

**Vital Therapies, Inc. (VTI) is developing the first human allogeneic cellular therapy for acute liver failure; ELAD® provides extracorporeal support for patients with liver failure by processing toxins and synthesizing proteins and metabolites that are the key processes of normal human liver function. VTI has completed seven clinical trials including a pivotal trial in China, which was used to file for marketing approval in China. A US/EU pivotal trial plan to secure BLA and MAA approval is under review by the FDA and EMA and should start by end of 2011.**

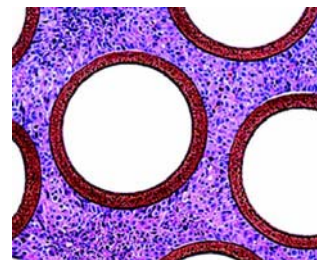
**Product** ELAD is comprised of four cartridges containing 440 grams of immortalized human liver cells mounted on a bedside unit. The patient's plasma flows between the cells through 32,000 hollow fibers in the cartridges, allowing two-way transfer of metabolites with the liver cells. During ELAD therapy the cells metabolize toxins and synthesize proteins and other liver specific products essential for life, assisting liver function.

**Technology** The proprietary ELAD human liver cell line was licensed from the Wistar Institute in Philadelphia and further developed by Baylor College of Medicine. Derived from a human hepatoblastoma, the cells can be grown in unlimited quantities, stored and shipped worldwide. Once incorporated into the bedside unit, a set of cartridges enables continuous treatment for up to 17 days without the cells losing their ability to perform.

**Clinical Development** Seven human clinical trials have been completed in the USA, UK and China. 157 patients have received ELAD treatment including 16 in compassionate use programs in USA, UK, Saudi Arabia, and Singapore. Phase 1 and three phase 2 controlled, randomized, multicenter trials were run in USA and Europe with promising results. In China, VTI has completed a successful pivotal trial and has filed for market approval. A US/EU pivotal trial plan is currently undergoing FDA and EMA review.

**Regulatory Status** ELAD is regulated as a combination biologic in the USA by the FDA's Division of Cellular, Tissue and Gene Therapy in CBER, the biologics division. In the EU, ELAD is a Combined Somatic Cell Advanced Therapy Medicinal Product (cATMP) and in China, ELAD is regulated as a medical device. The California FDB has licensed VTI's San Diego plant as a cGMP compliant Drug Manufacturing Facility. The plant also passed a UK QP audit.

**U.S. Market** Transplantation is the sole therapy shown to improve survival in liver failure but only 6,400 transplants are performed annually with an average waiting time of about a year. There are about 30,000 patients that are not eligible for transplantation and have no therapeutic options available which comprise ELAD's main market. In a market study done for VTI by Easton Associates, a U.S. market potential for ELAD of \$1.08 billion per year was defined.



ELAD Cross-section (40X)

## CORPORATE INFORMATION

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**China Opportunity** Liver disease is pandemic in China. IMS China estimates that 12% of the population is infected with hepatitis B and C and there are more than 400,000 deaths from liver failure annually. In China's urban centers, IMS identified over 1 million patients who would be clinical candidates for ELAD. With the cooperation of eminent physicians who approached VTI through a long time VTI Chinese employee, a 100% owned subsidiary was formed in China to conduct the clinical trial and launch ELAD in China. The China trial was completed and a market application was filed. It is under review at the China SFDA.

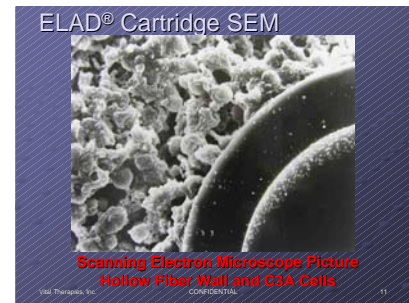
**China Partner.** In June 2011, VTI signed a Cooperation Agreement with Jilin Aodong Medicine Group Co Ltd, based in Dunhua, Jilin Province, China to work together to secure SFDA approval and to market ELAD in China. Aodong has made an investment in VTI and, on ELAD approval in China, will increase that investment and take two seats on VTI's Board.

**Compassionate Use** ELAD has been approved for compassionate use via an Expanded Access protocol with cost recovery by the US FDA and regulatory bodies in the UK, Singapore and Saudi Arabia. A total of 16 patients have been treated through these programs.

**Manufacturing** The bedside system, disposables and cartridges are externally sourced. The cells are grown in the cartridges at VTI's cGMP facility in San Diego, which was recently expanded to a capacity of 2,000 patient sets per year. The logistics of growing, storing, shipping and connecting the cartridges to the bedside unit have been proven during the US, UK and China clinical trials. On approval in China, plans will be initiated to build a large production plant there.

**Competition** Liver transplantation is the only therapy proven to extend survival in patients with acute liver failure. However, there are a limited number of livers available, the procedure is very expensive and requires a lifetime of immunosuppressive drugs. Existing toxin filtration systems include albumin dialysis, charcoal or resin filtration and plasma exchange. However, none of these has been shown to improve survival, nor are they accepted as a standard of care. Recently published data on trials of two albumin filtration devices showed no impact on survival despite removal of toxins and improvement in encephalopathy. There are currently no other living cell-based liver support devices in controlled clinical trials anywhere in the world. First mover advantage for VTI will include Orphan Drug, Fast Track and Priority Status in the USA creating substantial technical and regulatory advantages.

**Financing** VTI is financed by a syndicate of US and China venture capital funds led by Versant Ventures and MedVenture Associates, and by private investors including numerous US and three Chinese individuals.



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