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Pursuing a Highly-differentiated Approach to the Treatment of Orphan Liver Diseases and Ultra-rare Pediatric Urea Cycle Disorders

The Company

Versantis, founded in 2015 based on technology spun out of **ETH-Zurich**, is a **Phase 2-ready, clinical-stage** pharmaceutical company focused on the development and commercialization of highly differentiated **orphan drugs and diagnostics** for the management of high unmet medical needs in **advanced liver diseases and pediatric urea cycle disorders.** Versantis is currently in development with **four product opportunities,** each uniquely addressing an urgent, medical need.

Our pipeline of development candidates is as follows:

PROGRAMS	TECHNOLOGY	DISCOVERY	NONCLINICAL	PHASE 1	PHASE 2	PHASE 3
DRUG CANDIDATES						
VS-01 (ACLF)	Intraperitoneal scavenging liposomes	Acute-on Chronic Liver Failure (ACLF)				
VS-01 (UCD)		Urea Cycle Disorders				
VS-02	Oral small molecule	Hepatic Encephalopathy				
DIAGNOSTICS PROGRAMS						
TS-01	Dye-loaded polymersomes	Ammonia diagnostic				

Pipeline of growth opportunities focused on rare liver diseases and Urea Cycle Disorders

Our **lead FDA-designated orphan product candidate,** VS-01 (ACLF) , has completed a **successful Phase 1b clinical study** in Germany and is being advanced in early 2022 into a multi-national Phase 2 human trial in patients with Grades 1 and 2 *Acute-on-Chronic Liver Failure*. This indication is of high commercial value (**>$750M peak U.S. sales**[1]).

We are also developing **an FDA ultra-rare pediatric disease designated product candidate,** VS-01 (UCD) , in acute hyperammonemia in inborn errors of metabolism, including *Urea Cycle Disorders*.

Our **third product candidate,** VS-02 **, is an oral, small molecule** for the chronic maintenance treatment of *hepatic encephalopathy* in patients with decompensated cirrhosis. It has demonstrated decreased ammonia levels in the blood and glutamine levels in the brain.

Through InnoSuisse research grants, we are developing TS-01 , a unique point-of-care diagnostic device for the *at-home measurement of ammonia in blood*, the primary cause of hepatic encephalopathy.

Investment Highlights $40M+ Series C target

VS-01 (ACLF)
Lead product candidate; phase 2-ready in **FDA orphan-designated** acute-on-chronic liver failure (ACLF)

VS-01 (UCD)
Pre-clinical product candidate in **FDA rare pediatric disease designated** acute hyperammonemia in newborn and infants due to **ultra-rare Urea Cycle Disorders (UCD)**

VS –02*
Pre-clinical oral, small molecule product candidate **for the chronic and preventive treatment of hepatic encephalopathy (HE)**
*subject to license from ETH

TS-01
Point of care diagnostic device **in prototype development and grant-funded by Innosuisse** for **at-home blood ammonia measurement**

$21M raised from sophisticated European investors and **$2.0M in grants**

[1] US Market Research, Dec. 2021

Versantis-targeted liver diseases: high unmet needs in three substantial indications

Acute-on-Chronic Liver Failure (ACLF) is a rapidly evolving complication of cirrhosis with high short-term mortality, usually instigated by a precipitating event leading to acute decompensation of liver cirrhosis, and potentially progressing without intervention to multi-organ failure (i.e., liver, brain, and kidneys). The 28 Day-mortality rates of patients with Grades 1 or 2 ACLF range from 22 – 32%. No treatments have been approved to date and only supportive measures are available to patients, including extracorporeal liver support (hemodialysis, MARS…) which fail to improve patient survival. ACLF is a ***rare*** disease affecting 137,000[1] patients yearly in the US and leading to extremely high hospitalization costs (>$50,000[1] in the US), often driven by the need for intensive care.

Urea Cycle Disorders (UCDs) are ***rare*** congenital metabolic deficiencies affecting an estimated 2,000 newborns yearly (EU5+USA). Fast reduction of hyperammonemia is crucial to improve survival outcomes after birth. Available treatment options are costly (>$900,000/patient/year in US) and are limited to the chronic management of surviving UCD patients.

