early diagnosis • best treatment • better quality of life • finding a cure

PHA EUROPE for the patients

Call to action

April 2022

Gergely Meszaros

PHA Europe

Project manager

5 DAY 10 Years World Pulmonary Hypertension Day

MEP Lung Health Group

European Reference Network | ERN-LUNG European Reference Network for rare respiratory diseases

Call to action in 2012

Launched in the European Parliament

Call to action 2022

Medical review committee

Prof. Marc Humbert

Prof. Marion Delcroix

Prof. Adam Torbicki

Prof. Marius Hoeper

Ass. Prof. Gabor Kovacs

Launch event speaker

Ass. Prof. Laurent Godinas

Roundtable members

PULMONARY HYPERTENSION ROUNDTABLE MEMBERS

Health Care Professionals

Prof Dr Marion Delcroix
Clinical Department of Respiratory Diseases, Centre of Pulmonary Vascular Diseases, University Hospitals of Leuven; BREATHE, Department of Chronic Diseases and Metabolism (CHROMETA), KU Leuven
LEUVEN, BELGIUM

Prof. Marius M. Hoeper, MD
Senior Physician, Department of Respiratory Medicine, Hannover Medical School,
HANNOVER, GERMANY

Prof. Marc Humbert, MD
Professor and Director, Centre de Référence de l'Hypertension Pulmonaire, Service de Pneumologie et Soins Intensifs Respiratoires, Hôpital Bicêtre, Assistance Publique Hôpitaux de Paris, INSERM, Université Paris-Saclay
LE KREMLIN BICÊTRE, PARIS, FRANCE

Gabor Kovacs MD
Associate Professor, University Clinic for Internal Medicine, Medical University of Graz
Division of Pulmonology
Ludwig Boltzmann Institute for Lung Vascular Research
GRAZ, AUSTRIA

Adam Torbicki, MD
Professor, Department of Pulmonary Circulation, Thromboembolic Disease and Cardiology, Center of Postgraduate Medical Education at European Health Center - Otwock
OTWOCK, POLAND

Prof. Dr. T.O.F. Wagner
Coordinator ERN-LUNG, Frankfurter Referenzzentrum für Seltene Erkrankungen (FRZSE)
Universitätsklinikum Frankfurt
FRANKFURT, GERMANY

Non-Governmental Organizations (NGO)

Iain Armstrong, Dr
Chair, PHA UK
SHEFFIELD, UK

Shaun Clayton
Director of Membership Support, PHA UK
SHEFFIELD, UK

Migdalia Denis
President, Sociedad Latina de Hipertensión Pulmonar
FLORIDA, USA

Gerald Fischer
Managing Director, PHA Europe
VIENNA, AUSTRIA

Matt J. Granato, LL.M., MBA
President & CEO, Pulmonary Hypertension Association
WASHINGTON, DC, USA

Gergely Meszaros, JD
Project and advocacy manager, PHA Europe
VIENNA, AUSTRIA

Eva Otter
President, PHA Europe
VIENNA, AUSTRIA

Susanna Palkonen
Chairperson, Patient Access Partnership (PACT)
BRUSSELS, BELGIUM

Shalaka Parab
Cofounder & Patient Advocate
Pulmonary Hypertension India,
MUMBAI, INDIA

Joan Paulin
Director, PHA Canada
VANCOUVER, CANADA

ALLSCHWIL, SWITZERLAND

Timothy Pigot
Sr. Vice President Commercial, Aerovate Therapeutics
FOSTER CITY, CA, USA

Andy Hayes
Head Global Brand Management PAH, Bayer AG
BERLIN, GERMANY

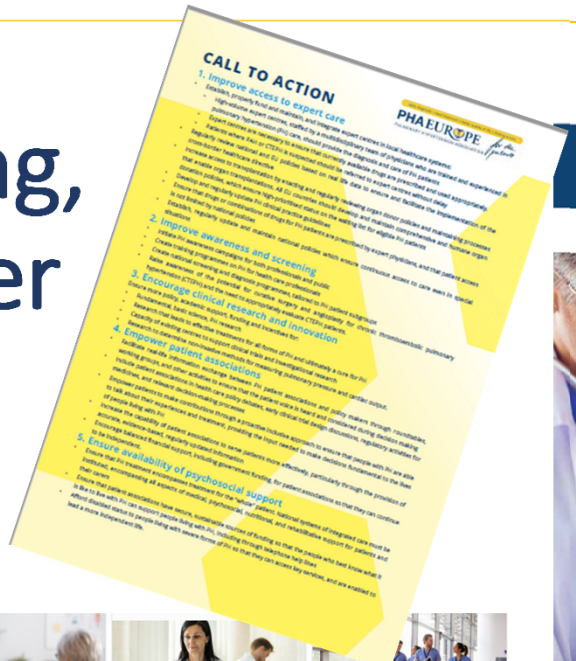
Medicine, Pfizer Biopharmaceuticals Group
NEW YORK, USA

