MAP to provide alpelisib (BYL719) for patients with PROS

Status: OPEN TO ACCRUAL

Eligibility Criteria

Age: 2 years to 99 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Patients eligible for inclusion in this Treatment Plan have to meet all of the following criteria: 1. Adult or pediatric patients ? 2 years of age, with a diagnosis of PROS preferably with evidence of a mutation in the PIK3CA gene 2. The treating physician has determined that the patient's condition is severe or life threatening, treatment is necessary and there are no other feasible alternatives for the patient. 3. Confirmed adequate bone marrow function Written patient informed consent must be obtained prior to start of treatment Exclusion criteria Patients eligible for this Treatment Plan must not meet any of the following criteria: 1. Patient has history of hypersensitivity to any drugs or metabolites of PI3K inhibitor or any of the excipients of alpelisib. 2. Patient with uncontrolled diabetes mellitus type I or not controlled type II (based on FPG and HbA1c, see inclusion criterion 2) 3. Patient who has other concurrent severe and/or uncontrolled medical conditions that would, in the Treating Physician's judgment, contraindicate administration of alpelisib (eg. active or uncontrolled severe infection, chronic active hepatitis, immuno-compromised, acute or chronic pancreatitis, uncontrolled high blood pressure, interstitial lung disease, etc.) 4. Patient has a known history of Severe Cutaneous Adverse Reactions (SCAR) like Steven Johnson's syndrome (SJS), Erythema Multiforme (EM), Toxic Epidermal Necrolysis (TEN), or Drug Reaction with Eosinophilia and Systemic Symptoms (DRESS). 5. History of pancreatitis within 1 year of screening or past medical history of chronic pancreatitis 6. Subject with Child Pugh score B or C 7. Subjects with unresolved osteonecrosis of the jaw 8. Subject is currently receiving any of the following medications and cannot be discontinued 7 days prior to the start of the treatment:

• Strong inducers of CYP3A4

• Inhibitors of BCRP 9. Patient has a known history of Human Immunodeficiency Virus (HIV) infection (testing not mandatory unless required by local regulations or requirements). 10. Patient who is concurrently being treated with drugs known to be strong inhibitors or inducers of the isoenzyme CYP3A; switching to different medications prior to start of program treatment is allowed within the last 5 days prior to starting program treatment 11. Patient is currently receiving or has received systemic corticosteroids ? 2 weeks prior to start of program treatment, or who have not fully recovered from side effects of such treatment. Note: The following uses of corticosteroids are permitted: single doses, topical applications (e.g., for rash), inhaled sprays (e.g., for obstructive airways diseases), eye drops or local injections (e.g., intra-articular). 12. Male patient who does not apply highly effective contraception during the treatment with alpelisib and through the duration as defined below after the final dose of alpelisib. Sexually active males should use a condom during intercourse while taking drug and for at least 4 weeks after stopping alpelisib and should not father a child in this period. A condom is required to be used also by vasectomized men in order to prevent delivery of the drug via seminal fluid 13. Subject is not able to understand and to comply with treatment instructions and requirements 14. Subject is a nursing (lactating) or pregnant woman as confirmed by a positive serum (hCG) test prior to initiating study treatment 15. Subject is a woman of child-bearing potential defined as all women physiologically capable of becoming pregnant, unless they are using highly effective methods of contraception during study treatment and at least for 1 week after the last dose of any study treatment. Highly effective contraception methods include:

• Total abstinence (when this is in line with the preferred and usual lifestyle of the subject). Periodic abstinence (e.g., calendar, ovulation, symptom-thermal, post-ovulation methods) and withdrawal are not acceptable methods of contraception. Female sterilization (have had surgical bilateral oophorectomy with or without hysterectomy), total hysterectomy or bilateral tubal ligation at least 6 weeks before taking study treatment. In case of oophorectomy alone, only when the reproductive status of the woman has been confirmed by follow up hormone level assessment

• Male sterilization (at least 6 months prior to screening). For female subjects on the study the vasectomized male partner should be the sole partner for that subject

• Use of oral (estrogen and progesterone), injected or implanted combined hormonal method of contraception or placement of an intrauterine device (IUD) or intrauterine system (IUS), or forms of hormonal contraception that have comparable efficacy (failure rate <1%), for example hormonal vaginal ring or transdermal hormone contraception. In case of use or oral contraception, women should have been stable on the same pill for a minimum of 3 months before taking study treatment. Note: Women are considered postmenopausal and not of child bearing potential if they have had 12 months of natural (spontaneous) amenorrhea with an appropriate clinical profile (i.e., age appropriate, history of vasomotor symptoms) or have had surgical bilateral oophorectomy (with or without hysterectomy), total hysterectomy, or bilateral tubal ligation at least 6 weeks before taking study treatment. In the case of oophorectomy alone, only when the reproductive status of the woman has been confirmed by follow up hormone level assessment is she considered not of child bearing potential. 16. Subject is a sexually active male unwilling to use a condom during intercourse while taking study treatment, and for 1 week after stopping alpelisib. A condom is required for all sexually active male participants to prevent them from fathering a child AND to prevent delivery of study treatment via seminal fluid to their partner. In addition, male participants must not donate sperm during study and up to the time period specified above.

Conditions & Interventions

Interventions:

Drug: alpelisib, drug: Byl719, Modality: No vcuhs billing

Conditions:

PIK3CA-Related Overgrowth Spectrum (PROS), Congenital Anomalies (740-759)

Keywords:

adult, pediatric, BYL719, PIK3CA, Overgrowth Spectrum, PROS, alpelisib

More Information

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Phase: N/A

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