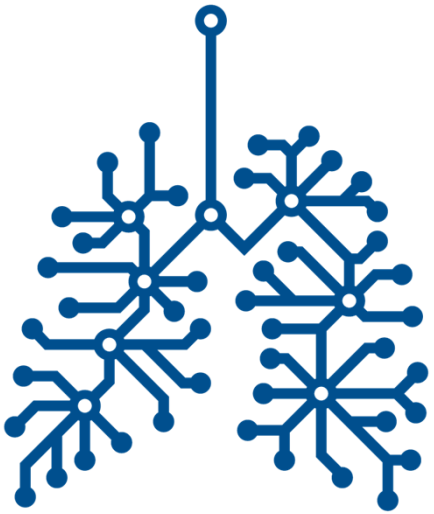
ERS

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

PHA EUROPE for the patients

PULMONARY HYPERTENSION ASSOCIATION

MEP Lung Health Group



Pulmonary hypertension, rare diseases and EU policies

Marion Delcroix, University Hospitals Leuven, Belgium

KU LEUVEN

UZ LEUVEN

ICA International CTEPH Association

PHAROS Severe Pulmonary Hypertension Management Across Europe

World Symposia on Pulmonary Hypertension Association

European Reference Network for rare or low prevalence complex diseases

Network Respiratory Diseases (ERN-LUNG)

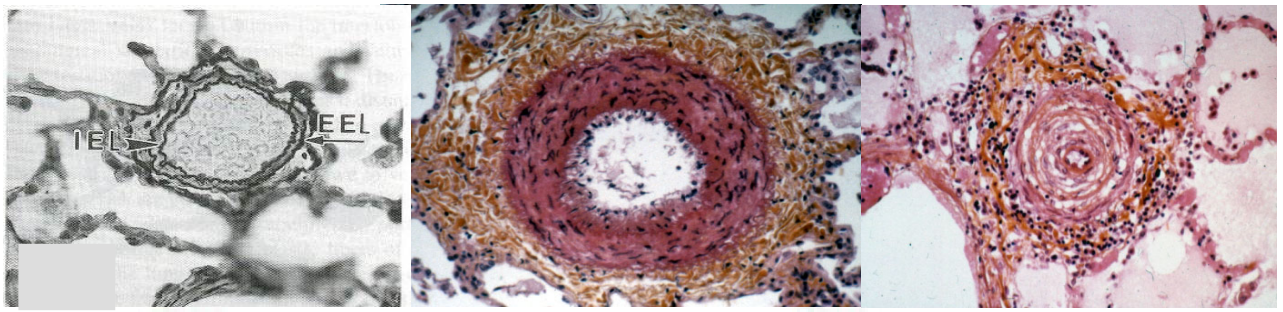
DISCLOSURES

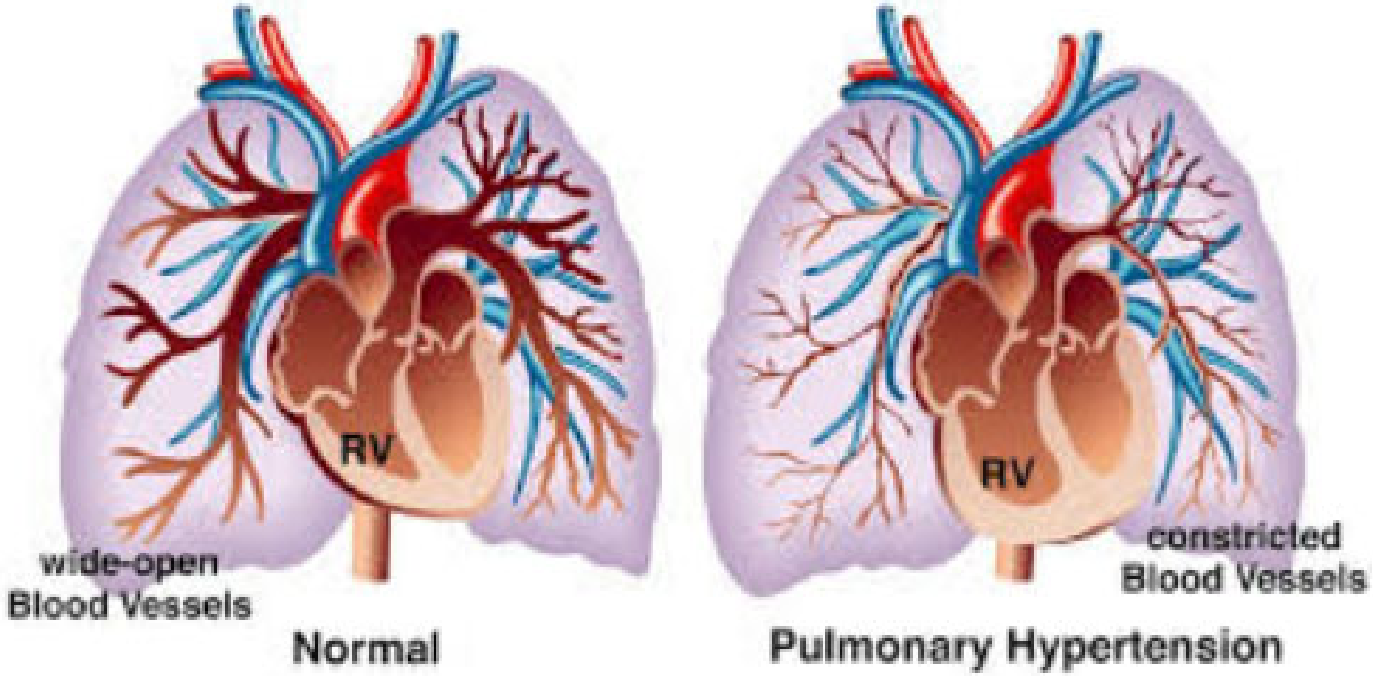
- **Speaker, consultant, or steering committee member:**
  - Acceleron, AOP, Bayer, Daiichi Sankyo, Ferrer, Inari, Janssen, MSD, Pfizer
- **Research grants:**
  - Janssen

