Halting Ornithine Transcarbamylase Deficiency With Recombinant AAV in ChildrEn

NCT05092685

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	University College, London
Enrollment	12 participants

Key Eligibility Criteria

Inclusion (9)

- Patient (male or female) aged d16 years at time of written informed consent. For the dose escalation phase patients must be aged 6-16, for the dose expansion phase patients must be aged 0-16 (at the time of written informed consent).
- OTC deficiency confirmed via enzymatic or molecular analysis. This may include identification of pathogenic mutations or liver OTC activity that is \<20% of normal activity.
- Patient has severe disease defined by reduced protein allowance and prescribed at least one ammonia scavenger drug.
- Patient (if capable of signing) and parents or legal representative have signed a written informed consent form.
- Females of childbearing potential must have a negative pregnancy test in serum or urine at the screening and Day 0 infusion visits, and use an adequate contraception method from the screening visit until 4 weeks after the first negative plasma sample monitoring vector genomes copies or the week 52 visit, whatever comes first.

... and 4 more (see full listing online)

Exclusion (12)

- Titres of the neutralising antibodies against AAV-LK03 \>1:5 serum dilution.
- Significant hepatic inflammation as evidenced by the following laboratory abnormalities: alanine aminotransferase or aspartate aminotransferase or bilirubin \>2 x upper limit of normal (ULN), alkaline phosphatase \>3 x ULN.
- Evidence of severe unexplained liver disease including but not limited to liver malignancy, liver cirrhosis, or acute liver failure.
- Evidence of active hepatitis B or C virus (HBV and HCV respectively) documented by hepatitis B surface antigen (HBsAg) or HCV RNA positivity.
- Positive PCR for human immunodeficiency virus (HIV).

... and 7 more (see full listing online)

Locations (1 total)

Great Ormond Street Hospital, London, United Kingdom