BARRIERS TO ACHIEVING NORMALIZATION AMONG PEOPLE LIVING WITH HAE ON EXISTING TREATMENTS

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BACKGROUND

- People living with hereditary angioedema (HAE) experience physical, emotional, logistical, and financial impacts associated with disease and treatment that prevent them from achieving the goal of "normalization", defined as living life without limitations from HAE.[1,2]
- A quantitative survey was conducted to estimate the burden of HAE and treatments for people living with HAE receiving long-term prophylactic (LTP) and/or on-demand (OD) therapies, aiming to identify unmet needs that are associated with the ability to achieve normalization.
- Here, we present a subset of survey data focused on perceptions of HAE control and normalization as well as emotions associated with HAE and its management.

METHODS

- A cross-sectional, IRB-exempt, quantitative survey was conducted from March to April 2025 among people living with HAE in the United States.
- All patients were recruited in collaboration with the US Hereditary Angioedema Association (HAEA).
- The study instrument included a screener, informed consent form and a web-enabled questionnaire.
- Data were analyzed with descriptive statistics using Q Research Software.

INCLUSION CRITERIA

- ✓ Aged 18 or older with a self-reported confirmed diagnosis of HAE Type 1 or Type 2
- ✓ Currently prescribed a treatment for HAE (either OD, LTP, or both)
- ✓ Must have visited a physician in the past 1 year

RESULTS

Study Population

- A total of 100 respondents in the US completed the survey; the majority (63%) were between 18 to 45 years **[Table 1].**
- 96% of respondents reported an allergist or immunologist as primarily responsible for helping them manage and treat their HAE.

TABLE 1: CHARACTERISTICS OF RESPONDENTS (N=100)

Characteristic (N=100)	n (%)
Age Group 18 – 20 21 – 45 46 – 60 61+	 2 (2%) 61 (61%) 30 (30%) 7 (7%)
Female	80 (80%)
Race/Ethnicity* Caucasian/White Asian or South Asian (Indian Subcontinent) Black/African American Hispanic/Latin American Other (Middle Eastern, Mediterranean, Caribbean Descent, etc.) American Indian/Alaskan Native	 83 (83%) 9 (9%) 8 (8%) 7 (7%) 6 (6%) 4 (4%)
Place of Regular HAE Care* Private Practice or Physician's Office Community Hospital, Health Center, or Clinic Academic Hospital or Medical Center	 68 (68%) 24 (24%) 20 (20%)
Insurance Status* Commercial/Private Insurance Medicaid Medicare Other (e.g., VA/Tricare)	 78 (78%) 14 (14%) 7 (7%) 6 (6%)
Current Prescribed HAE Medication OD and LTP LTP Only OD Only	 70 (70%) 19 (19%) 11 (11%)
Current Prescribed LTPs* Lanadelumab C1-INH SC [human] Berotralstat Other (C1-INH IV [human], Androgens)	 55 (55%) 21 (21%) 14 (14%) 2 (2%)
Current Prescribed OD* Icatibant (generic or branded) C1-INH IV [human] C1-INH IV [recombinant]	 66 (66%) 12 (12%) 11 (11%)
Healthcare provider(s) currently seen for HAE management* Allergist or Immunologist PCP/General Practitioner Other (Nurse Practitioner, Physician Assistant, Home Nurse)	 96 (96%) 6 (6%) 4 (4%)