PULMONARY HYPERTENSION, RARE DISEASES AND EU POLICIES

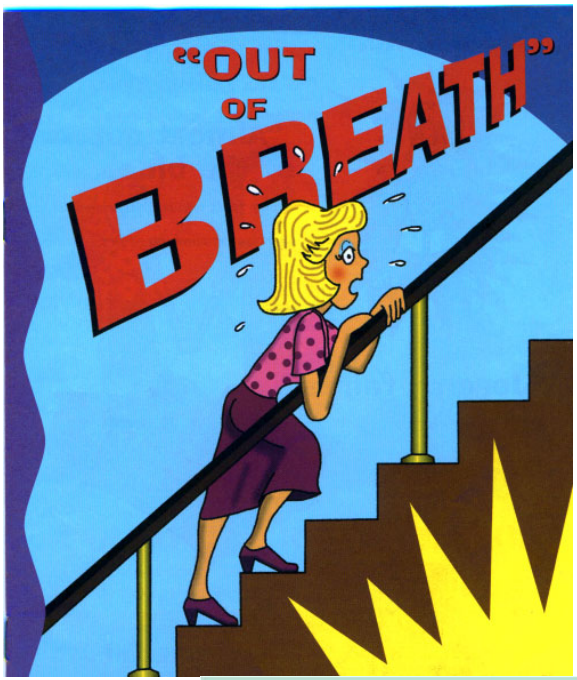
- **What is PH? When to suspect PH?**
- **From a frequent anomaly to rare forms of PH**
  - Pulmonary arterial hypertension (PAH)
- **Remarkable orphan drug development - Still high mortality**
  - Vasodilators
  - Pipeline
- **Knowledge dissemination**
  - Practice guidelines and statements
  - 1st-6th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

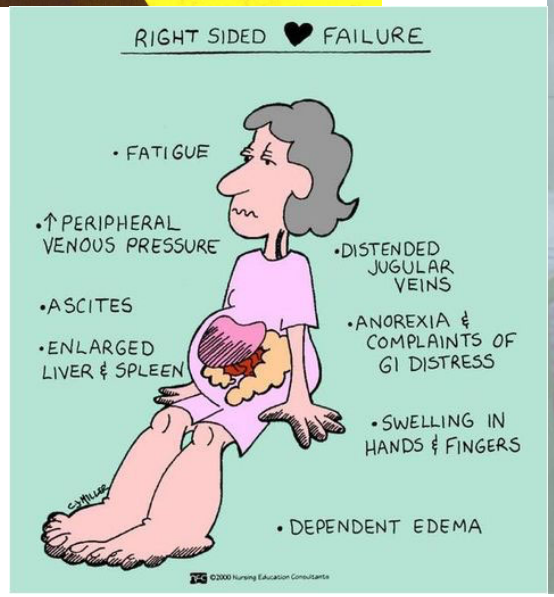
WHAT IS PULMONARY HYPERTENSION (PH)?





WHEN TO SUSPECT PH?





PH IS A FREQUENT ANOMALY (1%)

BUT SOME FORMS ARE RARE (<1/2.000)

