EPIK-P4: A Phase II Single-arm Study to Assess the Efficacy, Safety and Pharmacokinetics of Alpelisib (BYL719) in Pediatric and Adult Patients With PIK3CA-related Overgrowth Spectrum (PROS)

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 2 years to 100 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Key Inclusion Criteria:

1. Male or female participants aged ≥2 years at the time of informed consent/assent.
2. Participants with diagnosis of PROS (according to Clinical Diagnostic Criteria for PROS proposed by Keppler Noreuil et al 2014) with symptomatic AND progressive overgrowth, who have syndromic disease or isolated features (with the exception of isolated macrodactyly, macrocephaly or epidermal nevus) at the time of informed consent/assent.
3. Documented evidence of a somatic mutation(s) in the PIK3CA gene performed in local laboratories using a DNA-based test AND available archival tissue (if archival tissue sample is not available, a fresh biopsy should be performed, if it is not clinically contraindicated) at the time of informed consent/assent.
4. Karnofsky (in participants >16 years of age at study entry) or Lansky (≤16 years of age at study entry) performance status index ≥50.
5. PGI-S score of mild, moderate, severe, or very severe at screening.
6. Adequate bone marrow and organ function.
7. Presence of at least 1 PROS-related measurable lesion (longest diameter ≥2 cm) confirmed by BIRC assessment and associated with complaints, clinical symptoms or functional limitations affecting the participant's everyday life.

Key Exclusion Criteria:

1. Participant with only isolated macrodactyly, epidermal nevus/nevi and macroencephaly (the only clinical feature or a combination of any of three of them), in absence of other PROS-related lesions at the time of informed consent/assent.
2. Previous treatment with alpelisib and/or any other phosphatidylinositol 3-kinase (PI3K) inhibitor(s) (except treatment attempt, defined as the attempt to treat PROS with any of PI3K inhibitors, with treatment duration less than 2 weeks and stopped at least 2 weeks prior to the first dose of study medication with alpelisib).
3. Debulking or other major surgery performed within 3 months at the time of informed consent/assent.
4. Radiation exposure for PROS treatment purpose within 12 months prior to informed consent/assent.
5. Clinically meaningful PROS-related thrombotic event (Grade 2 and more as per CTCAE v4.03) within 15 days before study treatment initiation, and/or sclerotherapy/embolization for vascular complications performed within 6 weeks before informed consent/assent.
6. Clinically meaningful bleeding from PROS-related lesion (Grade 2 and more as per CTCAE v4.03) within 15 days before study treatment initiation.
7. Participants with clinically significant worsening of PROS-related laboratory abnormalities, physical signs and symptoms (such as, but not limited to increase of D-dimers, worsening of underlying pain, newly occurring swelling or redness) indicating an uncontrolled condition during the screening phase.

Other inclusion/exclusion criteria may apply

Conditions & Interventions

Interventions:
DRUG: Alpelisib
Conditions:
PIK3CA-related Overgrowth Spectrum (PROS)
Keywords:
PIK3CA-related overgrowth spectrum (PROS), Alpelisib (BYL719), Phase II, Adult, Pediatric, Adolescent

More Information

Description:
This study is designed to demonstrate the efficacy and assess safety and tolerability of oral daily alpelisib in participants with PIK3CA-related overgrowth spectrum (PROS).

Contact(s): - sarah.price@cchmc.org
Phase: PHASE2
System ID: NCT06997588

Thank you for choosing StudyFinder. Please visit http://studyfinder.cchmc.org to find a Study which is right for you and contact clinicalstudies@cchmc.org if you have questions or need assistance.