A Phase 1, Dose Escalation Trial of RP-A601 in Subjects With PKP2 Variant-Mediated Arrhythmogenic Cardiomyopathy (PKP2-ACM)

NCT05885412

Status	RECRUITING
Phase	Phase 1
Sponsor	Rocket Pharmaceuticals Inc.
Enrollment	9 participants

Key Eligibility Criteria

Inclusion (7)

- Male or female e18 years at the time of signing the informed consent
- Capable and willing to provide signed informed consent
- Clinical diagnosis of ACM as defined by the 2010 revised Task Force Criteria (TFC)
- Documentation of a pathogenic or likely pathogenic truncating variant in PKP2
- History of Implantable Cardioverter-Defibrillator (ICD) implantation e6 months prior to enrollment

... and 2 more (see full listing online)

Exclusion (5)

- Anti-AAVrh.74 capsid neutralizing antibody titer of \>1:40
- Cardiomyopathy related to a genetic etiology other than PKP2 truncating variant
- Previous participation in a study of gene transfer or gene editing
- Severe Right ventricular (RV) dysfunction
- New York Heart Association (NYHA) Class IV heart failure.

Locations (3 total)

University of California, San Diego, La Jolla, California, United States
Duke University, Durham, North Carolina, United States
Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States