THE PHEDOM STUDY - A GENE THERAPY CLINICAL TRIAL OPPORTUNITY FOR PATIENTS DIAGNOSED WITH PHENYLKETONURIA (PKU)

PHEdom Study Details

This is an open label Phase I/II study for the safety and efficacy of NGGT002.

NGGT002 is a gene replacement therapy. It delivers a working PAH gene in your body via the adeno-associated virus serotype 8 (AAV8) delivery system, and this has the potential to reduce symptoms of your classic phenylketonuria. It is administrated by one dose intravenous infusion, using a small tube and a needle that deliver the medication directly into your vein.

NGGT002 is specifically formulated to target liver cells and facilitate the production of PAH, the crucial liver enzyme whose dysfunction causes PKU. NGGT002 can potentially restore PAH activity in regulating phenylalanine metabolism. If this proves effective, it has the potential to provide long-term relief for individuals who must undergo life-time diet management to control their abnormal high blood phenylalanine level, possibly even for their lifetime.

Participants must be diagnosed with classic PKU; between the age of 18-55 and not on any available medical therapies for PKU. Eligible patients will enter the study followed by a 52-week post infusion follow-up period, and an additional 4-year long-term follow-up for safety and efficacy assessments.

Please speak with your physician or study coordinator for additional details.

FOR MORE INFORMATION:

www.clinicaltrials.gov
ClinicalTrials.gov ID: NCT06332807

NGGT
NEXT GENERATION GENE THERAPEUTICS INC.