

Quality of Life Among Prophylaxis Plus On-Demand and On-Demand Only Therapy Users in HAE

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Background

- Hereditary angioedema (HAE) is characterized by unpredictable swelling attacks affecting cutaneous and submucosal tissues, which are often painful and debilitating
- While long-term prophylaxis (LTP) has been shown to reduce the frequency of HAE attacks, the burden of these attacks and the impact on quality of life (QoL) have not been fully described
- The current analysis examined the impact of HAE attacks on QoL in patients receiving on-demand therapy only or a combination of LTP and on-demand therapy

Methods

- People with Type 1 and 2 HAE were recruited between April and June 2023 by the US Hereditary Angioedema Association
 - The study aimed to recruit approximately 80% adults and 20% adolescents
- Participants completed a 20-minute, self-reported, online survey that asked about their last treated HAE attack
- Participants ≥12 years old, who had treated ≥1 HAE attack within the prior 3 months with an approved on-demand treatment were included. Quality of life (QoL) was assessed at the time of the last treated attack and "today" (i.e., current QoL), utilizing the index score from the EuroQoL Five-Dimensions Five-Levels (EQ-5D-5L)
- Descriptive analyses were performed

Results

Table 1. Participant Demographics at the Time of Survey

	Total (n = 94)	On-Demand Only (46% n = 43)	Long-Term Prophylaxis (54% n = 51)
Current Mean Age, (SD)	39.4 (17.4)	42.6 (18.7)	36.7 (15.8)
Mean Age of Diagnosis, Years (SD)	18 (12.6)	19 (12.7)	17 (12.5)
Gender			
Male	28%	23%	31%
Female	72%	77%	69%
Race/Ethnicity			
White	87%	91%	84%
Hispanic or Latino	9%	2%	14%
Black/African American	3%	2%	4%
American Indian or Alaskan Native	2%	2%	2%
Asian	3%	5%	2%
Other	1%	-	2%
HAE Type			
Type 1	81%	79%	82%
Type 2	19%	21%	18%

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Disclosures

Maeve O'Connor reports speaker/consultant/advisor or research: KalVista, Pharming, CSL, GSK, Blueprint, TEVA, AZ, Sanofi, Grifols, Abbvie. Paula Busse reports receiving consulting fees from Takeda, KalVista, CVS Specialty, BioCryst, CSL, Behring, ADARx, Astra, Pharvaris. Sandra Christiansen reports advisory boards: KalVista, BioCryst, US HAEA Medical Advisory Board. Cristine Radojicic reports honorarium from the following participation: Medical Advisory Board- KalVista, BioCryst, CSL Behring, Astra, Safety Monitoring Board- Astra, Speakers Bureau- CSL Behring. Julie Ulloa and Sherry Danese have received consulting fees from KalVista. Vibha Desai and Paul Audhya are employees of KalVista Pharmaceuticals.

