

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 term the 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

### **PURI**

<https://redirect.ema.europa.eu/resource/40614>

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### **EU PAS number**

EUPAS13818

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### **Study ID**

40614

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## **DARWIN EU® study**

No

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### **Study countries**

Belgium

France

Germany

Italy

United Kingdom

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### **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 diurnal somnolence and other symptoms of narcolepsy, 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.

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## Study status

Ongoing

## Research institution 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

Isabelle Lecomte

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

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**Study start date**

Planned: 03/10/2016

Actual: 15/12/2016

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**Data analysis start date**

Planned: 30/11/2024

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**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

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

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

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**Regulatory procedure number**

EMA/H/C/002616

## Methodological aspects

### Study type

### Study type list

**Study type:**

Non-interventional study

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**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

**Name of medicine**

WAKIX

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**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)

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## **Estimated number of subjects**

300

# 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.

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## **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

### Data sources

#### Data sources (types)

Other

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#### 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

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#### Check completeness

Unknown

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#### Check stability

Unknown

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## **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No