Reductions in Healthcare Utilization Among Individuals with HAE Who Switched to Berotralstat from Another Prophylaxis

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BACKGROUND

- Hereditary angioedema (HAE) is a rare inherited condition marked by recurrent attacks of painful and unpredictable swelling of the skin and mucous membranes, which can be life-threatening when affecting the upper airway.[1]
- Berotralstat is the only targeted, once-daily, oral prophylactic therapy for the prevention of HAE attacks in adults and pediatric patients aged 2 years and older.[2]
- Real-world analyses using Optime Care Specialty Pharmacy data observed significant reductions in HAE attacks following berotralstat initiation.[3]
- Additionally, significant reductions in healthcare resource utilization (HRU) following berotralstat initiation have been reported in Komodo Health claims data.[4]
- This study evaluated changes in angioedema-related HRU following berotralstat initiation among individuals switching from another long-term prophylaxis (LTP) using linked Optime Care and Komodo Healthcare Map data.

METHODS

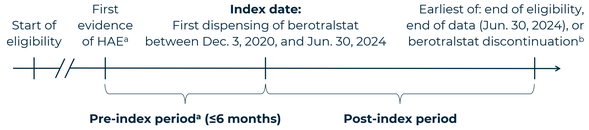
Data Source

- Optime Care Specialty Pharmacy data (Dec. 3, 2020 – Jun. 30, 2024) were linked with Komodo Healthcare Map administrative claims data (Oct. 1, 2015 – Jun. 30, 2024) using de-identified Datavant tokens.
- Optime Care was the sole dispenser of berotralstat in the United States, and the database includes berotralstat shipment information, self-assessments of HAE attacks, and laboratory results (C1INH levels, C1INH function, and C4 levels) for HAE type.
- Komodo Healthcare Map contains pharmacy and medical claims data for more than 320 million patient-lives in the United States.

Study Design and Outcomes

- This retrospective pre–post study selected patients with ≥2 berotralstat dispensings (first=index), aged ≥12 years at index, had no pre-index enrollment in a clinical trial or expanded access program, had ≥6 months of continuous insurance eligibility before and on the index date, had evidence of HAE during continuous eligibility before index, and met the prespecified switch criteria (another LTP in the 60 days before starting berotralstat and without resumption in the 60 days after).
- The pre-index period spanned from first evidence of HAE to the index date, with a maximum of 6 months. The post-index period spanned the index date to the earliest of the end of continuous eligibility, end of data, or date of berotralstat discontinuation.
- Angioedema-related medical visits were identified using claims with a primary diagnosis code for HAE or angioedema, provider-administered on-demand or LTP medications, and diagnostic tests for complement components associated with HAE. Rates of medical visits, outpatient (OP)/emergency department (ED) visits, and hospitalizations per person-year were weighted by patients' length of observation.
- Rates of visits post-index were compared with pre-index using rate ratios, 95% confidence intervals (CIs), and p-values from generalized estimating equation (GEE) Poisson regression models with robust standard errors.

Figure 1. Pre-Post Study Design

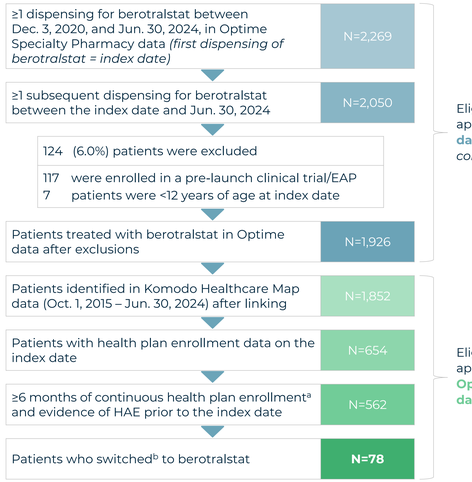


HAE, hereditary angioedema; LTP, long-term prophylaxis. [a]First evidence of HAE was assessed any time pre-index (during continuous eligibility). Evidence of HAE was defined as a pharmacy or medical claim for an HAE on-demand or LTP medication, an angioedema or HAE diagnosis (in any position), or measurement of complement function. If the first evidence of HAE was ≥6 months pre-index, then the pre-index period was 6 months; if the first evidence of HAE was <6 months pre-index, then the pre-index period spanned from the first evidence of HAE to the index date. [b]Discontinuation was defined as a gap of ≥60 days of berotralstat supply between consecutive dispensings or between the end of data availability or continuous eligibility. The discontinuation date was the final day of berotralstat supply before the gap.

RESULTS

- The study population comprised 78 individuals with HAE who switched from another LTP to berotralstat (**Figure 2**).
- Mean age was 41.8 years, 78.2% were female, and 67.9% had commercial insurance at index (**Table 1**).
- 73% of the study population used lanadelumab (42%), Haegarda (26%), or Cinryze (5%) prior to switch to berotralstat. Danazol (19%), tranexamic acid (5%), and oxandrolone (3%) comprised the remaining pre-switch LTPs (**Figure 3**).
- Prior to berotralstat initiation, the mean rates of angioedema-related OP/ED visits and hospitalizations were 5.86 and 0.27 per person-year, respectively (**Figure 4**).
- Rates of angioedema-related visits decreased significantly by 30% for OP/ED visits after berotralstat initiation (p<0.05) (**Figure 5**).
- Rates of angioedema-related hospitalizations decreased to 0.12 per person-year post-index (rate ratio [95% CI]: 0.54 [0.21–1.39], non-significant) (**Figure 4** and **Figure 5**).

