

# 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:** 06/07/2026

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS37943

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### Study ID

46833

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### DARWIN EU® study

No

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### Study countries

 France

 Germany

## 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).

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## 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

Funke Katja EUQPPV@lupin.com

Study contact

[EUQPPV@lupin.com](mailto:EUQPPV@lupin.com)

### Primary lead investigator

Beatriz Borredá

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 28/10/2019

Actual: 28/10/2019

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### Study start date

Planned: 05/11/2020

Actual: 17/12/2020

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### Data analysis start date

Planned: 20/11/2025

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### Date of interim report, if expected

Planned: 05/11/2021

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### Date of final study report

Planned: 31/07/2026

## 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

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### **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 topic:**

Human medicinal product

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**Study type:**

Non-interventional study

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**Scope of the study:**

Effectiveness study (incl. comparative)

Evaluation of patient-reported outcomes

Safety study (incl. comparative)

**Data collection methods:**

Primary data collection

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**Study design:**

This is a non-interventional, prospective, observational, multicentre study

**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

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**Non-interventional study design, other**

Non-interventional Prospective Post Authorisation Safety Study (PASS)

## Study drug and medical condition

**Medicinal product name**

NAMUSCLA

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

MEXILETINE HYDROCHLORIDE

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**Anatomical Therapeutic Chemical (ATC) code**

(C01BB02) mexiletine

mexiletine

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**Medical condition to be studied**

Myotonia

## Population studied

**Short description of the study population**

The study population will comprise patients who are diagnosed with non-dystrophic myotonic disorders and considered suitable candidates for the treatment by Namuscla by the investigators according to the approved SmPC and who meet the eligibility criteria. This includes:

1. Patients newly initiated on Namuscla for the treatment of NDM (newly exposed)
2. Patients already on Namuscla/ mexiletine at enrolment - For patients receiving mexiletine other than Namuscla, only those who switch to Namuscla will be included in the study.

Patients already being treated with Namuscla/ mexiletine at the time of enrolment will be considered for enrolment provided they meet the eligibility criteria.

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## **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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## **Special population of interest**

Hepatic impaired

Immunocompromised

Renal impaired

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## **Estimated number of subjects**

50

# Study design details

## **Setting**

The study will be conducted at specialized centres for the treatment of myotonic disorders that see at least 20-30 patients annually (“reference centres”).

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## **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: 1. Proportion 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, fatigue 7. Clinical myotonia evaluation 8. Change INQoL&MBS.

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### **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  $\geq 65$  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

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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