MariposaNews

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

- **Austria**
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Dear friends,
first of all I would like to thank you again for electing me President of PHA Europe. I am honoured by the trust you placed in me and look forward to working closely with the Board and Members of PHA Europe in promoting the cause of Pulmonary Hypertension patients in Europe.

I am delighted to introduce the first issue of our newsletter which will be circulated in PDF format to our members and other interested parties.

This issue includes a report on our recent elections and biographical notes of the new Board, news from PH patient associations in Europe and around the world, updates on PH treatment and research, information about recent conferences and European Union policy and NGO activities in PH and related areas (rare diseases, orphan drugs, organ donation and transplantation). The newsletter also includes a summary of upcoming events for the year and a section on useful links and reading suggestions.

2010 is already proving a very interesting and challenging year for our association.

We will take part to both the European Society of Cardiology (ESC) and European Respiratory Society (ERS) Annual Congresses, respectively in Stockholm at the end of August and in Barcelona in mid September.

We are going to hold our Annual General Assembly within the context of the ERS Congress, from 18th to 20th September, and are very pleased with the exceptional turnout, 40 confirmed delegates from 18 European countries. The General Assembly has a very interesting and wide ranging program and we will be holding our first corporate meeting with representatives of pharmaceutical industries.

There are many positive developments at European Union level, which we will be monitoring closely. In particular, we are very pleased that the Council Recommendation on European Action in the field of Rare Diseases will provide more focus on this entire field. We also welcome the new trend towards closer involvement of patient associations in the European Committee of Experts on Rare Diseases (EUCERD) and in the European Medicines Agency and look forward to new developments with regard to improving access to orphan drugs for patients in Europe.

I would like to thank our member associations and their leaders for their precious input without which this newsletter would not have been possible and Pisana Ferrari for putting it all together and editing the texts as well as for her contributions on EU and NGO activities.

I look forward to your comments and to working with you.

Gerry Fischer
President

Contents

PHA EUROPE

PHAE elections and new Board 2010-2012

Elections for the new Board of PHA Europe were held in January 2010. PHA Europe wishes to thank Past President Bruno Kopp and the other members of his team for the work carried out in the two years of his Presidency and is delighted to introduce the new Board of Members, composed of extremely qualifed and dedicated individuals who are very active at national level in providing invaluable support to Pulmonary Hypertension patients and their families. All have been affected, either directly or indirectly, by Pulmonary Hypertension.

PRESIDENT AND VICE TREASURER

Gerry Fischer

University studies in world trade. Lives in Vienna where he works in tourism and, since 2001, manages the family liquor and schnaps business. Married and father of a 21 year old boy and a teenage girl with PH. Founding Member and President of the Austrian PH association.

1ST VICE PRESIDENT

Pisana Ferrari

Political Science graduate. Has worked for EU institutions and consultancy firms and then in event management. Lives in Milan. Has a 21 year old daughter. Diagnosed with PH in 1988, underwent double lung transplant surgery in 2002. Founding Member and President of AIPI, Italian PH patient association.

2ND VICE PRESIDENT

Umit Atli

Chemistry graduate. Has been working for the Turkish Prime Minister's Press and Public Relations Office as a consultant since 2000. Lives in Ankara. Diagnosed with PH in 2001, he underwent double lung transplant surgery in 2007. Founding Member and President of the Turkish PH patient association.

SECRETARY

Luisa Sciacca della Scala

University studies in Languages and Literature. Married and mother of three grown up children, lives and works in Rome. Has been involved in PH since 1998, when her close friend Maria Pia Proia was diagnosed with PH and when in 2000 she founded AMIP, Italian Patient association. President of AMIP.

VICE SECRETARY

Ebru Arisoy

Economics graduate. Has been working for Undersecreteriat of Foreign Trade since 1996. Lives in Ankara. Has been involved in PH since 2008 when she was working for the Turkish Embassy in Vienna and she met Umit Atli, an old school friend, recovering from lung transplant surgery, and other PH patients. Member of the Board of Turkish PH patient association.

TREASURER

Sylvain Reydellet

Engineering studies and Master in Management. Lives in Lyon and works as Managing Director of a company which produces Air Conditioning systems for special vehicles. Diagnosed with PH in 2004 he started collaborating with the French PH association in 2006. Current President of HTAP France, the French patient association.

AUDITOR I

Iris Tal

PhD in Biology and Homeopathy. Lives in a small town near Haifa. Married and mother of two girls. Diagnosed with PH in 1999. Member of the Board of the Israeli PH patient association.

AUDITOR II

Mélanie Gallant Dewawrin

Lawyer and Business School Professor. Is married and has a young daughter. Lives in Beaune, near Dijon (Bourgogne/Burgundy). Was diagnosed with PH as a child. She was on Flolan treatment for 16 years before double lung transplant surgery in 2009. Past President of the French PH patient association, she is currently Managing Director.

During the 2009 General Assembly held in Paris in September, there was a discussion about future activities and strategies for PHA Europe and it was felt that PHA Europe would greatly benefit by having a European coordinator working for the association.

The decision was taken to employ someone for the position and the name of Pisana Ferrari was put forward. Pisana Ferrari, who is also first Vice President, will be working in close contact with the President and the Board, will maintain contacts with the members, coordinate common activities and projects, organize meetings and participation in conferences, take charge of the web site, newsletter and printed material and general organization of the association.

AUSTRIA

Awareness campaign

Vienna's bus stops last January were plastered with billboards from the awareness campaign promoted and organized by the Austrian PH Association. The campaign deliberately included very powerful and provocative images of women, men and children standing with one foot in a coffin whilst carrying our every day activities (see photos). This campaign aims to convey the message that there are many people who risk dying, without even knowing it! Shortness of breath can be a symptom of a very dangerous disease, PH. By increasing awareness of the disease, the Austrian Association hopes to encourage more people with breathing problems to see their doctor and ask if their symptoms are consistent with PH. As we know, early diagnosis and treatment can make all the difference. The campaign provoked a lot of comments and even some criticism, but it certainly reached its objective, that is to raise awareness of PH.

Patient meeting

PHA Austria held a PH patient meeting on the 22nd of January in Vienna. Special guests included Prof. Irene Lang, cardiologist and PH specialist, and Prof. Walter Klepetko, world renowned lung transplant surgeon, both at the AKH, Vienna General Hospital, and Prof. Martin Langer, gynaecologist, who spoke about PH and women issues.

From left: Eva Huetter, Prof Walter Klepetko, Dr. Irene Lang. Third from right Prof. Martin Langer next to Gerry Fischer

Wiener Philarmoniker

As part of the events of the Vienna Marathon, the Wiener have given to the *Initiative Heilung für Lungenhochdruck* a number of tickets for their concert of the 15th April in the Wiener Konzerthaus. Proceeds from the ticket sale were entirely for the benefit of the Initiative.

Photos from other fundraising events: Circus Roncalli PH Gala in front of the Vienna City Hall (Gerry and his daughter Maleen with Bernhard Paul, ringmaster)

Maleen becomes a little Fashionista and was able to meet with Karl Lagerfeld, Philipp Plein & Tang de Hoo

Billboards in Vienna with PHA Austria's latest awareness campaign

News from European PH patient associations

BELGIUM - HTAP BELGIQUE

HTAP Belgique's President at Eurordis

HTAP Belgique's President, Rosie Matthysen, contributed to raising awareness of PH by recently sharing her personal experience as a PH patient with the European Association for Rare Diseases. Her story features in six languages on the Eurordis website at: www.eurordis.org/content/pulmonary-hypertension-courage-start-new-life-50-rare-disease

EURORDIS

The Voice of Rare Disease Patie

LIVING WITH A RARE DISEASE

Participation at scientific events

HTAP Belgique has recently taken part in a workshop organised by the *Cellule d'Education Permanente des Médecins* (Belgian ongoing education for medical professionals) on the safe use of pharmaceutical products. It has also participated to the Rare Disease Day organized by the Belgian Ministry of Health for physicians, nurses and patient representatives.

Master Classes

Erasme University regularly organizes "Master Classes" on PAH for medical professionals (cardiologists, pneumologists, rheumatologists) from all over the world. The training program includes a two hour PH patient-led workshop organized by HTAP Belgique.
Through the workshop the patients association has contributed to the training of professionals from Venezuela, Greece, Spain, Italy. Its work was greatly appreciated and, in particular, the *syllabus* written by a PH patient with information about the disease in every day life of patients.

