

Pulmonary hypertension, rare diseases and EU policies

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European
Reference
Network
for rare or low prevalence
complex diseases
Network
Respiratory Diseases
(ERN-LUNG)

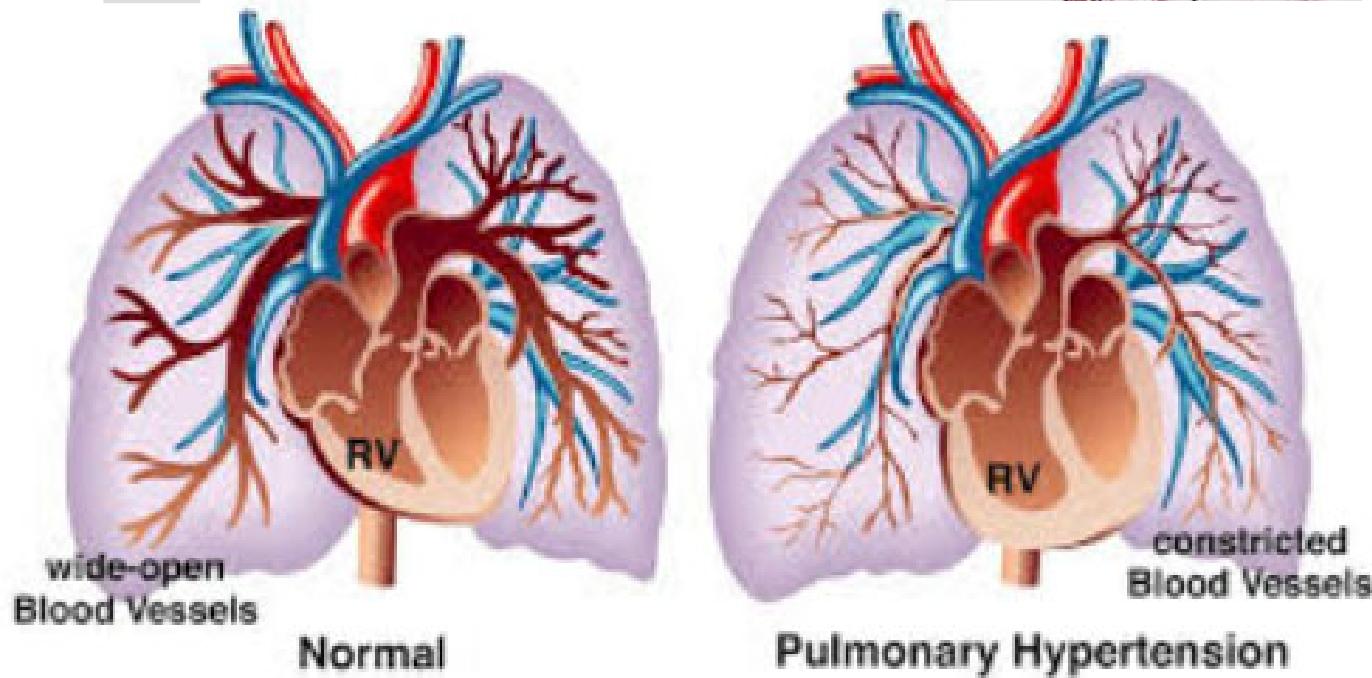
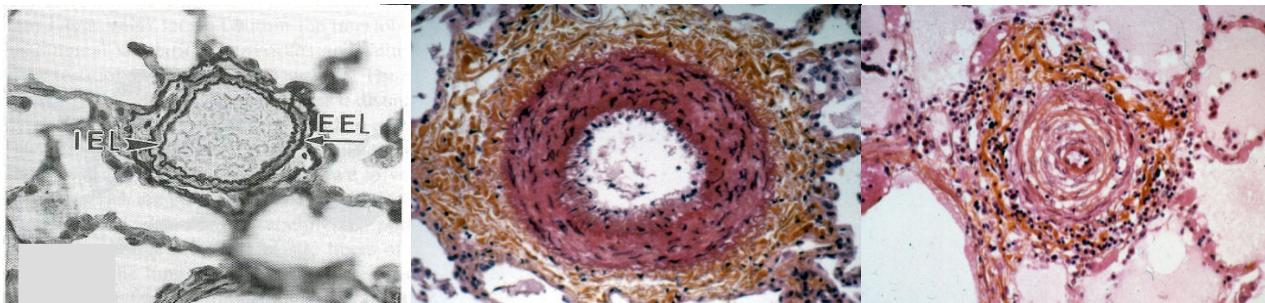
DISCLOSURES

- **Speaker, consultant, or steering committee member:**
 - Acceleron, AOP, Bayer, Daiichi Sankyo, Ferrer, Inari, Janssen, MSD, Pfizer
- **Research grants:**
 - Janssen

PULMONARY HYPERTENSION, RARE DISEASES AND EU POLICIES

- **What is PH? When to suspect PH?**
- **From a frequent anomaly to rare forms of PH**
 - Pulmonary arterial hypertension (PAH)
- **Remarkable orphan drug development - Still high mortality**
 - Vasodilators
 - Pipeline
- **Knowledge dissemination**
 - Practice guidelines and statements
 - 1st-6th World Symposia on PH
- **National policies vs. EU policies**
- **Key messages and action points**

WHAT IS PULMONARY HYPERTENSION (PH)?





WHEN TO SUSPECT PH?



