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 Authorisation 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.

Design

This multi-centre, multi-national, prospective, observational registry is now launched and will:

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

Hepatic Veno Occlusive Disease

Hepatic veno-occlusive disease is a life-threatening regimen-related toxicity that constitutes a barrier to successful allogeneic and autologous HSCT.

The severe form of the disease, usually associated with pulmonary and/or renal dysfunction, and a mortality rate of more than 80% by 100 days after HSCT, is reported to occur in about 40%-60% of VOD patients, that is approximately 3-5% of transplanted patients.

Progress of the Registry

- ✓ So far, the regulatory approvals are obtained in Denmark, France, Ireland, Italy, the Netherlands, Poland, Portugal, Sweden and United Kingdom.
- ✓ Over 350 sites were invited across Europe, and to date, 90 of them showed their willingness to participate.
- ✓ The list of participating countries is expanding progressively and new countries in Europe are requesting to be included (Greece, Austria, Norway, Finland, Croatia…).
- ✓ Some sites in Italy and France have already been initiated and are ready to register patients.
- ✓ EBMT developed a dedicated electronic Med C with ProMISe 3 for this specific project.

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.

Contact

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

EBMT Data Office Paris:
jessica.lemaitre@upmc.fr
Tel:+33.1.70.64.24.15
Fax:+33.1.71.97.05.88

▼ **This medicinal product is subject to additional monitoring**