



Drug Patterns, Clinical Outcomes, and Economic Costs among Hereditary Angioedema Patients Initiating Long-Term Prophylaxis

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INTRODUCTION

- Hereditary angioedema (HAE) is a rare autosomal dominant condition characterized by recurrent episodes of nonpruritic, nonpitting, subcutaneous, or submucosal swelling without urticarial lesions
 - Clinical events associated with HAE are called “attacks”, and can involve multiple body areas, including hands, feet, intestinal wall, genitalia, face, tongue, and/or larynx
- Until recently, the management of HAE attacks was largely limited to prophylactic use of an intravenous C1 esterase inhibitor (C1-INH) and/or reactive use of acute (on-demand) treatment¹
- Since 2017, three additional agents have been approved for the prevention of attacks in patients with HAE: C1-INH (subcutaneous [SC]), lanadelumab, and berotralstat²⁻⁴
- Real-world evidence on the use of long-term prophylaxis (LTP), as well as the burden of attacks among HAE patients receiving LTP, is sparse

OBJECTIVES

- To explore patterns of LTP, attack rates, and attack-related healthcare expenditures among HAE patients initiating C1-INH-SC or lanadelumab in United States (US) clinical practice
 - Berotralstat was not considered in analyses due to small sample size

METHODS

Study Design and Data Source

- Retrospective observational cohort design
- Paid healthcare claims data (02/23/18-06/30/2022) from the Merative Commercial Claims and Encounters (CCA) and Medicare Supplemental and Coordination of Benefits (MDCR) Databases

Study Population

- Study population comprised patients aged ≥12 years who initiated LTP with C1-INH-SC or lanadelumab
 - Use of C1-INH-SC and lanadelumab was identified based on medical claims and outpatient pharmacy claims with a corresponding drug (HCPCS/NDC) code
 - Date of first claim for LTP was defined as the initiation date

Study Measures

- Study measures (LTP adherence, LTP discontinuation, number of attacks, attack-related expenditures) were evaluated from LTP initiation through loss of healthcare coverage or end of study period
- LTP adherence was defined using the medication possession ratio (MPR; ratio of prophylaxis days to follow-up days), and LTP discontinuation was defined as a gap in drug supply of ≥30, ≥60, and ≥90 days, respectively
- HAE attacks and attack-related expenditures (2022 USD) were ascertained based on all-cause hospitalizations, all-cause emergency department (ED) visits, and administration of acute treatment
 - Evidence separated by ≤5 days was considered a single attack⁵

Baseline Characteristics

- Demographic, clinical, and treatment profiles were ascertained based on healthcare claims during the 6-month period prior to initiation of LTP