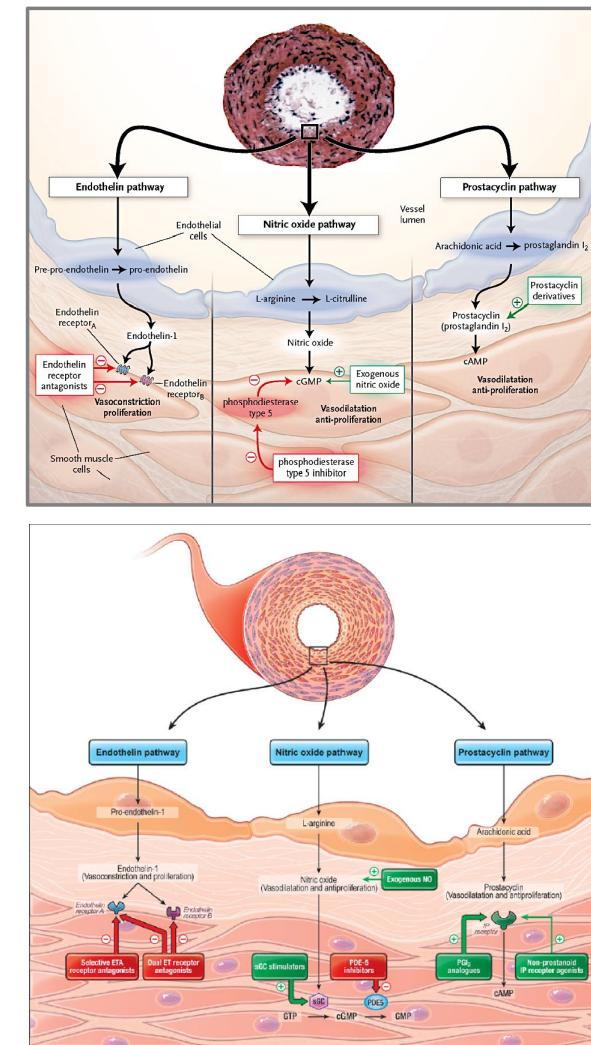
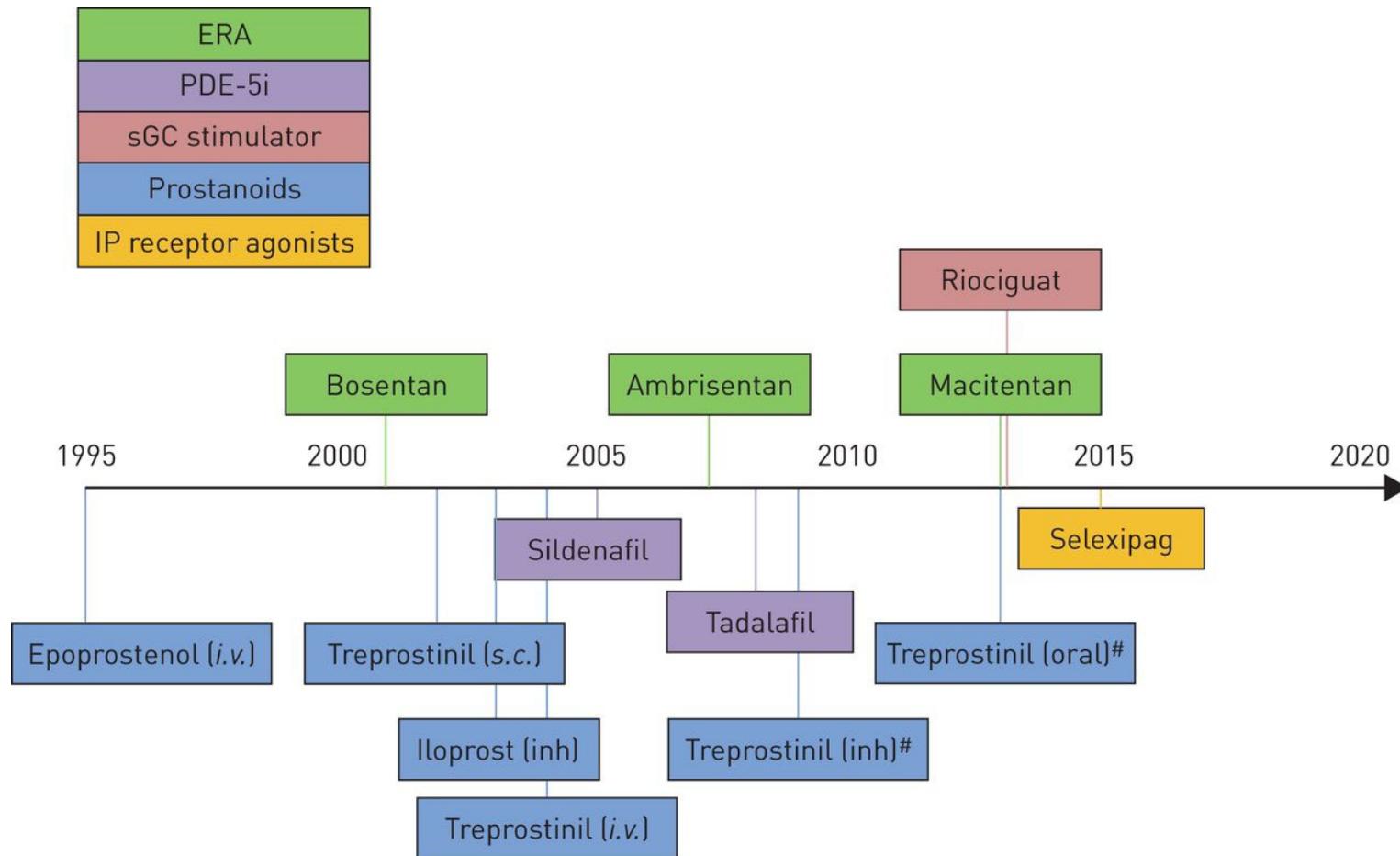
PH IS A FREQUENT ANOMALY (1%)

BUT SOME FORMS ARE RARE (<1/2.000)

Group	Description	Prevalence
Group 1	Pulmonary arterial hypertension (PAH)	50 per million 1/20.000
Group 2	Pulmonary hypertension due to left heart disease	very frequent
Group 3	Pulmonary hypertension due to lung disease	frequent
Group 4	Chronic thromboembolic pulmonary hypertension (CTEPH)	30 per million
Group 5	PH due to unclear or multifactorial causes	very rare

