Burdens of Disease and Treatment in Hereditary Angioedema: Interview Insights from HAE Patients

Anne-Elise Tobin, PhD • Astria Therapeutics • Boston, MA, USA

Introduction

Purpose
To share the diagnosis and treatment journey from 10 US Hereditary Angioedema (HAE) patients and identify disease burden and unmet needs in treatment.

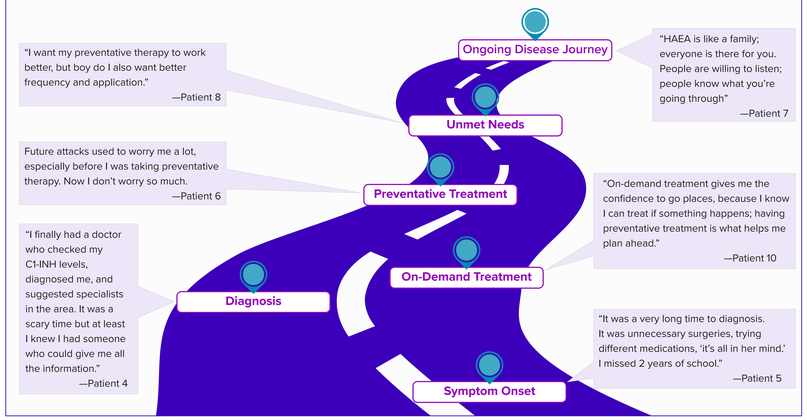
Background
The options for HAE treatment in the US have increased over the past 13 years since the 2008 introduction of the first HAE-specific treatment designed to prevent HAE attacks (intravenously administered C1 esterase inhibitor [human]). Since then, 3 additional preventative treatments, and 4 on-demand treatments have been approved in the U.S. The evolution of treatment options has centered on easing administration burden (shifting away from IV administration, and, for preventative medicines, decreasing dosing frequency). Given the advancement in treatment options, we sought to understand patients' past experiences with obtaining accurate HAE diagnosis, past and current experiences managing treatment and disease burdens, and their future expectations for improvements to HAE preventative treatments.

Methodology

- A case study methodology was used
- Patients with diagnosed HAE were identified across the U.S. by Rare Patient Voice, LLC and screened for inclusion based on self-identified disease type (HAE Types 1 and 2), disease severity (moderate or severe when not on treatment), and eligibility for preventative treatment (having received a prescription for, or participated in a clinical trial for, preventative treatment)
- Ten patients were interviewed during 60-minute telephone discussions in February 2021 using a structured one-hour interview guide
- Respondents were blinded to the identity of interviewer and sponsoring company
- Results were qualitatively analyzed; where numerical ratings were provided, or where responses could be categorized, quantification was performed to show relative differences in responses and was not intended for statistical analysis

HAE Patient Journey

Patients often experience a long, painful, and frustrating disease journey. Although treatments have been life-changing, patients hope for improvements. HAE community connection provides support and education.

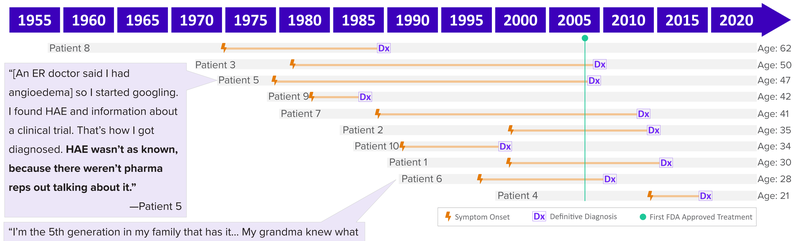


Results

Disease Awareness

- HAE patients' symptom onset ranged from 4 to 16 years old with an average delay to diagnosis of 16 years
- Most believed diagnosis was delayed by poor HAE awareness among the variety of treatment providers they visited (PCP, ER, dermatologist, GI specialists), delaying referral to Allergists/Immunologists for diagnosis
- Only 3 of the 10 patients were diagnosed prior to the 2008 availability of the first HAE-specific treatment
- Although three-quarters of HAE patients likely have a family history of the disease (Pappalardo, 2000), only one third of interviewed patients were aware of their family history prior to diagnosis. After diagnosis, one-third suspected a family history
- Currently, to stay informed on disease management and treatments, patients relied on a variety of sources, especially advocacy organizations (the U.S. HAE Association HAEA) and patient communities they met through advocacy-sponsored events and social media

