

Eligibility Criteria

- Select inclusion criteria:
 - Adults (age 18–65 years)
 - Nonobstructive hypertrophic cardiomyopathy
 - Pathogenic/likely pathogenic truncating mutations in the MYBPC3 gene
 - Symptomatic (NYHA Functional Class II or III)
 - NT-proBNP ≥ 300 pg/ml
 - Functioning ICD
- Exclusion criteria:
 - Seropositive for AAV9-neutralizing antibody titer

MyPEAK-1 Phase 1b/2 clinical trial design



Study Objectives

- Safety, tolerability
- Dose-finding
- Pharmacodynamics

Design

- Open-label, multi-center, dose-escalation and dose-expansion
- 52-week trial period with four-year safety and efficacy follow-up
- Cardiac biopsies at baseline, post-dose and ~52 weeks (effective with Cohort 1, patient 3)

