A prospective, multi-national, multicentre, non-interventional study to evaluate the long term safety of Esmya, in particular the endometrial safety, and the current prescription and management patterns of Esmya in a long term treatment setting

First published: 20/12/2016

Last updated: 09/01/2025

Study Finalised

Administrative details

EU PAS number

EUPAS16792

Study ID

28785

DARWIN EU® study

No

Study countries

- [ ] Belgium
- [ ] Czechia
- [ ] Denmark
- [ ] France
- [ ] Germany
- [ ] Hungary
- [ ] Italy
- [ ] Latvia
- [ ] Lithuania
- [ ] Netherlands
- [ ] Poland
- [ ] Portugal
- [ ] Romania
- [ ] Spain
- [ ] Sweden
- [ ] United Kingdom

Study description

This is a multi-centre, multinational, prospective, non-interventional study in females with a diagnosis of moderate to severe uterine fibroids, and for whom a treatment with Esmya in a long term manner is planned, and in subjects who were previously exposed to UPA in the long term Phase III studies. It is planned to enroll approximately 1,500 patients. Consecutive, eligible, patients will be invited to enroll from approximately 100-150 European Union (EU) clinical practice sites in approximately 15 countries. Patients will be followed for an observation period of 60 months (5 years) from treatment start. Investigators are to manage and treat patients according to their standard medical practice.

Study status

Finalised

Research institutions and networks

Institutions

PregLem

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Institution

Gedeon Richter

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Institution

Multiple centres: 153 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Daniela Ciobanu

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 22/12/2015

Actual: 18/12/2015

Study start date

Planned: 31/12/2015

Actual: 22/12/2015

Date of final study report

Planned: 16/09/2024

Actual: 04/10/2024

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Gedeon Richter Plc

Study protocol

PREMIUM_Protocol Version 1.3_including Amendment 04_21Aug2018_PGL_FINAL.pdf (4.97 MB)

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

NCT02748460

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Main study objective:

The specific objectives of the study are to:
1. Assess the long term safety, including endometrial safety, of Esmya in standard medical practice
2. Assess prescription patterns of Esmya in standard medical practice.

Exploratory objective: Assess patients’ quality of life in a long-term treatment setting.

Study drug and medical condition

Medicinal product name

ESMYA

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

ULIPRISTAL ACETATE

Anatomical Therapeutic Chemical (ATC) code

(G03XB02) ulipristal

ulipristal

Medical condition to be studied

Uterine leiomyoma

Population studied

Age groups

- Adults (18 to < 46 years)

- Adults (46 to < 65 years)

Estimated number of subjects

1500

Study design details

Data analysis plan

The majority of statistical analyses will be descriptive, reporting patient counts, means, standard deviations, medians, minima, and maxima for continuous variables (e.g. age and duration of symptomatic uterine fibroids) and frequencies and percentages for categorical variables (e.g. disease symptoms, prescription pattern, diagnostic test results). Two-sided 95% confidence intervals will be estimated as appropriate and reported along with p-values to facilitate the interpretation of the significance of the findings. These findings will be compared to published epidemiological data on pre-menopausal women with abnormal uterine bleeding (AUB), where available.Baseline data (i.e. data collected prior to the first administration of Esmya), including relevant demographic characteristics will be summarised for all treated subjects.Full details of all analysis to be carried out will be presented in the Statistical Analysis Plan (SAP).

Summary results

This was a prospective, multicentre, non-interventional study in female patients who had been diagnosed with moderate to severe symptoms of uterine fibroids and were initiating long-term treatment with Esmya (PGL14-001, PREMIUM) or patients who were previously exposed to ulipristal acetate 5 or 10 mg in the long-term Phase III studies.

Eligible, consenting patients were enrolled from approximately 100 to 150

clinical practice sites in the EU and United Kingdom and were followed for 60 months from treatment start according to the standard medical practice of their physician.

This study provided real-world data related to the longterm safety, including endometrial safety, and current prescription patterns of Esmya as treatment for symptomatic uterine fibroids from 1359 patients who had received at least one dose of Esmya/ulipristal acetate. This study also aimed to address the following safety concerns included in the current ulipristal acetate Risk Management Plan and identified:

• 7 (0.5%) patients with inappropriate management of endometrium thickening.

• 2 (0.15%) patients with suspected inappropriate diagnosis of endometrial hyperplasia.

• 11 (0.8%) patients with suspected acute uterine bleeding requiring immediate intervention. No trend could be identified for these cases.

• 104 (7.7%) patients with continuous treatment courses beyond 3 months. No safety concern was identified among these patients compared to the rest of the study population.

• No long-term effects of prolonged treatment on the endometrium (continuous course beyond 3 months or more than 4 intermittent treatment courses).

• One case of atypical hyperplasia and one case of endometrial adenocarcinoma, however, there were no cases identified with delayed diagnosis of atypical endometrial hyperplasia or adenocarcinoma.

• No identified reports of Esmya treatment having either a beneficial or adverse impact on (uterine fibroid) surgery.

No new safety concerns have been identified in this study

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

Unknown