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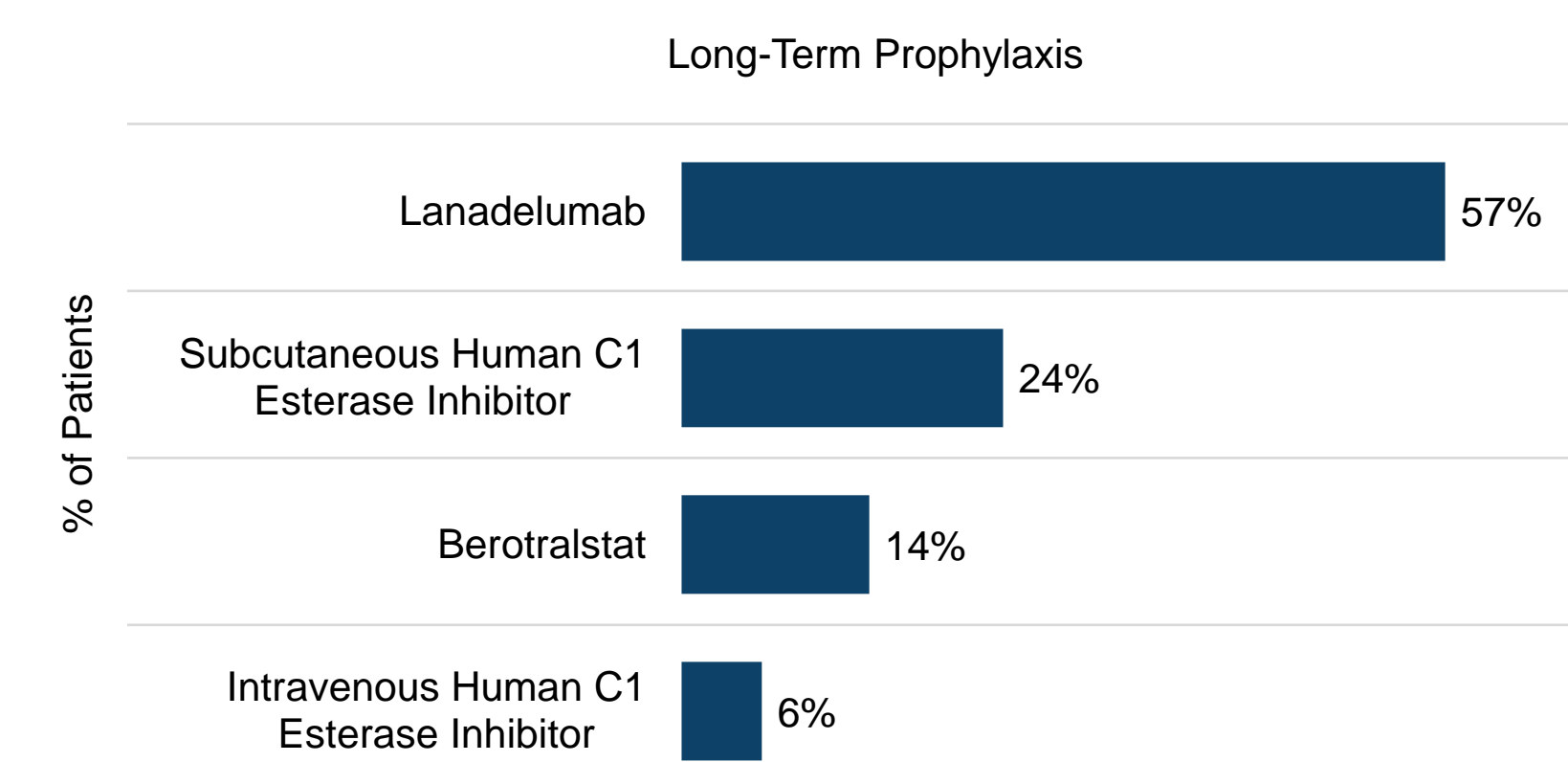
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Results