Good news from Hôpital Erasme

Hôpital Erasme-ULB Brussels has created an independant *Clinique d'Hypertension Pulmonaire*, headed by Pr. Jean-Luc Vachiéry, fully and only dedicated to PH patients.

Other Belgian hospitals

A number of Belgian hospitals have a *Cellule Hôpital-relais* HTAP for PH patients. HTAP Belgique representatives often visit PH patients in these hospitals.

BELGIUM - VZW

New President

The Flemish Belgian patient association has a new President since April 2009, Mr Gust Caelen. We are very happy that our friend and past President Hendrik Ramaker will continue to represent the association in PHA Europe.

Patient to patient meetings

Meetings were held in February in five regions of Flanders (Antwerp, Brussels, West-Vlaanderen, Oost-Vlaanderen and Limburg) to give the opportunity to patients to talk to each other in a very informal context, without doctors or other medical professionals present.

New Patient Day

In May all new patients will be invited to a meeting where they will be provided with basic information about the disease and the patient organisation and its activities.

Photos from the patient meeting in Limburg

BULGARIA

New Member

PHA Europe is very happy to welcome the Bulgarian PH patient association as a new member. The President is Mr Todor Mangarov. The organization was set up at his initiative at the beginning of 2009 and is a part of National Alliance of People with Rare Diseases in Bulgaria.

In the last months of 2009 Mr Vladimir Tomov, Chairman of the National Alliance for Rare Diseases, launched a campaign to ensure that treatment for PH be paid for by public funding.

Future plans

Mr Mangarov is planning to hold a PH patient meeting in mid May in Plovdiv, with the support of the National Alliance for Rare Diseases and the Information Centre for Rare Diseases and Orphan Drugs. He is also planning to set up a website in order to increase awareness of the diseases and provide information to patients.

CZECH REPUBLIC

New President and Board

The Czech Association has a new Board as of February 2010. The President is Katerina Novakova, the Vice-President is Veronika Schächterova. Other members of the Board are Dr. Josef Oškera and Jiří Buneš. Andrea Hýblova is the manager.

Annual Congress of the Czech Society of Cardiology

The Czech association has been taking part in the Annual Congress of the Czech Society of Cardiology as an exhibitor since 2008.

This year's Congress is scheduled in Brno from 16 to 19 May. The Congress is a very important opportunity to interact with doctors, nurses and PH specialists. The association will be distributing brochures and other material and thus contributing to spreading awareness about the organization.

Shiri Ben Artzi at the 2009 General Assembly

Patient weekend and General Assembly

Each year the Czech association organizes a weekend for its members.

The General Assembly is held during the weekend. This year's event is scheduled is from 29 to 30 May 2010 in Seã. There will be lectures on PH diagnosis and treatment, surgery and rehabilitation programs. Patients will take part in workshops where they can discuss their problems. The Saturday evening after the first part of the conference is always free and patients may, if they wish, relax and enjoy beauty treatments in the wellness area.

Physical rehabilitation program-stay

The third fitness/rehab program for PH patients will be organized during the summer of 2010 in Podebrady with the support of the Czech Government. Physical rehabilitation will represent the main part of the program which brings together doctors and physiotherapists cooperating with the association.

Photos from 2009 rehab program

Publications

A brochure on "PH - diagnosis and treatment" has recently been printed and another is planned on the social and legal aspects of living with PH.

FRANCE

President
Sylvain Reydellet

Annual General Assembly

HTAP France held its Annual General Assembly on the 13th of March in Paris. About 110 people attended the meeting, including patients, family members and healthcare professionals. Items on the agenda included elections of the new Board and a break-out session with medical professionals. The President presented the Annual Report with details of 2009 activities and program for 2010. The General Assembly also voted on the 2009 financial report. The meeting ended with drinks and snacks and time for socializing.

Second from left Prof. Gerald Simonneau, fourth from left Prof. Marc Humbert, third from right Dr Xavier Jaïs

Study on PH patients and families, March

A study on PH on everyday life of patients started in March. It will cover all aspects of the disease: diagnosis, referral, treatments, consequences of the disease on the family, social and professional life including psychological and emotional aspects. All French PH patients and their families can take part in this study.

Regional patient meetings, March-April

Patient meetings were held on the 27th of March in Martillac, in Aquitaine, and on the 24th of April in Mollkirch, in Alsace.

PH Awareness day, Sète, 8th of May

This event is being organized by Xavier Gine, father of a little girl who just passed away last April, in collaboration with HTAP France, in Sète, a town by the sea in the South of France, in Languedoc-Roussillon. Alexandra Rosenfeld, Miss France 2006, is patron of the event, which is supported by the local press, TV and radio, tourist office, hotels and other commercial activities. The funds raised will be devolved to HTAP France for research. The program includes a walking/cycling excursion and other activities and the highlight of the day is a concert by Michel Fugain, a very famous French singer and composer. The first edition, in 2009, was a great success.

Family weekend, Chédigny, 8-9th of May

HTAP France is organising a weekend where children with PH (up to 20 years of age) and their families and friends can meet and share experiences at the Village des Vacances in Chédigny, in Touraine. During the weekend patients and their families will be able to meet Professeur Bonnet of the Necker Hospital.

PH Awareness day, Lyon, 6th of June

The 5th edition of “Running for PH” (*À pleins poumons*) is being held in Lyon in June. The race is open to anyone over 6 years of age. Adults will run 10km. This event is being organised in a park in the centre of Lyon and will be supported by local press and TV. Funds raised will be devolved to HTAP France.

PH Marathon, Toulouse, 11-12th of June

The 6th edition of the HTAP marathon is being run on 11th and 12th of June. The marathon starts in Toulouse and ends in Port La Nouvelle (205 km). Last year’s marathon was very successful and managed to raise considerable attention on PH in TV, radio and press.

First French speaking Patients Conference Congress for PH patients and their families, Lyon, 8-10th of October.

HTAP France is organizing its first French speaking patient Congress in Lyon 8-10 October. The 2-day event is specifically organized for patients and their families. PH experts from the PH reference centres will make presentations and there will sessions where patients can meet doctors, psychologists, nurses etc.

GERMANY

René Baumgart Foundation award


The two winners with their award

The 7th edition of the Prize instituted by the René Baumgart Foundation and dedicated to "A life without Pulmonary Hypertension" was held on 19th of March in Hannover. This event was held within the context of the 51st Congress of the German Society for Respiratory Medicine. For the second time the prize was awarded to a project in basic research (Dr. Matthias Brock from the University of Zurich) and in clinical practice (Dr. Nika Skoro Sajer, University Clinic, AKH Vienna). Dr. J. Winkler from Leipzig, Chairman of the René Baumgart Foundation, and Professor Dr. E. Grünig, Member of the Executive Committee of the Foundation, presented the award.

National Patient meeting

In October 2010 the association will hold its 13th national patient meeting in Frankfurt with lectures given by PH experts and discussions on PH related issues, with an expected attendance of 300 to 400 attendees.

The program is expected to include sessions about:

- combination therapy
- interaction of drugs
- risks and advantages of pharmaceutical studies
- German health policy issues e.g. advanced health care directive
- surgery for chronic thromboembolic pulmonary hypertension
- physical exercise in patients with PH
- yoga
- tai chi
- Feldenkrais
- workshops for parents of PH children
- workshops for family members of patients
- workshops on quality of life and psychological aspects of the disease.

Anniversary celebrations

In 2011 the PH Association will celebrate its 15th anniversary and the René Baumgart Foundation its 10th anniversary.

Two big events are being scheduled to raise awareness and funds for research: a football match with charity run and autograph session on 19 July 2010 and a concert on 7 May 2011.

Speaking engagements

President Bruno Kopp was invited to present the PH patient point of view in January at a Lilly event in Prague and in March at a Pfizer event in Munich. He will be speaking on the 27 April at a Bayer Vital event in Berlin, on 25/26th of June at an Actelion event in Cologne and on 28th of June at a Bayer Schering event in Berlin.

HUNGARY

Patient meetings

The Hungarian PAH Association is planning to start holding regular patient meetings that would provide a good oppportunity for patients to share experiences and network.

Web site

The association is also planning to improve their web site by uploading a video interview with a PH specialist and by making the necessary contacts in order to have their URL inserted in the links section of different Hungarian medical web pages.