Symptom Onset to Diagnosis



"[An ER doctor said I had angioedema] so I started googling. I found HAE and information about a clinical trial. That's how I got diagnosed. **HAE wasn't as known, because there weren't pharma reps out talking about it."** —Patient 5

"I'm the 5th generation in my family that has it... My grandma knew what it was, but **there were not a lot of great options at the time, so we never had it diagnosed or treated."** —Patient 6

Patients Trust Their Doctors, Also Seek Additional Information

All are connected with HAEA for the following needs:

- Information on HAE and treatments
- Peer support and to feel part of a community
- Recommendations of providers
- Information on research studies

Most patients did not seek additional information when their doctor first recommended preventative treatment, owing to trust and eagerness to start treatment

"I trusted her, and when you're on nothing, they could have said they would put peanut butter in my veins, and I would have said, 'ok.' I was willing to try anything." —Patient 8

Disease Burden

- Despite all patients taking preventative therapy, half of the patients stated they think about future attacks often or always; others indicated it was on their mind sometimes
- 3 respondents mentioned preventative therapy has helped them worry less

"I was tiptoeing around life before [preventative treatment]. If I did activities, I would do them more in fear than in enjoyment. Now, if something happens, I know there's medication working in my body, so I don't have to live in that fear." —Patient 4

HAE Effects on Daily Life

Ongoing Functional Impacts:

- Avoiding certain activities (e.g., exercise, hobbies, travel)
- Missing/avoiding events
- Being unreliable
- Work issues:
  - Missing work/modified job to avoid missing work
  - Unable to maintain a job
  - Difficulty explaining disease to employer

Frequent Worries/Fears:

- Running out/losing access to treatment (esp. on-demand)
- Threat of fatal laryngeal attacks
- Managing attacks (esp. outside of the house)
- Being on the lookout for triggers
- Financial burden of treatment

HAE can severely disrupt patients' lives and their ability to maintain a job, social life, and hobbies.

Treatment Burden

Administration Convenience Drives Treatment Preference

- On average, patients had tried 2-3 preventative treatments, switching for more convenient administration
- Improvements in efficacy and dosing are equally compelling for patients to switch
- Most patients would be compelled to try a therapy dosed every 2 months or less frequent

Primary Driver for 1st Preventative Treatment

Primary Driver for Switches

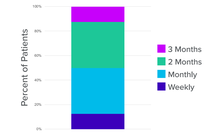
"I was really struggling with the IV treatment. I had a nurse come to my house every 3 days. We have very active lifestyles and this tied me down a bit. So my doctor was on the lookout for a subcutaneous formulation." —Patient 9

Factors to Compel Patients to Switch to a New Therapy

"I'd choose 'efficacy,' but I don't know how much more effective it could get --Takhzyro is supposed to be 80%-90% effective. But if something is equally effective, and it's easier to integrate into my life, that would make me change." —Patient 9

Top Therapeutic Improvement to Compel Switch

Minimal Desired Improvement in Dose Frequency



Conclusions

Since the 2008 availability of the first HAE-specific treatment in the U.S., the selection of HAE treatment options has grown. Along with advocacy efforts in driving disease awareness, the advent of new medicines has had the added benefit of supporting disease awareness and potentially increasing HAE diagnosis rates.

While treatments can help decrease the severity and frequency of HAE attacks, there is substantial need to decrease the psychological burden that the potential for unpredictable and debilitating HAE attacks can have on patients' lives.

Improved dosing (route and frequency) substantially influenced patients' past choices for preventative therapy. Reducing dosing frequency was as important as improving efficacy for patients to consider a future switch in therapy. These findings suggest that patients are seeking to reduce their treatment burden as much as their disease burden and may be open to trying new therapies that can address these needs.

Advocacy plays an important role in informing patients with disease education, treatment options and other resources for disease management, and maintaining a supportive and connected community.

References

Pappalardo E, et al. Frequent de novo mutations and exon deletions in the C1 inhibitor gene of patients with angioedema. Journal of Allergy and Clinical Immunology. 2000;106(6):1147-1154.

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