

# A Gene Therapy Study of RP-A501 in Male Patients With Danon Disease

NCT06092034

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| Status     | RECRUITING                  |
| Phase      | Phase 2                     |
| Sponsor    | Rocket Pharmaceuticals Inc. |
| Enrollment | 14 participants             |

## Key Eligibility Criteria

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### Inclusion (9)

- Documentation of a pathogenic or likely pathogenic variant of the LAMP2 gene.
- Male.
- Age ≥8 years.
- Evidence of left ventricular hypertrophy with preserved systolic function phenotype as defined by each of the following:
- Abnormal thickening of Left ventricular wall,  
... and 4 more (see full listing online)

### Exclusion (4)

- Anti-AAV9 neutralizing antibody titer  $\geq$ 1:40.
- Severe heart failure or requirement for advanced therapies.
- History of intracardiac thrombosis or arterial thromboembolic events including stroke, transient ischemic attack (TIA), acute coronary syndrome, myocardial infarction or unstable angina.
- Prior cardiac or other organ (lung, liver, other) transplantation.

## Locations (6 total)

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University of California, San Diego, La Jolla, California, United States  
Boston Children's Hospital, Boston, Massachusetts, United States  
Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States  
... and 3 more locations