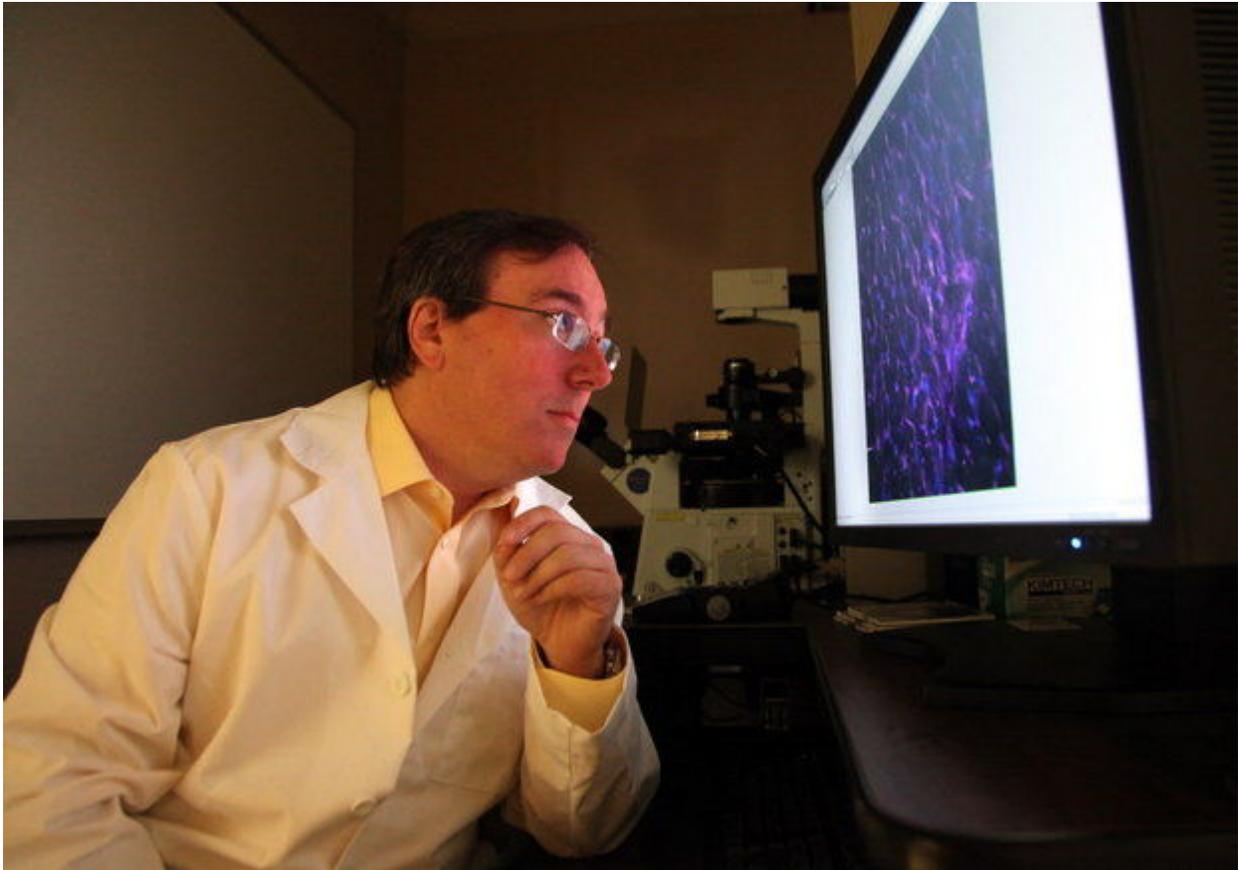


Local biotech's heart failure treatment shows promise; Juventas to launch Phase 3 study next year



Dr. Marc Penn studies a slide of mouse heart tissue in this 2012 photo taken in Rootstown. Penn and his team at Juventas have been working on JVS-100, a method of treating damaged heart muscle that recruits the body's innate healing abilities, for the past decade. The company was a Cleveland Clinic spinoff formed in 2007 when Penn still worked for the hospital system; he is now Director of Cardiovascular Research at Summa Health System in Akron. *(Thomas Ondrey/ The Plain Dealer)*



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CLEVELAND, Ohio— A potential new treatment for heart failure developed by Cleveland Clinic spinoff **Juventas Therapeutics** has shown new promise in a Phase 2 trial, according to data recently released by the company.

The treatment, a gene therapy called **JVS-100** that recruits the body's own stem cells to repair damaged areas of the heart, was safe and improved cardiac function in patients with advanced heart failure who had heart attacks more than a decade ago.

In the trial, called STOP-HF, 93 participants received either a single treatment of a low dose of JVS-100, a high dose of JVS-100, or placebo. They were then followed and evaluated at two points -- four months and one year later -- to test their heart function.

"We're very excited by the results," said **Dr. Marc Penn**, a former Cleveland Clinic doctor and founder and Chief Medical Officer for Juventas, who is now Director of Cardiovascular Research at Summa Health System in Akron. "This is a high risk population probably approaching most cancer mortalities."

About 6 million Americans suffer from **heart failure**, which happens when the heart can no longer keep up with its workload. About half of people diagnosed with heart failure die within five years, according to the Centers for Disease Control and Prevention.

Penn estimates that about 1.5 million people with heart failure do not benefit from current treatments, and still suffer daily symptoms, which include shortness of breath, fatigue and swelling from fluid buildup in the limbs. As the heart muscle weakens over time, the heart grows larger to compensate and becomes less efficient, pumping less blood (a measure referred to as ejection fraction) per heartbeat.

In the STOP-HF trial, JVS-100 reduced the size of the heart by about 8 percent and improved ejection fraction by about 2 percent overall, and higher doses of the treatment worked better. Participants in the trial had symptomatic heart failure, were about 65 years old on average, and had suffered a heart attack an average of 11 years prior to enrolling in the trial. JVS-100 was delivered via catheter to 15 spots in the heart muscle that showed signs of damage from a previous heart attack.

In a subgroup of high-risk patients within the trial who had more advanced heart failure, there was a decrease in a key heart function measure called end systolic volume, which indicates the size of the heart after it finishes its squeeze. Smaller values correlate with better heart function.

"This level of reduction correlates with a greater than 80 percent chance of us having a meaningful mortality benefit over two years -- at least a 20 percent reduction in mortality," Penn said.

STOP-HF was not designed to measure the impact of the drug on the risk of dying from heart failure over time, so these interim Phase 2 results can only give a suggestion of how the therapy may affect a patient's survival. A definitive test of the drug, in a Phase 3 trial, is set to launch next summer and should enroll several hundred heart failure patients.

Participants in the STOP-HF trial are still being followed and the company will report final results by the end of this year.

Juventas, which was spun out of the Cleveland Clinic in July 2007, has since attracted \$32 million in private investment and grants. The company is also testing JVS-100 for people with critical limb ischemia, a severe blockage of blood flow in the legs that causes pain, blood clots and amputations.

The company believes the treatment works by stimulating the patient's stem cells to heal and regenerate tissue that has been damaged, reduce the amount of scarring, and block any ongoing cell damage caused by the chronic disease.

"This repair mechanism isn't just about the heart, it's really about any organ in the body and having a specific drug that activates this repair pathway that allows us now to attack disease in a new and fundamentally novel way," Penn said.

"It's very exciting to be working on this for so many years and to see this pathway become so important."