Eszter Csabuda, President and Sara Marton

ISRAEL

Mr. Joni Berg, Chairman of the Israeli PH Association

Annual meeting

PHA-Israel held its Annual Meeting last December in Hertzelia (near Tel-Aviv). About 100 people attended, including patients, family members and medical stuff. Mr. Joni Berg (Chairman) and Mr. Aryeh Copperman (Director) reported on the year's activities and presented the program for 2010. One of the highlights of the meeting was the presentation by a speaker from the National Transplant Service who explained the procedures through which people are listed for transplant, donors found and organs allocated. There was great interest from the patients on this issue and a lot of questions were put forward.

Learning how to make beaded necklaces

A PH patient who had undergone a lung transplant then described her experience and how it affected her life. The last speaker was a PH specialist who spoke about combination therapy, describing the different pathways and mechanisms by which the drugs operate. He also spoke about side effects of combination therapy.

After the conference, for the very first time, a raffle was held. The first prize was an iPod Touch, donated by a pharmaceutical company. The initiative was so successful that people where queuing up to buy tickets. These eventually ran out and the organizers had to write numbers and names on pieces of paper! During the dinner a pianist and flutist played background music.

Chamber music being played after the Annual meeting

Support group

PHA Israel runs a professionally facilitated emotional support group for its patients. This support group was started with the help of generous funding from PHA USA in the form of a seed grant. The group meets monthly and it provides a unique opportunity for PH patients to share and support each other and to deal with the emotional issues involved in living with PH. In January the ladies of the group were treated to a morning of relaxation, fun and creativity. They started with relaxation exercises and guided imagery. A professional jewelry designer then explained the basic techniques of making a beaded necklace. She had brought along hundreds of assorted beads in all different colours, shapes and sizes and the ladies enjoyed making necklaces whilst chatting and sharing coffee and cakes.

Raising awareness

The Israel PH Association plans to sponsor a series of consciousness-raising events this spring and summer as part of the international (Pucker-Up) for Blue Lips campaign. These include a "Blue Lips" wall pasted with a hopefully astronomical number of paper slips adorned with supporter kisses during a major media event in one of Tel Aviv's most central squares.

ITALY – AIPI

Annual Patient meeting

AIPI held its Annual patient meeting and General Assembly in Bologna on 11 April. Very qualified speakers provided updates on PH treatments and research. Italian journalist and writer Margherita De Bac presented her second book on rare diseases, recently published, which contains a section on PH based on an interview with AIPI President Pisana Ferrari. As in past years, there was a buffet lunch followed by a raffle and time for socializing. Over 100 patients and family members attended.

Pisana Ferrari, President of AIPI, and Margherita De Bac, journalist and writer

Increasing awareness

As part of its efforts to increase awareness of the disease AIPI has recently offered articles on PH to a number of health and health related magazines and succeeded in having them published in a number of magazines, some of which are distributed free of charge in Italian pharmacies. The next issue of the Italian Organ Donor Association (AIDO) national magazine will carry an interview with Cristina Gandola, an ex PH patient who underwent lung transplant surgery 15 years ago.

Walking for PH

Board member Cristina Gandola took part in the Milano STRAMILANO 10 km walk on 21 March to celebrate 15 years since her lung tranplant and to contribute to raise awareness of the disease. She walked 10 km in 2 h and 9 minutes!

ITALY – AMIP

Annual patient weekend

AMIP is holding its annual patient day in Pavia from 14th to the 16th of May. The three day program includes a meeting with the physicians at the PH specialist centre of the San Matteo Hospital in Pavia and a social program with a visit of Pavia and its famous 14th century Certosa.

PH on Sky TV

An 8 minute video on PH featuring interviews with Luisa Bonelli and Maria Pia Proia (President and member of the board) from AMIP Italy and PH physicians from the Umberto I Hospital in Rome was shown on 23 February on SKY TG 24 as part of a program on rare diseases conducted by well-known Italian journalist Paola Saluzzi.

Bambin Gesù Hospital and Echocardiogram equipment for Umberto I Hospital in Rome

Over the last few years AMIP has organized a number of fundraising events. With part of these funds it has financed a project at the Bambin Gesù Paediatric Hospital in Rome to support medical professionals dedicated to paediatric PH. AMIP is also donating a VIVID S6 - echocardiogram equipment - to the Umberto I Hospital in Rome. This equipment will be invaluable to the hundreds of PH patients being treated in the PH unit. This equipment is normally supplied by the National Health Service, but it can take years before it does - this is not the first time that patient associations step into the role of the public institutions…

Footbal for PH

AMIP members Vittorio and Laura, parents of two children with PH, are extremely active in fundraising for PH. On 21 December 2009 they took part in the annual *Derby del cuore*, a match where famous singers and actors compete against each other to raise money for charities.

PH and Facebook

Check out and join Vittorio and Laura's Facebook page called *Ipertensione Polmonare: sai cos'è?* (Do you know what Pulmonary Hypertension is?).

It has over 1,200 members and an extremely lively news feed section.

www.facebook.com/search/?q=AIPI+Ass&init=quick#!/group.php?gid=56286148556&ref=ts

News from European PH patient associations

NETHERLANDS

Useful and interesting merchandising ideas

Medikeeper is a USB-stick in the shape of a medical doctor on which one can store relevant personal information: address, contact details, name of persons to be warned in case of accident, illnesses, medicines being used, type of blood, addresses of the treating medical specialists and hospitals, vaccinations, allergies and so on. It is available on request from PHA NL web site.

Raising awareness

An interesting initiative is due to be launched shortly. PHA NL will be sending to all known patients 10 postcards. These postcards contain a brief text about PH and the need to raise money for research. Patients can paste a photo of themselves on the postcard under the wording "Am I familiar to you?'' This postcard should be sent to ten friends/aquaintances or family members, with the request to donate and to make ten copies of the postcard in order to send them on to other friends. In turn these friends should also send ten cards to their friends. This way PHA NL hopes to create a "chain of donators".

Subsciption fees and fundraising

PHA NL has created a "PH Club of 100" for donors who contribute either individually or as a group 100 euros.

www.pha-nl.nl

NORWAY

Situation in Norway

Norway's population is less than 5 million. This means that the number of PH patients is very small (it is estimated that about 100 people in Norway are diagnosed with PH). One patient's search for other patients to share experiences with initiated the start of the Norwegian organization about two years ago. A web page and a forum were set up, and patients had, in this way, an opportunity to find each other and to exchange valuable information and encouragement with each other.

PHA Norway

Due to the low membership number (about 25), PHA Norway decided to join forces with the much bigger Heart and Lung Association (LHL) in 2009.
PHA Norway is defined as an independent interest group within LHL and has its own web page and forum.

Report from Hall Skara, President of the PH group.
www.pha-no.com

POLAND

New Board

The Polish patient association held elections in March 2010 and has a new Board of Members. The President is Wlodzimierz Galuszkiewicz and the Vice-President is Marek Babecki. Other members of the Board are Piotr Manikowski, Dariusz Sakowski, Roman Cichoń, Agnieszka Bartosiewicz and Małgorzata Serwicka-Walczak.

Web site

The PHA Poland web site was launched in the second half of 2009. The address is www.tetniczenadcisnienieplucne.pl

It includes a patient forum and a calender of future events. The association plans to upload a video interview with a PH expert in the near future.

Patent meeting

The annual meeting of PHA Poland is planned for May 2010. This is intended to be a social occasion and will include family members and friends, with time for relaxation and socializing

SPAIN

Respiratory Medicine Congress

The 8th Winter Congress of the Spanish Society of Pulmonology and Thoracic Surgery (SEPAR) was held in Madrid in January. Irene Delgado, President of the Spanish PH association, was invited to present the patient perspective during the session on Pulmonary Hypertension.

This was the first time a patient association participated and was given the opportunity to explain what PH means in the everyday life of a patient.

Irene Delgado speakiing at the Congress

General Assembly

The Spanish PH Association's Annual General Assembly will take place from 11-13 June in Marina D'Or (Castellón). The meeting will bring together patients from all over Spain to talk about PH and PH related issues in an informal atmosphere and pleasant location (by the sea) which will also allow for socializing and relaxing. Last year's Annual Assembly was organized in a mountainous area and was such a success that it was decided to make the stay longer.

