An Observational Study to Describe the Long-term Safety and Effectiveness of Namuscla in the Symptomatic Management of Myotonia in Adult Patients with Non-dystrophic Myotonic Disorders

First published: 10/11/2020

Last updated: 14/03/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS37943

Study ID

46833

DARWIN EU® study

No

Study countries

- ☐ France
- ☐ Germany

United Kingdom

Study description

Namuscla™ is approved in European Union (EU) for the symptomatic treatment of myotonia in adult patients with non-dystrophic myotonic disorders. Active ingredient of Namuscla is 167 mg mexiletine, a class Ib antiarrhythmic. To date, randomised studies conducted for mexiletine have assessed only short-term efficacy and safety with little supporting data for long-term use from observational research. This non-interventional study will collect data on the long-term (12 months to 3 years) safety of Namuscla in a real-world setting for the symptomatic treatment of myotonia in adult patients with non-dystrophic myotonic disorders over a period of up to 3 years. This study is being conducted as part of the agreed European Risk Management Plan (RMP).

Study status

Ongoing

Research institutions and networks

Institutions

United BioSource Corporation (UBC)

Switzerland

First published: 25/04/2013

Last updated: 06/03/2024

Institution | Non-Pharmaceutical company | ENCePP partner

University Hospital of Ulm

Germany

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Educational Institution | Hospital/Clinic/Other health care facility

Hôpital Universitaire de La Pitié Salpêtrière 47-83 bd de l’hôpital Batiment Babinski Paris, Cedex 13 75013, CHRU Lille 2 Avenue Oscar Lambret Lille, 59000, St. Josef-Hospital Klinikum der Ruhr Universitaet Bochum Gudrunstraße 56 44791 Bochum Bochum, North-Rhine Westphalia 44791, Universitätsklinikum Ulm, Klinik für Neurologie Oberer Eselsberg 45 Ulm, 89081, Nottingham University Hospitals NHS Trust Queen’s Medical Centre, South Block, Derby Road, Nottingham, England NG7 2UH, Institute of Neurology, Queen Square London, England WC1N 3BG

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Beatriz Borredá

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 28/10/2019

Actual: 28/10/2019

Study start date

Planned: 05/11/2020

Actual: 17/12/2020

Data analysis start date

Planned: 20/11/2025

Date of interim report, if expected

Planned: 05/11/2021

Date of final study report

Planned: 24/02/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Lupin EU GmbH

Study protocol

Lupin_PASS_Protocol_V1.6_25Nov2019.pdf(441.82 KB)

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)

Other study registration identification numbers and links

NCT04616807

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

Primary Objective: To describe the long-term safety and tolerability of Namuscla for the symptomatic treatment of myotonia in adult patients with non-dystrophic myotonic disorders.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Non-interventional Prospective Post Authorisation Safety Study (PASS)

Study drug and medical condition

Study drug International non-proprietary name (INN) or common name

MEXILETINE HYDROCHLORIDE

Anatomical Therapeutic Chemical (ATC) code

(C01BB02) mexiletine

mexiletine

Medical condition to be studied

Myotonia

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)

Special population of interest

Hepatic impaired

Immunocompromised

Renal impaired

Estimated number of subjects

50

Study design details

Outcomes

Outcome Variables: Primary: 1. Proportion of patients with treatment-emergent AEs (TEAEs, including SAEs) from study enrolment to 6, 12, 24 and 36 months on Namuscla, 2. Proportion of patients requiring dose reduction or treatment discontinuation due to AEs (including SAEs). Safety:1Proportion of pts with

AEs/SAEs/AESI from study enrolment to 6,12,24,and 36 months.2. Proportion of AEs in pts with abnormal hepatic function 3.Number of AEs in pts with abnormal renal function 4.Number of AEs in geriatric pts 5.Number of pts with cardiac arrhythmia,SCAR,DRESS,SJS,seizures.Efficacy:6.Change VAS,stiffness,fatigue7.Clinical myotonia evaluation8.Change INQoL&MBS.

Data analysis plan

All statistical analyses will be performed using SAS® Version 9.4 or higher.Data will be presented by myotonic disorder type, by age group (18 to 64 years, inclusive, and ≥65 years), other subgroups of interest, and by Namuscla use (newly exposed and prior mexiletine exposure, average dose). Standard descriptive summaries: Descriptive statistics for continuous data: The continuous data will be summarized using the number of observations (n), arithmetic mean, standard deviation, median, minimum value, maximum value and 95% CI. The (n) will be presented with no decimal place, mean and median will be presented up to one decimal place from the original value, SD up to two decimal places from the original value and (min, max) as an original value. Descriptive statistics for categorical data: The categorical variables will be summarized using the frequency count & % for each possible value. The frequencies will be presented up to 0 decimal places and % up to 1 decimal place.

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