# Linking Specialty Pharmacy Data with Healthcare Claims Data in a Rare Disease: Comprehensively Characterizing Patients with Hereditary Angioedema Treated with Berotralstat

MSR138

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## BACKGROUND

- Hereditary angioedema (HAE) is a rare disease characterized by painful, recurrent swelling attacks of skin and mucous membranes, which can be life-threatening when affecting the upper airway.[1]

- Studies have observed significant reductions in HAE attacks following berotralstat initiation using Optime Care Specialty Pharmacy data[2-3] and significant reductions in healthcare resource utilization (HRU) following berotralstat initiation using Komodo's Healthcare Map claims data.[4]

- This study characterized patients treated with berotralstat for long-term prophylaxis (LTP) of HAE in the linked Optime-Komodo database.

## METHODS

### Data Source

- Optime Care Specialty Pharmacy data (Dec. 3, 2020 – Jan. 31, 2024) was linked with Komodo Healthcare Map administrative claims data (Oct. 1, 2015 – Jan. 31, 2024) at the patient level using de-identified Datavant patient tokens based on patients' date of birth, sex, and first and last name, accounting for spelling variations.

- Optime Care is the sole dispenser of berotralstat in the United States, and the database includes berotralstat shipment information, self-assessments of HAE attacks, and laboratory results (C1 inhibitor [C1INH] levels, C1INH function, and C4 levels) for HAE type identification.

- Komodo Healthcare Map contains pharmacy and medical claims data for more than 320 million individuals in the United States, including information on diagnoses, treatments, and HRU.

### Study Design and Analysis

- This retrospective, real-world study selected patients with ≥2 berotralstat dispensings based on Optime data (first dispensing = index date), who had ≥6 months of continuous insurance eligibility prior to the index date based on Komodo data and were ≥12 years of age at index.

- Patient demographics and clinical characteristics were described during the 6 months pre-index or on the index date.

- Baseline HAE attack rate was calculated from the Optime onboarding assessment. The number of patient-reported attacks in the 90 days prior to berotralstat initiation was divided by 3 to obtain a 30-day attack rate. The maximum rate of HAE attacks that patients could experience was assumed to be 1 attack per 2 days.

- Continuous variables were reported using mean, standard deviation, and median values. Categorical patient characteristics were reported using frequencies and proportions.

