OUTCOMES OF THE SPANISH COHORT OF EARLY ACCESS TO PERTUZUMAB AND TRASTUZUMAB EMTANSINE (KNOWHER STUDY)

First published: 30/01/2017

Last updated: 03/09/2024

Study Finalised

Administrative details

EU PAS number

EUPAS17462

Study ID

17463

DARWIN EU® study

No

Study countries

☐ Spain

Study description

Currently, four targeted anti-HER2 agents are available in the EU for the treatment of advanced HER2+ breast cancer: trastuzumab (a humanized monoclonal antibody that targets subdomain IV of HER2), lapatinib (a HER1/HER2 dual tyrosine kinase inhibitor) and, more recently, pertuzumab (a humanized monoclonal antibody that targets domain II of HER2) and trastuzumab emtansine (an antibody-drug conjugate). Three of these agents have been approved in combination with chemotherapy: trastuzumab, lapatinib (also authorized in combination with trastuzumab), and pertuzumab. Trastuzumab emtansine (T-DM1) is the only targeted agent currently approved as monotherapy. The addition of pertuzumab (Perjeta®) to trastuzumab plus chemotherapy (docetaxel) in first line treatment of HER2-positive metastatic breast cancer obtains a significant and clinically relevant increase in median PFS of 6.1 months. Both Kadcyla® and Perjeta® were available under those special access systems. In order to be eligible for CU of Trastuzumab emtansine (T-DM1) or Pertuzumab, patients had to meet the some prespecified criteria. After the EU approval, oncologists could prescribe the product to specific patients through the Early Access Program, provided that the local or regional responsible people accept payment.In order to evaluate the effectiveness and safety of Trastuzumab emtansine (T-DM1) and Pertuzumab in HER2-positive metastatic breast cancer under real-world disease conditions, is proposed a retrospective observational cohort non-comparative study / registry in Spain.

Study status

Finalised

Research institutions and networks

Institutions

Puerta de Hierro-Majadahonda University Hospital

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Clinical Pharmacology Service, Puerta de Hierro-Majadahonda University Hospital (HUPHM)

Spain

First published: 26/12/2012

Last updated: 20/08/2024

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

Clinical Pharmacology Department, Area del Medicament, Hospital Clínic de Barcelona

Spain

First published: 29/03/2010

Last updated: 24/08/2023

Institution | Hospital/Clinic/Other health care facility | ENCePP partner

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Avendaño-Sola Cristina

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/12/2016

Actual: 01/12/2016

Study start date

Planned: 03/04/2017

Actual: 30/01/2017

Data analysis start date

Planned: 03/07/2017

Actual: 30/01/2017

Date of final study report

Planned: 31/10/2017

Actual: 30/01/2017

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Roche Farma, S.A

Study protocol

Study protocol ML29844 V2.0_25_11_2016.pdf(769.45 KB)

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Other study registration identification numbers and links

ClinicalTrials.gov Identifier: NCT03025711

https://clinicaltrials.gov/ct2/show/NCT03025711?term=NCT03025711&rank=1

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)

Data collection methods:

Secondary use of data

Main study objective:

To evaluate the effectiveness of Trastuzumab emtansine (T-DM1) and Pertuzumab in patients with HER2-positive MBC treated under compassionate use or early access program.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Case-series

Study drug and medical condition

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

PERTUZUMAB

TRASTUZUMAB EMTANSINE

Medical condition to be studied

HER2 positive breast cancer

Population studied

Short description of the study population

Adult patients (age ≥ 18 years at enrolment) from the Spain with HER2-positive metastatic or locally recurrent unresectable breast cancer and who were treated with Trastuzumab emtansine (T-DM1) and Pertuzumab under compassionate use or early access program.

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

Other

Special population of interest, other

Breast cancer patients

Estimated number of subjects

700

Study design details

Outcomes

Overall survival. Defined as the time between the date of start of treatment and the date of death. For subjects without documentation of death, OS will be censored on the last date the subject was known to be alive, •Progression free survival. •Best overall response rate•Duration of response •Time to treatment failure •Time to Objective Response•Time to change treatment•Time to next treatment

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Data analysis plan

The analysis of the present study will primarily make use of descriptive statistical methods (i.e. number, mean, median, standard deviation, rate, range, and IC95% for the estimated parameters).Where possible, and if allowed by the number of enrolled patients receiving different treatment regimens, a comparative analysis of the outcomes across various groups will also be performed.If applicable, it will be calculated IC 95% for the estimated parameters in relevant subgroups, analysis of variance (t test or F test) or non-parametric testing, such as Wilcoxon’s rank-sum test or Kruskal–Wallis test, will be used to test group differences on the continuous variables. All test performed will be two-sided and carried out with a 5% α-error rate without correction for multiplicity.Categorical variables will be summarized by numbers and proportions, and, where applicable, chi-squared testing will be used to test group differences.

Data management

Data sources

Data sources (types)

Drug registry

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