NT13-136
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Novel Therapies for Congestive Heart Failure

Summary

Congestive heart failure (CHF) is defined as abnormal heart function resulting in inadequate cardiac output for metabolic needs. Dr. Paul Grayburn, a cardiologist in Baylor Scott & White Research Institute, is a pioneer in the use of ultrasound targeted microbubble destruction (UTMD) technology to deliver therapeutic genes and proteins to treat cardiac disease. Our technology can reverse established Adriamycin (ADM) cardiomyopathy by stimulating myocardial regeneration in an in vivo rat model. It is a potential breakthrough for treating CHF.

Key Investigators
Paul Grayburn, M.D.
Cardiologist

Field
Cardiology

Technology
Novel therapy for congestive heart failure

Key Features
Gene therapy
Protein therapy

Stage of Development
Preclinical proof of concept

Status
Available for licensing

Patent Status
Patents pending
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Market

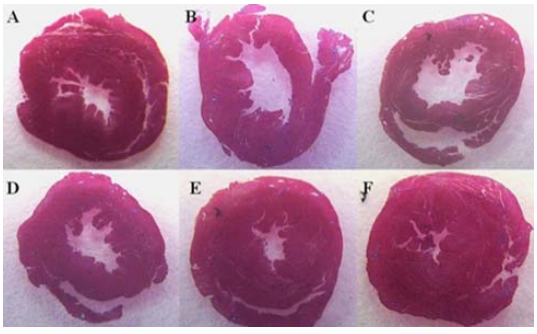
Congestive heart failure (CHF) is defined as abnormal heart function resulting in inadequate cardiac output for metabolic needs. It has been reported that 3-4 million adults in the United States have CHF. Once symptoms of heart failure are moderately severe, the prognosis is worse than most cancers in that 50% of such patients are dead within 4 years. The global market for CHF pharmaceuticals was valued at nearly $11.2 billion in 2010. Present treatments for CHF include pharmacological therapies, coronary revascularization procedures (e.g. coronary artery bypass surgery and angioplasty), and implantable cardiac defibrillators and biventricular pacemakers. However, even with optimal therapy, approximately half of the patients with severe CHF die within 4 years. Cardiac transplantation provides a better solution, but is available for only 1 patient per 1000 with CHF.

CHF affects people of all ages. It is very crucial to find new resource of cardiac muscle regeneration for CHF treatments.

Technology

1. **UTMD-GLP-1.** GLP-1 was successfully delivered through UTMD technology to rat heart cells with evidence that transfected cardiac cells had undergone proliferation. UTMD-GLP-1 gene therapy restored left ventricular (LV) mass, fractional shortening index, and LV posterior wall diameter.
2. **Undisclosed therapeutic agent.** The protein can be used as a stand alone therapeutic agent. The gene can be delivered using UTMD technology.

The technologies listed above have shown their function to stimulate myocardial regeneration and also reverse the cardiomyopathy itself in our in vivo rat model.



Treating and Reversing Adriamycin (ADM) Cardiomyopathy Using UTMD-GLP1 Gene Therapy

(A) Normal rat heart
(B) ADM injection only
(C) ADM injection + GLP1 peptide
(D) ADM injection + UTMD-GLP1 peptidein treatment
(E) ADM injection + UTMD-GLP1NLS gene therapy
(F) ADM injection + UTMD-GLP1NLS gene therapy 14 days later