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European
Reference
Networks

ERN-LUNG
RARE RESPIRATORY DISEASES

Evolution of the Role of Patient Associations and challenges for their Future in PH care

GERGELY MESZAROS

2023/12/12

Patient – Physician relationship



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Networks
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RARE RESPIRATORY DISEASES

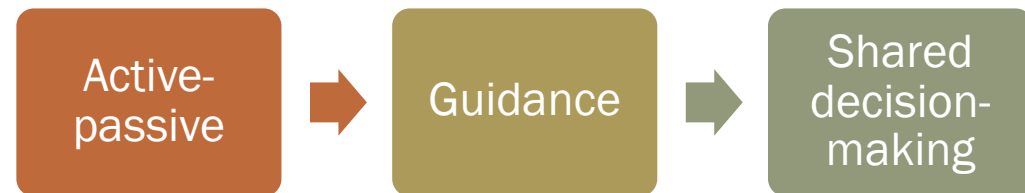


Photo from <https://cancerworld.net/>

Patient organizations



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First mutual
self-help groups
in the 1940s
and 50s

Defending
stigmatizations
in the 60s

Modern
advocacy in the
80s and 90s
(HIV, breast
cancer)

Patients' organisations are defined as not-for-profit organisations which are patient focused, and where patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies.

Patient organizations in the 2022 ESC/ERS guidelines



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12.3. Patient associations and patient empowerment

Pulmonary hypertension centres should inform patients about patient associations and encourage them to join such groups. Patient associations are a valuable resource for managing patients, as they provide educational and emotional support, and can have positive effects on coping, confidence, and outlook [845]. It is recommended that PH centres collaborate with patient associations on initiatives to empower patients and improve the patient experience, addressing issues such as health literacy, digital skills, healthy lifestyles, mental health, and self-management. Health care can be delivered more effectively and efficiently if patients are full partners in the process.

European Scientific Societies

ERN-Lung

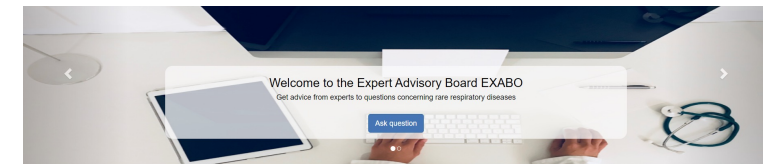
European Reference Network for Rare Respiratory Diseases



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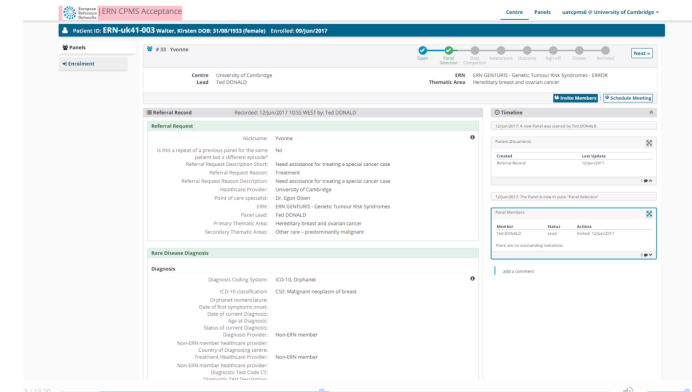
Online tools (CPMS, EXABO)



- About EXABO / Our Mission**
The Online Expert Advice system is designed and implemented in accordance with the EU funded Expert Advice System on Cystic Fibrosis. People living with rare diseases face to their every common challenge, such as delayed or inaccurate...
- Expert Team**
With the Online Expert Advice System, patients and clinicians can address their specific questions concerning rare respiratory diseases to experts who are specialized and experienced in this field. Having access to expert systems on complex health conditions, makes it...
- Target Audience**
Many patients experience difficulties in receiving health care services in their region or country due to sparse, fragmented and uncoordinated services, which means that they don't all have equitable access to treatment services, especially for patients who are suffering...

