A multi-centre, multinational, prospective observational registry on safety and outcome in patients diagnosed with severe hepatic VOD following hematopoietic stem cell transplantation (HSCT) and treated with Defitelio® ▼

EBMT
European Society
for Blood and Marrow Transplantation

CONTEXT

The European Commission in 2013 granted Gentium (a Jazz Pharmaceuticals company) the Marketing Authorization for Defitelio® (defibrotide) for the treatment of severe hepatic veno-occlusive disease (sVOD) in adults, children and infants undergoing hematopoietic stem cell transplantation.

In order to fulfil the specific post-approval obligation from the EMA, Gentium - as Marketing Authorization Holder - set up a disease registry to collect safety and outcome data in sVOD patients who were treated with Defitelio® as well as to assess patterns of utilization of Defitelio® in the post-approval setting.

STUDY DESIGN

This multi-centre, multi-national, prospective and observational registry:

- ✓ Took place in Europe
- ✓ Collects data until end of June 2019 on serious adverse events (SAEs) as well as endpoints of interest in relation to clinical outcome and standard baseline information
- ✓ EBMT developed a dedicated electronic database under ProMISe for this specific project
- ✓ The follow up of patients consists of 4 visits up to 12 month post HSCT

COLLABORATION

An important collaboration has been established between Gentium and EBMT for the development and the management of this registry which will provide extremely valuable safety and outcome data in this orphan indication.

PROGRESS OF THE REGISTRY

- ➢ Over 350 sites were invited across Europe, over 110 of them showed their willingness to participate.
- ➢ The European regulatory approvals were obtained in Austria, Belgium, Czech Republic, Denmark, Finland, France, Ireland, Italy, the Netherlands, Norway, Poland, Portugal, Sweden and United Kingdom.
- ➢ Enrollment was finally effective in 4 countries: France, Italy, Portugal and United Kingdom.
- ➢ The study recruited in the end 176 patients in total: Patient recruitment was finally ceased on 18 Jul 2018 after discussions with the Pharmacovigilance Risk Assessment Committee (PRAC).

Final enrolment		
Countries	Sites (active/open)	Patients
France	14 / 22	80
Italy	16 / 19	74
UK	6 /11	21
Portugal	1 / 1	1

CONTACT

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▼ This medicinal product is subject to additional monitoring