

Characterizing the Perspective of Patients With HAE on Prophylactic Treatment

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Background

- Hereditary angioedema (HAE) is a rare genetic disease resulting in deficiency (type I) or dysfunction (type II) in the C1-inhibitor protein and subsequent uncontrolled activation of the kallikrein-kinin system
- People living with HAE experience unpredictable, painful and debilitating attacks of tissue swelling in various locations of the body that can be life-threatening depending on the location affected
- WAO/EAACI 2021 updated guidelines recommend that all HAE patients¹
 - consider treating all attacks as early as possible
 - have access to sufficient medication to treat two attacks
 - carry on-demand treatment at all times, regardless of prophylactic therapy
- Patients on prophylaxis still face the risk of having an attack and continue to experience burden in their day-to-day life and challenges associated with parenteral on-demand therapy

Objective

- The objective of this analysis was to characterize the patient perspective and experience related to on-demand therapy among those living with HAE on prophylactic HAE treatment

Methods

- The US Hereditary Angioedema Association (HAEA) recruited people living with HAE to complete an online survey
 - Recruitment was stratified to include 50% of patients taking on-demand only and 50% receiving LTP plus on-demand
- The survey was self-reported and took respondents approximately 20 minutes to complete
- The survey was completed by 107 individuals between September 6 and October 19, 2022; response rate 69% (107/155)
- Respondents provided consent for their data to be used anonymously or in aggregate
- Analysis was performed using descriptive statistics

References

1. Maurer M., et al. The international WAO/EAACI guideline for the management of hereditary angioedema - The 2021 revision and update. World Allergy Organ J. 2022 Apr 7;15(3):100627.

Disclosures

This study was sponsored by KalVista Pharmaceuticals. All authors met the ICMJE authorship criteria and had full access to relevant data. The authors had full editorial control of the data presented and provided final approval of all content. Neither honoraria nor payments were made for authorship.

Stephen Betschel - Speaker/Consultant: CSL Behring, Green Cross, Ionis, Octapharma, and Shire/Takeda, and KalVista Pharmaceuticals, Inc. Cristine Radojicic- Scientific Advisory Board: KalVista, CSL Behring, Takeda, BioCryst Sally van Kooten and Markus Heckmann - Employees of KalVista Pharmaceuticals Neil Malloy - Consultant fees from KalVista Pharmaceuticals Julie Ulloa - Consultant fees from KalVista Pharmaceuticals William Lumry - Consultant Arrangements: Astra, BioCryst, Biomarin, CSL Behring, Express Scripts/CVS, Fresenius Kabi, Intellia, KalVista, Magellan, Optum, Pharming, Pharisar, Shire/Takeda, Speakers' Bureau: BioCryst, CSL Behring, Optinose, Pharming, Shire/Takeda, Grifols, Astra Zeneca, Sanofi/Regeneron, GSK; Current Grants/Research Support: Astra, BioMarin, CSL Behring, Grifols, Ionis, KalVista, Shire/Takeda, Teva; Board Membership: US Hereditary Angioedema Association Medical Advisory Board, DFW Metroplex Allergy Society; Expert Witness: Vedder-Price; Henkel v. Reliastar and ESI, Murphy & King; Wellforce v. ANICO, Dorsey & Whitney; LGH v. Optum

