XERMELO Patient Registry: Improvements in Clinical Outcomes, Patient Satisfaction, and Weight with Telotristat Ethyl in the Real-World

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Background and Objective

- The effectiveness of telotristat ethyl (TE) to improve CS symptoms including carcinoid syndrome diarrhea (CSD) and to ameliorate weight loss has been demonstrated in clinical trials.[1,2]
- Observational studies have also shown effectiveness of TE added to somatostatin analogs (SSA) in clinical practice according to clinical and patient-reported outcomes.[3,4]
- This ongoing registry evaluates clinical and patient-reported outcomes in a larger cohort of patients receiving TE over a longer duration of treatment than observed in clinical trials.

Methods

- The design and methods of the non-interventional RELAX registry have been reported previously.[4]
- Patients with CS initiating TE treatment are invited through the specialty pharmacy.
- Online surveys are conducted at baseline (BL) and every 6 months up to 3 years.
- BL assessments include demographic and clinical characteristics, CS treatment history, and patient satisfaction with CS treatment before initiating TE.
- Six-month outcomes include:
  - Patient-reported outcomes related to CS symptom control since initiating TE, including CS symptoms overall, diarrhea, flushing, and daily bowel movement (BM) frequency
  - Patient satisfaction and global impression of change (PGIC)
  - Changes in rescue medication and long-acting SSA use
- This interim analysis presents demographic and clinical characteristics, and clinical and patient-reported outcomes at BL and after 6 months of TE treatment.

Results

- At the time of this analysis, 109 patients had initiated TE (BL) and 51 patients had 6 months of follow-up data available.
- The mean age of this cohort was 61 years; mean time since CS diagnosis was 5.3 years; and half were female (56%; Table 1).

Table 1. Baseline demographic and clinical characteristics

Characteristic	Patients (N = 109)
Age, mean (SD), years	60.9 (11.1)
Sex, n (%), female	61 (56)
Weight, mean (SD), kg	82.4 (24.0)
Race or ethnicity, n (%)	
White or Caucasian	92 (84)
Black or African-American	8 (7)
Hispanic or Latino	6 (6)
Asian	1 (1)
Other or prefer not to answer	2 (2)
US Region, n (%)	
Northeast	24 (22)
Midwest	19 (17)
South	44 (40)
West	22 (20)
CS diagnosis to baseline, mean (SD), years	5.3 (4.5)
Site of primary neuroendocrine tumor, n (%)	
Small intestine	72 (66)
Lung	3 (3)
Appendix	3 (3)
Other	25 (23)
Do not recall	6 (6)
SSA therapy use in past 1 month, n (%)	
Short-acting SSA rescue medication	21 (19)
Long-acting SSA	106 (97)
No other treatment	2 (2)
CS treatments in past 6 months, n (%)*	
Chemotherapy	14 (13)
Radionuclide therapy	6 (6)
Radiation	5 (5)
Embolization	5 (5)
Chemoembolization directly into liver	2 (2)
No additional treatments or do not recall	81 (74)

*Patients could have indicated more than 1 treatment

- Overall, patients reported greater treatment satisfaction related to CS symptoms after 6 months of TE (**Figure 1**).
- Most patients (71%, 95% CI: 0.58–0.83) reported being at least somewhat satisfied with overall CS symptom control at 6 months.
- The majority of patients reported reductions in daily bowel movements (80%, 95% CI: 0.69–0.91) and/or improvement in CS symptoms overall (75%, 95% CI: 0.63–0.87; **Figure 2**).

Figure 1. Treatment satisfaction at baseline (N=109) and after 6 months of TE (n=51)

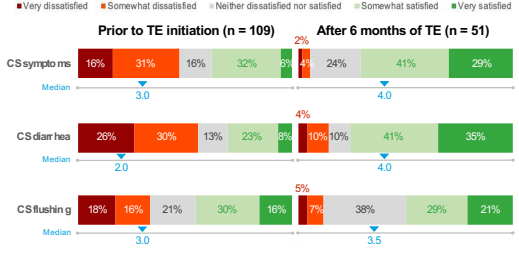
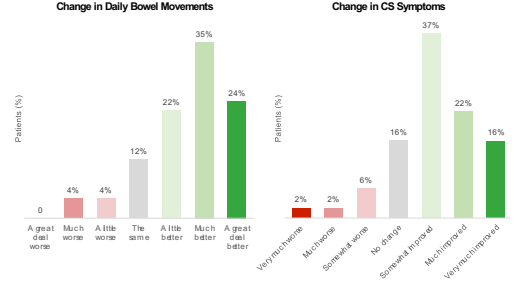
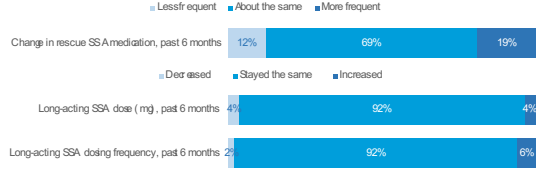


Figure 2. Patient-reported BM frequency and CS symptoms after 6 months of TE (n=51)



- Most patients reported weight maintenance or weight gain after 6 months of TE (75%, 95% CI: 0.63–0.87); 22% reported weight gain.
- Use of short-acting SSA rescue therapy and long-acting SSA therapy decreased or stayed the same for nearly all patients (**Figure 3**).
  - Among patients who continued using long-acting SSA therapy, 4% (2/51) reported reductions in dose (mg/month) and 6% (3/50) reported reductions in dosing frequency.

Figure 3. Changes in rescue and long-acting SSA therapy after 6 months of TE (n=51)



Conclusions

- These findings are consistent with previous demonstrations of the real-world effectiveness of TE in clinical practice, and with results from clinical trials.
- The majority of patients receiving TE for 6 months reported improved CS symptoms and treatment satisfaction with stabilization or reduction of background SSA therapy.
- Nearly all patients receiving 6 months of TE maintained or gained weight, a clinically relevant indicator of health for patients with CS.

References

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Disclosures

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