PHA EUROPE for the patients
European pulmonary hypertension association

A Call to Action for Appropriate Diagnosis, Treatment, and Care of Persons Living with Pulmonary Hypertension

CALL TO ACTION

1. IMPROVE ACCESS TO EXPERT CARE

- Establish centres of expertise:
  - High volume centres of expertise, staffed by a multidisciplinary team of physicians who are trained and experienced in pulmonary hypertension (PH) care, should provide for the diagnosis and care of PH patients.
  - Expert centres are necessary to ensure that currently available drugs are prescribed and used appropriately.
- Increase transplants by enacting donor legislation that ensures easier access to transplant. All EU countries should develop and maintain comprehensive and humane organ donation policies.
- Develop and regularly update clinical practice guidelines.
- Ensure that drugs or combinations of drugs for PH patients are prescribed by expert physicians, and that patient access is not arbitrarily limited by national policies.

2. IMPROVE AWARENESS AND SCREENING

- Initiate PH awareness campaigns and training programmes.
- Create national screening and diagnosis programmes, tailored to PH patient subgroups.
- Raise awareness of the potential for curative surgery for chronic thromboembolic pulmonary hypertension (CTEPH) and the need to appropriately evaluate CTEPH patients.

3. ENCOURAGE CLINICAL RESEARCH AND INNOVATION

- Ensure more support and funding for:
  - Fundamental PH research
  - Research that leads to effective treatments for all forms of PH
  - Research to determine non-invasive methods for measuring pulmonary pressure and cardiac output.

4. EMPOWER PATIENT GROUPS

- Facilitate real-life information exchange between PH patient organisations and policy makers through roundtables, working groups, and other activities.
- Include patient groups in health care policy debates and decision-making.
- Increase the capacity of patient organisations to allow them to more effectively serve patients, particularly through the provision of accurate, regularly updated information on websites.
- Encourage financial support for patient associations that ensures their independence.

5. ASSURE AVAILABILITY OF PSYCHOSOCIAL SUPPORT

- Ensure that PH treatment encompasses treatment for the “whole” patient. National systems of integrated care must be instituted, encompassing all aspects of medical, psychosocial, nutritional, and rehabilitative support for patients and their caretakers.
- Ensure that patient associations have secure and ongoing sources of funding for patient psychosocial support, including 24-hour telephone help lines and other programmes.
- Afford disabled status to PH patients.

The Need for a Call to Action in Pulmonary Hypertension

Pulmonary hypertension (PH) is a disabling, debilitating, and deadly disease. It severely impacts one's ability to perform the most routine daily life activities. It can be caused by any one of a number of conditions (such as lung or heart disease or blood clots), or it can occur for no known reason. In some cases it is hereditary; in others, not. It manifests in several different forms, some of which are rare conditions. It does not discriminate but occurs irrespective of age, race, or ethnicity, and it can affect one's neighbours, friends, family members, or colleagues.

The struggle to do something as automatic and essential to life as breathing is both frightening and disheartening. Persons who live with PH are challenged by this simple activity, which most of us take for granted and do not even think about. It is therefore critical that persons living with PH be appropriately diagnosed in a timely manner, that currently available treatments be prescribed only to those patients who will benefit, and that innovative research continue for new and improved treatments until a cure is found. These needs can be addressed by two relatively simple solutions that will not only enhance PH patient outcomes, but will also result in cost savings for health care systems and society at large.

The **establishment of centres of expertise for the diagnosis, treatment, and management of PH** will ensure that persons at risk of and living with PH are seen and appropriately diagnosed and treated by clinicians with significant experience in PH, who will also help ensure that costly medications are not over-prescribed. The **support and funding of innovative research** will help ensure that new and improved treatments are developed until a cure is found. Breathing is not an activity that persons living with PH take for granted. Centres of expertise and innovative research can help restore normalcy to their lives. We must not hold our breath, but move forward now.

Pulmonary Hypertension Overview

More than 25 million people live with PH around the world.[1] There are numerous potential causes, and it sometimes runs in families, but sometimes it occurs for no obvious or known reason. In PH, the arteries that carry blood from the heart to the lungs narrow for reasons that are not entirely understood. The heart struggles to pump blood through the narrowed arteries, resulting in high blood pressure in the lungs and enlargement of the heart. Eventually, the overworked heart wears out, and heart failure and death can result.