Mission statement of ERN-Lung

(...) (ERN-LUNG) (...) to develop **innovative care models** and improve **cross border care** (...). (...) provide eHealth tools, will contribute to medical solutions and device development, and will offer services such as cross border health care (...) will **boost research** through large clinical studies and contribute to the development of **new pharmaceuticals**, and will lead to economies of scale and ensure a **more efficient use of costly resources**, which will have a positive impact on the **sustainability** of national healthcare systems, and for (...) patients (...)



European Lung Health Group (ELHG) (I)



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ERN-LUNG
RARE RESPIRATORY DISEASES

- Launched in 2020 to define common advocacy goals and foster closer collaboration on the respiratory field
- Working together with various institutions (eg. WHO, EC, MEP Lung Health Group)
- Advocacy and information-sharing meetings and webinars

RE-NEWED CALL TO ACTION POSITION PAPER

CALL TO ACTION

Improve awareness and screening

- Initiate the awareness campaign for both professionals and public (including CTFPs)
- Create training programmes on PH for health care professionals
- Create national screening and diagnosis programmes, tailored to PH patient subgroups

Empower patient associations

- Facilitate real-life information exchange between the stakeholders, empower patients to ask about their experiences and treatment about PH and include PH patient associations in decisions and decisions which have effect on the lives of PH patients
- Increase the capability of patient associations to serve patients more effectively, particularly through the provision of accurate, evidence-based, regularly updated information and well-balanced financial support

Improve access to expert care

- Establish, properly fund and maintain, and integrate high-volume expert centres in local healthcare systems and patients are treated/drugs prescribed/medical interventions are made in such centres
- Establish, regularly review and maintain national and EU policies (also covering special situations) and PH clinical practice guidelines
- Ensure that drugs (combinations thereof), medical interventions (including transplantation with high-prioritized status for eligible PH patients) are available for PH patients without limitations of national policies

Ensure 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 carers
- Afford disabled status to people living with severe forms of PH

Encourage clinical research and innovation

- Ensure more policy, academic support, funding and incentives for:
 - Basic and PH research that leads to effective treatments for all forms of PH and to non-invasive methods for measuring pulmonary pressure and ultimately a cure for PH
 - Capacity of existing centres to support clinical trials and investigational research

Follow up campaign with 4+(!) MILLION POST IMPRESSIONS ON SOCIAL MEDIA

HYBRID LAUNCH EVENT IN THE EUROPEAN PARLIAMENT



European Lung Health Group (ELHG) (II)



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Vision for respiratory health in 2030:

- Common voice
- Access to treatment
- Early diagnosis
- Clean air
- Research
- Prevention
- Measurable progress



05 May 2021

Accelerating EU research for rare diseases – the case of pulmonary hypertension

To mark World Pulmonary Hypertension Day, on May 5th the MEP Lung Health Group organised a virtual event on April 21 to bring to the attention of the European Parliament the needs arising from the complex nature of rare diseases.





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ERN-LUNG
RARE RESPIRATORY DISEASES

ERS (European Respiratory Society)

ERS is made up of over 35,000 respiratory clinicians, scientists and allied healthcare professionals, spanning more than 160

Patient involvement in many task forces:

- Pharos CRC (severe Pulmonary Hypertension mAnagement acROs Europe)
- Task Force on BPA



2022 ESC/ERS Guidelines for the diagnosis and treatment of pulmonary hypertension

Marc Humbert, Gabor Kovacs, Marius M. Hoeper, Roberto Badagliacca, Rolf M.F. Berger, Margarita Brida, Jörn Carlsen, Andrew J.S. Coats, Pilar Escribano-Subias, Pisana Ferrari, Diogenes S. Ferreira, Hossein Ardeschir Ghofrani, George Giannakoulas, David G. Kiely, Eckhard Mayer, Gergely Meszaros, Blin Nagavci, Karen M. Olsson, Joanna Pepke-Zaba, Jennifer K. Quint, Göran Rådegran, Gerald Simonneau, Olivier Sitbon, Thomy Tonia, Mark Toshner, Jean-Luc Vachiery, Anton Vonk Noordegraaf, Marion Delcroix, Stephan Rosenkranz, the ESC/ERS Scientific Document Group