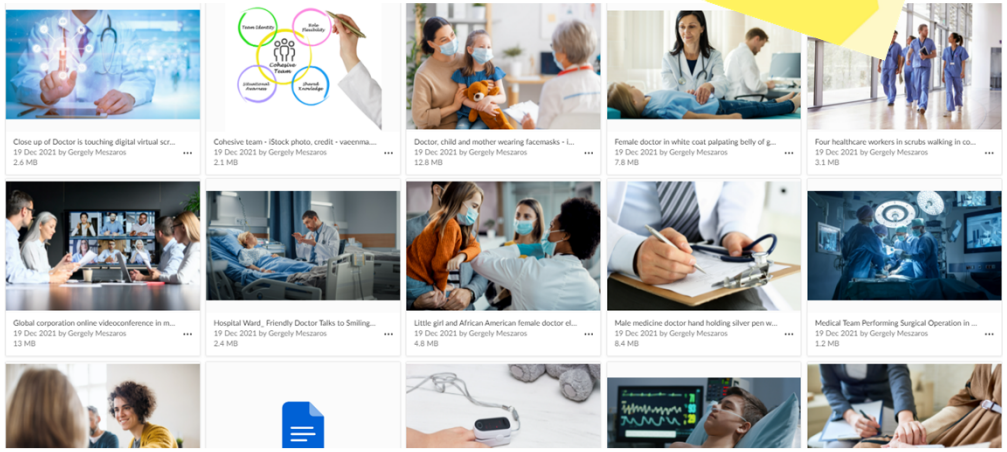
Clàudia Roca Herms
Head of Corporate Customer Onboarding Program, Patient Advocacy & Public Affairs, Ferrer
BARCELONA, SPAIN

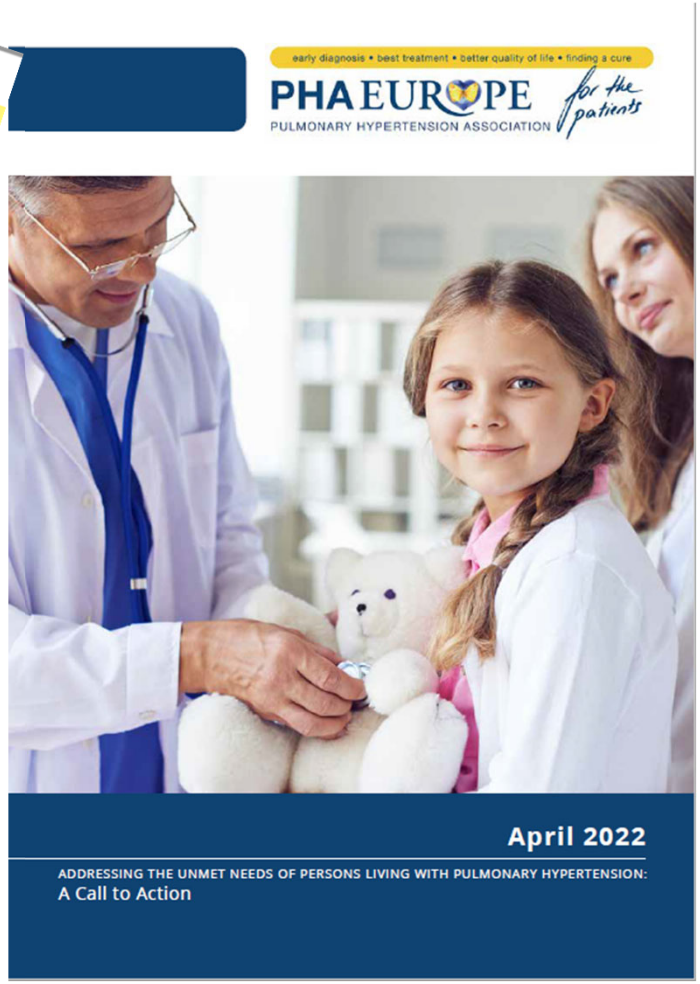
Eloi Torcal
Head of Corporate Marketing, Pulmonary Vascular & Interstitial Lung Diseases, Ferrer
BARCELONA, SPAIN

