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G-003 - OPEN-LABEL STUDY OF PATISIRAN IN PATIENTS WITH HEREDITARY TRANSTHYRETIN-MEDIATED AMYLOIDOSIS POST-ORTHOTOPIC LIVER TRANSPLANT

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Resumen

Objectives: Hereditary transthyretin-mediated (hATTR) amyloidosis is a progressive, life-threatening disease. Orthotopic liver transplant (OLT) suppresses mutant transthyretin production to slow progression in early disease. Patisiran suppresses production of mutant and wild-type transthyretin and has been shown to halt/reverse polyneuropathy and improve quality of life in patients. This study evaluates the safety, efficacy, and pharmacokinetics (PK) of patisiran in patients with hATTR amyloidosis with polyneuropathy and disease progression post-OLT.

Methods: Phase 3b open-label study (NCT0386280) where patients receive patisiran 0.3 mg/kg intravenously once every 3 weeks for 12 months. 6-month safety and efficacy analyses will be presented.

Results: 23 patients enrolled and received patisiran in the study. Median age was 58.0 years, 13 (56.5%) were males, and 15 (65.2%) had V30M mutation. At baseline, 1 (4.3%) patient had polyneuropathy disability (PND) score I, 9 (39.1%) PND II, and 13 (56.5%) PND IIIA/B. 5 patients (21.7%) had New York Heart Association (NYHA) classification I, 5 (21.7%) NYHA II, and none had NYHA III/IV at study baseline. Mean (SD) percent change in transthyretin at week 3 was -81.9% (11.3). 21 (91.3%) patients experienced at least one adverse event (AE); 3 (13.0%) patients experienced at least one serious AE. 5 (21.7%) patients had AEs related to treatment.

Discussion: Updated 6-month efficacy and safety data will be presented at the congress.

Conclusions: This study will continue to investigate the efficacy, safety, and PK of patisiran with the potential to address an unmet need in patients with hATTR amyloidosis with polyneuropathy with disease progression post-OLT.