European Respiratory Journal 2022; DOI: 10.1183/13993003.00879-2022

ERS statement on chronic thromboembolic pulmonary hypertension

Marion Delcroix, Adam Torbicki, Deepa Gopalan, Olivier Sitbon, Frederikus A. Klok, Irene Lang, David Jenkins, Nick H. Kim, Marc Humbert, Xavier Jais, Anton Vonk Noordegraaf, Joanna Pepke-Zaba, Philippe Brénot, Peter Dorfmueller, Elie Fadel, Hossein-Ardeschir Ghofrani, Marius M. Hoeper, Pavel Jansa, Michael Madani, Hiromi Matsubara, Takeshi Ogo, Ekkehard Grünig, Andrea D'Armini, Nazzareno Galie, Bernhard Meyer, Patrick Corkery, Gergely Meszaros, Eckhard Mayer, Gérald Simonneau

European Respiratory Journal 2021 57: 2002828; DOI: 10.1183/13993003.02828-2020

LIVING WITH RARE RESPIRATORY DISEASES THROUGH THE COVID-19 PANDEMIC

FREE REGISTRATION
LIVE PANEL DISCUSSION
23 MARCH, 2021 at 19:00 CET



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ESC - European Society of Cardiology

A community of healthcare professionals, including cardiologists, cardiac surgeons, nurses, and other allied health professionals, who are involved in the field of cardiovascular medicine.

2022 ESC/ERS Guidelines for the diagnosis and treatment of PH with the involvement of two patient representatives were included for the first time ever as co-authors

The largest and most influential cardiology organizations in the world.

European Society of Cardiology quality indicators for the care and outcomes of adults with pulmonary arterial hypertension. Developed in collaboration with the Heart Failure Association of the European Society of Cardiology

Suleman Aktaa ^{1 2 3}, Chris P Gale ^{1 2 3}, Margarita Brida ^{4 5}, George Giannakoulas ⁶, Gabor Kovacs ⁷, Yochai Adir ⁸, Raymond L Benza ⁹, Michael Böhm ¹⁰, Andrew Coats ¹¹, Michele D'Alto ¹², Pilar Escribano-Subias ¹³, Pisana Ferrari ¹⁴, Nazzareno Galiè ^{15 16}, J Simon R Gibbs ¹⁷, Wendy Gin-Sing ¹⁸, Marius M Hoeper ^{19 20}, Marc Humbert ²¹, Irene M Lang ²², Bradley A Maron ²³, Gergely Meszaros ²⁴, Anton Vonk Noordegraaf ^{25 26}, Laura C Price ^{27 28}, Joanna Pepke-Zaba ²⁹, Göran Rådegran ^{30 31}, Abilio Reis ^{32 33}, Olivier Sitbon ²¹, Adam Torbicki ³⁴, Silvia Ulrich ³⁵, Stephan Rosenkranz ^{# 36}, Marion Delcroix ^{# 37 38}



ELF/ESC/ACNAP



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Patient advisory group (PAG)
European Patient Advisory
Programme (EPAP)



DIAGNOSING AND TREATING
PULMONARY HYPERTENSION
Understanding the professional guidelines



Patient Forum at the European Heart House - October 2018

ESC – Patient Forum

The vision is to have professionals and patients working together to make a meaningful contribution to all objectives of the ESC (...). We want to develop and enhance patient engagement so that it is embedded like a golden thread through all ESC activities.



ESOT - European Society for Organ Transplant



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ESOT is a medical and scientific society dedicated to the exchange of knowledge in donation and transplantation.