* Respondents could select all that apply

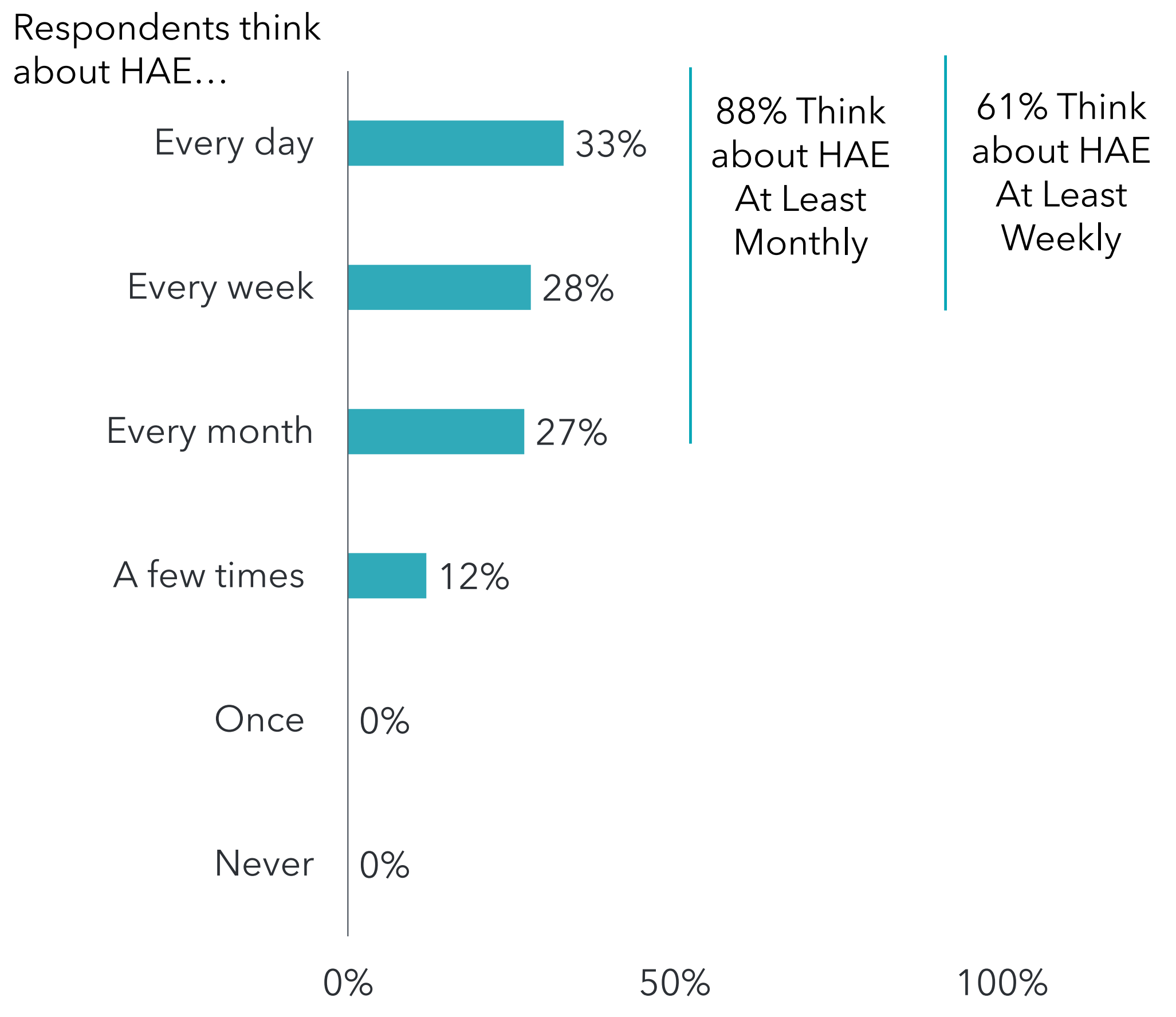
Frequency of Attacks and Thoughts of HAE

- Of the 100 respondents, 80% reported at least 1 attack in the past year; 34% experienced ≥1-2 attacks per month **[Table 2].**
- Among the 100 respondents, 88% thought about HAE at least once a month, 61% at least every week, and 33% every day in the past year **[Figure 1].**

TABLE 2: FREQUENCY OF ATTACKS IN THE PAST 1 YEAR (N=100)

Frequency of Attacks (N=100)	n (%)
0 times per year	20 (20%)
At least 1 time per year	80 (80%)
1-5 times per year	34 (34%)
6-11 times per year	12 (12%)
1-2 times per month	19 (19%)
3-5 times per month	12 (12%)
≥5 times per month	3 (3%)

