

# A Phase 1 AAV Gene Therapy Trial Evaluating Safety and Preliminary Efficacy of RP-A701 in Subjects With BAG3 Dilated Cardiomyopathy

NCT07137338

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Status	RECRUITING
Phase	Phase 1
Sponsor	Rocket Pharmaceuticals Inc.
Enrollment	8 participants

## Key Eligibility Criteria

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### Inclusion (10)

- Subjects are eligible for inclusion into the study only if all the following criteria apply:
- Male or female between 18 and 65 years of age at the time of signing the informed consent
- Capable of and willing to provide signed informed consent
- Clinical diagnosis of DCM defined as and requiring each of the following:
- Mild to moderate systolic dysfunction (LVEF e 25% and d 45%) by echocardiography or CMR performed within 3 months of enrollment.
- ... and 5 more (see full listing online)

### Exclusion (7)

- CV disease that may be related to a genetic etiology other than a BAG3 pathogenic or likely pathogenic variant.
- Previous participation in a study of gene transfer or gene editing.
- I.V. inotropic, vasodilator, or diuretic therapy d 30 days prior to enrollment.
- History of intracardiac thrombosis or arterial thromboembolic events
- Severe RV dysfunction assessed by echocardiogram or CMR d 12 months prior to screening
- ... and 2 more (see full listing online)

## Locations (3 total)

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University of California, San Diego, San Diego, California, United States  
Mayo Clinic, Rochester, Minnesota, United States  
Medical University of South Carolina, Charleston, South Carolina, United States