

# 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

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### Study ID

50372

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### DARWIN EU® study

No

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### 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
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## **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](mailto: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

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**Study start date**

Planned: 30/09/2017

Actual: 13/09/2017

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**Date of interim report, if expected**

Planned: 22/04/2020

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**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

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## 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

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#### Study type:

Non-interventional study

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#### Scope of the study:

Safety study (incl. comparative)

#### Data collection methods:

Primary data collection

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#### 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

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### **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

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### **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
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### **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)
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### **Special population of interest**

Other

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### **Special population of interest, other**

Patients with unresectable hepatocellular carcinoma

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### **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)

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### **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

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### 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

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### Check completeness

Unknown

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### Check stability

Unknown

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**Check logical consistency**

Unknown

**Data characterisation**

**Data characterisation conducted**

No