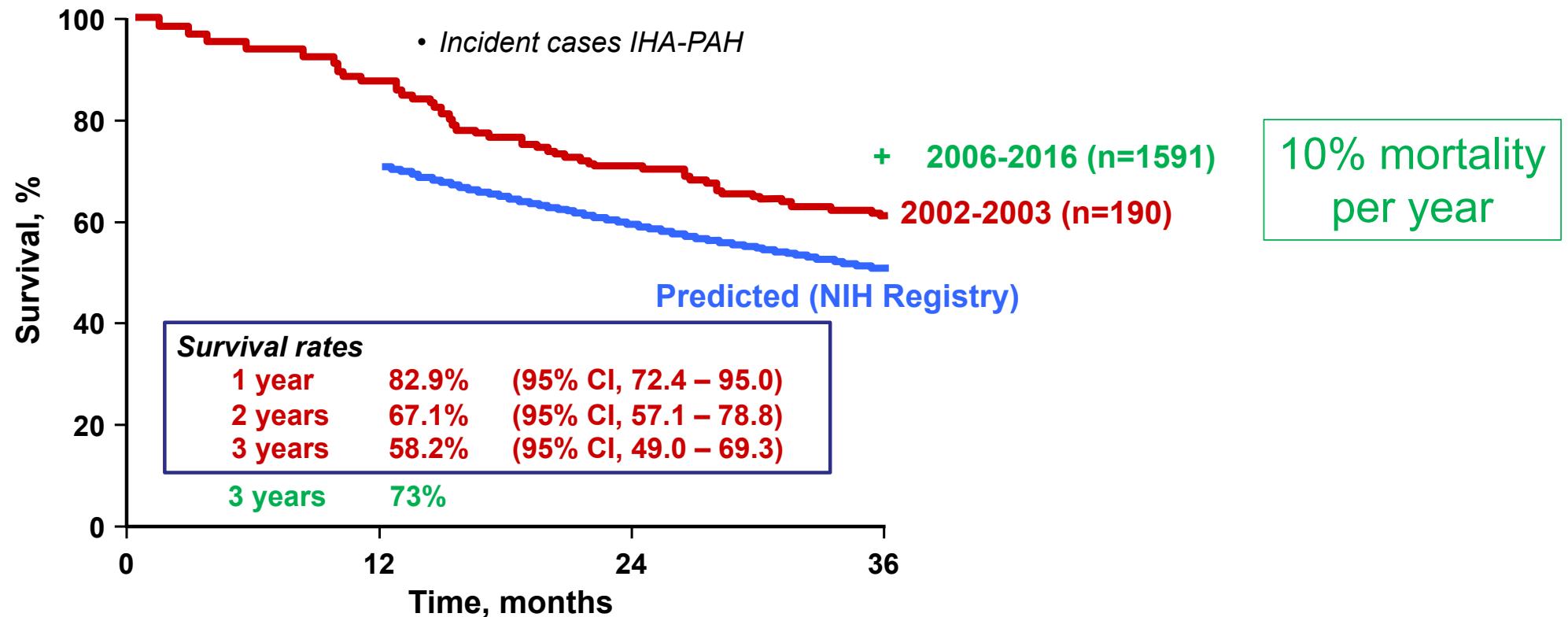
PAH TREATMENTS (vasodilators)

2004 - 2014



Gaine & McLaughlin, ERR 2017; Humbert et al. N Engl J Med 2004; Humbert et al. Circulation 2014