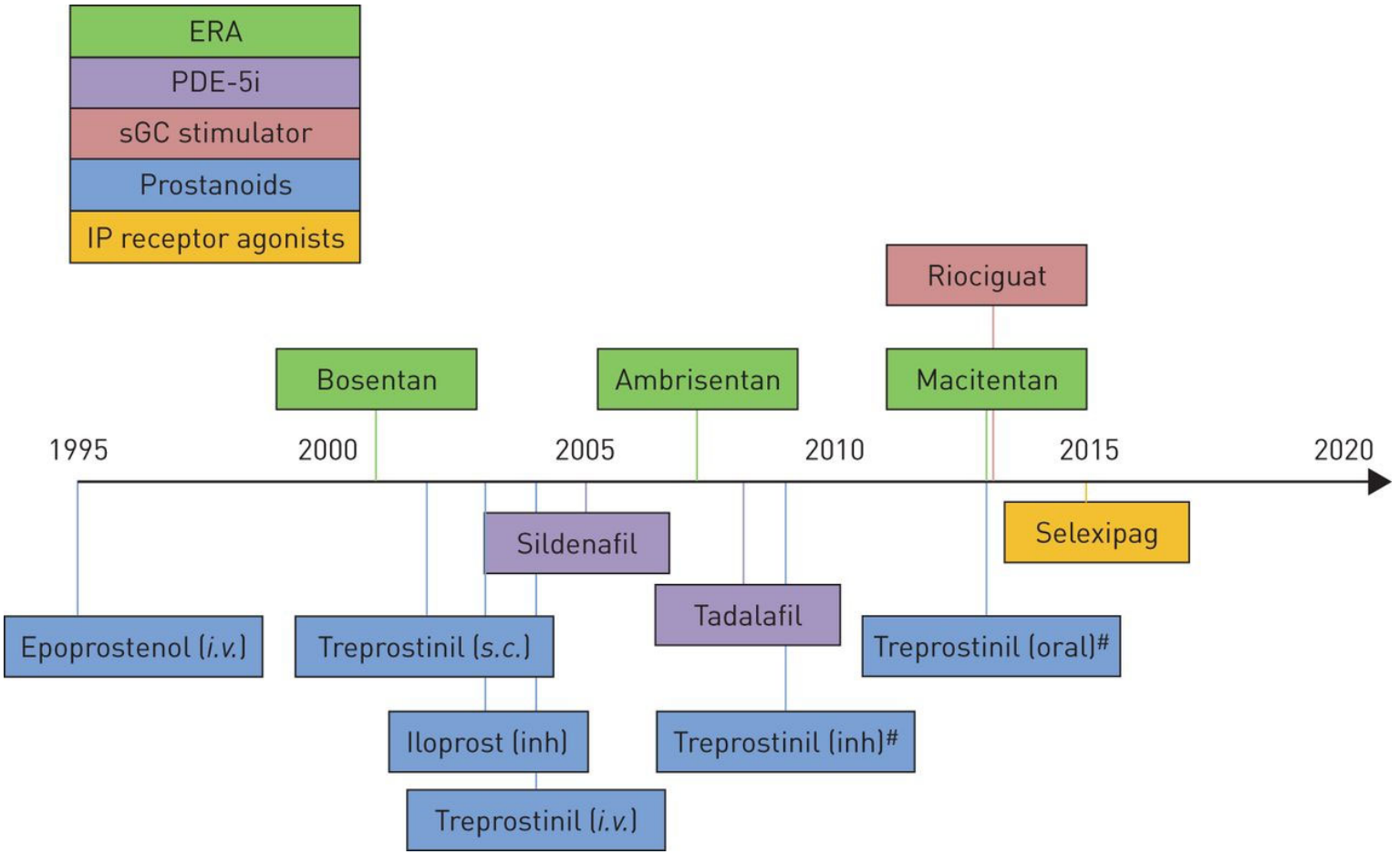
Group	Description	Prevalence
Group 1	Pulmonary arterial hypertension (PAH)	50 per million
Group 2	Pulmonary hypertension due to left heart disease	very frequent
Group 3	Pulmonary hypertension due to lung disease	frequent
Group 4	Chronic thromboembolic pulmonary hypertension (CTEPH)	30 per million
Group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

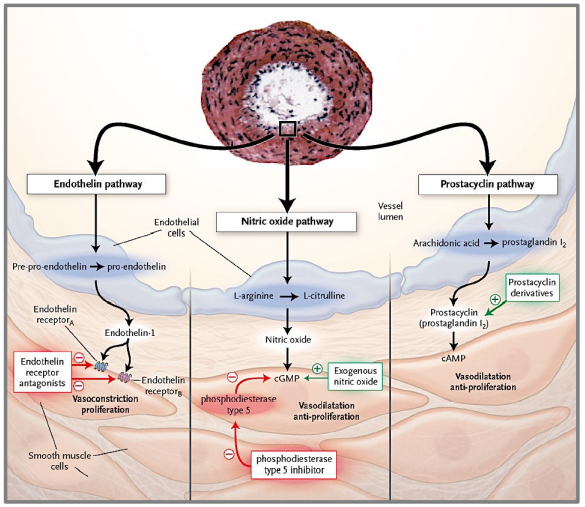
1/20.000

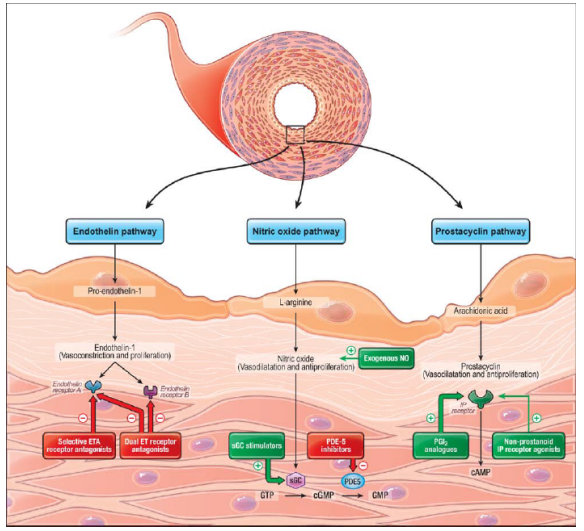
Galie et al, EHJ and ERJ 2015

PAH TREATMENTS (vasodilators)