Results

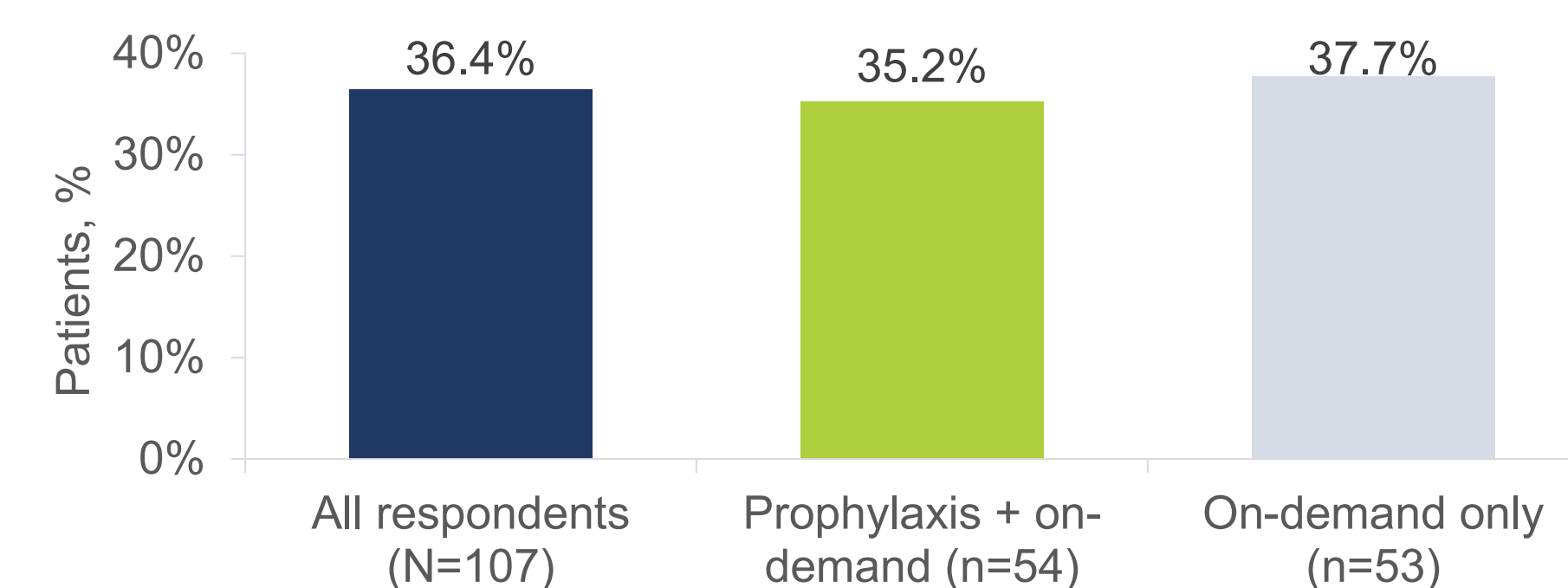
- Respondents included 107 Type I/II HAE patients, mean age 41 years (range 16-83); 50% using on-demand therapy only, 50% using prophylaxis + on-demand therapy (Table 1)
- Most common prophylactic treatment was lanadelumab injection (57.4%)

Table 1. Respondent characteristics

Characteristic	Total (N=107)
Age, mean (range), years	41 (16-83)
Gender, n (%)	
Female	86 (80.4)
Male	21 (19.6)
Type of therapy, n (%)	
On-demand only	53 (49.5)
Prophylaxis and on-demand	54 (50.5)
On-demand treatments used	
Icatibant	84 (78.5)
C1 esterase inhibitor (recombinant)	13 (12.1)
C1 esterase inhibitor (human)	9 (8.4)
Ecallantide	1 (0.9)
Prophylactic treatments used, n (%) of those using prophylaxis (n=54)	
Lanadelumab	31 (57.4)
Bertralstat	7 (13.0)
C1 esterase inhibitor (subcutaneous)	7 (13.0)
Androgens/steroids	5 (9.3)
C1 esterase inhibitor (intravenous)	4 (7.4)

