Medical resource utilization and quality of life of HAE patients based on data from the 2020 national survey

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Abstract

Introduction: Hereditary angioedema (HAE) is a rare genetic disease characterized by unpredictable, recurrent episodes of angioedema leading to swelling of limbs, face, larynx and the gastrointestinal tract. Episodes are painful and can be fatal. Most patients have deficient or dysfunctional C1 inhibitor (HAEC1INH), but a significant percentage have other mutations causing similar episodes of angioedema (HAEnC1INH).

Methods: Because randomized controlled trials may not fully reflect HAE patients' burden of illness, HAE Canada has conducted multiple surveys to obtain data which would otherwise be unavailable. Data obtained includes demographics, number and severity of attacks, treatment utilization and satisfaction, quality of life (QoL), burden of illness, health care utilization and economic costs to the patient.

Results: Results from our 2020 survey show that despite 88% of HAE patients using HAE medication, a significant proportion still have >12 attacks/year (HAEC1INH: 27%, HAEnC1INH: 50%) and unscheduled visits to the ER (HAEC1INH: 45%, HAEnC1INH: 54%), missed work (HAEC1INH: 53%, HAEnC1INH: 61%) and have high levels of anxiety (HAEC1INH: 61%, HAEnC1INH: 67%). Medications are evolving but due to heterogeneity of treatment effects, more treatment options are needed for attack prevention, acute attack management as well as more convenient treatment modalities (e.g., oral or subcutaneous versus intravenous). Better treatments and better access to treatment may offset costs to the health care system and improve patient QoL.

Conclusion: Our data has been used to raise awareness of HAE and to advocate for access to treatment. However, there are still unmet needs and further research is needed to better manage all forms of angioedema. A registry to collect real-world data would be highly beneficial.

Introduction

Hereditary angioedema (HAE) is a rare genetic disease characterized by unpredictable, recurrent episodes of angioedema leading to swelling of limbs, face, larynx and the gastrointestinal tract. Episodes are painful and can be fatal. Most patients have deficient or dysfunctional C1 inhibitor (HAEC1INH), but a significant percentage have other mutations causing similar episodes of angioedema (HAEnC1INH). A number of treatments are approved for the treatment of HAEC1INH, but none are approved specifically for HAEnC1INH. Therefore, resource utilization and quality of life of the two patient populations may differ.

Objective

To understand the burden of illness of patients with HAE by evaluating the frequency of HAE attacks, medical resource use and quality of life.

Methods

In 2020, a comprehensive email survey was sent to all members of HAE Canada to gather information on multiple aspects of HAE. Responses of patients who reported having HAE C1INH or HAEnC1INH were extracted to evaluate attack frequency, resource utilization and quality of life. Data was analyzed as the percent of responses to a given question.

Results

In the 2020 survey, 106 respondents self-identified as having HAEC1INH; 45 self-identified as having HAEnC1INH. The majority were female (HAEC1INH:76%, HAEnC1INH: 84%) with a median age of 53 (HAEC1INH) and 48 (HAEnC1INH).

Results (continued)

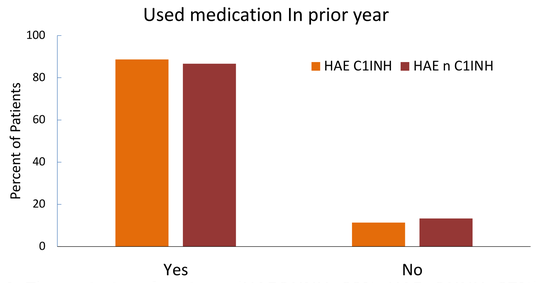

Figure 1. The majority of patients (HAEC1INH: 89%, HAEnC1INH: 87%) reported using medication to treat their HAE in the prior year.