FIGURE 1: FREQUENCY OF THOUGHTS RELATED TO HAE IN THE PAST 1 YEAR (N=100)



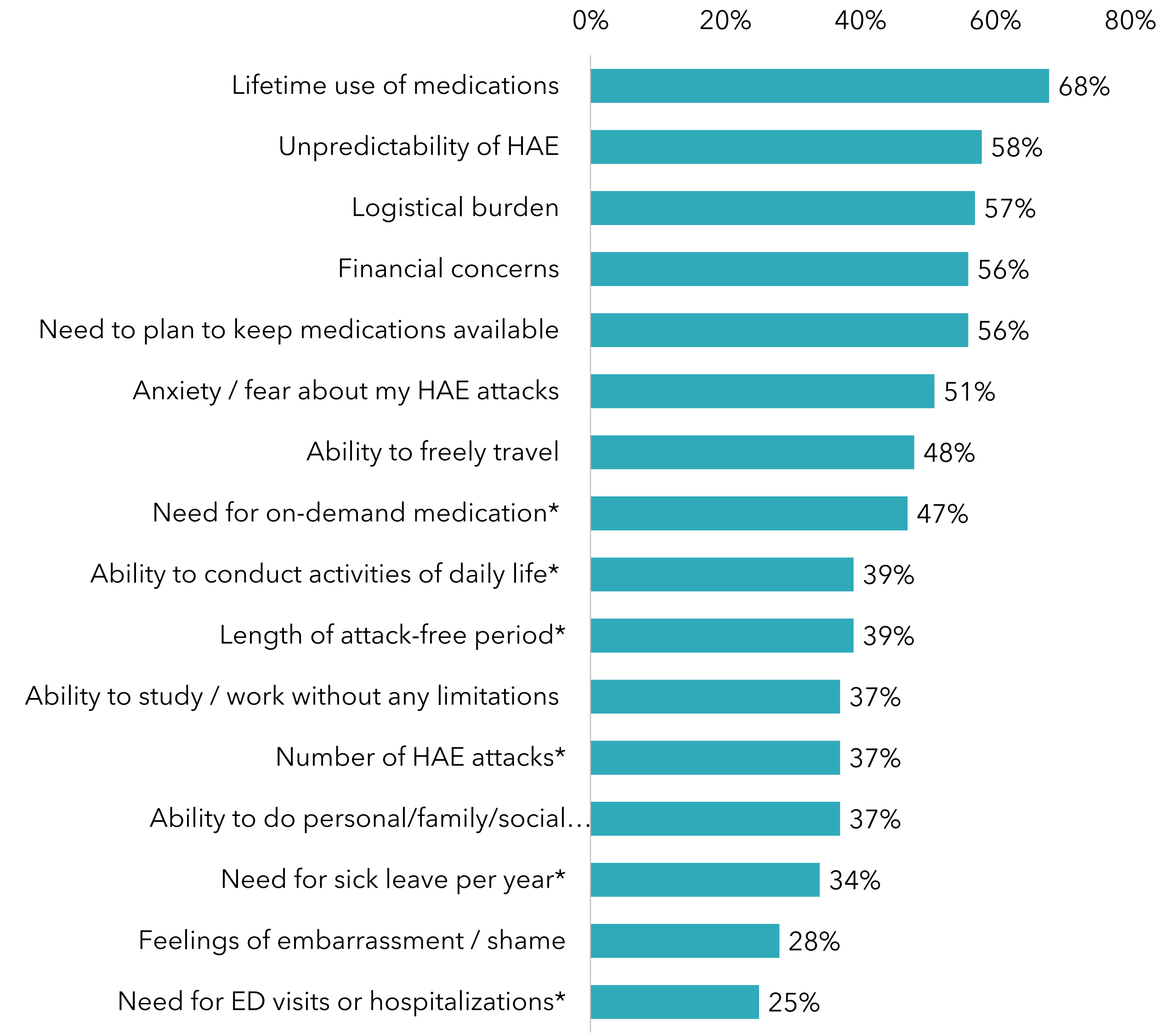
Perception of HAE Control and Normalization

- One-third of respondents (33/100) rated their HAE as not well-controlled (rated 1-7 on a 10-point scale) and nearly half (47/100) rated their HAE as not completely normalized (rated 1-7 on a 10-point scale).
- More patients considered themselves well-controlled than completely normalized.