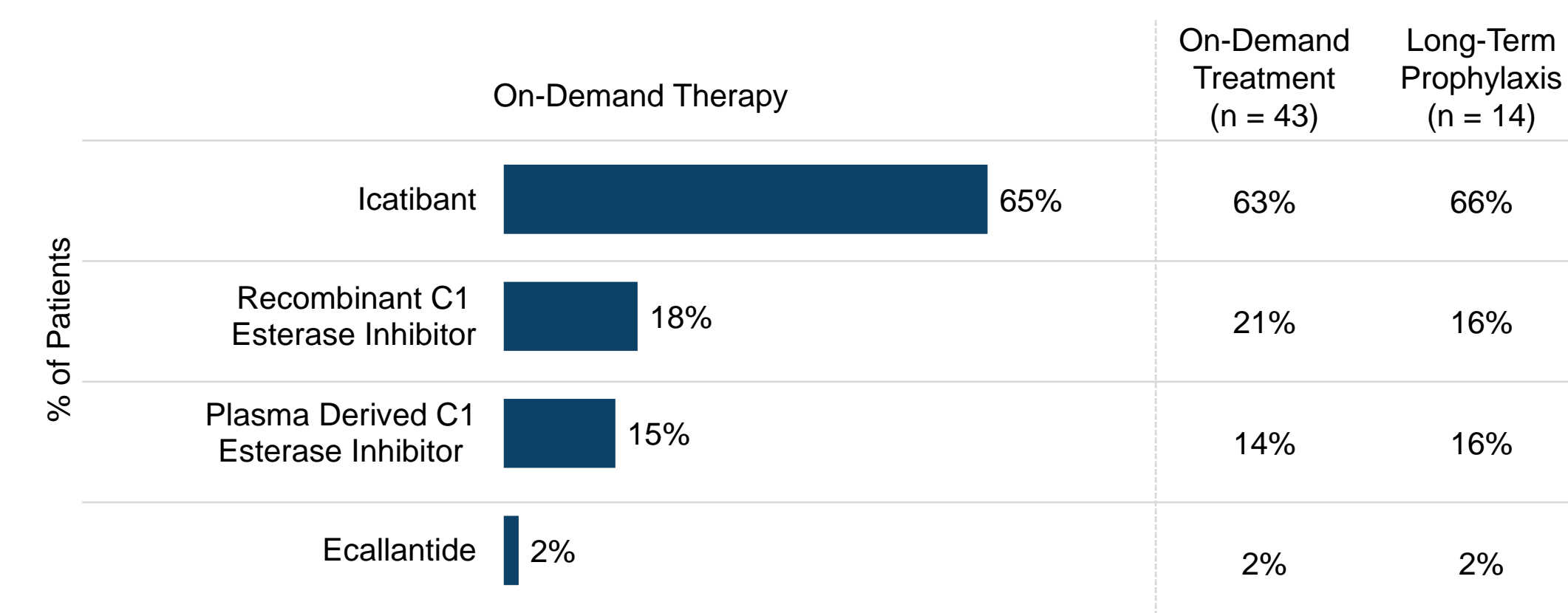
- Respondents included 80 adults and 14 adolescents with an average age of 39 years (Table 1)
- Respondents were predominantly female (72%) and White (87%)
- At the time of their most recent treated attack, 54% of participants were on prophylaxis, and 46% were using on-demand treatment only
- Most of the participants (81%) reported having Type 1 HAE

Figure 1. Long-Term Prophylaxis at Time of Last Treated Attack (n = 51)



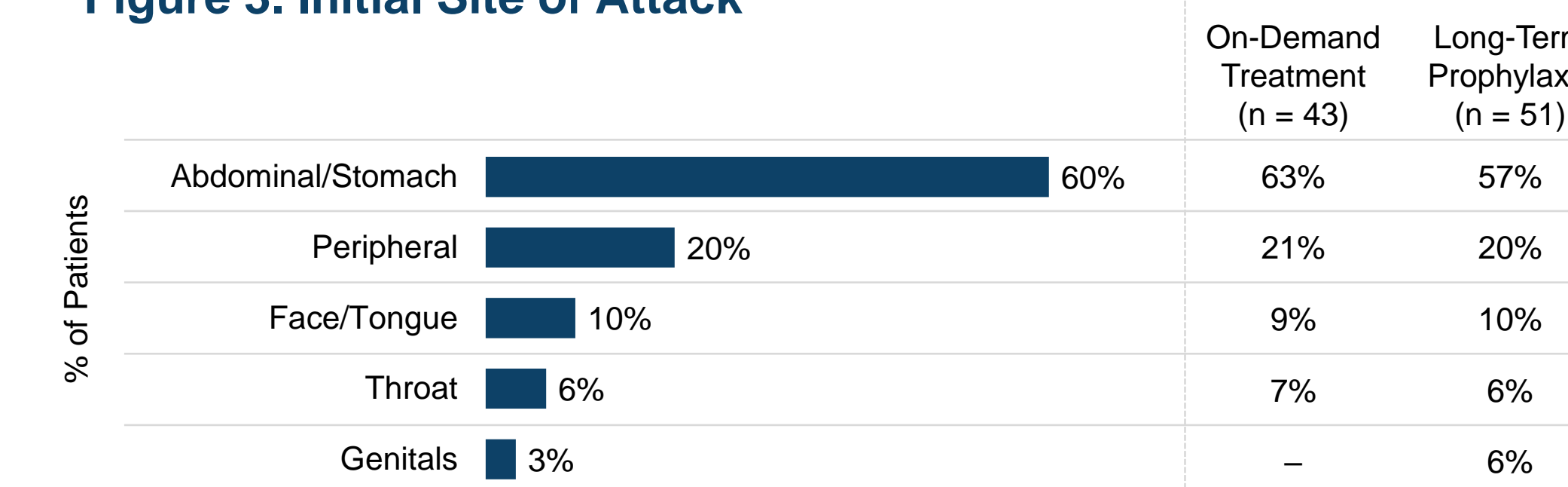
- The most frequently used long-term prophylaxis treatment was lanadelumab (Figure 1)