Yoga session at 2009 Assembly

Photo competition

The Spanish Association has launched a photo contest open to patients and families on the subject of "Living with PH". The winners will be announced during the General Assembly (see above). The 12 best photos will be used in the Spanish Association's 2011 calender which will be distributed to members, medical professionals, hospitals, etc. To apply click on www.fotovisualis.com

The photos submitted will be posted on this web site.

PH booklet for parents of children with PH

The Spanish PH Association has recently published a booklet for parents of children with PH *Guia para padres: Tengo un hijo con Hipertension Pulmonar* (see photo).

The publication contains not only scientific information about the disease, but also practical tips for day to day matters and advice on how to deal with the emotional aspects of the condition. It has a very attractive graphic layout, beautiful and evocative photos and it includes touching quotations from famous authors, poems and lyrics.

The booklet was distributed in a number of Spanish hospitals. Both PHA USA and Latina American PH Associations have requested it and put it on their websites. At the moment the booklet is available only in Spanish.

Spanish Association on Youtube

The Spanish Association has recently made a brief video about its activities which can be viewed on Youtube at:

www.youtube.com/watch?v=PBMEz7hXdT4

Spanish Association on Facebook

The Spanish Association's Facebook page has over 1.000 fans.

www.facebook.com/hipertensionpulmonar

SWEDEN

A PH patient association has recently been set up in Sweden. PHA Europe wishes the best of luck to its President, Annika Nordberg, as well as her colleagues Britt-Marie Forsstrum and Malin Ekstromm.

SWITZERLAND - Schweitzer PH-Verein

PH Association set up in January

On January 15 the Swiss PH self help group (PPH Selbsthilfegruppe Schweiz) which Bruno Bosshard has run successfully for the last ten years, has become the Swiss PH Association (Schweizer PH-Verein). Mrs Therese Oesch is the new President. For further information please see www.lungenhochdruck.ch (PH) and www.novaria.ch (transplant). Warmest congratulations to Bruno and his colleaugues from all at PHA Europe!

TURKEY

Office moved and patient meeting

PHA Turkey recently moved its central office from Istanbul to Ankara.

On January 10 it held a “Patient-info Meeting” during which future plans for 2010 were discussed.

One of the aims for the coming year is to open offices in Istanbul, Izmir and other regions of Turkey.

Awareness campaign

Over the last few months PHA Turkey members have concentrated their efforts on raising awareness of the disease and of the difficulties PH patients face in day to day life by contacting national and local media.

Umit Atli, PHA Turkey President and other members of the Turkish association recently appeared on TV programs in Istanbul and Ankara (see photo).

Participation at scientific events

PH Turkey took part in the “Patient Partner Central-Eastern European Regional Workshop on Defining the needs and means for more partnership between Patients, Patient Organisations and Other Stakeholders in Clinical Trials” on 24th and 25th March in Athens.

Web site

PHA Turkey has two new web-sites. The official site is www.phdernegi.org.tr and the web site for the support group is www.pha-turkey.com

On left: PHA Turkey members on national TV

Above: new web site

UNITED KINGDOM

Breathe freely campaign

PHA-UK's "Breathe Freely" campaign is in its final stage and has achieved considerable success in helping persuade British Airways, Cathay Pacific, Emirates and leading charter holiday provider Thomson Airways to drop charges for the provision of supplemental oxygen. It has also succeeded in persuading many others carriers to change their policies and allow people to bring their own oxygen equipment on board.

Puckerup4PH 2010

PHA UK is now focusing on the global Pulmonary Hypertension awareness campaign, PuckerUp4PH 2010. The original 2008 initiative to collect the largest number of "Blue Kisses" with the aim of entering the Guinness Book of records was thwarted by the H1N1 virus pandemic.
The alternate online Pucker Up4PH search for "The Lips of 2010", however, proved a great success and generated wideranging media interest. 24 PH patient support groups from around the world participated and almost a thousand photo were uploaded to the www. puckerup4ph.com /gallery.php photo gallery Most significantly, there were over 30,000 hits on the www.puckerup4ph.com website.
On the 14th of May 2010 PHA UK is launching this year's PuckerUp4PH Campaign, which will run through till the start of International PH Awareness Month in November 2010, with the participation of a number of PHA Europe member associations.
Blue Lips Kits will be sent to each participating PH patient group in order to help their members collect kisses from friends and loved ones, but also to encourage PH awareness raising and "kiss collecting" events.
Each Blue Lips Kit will contain enough forms to collect 100 kisses.

New publications

PHA UK has recently updated its Patient Survival Guide and has published two very interesting booklets, one on patient participation in clinical trials, and the other on PH and intimacy.
Intimacy is an important issue for PH patients in their day to day life with the disease, but is rarely addressed. This booklet provides very useful information and advice. The publications are available on request by contacting the association.

For further details see: www.phassociation.uk.com

Spring issue of newsletter

The Spring issue of the PHA UK newsletter is now out with many interesting articles.
Topics covered include:

- news of the Puckerup4 PH2010;
- results of the Breathe freely campaigns;
- disability living and attendace allowances;
- a Guide to accessible Britain;
- details of meetings planned;
- fundraising events for 2010.

The newsletter also features a number of patient stories.

For further details see: www.phassociation.uk.com

Next PHAE newsletter

The Summer issue of the PHA Europe newsletter is due out around the end of July/beginning of August. It will cover May-June-July. We would be very grateful to you if you could send a brief report and possibly some photos of any activities of your patient association that you wish to share with us. You may send your contributions in your own language and we can provide for translation. Many thanks in advance!

News from PH patient associations around the world

LATIN AMERICA

Sociedad Latina de Hipertensión Pulmonar

The Latin America PH Society was founded by Migdalia Denis, a Venezuelian PH patient living and working out of Miami. She is the current President. PHA Europe members had the opportunity of meeting her at the International PH association meeting held in Barcelona in 2006.

Quarterly newsletter

Migdalia Denis has recently sent us its second quarterly e-newsletter containing information on its activities and interesting reports from its member assocations of Argentina, Colombia, Brazil, Mexico, Puertorico, Venezuela as well as from the Spanish association.

www.sociedadlatinahp.org

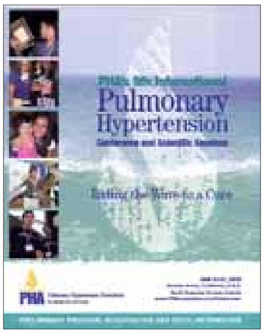
USA

New website

PHA USA's web site has recently undergone major restyling and has many new and interesting features. The international section is particularly interesting: the page on international commitment contains a time line mapping the development of the PH associations in different countries and their main activities over the last 30 years.

9th International Conference on PH

PHA's 9th International Pulmonary Hypertension Conference is taking place in Garden Grove, California, from the 25th to the 27th June. This year's scientific sessions for medical professionals will be on "Inflammation and Growth factors in PAH". There will be numerous patient/ family led sessions as well as medically led sessions for patients covering different aspects of PH and PH in every day life.



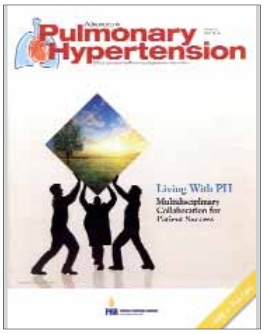
The three day Conference is packed with interesting events such as patient and caregiver meet-ups, support group meetings, helpline volunteers training sessions and the traditional PH Fashion Show. Two sessions on PH and PH treatments will be held in Spanish.

PHA Europe President Gerry Fischer has been invited to speak about "Fundraising for PH" at the Leaders Meeting.

Advances in Pulmonary Hypertension

The title of the new and extremely interesting issue of the Official Journal of PHA USA is "Living with PH: multidisciplinary collaboration for patient success". Items covered include:

- health-related quality of life as perceived by patients;
- depression and PH.



www.PHAssociation.org

VENEZUELA

Jornada Annual de Hipertension Pulmonar

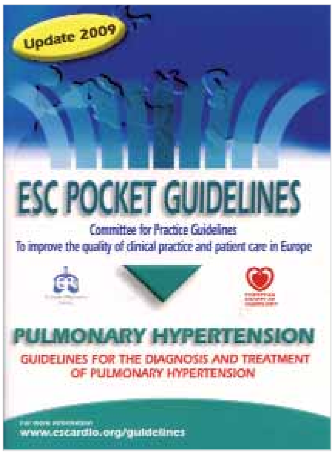
In February the Venezuelian Society for Cardiology, in collaboration with the Society for Respiratory Medicine, organized a conference on PH. One of the speakers was Prof. Nazzareno Galiè, from the University of Bologna, Italy (see photo with Annabel Sivira).

