A Gene Therapy Strategy To Restore Electrical And Cardiac Function In Arrhythmogenic Right Ventricular Cardiomyopathy

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Technology description

Researchers at UC San Diego have developed a novel treatment strategy for ARVC by rescuing the cardiac electrical and physiological dysfunction associated with arrhythmogenic disorders and thereby potentially prolonging life. This strategy is based upon the use of a novel mouse model of ARVC and using gene therapy as a therapeutic to improve cardiac rhythm and function as demonstrated in our mouse model of ARVC by improving electrical, contractile, and potential survival.

Arrhythmogenic right ventricular cardiomyopathy (ARVC) is a predominantly genetic-based heart disease characterized by right but also recently left ventricular dysfunction, fibrofatty replacement of the myocardium leading to fatal/severe ventricular arrhythmias leading to sudden cardiac death in young people and athletes. ARVC is responsible for 10% of sudden cardiac deaths in people ≥65 years of age and 24% in people ≤30 years of age. ARVC is thought to be a rare disease as it occurs in 1 in 1000-5000 people, although the prevalence may be higher as some patients are undiagnosed or misdiagnosed due to poor diagnostic markers. Growing evidence also reveals earlier onset since pediatric populations ranging from infants to children in their teens are also particularly vulnerable to ARVC, highlighting the critical need to identify and treat patients at an earlier stage of the disease.

At present there are no effective treatments for ARVC nor has there been any randomized clinical trials conducted to examine treatment modalities, screening regimens, or medications specific for ARVC. As a result, treatment strategies for ARVC patients are directed at symptomatic relief of electrophysiological defects, based on clinical expertise, results of retrospective registry-based studies, and the results of studies on model systems. The current standard of care is the use of anti-arrhythmic drugs (sotalol, amniodarone and beta-blockers) that transition into more invasive actions, which include implantable cardioverter defibrillators and cardiac catheter ablation, if the patient becomes unresponsive or intolerant to anti-arrhythmic therapies. However, current therapeutic modalities have limited effectiveness in managing the disease, 40% of ARVC patients (a young heart disease) die within 10-11 years after initial diagnosis, highlighting the need for development of more effective therapies for patients with ARVC.

Application area

Gene therapy as a therapeutic treatment for ARVC.

Advantages

The invention provides and advantage over the current standard of care.

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