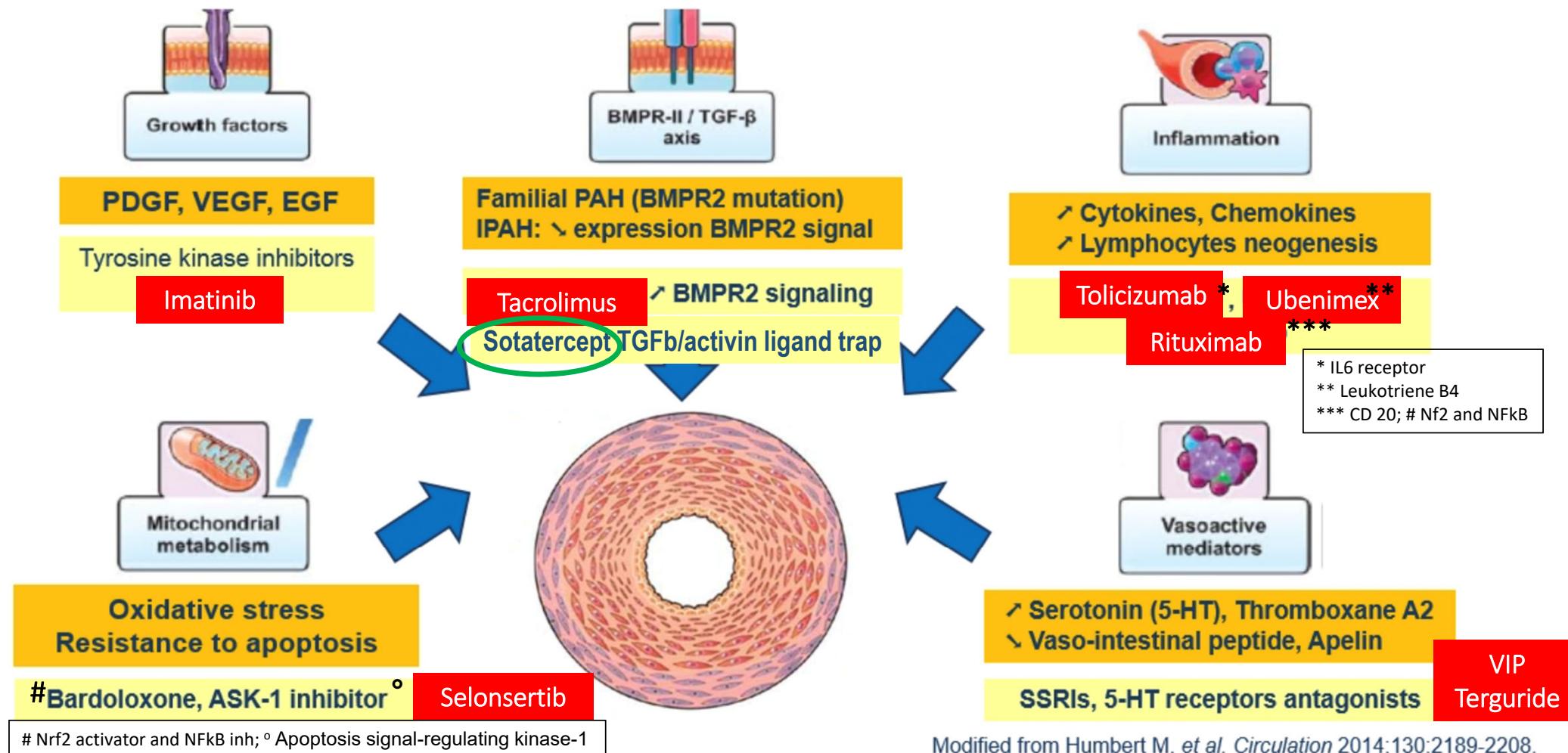
DESPITE DRUG DISCOVERY AND EDUCATION PAH REMAINS A DEVASTATING CONDITION



Humbert M, et al. *Circulation* 2010;122:156–63.

Boucly et al. *Eur Respir J* 2017; 50: p1700889.

OTHER APPROACHES (targeting proliferation)



Modified from Humbert M, et al. Circulation 2014;130:2189-2208.

PAH/PH/CTEPH GUIDELINES - STATEMENTS

 ELSEVIER

2004

 EUROPEAN SOCIETY OF CARDIOLOGY®

ESC Guidelines

Guidelines on diagnosis and treatment of pulmonary arterial hypertension

The Task Force on Diagnosis and Treatment of Pulmonary Arterial Hypertension of the European Society of Cardiology

 European Heart Journal
doi:10.1093/eurheartj/ehp297

2009

ESC/ERS GUIDELINES

 [†] **Guidelines for the diagnosis and treatment of pulmonary hypertension**

The Task Force for the Diagnosis and Treatment of Pulmonary Hypertension of the European Society of Cardiology (ESC) and the European Respiratory Society (ERS), endorsed by the International Society of Heart and Lung Transplantation (ISHLT)

 European Heart Journal
doi:10.1093/eurheartj/ehv317

2015

ESC/ERS GUIDELINES

 EUROPEAN RESPIRATORY SOCIETY

 **2015 ESC/ERS Guidelines for the diagnosis and treatment of pulmonary hypertension**

The Joint Task Force for the Diagnosis and Treatment of Pulmonary Hypertension of the European Society of Cardiology (ESC) and the European Respiratory Society (ERS)

Endorsed by: Association for European Paediatric and Congenital Cardiology (AEPC), International Society for Heart and Lung Transplantation (ISHLT)



ERS OFFICIAL DOCUMENT
ERS STATEMENT

 **2017**

CrossMark

An official European Respiratory Society statement: pulmonary haemodynamics during exercise

 **2019**

CrossMark

ERS statement on exercise training and rehabilitation in patients with severe chronic pulmonary hypertension

 **2021**

CrossMark

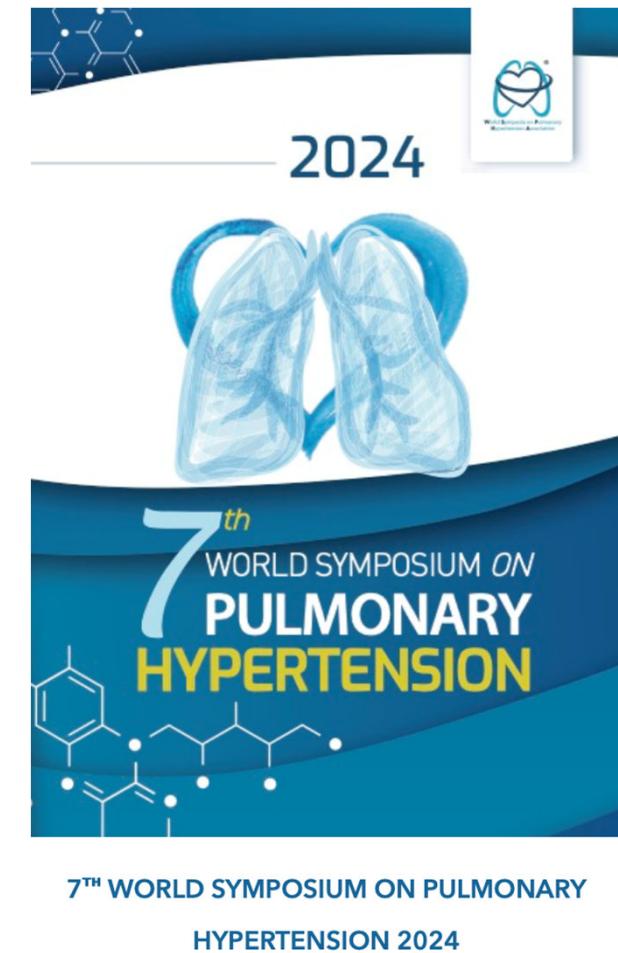
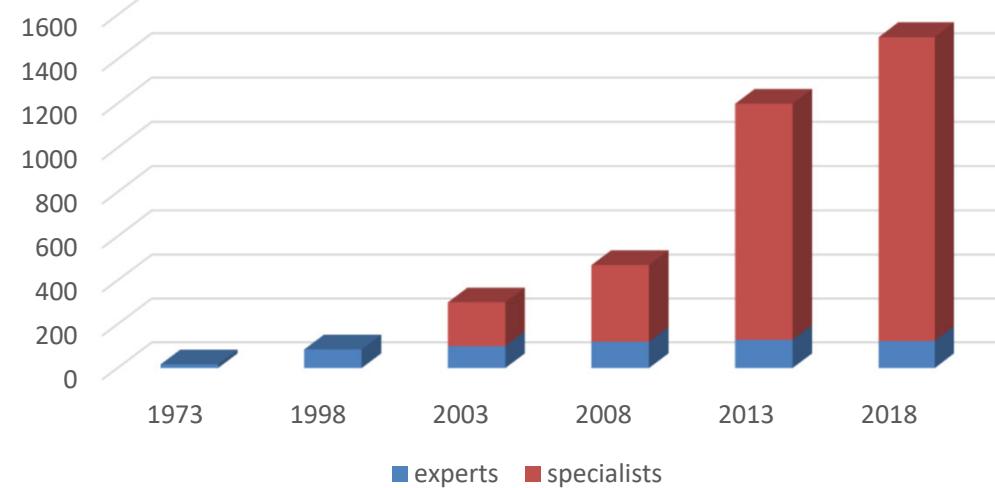
ERS statement on chronic thromboembolic pulmonary hypertension

