A multi-centre, multinational, prospective observational registry to collect safety and outcome data in patients diagnosed with severe hepatic veno-occlusive disease (VOD) following haematopoietic stem cell transplantation (HSCT) and treated with Defitelio® (DF VOD-2013-03-REG)

First published: 21/01/2014

Last updated: 15/07/2020

Study | Finalised

Administrative details

EU PAS number

EUPAS5592

Study ID

36318

DARWIN EU® study

No

Study countries

- [ ] France
- [ ] Italy
- [ ] Portugal
- [ ] United Kingdom

Study description

A multi-centre, multinational, prospective observational registry to collect safety and outcome data in patients diagnosed with severe hepatic VOD following hematopoietic stem cell transplantation (HSCT) and treated with Defitelio®.The registry is performed in collaboration with the European Society for Blood and Marrow Transplantation (EBMT). Participating sites are transplant centres that are members of EBMT.Following obtaining an informed consent, clinical data from patients who are treated with Defitelio® for severe hepatic VOD will be collected. In addition all patients prescribed Defitelio® regardless of indication will also be registered, and information will be collected on the indication for which Defitelio® has been administered.Patient clinical data will be collected on the registry form at 100 days, 6 months and 12 months post-HSCT. The objective of this non-interventional study is to assess the incidence rate of specific SAEs of interest (including fatalities) in patients with sVOD treated with Defitelio®. All SAEs of interest will be recorded by the treating physician or by the site staff, whether or not these are considered to be related to the treatment received.

Study status

Finalised

Research institutions and networks

Institutions

Jazz Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Multiple centres: 36 centres are involved in the study

Networks

EBMT

Contact details

Study institution contact

Raj Hanvesakul raj.hanvesakul@jazzpharma.com

Study contact

raj.hanvesakul@jazzpharma.com

Primary lead investigator

Raj Hanvesakul

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 10/01/2014

Actual: 10/01/2014

Study start date
Planned: 01/04/2015

Actual: 24/04/2015

Data analysis start date
Planned: 18/10/2019

Actual: 18/10/2019

Date of final study report
Planned: 03/07/2020

Actual: 18/06/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Jazz Pharmaceuticals

Study protocol

FINAL DF VOD_2013_03_REG protocol version 2 - Signed (2)_Redacted.pdf

(401.52 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)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Primary data collection

Main study objective:

The main objective of the registry is to assess the incidence rate of specific SAEs of interest (including fatalities) in patients with severe hepatic VOD

treated with Defitelio®.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Prospective observational study

Study drug and medical condition

Name of medicine

DEFITELIO

Medical condition to be studied

Venoocclusive liver disease

Population studied

Short description of the study population

The Study Population is defined as the population of patients with severe hepatic VOD postHSCT who are entered into the registry and treated with Defitelio. This population will be used for all summaries. The initial diagnosis of severe hepatic VOD will be performed by the treating physician according to clinical practice. The relevant sections of the e-form will be filled in accordingly.

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)

Special population of interest

Hepatic impaired

Estimated number of subjects

176

Study design details

Outcomes

Incidence rate of specific SAEs of interest. -To describe the population treated with Defitelio®-To determine the incidence rate of multi-organ failure (MOF) and Graft versus hostdisease (GvHD) -To determine survival by Day+100 post-HSCT, overall mortality and mortality due toVOD-To determine the rate of VOD/MOF resolution any time after treatment initiation

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Data analysis plan

MedDRA coding will be used to classify and tabulate SAEs of interest. Frequencies (absolute and percentages) across SOC and for individual events within those classes will be provided for each treatment group. Safety data will be obtained by collecting the serious adverse events of interest, those listed as

identified and potential risks in the Risk Management Plan, namely haemorrhage, coagulopathy, hypotension, immunogenicity (allergic and hypersensitivity reactions), injection site reactions, infection and septicaemia, thromboembolic events, pregnancy and lactation.Incidence rates will be obtained for each of the treatment groups and relative risks calculatedwhere appropriate together with 95% confidence intervals for both the incidence rates and the relative risk for the Defitelio® group and the control group for patients with severe VOD.

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

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