Concerns Impacted by HAE

- Across all 100 respondents, irrespective of how they rated their level of HAE control or normalization, 68% of respondents reported the lifetime use of medication to control their HAE was a concern (rated ≥5; 7-point scale) **[Figure 2]**.
- The logistical burden of getting medications/medical care, keeping medications available with them, and the financial burden of getting access to medications are also top concerns, and they are linked to lifetime use of medication for HAE control.
- Interestingly, aspects related to treatment use were generally rated highly by respondents (>50%), and they may not be fully appreciated by HAE experts as being high impact[1].

FIGURE 2: ASPECTS OF NORMALIZATION THAT CONTINUE TO BE A CONCERN (RATED ≥5 ON A 7-POINT SCALE) (N=100)



* *Aspects where HAE experts reached consensus on importance for achieving normalization as a treatment goal*[1]
Note: Respondents rated each aspect on a scale of 1 (not a concern at all) to 7 (continues to be a significant concern)

Feelings and Emotions Associated with Attacks and Treatment

FIGURE 3A: EMOTIONS FELT THINKING ABOUT LAST HAE ATTACK ON CURRENT TREATMENT (N=100)

- When asked about emotions felt when thinking about their last HAE attack, respondents reported feeling "anxious" (51%) and "frustrated" (51%) **[Figure 3A].**