Figure 2. On-Demand Therapy Used for Last Treated Attack



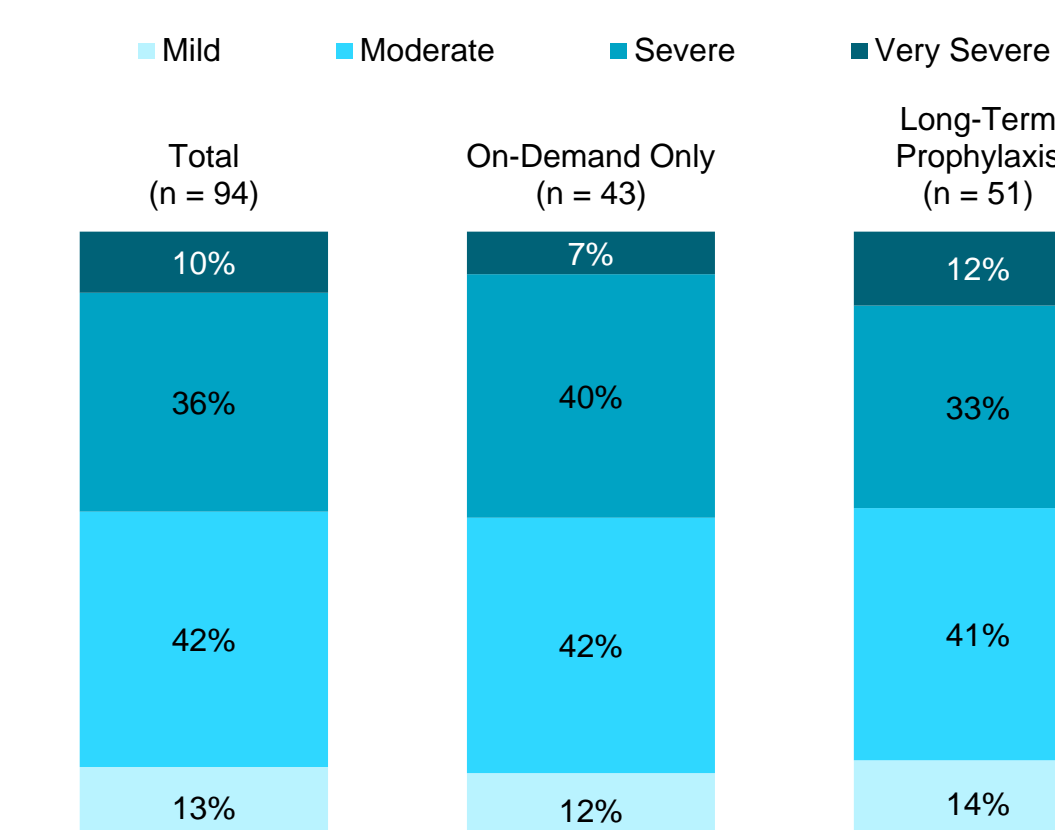
- Most frequently used on-demand treatment was icatibant (branded or generic) for both on-demand only and on-demand + LTP groups (Figure 2)

