Protocol Title: A Phase 1 Study to Assess the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Preliminary Efficacy of BLU-554 in Patients with Hepatocellular Carcinoma

Target Population: Hepatocellular Carcinoma (HCC)

Summary: This is a Phase 1, open-label, first-in-human (FIH) study designed to evaluate the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD), and preliminary antineoplastic activity of BLU-554 administered orally in patients with FGF19 IHC+ Hepatocellular Carcinoma (HCC). The study consists of 3 parts, a dose-escalation part (Part 1), an expansion part (Part 2) exploring a once daily (qd) dosing schedule at the recommended Phase 2 dose (RP2D), and a Part 3 expansion of the qd dosing schedule at the RP2D in TKI naive patients.

Key Inclusion Criteria:
- Confirmed diagnosis of HCC by histological examination or by non-invasive criteria according to European Association for the Study of the Liver (EASL) or American Association for the Study of Liver Disease (AASLD) Guidelines (Part 1, 2 and 3)
- For Part 1 and 2, the patient has unresectable disease and has been previously treated with Sorafenib, has declined treatment with Sorafenib, or does not have access to Sorafenib
- For Part 3, the patient has not received prior treatment with a TKI
- Child-Pugh Class A with no clinically apparent ascites
- ECOG Performance Status 0 - 1
- For Part 1, willing to provide archived tumor tissue (if available) and willing to undergo pre- and on-treatment tumor biopsy (if considered safe and medically feasible by the treating investigator)
- For Part 2 and 3, all patients must have an FGF19 IHC result available
  - Only FGF19 IHC+ HCC patients will be eligible for Part 3

Key Exclusion Criteria:
- Central Nervous System metastases
- Platelet Count <75,000/mL
- Absolute Neutrophil Count <1000/mL
- Hemoglobin <8 g/dL
- Aspartate Aminotransferase (AST) or Alanine Aminotransferase (ALT) >5x the upper limit of normal (ULN)
- Total Bilirubin >2.5 mg/dL
- International Normalized Ratio (INR) >2.3 or Prothrombin Time (PT) >6 seconds above control
- Estimated (Cockcroft-Gault formula) or measured Creatinine Clearance <40 mL/min

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For additional information: https://clinicaltrials.gov/ct2/show/NCT02508467

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