Annabel Sivira is the President of the Venezuelan PH Association. PHA Europe members had the opportunity of meeting her at the International PH Association meeting held in Barcelona in 2006. She sent us a report and photos of the meeting.

Legislation on the disabled

The PH patient association is lobbying for PH to be included in the new law on the disabled due to be approved in April 2010.

www.fundavhip.org.ve



The new ESC/ERS* Guidelines on the diagnosis and treatment of Pulmonary Hypertension were the object of a course organized by the European Society of Cardiology (ESC) in Sophia Antipolis, France, in October 2009, attended by over 300 medical professionals. These courses take place annually but the 2009 edition represents a landmark event as it included, for the first time ever, presentations from all the relevant stakeholders: medical professionals, patient representatives, pharmaceutical industry and regulatory authorities.

Over the course of the one day event, qualified speakers from all over the world discussed possible gaps in evidence of the current guidelines and future challenges in treatment and research for PH. The course faculty included medical professionals and researchers Marion Delcroix and Jean Luc Vachièry (Belgium), Marius Hoeper (Germany), Adam Torbicki (Poland), Gerald Simmoneau and Oliver Sitbon (France), Simon Gibbs (U.K.), Nazzareno Galiè (Italy). Frédéric Bodin (Actelion France) represented the industry, Amani El-Gazayerly (Netherlands) the European Medicines Agency (EMA) and Pisana Ferrari (Italy) PH patients and PHA Europe.

Role of patient associations

Very interestingly, one of the gaps identified in the course of the discussion was involvement and input from patients in the drawing up of the guidelines. This represents an important step towards increased patient involvement and we look forward to further developments in this respect.

The role of patient associations in providing invaluable support to patients is explicitly recognized by the new ESC/ERS Guidelines in several instances:

- Paragrah 7.3.1 General measures: “Encouraging patients and their families to join patient support groups can have positive effects on coping, confidence and outlook” and “Patient support groups may also play an important role in this area (psycho-social support) and patients should be advised to join such groups” (page 3).
- Paragraph 12.7 “(Medical) Referral centers will have a link to their national and/or European pulmonary hypertension patients association” (page 38).

Main changes in the Guidelines

Without going into details, we would briefly like to review the main changes/improvements introduced by the new Guidelines:

- Updated clinical classification.
- Revised diagnostic algorithm.
- Revised therapeutic algorithm (with indication for early treatment, functional class II).
- Detailed analysis of all drugs and possible drug interactions.
- Specific recommendations for treatment for different types of pulmonary hypertension.
- Definition of the characteristics of centers of excellence.

Also, compared to prior versions, the new guidelines contain more details on associated forms, risk and prognosis factors, diagnosis and treatment in children, etc.

Obviously, the limits of the Guidelines were also brought to light, with indications for possible improvements. One limit in particular is the fact that the Guidelines, as detailed as they may be, cannot cover all the possible forms of the disease, especially in cases of various etiologies or co-morbidity.

Need for further evidence

Another issue raised during the discussions is the need for further evidence and problems related to recruiting volunteers for clinical studies. The reasons for these difficulties include the following:

- Three classes of effective drugs already exist and patients are reluctant to enter new studies.
- Many patients still arrive at medical centers in an advanced stage of the disease and cannot be enrolled;
- The presence of a control group with placebo replacing the active principle is considered by some to be ethically questionable.
- Clinical trials on *naïve* patients (not treated with other drugs) are almost impossible to conduct: the drugs being tested are usually added to the treatment already underway.
- The proliferation of small centers makes the organization of clinical studies more difficult.

Improving future clinical trials

For all of these reasons and despite the fact that there are already several substances ready for experimentation, it was felt that it will be difficult to complete clinical studies to verify their efficacy in the short-medium term.

Suggestions were put forward on ways to improve clinical trials:

- Redefine the endpoints of clinical studies (replacing or adding to the 6 minute test); for example, evaluating the number of patients who improve significantly or introducing an absolute value.
- Diversify the population of clinical study participants (currently, most are patients with the idiopathic form of the disease).

Patient involvement in ESC/ERS Guidelines for PH

- Differentiate the clinical studies by their functional class: perhaps specific tests for class II (mild symptoms) are necessary?
- Carefully evaluate the effects of first line combination therapy compared to the system currently in use in which the second or third drug is subsequently added.

ESC/ERS Guidelines and PH patient perspective

From the patients' perspective, the existence and the use of Guidelines by physicians who are involved with pulmonary hypertension are, without a doubt, to be received positively.

Guidelines summarize and evaluate all of the latest and most up to date evidence on a given disease. If they are correctly implemented they guarantee the application of systematically and widely tested diagnostic techniques and therapeutic strategies.

PHA Europe will be monitoring future developments.

Next ESC course on PH, Sophia Antipolis

PHA Europe President Gerry Fischer has been invited to attend the next ECSC course on PH being held in Sophia Antipolis in the Fall of 2010.

** A joint task force of the ESC, European Society of Cardiology, and of the ERS, European Society of Respiratory Medicine have developed these guidelines with the participation of the International Society of Transplantation and the Association for European Paediatric Cardiology who have subsequently endorsed them. Full text at* www.escardio.org/guidelines-surveys/esc-guidelines/GuidelinesDocuments/guidelines-PH-FT.pdf

Update on PH treatments and research on PH drugs

In this section of the newsletter we will be reporting on ongoing research on PH treatments, both on approved drugs and new substances being tested. We will also advise on any new indications or new ways of administering approved drugs. Clinical trials involve long procedures for design, recruitment of volunteers, testing period and publication of results so we do not expect to have significant developments to report in each issue.

At the present moment new developments include:

Extensions/new approvals

- In January 2010 ambrisentan/Volibris' (GSK) use was extended to functional class II.

New ways of administering approved drugs

- Sildenafil/Revatio has been recently approved for intravenous use (Pfizer).
- Clinical trials under way to assess efficacy and safety of oral treprostinil/Remodulin (United Therapeutics) - FREEDOM-C2 and FREEDOM M-M studies.

Combination therapy clinical trials

- Effects of combination of bosentan/Tracleer (Actelion) and sildenafil/Revatio (Pfizer) Versus sildenafil/Revatio monotherapy - COMPASS-2 study.
- Upfront combination of tadalafil/Adcirca (Eli Lilly) and ambrisentan/Volibris (GSK) versus monotherapy with either tadalafil or ambrisentan - AMBITION study.

New substances being tested

- Riociguat, NO-independent stimulators and activators of cGMP in PAH (PATENT study) or CTEPH (CHEST study) (Bayer).
- Imatinib - tyrosine kinase inhibitors - platelet-derived growth factor inhibitors - IMPRES study (Novartis).
- PF-00489791 - novel long-acting phosphodiesterase 5 inhibitor (Pfizer).
- Macitentan - a new, potent tissue-targeting endothelin receptor antagonist (Actelion) - SERAPHIN study.
- Selixipag (previously ACT-293987), a potent non-prostanoid prostacyclin receptor agonist which exerts vasodilating effects (Actelion) - GRIPHON study.

For further information and details:

- www.orpha.net/consor/cgi-bin/index.php?lng=EN
- www.clinicaltrials.gov
- www.emea.eu
- web sites of pharmaceutical companies

The European Organization for Rare Diseases (Eurordis) organized a workshop on 1st of March in Brussels on Research in Rare Diseases (RD) in Europe. PHA Europe delegates Rosie and Luc Matthysen (Belgium) and Pisana Ferrari (Italy) attended this meeting which provided an excellent opportunity to listen to some of the key players on the Brussels scene in the area of rare diseases: EU Commission DG SanCo (Health and Consumer Protection) and DG Research officials, representatives of European Medicines Agency (EMA), Eurordis, Orphanet, E-rare network, Europlan, and national research Institutes. About 100 persons attended the workshop, including many patient organisations, both national and European, national health authorities and research institutes and representatives of the pharmaceutical industry. What follows is a brief summary of three of the presentations, felt to be the most relevant as they refer to research on PH or to the role of patient associations in Europe. There is only a brief mention of the other topics covered but further details can be found at:
www.eurordis.org/content/european-workshop-rare-disease-research

RDPlatform

Ségolène Aymé

RareDiseasePlatform (RDPlatform) is 3-year support (2008-2012) action project of the EU involving 13 countries. RD Platform provides information to the RD research community on: databases, bio-banks, patient registries, technology platforms and academic and industrial know-how. The aim is to provide decision makers and the community with a clear view of what is currently funded at the national and international levels in the field of RD. The project leader is Ségolène Aymé, and the coordinating team is composed of members of Orphanet Paris. Ségolène Aymé in her presentation reviewed the current situation with regard to RD research in the EU.

