NORD, aims to sustain a strategic collaboration between both sides of the Atlantic (EU and Canada/US) and to better position rare diseases on the international scene. The meeting will bring together policy-makers, health professionals and academia, patients and patients representatives and industry representatives. PHA Europe will be attending the forum as well as the membership meeting.



August 31 - September 4, Amsterdam, The Netherlands

### 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 7-11, Barcelona, Spain

### EUROPEAN RESPIRATORY SOCIETY'S ANNUAL CONGRESS

PHA Europe will also be present with a booth at the EUROPEAN SOCIETY FOR RESPIRATORY MEDICINE'S ANNUAL CONGRESS 2012. This Congress involves not only leading experts in respiratory medicine, but also in the entire spectrum of research and practice.



September 11-15, Barcelona, Spain

### PHA EUROPE GENERAL ANNUAL MEETING

PHA Europe will be holding its 2013 GENERAL ANNUAL MEETING in Castelldefels (Barcelona) September 11-15. PH Patient Leaders from 20 European countries attended the 2012 meeting (see photo on left).

October (date to be defined), Sophia Antipolis, France

### PH EDUCATIONAL COURSE

This yearly international course has as its target audience mainly cardiologists, pulmonologists and other specialists involved in PH care. PHA Europe is regularly invited to attend and to present the patient perspective in the context of the G6, a meeting which brings together the major stakeholders in PH.







## EU Health programmes 10 YEARS ANNIVERSARY



This is a recent promotional video created to celebrate 10 years of the EU Health Programmes. Starting with the Treaty of Maastricht in 1992 and the Title X on Health, these programmes have gradually increased over the years from the provision of health information, health promotion and reaction to health threats to a more active role in coordination and guidance of national policies. The video lists the various areas in which the EU has been active over the past years, the financial resources employed and projects accomplished (over 350).

<http://app.sliderocket.com/app/fullplayer.aspx?id=231A3C76-0CDD-81DD-6EFF-E8F6C4FBB100>  
[http://ec.europa.eu/health-eu/videos/index\\_en.htm](http://ec.europa.eu/health-eu/videos/index_en.htm)

## EU Health and rare diseases DIRECTORATE GENERAL FOR HEALTH AND CONSUMERS



This video created by the Directorate General for Health and Consumers and Executive Agency for Health and Consumers focuses specifically on rare diseases. It describes how the EU is funding a number of programmes to support the networking between patient associations, including EURORDIS and ORPHANET.

[http://ec.europa.eu/health/rare\\_diseases/videos/videos/rare\\_en.wmv](http://ec.europa.eu/health/rare_diseases/videos/videos/rare_en.wmv)

## EU Patient rights CROSS BORDER HEALTHCARE



This video gives an overview of the EU legislation that clarifies the right for patients to be treated in another EU country and to be reimbursed for it (Directive 2011/24/EU on patients' rights in cross border healthcare). The directive is part of a package of measures under the Renewed Social agenda. It sets out responsibilities for Member States and healthcare providers, and promotes co-operation between health systems to provide better access to specialised care.

[http://ec.europa.eu/dgs/health\\_consumer/video/healthcare\\_without\\_barriers\\_long.wmv](http://ec.europa.eu/dgs/health_consumer/video/healthcare_without_barriers_long.wmv)

## Medicines for Rare Diseases ROLE OF EMA



This video describes the main steps in the procedure that leads to the approval of drugs for rare diseases in Europe. The European Medicines Agency, founded in 1995, is a decentralised agency of the European Union, located in London. The Agency is responsible for the scientific evaluation of applications for European Union (EU) marketing authorisations for human and veterinary medicines in the centralised procedure. Under the centralised procedure, pharmaceutical companies submit a single Marketing Authorisation Application (MAA) to the Agency.

<http://www.youtube.com/watch?v=z-5UTc0OIHQ>

Pisana Ferrari

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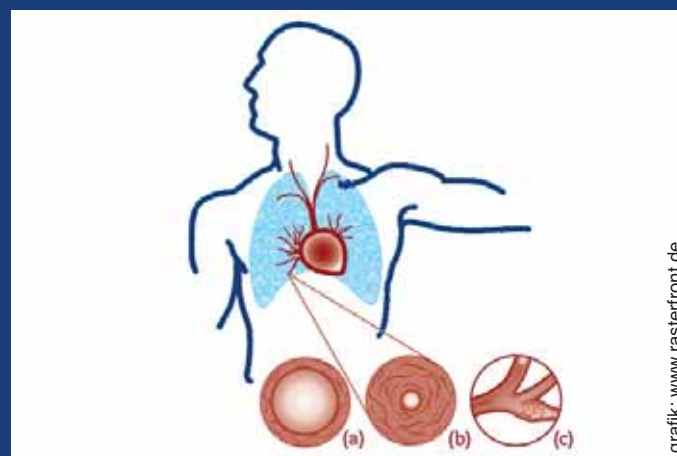


## AIMS OF PHA EUROPE

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.