A Study of PBFT02 in Participants With FTD and Mutations in the Granulin Precursor (GRN) or C9ORF72 Genes

NCT04747431

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Passage Bio, Inc.
Enrollment	30 participants

Key Eligibility Criteria

Inclusion (4)

- Documented to be a pathogenic carrier of GRN or C9orf72 mutation
- Clinical diagnosis of frontotemporal dementia
- Have a reliable informant / caregiver (and back-up informant / caregiver) who personally speaks with or sees the subject at least weekly
- Living in the community (i.e., not in a nursing home); assisted living may be permitted at the discretion of the investigator

Exclusion (43)

- Classification of the GRN mutation as "not pathogenic," "likely benign variant," "benign variant," or "pathogenic nature unclear" (FTD- GRN Cohorts 1-3) or C9orf72 HRE length d 30 (FTD-C9orf72 Cohorts 4-5).
- Previous treatment with any gene therapy. Any other therapies with the potential to alter PGRN levels must be washed out for at least 5 half-lives prior to entry into this study
- Homozygous GRN mutation carrier (FTD-GRN Cohorts 1-3) or homozygous C9orf72 mutation carrier (FTD-C9orf72 Cohorts 4-5).
- Rosen-modified Hachinski Ischemic Scale score \> 7
- Known presence of a structural brain lesion (eg, tumor, cortical infarct) that could reasonably explain symptoms in a symptomatic subject

... and 38 more (see full listing online)

Locations (10 total)

Michigan Alzheimer's Disease Center, Ann Arbor, Michigan, United States
University of Pennsylvania, Philadelphia, Pennsylvania, United States
Vanderbilt University Medical Center, Nashville, Tennessee, United States
... and 7 more locations