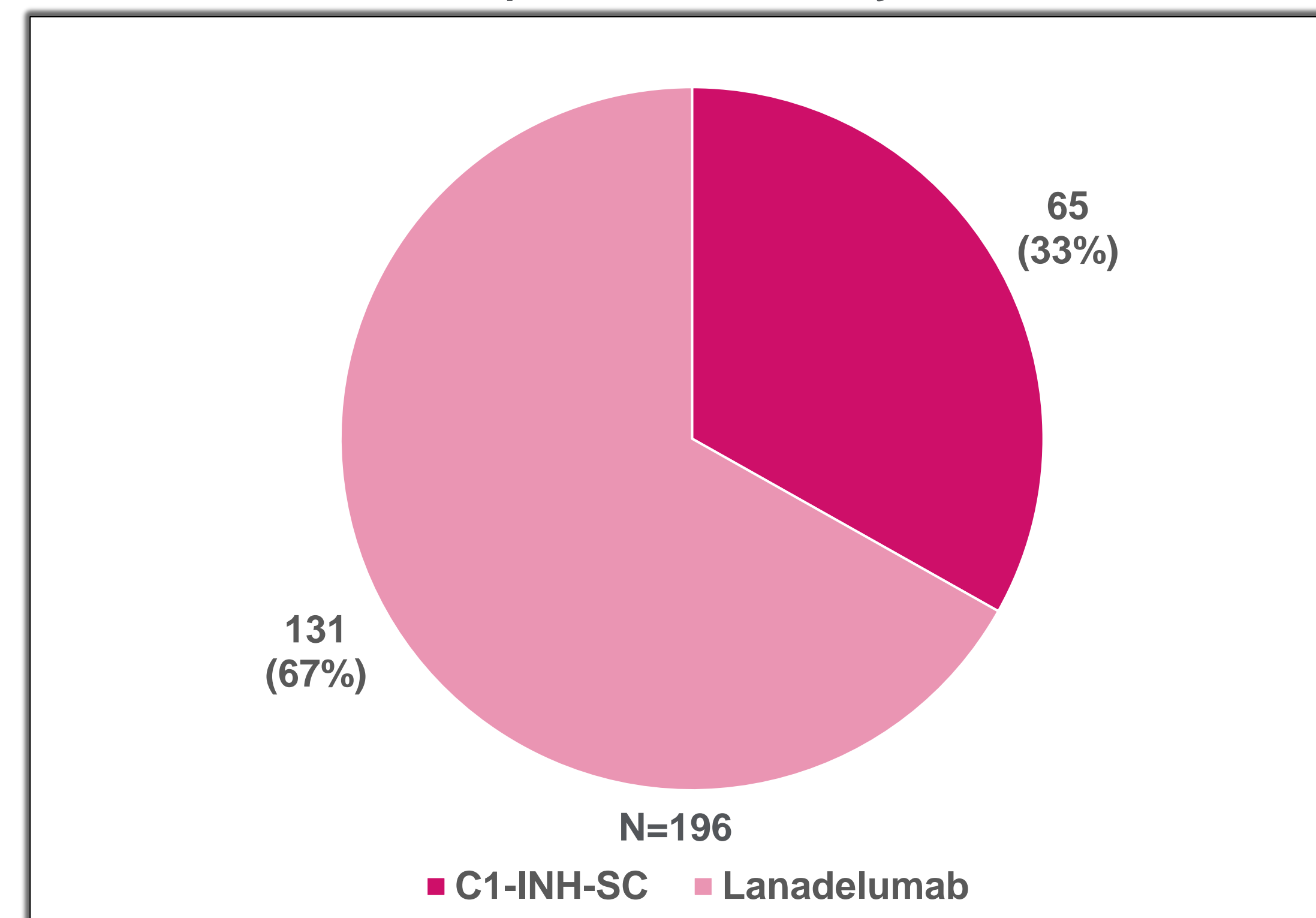
Statistical Analyses

- Study measures were summarized using means, percentages, and 95% confidence intervals (CI)
 - Number of attacks and attack-related expenditures were annualized using population-based approach

Patient Characteristics

- A total of 196 patients who initiated LTP were included in the study population

