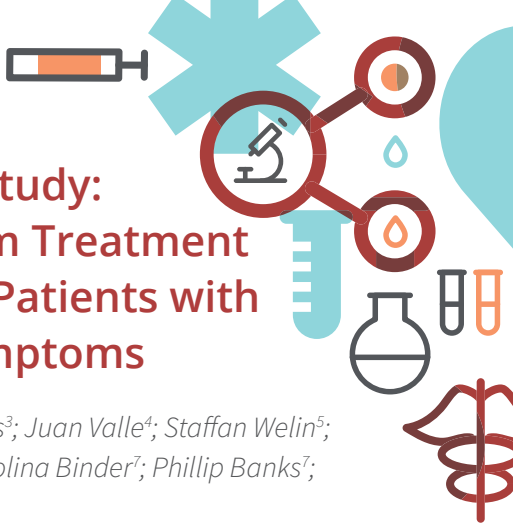

C-1

The TELEPATH Phase 3 Study: An Analysis of Long-Term Treatment with Telotristat Ethyl in Patients with Carcinoid Syndrome Symptoms

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BACKGROUND: TELEPATH (NCT02026063) is an open-label extension phase 3 study that evaluated the long-term safety and changes in patient reported outcomes of telotristat ethyl (TE) in patients enrolled in prior Phase 2 (NCT00853047, NCT01104415) or Phase 3 (NCT01677910, NCT02063659) trials.

METHODS: Patients continued receiving open-label TE 250 or 500 mg three times per day for treatment of carcinoid syndrome (CS) symptoms as administered in the original study they were enrolled in. The primary endpoint was the incidence of treatment-emergent adverse events (TEAEs) and the secondary endpoint was evaluation of long-term changes in patient-reported outcomes over the first 84 weeks of TELEPATH treatment (i.e., during continuation of TE therapy).

RESULTS: 124 patients were enrolled and 53.2% completed the study. Mean cumulative duration of exposure was 102.6 weeks (max, 234 weeks) and treatment compliance was 88.2%. TEAEs were reported in 98.4% of patients (serious in 53.2% with 2.4% [n=3 patients] experiencing treatment related SAEs). Study drug discontinuation due to TEAEs was required in 17.7% of patients.

Death due to TEAEs was reported in 16.1% (n=20 patients) of patients, 12 were due to progressive disease/underlying malignancy and only 1 was considered possibly related to treatment. The most frequent TEAEs by preferred term were diarrhea (35.5%), nausea (33.1%), and abdominal pain (32.3%). There was no significant change in the proportion of patients reporting adequate relief of CS symptoms associated with gastrointestinal events. A minimal (-0.9, P=0.29) change was reported in the EORTC QLQ-30 Global Health Status score at Week 84. The mean severity score of patients overall with CS generally remained stable.

CONCLUSION: Long-term administration of TE was well-tolerated in majority of patients and AEs were consistent with the known safety profile. In general, benefits in quality of life and relief of CS symptoms experienced in the prior studies were maintained with extended therapy.