**EFFICACY AND SAFETY OF DEFIBROTIDE IN THE TREATMENT OF HEPATIC VENO-OCCLUSIVE DISEASE/SINUSOIDAL OBSTRUCTION SYNDROME FOLLOWING HAEMATOPOIETIC CELL TRANSPLANTATION: INTERIM RESULTS FROM THE DEFIFRANCE STUDY**

**Anne Huynh**[1]; Myriam Labopin[2]; Ibrahim Yakoub-Agha[3]; Didier Blaise[4]; Régis Peffault de Latour[5]; Virginie Bouvatier[6]; Déborah Gutierrez[6]; Jean-Hughes Dalle[7]; Mohamad Mohty[2]

*[1]Institut Universitaire du Cancer de Toulouse-Oncopole, Toulouse, [2]Hôpital Saint-Antoine, Paris, [3]Hôpital Claude Huriez, Lille, [4]Institut Paoli Calmettes, Marseille, [5]Hôpital Saint-Louis, Paris, [6]Jazz Pharmaceuticals, Lyon, [7]Hôpital Robert-Debré, Paris, France*

Hepatic veno-occlusive disease/sinusoidal obstruction syndrome (VOD/SOS) is a potentially fatal complication after haematopoietic cell transplantation (HCT). Defibrotide is approved for severe hepatic VOD/SOS post-HCT in patients aged >1 month in the EU. DEFIFrance is an ongoing, post-marketing study collecting retrospective and prospective real-world data on patients receiving defibrotide at 36 HCT centres in France since July 2014. VOD/SOS severity was categorised using EBMT criteria (adults) or study steering committee member adjudication (paediatric patients). Primary endpoints include Kaplan-Meier–estimated Day 100 (post-HCT) survival and Day 100 complete response (CR; total serum bilirubin <2 mg/dL and multi-organ failure [MOF] resolution per investigators' assessment). Of the 324 patients enrolled at interim analysis data cut-off (November 2018), 120 had VOD/SOS post-HCT. Day 100 Kaplan-Meier–estimated survival was 59%; the CR rate was 53%. MOF occurred in 37/120 patients; 30/35 patients with renal failure underwent dialysis, and 28/35 patients with respiratory failure required a ventilator. Estimated Day 100 survival rates were 85% and 32% with severe (n=40) and very severe (n=49) VOD/SOS, respectively; Day 100 CR rates were 82% and 27%, respectively. Adverse events (AEs) of interest occurred in 56% of patients; the most common (>5%) were BK virus infection and viral haemorrhagic cystitis (6% each). Among patients receiving defibrotide for VOD/SOS post-HCT, outcomes were better with severe versus very severe (per EBMT criteria in adults and expert adjudication in paediatric patients) disease, highlighting the importance of VOD/SOS diagnosis/treatment before patients reach the most severe VOD/SOS stages. AEs of interest were consistent with previous studies.