Prospective Case-Control Safety Study of Bronchitol (inhaled mannitol) in Patients with Cystic Fibrosis from the UK CF Registry

First published: 22/08/2017

Last updated: 23/04/2024

Study Finalised

Administrative details

EU PAS number

EUPAS20668

Study ID

33911

DARWIN EU® study

No

Study countries

☐ United Kingdom

Study description

Post-authorisation safety study is being conducted pursuant to an obligation imposed by the EMA as a condition of the granting of a marketing authorisation for Bronchitol (mannitol) to confirm the safety profile of Bronchitol, identify, characterise and/or quantify any emergent safety issues, and measure the effectiveness of agreed risk management strategies.

Study status

Finalised

Research institutions and networks

Institutions

Cystic Fibrosis Trust

United Kingdom

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Institution | Not-for-profit

Contact details

Study institution contact

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Primary lead investigator

Siobhán Carr

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 29/06/2012

Actual: 29/06/2012

Study start date
Planned: 01/07/2012

Actual: 01/07/2012

Date of final study report
Planned: 30/04/2018

Actual: 05/11/2018

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pharmaxis Ltd

Study protocol

Registry Study Protocol V1.0 Final.pdf (296.67 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

To assess the rates of identified and potential risk of Bronchitol in CF through a comparison of Bronchitol treated vs. non-treated patients in a cohort matched for key characteristics from the CF registry over a 1 to 5 year period. Safety analysis would be conducted on a 6 monthly basis for three years and then annually for two further years..

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

BRONCHITOL

Medical condition to be studied

Cystic fibrosis

Population studied

Short description of the study population

Adult (≥ 18 years) patients in the CF registry database who were prescribed Bronchitol. The unexposed group were untreated patients.

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 Cystic Fibrosis

Estimated number of subjects

446

Study design details

Outcomes

To assess the rates of identified and potential risks of Bronchitol through a comparison of treated vs. non-treated patients, - To compare the rates of identified and potential risks against background rates in the general CF population.- To assess the rate of identified and potential risks in the population below 18 years of age- To compare the effect on lung function in treated vs. non-treated patients- To compare the effect on CF exacerbations in treated vs. non-treated patients

Data analysis plan

At each analysis time a propensity score analysis will be performed. Here we will use logistic regression to estimate the probability of being treated with Bronchitol. This will generate a propensity score for each patient which will be used in subsequent analyses. Full Analysis PopulationAll patients (treated and

untreated) from CF Registry will be included in the analyses. For this population, we will include:- Simple descriptive analyses (frequency and percentage) for AEs- Negative Binomial regression for the count of events for each AE with adjustment for propensity scores.- Cox regression for time to first event for each AE (Hazard Ratio), with adjustment for propensity scores.Cox regression will be used to study the time to first event for each AE. As for the negative binomial regression models these will be done both with and without adjustment for potential confounders.Random effects linear models will be used to study changes in FEV1 over time

Documents

Study results

5 yr PASS - NACF poster 2018 Final.pdf (526.73 KB)

Abstract 484 NACF 2018.pdf (200.14 KB)

Study publications

Carr S et al., Pediatric pulmonology Vol 53, Issue S2 Abstract 484

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 sources (types)

Disease registry

Other

Data sources (types), other

Prospective patient-based data collection

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