34 members

Freshly looking, modern paper





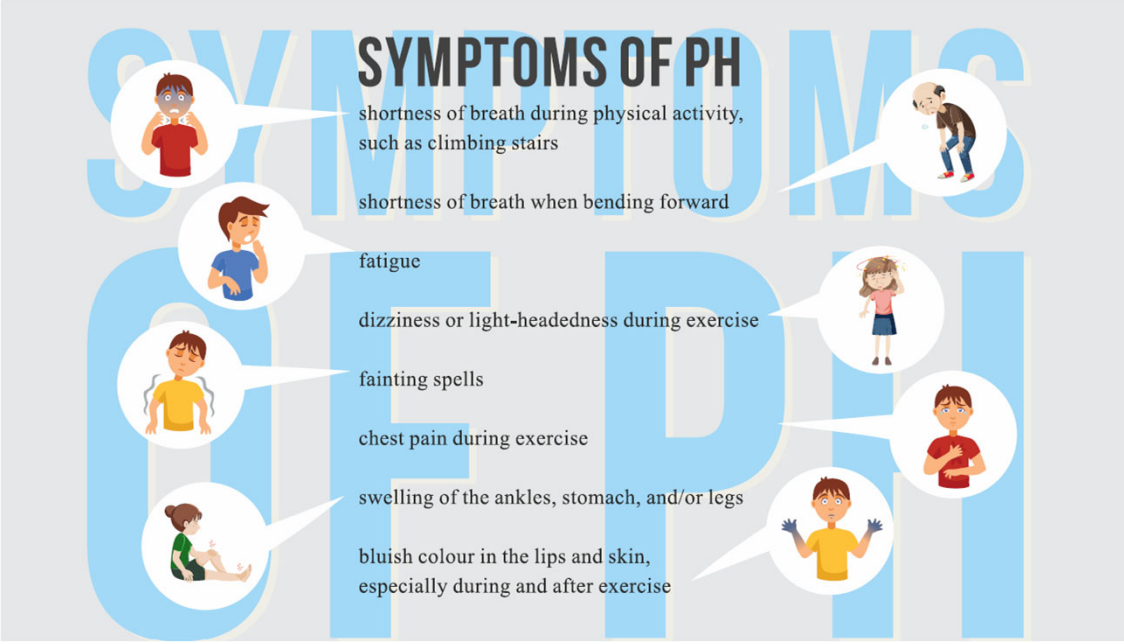


Call to action 2022

New visuals

Multiple purposes

- Standalone use
- Space saving
- More emphasis on these lists & content



What we kept…

- 5 main pillars
  - Access to treatment and expert centres
  - Clinical research and innovation
  - Psychosocial support
  - Empowered patient groups
  - Improved awareness and screening

PHA EUROPE for the patients

… heavily re-written

Call to action: Combined sections working doc

Why Action is Needed for Pulmonary Hypertension

Most of us take breathing for granted. People living with pulmonary hypertension do not. They often struggle to breathe and doing any kind of activity can be difficult. Pulmonary hypertension (PH) is the broad, inclusive name for a group of chronic diseases that affect the lungs and the heart. Some forms of PH are rare, progress rapidly and are debilitating and deadly. In PH, the arteries that carry blood from the heart to the lungs narrow for reasons that are not yet entirely understood. The heart struggles to pump blood through the narrowed arteries, resulting in high blood pressure in the lungs and the enlargement of the heart. Eventually, the overworked heart wears out, and heart failure and death can result.

Global estimates suggest that PH affects 20-70 million people worldwide. It may be caused by several other underlying conditions, but often there is no identifiable cause. Symptoms usually do not appear until the disease has progressed, meaning diagnosis and treatment may be delayed. PH does not discriminate, and while it is more common among females it occurs irrespective of age, race, and ethnicity. It can strike our colleagues, our neighbours, our family, and ourselves[1].

Unfortunately, approved treatments exist for only around one percent of people diagnosed with PH – those with three rare forms of the disease: Pulmonary arterial hypertension (PAH), Chronic thromboembolic pulmonary hypertension (CTEPH) and PH due to chronic pulmonary embolism. Even with therapy, most patients with these forms of the disease have a reduced life expectancy, although timely diagnosis and better disease management can significantly improve that timeline.