ESOT provides an extensive education programme and best-practice guidelines for professionals as well as opportunities for knowledge exchange

In 2019 ESOT launched a “Patient Inclusion Initiative”

In 2020 a European Transplant Patient Organisation (ETPO) was established under the aegis of ESOT, followed by the launch of the ESOT-ETPO Alliance



Credit to Pisana Ferrari for the slide

WSPH

World Symposium on Pulmonary Hypertension



Patient involvement



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ERN-LUNG
RARE RESPIRATORY DISEASES

First congress in Geneva in 1973 (17 participants).

Last in Nice in 2018 (1376 participants):

- Task force for patient perspective
- The importance of patient perspectives in pulmonary hypertension

Michael D McGoon, Pisana Ferrari, Iain Armstrong, Migdalia Denis, Luke S Howard, Gabi Lowe, Sanjay Mehta, Noriko Murakami, Brad A Wong

7TH World Symposium on Pulmonary Hypertension Barcelona, Spain 2024

7TH World Symposium on Pulmonary Hypertension

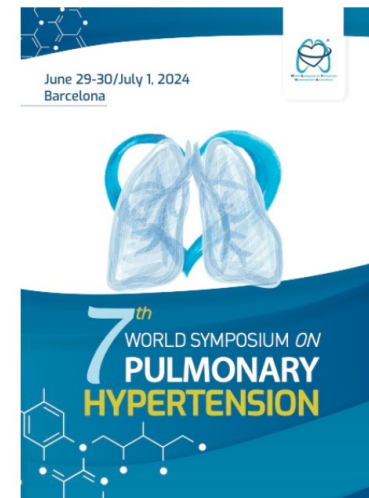
12 Members Scientific

129 Members of Task Forces

(15 Working Groups)

1400 Attendees

Proceedings published on ERJ suppl. 2019



European Regulatory Authority



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European Medicines Agency (EMA) (I.)

Centralised marketing authorization system in EU (reimbursement still at membership level)

Patients and consumers involvement since 1996:

Patients representing their community

- Management Board
- Scientific Committee(s)

Patients representing their organisation

- Patients' and Consumers' Working Party (PCWP)
- EMA consultations
- Workshops

Patients as individual experts

- Scientific advice / protocol assistance procedures
- Scientific advisory / ad hoc expert groups
- Scientific committee consultations
- Review of documents



