

Real-World Patient Characterization, Prior Long-Term Prophylactic Prescribing Patterns, and Treatment Outcomes for Adults on Berotralstat With Hereditary Angioedema in Japan

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INTRODUCTION

- 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
- In 2021, berotralstat became the first targeted long-term prophylactic (LTP) treatment for HAE approved by the Ministry of Health, Labour and Welfare in Japan.²
- This study aimed to describe patient characteristics, prior treatments, treatment outcomes, and reasons for switching to berotralstat among patients with HAE receiving berotralstat as an LTP in Japan.

METHODS

- Data from the Adelphi HAE Wave II Disease Specific Program (DSP), a real-world cross-sectional survey of physicians who manage patients with HAE in Japan, were assessed.³
- Data were collected between January 2023 and January 2024 for patients aged ≥12 years receiving berotralstat at the time of data collection.
- Physicians were eligible for inclusion if:
 - they specialized as an allergist, allergist-immunologist, dermatologist, pulmonologist, ear, nose, and throat specialist, hematologist, or rheumatologist
 - they managed at least two patients with a diagnosis of HAE in a typical month
 - they treated at least one patient receiving berotralstat.
- Patients were eligible for inclusion if they were aged ≥12 years, had a physician-confirmed diagnosis of HAE, and experienced at least one HAE attack following HAE diagnosis.
- Physicians completed the survey using patients' medical charts alongside clinical judgment, reporting patient demographics, reasons for selecting berotralstat, and the effectiveness of berotralstat.

RESULTS

- In total, three physicians participated in the survey and provided data for 15 adult patients (aged ≥18 years) receiving berotralstat.

Demographics and Clinical Characteristics

- Patient demographics and clinical characteristics are reported in **Table 1**.
 - The mean (standard deviation) age was 44.7 (13.9) years, and 53.3% of patients were male.
 - Overall, 66.7% of patients had HAE Type 1, 26.7% had Type 2, and 6.7% had unknown type.

