Clinical Therapeutics/Volume ▮, Number ▮, 2017

Budgetary Impact of Telotristat Ethyl, a Novel Treatment for Patients with Carcinoid Syndrome Diarrhea: A US Health Plan Perspective

Vijay N. Joish, PhD*; Feride Frech, PhD†,‡; and Pablo Lapuerta, MD§

Lexicon Pharmaceuticals, Inc, Basking Ridge, New Jersey

ABSTRACT

Purpose: Telotristat ethyl (TE) was recently approved for carcinoid syndrome diarrhea (CSD) in patients not adequately controlled with somatostatin analog long-acting release (SSA LAR) therapy alone. A budget impact model was developed to determine the short-term affordability of reimbursing TE in a US health plan.

Methods: A budget impact model compared health care costs when CSD is managed per current treatment patterns (SSA LAR, reference drug scenario) versus when TE is incorporated in the treatment algorithm (SSA LAR + TE, new drug scenario). Prevalence of CSD, proportion of patients not adequately controlled on SSA LAR, monthly treatment costs (pharmacy and medical), and treatment efficacy were derived from the literature. In the reference drug scenario, an escalated monthly dose of SSA LAR therapy of 40 mg was assumed to treat patients with CSD not adequately controlled on the labeled dose of SSA LAR. In the new drug scenario, TE was added to the maximum labeled monthly dose of SSA LAR therapy of 30 mg. The incremental budget impact was calculated based on an assumed TE market uptake of 28%, 42%, and 55% during Years 1, 2, and 3, respectively. One-way sensitivity analyses were conducted to test model assumptions.

Findings: A hypothetical health plan of 1 million members was estimated to have 42 prevalent CSD patients of whom 17 would be inadequately controlled on SSA LAR therapy. The monthly medical cost per patient not adequately controlled on SSA LAR in addition to pharmacotherapy was estimated to be $3946 based on the literature. Based on the observed treatment response in a clinical trial of 20% and 44% for the base case reference and new drug scenarios, total per patient per month costs were estimated to be $7563 and $11,205, respectively. Total annual costs in the new drug scenario were estimated to be $2.3 to $2.5 million during the first 3 years. The overall incremental annual costs were estimated to be $154,000 in Year 1, $231,000 in Year 2, and $302,000 in Year 3. This translated to an incremental per patient per month cost of $0.013, $0.019, and $0.025 for Years 1, 2, and 3. These results remained robust in 1-way sensitivity analyses.

Implications: The availability of TE for patients not adequately controlled on SSA LAR therapy provides a novel treatment option for CSD. This model showed that providing access to this first-in-class oral agent would have a minimal budget impact to a US health plan. (*Clin Ther*. 2017;▮:▮▮▮–▮▮▮) © 2017 Elsevier HS Journals, Inc. All rights reserved.

Key words: budget impact, carcinoid syndrome diarrhea, neuroendocrine tumors, somatostatin analogs, telotristat ethyl.

INTRODUCTION

Patients with well-differentiated neuroendocrine tumors (NETs) may develop carcinoid syndrome (CS), which is characterized by flushing, abdominal cramps, diarrhea, bronchoconstriction, and carcinoid heart disease.[1] The incidence of carcinoid tumors in the United States is 1.9 per 100,000 people, and approximately 10% to 20% of commercially insured and Medicare patients with gastrointestinal carcinoid tumors have CS.[1–3] CS-associated diarrhea (CSD) is the most burdensome

*Executive Director Epidemiology and Outcomes Research.
†Current affiliation Former employee of Lexicon Pharmaceuticals.
‡Former HEOR Employee.
§Executive Vice President and Chief Medical Officer.

