Understanding Patient Reasons Not to Treat All Hereditary Angioedema (HAE) Attacks and Characteristics of Untreated HAE Attacks: Results From a Real-World Survey

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Conflicts of interest disclosure

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Why do people living with hereditary angioedema (HAE) opt to not treat all attacks with on-demand medication?

Clinical guidelines[1-3] recommend people with HAE carry sufficient on-demand treatment for use with all attacks to reduce:

- Progression, morbidity, and duration of symptoms
- Functional disability
- Risks of life-threatening consequences

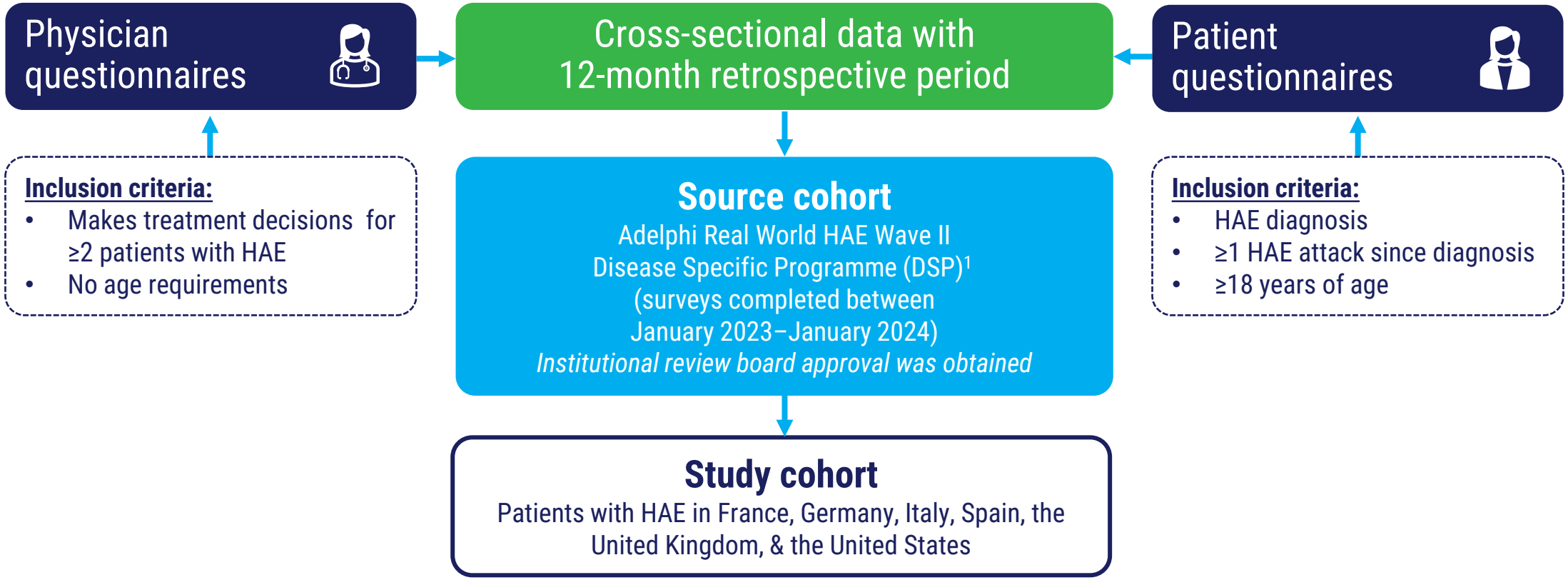
However, people with HAE do **NOT** treat all their attacks[4]

Analysis objectives:

- Understand the reasons why people choose to not treat all their HAE attacks
- Explore physician and patient satisfaction with current on-demand treatment options

1. Busse PJ, et al. *J Allergy Clin Immunol Pract*. 2021;9:132-50.e3. **2**. Maurer M, et al. *Allergy*. 2022;77:1961-90. **3**. Betschel S, et al. *Allergy Asthma Clin Immunol*. 2019;15:72. **4**. US Food and Drug Administration, Center for Biologics Evaluation and Research. The voice of the patient – hereditary angioedema. May 2018. https://www.fda.gov/media/113509/download. Accessed March 4, 2024.

Disease Specific Programme™ (DSP™) methodology



HAE, hereditary angioedema. **1**. Anderson P, et al. *Curr Med Res Opin*. 2023;39:1707-15.

