### RX-3117 Proposed Mechanism

**Phase 1b/2a Pancractic Subjects Demographics and Safety Profile**

**Stage 1 of Phase 1b/2a: Demographics**

- **Total Number of study**: 13
- **Gender**: 1 Male, 12 Female
- **Median age (range)**: 69 (49-79)
- **Race**: White, 10; Black, 3
- **ECOG performance status**: 0 (0%), 1 (15.4%), 2 (84.6%)
- **Number of prior antitumor treatments**: 1 (33.3%), 2 (66.7%)

**Stage 1 of Phase 1b/2a: Safety**

- **Adverse Events**:
  - **ALT elevated**: 1 (33.3%), 2 (66.7%)
  - **AST increased**: 1 (33.3%), 2 (66.7%)
  - **Decreased appetite**: 1 (33.3%), 2 (66.7%)
  - **Fatigue**: 2 (66.7%)
  - **GI disorder**: 1 (33.3%), 2 (66.7%)
  - **Hypophosphatemia**: 1 (33.3%), 2 (66.7%)
  - **Leukopenia**: 2 (66.7%) 3 (100%)
  - **Neutropenia**: 1 (33.3%), 2 (66.7%)
  - **Thrombocytopenia**: 1 (33.3%), 2 (66.7%)

**Conclusion**

- RX-3117 is safe and well tolerated at the doses and schedules tested. The recommended Phase 2 dose is 700 mg administered for 5 consecutive days with 2 days off for 3 weeks with 1 week off. Subjects enrolled into stage 1 of the clinical trial had actively progressing disease, with 88% of them having failed ≥4 prior cancer therapies (including 5-FU and gemcitabine-based therapies).
- RX-3117 was shown to be safe and tolerable.
- Two subjects met the predefined protocol efficacy criteria by having progression free survival for more than 6 months.
- Forty additional subjects are now being recruited in stage 2 of the Phase 1b/2a.
- Future clinical studies include combination of RX-3117 with other agents for the treatment of pancreatic and bladder cancer.

**Author Disclosures**

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