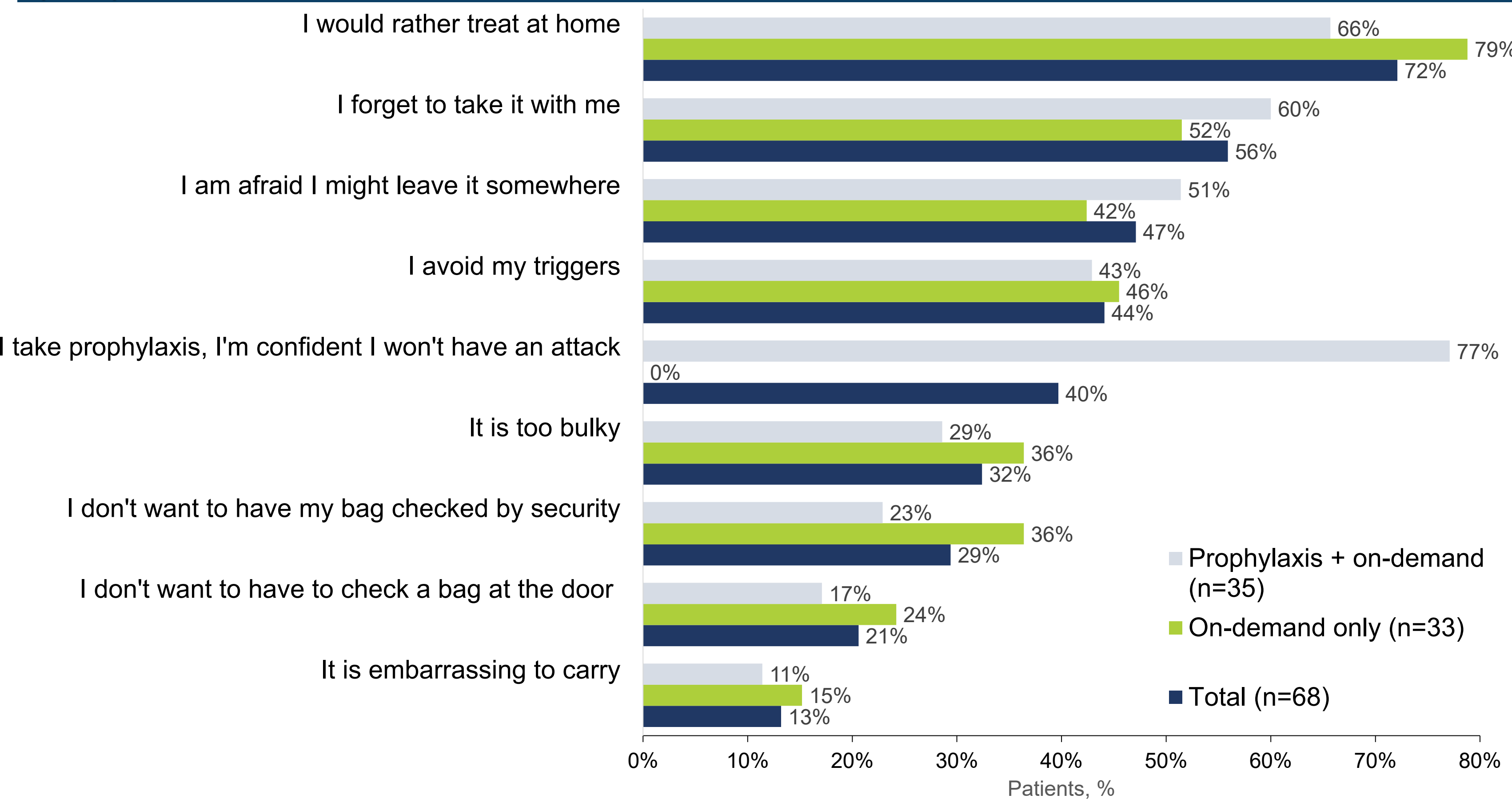
- Only 35.2% of prophylaxis patients always carry on-demand treatment when away from home, as part of day-to-day life (Figure 1)

Figure 1. Proportion of respondents who always carry their HAE on-demand treatment when they are away from home, as part of day-to-day life