Figure 3. Initial Site of Attack



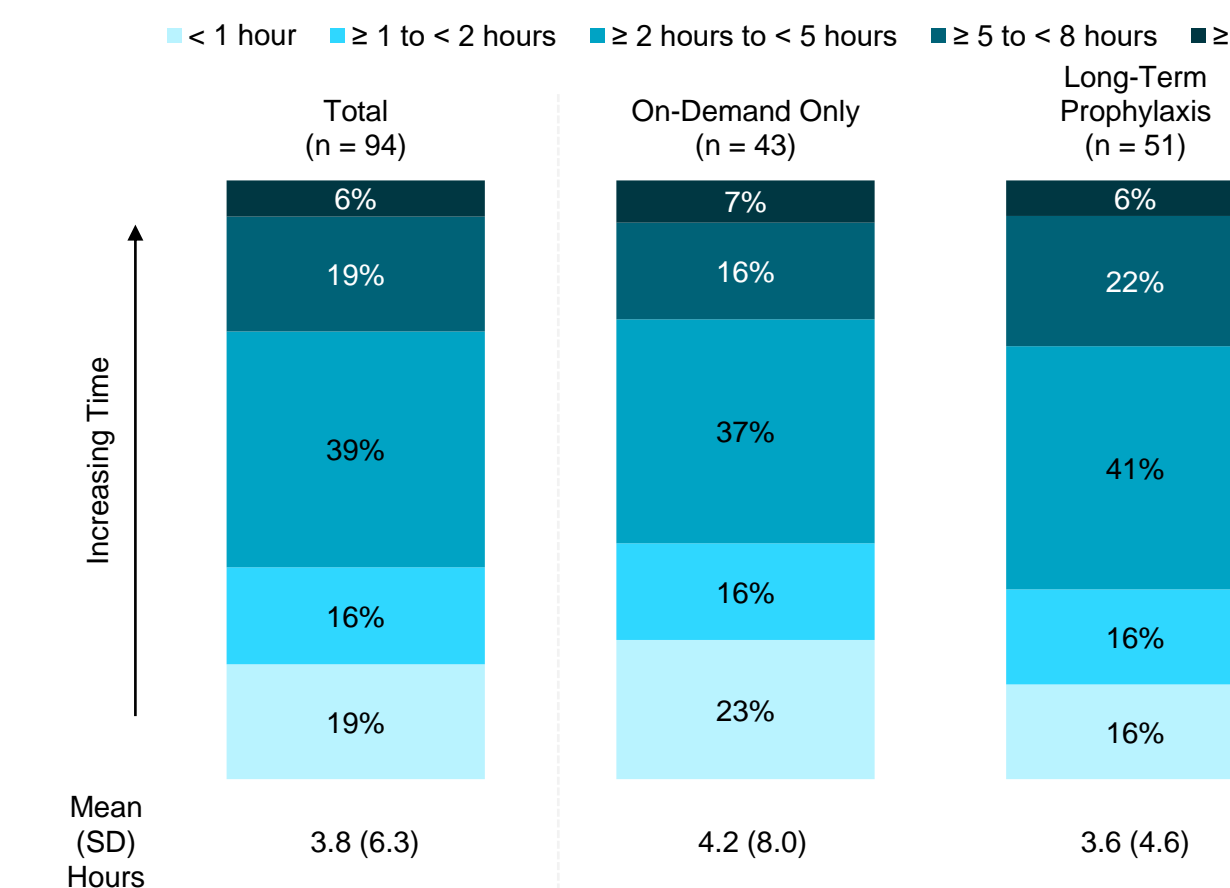
- Sixty-three percent (n = 27) of patients receiving on-demand only and 57% (n = 29) of patients receiving LTP reported that their last treated attack originated in the abdomen (Figure 3)
- Symptoms spread from the initial site to another site in 28% of those receiving on-demand only (n=12) and 31% of those receiving LTP (n=16)

Figure 4. Peak Attack Severity



- Forty-seven percent of patients receiving on-demand only and 45% of patients receiving LTP rated their last attack as severe or very severe (Figure 4)

