Versantis Receives FDA Rare Pediatric Disease Designation for VS-01 for the Treatment of Urea Cycle Disorders

Designation qualifies Versantis to receive FDA priority review voucher (PRV) upon marketing approval of VS-01

Zurich, Switzerland, October 19th, 2020 – Versantis AG, a clinical-stage company developing novel therapies for orphan liver diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted a rare pediatric disease designation (RPDD) to its lead product candidate VS-01, an ammonia clearance enhancer, for the treatment of Urea Cycle Disorders (UCD). UCD is a rare and life-threatening condition caused by an inherited inborn error of metabolism. Current treatment options are associated with poor outcomes.

"The rare pediatric disease designation highlights the potential of using VS-01 to treat the serious and life-threatening manifestations of UCD," said Meriam Kabbaj, Chief Operations Officer and Co-founder of Versantis. "It is valuable recognition that will help Versantis to ramp up its pediatric program by fostering collaborations with key opinion leader and patients' associations."

The FDA grants rare pediatric disease designation for serious or life-threatening diseases primarily affecting children from birth to 18 years and affecting fewer than 200,000 people in the USA. Upon approval of a new drug application, the RPDD renders sponsor companies eligible for a priority review voucher, which can be redeemed to obtain accelerated FDA review of a drug candidate, in any indication, potentially gaining early market access. This voucher may be sold or transferred to another sponsor.

Versantis recently completed a $16M Series B financing round and is currently raising new funds to fuel VS-01 clinical development in rare indications, including UCD.

"This recognition acknowledges the unmet medical needs of children with UCD. Importantly, with this designation, we may qualify to receive a priority review voucher from FDA, at the time of marketing approval of VS-01. It provides significant value to our current effort to develop effective treatments for this devastating rare disease," said Vincent Forster, Versantis' Co-founder and CEO.

VS-01 is an innovative liposomal-based detoxification therapy that acts as a clearance enhancer for a large spectrum of toxic metabolites accumulated during liver and kidney failures. More specifically, VS-01 clears ammonia from the body, which is the main neurotoxic metabolite and can lead to brain edema. With its rapid onset, VS-01 could be the first-line medication of choice for acute hyperammonemia in an emergency setting. VS-01 is currently being evaluated in clinical trials in decompensated cirrhotic patients.

About UCD

Urea cycle disorder is an orphan condition that predominantly affects children where patients present with hyperammonemia either shortly after birth (about 50%) or, later at any age, leading to death or to severe neurological handicap. The clinical course in newborns is characterized by an initial hyperammonemic crisis shortly after birth progressively leading to somnolence, lethargy, coma and death. In hyperammonemic crises, dialysis is used to drastically lower ammonia levels, however, it can only be performed in highly specialized tertiary centers and is often initiated too late. Up to 90% of newborns do not survive the initial event of acute hyperammonemia or suffer from severe neurological sequelae. Curative orthotopic liver transplantation can be performed in children from about 10 kg body weight. VS-01 is principally intended as bridging therapy until the children are old enough and have reached the appropriate body weight and/or until they can access a tertiary center.

About Versantis

Versantis is a clinical-stage biotech company focused on the development of orphan drugs in liver and pediatric diseases. Founded by leading scientists from ETH Zurich and industry experts, Versantis is pursuing innovative therapies for the critical care of serious conditions based on its proprietary detoxification platform technology. The company's mission is to bring therapeutic solutions to many patients in need by timely supporting hyperammonemic crises in children (now supported by the FDA RPDD) as well as acute liver conditions (Versantis' most advanced program). In addition to the RPDD mentioned herein, VS-01 was granted orphan drug designations in acute liver failure by the EMA and in acute-on-chronic liver failure (ACLF) by the FDA, which may help to streamline its clinical development pathway. Versantis is headquartered in Zurich, Switzerland. For additional information, visit: www.versantis.ch.

Contacts

Versantis AG
Vincent Forster, +41 44 500 8891
info@versantis.ch

Halsin Partners
Mike Sinclair, +44 20 7318 2955
msinclair@halsin.com