



## Xermelo™ (telotristat ethyl) – New orphan drug approval

- On February 28, 2017, the [FDA announced](#) the approval of [Lexicon's Xermelo \(telotristat ethyl\)](#), for the treatment of carcinoid syndrome diarrhea in combination with somatostatin analog (SSA) therapy in adults inadequately controlled by SSA therapy.
- Carcinoid syndrome is a cluster of symptoms that occurs in patients living with metastatic neuroendocrine tumors (mNETs). It is characterized by frequent, debilitating diarrhea as well as facial flushing, abdominal pain, fatigue, and over time, heart valve damage.
- Xermelo is an inhibitor of tryptophan hydroxylase, an enzyme that disrupts the production of serotonin by mNETs, thereby, reducing the frequency of carcinoid syndrome diarrhea.
- The safety and efficacy of Xermelo were based on a placebo-controlled trial in adult patients with well-differentiated mNETs and carcinoid syndrome diarrhea. The primary endpoint was the change from baseline in the number of daily bowel movements averaged over the 12-week treatment period.
  - Patients receiving Xermelo added on to their SSA treatment experienced a greater reduction in average bowel movement frequency vs. placebo and SSA ( $p < 0.001$ ). Specifically, 33% of participants in the Xermelo arm experienced an average reduction of two bowel movements per day vs. 4% in the placebo arm.
- Warnings and precautions of Xermelo include constipation.
- The most common adverse events ( $\geq 5\%$ ) with Xermelo use were nausea, headache, increased gamma glutamyl transferase, depression, flatulence, decreased appetite, peripheral edema, and pyrexia.
- The recommended dose of Xermelo is 250 mg orally three times daily for patients whose diarrhea is inadequately controlled by SSA therapy.
  - When short-acting octreotide is used in combination with Xermelo, administer short-acting octreotide at least 30 minutes after Xermelo.
  - Discontinue Xermelo if severe constipation develops.
- The drug cost for Xermelo is projected to be \$61,000 - \$72,000 per patient per year.
- Xermelo will be in select specialty pharmacies beginning on March 6, 2017. Xermelo will be available as 250 mg tablets.



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