## REFERENCES

**1.** Betschel S, et al. *Allergy Asthma Clin Immunol.* 2019;15(1):1-29. **2.** Tachdjian R, et al. Real-World Attack Rates Before and After Berotralstat Initiation Among Patients With Hereditary Angioedema With C1-Inhibitor Deficiency (Type I/II) Stratified by Monthly Baseline HAE Attack Frequency. Presented at AAAAI/WAO Joint Congress; Feb 28-Mar 3, 2025; San Diego, CA. **3.** Davis-Lorton M, et al. Real-World Attack Rates Before and After Berotralstat Initiation Among Patients With Hereditary Angioedema Without C1-Inhibitor Deficiency (HAE-nl-C1-INH) Stratified by Monthly Baseline HAE Attack Frequency. Presented at AAAAI/WAO Joint Congress. Feb 28-Mar 3, 2025; San Diego, CA. **4.** Christiansen S, et al. *J Manag Care Spec Pharm.* 2025;31(6):578-589.
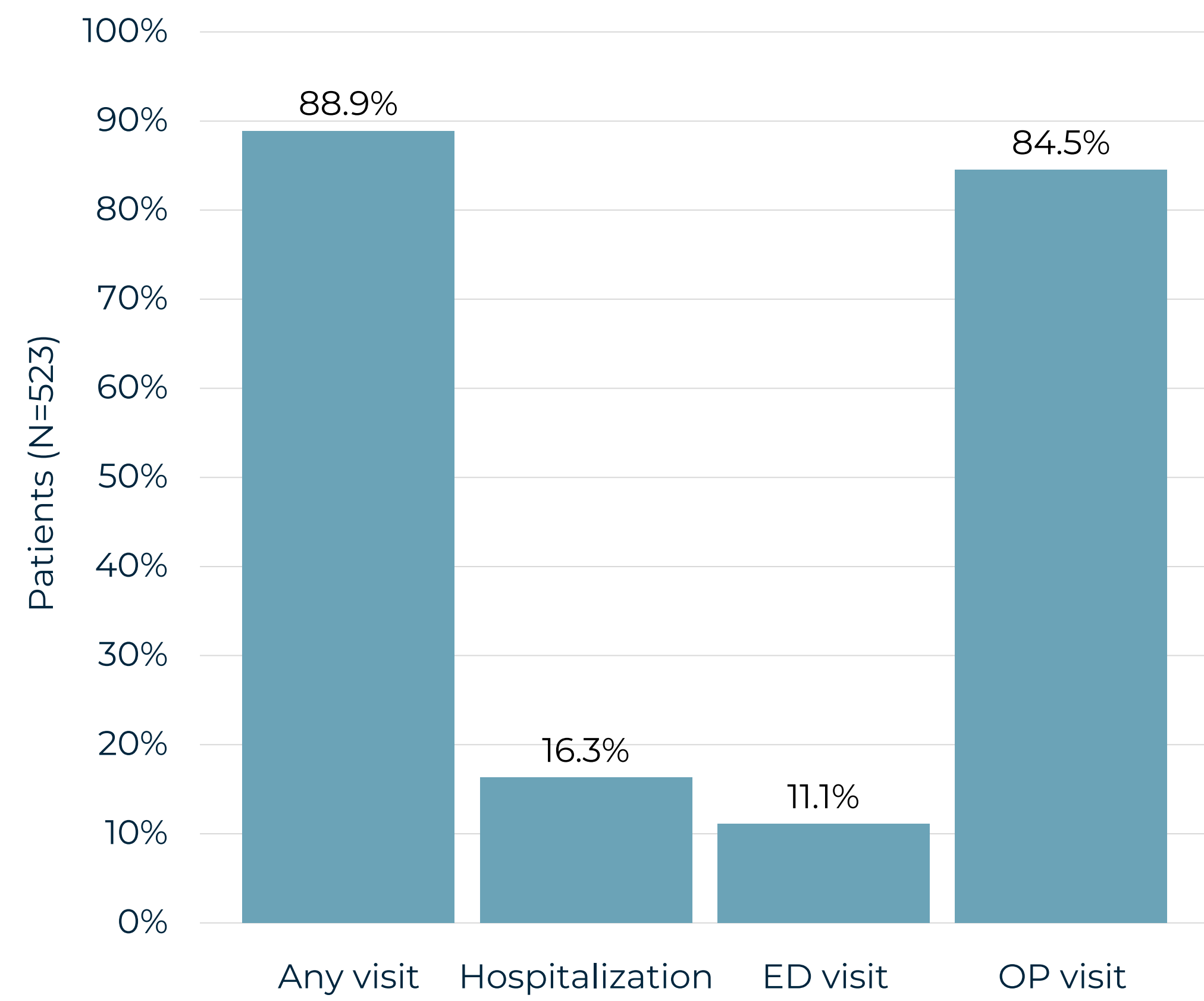
## RESULTS

- The study population comprised 523 patients who met the eligibility criteria (**Figure 1**).

- 96% of patients with berotralstat dispensings in Optime data also had linked Komodo data (**Figure 1**).

- Mean age was 41 years, 75.0% were female, and 69.4% had commercial insurance at index (**Table 1**).

- Among the subset of patients with self-assessments of HAE attacks (n=435), 23.9% had 0 attacks/month, 24.8% had 1 attack/month, 26.4% had 2–4 attacks/month, and 24.8% had ≥5 attacks/month at baseline (**Figure 2**).

- In the 6 months pre-index, 16.3% and 11.1% of patients had an angioedema-related hospitalization and emergency department visit, respectively (**Figure 3**).

- Based on laboratory values in Optime data, 37.5% had C1INH deficiency (HAE type 1 or 2) and 30.8% had HAE with normal C1INH, with the remainder having undetermined HAE type (i.e., laboratory values in database missing or not classifiable) (**Figure 4**).

### Table 1. Demographics and Clinical Characteristics

| Characteristics | Patients (N=523) |
|---|---|
| **Demographics***,[a] | |
| Age, years, mean ± SD [median] | 40.7 ± 16.5 [40] |
| Female, n (%) | 392 (75.0) |
| *Region of residence, n (%)* | |
| South[b] | 223 (42.6) |
| West | 108 (20.7) |
| Midwest | 100 (19.1) |
| Northeast | 92 (17.6) |
| **Insurance plan type**, n (%)[†,a] | |
| Commercial | 363 (69.4) |
| Medicaid | 107 (20.5) |
| Medicare | 52 (9.9) |
| Unknown | 1 (0.2) |
| **Healthcare practitioner specialty**, n (%)*,[a] | |
| Allergist/Immunologist | 484 (92.5) |
| Nurse practitioner | 21 (4.0) |
| Other | 18 (3.4) |
| **Quan-CCI score**, mean ± SD [median][†,c] | 0.54 ± 1.12 [0] |
| **Patients with ≥1 claim for an LTP pre-index**, n (%)[†,d] | 212 (40.5) |
| **Patients with ≥1 claim for an ODT pre-index**, n (%)[†,d] | 289 (55.3) |

CCI, Charlson comorbidity index; LTP, long-term prophylaxis; ODT, on-demand therapy; SD, standard deviation. *Identified from Optime Care Specialty Pharmacy data. †Identified from Komodo Healthcare Map data. [a]Assessed on the index date. [b]South census region includes Puerto Rico. [c]Assessed during the 6-months pre-index. [d]Assessed any time pre-index, from the start of continuous eligibility to the index date.