ANNOUNCED

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



WORLD SYMPOSIA ON PULMONARY HYPERTENSION



RARE DISEASE POLICIES IN EU

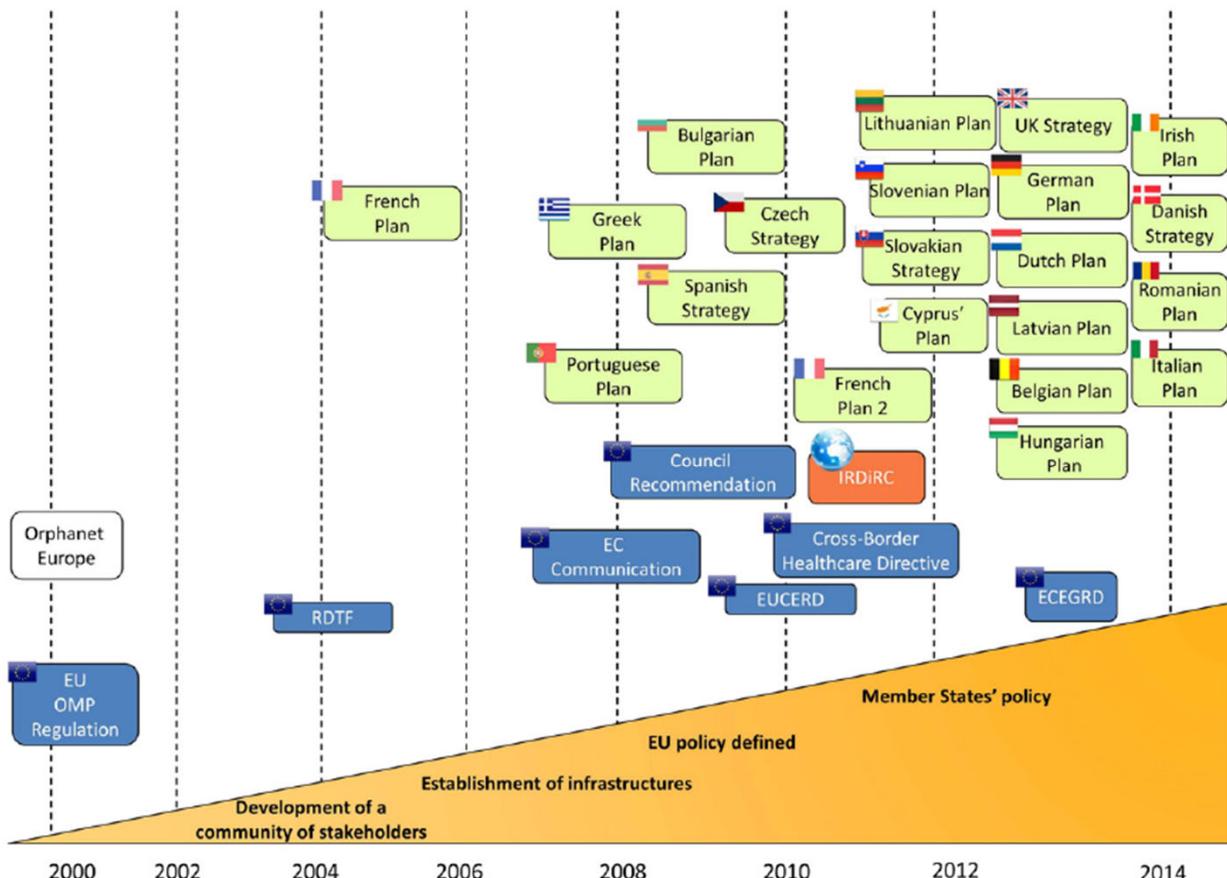
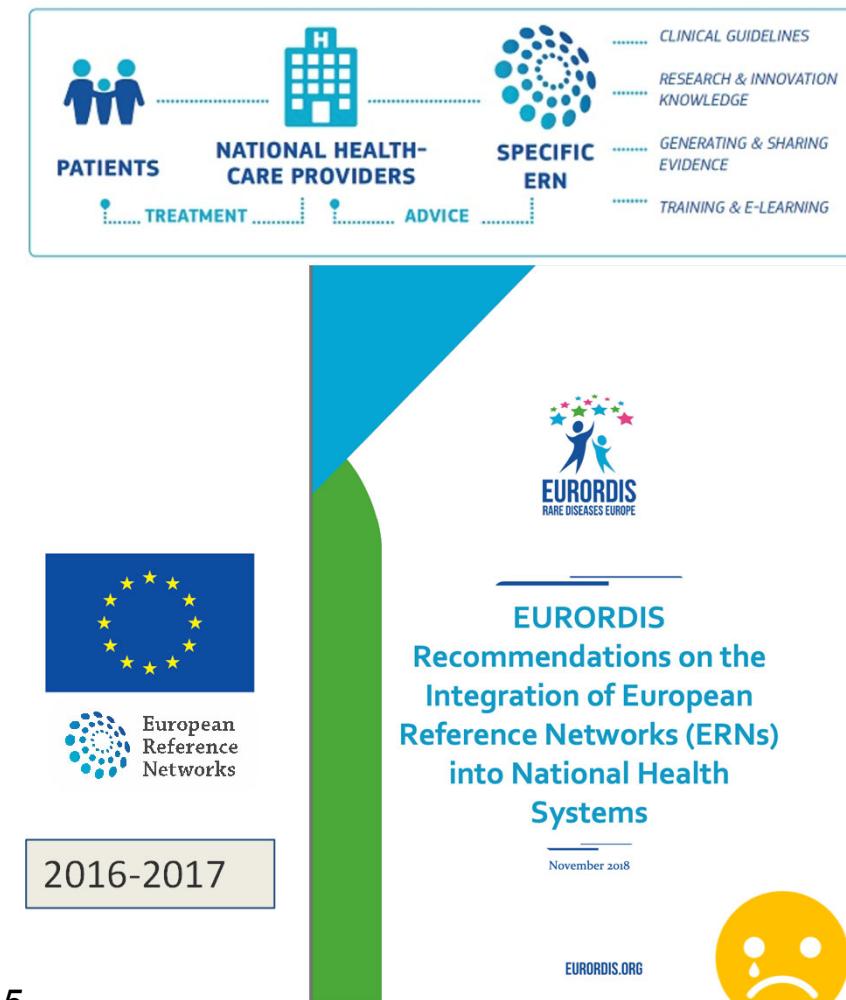


