Checkpoint Inhibition In Pediatric Hepatocellular Carcinoma

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 0 years to 30 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Age: Patients must be <30 years of age at the time of study enrollment.
- Diagnosis: Patients must have relapsed/refractory, histologically confirmed HCC to be eligible for enrollment. Patients with hepatocellular neoplasm not otherwise specified (HCN NOS) will also be eligible.
- Disease Status: Participants must have measurable disease by RECIST criteria, defined as at least one lesion that can be accurately measured in at least one dimension (longest diameter to be recorded for non-nodal lesions and short axis for nodal lesions) measured at ≥20 mm with conventional technique or ≥10 mm with spiral CT scan, MRI, or calipers by clinical exam. See Section 10 for the evaluation of measurable disease.
- Performance Level: Karnofsky performance status ≥ 60% for patients ≥ 16 years of age or Lansky ≥ 60% for patients < 16 years of age.
- Prior Therapy: Patients must have fully recovered from the acute toxic effects of all prior anti-cancer therapy.
- Patients must not have received standard or targeted treatment regimens within 14 days of initiation of treatment with pembrolizumab.
- Patients must not have received prior radiotherapy within 7 days of initiation of treatment with pembrolizumab. Patients who have experienced radiation-induced adverse events must recover to a grade 1 prior to enrollment.
- Organ Function Requirements: Participants must have normal organ and marrow function as defined below:
    - Adequate Bone Marrow Function defined as:
    - Peripheral absolute neutrophil count (ANC) ≥ 750/µL
    - Platelet count ≥ 75,000/µL (can be transfused)
    - Adequate Liver Function defined as:
    - Total bilirubin < 1.5 x institutional upper limit of normal (ULN)
    - AST(SGOT) ≤ 2.5 x ULN
    - ALT(SGPT) ≤ 2.5 x ULN
    - If liver function studies are more elevated than the thresholds above, and if if this elevation is felt secondary to tumor, patients may still be eligible for enrollment after discussion with the study PI.
    - Adequate Renal and Metabolic Function defined as:
    - A serum creatinine based on age/gender as follows:
    - Age Maximum Serum Creatinine (mg/dL) Male Female
    - 1 to <2 years 0.6 0.6
    - 2 to <6 years 0.8 0.8
    - 6 to <10 years 1.0 1.0
    - 10 to <13 years 1.2 1.2
    - 13 to <16 years 1.5 1.4
    - >16 years 1.7 1.4
    - OR
    - Creatinine clearance ≥60 mL/min/1.73 m2 for participants with creatinine levels above institutional normal.
    - Amylase ≤ 1.5 x ULN
    - Lipase ≤ 1.5 x ULN
- If liver function studies are more elevated than the thresholds above, and if this elevation is felt secondary to tumor, patients may still be eligible for enrollment after discussion with the study PI.

-- Adequate Thyroid Function defined as:

--- TSH ≤1.5 ULN. Patients can be receiving thyroid supplementation.
* Confirmation of Insurance Pre-authorization approval for Pembrolizumab.
* Patients, their parent, and/or legally authorized representative must be able to understand and be willing to sign a written informed consent document. Assent for participants < 18 years will follow institutional guidelines. The protocol will require approval by each institution's Institutional Review Board.
* The effects of pembrolizumab on the developing human fetus are unknown. For this reason, patients of child-bearing and child-fathering potential must agree to use adequate contraception (hormonal or barrier method of birth control; abstinence) prior to study entry, for the duration of study participation, and for 4 months after completion of pembrolizumab administration. Should a female become pregnant or suspect she is pregnant while she or her partner is participating in this study, she should inform her treating physician immediately. Males treated or enrolled on this protocol must also agree to use adequate contraception prior to the study, for the duration of study participation, and 4 months after completion of pembrolizumab administration.
* Women of child bearing potential (WOCBP) must have a negative serum or urine pregnancy test within 24 hours of each treatment.
* A tumor sample must be available for submission to the central laboratory (Dana-Farber Cancer Institute, see Section 9). If surgery was performed at the time of recurrence, this sample, in addition to a diagnostic sample should be submitted. If no re-operation was performed, archived tissue from diagnosis or the most recent procedure should be submitted (see section 9 for further details regarding tissue specifications).

Exclusion Criteria:

- Participants who are receiving any other investigational agents are not eligible.
- Participants who have received checkpoint inhibitors (PD-1, PD-L1, and CTLA-4 inhibitors) are not eligible.
- Participants who have received antibody-based therapies are not eligible if they are within 3 half-lives of receipt of the last antibody dose.
- Participants who are receiving chronic steroids are not eligible.Chronic steroids are defined as either > or = 2mg/kg/day of body weight or > or = 20mg/day of prednisone or equivalent for persons who weigh > or = 10kg administered for > or = 14 consecutive days.
- Participants who are receiving anti-inflammatory or immunosuppressive medications are not eligible.
- Participants with known autoimmune disease, with the exceptions of childhood asthma or atopic dermatitis, are not eligible.
- Patients with a history of a positive test for human immunodeficiency virus or acquired immunodeficiency syndrome are not eligible.
- Patients with a history of allergic reactions attributed to compounds of similar chemical or biologic composition to pembrolizumab are not eligible. History of severe allergy to monoclonal antibody therapies (i.e. anaphylaxis) are likewise an exclusion.
- Patients with uncontrolled intercurrent illness including, but not limited to, ongoing or active infection, symptomatic congestive heart failure, or psychiatric illness/social situations that would limit compliance with study requirements are not eligible.
- Patients with prior solid organ transplantation are not eligible.

Conditions & Interventions

Interventions:
DRUG: Pembrolizumab
Conditions:
Hepatocellular Carcinoma, Childhood, Fibrolamellar Carcinoma, Liver Cancer, Liver Cancer, Pediatric
Keywords:
Hepatocellular Carcinoma Childhood, Fibrolamellar Carcinoma, Liver Cancer, Liver Cancer Pediatric

More Information

Description:
This research study is studying an immunotherapy drug (pembrolizumab or KEYTRUDA) as a possible treatment for pediatric hepatocellular carcinoma or hepatocellular neoplasm not otherwise specified (HCN NOS).

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IRB Number:
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