Chronic Medications Pose Challenges for People Living with Hereditary Angioedema

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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.
- It has been previously reported that nearly half of patients with HAE do not feel normalized with treatment due to chronic medication use and financial/logistical burdens.[3]
- Here, we present a subset of survey data focused on assessing the burden and unmet needs of currently available treatments.

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; most were managed in private or community settings and 89% were being treated with LTP **(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

Characteristic (N=100)	n (%)
Age Group	
18 – 20	2 (2%)
21 – 45	61 (61%)
46 – 60	30 (30%)
61+	7 (7%)
Female	80 (80%)
Race/Ethnicity*	
Caucasian/White	83 (83%)
Asian or South Asian (Indian Subcontinent)	9 (9%)
Black/African American	8 (8%)
Hispanic/Latin American	7 (7%)
Other (Middle Eastern, Mediterranean, Caribbean, etc.)	6 (6%)
American Indian/Alaskan Native	4 (4%)
Place of Regular HAE Care*	
Private Practice or Physician's Office	68 (68%)
Community Hospital, Health Center, or Clinic	24 (24%)
Academic Hospital or Medical Center	20 (20%)
Insurance Status*	
Commercial/Private Insurance	78 (78%)
Medicaid	14 (14%)
Medicare	7 (7%)
Other (e.g., VA/Tricare)	6 (6%)

Characteristic (N=100)	n (%)
Current Prescribed HAE Medication	
OD and LTP	70 (70%)
LTP Only	19 (19%)
OD Only	11 (11%)
Current Prescribed LTP*	
Lanadelumab	55 (55%)
C1-INH SC [human]	21 (21%)
Berotralstat	14 (14%)
Other (C1-INH IV [human], Androgens)	2 (2%)
Current Prescribed OD*	
Icatibant (generic or branded)	66 (66%)
C1-INH IV [human]	12 (12%)
C1-INH IV [recombinant]	11 (11%)
Healthcare provider(s) currently seen for HAE management*	
Allergist or Immunologist	96 (96%)
PCP/General Practitioner	6 (6%)
Other (Nurse Practitioner, Physician Assistant, Home Nurse)	4 (4%)

** Respondents could select all that apply*

Frequency of Attacks and Thoughts of HAE

- 80% (80/100) of respondents reported at least 1 attack in the past year; 34% experienced ≥1-2 attacks per month **(Table 2)**.

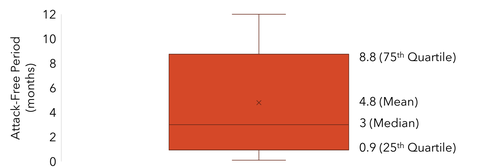
TABLE 2. Frequency of attacks in the past 1 year (N=100)

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%)

Attack-Free Period

- There was significant variability on the longest attack-free period experienced among the 80 respondents who had attacks in the past year.
- The median (IQR) longest attack-free period was 3 (0.9-8.8) months; respondents with minimal attacks were outliers who were attack-free most of the year **(Figure 1)**.
- Most respondents (61%) felt staying attack-free for a minimum of 6-12 months would be meaningful to them **(Table 3)**.
- Mental health impact was the primary driver for choosing the minimum meaningful attack-free period, selected by 33% of respondents **(Figure 2)**.

FIGURE 1. Longest attack-free period in the past 1 year (n=80*)



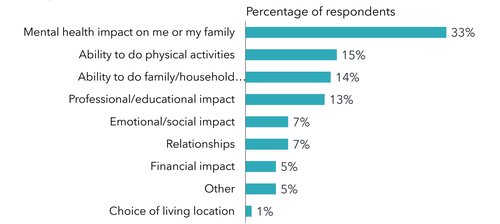
** Respondents who reported an HAE attack in the past year reported the longest time they were attack-free in the past year*

TABLE 3. Minimum meaningful attack-free period (N=100)

1 day	3 days	1 week	1 month	3 months	6 months	1 year
1%	1%	8%	12%	17%	**19%**	**42%**

61%

FIGURE 2. Primary reason* influencing the meaningful attack-free period (N=100)

