

Phase IA and IB Study of AAVrh.10hFXN Gene Therapy for the Cardiomyopathy of Friedreich's Ataxia

NCT05302271

Status	RECRUITING
Phase	Phase 1
Sponsor	Weill Medical College of Cornell University
Enrollment	25 participants

Key Eligibility Criteria

Inclusion (25)

- Males and females, age 12 to 50
- Willing and able to provide informed consent
- Definitive diagnosis of FA, based on clinical phenotype and genotype (GAA expansion on both alleles)
- ≥ 600 GAA repeats in intron 1 in at least one allele
- FARS and SARA neurologic scores consistent with diagnosis of Friedreich's ataxia
- ... and 20 more (see full listing online)

Exclusion (23)

- Individuals receiving corticosteroids or other immunosuppressive medications
- Individuals with uncontrolled diabetes (glycated hemoglobin, HbA1c levels $\geq 7\%$)
- Genotype FA missense mutation on one or both alleles
- Evidence of infection defined by elevated white blood cell count, temperature ≥ 38.5 C, infiltrate on chest x-ray
- Decompensated heart failure (NY4A class III-IV at time of baseline clinical assessment)
- ... and 18 more (see full listing online)

Locations (1 total)

Weill Cornell Medicine, New York, New York, United States