Table 1. Patient Demographics and Clinical Characteristics

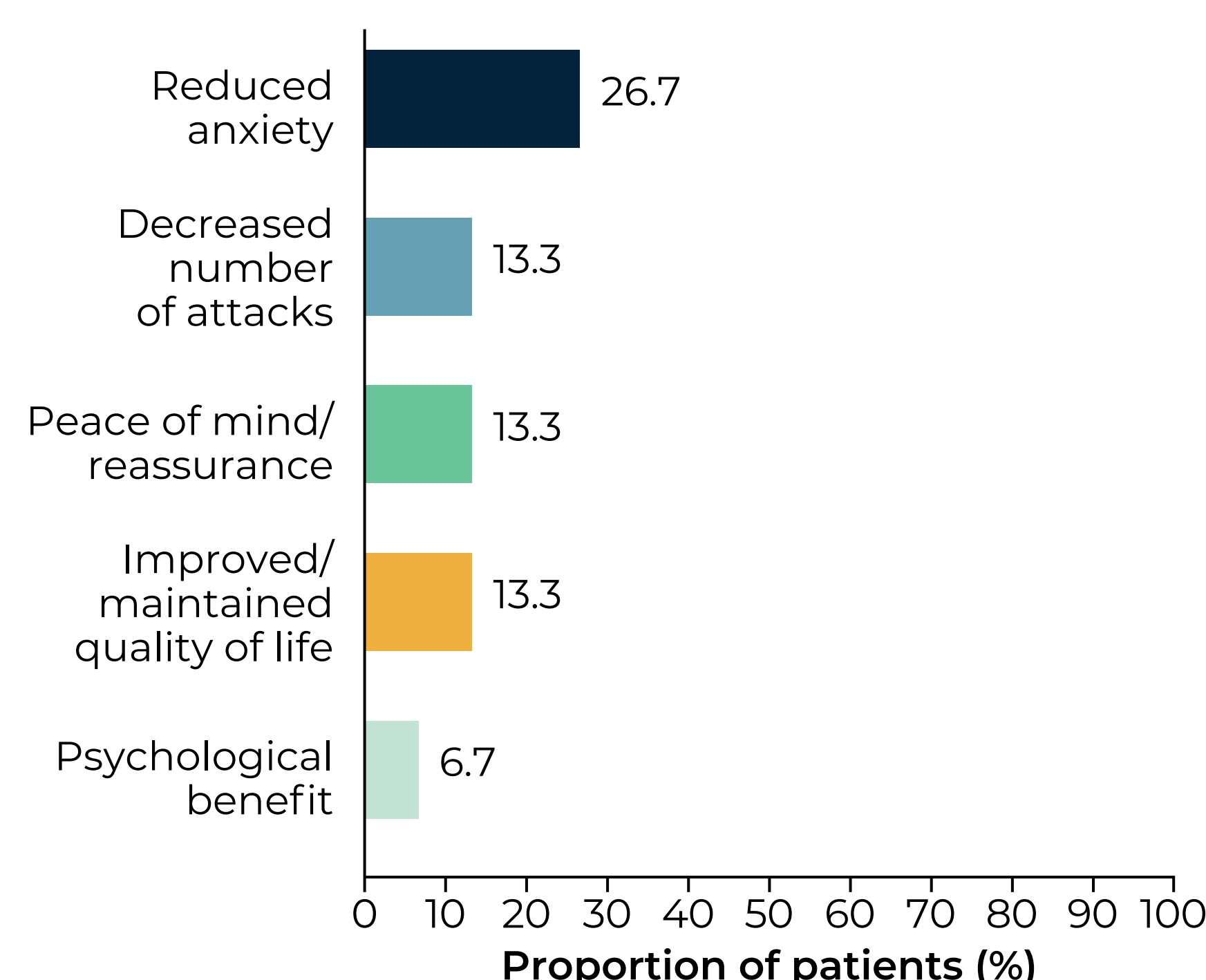
Characteristics	Total patients (N=15)
Age, mean (SD), years	44.7 (13.9)
Male, n (%)	8 (53.3)
BMI, mean (SD), kg/m ²	25.2 (4.2)
HAE type, n (%)	
Type 1	10 (66.7)
Type 2	4 (26.7)
Unknown	1 (6.7)
At least one concomitant condition, n (%)	13 (86.7)
Concomitant conditions, n (%)	
Anemia	5 (33.3)
Hypothyroidism	4 (26.7)
Anxiety	3 (20.0)
Depression	2 (13.3)
Diabetes without chronic complications	2 (13.3)
At least one suspected condition before HAE diagnosis, n (%)	14 (93.3)
Most common suspected conditions before HAE diagnosis, n (%)	
Urticaria	12 (80.0)
Contact dermatitis	4 (26.7)
Irritable bowel syndrome	4 (26.7)
Allergic reaction/allergic angioedema	3 (20.0)
Psychological disorder	2 (13.3)
Employment status, n (%)	
Full-time employment	8 (53.3)
Part-time employment	2 (13.3)
Homemaker	3 (20.0)
Student	2 (13.3)
Living setting, n (%)	
Urban	6 (40.0)
Suburban	6 (40.0)
Rural	3 (20.0)

HAE, hereditary angioedema; SD, standard deviation.

HAE Treatment Goals

- Physicians reported that the most important benefits that HAE treatment could provide for patients were reducing anxiety (26.7%), decreasing the number of attacks (13.3%), peace of mind/reassurance (13.3%), and improving/maintaining quality of life (13.3%) (**Figure 1**).

Figure 1. Most Important HAE Treatment Benefits for Patients Reported by Physicians

