

A Study of SGT-501 Gene Therapy in Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)

NCT07148089

Status	RECRUITING
Phase	Phase 1
Sponsor	Solid Biosciences Inc.
Enrollment	18 participants

Key Eligibility Criteria

Inclusion (8)

- Type of Participant and Disease Characteristics:
- Clinical diagnosis of CPVT, based on documented history of polymorphic or bidirectional non-sustained ventricular tachycardia with exercise or ventricular ectopy in a pattern consistent with CPVT on EST.
- Central Screening laboratory determination of a RYR2 variant that is pathogenic or likely pathogenic for CPVT.
- Documented history of life-threatening ventricular arrhythmic event defined as: survived sudden cardiac arrest, sudden cardiac arrest with appropriate implantable cardioverter defibrillator (ICD) shock, arrhythmic syncope, or sustained ventricular tachycardia (30 seconds or more) with or without ICD shock.
- On stable dose (defined as no change in dose by more than 50% for at least 1 month prior to Screening) of standard-of-care therapy defined as a beta-blocker and/or flecainide.

... and 3 more (see full listing online)

Exclusion (8)

- Abnormal liver function: gamma-glutamyl transferase (GGT) $> 1.5 \times$ upper limit of normal [ULN] or total bilirubin $> \text{ULN}$.
- Abnormal renal function defined by estimated glomerular filtration rate < 60 milliliter /minute (mL/min)/1.73-square meter (m²) using Chronic Kidney Disease Epidemiology Collaboration (CKD-EPI) Formula.
- Clinically significant abnormalities of coagulation including international normalized ratio or activated partial thromboplastin time $> 1.2 \times \text{ULN}$ or platelets $< 150,000$ cells/cubic millimeter (mm³).
- Potential concomitant cardiomyopathy or inherited arrhythmia as evidenced by pathogenic or likely pathogenic mutation other than RYR2 obtained on cardiac panel during Screening.
- Current or prior treatment with an approved or investigational gene transfer drug.

... and 3 more (see full listing online)

Locations (5 total)

Boston Children's Hospital, Boston, Massachusetts, United States

Mayo Clinic, Rochester, Minnesota, United States

Cleveland Clinic, Cleveland, Ohio, United States

... and 2 more locations