Baseline demographics and clinical characteristics

	Physician reported[a] (N=1027)	Patient reported (N=260)
Age (years), mean ± SD	34.2 ± 14.5	33.6 ± 13.0
Female, n (%)	542 (52.8)	131 (50.4)
Patients in employment or education, n (%)	**(n=1018)** 919 (90.3)	**(n=259)** 235 (90.7)
Number of comorbidities, mean ± SD	0.6 ± 0.9	0.5 ± 0.8
Time (years) since diagnosis, mean ± SD	**(n=944)** 7.3 ± 8.1	**(n=244)** 8.0 ± 7.7
HAE type,[b] n (%)	**(n=1001)**	**(n=258)**
Type 1	770 (76.9)	190 (73.6)
Type 2	188 (18.8)	56 (21.7)
HAE with normal C1-INH	43 (4.3)	12 (4.7)
Prescribed long-term prophylaxis treatment, n (%)	620 (60.4)	150 (57.7)
Prescribed ODT, n (%)	789 (76.8)	196 (75.4)
Number of HAE attacks in the 12 months prior to data collection, mean ± SD	**(n=1027)** 2.2 ± 3.2	**(n=239)** 2.6 ± 2.7

C1-INH, C1-inhibitor; HAE, hereditary angioedema; ODT, on-demand treatment; SD, standard deviation. [a]196 physicians reported data for 1027 patients. [b]Unknown HAE type excluded from base.

On-demand treatments were widely prescribed to patients with HAE, but nearly 25% of those surveyed did not have a current prescription

	Physician reported[a] (N=1027)	Patient reported (N=260)
Prescribed ODT, n (%)	789 (76.8)	196 (75.4)
Icatibant (branded or generic)	498 (63.1)	98 (50.0)
Plasma-derived C1-INH concentrate	222 (28.1)	64 (32.7)
Recombinant human C1-INH concentrate	93 (11.8)	28 (14.3)
Ecallantide	14 (1.8)	3 (1.5)
Other treatments	8 (1.0)[b]	3 (1.5)[c]
Time (years) receiving current ODT, mean ± SD	**(n=734)** 4.0 ± 4.0	**(n=187)** 4.7 ± 4.3

C1-INH, C1-inhibitor; HAE, hereditary angioedema; ODT, on-demand treatment; SD, standard deviation. More than one ODT can be prescribed. [a]196 physicians reported data for 1027 patients. [b]Other treatments used include steroids (n=3), antihistamine (n=2), human C1-INH concentrate (n=2), and epinephrine (n=1). [c]Other treatments used include antihistamine (n=1), human C1-INH concentrate (n=1), and steroids (n=1).

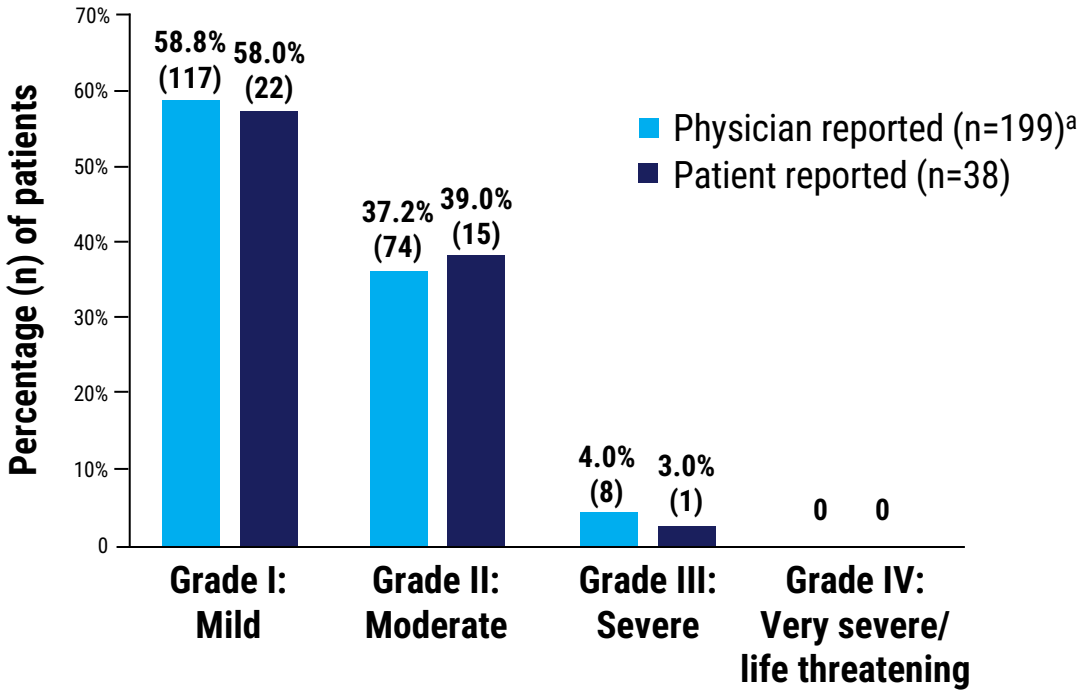
The majority of patients with HAE treated their most recent attack, but 15–20% of patients did not

