A Clinical Study of Glycerol Phenylbutyrate in Chinese Patients With Urea Cycle Disorders

NCT06904027

Status	RECRUITING
Sponsor	Tongji Hospital
Enrollment	40 participants

Key Eligibility Criteria

Inclusion (5)

- Male or female aged 0-18 years;
- Subject and/or subject's legally authorized representative willing to follow the therapeutic regimen, dietary management and visit plan of the study, and voluntarily signing informed consent form;
- Patients with the following subtypes of UCD: Carbamoyl phosphate synthetase I deficiency, Ornithine translocase deficiency, citrullinemia type I, argininosuccinic aciduria, argininemia, and hyperornithinemia-hyperammonemia-homocitrullinuria (HHH) syndrome;
- Patients planned to use glycerol phenylbutyrate who have not used it in past 3 months (including at the time of 3 months);
- Men with fertility and women of childbearing potential (with menstruation) who are willing to take effective contraceptive measures during the period from the date of signing the informed consent to 1 months after the last dose of the study drug, such as abstinence, condoms, intra-uterine contraceptive devices, and double barrier methods (such as condoms + contraceptive diaphragms). Pregnancy test results must be negative for women of childbearing age within d 7 days before the initial administration of study drug.

Exclusion (5)

- Hypersensitivity to any of the active ingredient, including phenylbutyrate (PBA), phenylacetate acid (PAA) and phenylacetyl glutamine (PAGN), or excipients;
- Use of any drug known to significantly affect renal clearance (such as probenecid) or increase protein catabolism (such as corticosteroids) or other drugs known to increase blood ammonia levels (such as valproate) within 24 h before the first administration;
- Use of other nitrogen-scavenging agent at the same time after enrollment, such as sodium phenylbutyrate and sodium benzoate;
- Pregnant or breastfeeding females.
- Other reasons, in the opinion of the investigator, that may affect the patient's compliance and safety in participating in the study.

Locations (5 total)

Peking University First Hospital, Beijing, Beijing Municipality, China
Guangzhou Women and Children's Medical Center, Guangzhou, Guangdong, China
Tongji Hosipital, Wuhan, Hubei, China
... and 2 more locations