2004 - 2014

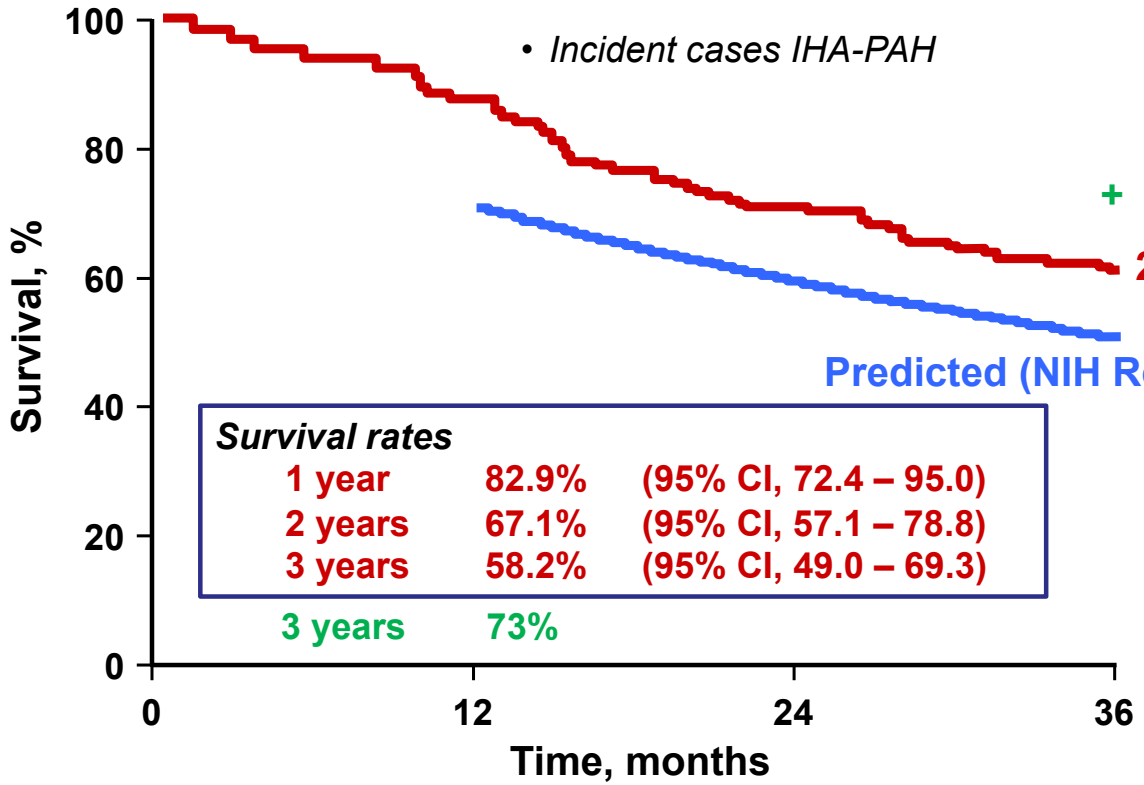






Gaine & McLaughlin, ERR 2017; Humbert et al. N Engl J Med 2004; Humbert et al. Circulation 2014

