Safety and Efficacy Study of NGGT002 in cPKU Adult Subjects

NCT06687733

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	NGGT (Suzhou) Biotechnology Co., Ltd.
Enrollment	18 participants

Key Eligibility Criteria

Inclusion (7)

- Voluntarily participating in the study and signing the informed consent form;
- Gender is not limited; patients must carry biallelic pathogenic or likely pathogenic variants in the PAH gene;
- Adult patients aged 18 to 55 years;
- In the past 24 months, at least two blood Phe concentrations have been e600μmol/L (10 mg/dL), with at least one of these measurements taken within 6 months prior to the screening period;
- Willing and able to manage their diet;

... and 2 more (see full listing online)

Exclusion (25)

- Presence of anti-AAV8 neutralizing antibodies e1:5
- Subjects whose disease is well-controlled with existing therapies, such as those currently receiving medications like Sapropterin Dihydrochloride tablets, Pegvaliase-pqpz, etc.;
- Before dosing, the patient's hematological laboratory tests exceed any of the following limits:
- Alanine Transaminase (ALT) \> 1.5×ULN and/or Aspartate Aminotransferase (AST) \> 1.5×ULN
- Alkaline Phosphatase (ALP) \> 1.5×ULN

... and 20 more (see full listing online)

Locations (2 total)

First Affiliated Hospital of Bengbu Medical College, Bengbu, Anhui, China
Xinhua Hospital Affifiated to Shanghai Jiao Tong University School of Medicine, Shanghai, China