As far as basic research is concerned at the present moment there are 4.770 ongoing projects covering 2121 diseases. The situation varies greatly according to the disease. Very few diseases have more than 10 research projects, a few diseases have over 5 projects going on, the vast majority of diseases have only one or two teams working. Cystic fibrosis is the disease with the highest number of research projects going on, followed by neuromuscular diseases, scleroderma, some rare forms of non rare diseases, rare cancers, etc. In this area the main progresses have been made in gene identification, which translates into diagnostic tests.

With regard to products in development Mrs Aymé takes the example of orphan drugs as a proxy. Five hundred and eighty one designations have been made for potentially treating 343 diseases affecting 8.2 million people. There are 666 ongoing national or international unique clinical trials relating to 312 diseases potentially affecting 6.8 million people. Looking at products on the market, there are 99 marketed drugs for 141 diseases. Interestingly, pulmonary arterial hypertension is second on the list of (non tumor) RD list (after cystic fibrosis) with 13 orphan drug designations (see slide below). Ségolène Aymé stressed the importance of Registries, European networks and patient organisations and disease-specific scientific output as determinants of R&D. Research on RD requires a community of interested-parties to reach the critical mass.

List of non-tumours RD
with over 3 orphan designations

Cystic Fibrosis	30	Pulmonary fibrosis, idiopathic	5
Pulm. Art. hypertension	13	Retinitis pigmentosa	5
Chronic pulm. hypertension	11	Sickle cell anaemia	5
Graft versus host disease	9	Tuberculosis	5
Acute respiratory distress	7	Beta-thalassemia	4
Amyotrophic lateral sclerosis	7	Haematopoietic cell transpl.	4
Spinal cord injury	7	Duchenne musc. dystrophy	4
Alpha-1 antitrypsin deficiency	6	Rejection corneal transplant.	4
		Scleroderma	4

orphanet www.orpha.net

For further details see:
www.rdplatform.org/
www.orpha.net/consor/cgi-bin/index.php?lng=EN

Eurordis survey on patient role in research

Fabrizia Bignami

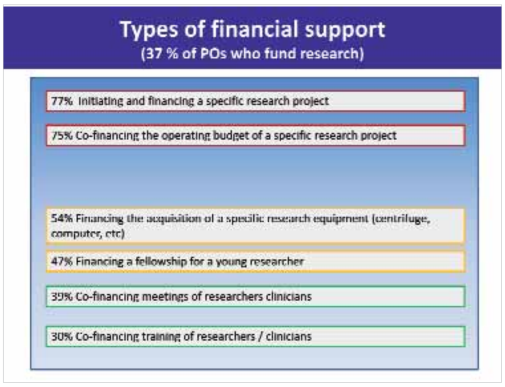
This survey was carried out in 2009 by Eurordis in collaboration with the *Centre de Sociologie de l'Innovation* (*Ecole des Mines*, Paris, France) and the aim was to:

- measure patient organisations' (POs) interest for research;
- evaluate POs support to research: in what ways and to what extent;
- learn about POs experience of collaboration with researchers;
- collect POs opinion on priorities and difficulties for RD Research.

Seven hundred and nine patient associations were involved in this survey. The response rate was 40%, with 309 valid responses from 29 European countries representing 110 diseases affecting 1.3 million persons.

There were considerable differences between the POs, in particular in terms of annual budget, number of members, time since setting up.

As far as budget is concerned, about 6% of POs have no budget, 10% have less than 300 euros, 25% have less than 3.000 euros. 25% of POs have over 100.000 euros, and 5% has more than 1.200.000 euros. There are big differences also in number of members, with 6% having less than 50 members, 21% between 50 and a 99 and 33% between 100 and 499. In other words, 60% of POs have less than 500 members. Another 26% have between 500 and 3000 members and only 6% over 3.000. The number of years of activity



Types of financial support
(37 % of POs who fund research)

77% Initiating and financing a specific research project

75% Co-financing the operating budget of a specific research project

54% Financing the acquisition of a specific research equipment (centrifuge, computer, etc)

47% Financing a fellowship for a young researcher

39% Co-financing meetings of researchers clinicians

30% Co-financing training of researchers / clinicians

varies between 1 and 30 (only very few POs are older than this) with the median being 11 years.

The main conclusions of the survey are that POs have a very high commitment to research are very conscious of obstacles and priorities. 56% of POs have a scientific board and thus integrate external scientific competences within their structures. Overall, 37% of POs finance research. Of these a total contribution of 13 M euros was provided over the last year. The majority of POs financially support research using their own budget, others organise specific fundraisers for this purpose. The above slide shows some of the types of research which is funded by POs.

One very interesting point made by Fabrizia Bignami is that POs contribute to research in many other ways and not just financially. They also contribute by:

- creating links between patients, researchers and physicians;
- helping to identify patients to participate in clinical trials and providing information for them;
- participating in scientific committees within institutions;
- collaborating in clinical trials design.

Finally, POs can contribute to defining research projects by highlighting patient's needs and expectations.

PO concern about funding clearly emerged in the survey as a key issue. POs do not expect to (and cannot) replace public or private research institutions, but rather aim to collaborate with them on an equal footing as fully recognised partners bringing important contributions.

For further details:
www.eurordis.org/sites/default/files/publications/3_FBignami_RDD2010.pdf

Eurordis conclusions

Yann Le Cam

Mr Le Cam concluded the day's debate by pointing out that nothwithstanding the significant amount of research on RD going on there are 500 RD for which there are only five projects and 3.000 RD with no research at all being carried out! Most research is focussed on few diseases and more must be done to progressively address other RDs.

There have been two major achievements:

- gene and mutation identification has led to tests being developed;
- almost 100 products for RD are on the market in Europe.

Basic research is not linked to prevalence of the disease. As far as clinical trials are concerned this is more difficult, because of the numbers (1 in 10.000), even if there are designations of drugs. European networking, the existence of registries and role of patient associations are three major determinants for research on RD and should be put at the centre of any policy on RD.

Research on RD being carried out is of high quality, there is excellence and this is a very positive message for all involved. Despite this, there is a big issue of discontinuity of funding and insufficient funding and this has emerged as a regular message throughout the workshop. This is particularly evident for infrastructure and tools (eg registries). More awareness needs to be raised about funding on RD. Funding should follow two parallel paths:

- openness of all research on the basis of excellence (bottom up);
- specific lines of funding for RD research for networking, tools, bio-banks, registries, clinical trials.

It is also important to ensure long term sustainibilty of research through continuity in funding.

Europlan

Domenica Taruscio

The European Project for Rare Diseases National Plans Development (EUROPLAN) is a 3-year project (2008-2011). The main goal is to provide National Health Authorities with supporting tools for the implementation of the Council Recommendation on an action in the field of RDs.

For further details:
http://89.97.167.162/

E-rare network

Manuela Posada

E-Rare (ERA-Net for research programs on rare diseases) is a network of 10 partners - public bodies, ministries and research management organizations - from 8 countries, responsible for the development and management of national/regional research programs on rare diseases. E-Rare is a 4-year project (2006-2010). The aim of E-Rare is to foster research on rare diseases in Europe.

For further details:
www.e-rare.eu/

In this section of the newsletter we would like to highlight some of the items currently on the EU agenda which can be of interest, either directy or indirectly, to PHA Europe members and PH patients in Europe. These include:

- an increased focus o rare diseases through the implementation of the Council Recommendation to the Members States (2009-2013);
- new developments with respect to access to orphan drugs throughout Europe;
- closer involvement of patient assocations in the Working Party in the EU Committee of Experts on Rare diseaases and in the European Medicines Agency;
- expected approval of the proposed Council and EP directive on Organ Donation and Transplantation.