The struggle to do something as essential as breathing is both frightening and debilitating and has a dramatic impact on people's lives.

That is why this call to action is urgently needed:

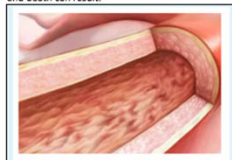
- People with PH need timely diagnosis in order to receive appropriate treatment as managing the disease in the early stages helps to reduce both the physical damage and the impact on quality of life
- Expert centres are needed within reach of people with PH. These need to have multidisciplinary medical teams who are trained and experienced in the diagnosis and treatment of PH to ensure that the right treatment is provided to the right people
- Researchers must continue to investigate innovative and improved treatments until a cure is found

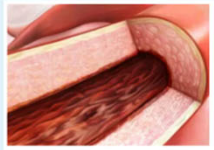
1

OF People LIVING WITH PULMONARY HYPERTENSION

Introduction

The term *Pulmonary Hypertension* (PH) describes a condition where high blood pressure affects the right heart and the vessels of the lungs. Some forms or "subtypes" of PH are rare and can progress rapidly, as well as being debilitating and deadly. Other forms are more common and less aggressive, yet still impact people's lives. In PH, the arteries that carry blood from the heart to the lungs narrow for reasons that are not yet entirely understood. The narrowed arteries force the right side of the heart, which pumps blood to the lungs, to work harder. Eventually, this wears out the heart, symptoms worsen, and heart failure and death can result.


Healthy blood vessel


Blood vessel of a person with PAH

PH does not discriminate. Although some forms are more common in young adults and in women and it sometimes runs in families, it occurs irrespective of age, race, and ethnicity. The symptoms of PH, which may include shortness of breath during exercise, fatigue, dizziness and fainting spells, vary from patient to patient, and usually do not occur until the disease has progressed. This delays diagnosis and treatment.

Diagnosis and treatment of PH is complex and belongs in expert hands. Fortunately, most regions in Europe now have established PH centres and it is important that patients with suspected or confirmed PH are referred to these centres.

Types of Pulmonary Hypertension

Pulmonary hypertension is the broad classification for a group of debilitating diseases that affect the heart and lungs. There are five subtypes of PH. Some are rare; some occur more frequently. Each subgroup has different underlying causes and must be treated differently. In fact, treatments that work in some forms of PH may be ineffective and even harmful in other forms of PH.[2,3]

5

Gergely Mesza... formázott
Gergely Mesza... törölt: PERSONS
Gergely Mesza... törölt:
Gergely Mesza... törölt: hypertension
Gergely Mesza... törölt: is
Gergely Mesza... törölt: broad, inclusive name

IMPROVE ACCESS TO EXPERT CARE

- Establish expert centres:
  - High-volume expert centres, staffed by a multidisciplinary team of physicians who are trained and experienced in pulmonary hypertension (PH) care, should provide 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 promoting clean donor policies and transplant processes that enable organ transplantation. An EU consensus should develop and maintain comprehensive and humane organ donation policies.
- Ensure that drugs or combinations of drugs for PH patients are prescribed by expert physicians, and that patient access is not limited by national policies.
- Develop and regularly update PH clinical practice guidelines

To reliably diagnose and to effectively treat rare diseases, such as pulmonary arterial hypertension (PAH) and chronic thromboembolic pulmonary hypertension (CTEPH), resources and experience need to be concentrated in dedicated expert centres. However, these centres need to be well positioned within the healthcare system, so that they deal with the right patients. Clear and realistic pathways need to be established to ensure that patients receive advanced care without overwhelming expert centres with unnecessary referrals where common causes of PH can be more easily identified.

Establish and Support Networks of Expert Centres [2]

High-volume expert centres, staffed by a multidisciplinary team of physicians who are trained and experienced in PH care, should provide the diagnosis and care of PH patients.

High-volume expert centres have repeatedly been shown to lengthen the best outcomes for patients for health care payers. In fact, the European Union Committee of Experts on Rare Diseases (ECERD) recognises the European added-value of networks of expertise in the field of rare diseases, and cites the need to improve universal access to high quality healthcare for rare diseases, in particular through the development of national/regional centres of expertise.[4,5]

I experienced a progressive shortness of breath for about two years. I was prescribed several asthma medications which didn't work. Eventually my physician referred me to a cardiologist. He performed an echo and sent me to [an expert] centre. Then I was diagnosed with PAH and started treatment.