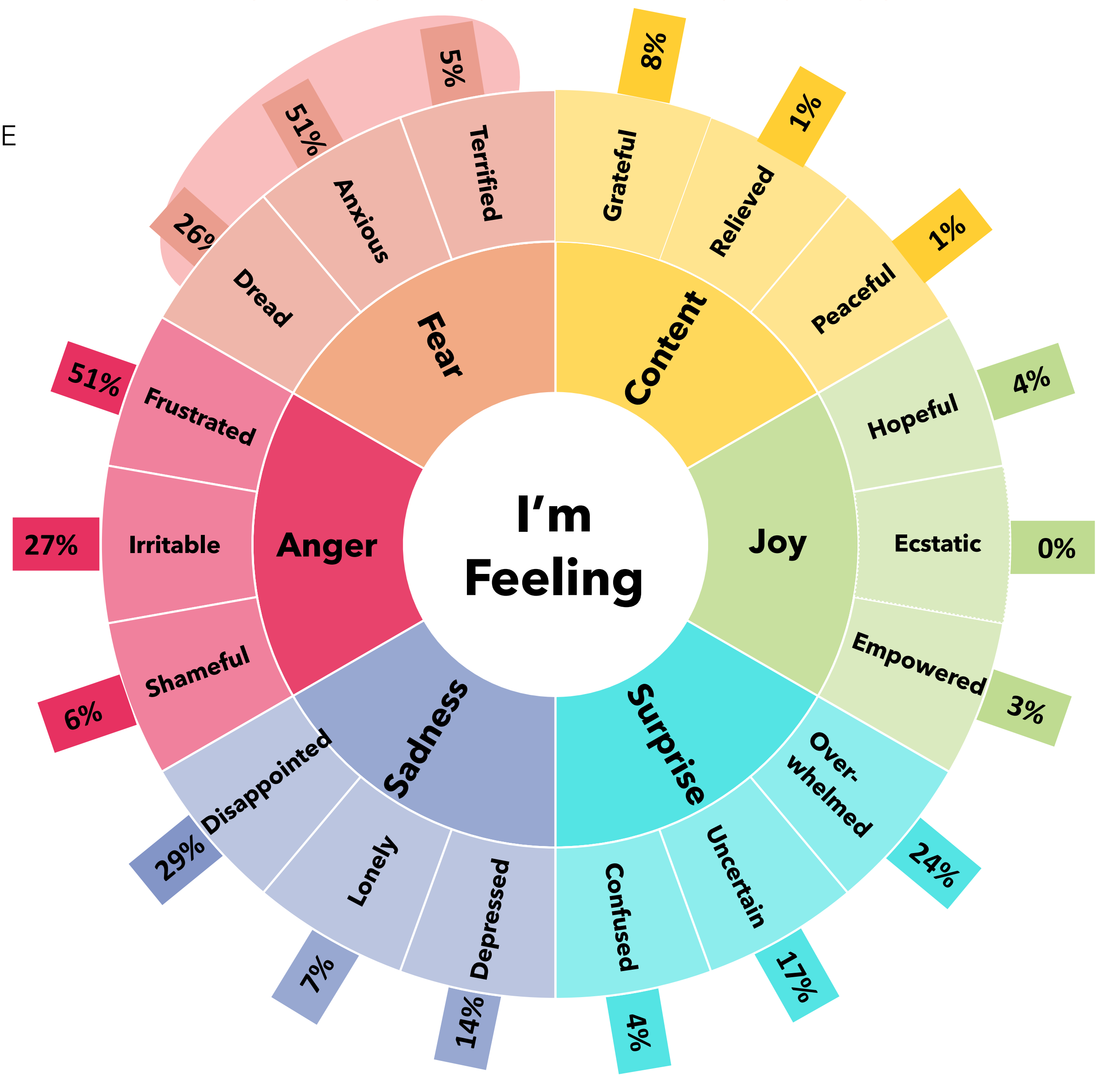
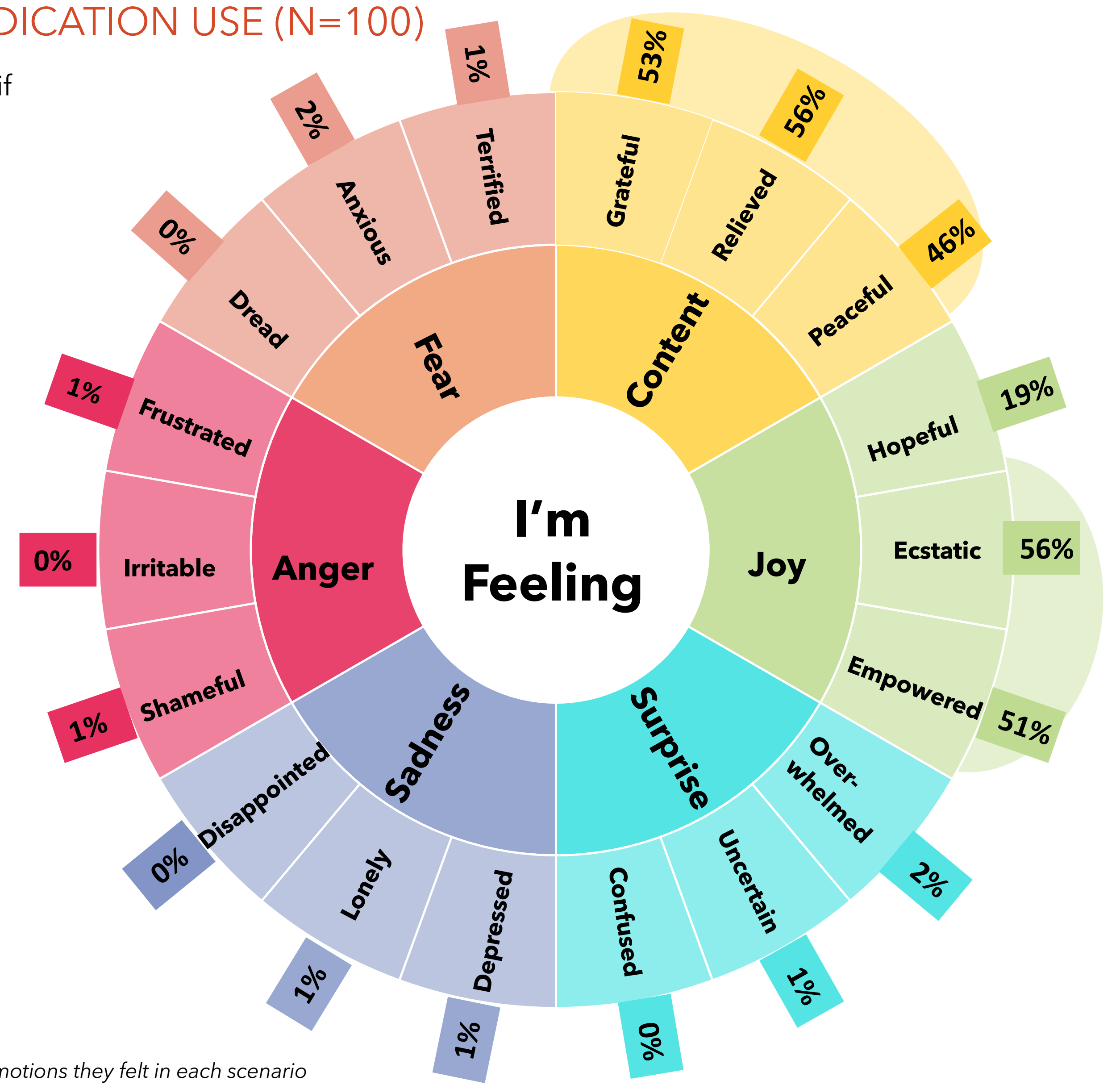