	Physician reported (N=1027)[a]	Patient reported (N=260)
Did patients treat their most recent attack?,[b] n (%)	**(n=976)**	**(n=250)**
Yes	775 (79.4)	212 (84.8)
No	201 (20.6)	38 (15.2)
Time (minutes) waited before administering ODT,[b] median (IQR)	NR	**(n=209)** 0.0 (0.0–12.5)
Time (minutes) to symptom improvement,[b] median (IQR)	NR	**(n=202)** 30.0 (20.0–60.0)
ODT used to treat the attack,[b] n (%)	**(n=775)**	**(n=210)[c]**
Icatibant (branded or generic)	428 (55.2)	101 (48.1)
Plasma-derived C1-INH concentrate	240 (31.0)	74 (35.2)
Recombinant human C1-INH concentrate	72 (9.3)	26 (12.4)
Ecallantide	12 (1.5)	2 (1.0)
Other treatments	27 (3.5)[d]	8 (3.8)[e]

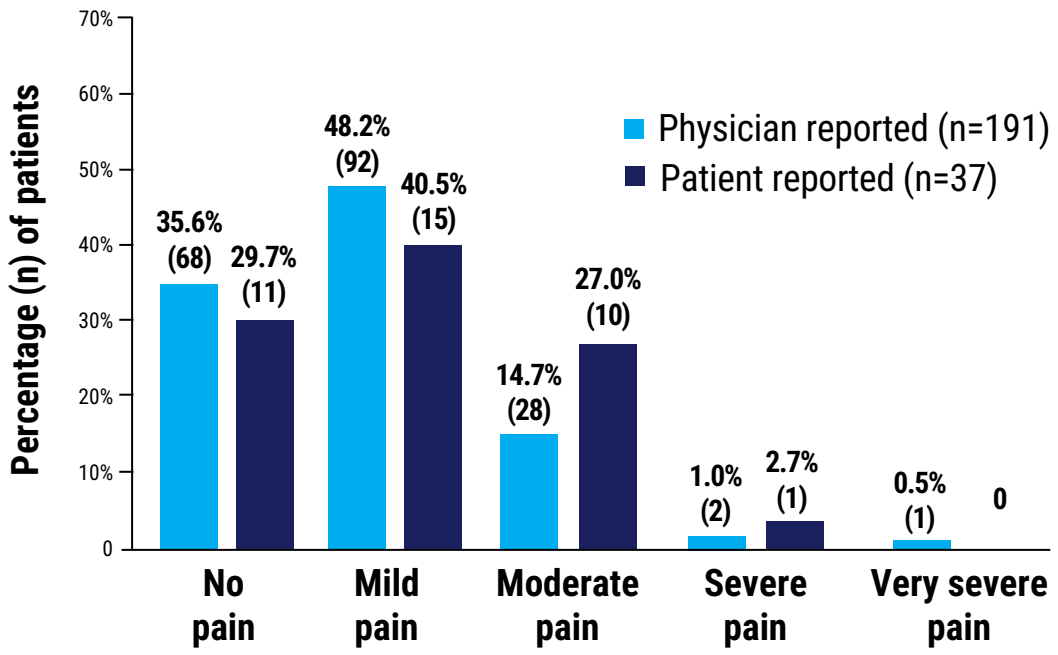
C1-INH, C1-inhibitor; HAE, hereditary angioedema; IQR, interquartile range; NR, not recorded; ODT, on-demand treatment. [a]196 physicians reported data for 1027 patients. [b]Unknown responses excluded from base. [c]One patient used 2 treatments, and 1 patient used 3 treatments. [d]Other treatments used include steroids (n=10), antihistamine (n=8), danazol (n=3), human C1-INH concentrate (n=3), epinephrine (n=2), tranexamic acid (n=2), non-steroidal anti-inflammatory drug (n=1), and prednisone (n=1). [e]Other treatments used include antihistamine (n=4), danazol (n=2), human C1-INH concentrate (n=1), and steroids (n=1).