### Figure 1. Berotralstat Patient Disposition



EAP, expanded access program.
*Continuous health plan enrollment was defined as continuous periods with both medical and pharmacy insurance eligibility.

### Figure 2. Monthly Baseline Attack Frequency



- Patients with ≥5 attacks per month
- Patients with 2–4 attacks per month
- Patients with 1 attack per month
- Patients with 0 baseline attacks per month

[a]Among patients with a baseline self-assessment and ≥1 follow-up self-assessment of attacks

### Figure 3. Healthcare Resource Utilization (6 Months Pre-Index)



ED, emergency department; IP, inpatient; OP, outpatient

### Figure 4. HAE Type



HAE, hereditary angioedema; HAE-C1INH, HAE with C1 inhibitor deficiency; HAE-nC1INH, HAE with normal C1 inhibitor.

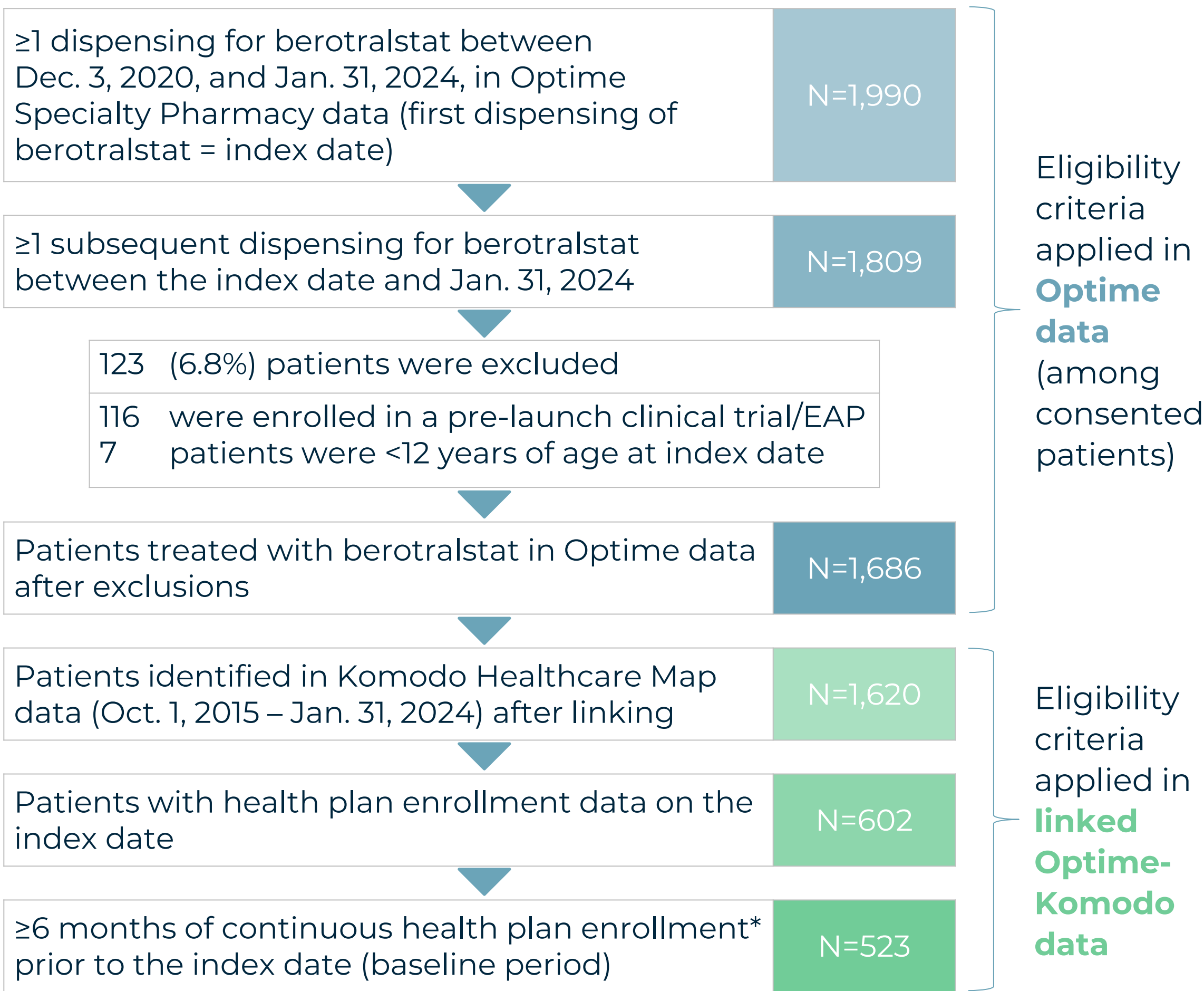## Limitations

- The presence of a pharmacy dispensing in Optime or Komodo data does not indicate that the medication was consumed or that it was taken as prescribed.

- While the Datavant Match method 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.

## CONCLUSIONS

1. The linking of Optime Care Specialty Pharmacy and Komodo Healthcare Map data allows for comprehensive characterization of patients with HAE treated with berotralstat.