Figure 5. Time to On-Demand Treatment



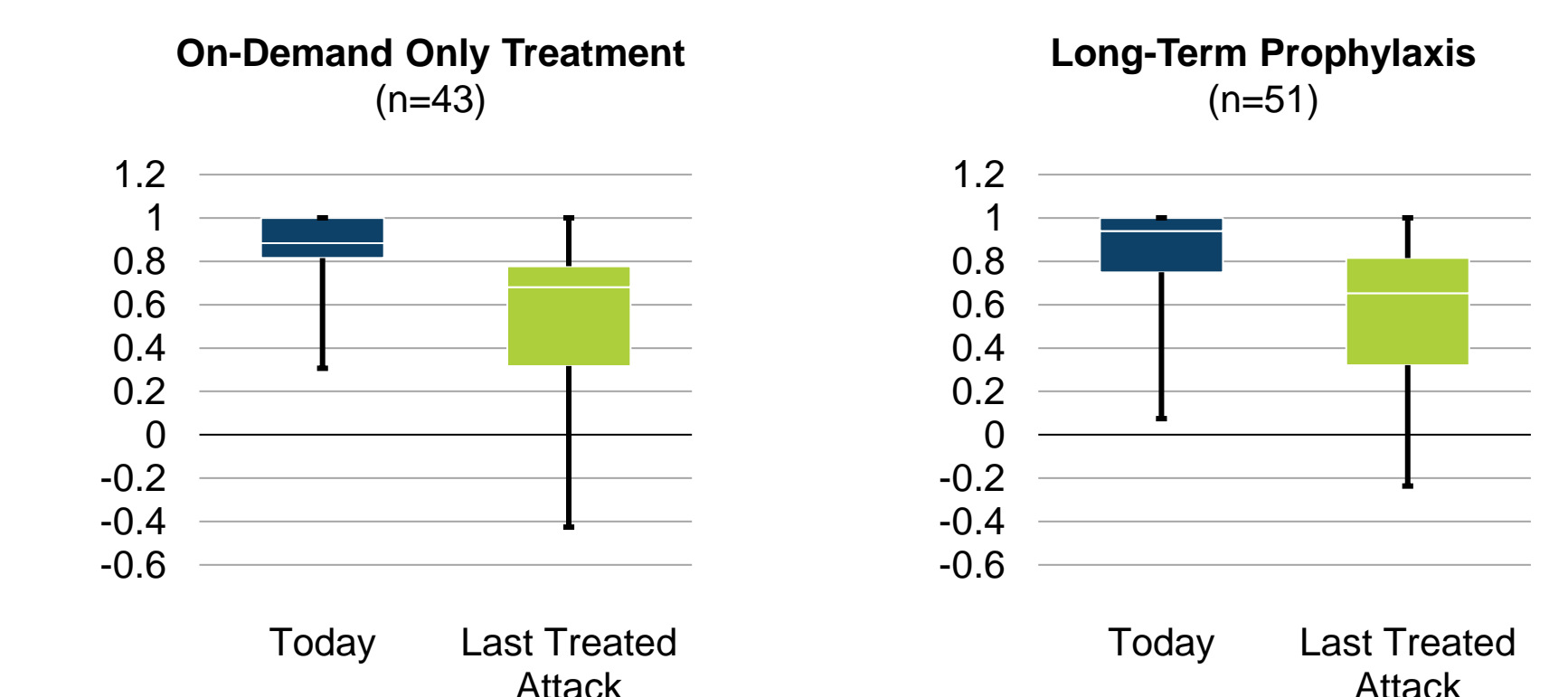
- The median reported time from attack onset to on-demand treatment (Figure 5) was 2 hours (IQR = 1-4 hours) for on-demand only patients and 3 hours (IQR = 1-5 hours) for LTP patients

Figure 6. Mean Duration of Last Treated Attack



- The duration of the HAE attack (Figure 6) was numerically lower for on-demand only patients (median = 1-day, interquartile range [IQR] = 1-2 days) than for LTP patients (median = 2 days, IQR = 1-2 days)

Figure 7. EQ-5D-5L Index Values "Today" and During the Last Treated Attack for Patients Taking On-Demand Treatment Only or Those Taking LTP Plus On-Demand



- As seen in Figure 7, patients receiving on-demand only and patients receiving LTP, QoL scores were meaningfully lower (based on minimal important difference: 0.08) at the time of the attack (on-demand only, median = 0.68 IQR = 0.32-0.781; LTP, median = 0.65, IQR = 0.32-0.82) than patients' current scores (on-demand only, median = 0.94, IQR = 0.82-1.0; prophylaxis, median = 0.93, IQR = 0.74-1.0)

Conclusions

- The current study showed that patients receiving LTP therapy who treated an attack in the last 3 months and those receiving on-demand only therapy experienced attacks with similar locations, severity, and impact on QoL
- Patients receiving LTP took longer to treat their attacks than patients receiving on-demand only and their attacks appeared to be longer (median 2 days LTP vs 1 day on-demand)
- Patients in both groups experienced significant burden with their attacks