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A Pilot Phase 2 Study of Albumin-Bound Rapamycin Nanoparticles, ABI-009, in Patients with Metastatic, Unresectable, Low or Intermediate Grade Neuroendocrine Tumors (NETs) of the Lung or Gastroenteropancreatic System

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BACKGROUND: Low and intermediate grade NETs lung or gastroenteropancreatic system are heterogeneous malignancies with few treatment options beyond surgical resection. Studies with the mammalian target of rapamycin (mTOR) inhibitor everolimus (RADIANT 1-4 trials) demonstrated its safety and efficacy; however, progression-free survival is generally less than 12 months. Preclinical data demonstrate improved tumor growth inhibition and survival with the mTOR inhibitor ABI-009 vs everolimus (dose for dose), indicating that ABI-009 may result in disease control even after everolimus failure. The goal of this phase 2 pilot study is to evaluate the utility of ABI-009 in NETs to warrant a full phase 2 clinical study.

METHODS: This study is a prospective, single arm, single institution pilot phase 2 study to evaluate the efficacy and safety of ABI-009 in patients with gastroenteropancreatic NETs (GEPNETs) or NETs of the lung and prior exposure to everolimus. ABI-009 will be administered intravenously at 100 mg/m^2 on days 1 and 8 of a 21-day cycle. Patients will be treated until disease progression.

Tumor response will be assessed by CT at baseline then every 9 weeks for 1 year, then every 12 weeks thereafter until progression. The primary endpoint is disease control rate at 6 months measured by RECIST 1.1.

The study will enroll 10 patients with ECOG performance status of 0 or 1. The key eligibility criteria is measurable unresectable or metastatic disease with typical or atypical carcinoid tumors of the lung or low or intermediate grade GEPNETs. Patients must have progressed or have been intolerant to everolimus.

RESULTS: This study is now active and open for enrollment. The anticipated enrollment period is 12 months.

CONCLUSION: This pilot phase 2 study may show evidence of promising safety and efficacy of ABI-009 to warrant a full phase 2 study in patients who progressed on or failed everolimus treatment.