2. The linked database will enable future innovative research not otherwise possible in a rare disease to address knowledge gaps in HAE and support decision-m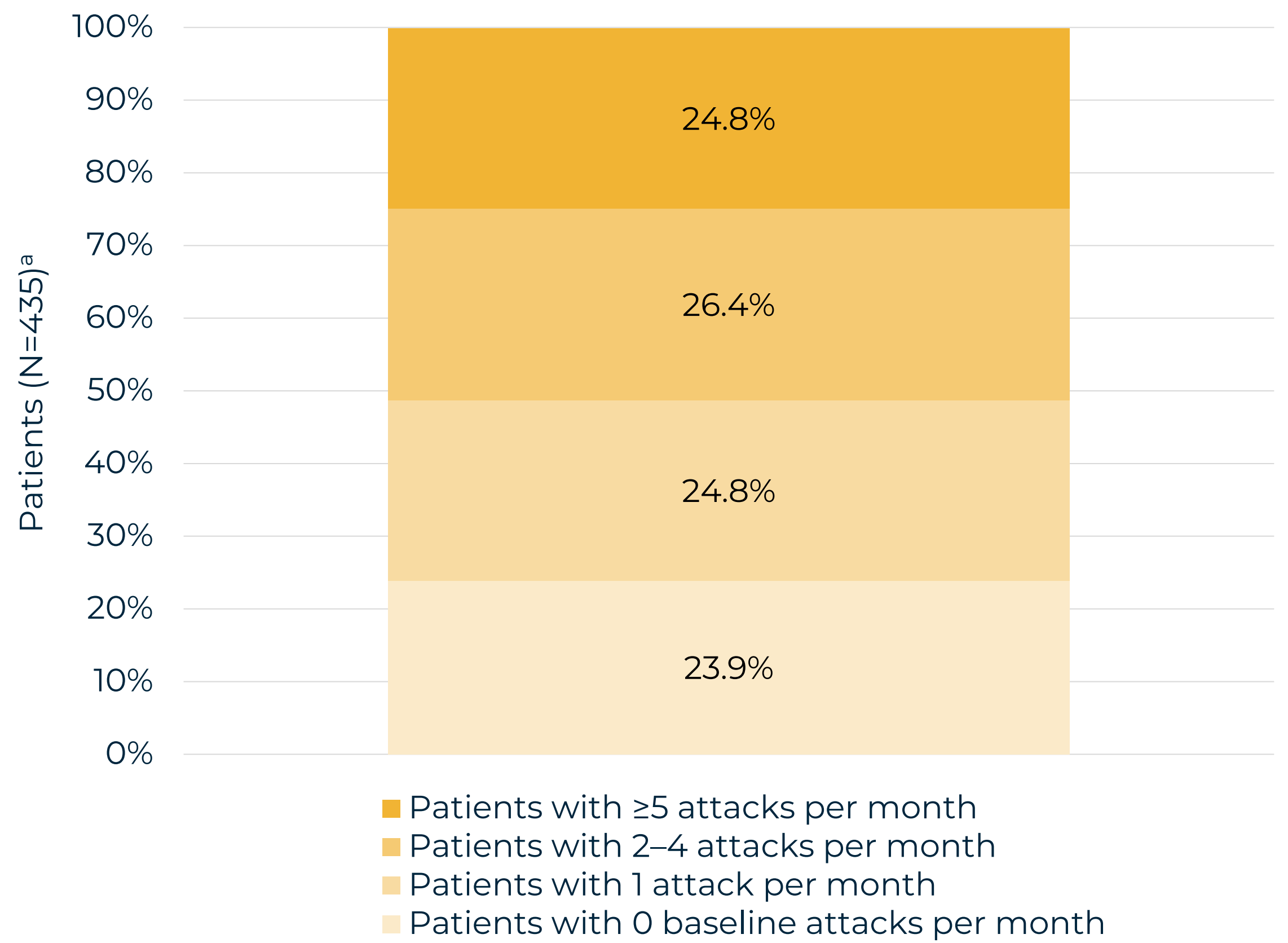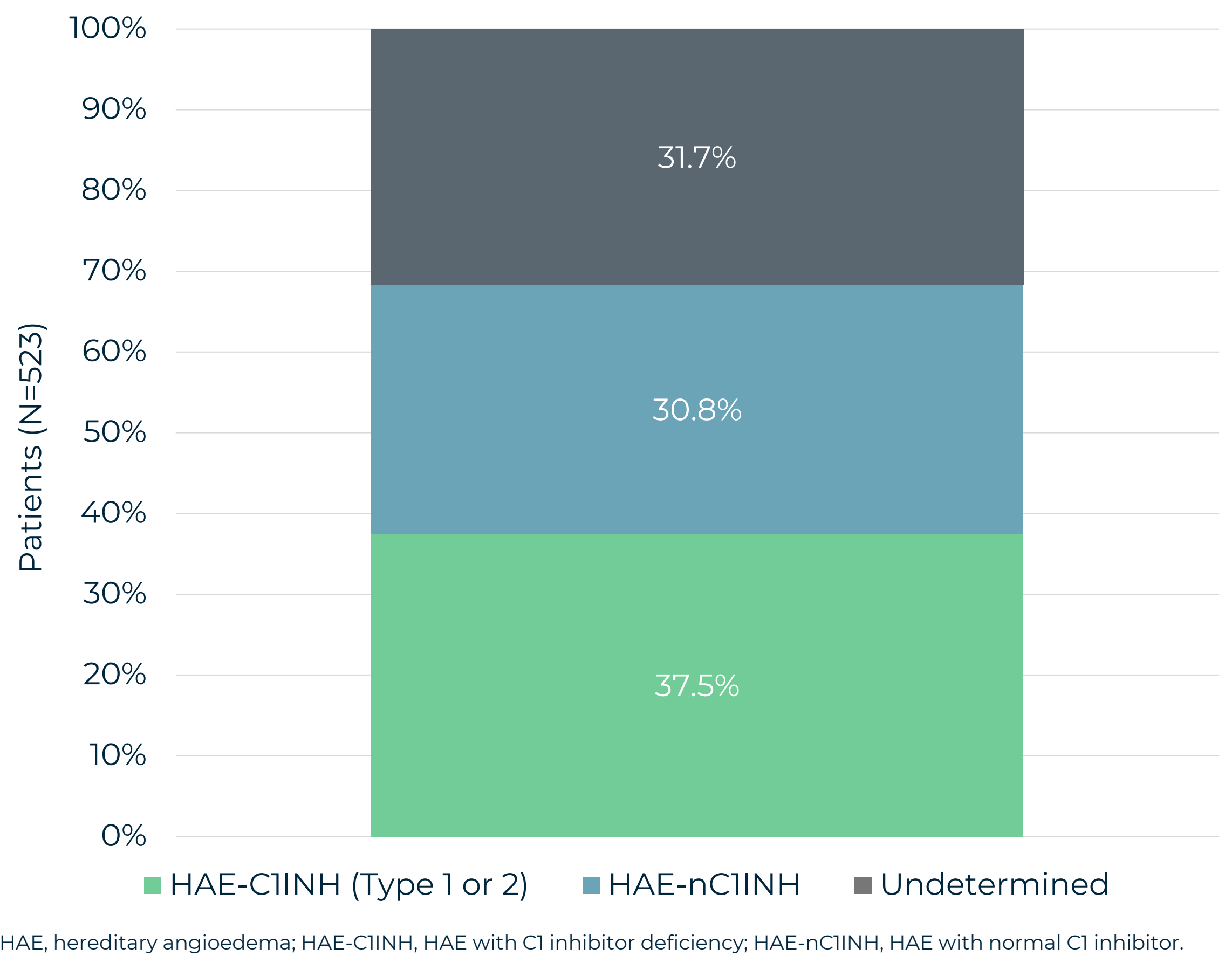aking by healthcare practitioners and payers.