Maria, Slovakia

3

Group 1	Pulmonary Arterial Hypertension	associated with connective tissue disease, portal hypertension, congenital heart disease, HIV infection	5-10 patients per million adults)	intravenous and subcutaneous drugs), often used in combination
Group 2	PH associated with left heart disease	Systolic and/or diastolic dysfunction of the left heart, valvular heart disease	Relatively common, affecting up to 50% of patients with advanced left heart disease	Treatments primarily target the underlying condition, no established treatment. PH, studies are on the way. PH center should be contacted in case of severe PH
Group 3	PH associated with to lung disease or hypoxia	Various chronic lung diseases including COPD, interstitial lung disease (ILD), hypoventilation syndromes, high altitude, asthma is usually not associated with PH, but can be a co-morbidity	Relatively common, affecting 10-30% of patients with COPD or ILD	Treatments primarily target the underlying condition, no established treatment. PH studies are on the way. PH center should be contacted in case of severe PH
Group 4	CTEPH (chronic thromboembolic pulmonary hypertension)	Organized clots in the lungs which obstruct pulmonary arteries	Rare (newly diagnosed in 3-6 patients per million adults and in 2-3% of patients who have recovered from acute pulmonary embolism)	Several treatment options including surgery, balloon angioplasty and medication. Therapy should be determined at CTEPH center
Group 5	PH associated with other diseases	Includes sarcoidosis, pulmonary histiocytosis, hematologic disorders, and other conditions	Rare, insufficient data for most of these conditions	Treatments primarily target the underlying condition. Patients with severe PH should be referred to PH centers

PH Groups

Group 1: Pulmonary Arterial Hypertension (PAH) is a rare, chronic, progressive condition that is disabling and can lead to death. The cause of PAH is often unknown, in which case it is known as idiopathic PAH. It can be hereditary with or without other affected family members or can be associated with conditions such as connective tissue diseases, HIV infection, liver disease, congenital heart disease, sickle cell disease and the use of certain drugs. PAH affects around 50,000 people in Europe and the symptoms are treatable, but there is medicine that targets the underlying cause and no cure. The median survival rate is five to six years following diagnosis.

Group 2: PH associated with left ventricular disease (PH-LVD) 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 one of the most common causes of PH.

Group 3: PH associated with lung disease and/or hypoxemia, including PH with chronic obstructive pulmonary disease (PH-COPD) and PH with interstitial lung disease (PH-ILD), is another common form of PH. The cause is not always known, but it is associated with COPD, sleep breathing disorders and damage tissue from exposure to disease or hazardous materials.

thromboembolic pulmonary hypertension (CTEPH) is caused by persistent blood clots in TEPH is a relatively rare but important form of PH, for which multiple treatment istreated, and patients often lack information or access to surgical and

