



Versantis Receives U.S. FDA Orphan Drug Designation for VS-01

Designation Awarded for Treatment of Hyperammonemia in Inborn Errors of Metabolism

Zurich, Switzerland, September 7th, 2021 – Versantis AG, a clinical-stage company developing novel therapies for rare liver diseases and rare pediatric diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to its lead product candidate VS-01 for the treatment of hyperammonemia (HA) in inborn errors of metabolism (IEM).

“This orphan drug designation from the FDA validates the scientific rationale of VS-01 in a new indication encompassing multiple genetic disorders where there is a vital need to remove the life-threatening build-up of ammonia,” said Meriam Kabbaj, Chief Operations Officer and Co-founder of Versantis. “VS-01 has now received U.S. FDA ODD for two main indications, hyperammonemia in inborn errors of metabolism and Acute-on-chronic Liver Failure (ACLF). Orphan drug incentives considerably strengthen the clinical development plan of our liposomal scavenging platform technology and provide a huge opportunity to soon address the growing burden of metabolic / liver diseases.”

The U.S. FDA ODD program is designed to provide incentives to sponsors developing drugs and biologics for rare diseases and conditions, in part defined as affecting fewer than 200,000 people in the United States. Sponsors of designated orphan drugs are eligible for tax credits for clinical trial costs, waiver of the user fee for marketing applications and, upon approval, consideration for seven years of marketing exclusivity.

“VS-01 is potentially a game-changing medication for the treatment of acute hyperammonemia in an emergency setting. The new designation from the FDA continues to validate our approach and development of VS-01, which was shown to be safe and well tolerated in our Phase 1b clinical study in patients with decompensated liver cirrhosis,” said Vincent Forster, Versantis’ Co-founder and CEO. “Building on this encouraging first experience in patients, we are in the process of raising new funds to continue advancing VS-01 towards its much-needed use in clinical practice.”

About Inborn Errors of Metabolism causing Hyperammonemia

IEMs causing HA comprise a group of rare genetic metabolic diseases in which a defect in an enzyme blocks the metabolic clearance of ammonia from the blood. This leads to a toxic

accumulation of ammonia in the body (hyperammonemia), which particularly affects the brain by causing cerebral edema and severe neurologic impairment through a variety of mechanisms. The most relevant group of inherited genetic defects associated with HA are Urea Cycle Disorders (UCDs) and organic acidemia. Currently, most patients are first diagnosed at clinical presentation when HA can rapidly progress to coma and death if not treated immediately.

About Versantis

Versantis is a clinical stage company focused on addressing the growing, un-met medical need in liver diseases. With a pipeline of drug and diagnostic products covering chronic and acute indications, Versantis believes it can revolutionize current standard of care for patients suffering from acquired and genetic hepatic deficiencies. Versantis' lead program, VS-01, is in clinical development as a first-line therapy for the timely reversal of ACLF. It harnesses Versantis' proprietary scavenging liposomes to extract toxins from the body and, if approved, will be the first drug to take advantage of the intraperitoneal route to simultaneously support the liver, kidney and brain, the organs that most often fail in cirrhotic patients. VS-01 has received ODD from the EMA and U.S. FDA, as well as a Rare Pediatric Diseases Designation from the U.S. FDA. Founded by scientists from ETH Zurich with entrepreneurial drive, Versantis has built a team and Board of seasoned industry executives with a proven ability to advance novel therapies from the idea stage into clinical development. The company is headquartered in Zurich, Switzerland. For additional information, visit: www.versantis.ch.

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