

AAV Gene Therapy Clinical Study in Adult Classic PKU (PHEdom)

NCT06332807

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	NGGT INC.
Enrollment	12 participants

Key Eligibility Criteria

Inclusion (16)

- Is willing and able to provide written, signed informed consent after the nature of the study has been explained and prior to any research-related procedures; a legally authorized representative may provide written consent and assent may be requested.
- Male and female subjects with diagnosis of classic PKU, a condition characterized by severe PAH deficiency with confirmed PAH mutations predicted with no residual enzyme activity. A list of PAH mutations for classic PKU based on in vitro PAH activity (Himmelreich et al., 2018) and the genotype-phenotype correlation (Garbade et al., 2019) can be found in BIOPKU genotypes database (<http://www.biopku.org/pah>).
- Adults aged 18-55 at the time of informed consent
- Subjects intolerant or unresponsive to available medical therapies, such as Kuvan, Playnzip, etc.
- Subjects who have been on medications, such as Kuvan, Palynziq, etc but have come off for medical reasons or the patient's decision at least 28 days prior to signing the consent form (Subjects who have good disease control on these existing therapies will not be included in this study).

... and 11 more (see full listing online)

Exclusion (27)

- Subjects with PKU that is not due to PAH mutation
- Presence of anti-AAV8 neutralizing antibodies
- Prior to dosing, subjects exceed the limit of any of the following liver function and hematology tests in two consecutive blood laboratory tests:
 - Alanine aminotransferase (ALT) $\gt 1.5 \times \text{ULN}$ and/or aspartate aminotransferase (AST) $\gt 1.5 \times \text{ULN}$
 - Alkaline phosphatase (ALP) $\gt 1.5 \times \text{ULN}$

... and 22 more (see full listing online)

Locations (5 total)

Children's Hospital of Orange County Hospital, Orange, California, United States

University of Minnesota, Minneapolis, Minnesota, United States

Atlantic Health System, Morristown, New Jersey, United States

... and 2 more locations

<https://clinicaltrials.gov/study/NCT06332807>

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