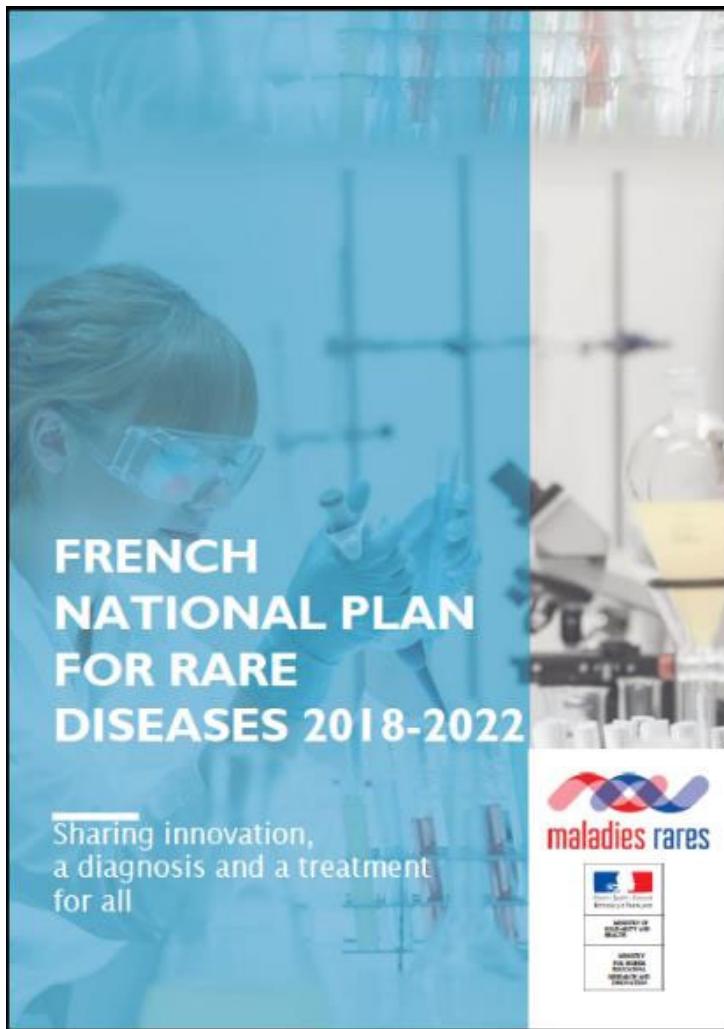
Fig. 1. Evolution of rare disease policy in Europe (December 2014) [4].