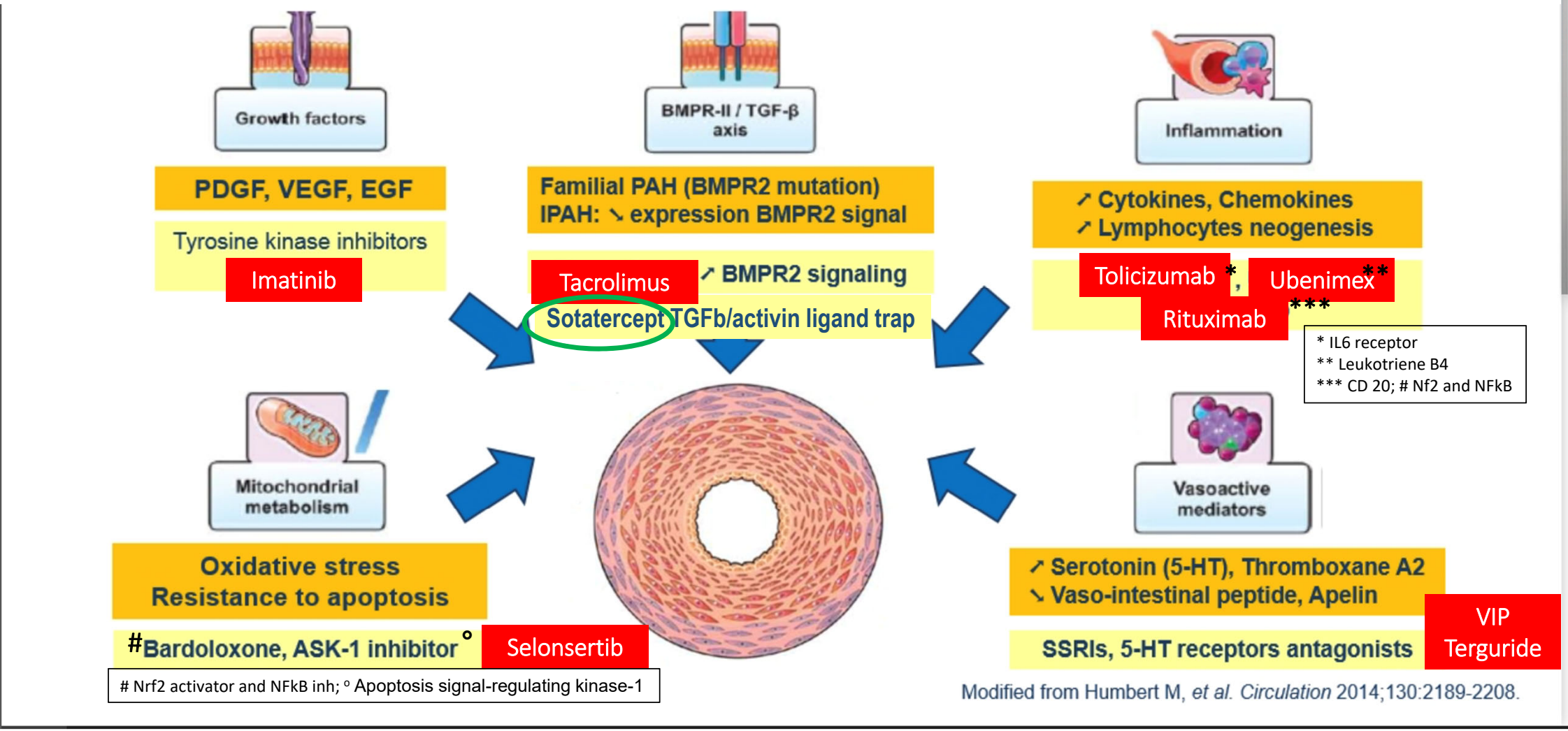
DESPITE DRUG DISCOVERY AND EDUCATION PAH REMAINS A DEVASTATING CONDITION



10% mortality per year

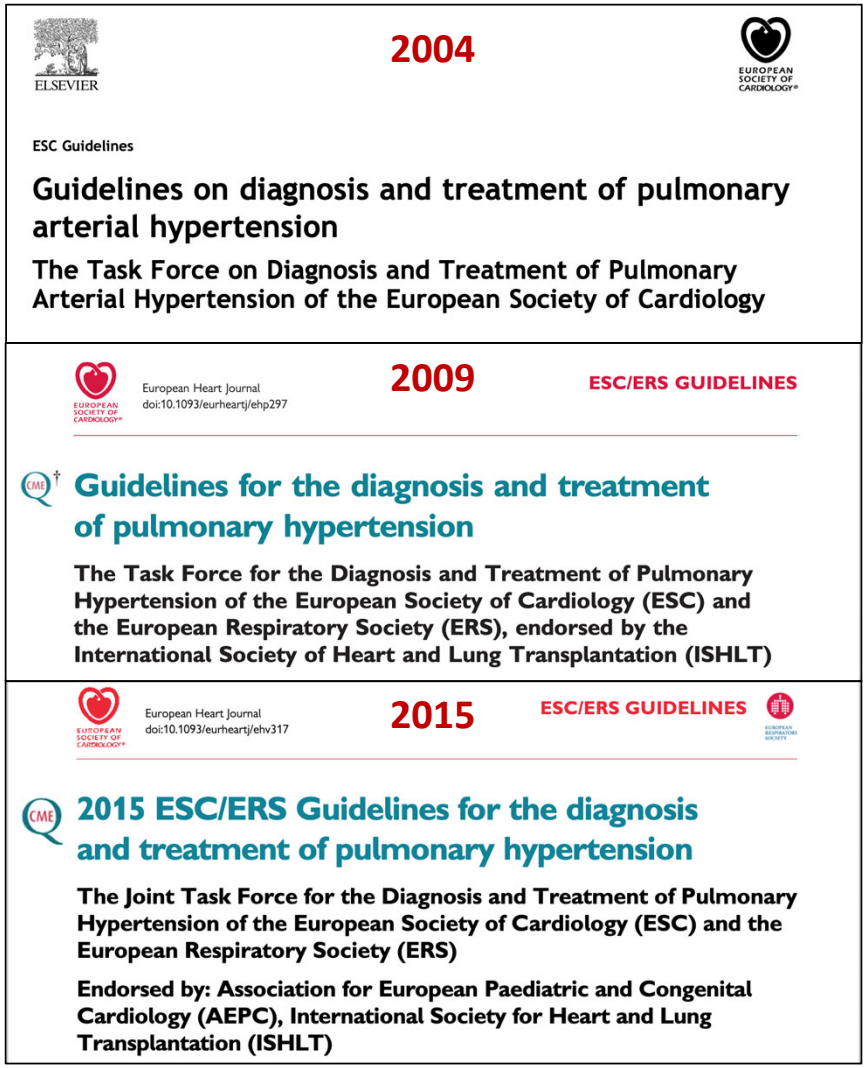
Humbert M, *et al. Circulation* 2010;122:156–63.
Boucly et al. *Eur Respir* J 2017; 50: p1700889.

OTHER APPROACHES (targeting proliferation)



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

PAH/PH/CTEPH GUIDELINES - STATEMENTS



ERS OFFICIAL DOCUMENT
ERS STATEMENT

An official European Respiratory Society statement: pulmonary haemodynamics during exercise