Untreated HAE attacks were primarily of mild to moderate severity, with most patients experiencing no or mild to moderate pain

Severity of untreated attacks



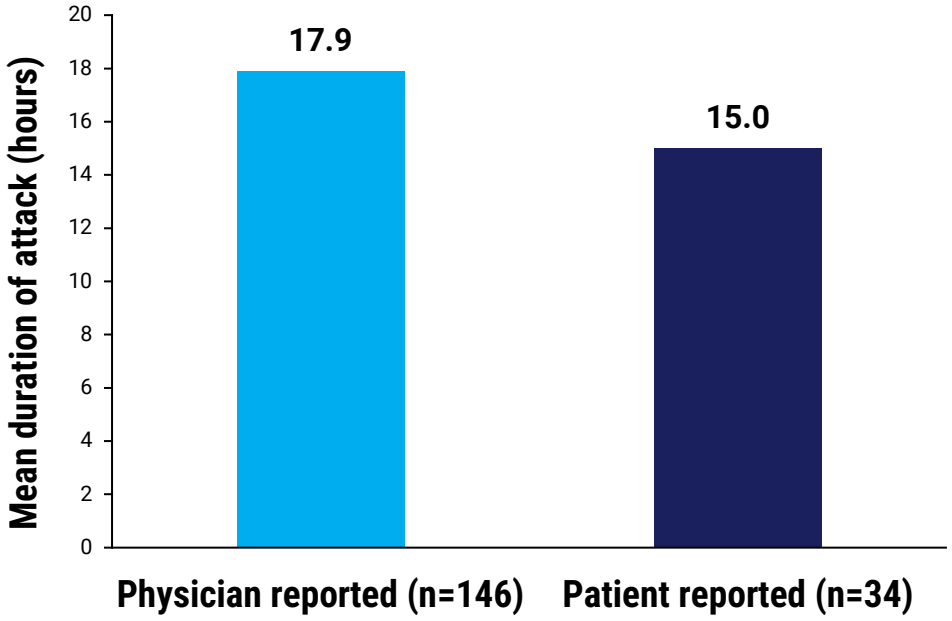
Pain experienced during untreated attacks



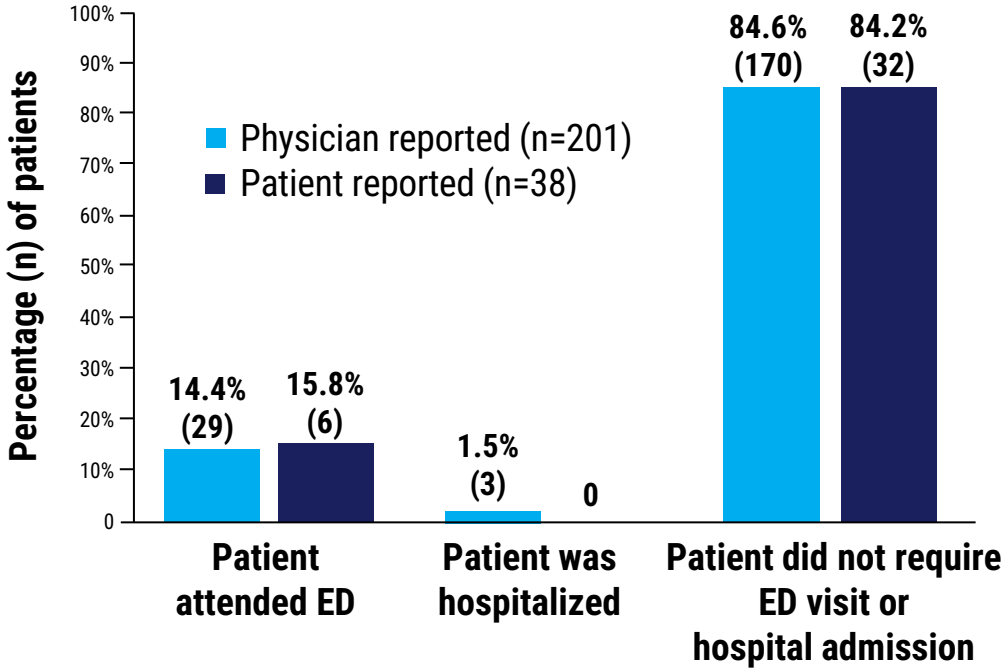
HAE, hereditary angioedema. Only the last attack for each patient is reported. [a]n=2, no response, excluded from base.