Figure 2. Berotralstat Patient Disposition



EAP, expanded access program; HAE, hereditary angioedema; LTP, long-term prophylaxis.
[a]Continuous health plan enrollment was defined as continuous periods with both medical and pharmacy insurance eligibility. [b]Patients who switched to berotralstat were identified as those with ≥1 day of non-berotralstat LTP supply within 60 days before the index date and no non-berotralstat LTP dispensing on the index date or during the first 60 days after index.

REFERENCES

1. Betschel S, et al. *Allergy Asthma Clin Immunol.* 2019;15(1):1-29. **2.** Berotralstat [package insert]. Durham, NC: December 2025. **3.** Davis-Lorton M, et al. *Allergy Asthma Clin Immunol.* 2026;22(1):10 **4.** Christiansen S, et al. *J Manag Care Spec Pharm.* 2025;31(6):578-589.

FUNDING

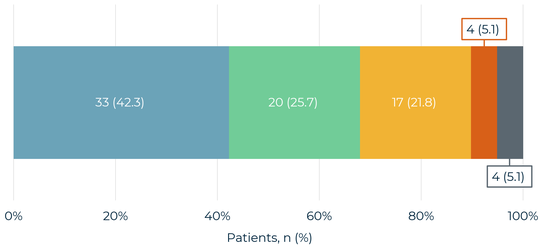
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Table 1. Demographics and Clinical Characteristics

Characteristics	Patients (N=78)
Demographics[a,a]	
Age, years, mean ± SD [median]	41.8 ± 17.5 [40]
Female, n (%)	61 (78.2)
Region of residence, n (%)	
South[b]	37 (47.4)
West	16 (20.5)
Midwest	15 (19.2)
Northeast	10 (12.8)
Insurance plan type, n (%)[†,a]	
Commercial	53 (67.9)
Medicaid	15 (19.2)
Medicare	10 (12.8)
HAE type, n (%)*[,a,c]	
HAE-C1INH (Type 1 or 2)	30 (38.5)
HAE-nC1INH	15 (19.2)
Undetermined	33 (42.3)
Healthcare practitioner type, n (%)*[,a]	
Allergist/Immunologist	70 (89.7)
Nurse practitioner	5 (6.4)
Other	3 (3.9)
Quan-CCI score, mean ± SD [median][†,d]	0.6 ± 1.1 [0]
Patients with ≥1 claim for an ODT pre-index, n (%)[†,e]	40 (51.3)

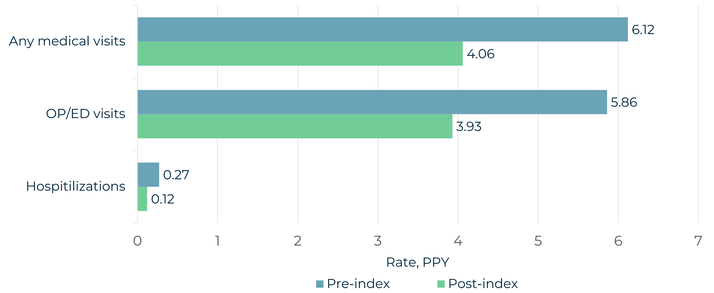
HAE-C1INH, hereditary angioedema with C1 esterase inhibitor deficiency; HAE-nC1INH, hereditary angioedema with normal C1 esterase inhibitor; ODT: on-demand therapy; Quan-CCI, Quan–Charlson Comorbidity Index; SD, standard deviation.
*Identified from Optime Care Specialty Pharmacy data. †Identified from Komodo Healthcare Map data.
[a]Assessed on the index date. [b]The South region includes individuals from Puerto Rico and Guam. [c]HAE type was identified using laboratory measurements of C1INH levels, C1INH function, and C4 levels. Patients with undetermined HAE type had missing laboratory measurements or a combination of measurements that could not be classified. [d]Assessed during the 6 months pre-index. [e]Assessed any time pre-index (from the start of continuous eligibility to the index date).

Figure 3. Non-Berotralstat LTP that Patients Switched From[a]



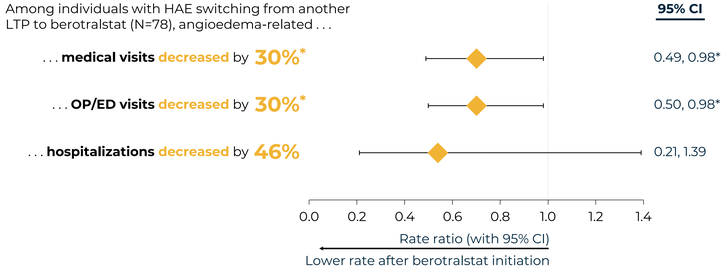
LTP, long-term prophylaxis.
[a]Categories are mutually exclusive and represent the final LTP switch classification. Individuals switching from attenuated androgens included 15 from danazol and 2 from oxandrolone. Individuals switching from antifibrinolytics all switched from tranexamic acid. One patient classified under lanadelumab had overlapping tranexamic acid supply, and one classified under Haegarda had overlapping danazol supply.

Figure 4. Mean Annual Rates of Angioedema-Related Healthcare Resource Utilization



ED, emergency department; OP, outpatient; PPY, per person-year.

Figure 5. Comparison of Angioedema-Related Healthcare Resource Utilization



CI, confidence interval; ED, emergency department; HAE, hereditary angioedema; LTP, long-term prophylaxis; OP, outpatient. *p<0.05.
Percent reduction was calculated as (1 − model-estimated rate ratio) × 100; percentages may differ from reductions calculated directly from the rounded rates in Figure 4.

Limitations

- Dispensed medications do not indicate that the medication was taken as prescribed.
- While matching data sources via tokenization achieves high precision, it does not ensure perfect accuracy in matching patients between different sources within the Komodo database or between Optime and Komodo data.

CONCLUSION

Among individuals with HAE who switched from another LTP to berotralstat, rates of angioedema-related medical visits decreased after berotralstat initiation.

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