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

Gentium
A Jazz Pharmaceuticals Company

CONTEXT

- ✓ The European Commission in 2013 granted Gentium SpA (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 (over 1 month of age) undergoing hematopoietic stem cell transplantation.
- ✓ In order to fulfil a specific post-approval obligation from the EMA, Gentium SpA - 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.
- ✓ Is open 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) of interest as well as endpoints of interest in relation to clinical outcome and standard baseline information.

COLLABORATION

An important collaboration has been established between Gentium SpA and the 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, 115 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.
- ✓ The list of participating countries is expanding progressively and new countries in Europe are requesting to be included (Greece, Croatia…).
- ✓ EBMT developed a dedicated electronic Med C under ProMISe 3 for this specific project.

On March, 3rd 2016:

Country	Open sites	Pending sites	Registered patients
France	18	3	20
Italy	14	13	21
Portugal	1	1	0
UK	0	11	0
Belgium	0	10	0
Ireland	0	2	0

CONTACT

For further information about your participation to this registry, please contact:

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▼This medicinal product is subject to additional monitoring