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ElevatION:NET-201 An Open-Label Phase II Study to Evaluate the Efficacy and Safety of PDR001 in Patients With Advanced or Metastatic, Well-Differentiated, Non-Functional NET of Pancreatic, Gastrointestinal (GI), or Thoracic Origin or Poorly-Differentiated

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BACKGROUND: Monoclonal antibody (mAb) inhibitors of immune checkpoints, including anti-PD-1 and anti-PD-L1, have become established treatment options in various solid tumors. However, there is a paucity of data on checkpoint inhibitors in NET. In a phase I trial of PDR001 (mAb checkpoint inhibitor targeting PD-1) conducted in patients with multiple solid tumor types, a patient with histologically confirmed metastatic atypical pulmonary carcinoid demonstrated a RECIST-based tumor response and clinical benefit.

This study will evaluate the antitumor activity, safety, and tolerability of single-agent PDR001 in patients with progressive, non-functional well-differentiated NET and poorly-differentiated GEP-NEC.

METHODS: Patients with non-functional unresectable advanced well-differentiated grade 1/2 NET of GI, pancreatic or thoracic origin and poorly-

differentiated GEP-NEC who have progressed on or after prior available treatment will be included. Overall response rate (by RECIST 1.1.and Blinded Independent Review Committee) in the well-differentiated NET and poorly-differentiated GEP-NEC groups is the primary outcome and duration of response in each group is the key secondary outcome.

RESULTS: Ninety patients will be treated by grouping in 3 cohorts of approximately 30 patients each, as per the site of primary NET: GI, pancreatic, or thoracic (including lung and thymic origin) and approximately 20 patients will be treated in the poorly differentiated GEP-NEC group, for a total of approximately 110 patients. Patients will receive PDR001 (400 mg, once every 4 weeks) via 30 minutes IV infusion until disease progression or unacceptable toxicity.

CONCLUSION: This phase II, open-label, multicenter study is currently enrolling patients in United States, Europe, Canada, Australia, Israel, and Japan (NCT02955069) to investigate the role of immunotherapy in NET after prior treatment.