

Safety and Efficacy Study of NGGT002 in PKU Adult Subjects

NCT06061614

Status	RECRUITING
Phase	Early Phase 1
Sponsor	The First Affiliated Hospital of Bengbu Medical University
Enrollment	15 participants

Key Eligibility Criteria

Inclusion (8)

- Voluntarily sign informed consent form;
- Male and female subjects with diagnosis of PKU caused by confirmed phenylalanine hydroxylase(PAH) mutation according to the "Clinical Practice Guidelines for Phenylketonuria, 2020 Edition";
- Age \geq 18 years;
- Blood phenylalanine (Phe) concentration \leq 600 μ mol/L at least once within 2 years prior to screening, with one measurement confirmed within six months of enrollment;
- Subjects are able to maintain their baseline diet throughout the study (regardless of dietary phenylalanine restriction), and willingness to follow the instruction of investigators to manage the diet for the duration of the trial;

... and 3 more (see full listing online)

Exclusion (14)

- Anti-AAV8 neutralizing antibody $>$ 1:10
- Prior gene therapy
- Positive hepatitis B virus surface antigen, hepatitis C virus antibody, anti-human immunodeficiency virus antibody or treponema pallidum-specific antibody
- Hepatic function abnormal: alanine aminotransferase (ALT) or aspartate aminotransferase (AST) $>$ 1.5 \times ULN; alkaline phosphatase (ALP) $>$ 1.5 \times ULN; total bilirubin (TBil) $>$ 1.5 \times ULN; international normalized ratio (INR) $>$ 1.3
- Hematology values outside of the normal range (Hemoglobin $<$ 110 g/L (male), $<$ 100 g/L (female), white blood cells $<$ 3.0 \times 10⁹/L, neutrophils $<$ 1.5 \times 10⁹/L, platelet counts $<$ 100 \times 10⁹/L;

... and 9 more (see full listing online)

Locations (1 total)

The First Affiliated Hospital of Bengbu Medical College, Bengbu, Anhui, China

<https://clinicaltrials.gov/study/NCT06061614>

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