Rodwell & Aymé, *Biochimica et Biophysica Acta* 2015



EURORDIS.ORG

FR as the best example of a large country with a well defined RD plan

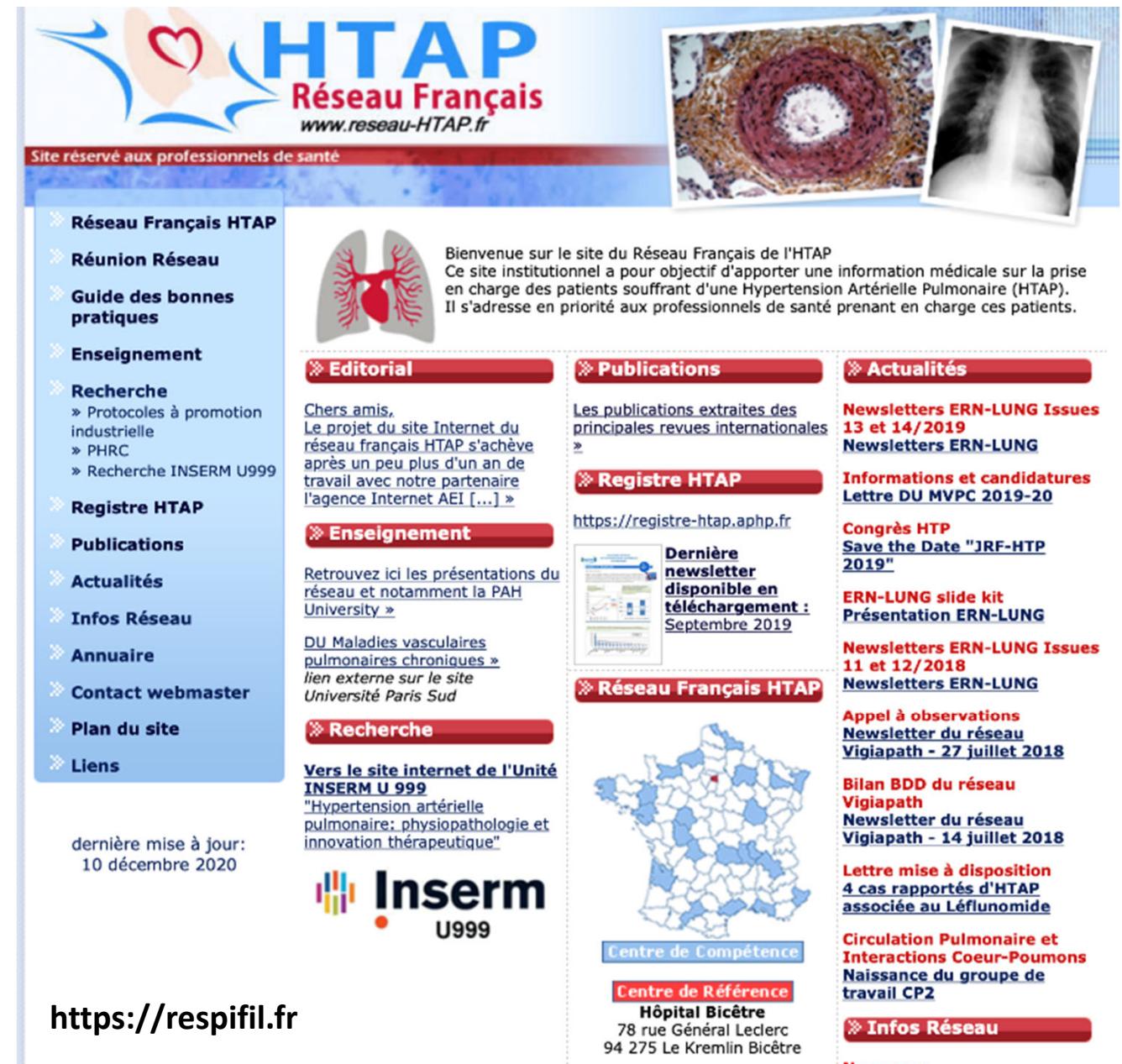


FRENCH NATIONAL PLAN FOR RARE DISEASES 2018-2022

Sharing innovation, a diagnosis and a treatment for all

maladies rares

Ministère de la Santé et des Solidarités
Ministère de l'Éducation Nationale et de la Recherche
Ministère de l'Europe et des Affaires Étrangères



HTAP Réseau Français
www.reseau-HTAP.fr

Site réservé aux professionnels de santé

Réseau Français HTAP

- Réunion Réseau
- Guide des bonnes pratiques
- Enseignement
- Recherche
 - » Protocoles à promotion industrielle
 - » PHRC
 - » Recherche INSERM U999
- Registre HTAP
- Publications
- Actualités
- Infos Réseau
- Annuaire
- Contact webmaster
- Plan du site
- Liens

dernière mise à jour:
10 décembre 2020

Bienvenue sur le site du Réseau Français de l'HTAP. Ce site institutionnel a pour objectif d'apporter une information médicale sur la prise en charge des patients souffrant d'une Hypertension Artérielle Pulmonaire (HTAP). Il s'adresse en priorité aux professionnels de santé prenant en charge ces patients.

Editorial

Chers amis,
Le projet du site Internet du réseau français HTAP s'achève après un peu plus d'un an de travail avec notre partenaire l'agence Internet AEI [...] »

Enseignement

Retrouvez ici les présentations du réseau et notamment la PAH University »

DU Maladies vasculaires pulmonaires chroniques » lien externe sur le site Université Paris Sud

Recherche

Vers le site internet de l'Unité INSERM U 999
"Hypertension artérielle pulmonaire: physiopathologie et innovation thérapeutique"

Inserm
U999

Centre de Compétence

Centre de Référence
Hôpital Bicêtre
78 rue Général Leclerc
94 275 Le Kremlin Bicêtre

Publications

Les publications extraites des principales revues internationales »

Registre HTAP

<https://registre-htap.aphp.fr>

Dernière newsletter disponible en téléchargement : Septembre 2019

Réseau Français HTAP



Actualités

Newsletters ERN-LUNG Issues 13 et 14/2019
Newsletters ERN-LUNG

Informations et candidatures Lettre DU MVPC 2019-20

Congrès HTP
Save the Date "JRF-HTP 2019"

ERN-LUNG slide kit
Présentation ERN-LUNG

Newsletters ERN-LUNG Issues 11 et 12/2018
Newsletters ERN-LUNG

Appel à observations
Newsletter du réseau
Vigiapath - 27 juillet 2018

Bilan BDD du réseau
Vigiapath
Newsletter du réseau
Vigiapath - 14 juillet 2018

Lettre mise à disposition
4 cas rapportés d'HTAP associée au Léflunomide

Circulation Pulmonaire et Interactions Coeur-Poumons
Naissance du groupe de travail CP2

Infos Réseau

UK: another large country with advanced care organisation



Publication, Part of National Pulmonary Hypertension Audit

National Pulmonary Hypertension Audit, 12th Annual Report

Audit, Open data

Publication Date: 20 Jan 2022
Geographic Coverage: England, Scotland
Geographical Granularity: NHS Trusts, Country, Hospital Trusts
Date Range: 01 Apr 2020 to 31 Mar 2021

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The following documents are published alongside the main report:

- Tables and Charts (Excel)
- Open Data (csv)
- Data Quality Report
- Dana Point Classification
- Staff Involved in Audit



ERN-LUNG is made up of **9 Core Networks** representing the diversity of diseases and conditions affecting the lungs, and **9 Functional Committees** that coordinate transversal activities affecting all of the current and future Core Networks.