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

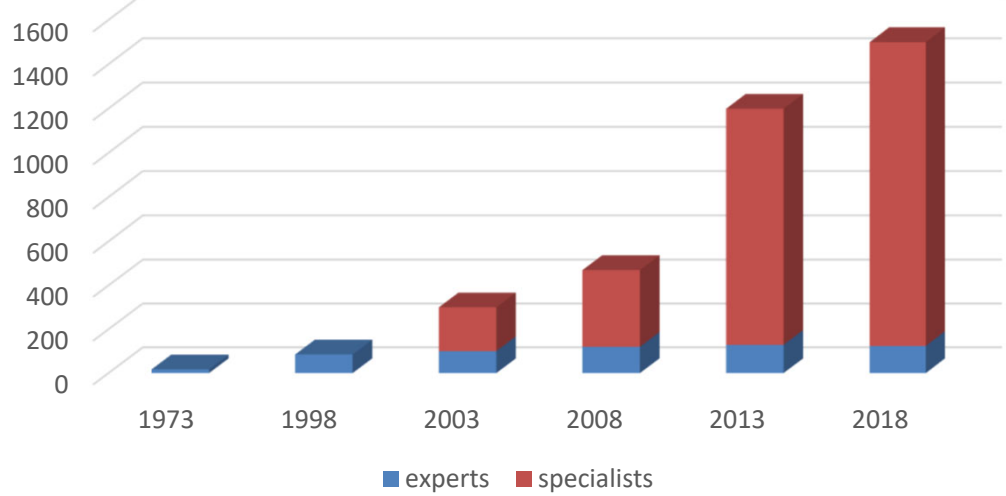
ERS statement on chronic thromboembolic pulmonary hypertension