ctorial (i.e., multiple) causes: This group encompasses various

6

Call to action 2022

CURE PH

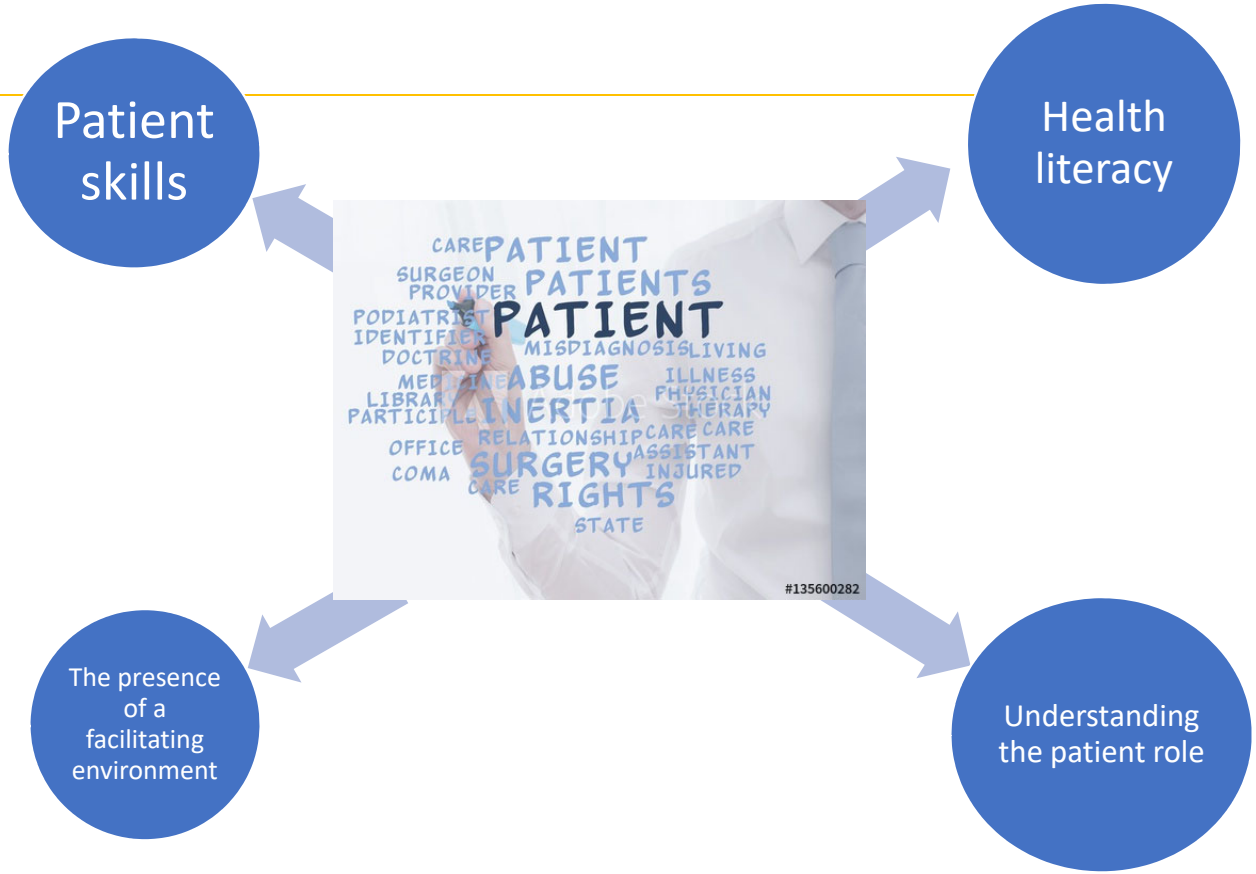
early diagnosis • best treatment • better quality of life • finding a cure

PHA EUROPE for the patients

Patient empowerment

"a process through which people gain greater control over decisions and actions affecting their health" and should be seen as both an individual and a community process.

World Health Organization



Empower patients to make contributions through a proactive inclusive approach to ensure that people with PH are able to talk about their experiences and treatment, providing the input needed to make decisions fundamental to the lives of people living with PH

Angelmar R, Bermann BP. Patient empowerment and efficient health outcomes. Financing sustainable healthcare in Europe. 2007 :139–162. [accessed 26 February 2009]
Health promotion glossary. Geneva: World Health Organization; 1998.

CURE PH

Patient empowerment - health literacy

High or low literacy levels have different impacts, notably on the individual's health and the efficiency of the health system. People with higher health literacy levels demonstrate healthier behaviours, are more adherent to treatment, report less chronic illness, feel healthier, and live longer.*

Importance:

- New technologies
- Demographic change and sustainability
- Self care and better adherence to therapies

- more efficient use of resources
- mitigate the adverse effects of chronic diseases
- increase the patients' quality of life

Bopp M, Minder CE (2003), Mortality by education in German speaking Switzerland, 1990-1997: results from the Swiss National Cohort; International Journal of Epidemiology; 32:346-354

… on a patient associations level

Facilitate real-life information exchange between PH patient associations and other stakeholders, and involve them in debates and discussions from the beginning

Participation in

- **the creation and review of medical guidance**
- **steering committees for clinical trials**
- **scientific meetings at regulatory bodies**
- **advocacy activities in European and national level (eg. ERN, HTA)**

Policy and decision makers at all levels should actively seek and involve patient associations to incorporate the perspectives of people living with PH

EMPOWER PATIENT ASSOCIATIONS

Increase the capability of patient associations to serve patients more effectively, particularly through the provision of accurate, evidence-based, regularly updated information

Up-to-date and reliable information

Brochure, webpage, webinars

EMPOWER PATIENT ASSOCIATIONS

Encourage balanced financial support, including government funding, for patient associations so that they can continue to be independent.

Long term commitment from various sponsors

Diversified source of fundings

Financial calls for NGOs

PLEASE SUPPORT US, PH ASSOCIATIONS, SO WE CAN HELP PATIENTS LIVING WITH PULMONARY HYPERTENSION!

CURE PH