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ASIA EDITION

# Versantis receives FDA RPDD for treatment of urea cycle disorders

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**UCD is a rare and life-threatening condition caused by an inherited inborn error of metabolism**



Versantis AG, a clinical-stage company developing novel therapies for orphan liver diseases based in Switzerland, has recently announced that the US 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 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.

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.

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.