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## **Real-world treatment patterns, adherence, costs and healthcare resource utilization associated with Waldenström macroglobulinemia in the United States.**

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**Background:** Waldenström macroglobulinemia (WM) is a rare, incurable non-Hodgkin lymphoma. Given limited real-world data on WM treatment utilization, this study evaluates real-world treatment patterns and associated outcomes in the US commercially insured population.

**Methods:** A retrospective observational study was conducted using the IBM MarketScan® commercial claims and Medicare supplement database. Adults with  $\geq 2$  WM diagnoses and  $\geq 1$  WM treatment between 2014 and 2019 were identified. Patients included were newly diagnosed, initiating treatment, and enrolled continuously for 6 months before and  $\geq 60$  days following the index date, defined as the first date of WM treatment. Treatment regimens were categorized as: rituximab monotherapy, chemotherapy-based (alone or in combination), proteasome inhibitor-based (alone or in combination with rituximab), ibrutinib (alone or in combination with rituximab), and other regimens. Treatment patterns were evaluated by frequency and duration of treatment regimen. Adherence is measured by discontinuation and switching rates. Healthcare resource utilization examined included inpatient, outpatient, and pharmacy visits. Total costs were calculated as sum of inpatient, outpatient and pharmacy costs per-patient-per-month (PPPM). Treatment regimens, costs, and hospitalizations were examined overall, and by line of therapy.

**Results:** A total of 453 patients (mean age: 67 years, 51% male) received 1<sup>st</sup>-line therapy (mean duration: 246 days); 143 (32%) patients received 2<sup>nd</sup>-line therapy (mean duration: 231 days), and 24 (5%) received 3<sup>rd</sup>-line therapy (mean duration: 212 days). The most commonly used treatment regimens by line of therapy are shown in the treatment pattern table. Discontinuation rates were 43.3%, 50.4%, and 45.8%, and the switching rates were 25.4%, 10.5%, 20.8% during 1<sup>st</sup>, 2<sup>nd</sup>, and 3<sup>rd</sup> line of therapy, respectively. The overall hospitalization rate was 20% with an average length of stay (LOS) of 2.3 days. Approximately 17% (LOS: 1.4 days), 20% (LOS: 1.8 days), and 25% (LOS: 7.0 days) of patients had a hospitalization, during 1<sup>st</sup>, 2<sup>nd</sup>, and 3<sup>rd</sup> line of therapy, respectively. Mean total PPPM costs were \$26,688 in overall population, and increased by line of therapy (1<sup>st</sup>: \$18,682; 2<sup>nd</sup>: \$19,171; and 3<sup>rd</sup>: \$36,878).

**Conclusions:** There remains a significant clinical and economic burden with suboptimal treatment adherence in US commercially-insured patients with WM. Future studies are needed to further understand factors associated with treatment selection.

**Table. Real-world Treatment Patterns In WM Patients**

<b>Regimen</b>	<b>Line of therapy</b>		
	<b>N (%)</b>		
	<b>1<sup>st</sup></b>	<b>2<sup>nd</sup></b>	<b>3<sup>rd</sup></b>
<b>Rituximab monotherapy</b>	144 (31.8%)	34 (23.8%)	7 (29.2%)
<b>Chemotherapy-based</b>	101 (22.3%)	24 (16.8%)	5 (20.8%)
<b>Proteasome-inhibitors</b>	79 (17.4%)	14 (9.8%)	1 (4.2%)
<b>Ibrutinib</b>	69 (15.2%)	45 (31.5%)	8 (33.3%)
<b>Other</b>	60 (13.3%)	26 (18.2%)	3 (12.5%)