CF

Cystic Fibrosis

PCD

Primary Ciliary Dyskinesia

nCF-BE

Non-CF Bronchiectasis

PH

Pulmonary Hypertension

ILD

Interstitial Lung Disease

CLAD

Chronic Lung Allograft Dysfunction

MSTO

Mesothelioma

AATD

Alpha-1 AntiTrypsin Deficiency

ORLD

Other Rare Lung Diseases

1

Research & Clinical Trials

2

Clinical Guidelines & Best Practice of Care

3

Registries & Biobanks

4

Communication & Outreach

5

Ethical Issues

6

Cross Border Care

7

Professional Training & Continued Medical Education

8

Patient Recorded Outcomes (PROs) & Quality of Life

9

Quality Management



HOME



European Reference Network

for rare or low prevalence complex diseases

Network

Respiratory Diseases
(ERN-LUNG)

Domain: PH

- 27 full members
- 4 affiliated partners
- 4 UK supporting partners

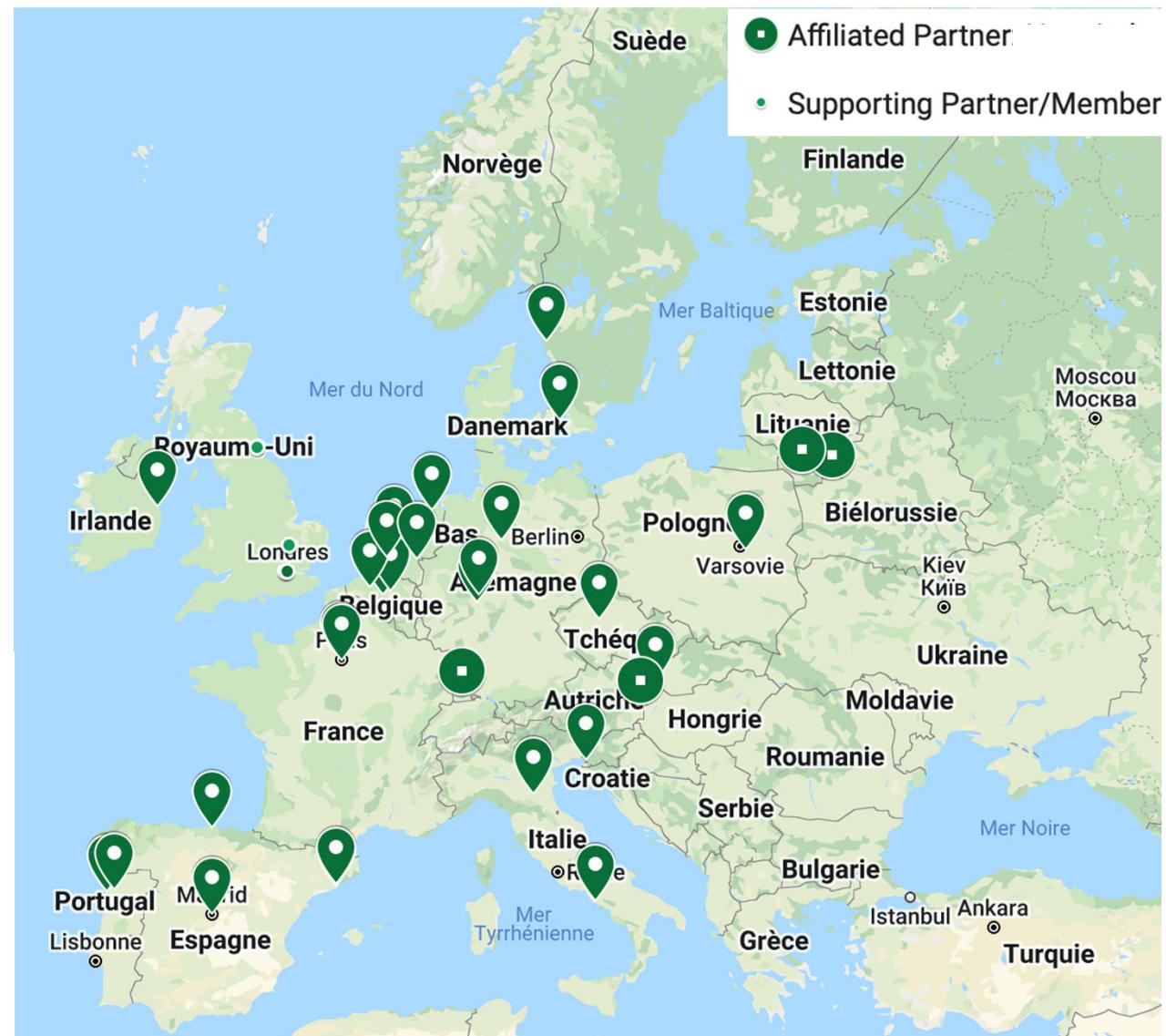


PHAROS
Severe Pulmonary Hypertension Management Across Europe

EDITORIAL
ERS CLINICAL RESEARCH COLLABORATIONS

Severe Pulmonary Hypertension Management Across Europe (PHAROS): an ERS Clinical Research Collaboration

Olivier Sitbon ^{1,2,3}, Abílio Reis ⁴, Marc Humbert ^{1,2,3}, Anton Vonk Noordegraaf⁵ and Marion Delcroix ^{6,7}, on behalf of the PHAROS Clinical Research Collaboration



Sitbon et al, Eur Respir J 2020

Key messages

rare

The prevalence of PAH averages 50 per million inhabitants

drugs

14 agents approved in the last 25 years

fatal

Median survival still averages 6 years

EU

Diverse care organisations and recognition/ignorance of expertise

ACTION POINTS

- **Stronger EU incentives for — and control of — the implementation of national RD plans, including**
 - Clear identification and financing of dedicated expert centres
 - Improved RD care organisation and audits
- **Expedited new drugs approval/reimbursement**
 - Closer collaboration between EMA and EU Member States
- **More effective incentives on RD research**
 - Not restricted to genetic diseases; embedded in the EU scientific societies
- **ERNs**
 - Less administrative burden, more transparency, more coordinated actions between ERNs
 - Global solutions concerning registries, data sharing regulation, ...
 - Reconsidering the funding structure (project-driven, ...)