**Hypothesis and objectives**

- A Phase I study was initiated to evaluate BLU-554, a potent, highly selective FGFR4 inhibitor designed for hepatocellular carcinoma (HCC) with FGFR4 pathway activation.

- **Key objectives** were to determine the maximum tolerated dose (MTD) and preliminary anti-tumor activity of BLU-554, an investigational, potent, highly-selective, orally available FGFR4 inhibitor.

- BLU-554 has high selectivity and affinity for FGFR4, with the potential to inhibit tumor growth and progression in patients with advanced HCC.

**HBV, hepatitis B virus; HCV, hepatitis C virus; NASH, non-alcoholic steatohepatitis**

**Methods**

First-in-human study of BLU-554, a potent, highly selective FGFR4 inhibitor designed for hepatocellular carcinoma (HCC) with FGFR4 pathway activation.

- **Part 1 dose escalation**

  - **Dose levels**:
    - BLU-554 100 mg/kg
    - BLU-554 10 mg/kg
    - BLU-554 30 mg/kg
    - BLU-554 10 mg/kg

  - **Dose expansion now enrolling**

**Key inclusion criteria**

- Tumor volume (mm³) ≤ 15 due to disease progression
- 2 due to AEs
- 1 due to investigator's decision

**Key exclusion criteria**

- Dose-limiting toxicities (DLTs)
- Additional accrual to dose levels

**Pharmacokinetics and pharmacodynamics**

**Anti-tumor activity and duration of study**

**Conclusions**

- Proof-of-concept established for highly selective targeting of FGFR4 with BLU-554 in advanced HCC.

- Proof-of-concept is shown for a FGFR4 pathway inhibitor in patients with advanced HCC.

- A Phase I study was initiated to evaluate BLU-554, a potent, highly selective FGFR4 inhibitor designed for hepatocellular carcinoma (HCC) with FGFR4 pathway activation.

- The objectives were to determine the maximum tolerated dose (MTD) and to evaluate the safety, pharmacokinetics (PK), pharmacodynamics (PD), and preliminary anti-tumor activity of BLU-554, an investigational, potent, highly-selective, orally available FGFR4 inhibitor.

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