

# Danon Disease Natural History Study

NCT06214507

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<b>Status</b>	RECRUITING
<b>Sponsor</b>	Rocket Pharmaceuticals Inc.
<b>Enrollment</b>	60 participants

## Key Eligibility Criteria

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### Inclusion (14)

- Prospective Cohort:
  - Documentation of a pathogenic or likely pathogenic variant of the LAMP2 gene by a CLIA-certified genetic testing laboratory
  - Patient or parent/legal guardian are capable and willing to provide signed informed consent
  - Age e 8 years at enrollment
  - Female Prospective Cohort:
- ... and 9 more (see full listing online)

### Exclusion (8)

- All Cohorts:
  - Concurrent enrollment in any other clinical investigation involving use of an investigational agent for any condition at time of enrollment to this study that could confound interpretation of this study
  - Previous treatment with a gene therapy
  - Prospective Cohort:
  - Prior mechanical circulatory support at time of enrollment to this study
- ... and 3 more (see full listing online)

## Locations (13 total)

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Phoenix Children's Hospital, Phoenix, Arizona, United States  
University of California, San Diego, La Jolla, California, United States  
Children's Hospital Colorado, Aurora, Colorado, United States  
... and 10 more locations