Alpelisib in Pediatric and Adult Patients With Lymphatic Malformations Associated With a PIK3CA Mutation.

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 0 years to 100 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Key inclusion criteria:

1. Signed informed consent and assent (when applicable) from the participant, parent, legal authorized representative or guardian.
2. Participant must be willing to remain at the clinical site as required by the protocol and be willing to adhere to study restrictions and examination schedules.
3. Participant has a physician confirmed and documented diagnosis of a symptomatic LyM at the time of informed consent (Note: the physician must confirm that the LyM cannot be included under the PROS diagnostic criteria).
4. Participant is not considered as a candidate for or is not willing to receive non-drug therapies including but not limited to sclerotherapy, embolization, and surgery until the completion of Week 24 in Stage 1 and 2.
5. Participant has evidence of a somatic mutation(s) in the PIK3CA gene prior to randomization.
6. Participant has at least one measurable LyM lesion confirmed by BIRC assessment prior to randomization.
7. Participants must be able to ingest study drug (either in tablet form or as a drinkable suspension [Groups 1 to 4] or granules or as an oral suspension [Group 5]) as assessed within 7 days before study treatment start. Drug administration via feeding tubes is allowed.

Key exclusion criteria:

1. Participant has a physician-confirmed and documented diagnosis of PROS at the time of informed consent.
2. Participant has a physician-confirmed and documented diagnosis of a Central Conducting Lymphatic Anomaly, General Lymphatic Anomaly, Gorham-Stout disease, Kaposiform lymphangiomatosis at the time of informed consent.
3. Participant has a known history of Stevens-Johnson syndrome, erythema multiforme, or toxic epidermal necrolysis at the time of informed consent.
4. Participant has an established diagnosis of type I diabetes mellitus or uncontrolled type II diabetes mellitus at the time of informed consent.
5. Participant had previous treatment with alpelisib and/or any other PI3K inhibitors with treatment duration longer than 2 weeks at the time of informed consent.

Other inclusion/exclusion criteria may apply

Conditions & Interventions

Interventions:
DRUG: Alpelisib, DRUG: Placebo
Conditions:
Lymphatic Malformations
Keywords:
Lymphatic malformations (LyM), Cystic lymphangioma, Cystic hygroma, Lymphangioma circumscriptum, Cavernous lymphangioma, Lymphangioma, Macrocystic lymphatic malformation, Microcystic lymphatic malformationn, Alpelisib

More Information

Description:
The main purpose of this study in participants with PIK3CA-mutated LyM is to assess the change in radiological response and symptom severity upon treatment with alpelisib film-coated tablets (FCT) as compared to placebo.

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System ID: NCT05948943

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