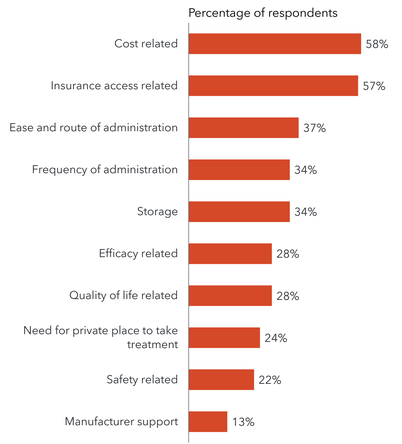


** Respondents could select one item only as the primary driver of their choice of the meaningful attack-free period*

Unmet Needs and Burden of LTP

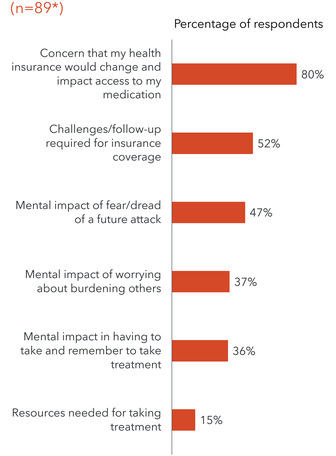
- The most commonly reported unmet needs associated with LTP included affordability (58%), insurance access (57%), ease of administration (37%), frequency of administration (34%), and storage (34%) **(Figure 3)**.
- For those on LTP, the highest burden associated with LTP was concern about changes in health insurance impacting medication access (80%) **(Figure 4)**.

FIGURE 3. Unmet needs with LTP (N=100)



Respondents indicated on a scale of 1 (very low unmet need) to 7 (very high unmet need)

FIGURE 4: Burden of currently available LTP (n=89*)



** Data were collected only for respondents who were on LTP currently*
Respondents indicated on a scale of 1 (very low burden) to 7 (very high burden)

Aspects of Current LTP that Need Improvement

- The majority of respondents ranked lifetime use of medications (55%) and efficacy (52%) in their top three aspects of LTP needing improvement to achieve normalization **(Table 4)**.

TABLE 4. Aspects of current LTP that need improvement to feel normalized (N=100)

Aspects of Current LTP	Proportion of Patients who ranked Aspect in the Top Three n (%)
Lifetime use of LTP to manage HAE	55 (55%)
Efficacy	52 (52%)
Financial burden	38 (38%)
Impact on Quality of life	37 (37%)
Frequency of administration	29 (29%)
Safety	28 (28%)
Route of administration	30 (30%)
Level of independence achieved	16 (16%)
Logistical burden	15 (15%)

Respondents ranked the aspects in order, from most important (rank 1) to least important (rank 9).

CONCLUSIONS

- These findings highlight the ongoing challenges and burdens related to lifelong HAE management that exist despite advances in treatment strategies over the years.
- The median time reported that respondents spent attack-free was only 3 months in the past year; however, the goal for meaningful attack-free time was reported as 6-12 months, suggesting patients may not be completely satisfied with the efficacy of their current HAE treatment.
- When asked to identify the primary driver for wanting to be attack-free for an extended period, a third of respondents indicated it may alleviate their personal or family's mental health burden, supporting the notion that attack control is related to psychosocial aspects of HAE.
- Cost considerations and challenges in access to HAE medications through healthcare insurance were identified as a key remaining unmet need of current LTP among these respondents from the US. The burdens are amplified with the need for lifetime use to achieve HAE control, and respondents rated need for lifetime use of medications as an aspect to improve in current LTP to feel normal (i.e., like someone without a chronic condition).
- These data provide more insight into the unmet needs and burdens that patients with HAE experience. Improving treatment efficacy and eliminating the need for lifelong HAE therapy may support achieving the treatment goal of normalization.

DISCLOSURES: *PB: received* fees or consulted for Intellia Therapeutics, KalVista Pharmaceuticals, ADARx Pharmaceuticals, Takeda Pharmaceuticals, CSL Behring, Astria Therapeutics, Ionis Pharmaceuticals; *KW, SF:* received fees from consulting for Intellia Therapeutics; *HF:* received grants/fees from or consulted for CSL Behring, Pharvaris, Shire Pharmaceuticals/Takeda Pharmaceuticals, Pharming Therapeutics, KalVista Pharmaceuticals, BioCryst Pharmaceuticals, Astria Therapeutics, Intellia Therapeutics, Ionis Pharmaceuticals and ONO Pharmaceuticals; *CM, SN:* employees of and hold equity in 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. 3. Busse et al. (2025). Barriers to Achieving Normalization among People Living with HAE on Existing Treatments. ACAAI, November 6-10, 2025, Orlando, Florida.

ABBREVIATIONS: *HAE:* Hereditary Angioedema; *HAEA:* Hereditary Angioedema Association; *IQR:* Interquartile Range; *IRB:* Institutional Review Board; *LTP:* Long-Term Prophylaxis Therapy; *OD:* On-Demand Therapy

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