



**A call to action on pulmonary hypertension:
The European Parliament launch event**
Wednesday, 6 June 2012

Introduction

Karin Kadenbach MEP opened the event and reminded participants of its objectives, i.e.

- Introduce the Pulmonary Hypertension Policy Brief and Call to Action
- Raise awareness of the urgent need for health policy, health services, and research to take account of pulmonary hypertension
- Explore the possibility for EU and national level action

Mrs Kadenbach MEP stated that she was struck by the fact that, despite the obviously serious nature of the disease, awareness is very low. She expressed her hope that this meeting would contribute to increased awareness and more suitable policy and research at EU as well as national levels. One of her reasons for hosting the event is that pulmonary hypertension (PH) is a *forgotten* disease. However, a variety of initiatives on the current EU health agenda can help bring about a greater focus on PH; Mrs Kadenbach MEP and co-host Hannes Swoboda MEP can be helpful in this respect.

Presentations

The first speaker, **Jean-Luc Vachiéry (Cliniques Universitaires de Bruxelles)**, addressed the issues in relation to pulmonary hypertension. High blood pressure in the lungs, i.e. pulmonary hypertension, kills silently, rapidly, and surely. The right side of the heart (connected to the pulmonary arteries) has to pump two to five times more than the left side (attached to the aorta). There are five PH subtypes, some of which are rare, i.e. pulmonary arterial hypertension. The disease is unknown to the public and barely known by many health care professionals.

In terms of diagnosis and prognosis, it takes around two years between the onset of the symptoms and diagnosis, largely because there are no specific symptoms. However, the consequences of a delayed diagnosis can be dramatic. Fifty percent of patients die if the disease is left untreated. Furthermore, beyond the symptoms and the disability, PH completely changes patient's lives.

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

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

The second speaker **Simon Gibbs (Imperial College, London)** emphasised that too many PH patients receive poor care. However, clinical experience and vigilant patient care can mean a longer and better life for patients and their families. The best way to provide this care is by means of expert centres:

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

A multi-professional team can support individuals throughout their patient-journey, which affects the physical, psychological, social, and spiritual aspects of their lives. Furthermore, expert centres are the right place to conduct clinical trials of new and potentially better medicines. In short, expert centres provide the opportunity for the best, most timely, and cost-effective care for PH patients.

No one model of expert centres will fit all European countries. Instead, a common set of principles of best clinical practice might drive a network of centres sharing their experience across borders. A European network of centres might be established as has been described for other rare diseases.

Paola Testori Coggi (European Commission, Director General DG SANCO) explained that the EU health remit is very limited; Member States are responsible in this area. However, the Commission takes many initiatives that complement Member States' activities and priorities, such as:

1. Chronic diseases feature high on the current Commission agenda, and this is supported by the European Parliament. In general, Member States do not invest enough in promotion and prevention.
2. The Commission also stimulates awareness-raising and knowledge dissemination through information campaigns; it helps to identify the health needs of the EU population.
3. Serious cuts in health budgets are occurring across the EU to keep health systems sustainable. The Commission works with health ministers to ensure greater efficiency in health care provision rather than just reducing spending. Making systems more efficient would also help reduce costs. For instance, more care delivered in home settings, better data analysis, and better use of eHealth would be part of the solution.
4. Making better use of the health workforce is another issue; it is already known that within the next 10 years, there will be a shortage of some 100.000 health staff. Providing the right services will be key, and training for these services will be essential.
5. The new research programme, Horizon2020, will have a substantially larger budget, and research on health will be part of this programme. This will focus on information and data collection on the prevalence and incidence of diseases and conditions, including pulmonary disease.
6. Paola Testori Coggi also recalled that the EU has been quite successful in the area of rare diseases. For instance, there are registries for rare diseases which are supported financially by the EU. In relation to orphan drugs, many drugs have now been authorised (70 drugs treating 60 rare diseases).

Key words in all of the Commission's health activities are "disease prevention" and "health promotion."

In her presentation, **Pisana Ferrari (Vice President, PHA Europe)** outlined four main challenges still facing PH patients:

Physical challenges

In addition to breathlessness and fatigue PH patients may experience syncope, arrhythmia, hemoptysis and disphonia. They are more at risk of infections. Some drugs are complex, invasive,

and painful and many have side effects. There may be sleeping difficulties and problems concentrating and reading; there can be changes in physical appearance, self-perception, and problems with intimacy. Co-morbidities, impaired physical capacity, and de-conditioning are frequent.

Emotional challenges

At the moment of diagnosis there will be shock, fear, and disbelief. There is an immediate need to rethink priorities and reorganize one's life around the disease. A difficult patient journey follows the diagnosis. There is a sense of isolation, of not being understood, and fear and uncertainty about the future. Anxiety and depression are common.

Organisational challenges

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

Medical challenges

There are delays in diagnosis or misdiagnosis as well as inequalities in access to treatments across the EU. Reimbursement for treatment can be problematic. There is a lack of expert centres and surgery facilities (PEA, lung transplant), and waiting lists for transplants are long. Despite progress and better medicines, the prognosis for PH is still very poor.

Ms Ferrari underscored that much remains to be done and that patient associations are the stakeholders best positioned to understand the needs and priorities of patients. Finding a cure for PH would benefit not only the PH patient, but society as a whole.

Gerald Fischer (President, PHA Europe) briefly introduced PHA Europe as an umbrella organization of 25 PH patient organisations located in 22 European countries. He then formally launched the Call to Action and provided a flavour of its content. The Call to Action has involved all key PH stakeholders: medical professionals, nurses, researchers, representatives of the health industry, and leaders of PH patient groups around the world. It addresses the five main unmet needs of PH patients, i.e.:

1. *Improved awareness and screening:*

Awareness is needed to make the patient conscious of the fact that breathlessness can be a sign of a severe condition. Primary care physicians have to be informed about specialized centres for PH. Screening programmes are needed to identify and diagnose at-risk patients to improve patient outcomes; physicians must know about the different subtypes of PH.

