MEASuRE: Metreleptin Effectiveness and Safety Registry

First published: 07/07/2026

Last updated: 08/07/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000001052

Study ID

1000001052

DARWIN EU® study

No

Study countries

France

Germany

Italy

United Kingdom

United States

Study description

A non-interventional, multicenter, prospective observational registry designed to evaluate the long-term safety and effectiveness of metreleptin under routine clinical practice conditions in the US and EEA.

The target study population will include patients who have taken at least one dose of commercial metreleptin. Inclusion criteria are broad so as to enable inclusion of a representative population of patients taking metreleptin as per usual clinical practice.

Study status

Ongoing

Research institutions and networks

Institutions

Chiesi Farmaceutici

First published: 01/02/2024

Last updated: 01/02/2024

Institution

IQVIA

United Kingdom

First published: 12/11/2021

Last updated: 22/04/2024

Institution | Non-Pharmaceutical company | ENCePP partner

N/A

Contact details

Study institution contact

Lori Hartnett lori.hartnett@chiesi.com

Study contact

lori.hartnett@chiesi.com

Primary lead investigator

Shir Fuchs Orenbach

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 08/03/2016

Actual: 08/03/2016

Study start date

Planned: 11/10/2016

Actual: 11/10/2016

Data analysis start date
Planned: 01/07/2017

Actual: 01/07/2017

Date of interim report, if expected
Planned: 30/09/2026

Date of final study report
Planned: 31/03/2032

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Chiesi Farmaceutici S.p.A

Study protocol

MEASuRE_ Protocol Amendment v11.0_Signed.pdf (352.52 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)

Regulatory procedure number

EEA: EMEA/H/C/4218/SOB001 IND 050259 US: PMR 2456-1

Other study registration identification numbers and links

MEASuRE AEGR-734-400

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Study design:

Prospective, non-interventional, observational registry of patients treated with metreleptin in routine clinical practice across US and EEA.

Main study objective:

Evaluate long-term safety and effectiveness of metreleptin under real-world conditions

Primary:

To determine the incidence and severity of the following safety events in patients treated with metreleptin as part of standard clinical practice:

• Acute pancreatitis associated with the discontinuation of metreleptin, and all cases of fatal or necrotizing pancreatitis

• Hepatic adverse events

• Hypoglycaemia stratified by severity and concomitant antidiabetics dose modifications

• Hypersensitivity reactions

• Serious and severe infections, including serious infections resulting in hospitalization and death

Loss of efficacy, potentially due to anti-drug antibodies with blocking activity

New diagnoses of autoimmune disorders

Exacerbation of existing autoimmune disorders

All cancers (excluding non-melanoma skin cancer) by cancer type

Exposed pregnancies and pregnancy outcomes stratified by planned or unplanned

All-cause deaths (including causes of death)

Medication errors

Secondary

• To describe the overall demographic and clinical characteristics, and metreleptin exposure in all patients treated with metreleptin (pattern of use analysis)

• To describe routine laboratory measurements that could be inferred as effectiveness endpoints (including HbA1c, FPG and TG) over time

Exploratory Objectives

Use in pregnancy and lactation

Use in elderly

Effect of metreleptin on brain development

Effect of metreleptin on bone metabolism

Effect of metreleptin on sexual maturation (Tanner staging)

Neuroendocrine parameters and levels of the following hormones:

Testosterone, Oestradiol, Luteinizing hormone , Follicle stimulating hormone and Free triiodothyronine and thyroxine. Contingent on study sample size, the study will also estimate the incidence rate of the primary outcomes of interest by patient characteristics.

In patients with results from immunogenicity testing, the incidence of ADAs with blocking activity will be estimated.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

MYALEPTA

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

METRELEPTIN

Anatomical Therapeutic Chemical (ATC) code

(A16AA07) metreleptin

metreleptin

Medical condition to be studied

Partial lipodystrophy

Congenital generalised lipodystrophy

Acquired generalised lipodystrophy

Population studied

Short description of the study population

All patients treated with metreleptin through commercial supply and enrolled in the registry will be categorized into one of two patient cohorts: metreleptin new user cohort or metreleptin prevalent user cohort.

• Metreleptin new user cohort includes patients who are initiating treatment with metreleptin through commercial supply at the time of providing written consent for registry enrolment.

• Metreleptin prevalent (prevalent current and prevalent prior) user cohort includes patients who are treated with metreleptin through commercial supply but before registry enrolment and/or patients treated with metreleptin through commercial supply but coming off metreleptin treatment from a non-commercial source (e.g., clinical studies).

Estimated number of subjects

100

Study design details

Setting

Routine clinical practice across US and EEA, using standard-of-care visits and medical record abstraction

---

Data analysis plan

The primary objective of this study is to estimate the incidence of primary outcomes of interest in the patients treated with metreleptin. A confidence interval approach is chosen for the rate of the events of interest.

The primary population for analysis will consist of all patients who have received at least one dose of commercial metreleptin, regardless of the indication for which the treatment is received.

Data will be analysed by subgroups; metreleptin new user cohort, metreleptin prevalent user cohort; by indication, GL and PL and if required by region, US and EEA. In addition, analyses of Paediatric cohorts and further sub-group analyses may be performed.

Summary results

Not applicable (ongoing registry)

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 source(s), other

At specified data collection points (bi-annual) data will be abstracted from patient medical records and charts and will be entered by the treating physicians or their site staff at centres into a computerized data system using structured electronic Case Report Forms (eCRF).

Data sources (types)

Electronic healthcare records (EHR)

Laboratory tests and analyses

Non-interventional study

Other

Data sources (types), other

Product registry

Use of a Common Data Model (CDM)

CDM mapping

Yes

CDM Mappings

CDM name (other)

CDM mapping is performed with data from a disease registry in the EEA, the European Registry for Lipodystrophy (ECLip)

Data quality specifications

Check conformance

Yes

Check completeness

Yes

Check stability

Yes

Check logical consistency

Yes

Data characterisation

Data characterisation conducted

Yes

Data characterisation details

Edit checks and data cleaning plan

Multi-step quality checks, including programmatic validation rules in the study eCRF and extensive cross-registry data reconciliation with the e disease registry, the European Registry for Lipodystrophy (ECLip), are continuously executed complete to ensure data integrity for regulatory updates.