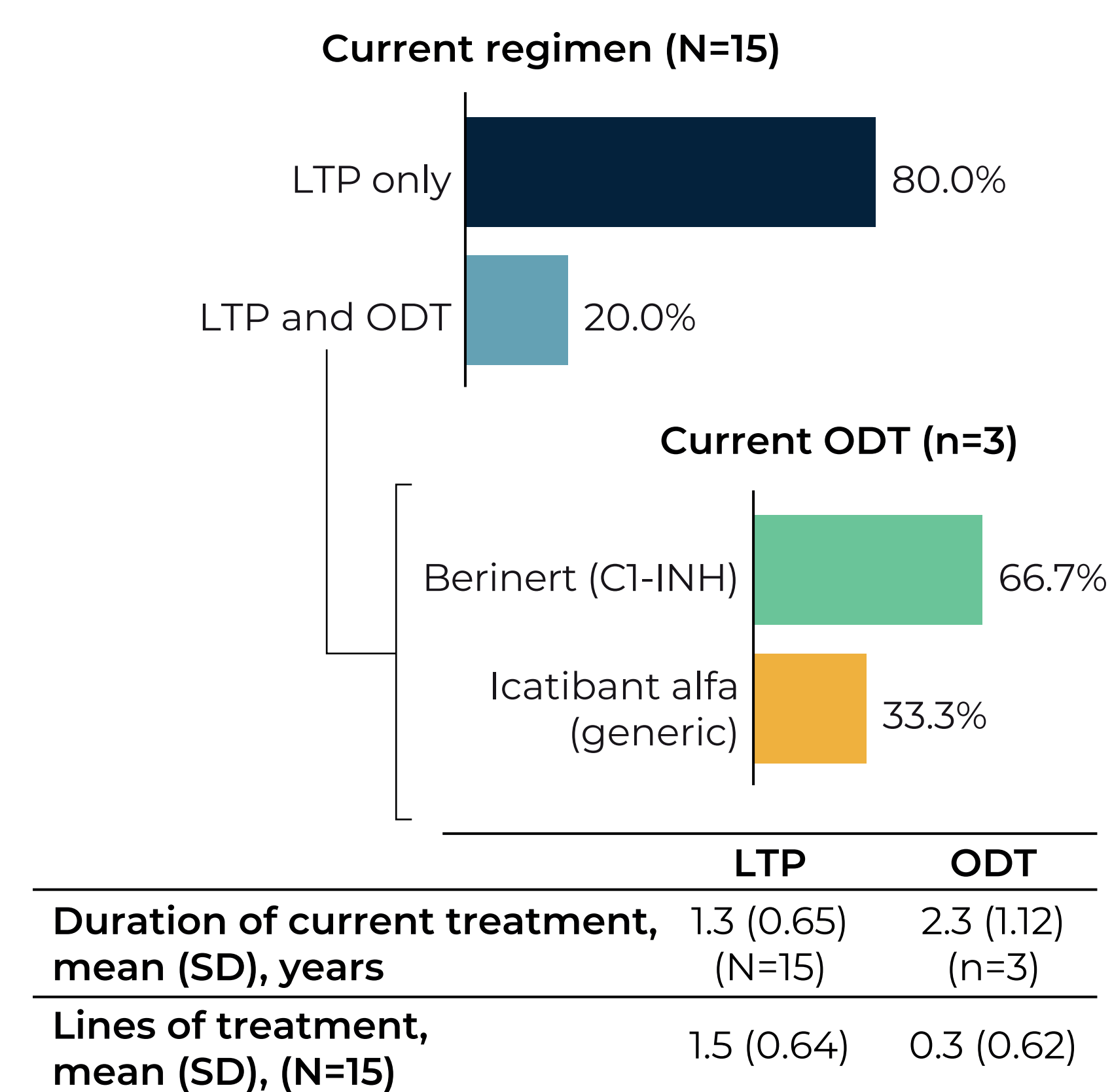


HAE, hereditary angioedema

Current and Prior HAE Treatments

- At the time of the survey, 80% of patients were only receiving an LTP medication (**Figure 2**).