Symptoms of PH typically include shortness of breath and fatigue, and sometimes dizziness and fainting spells. These symptoms may appear gradually over time, which may in turn delay patients from seeking diagnosis and treatment. As PH advances, it becomes increasingly difficult to perform even the simplest tasks. Patients curtail their daily activities and experience a substantially reduced quality of life.[2]

There are five different forms of the disease, some of which are rare as well as rapidly progressing, debilitating, and life-threatening. These are:

1. **Pulmonary Arterial Hypertension (PAH)** is a rare, chronic, progressive condition that is disabling, costly to society, and can lead to death. PAH may be idiopathic (occurring from no known cause), or it may be associated with congenital heart diseases, connective tissue diseases, HIV infection, or other diseases and conditions. In Europe, approximately 15 to 52 people per million live with PAH,[3] and approximately 2.4 new cases per million are diagnosed annually.[4]

2. **PH with left ventricular disease** is caused by disorders that affect the left side of the heart, including mitral valve disease and long-term high blood pressure. Left heart disease is thought to be the most common cause of pulmonary hypertension.
3. **PH with lung disease and/or hypoxemia** is a subtype of PH associated with lung diseases such as COPD and sleep-related breathing disorders such as sleep apnea. It can also be caused by diseases that result in scarring of the lung tissue.
4. **Chronic thromboembolic pulmonary hypertension (CTEPH)** is caused by blood clots in the lungs or blood clotting disorders. It is often inappropriately treated, and patients do not always have sufficient information about – or access to – indicated surgical options for care. While it is not known how prevalent CTEPH is, evidence suggests that the disease is under-diagnosed.[5]
5. **PH due to unclear or multifactorial (i.e., multiple) causes:** Pulmonary hypertension may also be caused by other diseases and conditions including blood, metabolic, or systemic disorders, or by other conditions, such as tumours that press on the pulmonary arteries, or kidney disease.

Until about 15 years ago, the prognosis for PH patients was grim because there were no PH-specific treatments. Even today, deteriorating quality of life, dependency, and eventual disability and decline are the norm. However, particularly in the last decade, management of patients has improved as new drugs have been introduced. Those new therapies notwithstanding, much remains to be done, as there are approved treatments only for PAH, and there are no cures.

About PHA Europe

A patient group with 25 national affiliates that together work to enhance awareness and understanding of pulmonary hypertension across Europe, PHA Europe is uniquely positioned to understand the needs, concerns, and hopes of persons who live with this devastating disease. In September 2011 PHA Europe convened a roundtable of practicing clinicians, researchers, and patient advocates – all of whom are preeminent in the field – to discuss a call for improved patient access to screening and diagnosis, integrated and appropriate care through centres of expertise, and psychosocial support.

Although focused on Europe, because PH patients around the globe share similar concerns and face the same challenges, this call to action is universal and transcends geographic boundaries. Our action items do not necessarily call for increased revenues, but rather a reallocation of currently available resources, because, as patients are properly diagnosed and treated in a timely fashion, they will be able to return to work and resume normal activities more quickly, as they simultaneously decrease their need for acute health care and supportive services.

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[1] Elliott, C, et al. *Worldwide physician education and training in pulmonary hypertension: pulmonary vascular disease: the global perspective*, Chest 2010; 137(6):85s-94s.

[2] Boyer Hayes, Gail, *Pulmonary Hypertension: A Patient's Survival Guide*, Pulmonary Hypertension Association, Fourth Edition, July 2011.

[3] Kirson NY, Birnbaum HG, Ivanova JI, Waldman T, Joish V, Williamson T, *Prevalence of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension in the United States*, Current Medical Research and Opinion. 2011 Sep; 27(9):1763-8. Epub 2011 Jul 27.

[4] Porhownik, Nancy R., MD, and Bshouty, Zoher, MD, PhD, FRCPC *Pulmonary Arterial Hypertension: A Serious Problem*, Perspectives in Cardiology, April 2007, pp 33-40.

[5] Tapson, VF, Humbert, M, *Incidence and prevalence of chronic thromboembolic pulmonary hypertension: from acute to chronic pulmonary embolism*, Proceedings of the American Thoracic Society, Sept 2006, available at http://pats.atsjournals.org/content/3/7/564.full.