FIGURE 3B: EMOTIONS FELT THINKING ABOUT COMPLETE HAE CONTROL WITHOUT ONGOING MEDICATION USE (N=100)

- When asked how they would feel if their HAE were controlled without need for ongoing medication, respondents reported they would feel "relieved" (56%), "ecstatic" (56%), and "grateful" (53%) **[Figure 3B].**



Note: Respondents had to choose up to 3 emotions they felt in each scenario

CONCLUSIONS

- These findings highlight the ongoing challenges and burdens related to the chronic HAE management despite advances in treatment strategies over the years.
- This report suggests that patients still experience HAE attacks, despite being on treatment; the majority of respondents reported attacks, which lead to a negative impact on their mental and emotional well-being.
- Nearly half of respondents did not feel normalized with their treatment today, primarily because of the need for chronic medication use and other logistical burdens related to medication use.
- These data provide more insight into how HAE patients may truly achieve normalization, which requires future HAE treatments to focus on reducing or eliminating attacks while reducing or eliminating the need for chronic treatment.

DISCLOSURES: *PB*: nothing to disclose; *KW, SF*: received fees from consulting for Intellia Therapeutics; *HF*: received grants/fees from or consulted for CSL Behring, Pharvaris, Shire/Takeda, Pharming, Kalvista, Biocryst, Astria, Intellia, Biocryst Ionis, and ONO Pharmaceutical; *CM, SN*: employees of and hold equity in Intellia Therapeutics; *SP*: previously employed by Intellia Therapeutics; *AA, AS*: employees of Trinity Life Sciences, funded by Intellia Therapeutics for this study.

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REFERENCES: 1. Maurer et al. (2021). Consensus on treatment goals in hereditary angioedema: A global Delphi initiative. doi:10.1016/j.jaci.2021.05.016 2. Busse, et al. (2021). US HAEA Medical Advisory Board 2020 Guidelines for the Management of HAE. doi:10.1016/j.jaip.2020.08.046

ABBREVIATIONS: *HAE*: Hereditary Angioedema; *HAEA*: Hereditary Angioedema Association; *IRB*: Institutional Review Board; *LTP*: Long-Term Prophylaxis Therapy; *OD*: On-Demand Therapy; *SD*: Standard Deviation

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