A 5-year multi-center, observational post-authorization safety study to document the drug utilisation of Wakix® in the treatment of narcolepsy with or without cataplexy and to collect information on its long termthe safety of Wakix® when used in routine medical practice (WAKIX PASS)

First published: 16/06/2016

Last updated: 16/04/2021

Study Ongoing

Administrative details

EU PAS number

EUPAS13818

Study ID

40614

DARWIN EU® study

No

Study countries

- [ ] Belgium
- [ ] France
- [ ] Germany
- [ ] Italy
- [ ] United Kingdom

Study description

International, multi-center, non-interventional, prospective, open-label long-term post-authorization safety study (PASS) on the use of Wakix (pitolisant) a new H3R antagonist approved for the treatment of narcolepsy with or without cataplexy in adults. The patients will be observed during routine clinical practice. Study is planned to follow up patients for five years or to the end of the study. The product (Wakix)will be prescribed and used based on the routine clinical practice as well as on the individual situation of each patient and according to the approved SPC. The main objectives are to collect information on the long term safety of pitolisant Wakix® (all reported adverse events) when used in a real-life setting, and to monitor and document the drug utilization patterns of Wakix® in routine medical practice. The other objectives are to assess the clinical benefit of a treatment with pitolisant (Wakix®) in a real-life setting on excessive diurne somnolence and other symptoms of narcolespy, and the health care resource use (hospitalizations, emergency visits, unscheduled visits) due to narcolepsy, to measure the treatment compliance, the quality of life and disease burden. Patients will be recruited in the 5 countries where Wakix will be first launched (i.e. France, UK, Italy, Germany, Belgium), by around 8 to 10 sites in each.

Study status

Ongoing

Research institutions and networks

Institutions

Kappa Santé

France

First published: 20/09/2010

Last updated: 06/03/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Stéphane SCHÜCK

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 16/06/2016

Actual: 21/07/2016

Study start date
Planned: 03/10/2016

Actual: 15/12/2016

Data analysis start date
Planned: 30/11/2024

Date of final study report
Planned: 30/03/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

bioprojet

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 1 (imposed as condition of marketing authorisation)

Regulatory procedure number

EMA/H/C/002616

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Main study objective:

To collect information on the long term safety of pitolisant Wakix® (all reported adverse events) when used in a real-life setting.

Study drug and medical condition

Medicinal product name

WAKIX

Medical condition to be studied

Narcolepsy

Population studied

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)

Estimated number of subjects

Study design details

Outcomes

Adverse events reported by the investigators will be coded using the MedDRA (preferred term) and assessed in terms of seriousness, severity, causality, recovery. Safety results will be described in accordance with GVP Module VI. ESS, CGI, BDI, BMI, NSAQ, 8-item Morisky, FOSQ-10, EQ5D, hospitalization, emergency visit, work day loss and drug utilization pattern : patient characteristics, medical history, co-medications, Wakix® dosage changes, Wakix® interruption and reason, co-medication changes, drop out.

Data analysis plan

Quantitative variables will be described using the following descriptive statistics: sample size, number of missing values, mean, standard deviation, median, minimum and maximum. Qualitative variables will be described using the following descriptive statistics: sample size, number of missing values, and percentage of each modality calculated on the responses expressed. The bivariate statistical analyses performed will undergo statistical tests, based on the nature of the variables analyzed: For qualitative variables, the Chi2 test will be applied, apart from the theoretical sample sizes are less than 5, in this case, Yates continuity correction or Fisher's exact test will be used. For quantitative variables, when distribution is close to normal (non-significant Shapiro-Wilk test), a Student's t-test or analysis of variance will be performed. If this is not the case, non-parametric tests will be used (Wilcoxon, Kruskal-Wallis).

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)

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