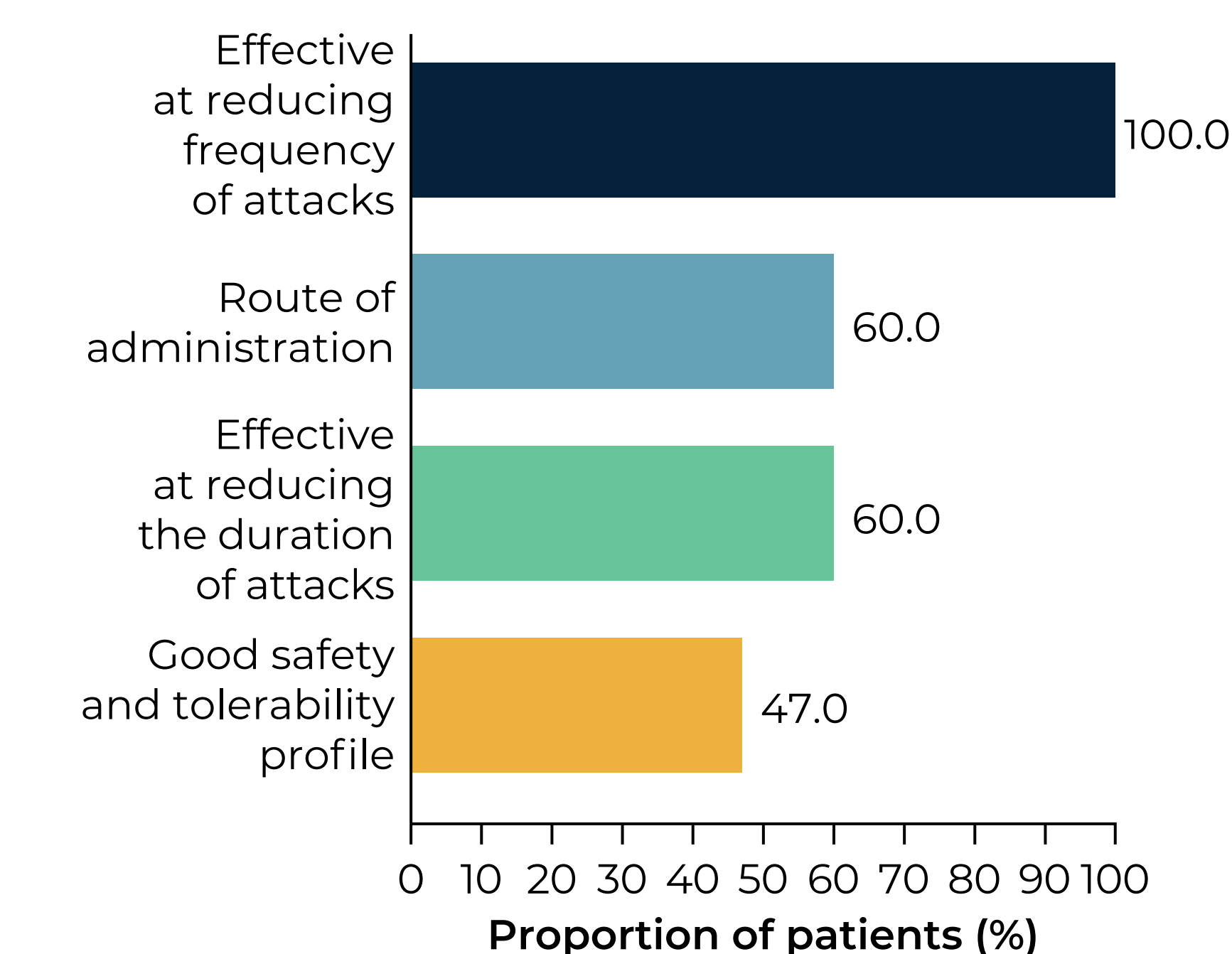
Figure 2. Current Treatment Regimens



C1-INH, C1 esterase inhibitor; LTP, long-term prophylactic; ODT, on-demand treatment; SD, standard deviation.

- Physicians reported that the most common reasons given by patients for initiating berotralstat, irrespective of prior LTP use, were perceived effectiveness in reducing the frequency of attacks (100.0%), the route of administration (60.0%), and effectiveness in reducing the duration of attacks (60.0%) (**Figure 3**).
- Of the patients receiving berotralstat, 86.7% were reported by the physicians as being satisfied or completely satisfied with the overall efficacy.

Figure 3. Most Common Reasons for Patients Choosing Berotralstat Reported by Physicians



- Before initiating berotralstat, 53.3% of patients had no prior LTP use (**Table 2**).
- Among patients with a prior LTP treatment (n=7), 85.7% received tranexamic acid before switching to berotralstat.
- Overall, 66.7% of patients had received berotralstat for ≥12 months.

Table 2. HAE LTP Treatment History

Characteristics	Total patients (N=15)
Prior LTP use, n (%)	
Yes	7 (46.7)
No	8 (53.3)
Prior LTP administration route (n=7), n (%)	
Targeted injectable	0 (0.0)
Nontargeted oral	7 (100.0)
Berotralstat treatment duration, n (%)	
≥6 months	15 (100.0)
≥12 months	10 (66.7)

HAE, hereditary angioedema; LTP, long-term prophylactic.

Limitations

- Owing to the small sample size, the data may not be representative of the overall population of patients with HAE in Japan.
- The data from the Adelphi DSP rely on the willingness of physicians to complete the survey and therefore do not represent a true random sample.

CONCLUSIONS

- Physicians reported that berotralstat was selected by patients in Japan as an LTP for HAE primarily for its effectiveness in reducing the frequency of HAE attacks.
- Upon initiating berotralstat, physicians reported high satisfaction with its effectiveness in reducing the frequency and duration of attacks among their patients.

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