COMP issued a positive opinion on orphan medicinal product designation for VS-01 for the treatment of acute liver failure.

LONDON (May 17, 2016) – The Committee for Orphan Medicinal Products (COMP), in light of the overall data submitted and the discussion within the Committee, issued a positive opinion on orphan medicinal product designation for VS-01 for the treatment of acute liver failure.

About Orphan Drug Designation

The EMA's COMP grants the orphan designation for medicines that are intended for the treatment of rare diseases. To qualify for orphan designation, a medicine must meet the following criteria: 1) it must be intended for the treatment of a disease that is life-threatening; 2) the prevalence of the condition in the EU must not be more than 5 in 10,000; no satisfactory method of treatment of the condition concerned can be authorised, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

It offers the sponsor incentives, which include:

- Protocol assistance during clinical development;
- Access to the centralised authorisation procedure; This allows companies to make a single application to the EMA resulting in a single opinion valid in all EU Member States;
- Access to conditional approval;
- Market exclusivity post approval of 10 years.
- Reduced fees for regulatory activities including protocol assistance, marketing-authorisation applications, inspections before authorisation and applications for changes to marketing authorisations made after approval, and reduced annual fees.
- Available funding from the European Commission

About acute liver failure

Acute liver failure is a condition in which the rapid deterioration of liver function results in hepatic encephalopathy, coagulopathy, and even progressive multi organic failure in the

absence of a pre-existing liver disease. The definition of 'acute' refers to a patient without pre-existing chronic hepatic disease and with an illness of less than 26 weeks duration.

Hepatic encephalopathy, cerebral oedema and infections are the most common complications of acute liver failure. Liver transplant is the gold standard in terms of treatment; however, due to the limited availability of donor organs the mortality remains high.

About VS-01

VS-01 is a sterile, white liposomal suspension. It contains citric acid in transmembrane pH-gradient liposomes that acts as a clearance enhancer of toxic substances once administered in the peritoneal space.

Following administration of VS-01 in the peritoneal cavity, citric acid contained within the liposomes' core protonates low molecular weight, ionizable toxic compounds (*e.g.*, ammonia) that remain entrapped since charged molecules cannot diffuse back. VS-01 acts as a standard peritoneal dialysis which is a well-established procedure for the treatment of chronic kidney failure. VS-01 also clears some of the toxic metabolites accumulated during liver disease and may enhance the evacuation of endotoxins.

The COMP has judged that Versantis has submitted sufficient data to support the medical plausibility of VS-01 for the purpose of designation.

About Versantis

Versantis reveals the potential of liposomal peritoneal dialysis to discover and develop a pipeline of drugs for acute liver diseases. Our first-product VS-01 has a dual mechanism of action targeting multiple failing organs to achieve a marked therapeutic benefit in a broad range of orphan diseases.