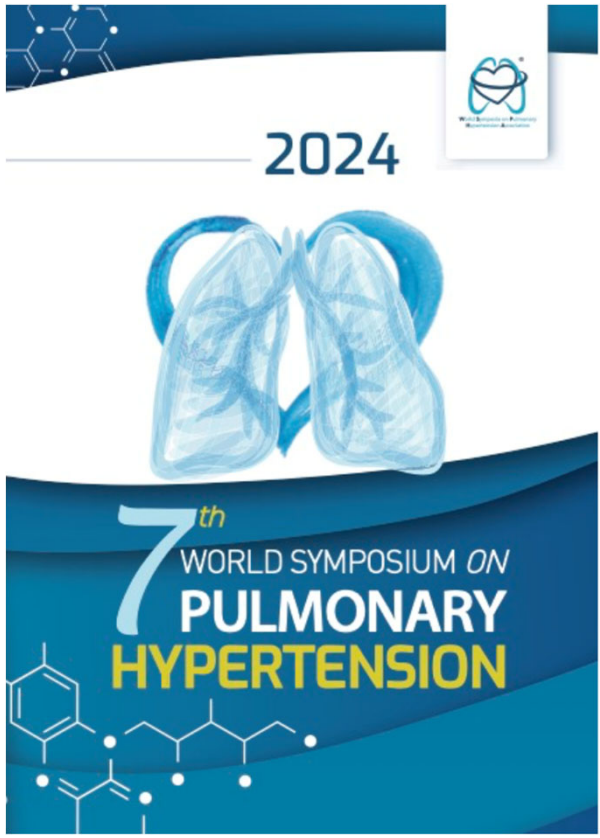
ANNOUNCED

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

World Symposia on Pulmonary Hypertension Association

WORLD SYMPOSIA ON PULMONARY HYPERTENSION





7TH WORLD SYMPOSIUM ON PULMONARY HYPERTENSION 2024

RARE DISEASE POLICIES IN EU

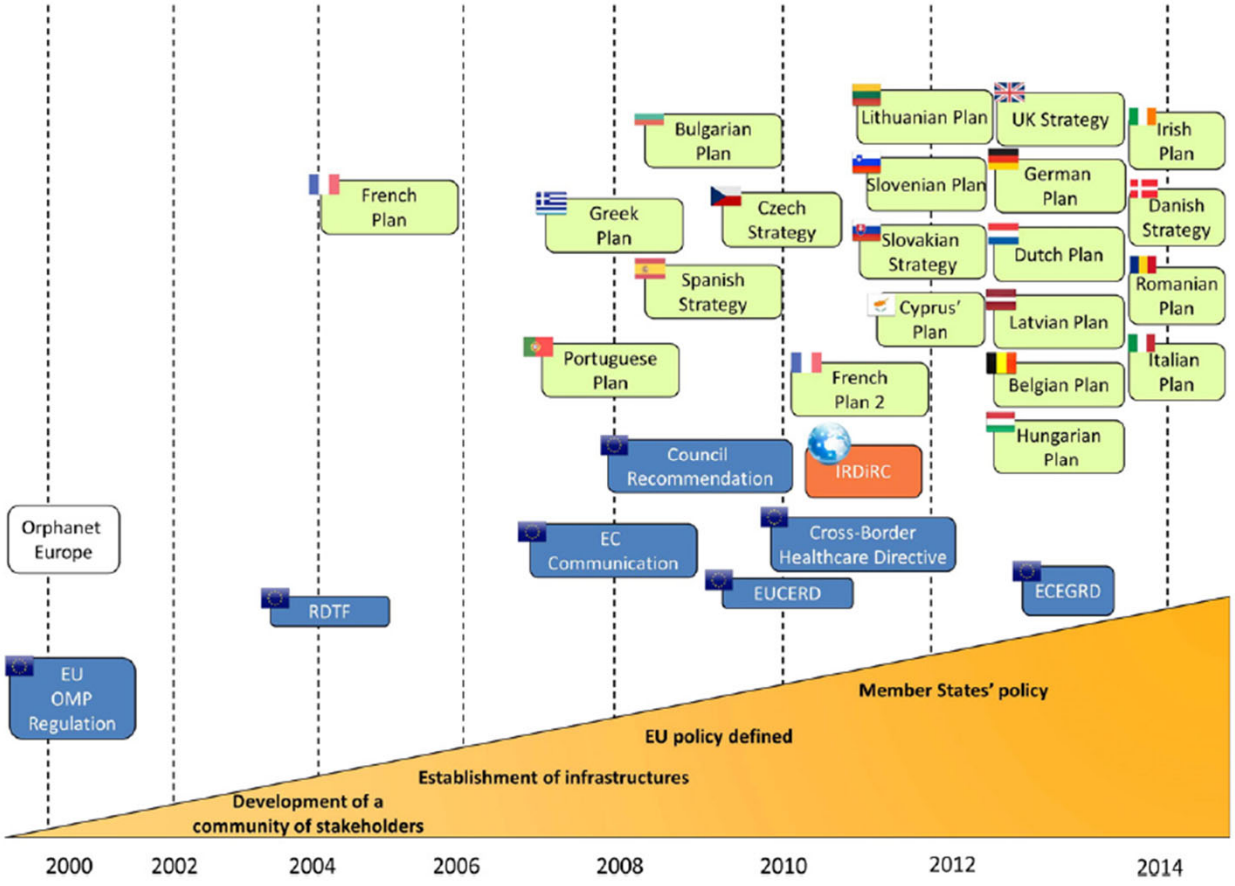


Fig. 1. Evolution of rare disease policy in Europe (December 2014) [4].

Rodwell & Aymé, Biochimica et Biophysica Acta 2015

European Reference Networks

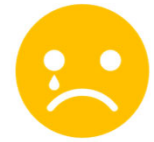
2016-2017

EURORDIS
RARE DISEASES EUROPE

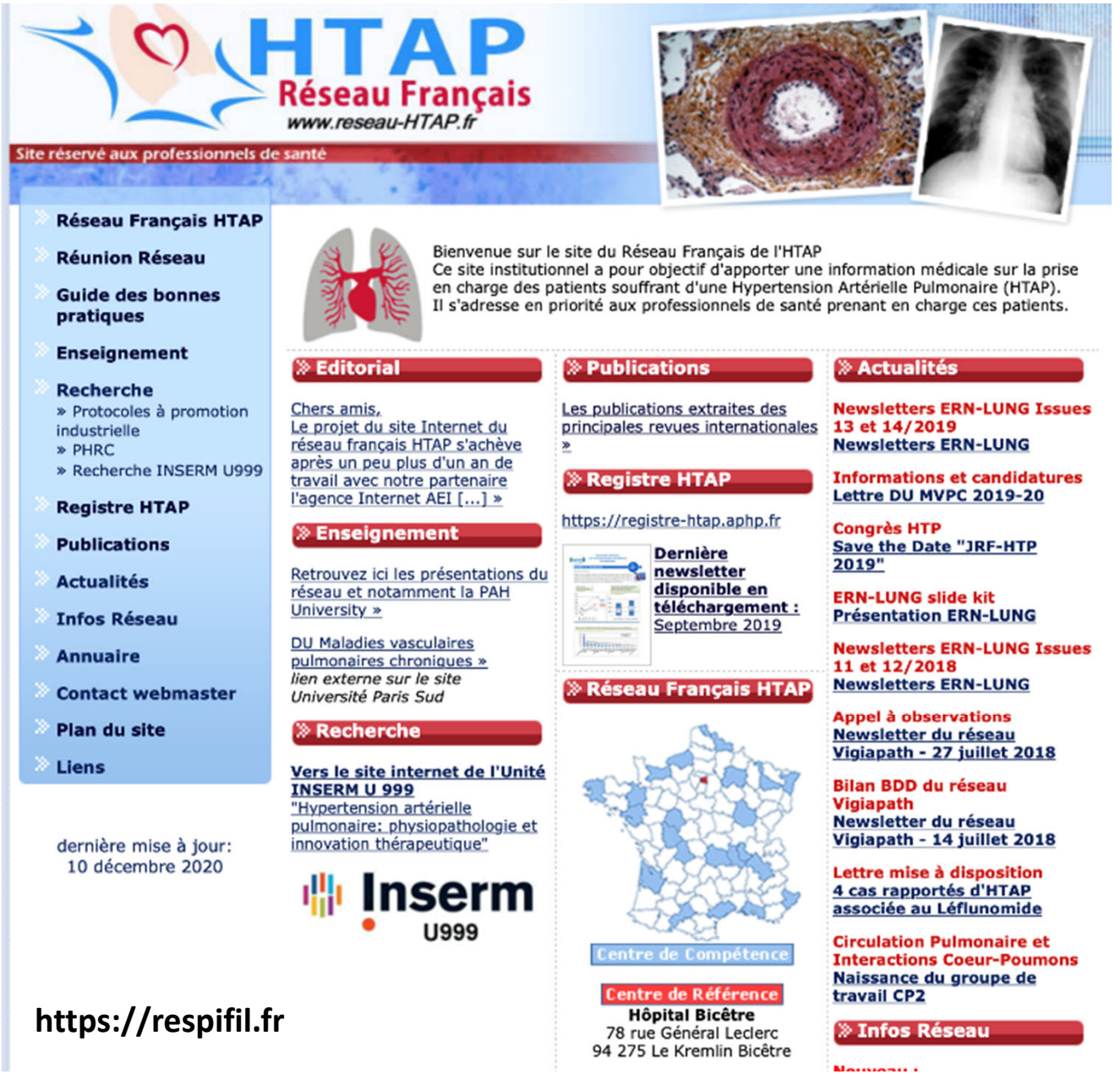
EURORDIS Recommendations on the Integration of European Reference Networks (ERNs) into National Health Systems

