Alpelisib (Piqray®) Post-Authorization Safety Study (PASS): a non-interventional study of alpelisib in combination with fulvestrant in postmenopausal women, and men, with hormone receptor positive (HR+), human epidermal growth factor receptor 2 negative (HER2-), locally advanced or metastatic breast cancer with a phosphatidylinositol-3-kinase catalytic subunit alpha (PIK3CA) mutation, in the real-world setting

First published: 11/06/2021

Last updated: 13/10/2025

Study Discontinued

Administrative details

EU PAS number

EUPAS41010

Study ID

48553

DARWIN EU® study

No

Study countries

- Austria
- Croatia
- Czechia
- Denmark
- Finland
- Greece
- Hungary
- Italy
- Netherlands
- Norway
- Poland
- Slovenia
- Spain
- Sweden

Study description

This Post-Authorization Safety study is a non-interventional study to further evaluate the safety of alpelisib in combination with fulvestrant in postmenopausal woman and men with HR+,HER2-, locally advanced or metastatic breast cancer with PIK3CA mutation, after disease progression following endocrine therapy as monotherapy, in the real-world setting.

Study status

Discontinued

Research institutions and networks

Institutions

Novartis Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

Novartis Clinical Disclosure Office

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Study contact

trialandresults.registries@novartis.com

Primary lead investigator

Novartis Clinical Disclosure Office

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 22/12/2020

Actual: 22/12/2020

Study start date
Planned: 03/10/2022

Actual: 21/06/2023

Data analysis start date
Actual: 18/10/2024

Date of final study report
Planned: 09/05/2025

Actual: 09/04/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novartis Pharma AG

Study protocol

BYL719C2404-v00--protocol Mar2021_Redacted.pdf (482.45 KB)

BYL719C2404-v02--protocol amendment_Redacted.pdf (576.04 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

CBYL719C2404

NCT04967248

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

The main objectives of this study is to evaluate the safety of alpelisib in combination with fulvestrant in the described population in the real-world setting.

As per the risk management plan approved by the European Medicines Agency,

this study will primarily focus on the risk of hyperglycemia and the risk factors for hyperglycemia.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

PIQRAY

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

ALPELISIB

Anatomical Therapeutic Chemical (ATC) code

(L01EM03) alpelisib

alpelisib

Medical condition to be studied

Breast cancer

Additional medical condition(s)

Hormone receptor positive HER2 negative breast cancer

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)

Estimated number of subjects

150

Study design details

Outcomes

- To Assess the incidence of hyperglycemia (adverse event of special interest, AESI) observed during follow-up of patients treated with alpelisib in combination with fulvestrant,
- To assess the risk factors of hyperglycemia,
- To estimate the incidence of complications of a non-compensated hyperglycemic state, such as ketoacidosis and hyperglycemic hyperosmolar non-ketotic syndrome,
- To assess the incidence of osteonecrosis of the jaw, and the risk factors for ONJ, To describe other AESIs, including GI toxicity, rash, hypersensitivity, pancreatitis, pneumonitis and SCARs

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Data analysis plan

Data analysis will include:

• cumulative incidence proportions estimated at different time points to assess specific events of interest

• descriptive analyses of relevant risk factors

• logistic regression to assess risk factors where appropriate
• where relevant, risk factors will be assessed taking time into consideration
• overall number and incidence proportion of patients with adverse events of special interest, will be summarized with 95% confidence intervals
• no statistical hypotheses will be tested in this study
• where inferential statistical methods are used their results are considered to be purely descriptive.

Documents

Study results

CBYL719C2404 Report Component Synopsis (1)_Updated Version_Redacted.pdf (227.98 KB)

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