FIGURE 1: Distribution of patients with HAE, by initial LTP



- Mean age of patients was 39 years, and 69% were female

TABLE: Baseline characteristics of study population

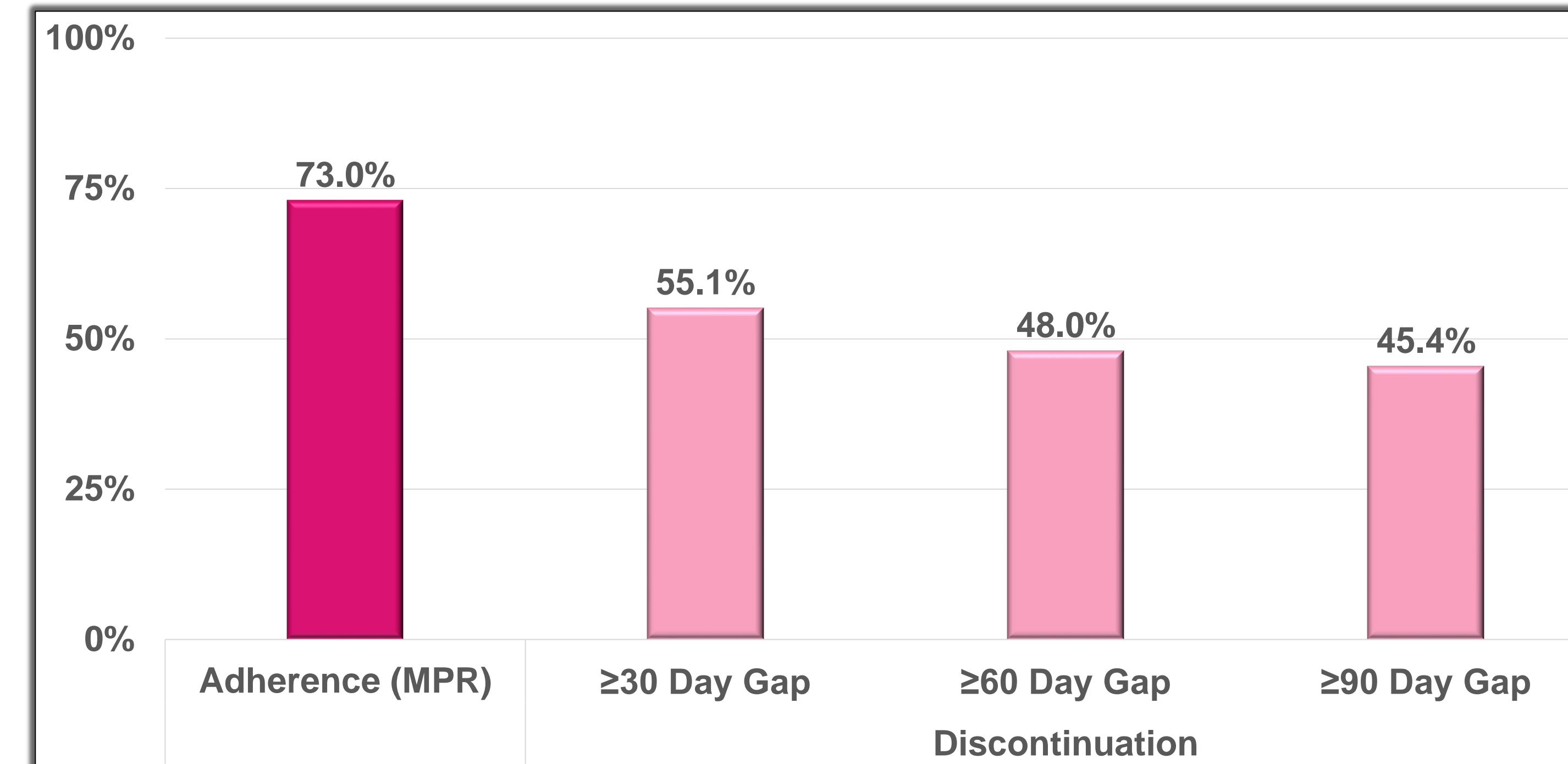
	All Patients (N=196)
Age (years)	
Mean (SD)	39.1 (15.3)
Female, %	69.4
Comorbidity Profile, %	
Metabolic disorders	31.6
Obesity (BMI >40)	24.0
Respiratory disease	18.9
Cardiovascular disease	10.7
Diabetes	9.7
Immunosuppressive conditions/treatments	7.7
Osteoarthritis	6.6
Neurologic disorders	5.1
Liver disease	4.6
Use of Drugs for HAE Management, %	
Androgens	10.7
Danazol	9.2
Oxandrolone	0.5
Tranexamic acid	0.5
Aminocaproic acid	0.0
Methyltestosterone	0.0

RESULTS

Patterns of LTP

- During mean (SD) follow-up of 18.9 (13.4) months, LTP adherence was 73.0% (95% CI: 67.8-77.3), and LTP discontinuation ranged from 45.4% (38.3-52.7) to 55.1% (47.9-62.2)