The mean duration of untreated HAE attacks was 15–18 hours and ~15% of these attacks prompted hospital treatment

Duration of untreated attack

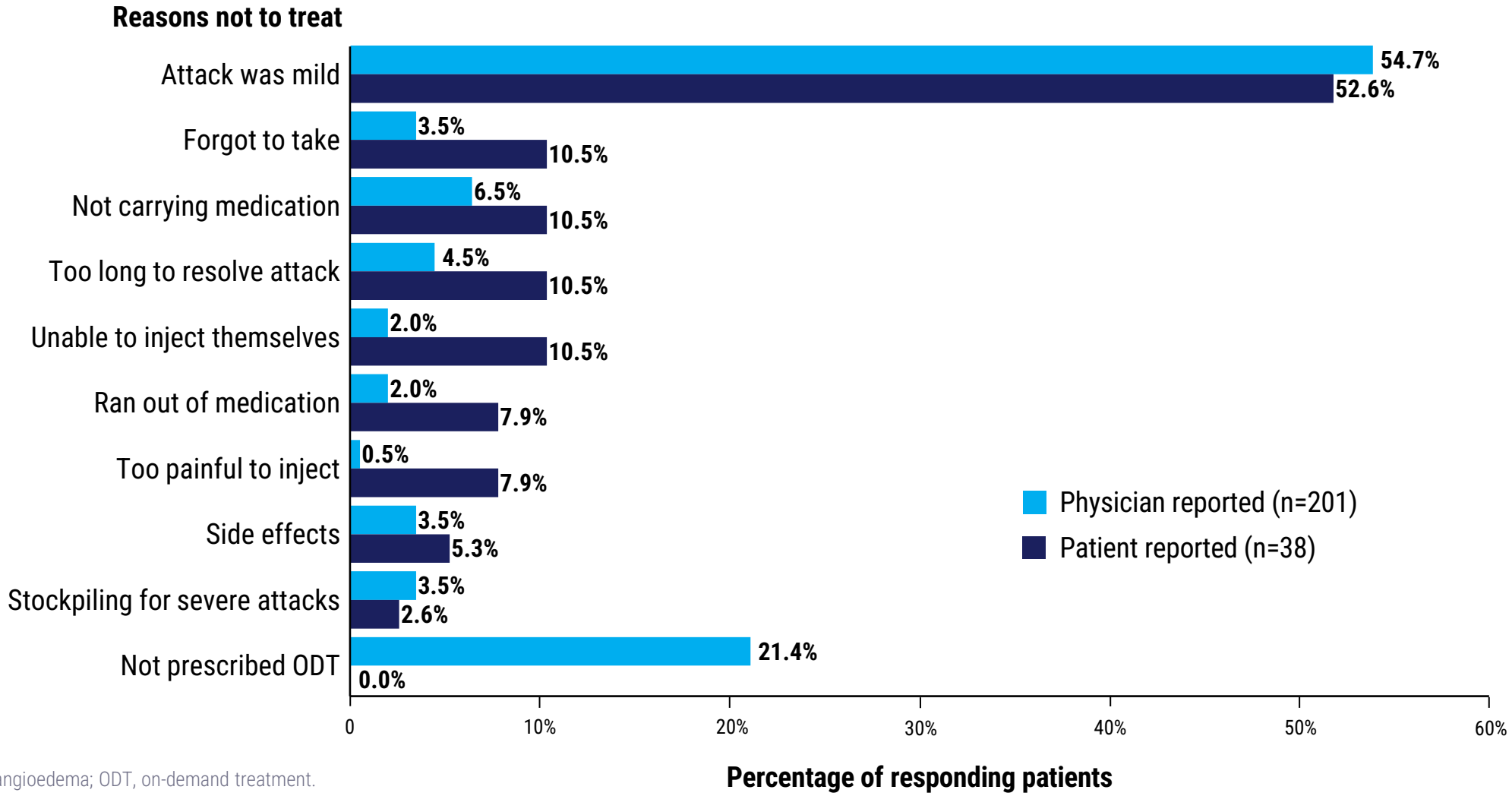


Use of hospital resources for untreated attacks



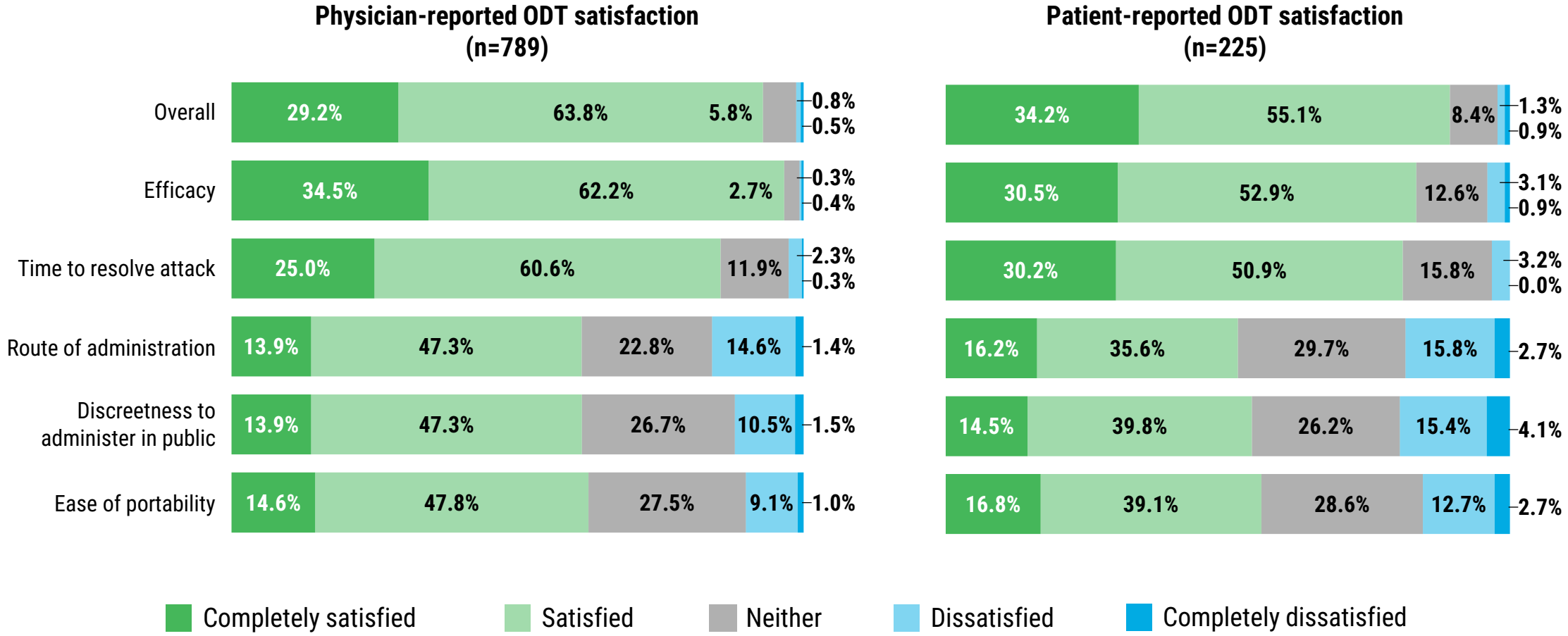
ED, emergency department; HAE, hereditary angioedema. Only the last attack for each patient was reported.

HAE attacks went untreated mainly due to perceived low attack severity or medication not available, taking a long time to resolve, or difficult to use



HAE, hereditary angioedema; ODT, on-demand treatment.

Primary reasons for lack of satisfaction with on-demand treatments included route of administration, discreetness, and portability



ODT, on-demand treatment.

Current on-demand medications for HAE have drawbacks that contribute to patients opting to not treat their attacks

- Factors impacting the choice not to treat:
  - Patient perceived attack symptoms to be mild
  - Patient forgot to take or did not have on-demand treatment with them
  - Patient felt time needed for medication to resolve attack was too long
  - Patient was unable to self-inject or medication was too painful to inject
- In the absence of novel alternatives, most patients who treated their attack were satisfied overall with their HAE treatment. However, almost half of these patients were not satisfied with their on-demand treatment's route of administration, discreetness, or portability
  - These aspects represent barriers to patient compliance with medical guidelines to promptly treat all HAE attacks
- Expanding the available treatment options and increasing on-demand treatment prescription and use for all HAE attacks may reduce HAE attack severity/duration and the need for emergency healthcare services
- Timely treatment of all HAE attacks may be facilitated by the availability of oral on-demand therapies

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DSP, Disease Specific Programme; HAE, hereditary angioedema.