Greater focus on rare diseases in Europe over the next few years

The Council of EU Health Ministers adopted in June 2009 a European strategy* that calls upon Member States to implement national plans for rare diseases before the end of 2013. The EU recognizes the specificities of rare diseases - limited number of patients and dirth of relevant knowledge and expertise - and singles them out as a field with very high European added value. The objective is to support Member States by ensuring effective and efficient identification, recognition, prevention, diagnosis, treatment, care, and research for rare diseases in Europe. The Council Recommendation also addressses the issue of difficulties in access to orphan drugs (paragraph 17(e)) and suppports the sharing of national assessment reports on the therapeutic or clinical added value of these drugs in order to minimize delays for rare disease patients (see also below). We will be monitoring developments in this area.

*COUNCIL RECOMMENDATION ON EUROPEAN ACTION IN THE FIELD OF RARE DISEASES (2009/C 151/02)

For further information and full text see:
http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2009:151:0007:0010:EN:PDF

Equal access to orphan drugs throughout Europe

Eurordis has frequently addressed the issue of rare disease patients in Europe not accessing life-saving EU-approved orphan drugs. This happens mainly because national authorities take too long to decide on the pricing/reimbursement of the drugs, thus delaying their being put on the market, and also because companies are faced with different procedures in the different countries.

In order to overcome this problem, Eurordis, representatives of industry and academic leaders in the field of orphan drugs have put forward a proposal to the European Commission and the European Medicines Agency (EMA) for the establishment of a WORKING PARTY FOR EUROPEAN COLLABORATION TOWARD COMMON SCIENTIFIC ASSESSMENT OF THE CLINICAL ADDED VALUE OF ORPHAN DRUGS (Eurordis call it CAVOD).

The proposed Working Party would facilitate collaboration amongst EU authorities and Member States in order to make the most of already existing scientific evaluations and information at the EU level and to help national health authorities make their pricing and reimbursement decisions, thus improving effective access to orphan drugs by rare disease patients.

The Commission responded to Eurordis recognizing that access to authorized medicinal products in Europe is a horizontal issue concerning all healthcare interventions but one that is particularly acute for orphan drugs. It also announced its decision to address the issue by launching a Call for Tenders to help identify and assess possible ways to implement a mechanism of coordination between Member States.

The Executive Agency for Health and Consumers has now published eight calls for tender in the area of public health, one of which relates to the creation of such a mechanism. The aim is to:

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

To access information about the calls for tenders see:
http://ec.europa.eu/eahc/health/tenders.html
For more information on Eurordis position see:
http://www.eurordis.org/content/eurordis-advocating-improve-patient-access-orphan-drugs-europe

Patient associations in the Eu Committee of Experts on Rare Diseases

Four representatives of patient associations will be sitting on the recently created European Committee of Experts on Rare Diseases (EUCERD). The aim of this Committee is to assist the Commission in formulating and implementing the Community's activities in the field of rare diseases. It should help all EU countries to tackle rare diseases more effectively and maximise the limited resources available by avoiding fragmented efforts and duplication.

The Committee will be composed of 51 members including representatives from departments or government agencies responsible for rare diseases (one for each Member State), from the pharmaceutical industry (4); from ongoing and/or past Community projects in the field of rare diseases (9); representatives of ongoing and/or past rare diseases projects financed by the Community Framework Programmes for Research and Technological Development (6); and one representative of the European Centre for Disease Prevention and Control.

According to a recent issue of Orphanews Europe (19 March), because of the delay in establishing the new European Commission (Barroso II), the EUCERD appointments have not yet been finalised and the first meeting has been postponed until May 2010.

For more information see:
www.orpha.net/actor/EuropaNews/2010/100319.html#17586
and the RAPID database of press releases at :
http://ec.europa.eu/health/ph_threats/non_com/rare_diseases_en.htm

Closer patient involvement in the European Medicinal Agency

The European Medicines Agency (EMA) has recently taken an important step forward in the direction of a closer involvement of patients and consumers.

In December 2009 the Management Board of EMA endorsed a a Reflection Paper (EMA/10723/2009) with proposed actions that will considerably widen the scope of patient and consumer contribution to to the Agency.

Community legislation already allows for participation of patients as members of three scientific committees:

- COMP (Committee on Orphan Medicinal Products);
- PDCO (Paediatric Committee);
- CAT (Committee for Advanced Therapies).

The legislation relating to patient and consumer participation in these Committees has been implemented, and all related activities are well established. However, in other areas where there is no specific legal basis patient involvement occurs but is not well structured (eg. at the level of the CHMP, Committee for Medicinal Products for Human Use).

The measures proposed by the Reflection Paper will provide a more structured framework for patient and consumer involvement. The proposed measures include:

- consultation during benefit-risk evaluation of medicines;
- participation as observers in meetings of the Pharmacovigilance Working Party;
- contribution to the Agency's safety communication.

The strategy also foresees financial support to facilitate the participation of patients' and consumers' representatives. The Reflection Paper specifically recognizes that "patients and consumers bring a unique input which contributes to achieve the best possible results within the regulatory process. Their contribution not only builds trust in the mentioned process but also incurs higher levels of transparency".

For further information see:
www.ema.europa.eu/pdfs/human/pcwp/1072309en.pd

Organ Donation and Transplantation

Organ donation and transplantation (ODT) is a major issue in the EU as the demand for organs currently exceeds the number of available organs in all Member States. The EU organized an Open Consultation on ODT that took place in June 2006. Patient representatives through Eurordis responded to this consultation. Eurordis underlined the importance of ODT for rare disease patients and explained how these were worse-off than the average organ recipient. In that same contribution Eurordis suggested that the Commission fund a pan-European awareness-raising and information campaign involving patient representatives, so as to educate the public and enlarge the pool of potential donors.

ODT is currently on the EU agenda with two specifc proposals:

- an Action Plan (2009-2015) on ODT which aims to reinforce coordination between Member States in this area;
- a proposed Directive of the European Parliament and of the Council on standards of quality and safety of human organs intended for transplantation.

The Action Plan identifies ten priority areas in order to reinforce the cooperation between Member States, by identifying and developing common objectives and guidelines, jointly-agreed benchmarks, regular reporting, and defining and sharing of best practices. Actions at Community level will complement Member States' efforts to improve the quality and safety of organ donation and transplantation, combat the organ shortage and make transplantation systems more efficient.

The Proposal for a Directive covers human organs used for transplantation, during all the phases of the process - donation, procurement, testing, preservation, transport and use - and aims to ensure their quality and safety, and hence a high level of health protection.

The European Parliament Committee on the Environment, Public Health and Food Safety was asked for its opinion (within the context of the co-decision procedure

- see below). At its meeting of 16th of March it agreed on a number of comments and suggestions on the Action Plan and has proposed amendments to the proposed Directive. These reflect concerns with regard in particular to the definition of donor, responsible authorities, post transplantation period, living living donors.

The Proposal for a Directive Directive is due to be approved by the European Parliament in plenary session 17-20 May.

For further information see:
www.europarl.europa.eu/oeil/FindByProcnum.do?lang=en&procnum=INI/2009/2104
www.eurordis.org/content/proposal-eu-directive-organ-donation-and-transplantation-renewed-hope-rare-disease-patients

Training transplant donor coordinators trainers

The Executive Agency for Health and Consumers has recently published eight calls for tender in the area of public health (see also page 19). One of these is related to the training of transplant coordinators.

The purpose of this contract is to train from one to three persons from each Member State, who have been designated as organ transplant coordinator by their hospital/local/regional/national government. These persons will become "national trainers" and will be charged with the task of further training more transplant donor coordinators in specific regions of each Member State.

For further information see:
http://ec.europa.eu/eahc/health/tenders_H03_2010.html

Rare Disease Blogs: a joint project of EURORDIS and NORD

Rare Disease Blogs is a recent joint project of Eurordis (European Organization for Rare Diseases) and NORD (National Organization for rare diseases, USA). It aims to bring together key international opinion leaders in the fields of rare diseases and orphan drugs in order to promote debate and foster interaction with members of the public. Invited bloggers include patient representatives, rare disease national and international patient federations and national health and research authorities. Comments or questions are welcome and there is a form for anyone who wishes to join.