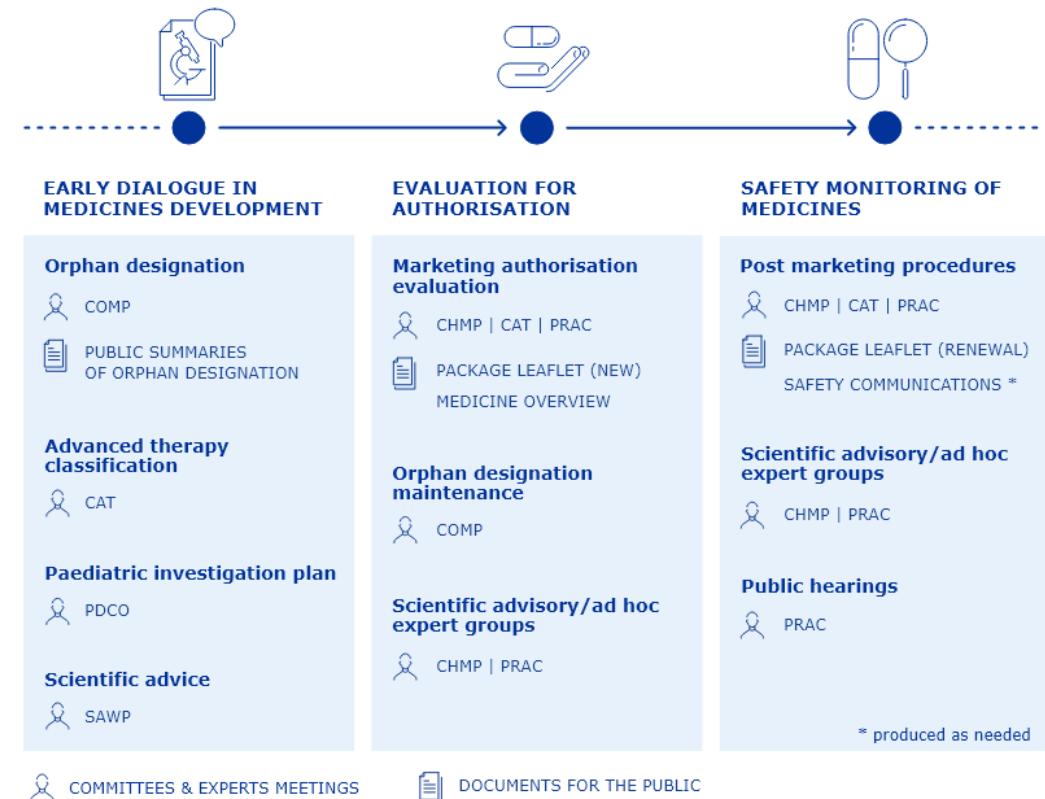
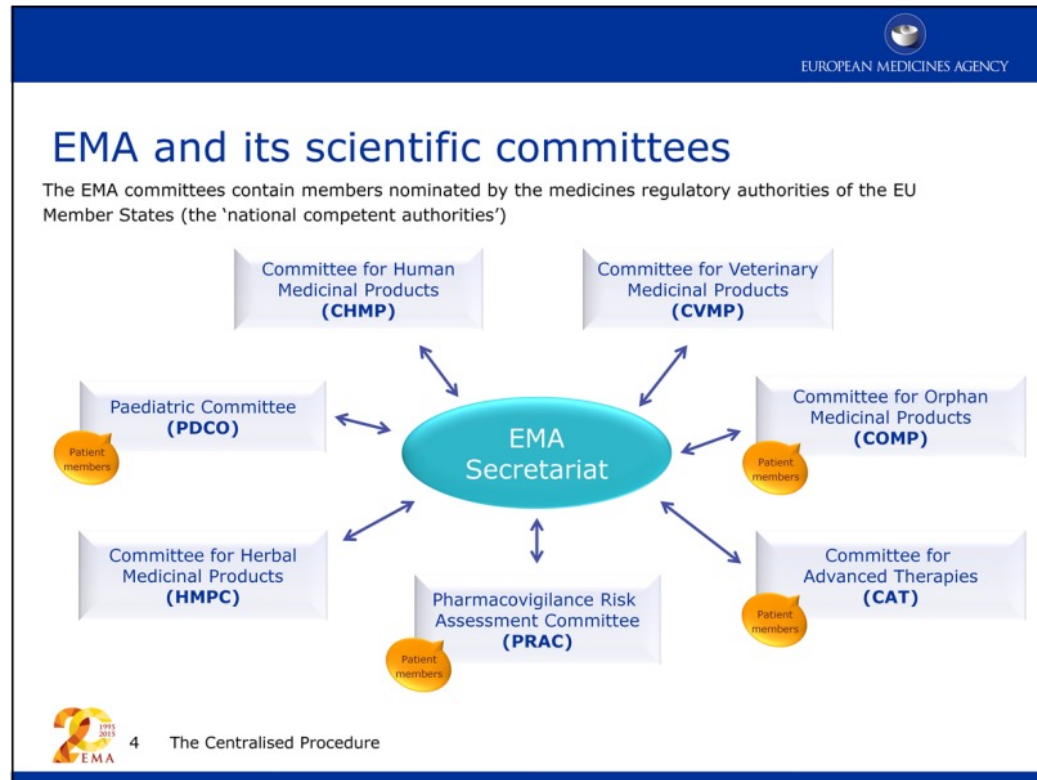
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH



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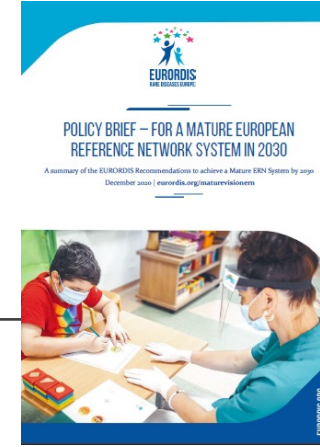
European Medicines Agency (EMA) (II.)



