Economic Benefits Associated with Resolution or Improvement of Carcinoid Syndrome Symptoms Following Treatment with Above-Standard Dose of Octreotide-LAR in Patients with Neuroendocrine Tumors: Assessment Based on Data from a Multicenter Retrospective Chart Review Study

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Background: In patients with neuroendocrine tumor (NET), major manifestations of carcinoid syndrome (CS) symptoms are diarrhea and flushing. A retrospective chart review study was previously performed at three tertiary oncology centers in the US (Moffitt, Northwestern, Dana-Farber; NET 3-Center study) that included 239 NET patients who had been treated with above-standard dose of octreotide-LAR for carcinoid or hormonal syndrome (62%) or radiographic progression (28%). Most common above-standard doses received were 40mg every 4 weeks (51%), 30mg/3 weeks (18%), and 60mg/4 weeks (18%). In this published study, many patients who had reported CS symptoms experienced symptom resolution/improvement (diarrhea: 79%, flushing: 81%) within one year after first dose escalation (*The Oncologist*, 2014). This study developed an economic model to evaluate potential cost savings associated with CS symptom resolution/improvement observed in the NET 3-Center study.

Methods: CS symptoms and treatment data from the NET 3-Center study were used. Healthcare resource utilization and cost inputs for a separate sample of patients from the Truven Health Analytics MarketScan healthcare claims database were used to estimate incremental costs for patients with vs. without CS symptoms. Patients in claims database may have received standard and above-standard doses of octreotide-LAR; sensitivity analyses will be presented to assess possible impact of variations in dose on cost. Total healthcare costs included inpatient, outpatient, emergency department (ED), and pharmacy services and were adjusted for age, gender, region, number of chronic conditions, and Charlson comorbidity index, using multivariable OLS regression. For each patient from the NET 3-Center study, the period after initiation of above-standard dose of octreotide-LAR (index date) was divided into days with and without CS symptoms; costs were calculated for each period. Total healthcare costs over the 12-month period post-index date were compared to annual total healthcare costs of patients with CS symptoms.

Results: One hundred and thirty-six patients had either diarrhea or flushing within 3 months prior to the index date; 108(79%) patients experienced CS symptom resolution/improvement within 12 months of index date. Patients with CS symptom resolution/improvement had statistically significantly lower mean annual total healthcare costs per patient by \$14,766 (p=0.03) than patients with CS symptoms. Cost savings were driven by resolution/improvement of diarrhea symptom. Among 107 patients with diarrhea within 3 months prior to the index date, 85(79%) patients experienced resolution/improvement post-index date. In the year following the index date, these patients had mean (SD) days with vs. without diarrhea of

147(123) and 218(123), respectively. Patients with resolution/improvement of diarrhea had statistically significantly lower mean annual total healthcare costs per patient by \$18,740 (p=0.01) than patients with diarrhea, with outpatient costs accounting for most of the difference (mean difference: \$10,467; p=0.02).

Conclusions: This economic model showed statistically significant mean annual total healthcare cost savings in patients having CS symptoms resolution/improvement after treatment with above-standard dose of octreotide-LAR. These economic benefits are in addition to any possible improvements in quality of life and functional status associated with CS symptom control. This model uses assumptions that need to be further validated in future studies and using alternative data sources.