REFINE: Regorafenib observational study in hepatocellular carcinoma

First published: 19/09/2017

Last updated: 27/03/2024

Study Finalised

Administrative details

EU PAS number

EUPAS20981

Study ID

50372

DARWIN EU® study

No

Study countries

- [ ] Albania
- [ ] Algeria
- [ ] Argentina
- [ ] Austria
- [ ] Belgium
- [ ] Brazil

- [ ] Canada
- [ ] China
- [ ] Denmark
- [ ] Egypt
- [ ] France
- [ ] Greece
- [ ] India
- [ ] Italy
- [ ] Japan
- [ ] Korea, Republic of
- [ ] Lebanon
- [ ] Luxembourg
- [ ] Mexico
- [ ] Netherlands
- [ ] Oman
- [ ] Russian Federation
- [ ] Saudi Arabia
- [ ] Spain
- [ ] Sweden
- [ ] Taiwan
- [ ] Thailand
- [ ] Türkiye
- [ ] United Arab Emirates
- [ ] United States

Study status

Finalised

Research institutions and networks

Institutions

Bayer AG

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Multiple centres: 150 centres are involved in the study

Contact details

Study institution contact

Bayer Clinical Trials BAYER AG clinical-trials-contact@bayer.com

Study contact

clinical-trials-contact@bayer.com

Primary lead investigator

Bayer Clinical Trials BAYER AG

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 17/03/2017

Actual: 23/02/2017

Study start date
Planned: 30/09/2017

Actual: 13/09/2017

Date of interim report, if expected
Planned: 22/04/2020

Date of final study report
Planned: 30/11/2022

Actual: 12/10/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Bayer AG

Study protocol

19244_REFINE_OS Protocol_v1.0_ Encepp.pdf(999.78 KB)

19244_Study Protocol_v2.1_2018-11-27_Redacted.pdf(1.43 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)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Main study objective:

To evaluate the safety of regorafenib in patients with uHCC, including incidences of all treatment-emergent adverse events (TEAEs) and dose

modifications due to TEAEs in real-world practice conditions.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

International, prospective, open-label, multicenter, observational study

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(L01XE21) regorafenib

regorafenib

Medical condition to be studied

Hepatocellular carcinoma

Population studied

Short description of the study population

The study population included male and female patients diagnosed with unresectable hepatocellular carcinoma (uHCC) initiated treatment with regorafenib under routine clinical practice.

Inclusion criteria:

• Patients with confirmed diagnosis of unresectable HCC

• Physician-initiated decision to treat with regorafenib (prior to study enrollment)
• Signed informed consent form

Exclusion criteria:
• Participation in an investigational program with interventions outside of routine clinical practice
• Past treatment with regorafenib

Age groups

Adolescents (12 to < 18 years)

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

Patients with unresectable hepatocellular carcinoma

Estimated number of subjects

1000

Study design details

Outcomes

Number of patients with treatment emergent adverse events (TEAEs) leading to dose modifications (including reductions, interruptions and permanent discontinuation), Overall survival (OS) Progression-free survival (PFS) Time to progression (TTP) Best overall tumor response (ORR) Duration of regorafenib treatment

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Data analysis plan

Statistical analyses will be of explorative and descriptive nature. The study is not aimed to confirm or reject pre-defined hypotheses. All variables will be analyzed descriptively with appropriate statistical methods: categorical variables by frequency tables (absolute and relative frequencies) and continuous variables by sample statistics (i.e. mean, standard deviation, minimum, median, quartiles, 5th and 95th percentiles, and maximum). Continuous variables will be described by absolute value and as change from baseline per analysis time point, if applicable.Patients who took at least one dose of regorafenib will be included in the safety analysis set (SAF).Patients who took at least one dose of regorafenib, did not violate a major inclusion/exclusion criterion, and had at least one follow-up assessment after receiving regorafenib will be included in the full analysis set (FAS). Safety data will be analyzed on the SAF, effectiveness data on the FAS.

Documents

Study results

19244_EU_PAS_Abstract_V1.0_2023-04-06.pdf(224.8 KB)

Study report

19244_Study_Report_Redacted_V1.0_2023-10-12.pdf(2.45 MB)

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