Disease characteristics and outcomes of pulmonary arterial hypertension in children and adolescents in real-world clinical settings: Sytematic Review of prospective, observational registries (Pediatric Systematic Review)

First published: 15/08/2013

Last updated: 14/09/2018

Study Finalised

Administrative details

EU PAS number

EUPAS4523

Study ID

25541

DARWIN EU® study

No

Study countries

- [ ] Australia
- [ ] Austria
- [ ] Brazil
- [ ] Canada
- [ ] China
- [ ] Denmark
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Hungary
- [ ] Italy
- [ ] Japan
- [ ] Mexico
- [ ] Netherlands
- [ ] Norway
- [ ] Poland
- [ ] Switzerland
- [ ] Türkiye
- [ ] United Kingdom
- [ ] United States

Study description

Systematic review of aggregated results from the prospective, observational disease registries : TOPP, French registry of PAH in children, Netherlands national registry, REVEAL (Registry to Evaluate Early And Long-Term PAH Disease Management, United States)

Study status

Finalised

Research institutions and networks

Institutions

Actelion Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Actelion Pharmaceuticals

Networks

TOPP Registry, FR Pediatric Registry, NL National Registry, REVEAL

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Pharmaceuticals Ltd Actelion

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 01/07/2009

Study start date
Actual: 01/07/2009

Date of final study report
Actual: 18/07/2018

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Actelion Pharmaceuticals Ltd

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study topic:

Disease /health condition

Other

Study topic, other:

Disease/Epidemiology study

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Data collection methods:

Secondary use of data

Main study objective:

For each registries, the objectives are to describe: -Patient demographics and disease characteristics -Outcomes of PAH in all pediatric patients (including patients on Tracleer). The main areas of interest are general development (growth and sexual maturation), clinical worsening, hospitalization and death-Safety experience in Tracleer-treated pediatric patients

Study Design

Non-interventional study design

Systematic review and meta-analysis

Study drug and medical condition

Medical condition to be studied

Pulmonary arterial hypertension

Population studied

Short description of the study population

Children and adolescents in real-world clinical settings with pulmonary arterial hypertension (PAH).

Age groups

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Estimated number of subjects

500

Study design details

Outcomes

-Outcomes of general development (weight, height, sexual maturation)-Outcomes of PAH such as clinical worsening -Outcomes of safety relevant experience in the sub-group of Tracleer treated patients.

Data analysis plan

Data analysis will be exploratory, and will be performed for each registry separately by the data owner using similar statistical methods based on a common SAP. Two groups – ‘All patients’ and ‘All Tracleer patients’ – will be described within each study.

Data management

Data source(s), other

Reveal United States, NL national registry Netherlands, FR pediatric registry France, TOPP United States

Data sources (types)

Other

Data sources (types), other

Aggregated tables from the contributing registries

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