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®▼ or supportive care (control group)

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 a 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 are either treated with Defitelio® or managed through supportive care only as well as to assess patterns of utilization of Defitelio® in the post-approval setting.

STUDY DESIGN

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

- ✓ Can take place in at least 14 countries in Europe.
- ✓ Collects data until June 2018 and plans to recruit 300 patients treated with Defitelio® and 300 patients managed through supportive care only who will act as a control group.
- ✓ Collects data on serious adverse events (SAEs) as well as endpoints of interest in relation to clinical outcome and standard baseline information.

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, and to date, over 110 of them showed their willingness to participate.
- ✓ So far, the European regulatory approvals are obtained in Austria, Belgium, Czech Republic, Denmark, Finland, France, Ireland, Italy, the Netherlands, Norway, Poland, Portugal, Sweden and United Kingdom.
- ✓ Is currently enrolling in 4 countries: France, Italy, Portugal and United Kingdom.
- ✓ EBMT developed a dedicated electronic database under ProMISe for this specific project.

On February, 13th 2017:

Country	Open sites	Pending sites	Registered patients
France	22	0	43
Italy	20	10	48
Portugal	1	1	0
UK	4	7	0
Cz. Rep.	0	1	0
Ireland	0	2	0

CONTACT

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▼This medicinal product is subject to additional monitoring