For further information see:
www.rarediseaseblogs.net

HOW THE EUROPEAN PARLIAMENT LEGISLATIVE PROCESS WORKS IN PRACTICAL TERMS

From the European Parliament web site:

- A Member of the European Parliament, working in one of the parliamentary committees, draws up a report on a proposal for a 'legislative text' presented by the European Commission, the only institution empowered to initiate legislation. The parliamentary committee votes on this report and, possibly, amends it. When the text has been revised and adopted in plenary, Parliament has adopted its position. This process is repeated one or more times, depending on the type of procedure and whether or not agreement is reached with the Council.
- In the adoption of legislative acts, a distinction is made between the ordinary legislative procedure (codecision), which puts Parliament on an equal footing with the Council, and the special legislative procedures, which apply only in specific cases where Parliament has only a consultative role. The codecision procedure was introduced by the Maastricht Treaty on European Union (1992), and extended and made more effective by the Amsterdam Treaty (1999). Two thirds of European laws are adopted jointly by the European Parliament and the Council, including the fields of transport, the environment and consumer protection.
- On 'sensitive' questions (e.g. taxation, industrial policy, agricultural policy) the European Parliament gives only an advisory opinion (the 'consultation procedure'). In some cases the Treaty provides that consultation is obligatory, being required by the legal base, and the proposal cannot acquire the force of law unless Parliament has delivered an opinion. In this case the Council is not empowered to take a decision alone.

Parliament has a power of political initiative: it can ask the Commission to present legislative proposals for laws to the Council. It plays a genuine role in creating new laws, since it examines the Commission's annual programme of work and says which laws it would like to see introduced.

For further details

www.europarl.europa.eu/parliament/public/staticDisplay.do?language=EN&id=46

Find your country's MEP sitting on the EP Committee on the Environment, Public Health and Food Safety at:

www.europarl.europa.eu/activities/committees/membersCom.do?language=EN&body=ENVI

Find your contry's MEP's at:

www.europarl.europa.eu/members/public/geoSearch.do?language=EN

Upcoming events for 2010

13-15 May, Cracow, Poland

INTERNATIONAL CONFERENCE ON RARE DISEASES

The biennial European Conference on Rare Diseases is organised by Eurordis and its partners, and is supported by the European Commission's Health Programme. It is the most important conference in Europe on rare disease policy and has attracted over 400 participants to each of the three last conferences. On May 13th, the day before the Conference officially starts, Eurordis will hold its General Assembly. Several satellite workshops will also take place on May 13th at the same venue, such as the 10th Council of National Alliances, the annual Orphanet Workshop, and the EuroPlan Workshop, which will gather health authorities from all 27 Member States. PHA Europe (Eurorids Member) will be represented by Mrs Marzia Predieri, Member of the Board of AIPI, Italian PH Patient Association.

24-27 June, Garden Grove, USA

PHA'S 9th INTERNATIONAL CONFERENCE ON PULMONARY HYPERTENSION

PHA's 9th International Pulmonary Hypertension Conference is taking place in Garden Grove, California, from the 25th to the 27th June. This year's scientific sessions for medical professionals will be on Inflammation and Growth factors in PAH. There will be numerous patient/family led sessions as well as medically led sessions for patients covering different aspects of PH and PH in every day life. The three day Conference is packed with interesting events such as patient and caregiver meet-ups, support group meetings, helpline volunteers training sessions and the traditional PH Fashion Show. Two sessions on PH and PH treatments will be held in Spanish. PHA Europe President Gerry Fischer has been invited to speak at the Leaders Meeting on Fundraising for PH. A number of other PHA Europe Member Associations will also be attending.

28 August - 1 September, Stockolm, Sweden

ESC ANNUAL CONGRESS

The European Society of Cardiology Annual Congress is currently the largest cardiovascular medicine meeting in the world. It covers all disciplines, from basic research to clinical practice.. Over 30.000 persons attended the 2009 ESC meeting in Barcelona. As in past years it is expected that there will be a number of sessions dedicated to Pulmonary Hypertension. PHA Europe will be present as an exibitor with a booth and will be represented by Mrs Pisana Ferrari, Vice President of PHA Europe, and Mrs Luisa Bonelli, Secretary of PHA Europe and President of AMIP, Italian PH Patient Association.

18-22 September, Barcelona, Spain

ERS ANNUAL CONGRESS AND PHA EUROPE GENERAL ASSEMBLY

PHA Europe will be present with an exibitor booth also at the European Society for Respiratory Medicine's Annual Congress in Barcelona. This Congress is the world's broadest respiratory gathering. It involves not only leading experts in respiratory medicine, but also in other areas, covering the entire spectrum of research and practice in respiratory medicine. Over 18.000 persons attended the 2009 edition in Vienna. As in past years it is expected that there will be a number of sessions dedicated to Pulmonary Hypertension. PHA Europe will be holding its 2010 General Assembly within the context of the ERS Congress (see below).

REGISTER NOW!

18-20 September, Barcelona, Spain

PHAE GENERAL ANNUAL ASSEMBLY

The three day program of the 2010 General Assembly includes presentations from national associations, sessions for sharing best practices and defining future strategies, updates on PH treatments and research from top medical experts and a meeting with representatives of the industry. There is also a social program and delegates will have to opportunity to visit Barcelona and spend an evening in town. All but one of our member associations have confirmed their attendance. We also have guests from other PH patient associations in Europe. Fourty delegates from eighteen countries will be present. All European PH patient associations are invited and it is still possible to apply. For further information please contact Pisana Ferrari at p.ferrari@pha-europe.org, tel +39 3484023432.

EU institutions:

European Parliament: www.europarl.europa.eu/
European Parliament Legislative Observatory: www.europarl.europa.eu/oeil/
European Council: www.consilium.europa.eu/showPage.aspx?id=&lang=en
European Commission: http://ec.europa.eu/
Economic and Social Committee: www.eesc.europa.eu/

European Health Portal

The Health-EU Portal contains a wide range of information and data on health-related issues and activities at both European and international level. The portal is intended to help meet EU objectives in the Public Health field, it is an important instrument to positively influence behaviour and promote the steady improvement of public health in the 27 EU Member States.
http://ec.europa.eu/health-eu/index_en.htm

EU Commission Directorate General for Health and Consumers (DG SanCo)

The EU Commission Directorate General for Health and Consumers has the task of keeping laws on the safety of food and other products, on consumers' rights and on the protection of people's health up to date. It is also in charge of verifying that EU laws are correctly implemented in the 27 Member States.
http://ec.europa.eu/dgs/health_consumer/index_en.htm

EUROPE IN ACTION
FOR HEALTHIER,
SAFER, MORE
CONFIDENT CITIZENS

Europe in action for healthier, safer, more confident consumers action

This booklet published by the EU Commission Directorate General for Health and Consumers provides a very interesting overview of EU action in this field.

European Patients Forum - EPF

EPF is the umbrella organisation of pan-European patient organisations active in the field of public health and health advocacy. It was founded in 2003 to become the collective patients' voice at EU level. It currently represent 42 patients organisations - which are chronic disease specific patient organisations operating at EU level and national coalitions of patients organisations. Eurordis is a Member of EPF.
www.eu-patient.eu

Pharmaceutical Forum

The Pharmaceutical Forum is a high-level political platform for discussion set up in 2006 by Commission Vice-President Günter Verheugen and Commissioner Markos Kyprianou. The aim is to enhance the competitiveness of the pharmaceutical industry in terms of its contribution to social and public health objectives. The Forum brings together Ministers from all European Union Member States, representatives of the European Parliament, patients, the pharmaceutical industry, health care professionals, and insurance funds. European Patient Forum (see above) represents patients' organisations in the Steering Group and in the three expert working groups: Relative Effectiveness, Pricing and Reimbursement and Information to Patients.
http://ec.europa.eu/pharmaforum/

PHA EUROPE

Aims of PHA Europe

Pulmonary 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.

Members of PHA Europe

AUSTRIA
Selbsthilfegruppe Lungenhochdruck
Wilhelmstraße, 19 - 1120 Wien
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BELGIUM
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Cantincrodelaan, 48 - 2150 Borsbeek (Antwerpen)
www.ph-vzw.be - hendrik.ramaker@skynet.be
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BULGARIA
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CZECH REPUBLIC
Sdružení Pacientů s Plicní Hypertenzí
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GERMANY
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Associazione Malati di Ipertensione Polmonare
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Avenida Dr. Luis Navega, 38-42 - 3050 Mealhada
www.aphp-hp.org - mariajsaraiva@gmail.com

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für Menschen mit pulmonaler Hypertonie
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bosshard@lungenhochdruck.ch
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www.saph.ch/SuisseRomande - monika.sorgemaitre@hcuge.ch

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