Assessment of the Efficacy and Safety of Alpelisib (BYL719) in Pediatric and Adult Patients With Megalencephaly-CApillary Malformation Polymicrogyria Syndrome (MCAP)

NCT05577754

Status	RECRUITING
Phase	Phase 2
Sponsor	Centre Hospitalier Universitaire Dijon
Enrollment	20 participants

Key Eligibility Criteria

Inclusion (21)

- Signed informed consent and assent (when applicable) from the patient, parent, or guardian must be obtained prior to any study related screening procedures are performed.
- Male or female patients age e2 years and d40 years at the time of informed consent
- Patients with diagnosis of MCAP* with neurodevelopmental disorder presentation (from specific learning disorder to severe intellectual disability)
- Documented evidence of a postzygotic or constitutional mutation(s) in the PIK3CA gene performed in local laboratories using a Deoxyribonucleic acid (DNA) based validated test at the time of informed consent.
- Adequate bone marrow and organ function (assessed during the screening visit):

... and 16 more (see full listing online)

Exclusion (34)

- Participants meeting any of the following criteria are not eligible for inclusion in this study:
- Patient previously treated with alpelisib
- Known impairment of GI function due to concomitant disease that may significantly alter the absorption of the study drug (e.g., ulcerative diseases, uncontrolled nausea, vomiting, diarrhea, malabsorption syndrome, or small bowel resection) at time of informed consent.
- Participant with uncontrolled diabetes mellitus (Type I or II) at time of informed consent.
- History of hypersensitivity to any drugs or metabolites of PI3K inhibitor or any of the excipients of alpelisib at time of informed consent.

... and 29 more (see full listing online)

Locations (13 total)

CHU Amiens, Amiens, France
CHU d'Angers, Angers, France
CHRU Brest, Brest, France
... and 10 more locations