

An Observational Study of Carbaglu® for the Treatment of MMA and PA in Adults and Pediatrics

NCT05040178

Status RECRUITING
Sponsor RECORDATI GROUP
Enrollment 20 participants

Key Eligibility Criteria

Inclusion (7)

- Provision of signed and dated informed consent/assent form
 - Prescribed and treated with Carbaglu®
 - Have an established diagnosis of PA or MMA defined as follows:
 - Diagnosed with PA by semi quantitative urine organic acid analysis, defined as presence of elevated methylcitric acid and normal methylmalonic acid levels and no evidence of biotin related disorders in the organic acid analysis; OR
 - Diagnosed with MMA by semi quantitative urine organic acid analysis, defined as elevation of methylmalonic acid and no evidence of vitamin B12 dependent disorder on plasma amino acid analysis (vitamin B12 dependency is defined by documented vitamin B12 responsiveness).
- ... and 2 more (see full listing online)

Exclusion (1)

- None

Locations (5 total)

Children's National Hospital, Washington D.C., District of Columbia, United States
University of South Florida, Tampa, Florida, United States
Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, Illinois, United States
... and 2 more locations