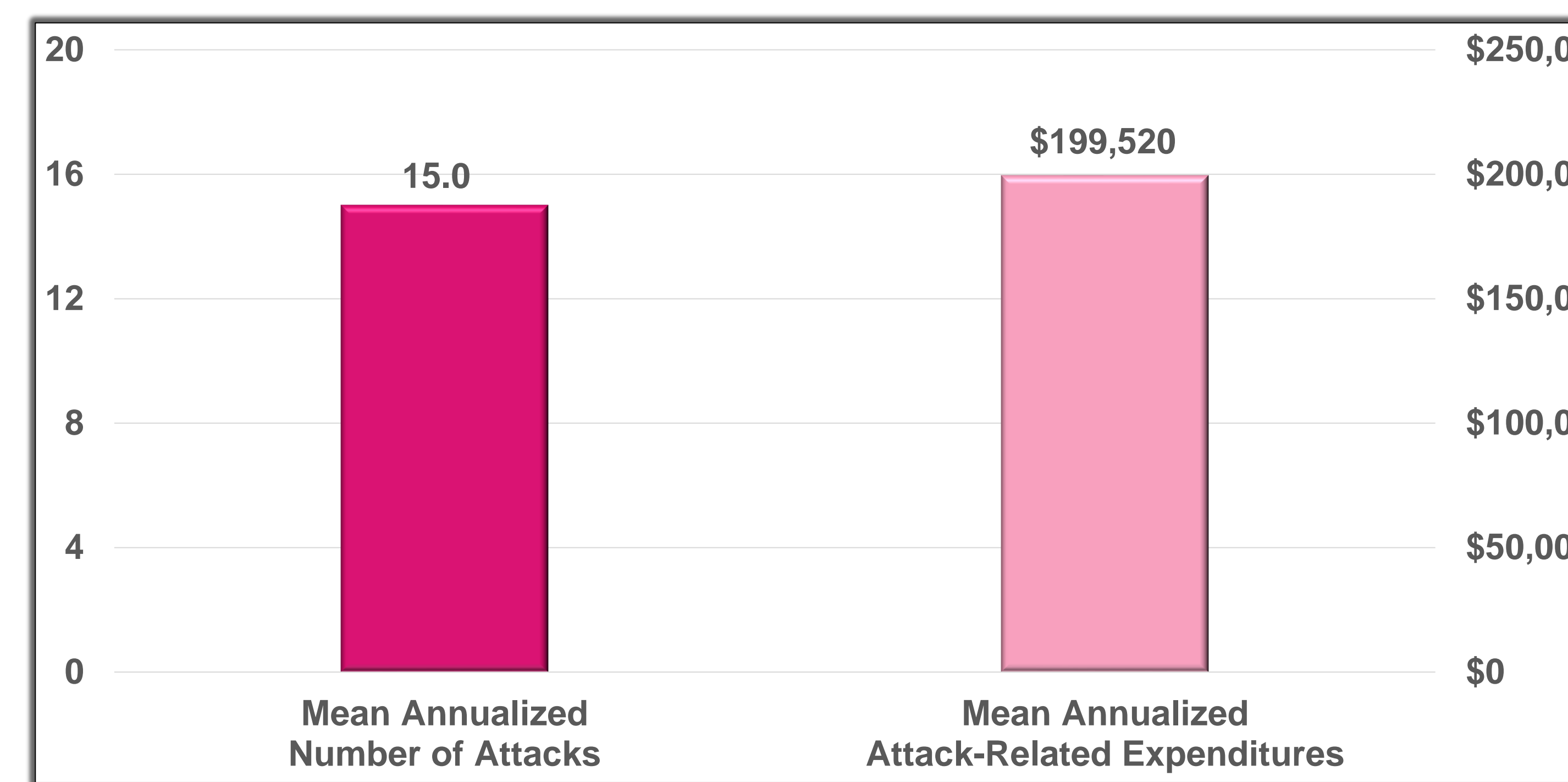
FIGURE 2: LTP adherence and discontinuation among patients with HAE



HAE Attacks and Attack-Related Healthcare Expenditures

- Mean annualized number of attacks identified via acute treatment was 15.0 (14.5-15.4) per patient
 - Attacks identified via hospitalizations and ED visits averaged 0.2 (0.1-0.2) and 0.5 (0.5-0.6)
- Mean annualized expenditures for attacks identified via acute treatment was \$199,520 (122,473-329,336)
 - Attack-related expenditures for hospitalizations/ED visits averaged \$37,423 (20,755-59,519)

FIGURE 3: Mean annualized number of attacks and expenditures for attacks identified via acute treatment



LIMITATIONS

- Patients with healthcare claims for LTP were assumed to have HAE; a unique diagnosis code for HAE does not currently exist
- Identification of HAE attacks was based on a published operational algorithm including all-cause hospitalizations, all-cause ED visits, and acute treatment⁵; the accuracy of this algorithm is unknown
 - All-cause hospitalizations/ED visits may have occurred for other reasons
 - Use of acute treatment as a proxy for attacks is subject to bias due to drug stockpiling and other factors; attack-related expenditures may thus be overestimated

CONCLUSIONS

- Despite the availability of newer LTP agents, adherence was not optimal, ~50% of patients discontinued, attacks were common, and associated costs were high
- Notably, annualized expenditures for attacks identified via acute treatment averaged ~\$200,000, even after initiation of LTP, thus demonstrating considerable unmet need in the management of HAE

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DISCLOSURES

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