A Gene Therapy for treating Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC)

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BACKGROUND

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.

TECHNOLOGY DESCRIPTION

Researchers at UC San Diego have developed a novel treatment strategy for ARVC. Using a mouse ARVC model which carries mutations in a specific gene, our researchers show that using an AAV (adenoviral vector) carrying the wild-type gene under the control of a cardiac-specific promoter the treated mice:

a) significantly improved ejection fractions, end-diastolic volumes, and end-systolic volumes;

b) improved electrical function (improved QRS duration and less premature ventricular contractions);

c) had significantly reduced cardiac fibrosis;

d) significantly improved survival;

e) even at a late disease stage had improved cardiac function, improved cell-cell junction proteins, and prevented mortality;

f) preserved the cell-cell junction and cardiac function several months post-treatment.

APPLICATIONS

Gene therapy as a therapeutic treatment for ARVC.

ADVANTAGES

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

STATE OF DEVELOPMENT

The current protocol has been developed in an ARVC mouse model.

INTELLECTUAL PROPERTY INFO

A provisional patent has been submitted and the technology is available for licensing.

RELATED MATERIALS

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OTHER INFORMATION

KEYWORDS

Gene therapy, ARVC, cardiomyopathies.

CATEGORIZED AS

- **Biotechnology**
  - Health
- **Medical**
  - Disease: Cardiovascular and Circulatory System
  - Gene Therapy
  - Therapeutics

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