European Public Health Organizations

EUROORDIS

1000+ member rare disease associations from 74 countries



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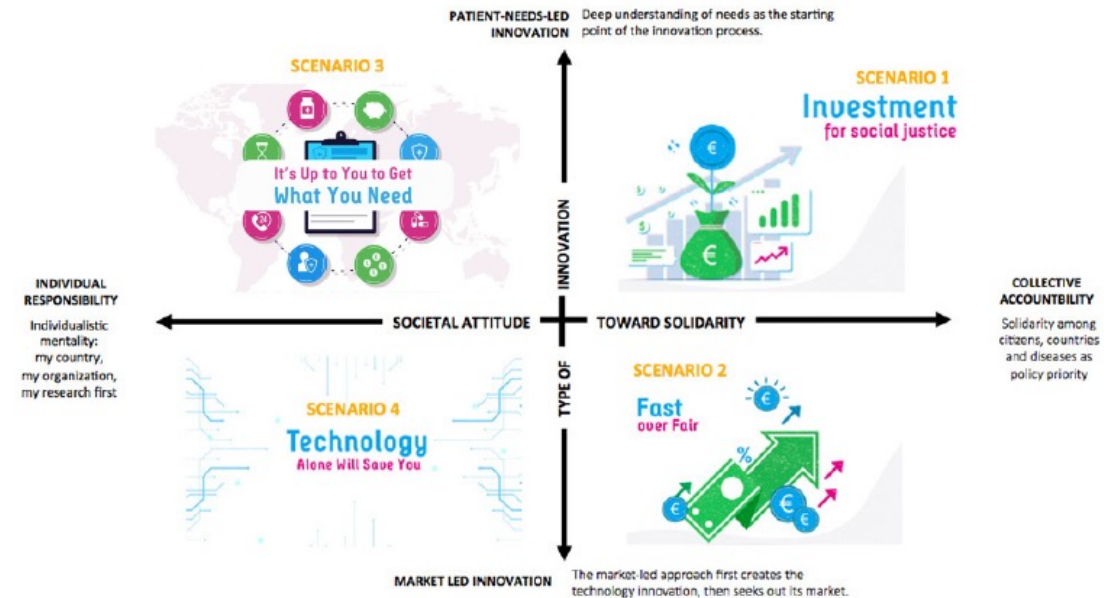


Rare disease policy

European action plan for rare diseases
#30millionreasons

Parliamentary advocates

Rare2030 study – 8 recommendations





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EPF - European Patients Forum



SHAPING A NEW EUROPEAN AGENDA FOR PATIENTS
MEANINGFUL PATIENT INVOLVEMENT IN CO-CREATING BETTER
HEALTH POLICY, PRACTICE, RESEARCH AND EDUCATION
DIGITAL TRANSFORMATION THAT DELIVERS FOR PATIENTS
ACCESSING THE HEALTHCARE WE NEED WITH NO
DISCRIMINATION
STRENGTHENING PATIENT COMMUNITIES ACROSS EUROPE



Universal Health Coverage
For All.
#Access2030

Challenges

Funding

Criticism about independence/reliability

Stability

Professionalism

„Outsourcing” PAs’ roles

„Insourcing” healthcare system’s roles



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Opportunities

HTA procedures

Role in drug research

Collaboration with stakeholders

Participation in scientific work



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Many thanks for your
attention!