A National, Post-Registration, Observational Study of the Longterm Safety and Health Outcome of Patients Treated With Defitelio®, Including Patients With Severe Hepatic VOD After HSCT. (DEFIFRANCE Registry)

First published: 13/06/2019

Last updated: 22/02/2024

Study Planned

Administrative details

EU PAS number

EUPAS29649

Study ID

46727

DARWIN EU® study

No

Study countries

France

Study description

DEFIFRANCE is a French national registry of patients treated with Defitelio® in order to conduct a multicentre observational study comprising descriptive analysis and follow-up assessment of patients treated with Defitelio®.

Study status

Planned

Research institutions and networks

Institutions

Jazz Pharmaceuticals

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Zakaria Medeghri

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 20/01/2017

Study start date
Planned: 20/01/2017

Date of final study report
Planned: 31/07/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Jazz Pharmaceuticals

Study protocol

DEFIFRANCE - PROTOCOLE V_7_16May2019_Redacted.pdf (7.58 MB)

DEFIFRANCE - PROTOCOLE V_7_22Jan2022.pdf (7.58 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

The primary objective of this study is to evaluate the efficacy in patients treated with Defitelio® in terms of complete response (CR) of severe hepatic VOD at Day+100 post HSCT and survival at Day+100 post HSCT.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

DEFITELIO

Population studied

Age groups

- Infants and toddlers (28 days – 23 months)
- Children (2 to < 12 years)
- 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)

Estimated number of subjects

200

Study design details

Outcomes

The primary objective of this study is to evaluate the efficacy in patients treated with Defitelio® in terms of complete response (CR) of severe hepatic VOD at Day+100 post HSCT and survival at Day+100 post HSCT. -Describe the main characteristics of patients treated with Defitelio®-Evaluate the rate of SAEs of interest in patients treated with Defitelio®-Determine the rate of acute and chronic GvHD after HSCT-Determine the overall mortality and VOD-related mortality after treatment-Identify prognostic factors influencing CR and survival

in patients treated with Defitelio® for severe hepatic VOD

Data analysis plan

MedDRA coding will be used to classify SAEs. The frequency (in absolute values and as a percentage) of SAEs according to SOC (System Organ Class) and events considered individually in each of these classes will be indicated.

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)

Disease registry

Other

Data sources (types), other

Prospective patient-based data collection, Retrospective 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