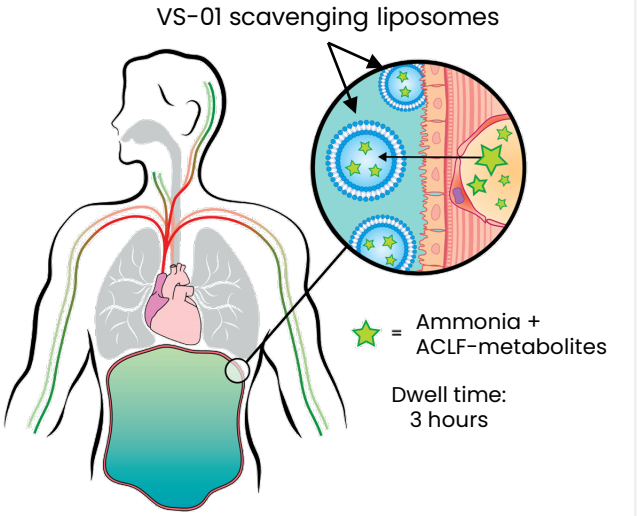
The unmet medical need is significant in the first weeks of life, where **VS-01 (UCD)** has the potential to be the first line of treatment. Versantis aims to develop this ***Priority Review Voucher eligible*** product candidate such that it is **IND-ready in 2024.**

Hepatic Encephalopathy (HE) is a serious neurologic condition caused when toxins, primarily ammonia, which are normally cleared by the liver, accumulate in the blood, affecting the brain. Annually, an estimated 350,000 cirrhotic patients are hospitalized for HE in the US and EU. Standard of care is limited (rifaximin, lactulose) and may impair quality of daily life or bear the risk of bacterial resistances.

Versantis plans to develop **VS-02, an oral, small molecule** to treat HE and **TS-01**, an ammonia point-of-care diagnostic device to serve this patient population such that both are ready for FDA IND submission in 2024 and clinical development commencing in 2025.

VS-01 (ACLF) target product profile:

- **To safely clear ACLF metabolites leading to improved mortality**
- **To support liver, brain, kidneys**
- 1st **To be first-line therapy following paracentesis**
- **To save costs by shortening hospital and ICU stays**



VS-01 (ACLF) is a liposomal formulation infused into the peritoneal cavity following paracentesis where it traps and concentrates ammonia along with other ACLF inflammatory metabolites involved in liver, kidney, and brain failures.

VS-01 (ACLF) is positioned early and seamlessly into the treatment algorithm of ACLF decompensations and is targeted to be the first therapy able to simultaneously support the liver, kidneys, and brain, potentially leading to an improved prognosis.

In Versantis' Phase 1b trial in patients with decompensated liver cirrhosis, VS-01 (ACLF) was safe and well-tolerated, while also showing activity in selected biomarkers and clinical cognitive tests.

[1] US Market Research, Dec. 2021

Planned value-creating milestones:

MILESTONE		TIMING
VS-01 (ACLF)	pre-IND meeting	Q1: 2022
VS-02	licensed from ETH	Q1: 2022
Selected EU countries P2 ACLF regulatory approval/first-patient in		Q2: 2022
VS-01 (ACLF)	IND cleared by FDA	Q2: 2022
VS-01 (ACLF)	Phase 2 50% enrolled	Q2: 2023
VS-02	Confirmatory non-clinical efficacy	Q3: 2023
VS-01 (UCD)	IND-enabling package	Q4: 2023
TS-01	Commercial prototype	Q1: 2024
VS-01 (ACLF)	Phase 2 clinical results	Q2: 2024
VS-01 (UCD)	IND submission	Q3: 2024
VS-02	IND-enabling package	Q3: 2024
VS-02	IND-submission	Q4: 2024

Experienced management team, regulatory advisors and board:

Mark Fitzpatrick, CEO; Formerly CEO of Chiasma, Inc. and CFO of Aegerion Pharmaceuticals, driving 2 orphan product regulatory approvals and launches

Meriam Kabbaj, PhD COO, co-founder; +18 years of experience in leading clinical research at CROs and in CMC, clinical and regulatory development of VS-01

Vincent Forster, PhD CSO, co-founder/ inventor at ETH; +8 years' experience developing VS-01; awarded prestigious entrepreneurial and scientific prizes

Prof Katharina Staufer, MD, CMO; gastroenterologist and hepatologist with a +16 years of strong clinical and scientific background in metabolic liver disease, liver cirrhosis and transplant hepatology

Regulatory advisors

Pam Williamson, MBA Acting Head of Regulatory Affairs; Former Global SVP of regulatory at Alexion, Genzyme, Serono

Donna Griebel, PhD Former Division Director FDA, Division of gastroenterology and inborn error products.

Andrew Mulberg, PhD Former Deputy Division Director FDA, Division of gastroenterology and inborn error products

Board

Peter Nicklin, Chairman; Former Corporate VP and EMEA President of Baxter, where he was responsible for the $4B business in the region.

Christopher Seaton, Director; 23 years at Bayer AG primarily in the U.S., culminating in Senior Vice President, Negotiations, Bayer HealthCare.

Michael Sidler, PhD, Managing Partner and co-founder, Redalpine Capital

Robert Schier, PhD, Investment Director, Swisscanto Invest