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

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Introduction

Purpose
To share the diagnosis and treatment journey from US Hereditary Angioedema (HAE) patients and identify disease burdens 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 (previously administered C1-inhibitor inhibitor [iFy]

Methodology

- A case study methodology was used.
- Patients with diagnosed HAE were identified across the US by Rare Patient Voice LLC and screened for inclusion based on self-identified disease type (HAE Types 1 and 2), disease severity (severe or moderate), 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

Disease State

- HAE patients’ symptom onset ranged from 1 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 10 patients were diagnosed prior to the 2008 availability of the first HAE-specific treatment.
- Although three-quarters of HAE patients deadly a family history of the disease (Pappalardo, 2005), only one-third were able to recognize symptoms prior to diagnosis.
- All patients had been diagnosed within 3 years of their first attack, with one-third aware of their family history prior to diagnosis. After diagnosis, one-third suspected a family history.

Dx

- To stay informed on disease management and treatments, patients relied on a variety of sources, especially referral opinions with the 1st HAE diagnosis. HAE and patient communities they meet through advocacy-sponsored events and social media.

Treatment burden

- Patient information was shared during the initial diagnosis.
- HCP recommendation was shared during the initial diagnosis.
- All patients had tried 2-3 preventative treatments and 4 on-demand treatments have been approved in the US. The evolution of treatment options has centered around decreasing the psychological burden (shifting away from IV administration, and, for preventative medicines, decreasing dosing frequency).

Factors to Compel Patients to Switch to a New Therapy

- Easier administration
- Improved efficacy
- HCP had samples
- Improved dosing (route and frequency) substantially improved patient’s past choice for preventative therapy.

Conclusions

Since the 2008 availability of the first HAE-specific treatment in the US, the selection of HAE treatment options has grown. Along with voluntary efforts in driving disease awareness, the advent of new medicines has had the added benefit of supporting disease management, and maintaining a supportive and connected community.

References


Our thanks to Rare Patient Voice • 71 Hampton Lane, Towson, MD 21286

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