Accepted for publication October 30, 2017.
https://doi.org/10.1016/j.clinthera.2017.10.019
0149-2918/$ - see front matter

symptom to patients with CS and to health insurers because it results in increased office visits, hospitalizations, and total health care spending compared with CS patients without diarrhea.[4] Successful treatment of CSD has been shown to significantly reduce health care costs compared with those without any symptom improvement.[5,6]

The standard of care for CSD in the United States has been the long-acting release formulation of the somatostatin analog octreotide long-acting release (SSA LAR) that blocks the release of vasoactive peptides and amines.[7] The long-acting formulations of SSA therapy have replaced the original short-acting formulations in the management of NETs and acromegaly.[8,9] SSA LAR therapy has been shown to reduce the symptoms associated with CS and slow tumor progression.[10] When response to SSA LAR therapy decreases, standard clinical practice is to escalate the dose of SSA LAR beyond the labeled dose and/or initiate additional treatments.[11] However, approximately 40% of patients with CSD treated with SSA LAR in the United States have been found to be unresponsive to therapy.[5]

Telotristat ethyl (TE) is a novel oral small-molecule tryptophan hydroxylase inhibitor recently approved in the United States for the treatment of CSD in combination with SSA LAR therapy in adults not adequately controlled by SSA LAR therapy alone.[12] TE demonstrated significant reductions in daily bowel movement frequency and improvements in other markers of disease among patients with CSD not adequately controlled by SSA LAR therapy alone in the randomized, controlled TELESTAR (Telotristat Etiprate for Somatostatin Analogue Not Adequately Controlled Carcinoid Syndrome) Phase III clinical trial.[13] An open-label extension to TELESTAR showed safety and tolerability of TE through 1 year.[13] Based on these results, TE has now been included as a recommended treatment option in the latest National Comprehensive Cancer Network Clinical Practice Guidelines in Oncology for NETs and in the National Comprehensive Cancer Network Drugs and Biologics Compendium where its use is recommended in combination with SSA therapy (octreotide or lanreotide) for persistent diarrhea due to poorly controlled CSD.[14,15]

Given the recent launch and inclusion of TE in the treatment guidelines, the objective of this study was to evaluate the potential budget impact of including TE with octreotide (SSA LAR + TE) compared with octreotide alone (SSA LAR) among patients with CSD whose symptoms remain inadequately controlled with SSA LAR-only therapy.

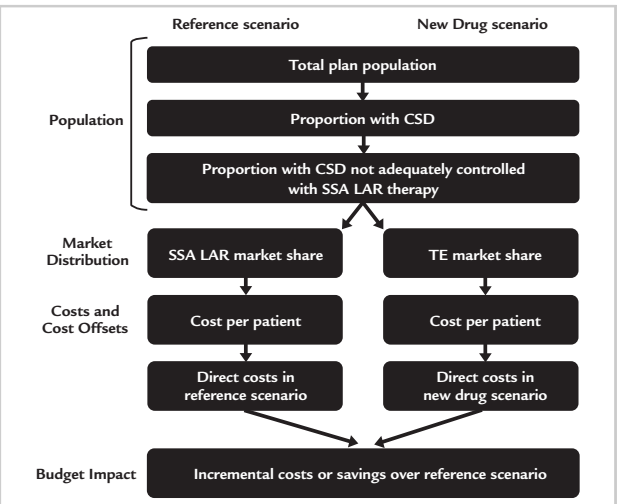

Figure 1. Study flow chart. CSD = carcinoid syndrome-associated diarrhea, SSA LAR = somatostatin analog octreotide long-acting release, TE = telotristat ethyl.

METHODS

Model Overview

An Excel-based budget impact model (Microsoft, Redmond, Washington) was developed for a hypothetical US health plan population with 1 million members (Figure 1). The analysis considered a 3-year time horizon for net changes in total health care costs per member per month (PMPM) and per patient per month between the reference scenario with above-label dose SSA LAR only (no TE) and the new drug scenario with on-label dose SSA LAR + TE as an available treatment option.

Eligible Population

The prevalence of CS is estimated to be 0.0042% as derived from the annual prevalence of NETs in the United States.[16,17] Of the total number of CS patients, Burton and Lapuerta[5] have reported 40% to be inadequately controlled by SSA LAR therapy. Based on a hypothetical 1 million-member plan, 42 patients (1 million x 0.000042) were estimated to have CSD of whom 17 (40%) would have inadequately controlled symptoms and therefore be eligible for the 2 treatment scenarios described above (above-label, escalated dose of monthly SSA LAR therapy or combination of TE