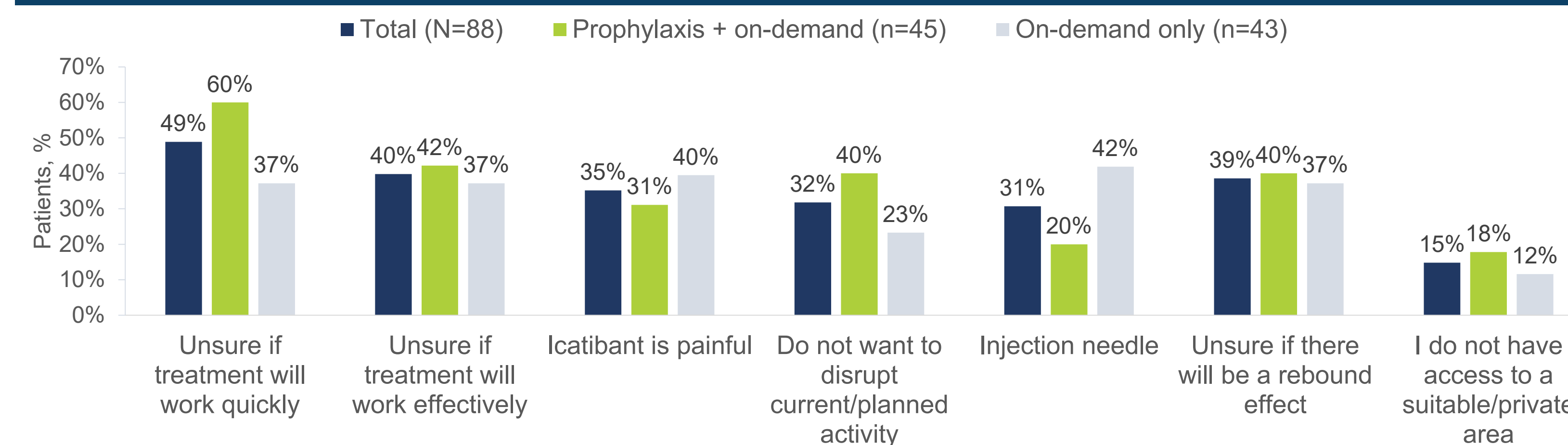
- Various reasons were given for not carrying on-demand treatment at all times, including 43% of prophylaxis patients avoiding potential triggers (Figure 2)

Figure 2. Reasons given for not carrying on-demand treatment at all times, by those who do not always carry (n=68)



Survey participants who indicated that they do not always carry an HAE on-demand treatment (n=68) were asked to select from a list of reasons they do not take on-demand treatment when away from home

Figure 3. Most common reasons* provided for experiencing anxiety when anticipating on-demand treatment



*Participants who reported having any degree of anxiety when anticipating on-demand treatment (>1 on scale; n=88) were asked to rank the top two reasons why their on-demand treatment makes them feel anxious.

- Time to feel fully recovered from an HAE attack following on-demand treatment was 1.7 days for patients taking prophylaxis and 1.6 days for those taking on-demand treatment only
- Common reasons for experiencing anxiety when anticipating on-demand treatment were uncertainty about whether on-demand treatment would work quickly (49%) or be effective (40%) (Figure 3)

Table 2. Reported level of anxiety when anticipating on-demand treatment, %

	Total (n=107)	Prophylaxis + On-demand (n=54)	On-demand Only (n=53)
'Extremely anxious' (7-10 on scale of 0 to 10)	32.7	35.2	30.2
'Moderately anxious' (4-6 on scale of 0 to 10)	15.9	16.7	15.1
'Mildly anxious' (1-3 on scale of 0 to 10)	33.6	31.5	35.8
'Not anxious' (0 on scale of 0 to 10)	17.8	16.7	18.9

Participants were asked, "Please rate your level of anxiety when you anticipate using your current on-demand treatment," and provided a scale to select from 0-Not anxious to 10-Extremely anxious.

- Overall, 48.6% of patients reported feeling moderately to extremely anxious when anticipating their on-demand treatment administration (Table 2); this proportion was even higher (57.2%) among those taking bertralstat.

Conclusions

- Only 35.2% of prophylaxis patients always carried on-demand treatment when away from home while 43% of prophylaxis patients cited avoiding potential triggers as a reason for not carrying on-demand treatment at all times
- Time to attack resolution after on-demand treatment was similar for patients using prophylaxis and those using on-demand treatment only
- 48.6% of prophylaxis patients experienced moderate to extreme levels of anxiety when anticipating on-demand treatment administration, often associated with uncertainty whether their on-demand treatment would work quickly or be effective

Presented at:

American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting - February 23-26, 2024, Washington, DC

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