# 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

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**Study start date**
Planned: 01/04/2015

Actual: 24/04/2015

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**Data analysis start date**
Planned: 18/10/2019

Actual: 18/10/2019

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**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

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**Non-interventional study design, other**

Prospective observational study

## Study drug and medical condition

**Medicinal product name**

DEFITELIO

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**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.

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**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)

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**Special population of interest**

Hepatic impaired

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**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

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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

**Check completeness**

Unknown

**Check stability**

Unknown

**Check logical consistency**

Unknown

# Data characterisation

**Data characterisation conducted**

No