November 2018

EURORDIS.ORG



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



https://respifil.fr

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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Contents: Navigation

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

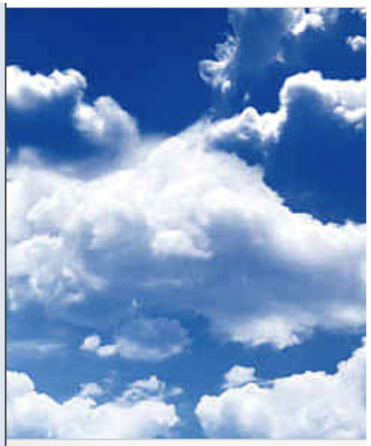
European Reference Network | Respiratory Diseases (ERN-LUNG)

ABOUT ERN-LUNG OUR EXPERTISE FOR CLINICIANS FOR PATIENTS SEEKING EXPERT OPINION? NEWS DONATE NOW

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

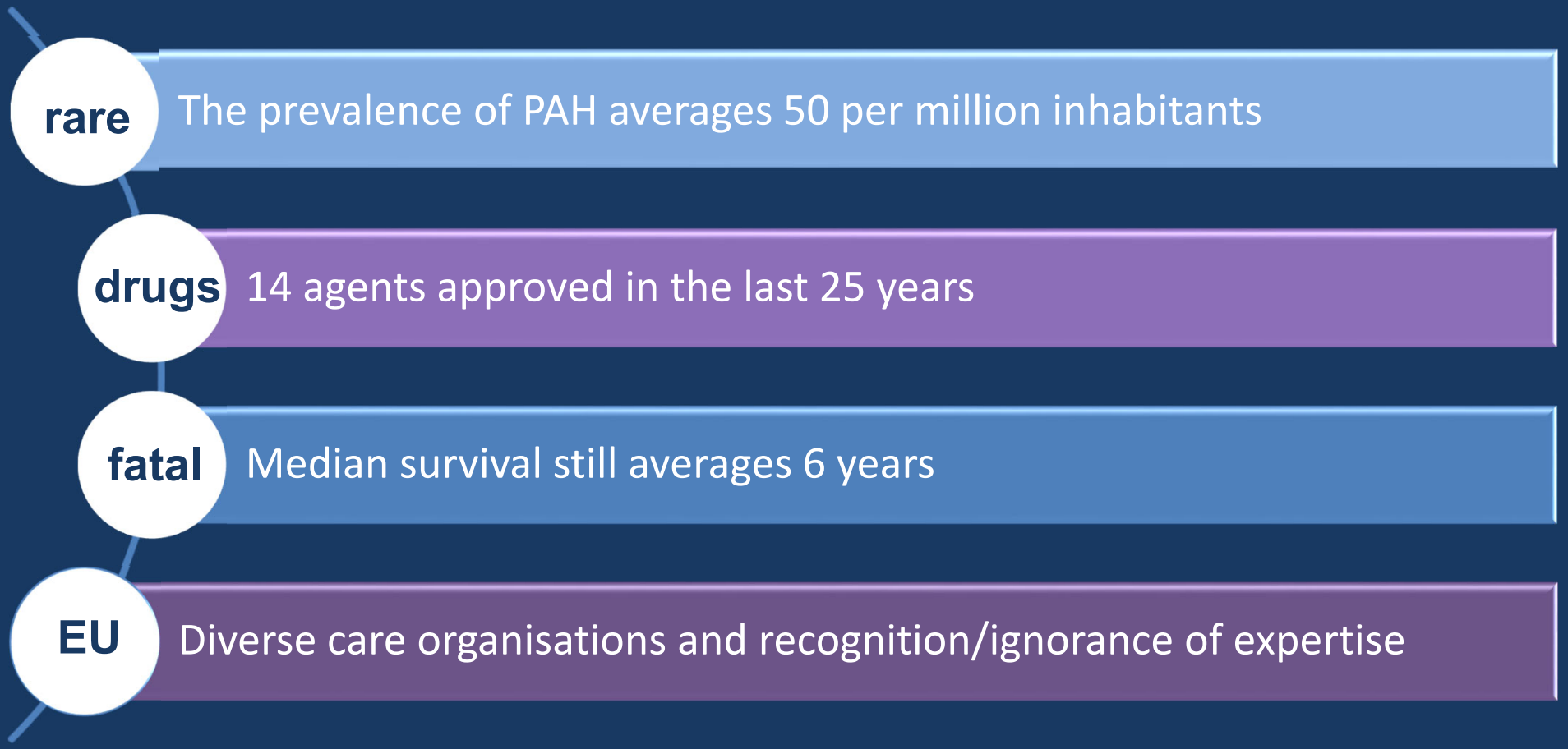
EDITORIAL
ERS CLINICAL RESEARCH COLLABORATIONS

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 Noordegraaf[5] and Marion Delcroix [6,7], on behalf of the PHAROS Clinical Research Collaboration

Sitbon et al, Eur Respir J 2020

Key messages



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 transparence, more coordinated actions between ERNs
  - Global solutions concerning registries, data sharing regulation, …
  - Reconsidering the funding structure (project-driven, …)