2. *Centres of expertise*

As PH is difficult to diagnose for non-specialists and as approved treatments are limited, expert centres with specialised physicians are essential for proper diagnosis and appropriate care. E--Health can help to establish important links between the centre and the nearby doctor. Treatments prescribed by centres of expertise must be available at affordable costs for every patient. Improved donor legislation will ensure easier access to transplantation.

3. *Psychosocial support*

Many patients feel isolated and alone after a PH diagnosis. Some treatments are complex to manage. Some patients suffer from depression and consider suicide. Therefore, there is a need for integrated care systems care which include psychosocial, nutritional, and rehabilitative support.

4. *Empowered patient groups*

Patient groups provide a wide range of supportive services and are the voice of patients and carers. Most patient groups are run by people living with or affected by PH. The patient perspective should be integrated by involving patient groups in health policy debates and decision-making. Financial support for patient groups should be encouraged.

5. *Clinical research and innovation*

There are approved drug treatments only for PAH, none for other forms of PH. Diagnosis is invasive and stressful. Endpoints are still very vague and clinical trials are the only option for some patients. Therefore, until a cure is found, patients need clinical trials in PH subtypes other than PAH, more paediatric studies, and non-invasive diagnostic tests. Finding a cure is the most important need.

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

The next speaker, **Prof Laurent P. Nicod (Scientific Committee Chair, European Respiratory Society)** briefly introduced ERS as the leading professional respiratory medicine organisation in Europe, which aims to alleviate suffering from respiratory diseases and promote lung health through research, sharing of knowledge, and medical and public education. The organisation launched a European Respiratory Roadmap in 2011, which includes some key recommendations for policy makers for the future of respiratory medicine under four headings: prevention, clinical care, research, and education.

Prof Nicod underlined the serious nature of PH, which, if left untreated, can lead to heart failure. The course of the disease depends on how quickly PH is diagnosed, how advanced the symptoms are and whether there is an underlying condition (e.g. COPD).

New treatment approaches for pulmonary hypertension and links with industry have created positive synergies. National and international registries have been established. Patient groups are an invaluable support. Despite this progress, more needs to be done.

There is a need for improved awareness and screening, as early diagnosis and detection is essential. Likewise, continued support for clinical research and innovation is vital. There is also a need to optimise care to improve patient outcomes. Research efforts need to be stepped up, as well as a strategic coordination amongst disease areas (as chronic diseases often occur together, particularly in older people). Turning biological knowledge into clinical progress will require new resources and support systems. The EU research and innovation programme, Horizon 2020, proposes a total of €8 billion for health research. Prof Nicod called on the European Parliament to defend and perhaps increase the health research budget.

The final speaker was **Nazzareno Galiè (Istituto di Cardiologia Università di Bologna)**, who articulated some recent successes in the field of PH. For example, there is now an organized international and multidisciplinary group of scientists and clinicians, patients associations are far more active, and G5 PH annual (stakeholder) meetings are being organized. The new PH classification was adopted by WHO, and eight drugs are approved, with more in the pipeline. Morbidity and mortality has improved, medical practice guidelines are in place, and the pharmaceutical industry is far more involved. A recent world symposium on PH, attended by 145 experts, addressed issues in 12 thematic working groups. Despite progress, Prof Galiè outlined some of the remaining issues, which include the need for:

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

Audience debate

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

How will we define success of the Call to Action in a year's time?

Participants stated that some of the indicators for success could be scientific progress and investment in research, with uniform cross-country PH care and disease management. Increased media attention would be another indicator. Others named increased and sufficient funding for patient groups; the EU could perhaps play a role in this respect. However, it was pointed out that funding of national patient organisations is a national responsibility rather than an EU one.

How do we raise awareness of PH?

The EU has limited powers within the field of health. Nevertheless, the Parliament can help to raise PH awareness by means of health-related topics on the EU policy agenda, e.g. the Transparency Directive, the Health for Growth programme, the Chronic Conditions Reflection Process, and Action on Rare Diseases. All these initiatives will need to be scrutinised for the effect on patients and health care. MEPs can table amendments that can render PH more visible, even if they are not adopted in the final text. Today's event is important as it has given the condition a face. The Call to Action is a useful tool as it comes forward with concrete recommendations. It gives guidance to policy-makers.

There is a need to implement existing programmes

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

How do we bring less advanced countries on board?

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

Medicines development

While it is true that progress has been made in the field of drug development for rare diseases (with 70 authorised drugs for 60 diseases), there are some 8000 different rare diseases. The industry's success rate in developing effective drugs is about 10 percent. Something needs to be done to improve the outcome of research: 90 percent of PH remains untreated and PAH medicines address only the one subtype. There are immense inequalities in terms of access to drugs between and within member states. A huge task lies ahead for member states to improve access to treatments. In this respect, the EU has made some small steps forward, e.g., the Directive on cross-border health care. This affords patient the legal certainty that they will receive treatment.

Drug authorisation and clinical trials

The precautionary principle is applied very strictly in this field; certification and verification are key words. While this principle may be applied too stringently on occasion, the safety of drugs remains paramount. There is no single or simple answer. Even if drug approval is centralised, reimbursement is the remit of the Member States. Equal access to treatment should follow central authorisation. However, while access to medicines should be improved, so should training of health professionals. If drugs are not being prescribed and managed appropriately, patient will suffer.

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

