Characterization of the epidemiology, treatment patterns and burden of Pulmonary Hypertension Group 1 and 3 in France, Germany and the UK: a real-word evidence study (PHILD_RWE_FR_DE_UK)

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Study Finalised

Administrative details

EU PAS number

EUPAS105806

Study ID

105807

DARWIN EU® study

No

Study countries

- [ ] France
- [ ] Germany

United Kingdom

Study description

Pulmonary hypertension (PH) is highly complex. There are five broad PH clinical categories recognized by the WHO: pulmonary arterial hypertension (PAH, Group 1), PH due to left heart disease (Group 2), PH with lung diseases and/or hypoxia (PH-LD, Group 3), Chronic Thromboembolic Pulmonary Hypertension (CTEPH, Group 4) or other disorders associated with PH (Group 5). This retrospective longitudinal cohort study uses claims data from France (PMSI, SNDS), Germany (SHI Funds), and electronic medical records (EMR) from the UK (CPRD/HES) from Jan-2015 to Dec-2021. The goal is to characterize the epidemiology, the patient profile, the treatment patterns, the resource utilization and the associated costs of PH-LD and the sub-group due to interstitial lung disease (PH-ILD, Group 3.2), and the treatment patterns of PAH patients over the 3-year inclusion period (Jan-2017 and Dec-2019) in the three countries. To identify PH-LD and PAH patients previously published decision trees will be applied to the data sources based on input of clinicians actively treating these patients. In the absence of ICD-10 codes, PH-LD and the sub-group PH-ILD patients will be identified based on the combination of PH-recorded diagnosis with LD/ILD records in the prior 24 months and up to 60 days after the first diagnosis. PAH patients will be identified based on a combination of PH diagnosis, visit to a PH center (if available), Right Heart Catheterization (RHC), and PAH-specific drugs. An estimation of prevalence and incidence will be reported per cohort and country. Patient baseline characteristics and comorbidities will be described, resource use measured, and real-world outcomes, including mortality, presented. Lastly, the treatment patterns will be described for the three cohorts. This pan-European RWE study will provide valuable insights into the epidemiology, management, and real-world outcomes of PAH, PH-LD, and PH-ILD. Limitations associated with claims and EMR data sources apply.

Study status

Finalised

Research institutions and networks

Institutions

Alira Health AG Swiss, Alira Health SAS France, Alira Health SLU Spain

Contact details

Study institution contact

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Study timelines

Date when funding contract was signed

Planned: 01/07/2022

Actual: 19/07/2022

Study start date
Planned: 13/07/2022

Actual: 25/11/2022

Date of final study report
Planned: 07/07/2023

Actual: 07/07/2023

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ferrer

Study protocol

20230517_Alira Health_Ferrer RWE Study Umbrella protocol_final_v2.0.pdf(2.03 MB)

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Drug utilisation

Healthcare resource utilisation

Data collection methods:

Secondary use of data

Main study objective:

To estimate prevalence and incidence of PH with lung diseases and/or hypoxia (PH-LD) and PH with interstitial lung disease (PH-ILD) in Germany, France, and the UK. To characterize baseline characteristics, comorbidities, resource use, and real-world outcomes of PH-LD and PH-ILD patients. To describe treatment patterns of three cohorts: pulmonary arterial hypertension (PAH), PH-LD, and PH-ILD.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Retrospective longitudinal study

Study drug and medical condition

Medical condition to be studied

Pulmonary hypertension

Chronic respiratory disease

Interstitial lung disease

Pulmonary arterial hypertension

Population studied

Short description of the study population

The study focused on two main cohorts and a subgroup: group 1 patients with pulmonary arterial hypertension (PAH) cohort, group 3 patients with pulmonary arterial hypertension (PAH) and/or hypoxia cohort, and subgroup 3.2 of patients with pulmonary hypertension associated with interstitial lung disease.

Age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Special population of interest

Other

Special population of interest, other

Patients with hypertension and lung disease

Estimated number of subjects

15000

Study design details

Outcomes

1. Estimation of the prevalence and incidence of PH-LD and PH-ILD in Germany, France, and the UK. - 2. The characterization of the treatment patterns of PAH, PH-LD, and the sub-group PH-ILD patients, 1. Estimation of burden of disease of PH-LD and PH-ILD patients in terms of resource use and costs, evaluating hospital care (inpatient and outpatient), expenditure on tests, procedures, diagnostics, and medications. - 2. Characterization of the patient profile, real-world outcomes in terms of demographic and clinical characteristics, comorbidity burden, survival rate, yearly hospitalization rate

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Data analysis plan

The queries, management and statistical analysis of the data used will be developed by Alira Health using R and RStudio software or Python for France and UK databases, whereas for the German claims data, SAS and Excel will be used. Descriptive statistics will be presented for all evaluation criteria for each population, as follows: - Quantitative variables a. Quantitative variables will be described with standard statistics including, the mean, standard deviation (SD), median, quartiles, and minimum and maximum. The number of patients with missing data will be reported for each variable and will be described accordingly. Quantitative variables may be categorized into quantiles as required. - Categorical variables - Counts and percentages will be computed for categorical variables. - If relevant, all statistical tests will be two-sided and considered significant at the 5% level. A more detailed Statistical Analysis Plan of the study will be developed.

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data source(s)

Clinical Practice Research Datalink

Hospital Episode Statistics

Data source(s), other

SNDS France, PMSI France, SHI Germany

Data sources (types)

Administrative healthcare records (e.g., claims)

Electronic healthcare records (EHR)

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No