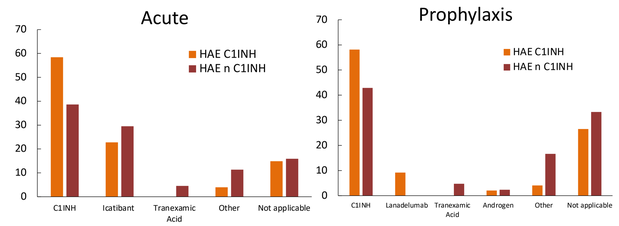

Figure 2. C1INH was the predominant treatment for both acute and prophylactic care for all HAE patients. About ⅓ HAEC1INH and ¼ HAEnC1INH patients used no prophylactic treatment. Other = HAE C1INH: essential oils, diazepam; HAEnC1INH: prednisone, adrenalin, antihistamine.

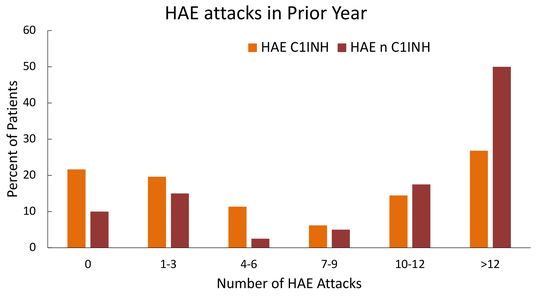

Figure 3. Despite using medication, a significant proportion of patients had >12 HAE attacks/year (HAEC1INH: 27%, HAEnC1INH: 50%).

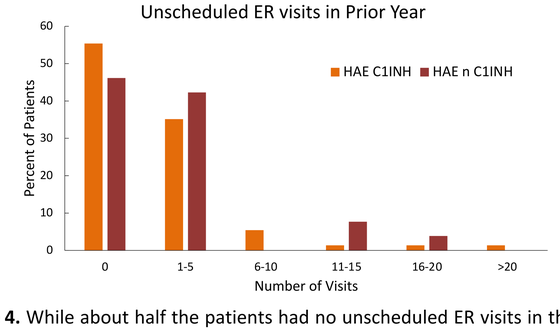

Figure 4. While about half the patients had no unscheduled ER visits in the prior year, a significant proportion (HAEC1INH: 45%, HAEnC1INH: 54%) had 1 or more visits.

Results (continued)

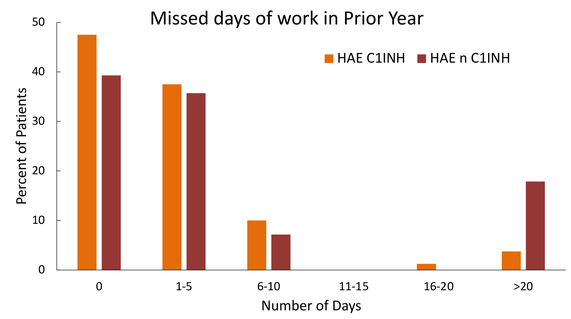

Figure 5. A significant proportion of patients reported missing at least one day of work (HAEC1INH: 53%, HAEnC1INH: 61%) in the prior year.

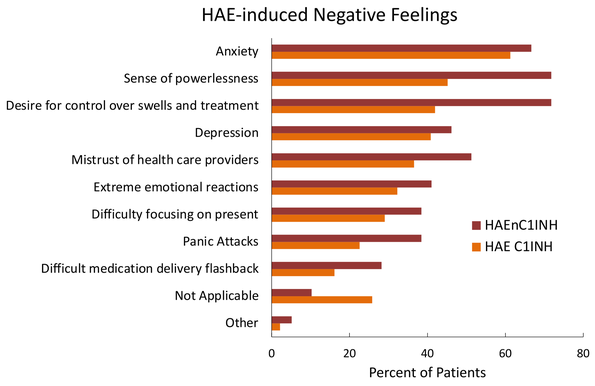

Figure 6. Higher proportions of HAEnC1INH patients experienced negative feelings on all domains questioned.

Conclusions

- In 2020, a majority of patients with HAE used medication to treat their disease. However, usual treatment was C1INH, irrespective of the type of HAE and many did not treat prophylactically.
- Only a small proportion were attack free and those with HAEnC1INH tended to have more frequent attacks.
- About half of HAE patients had unscheduled ER visits and missed work. Those with HAEnC1INH were more likely to have frequent ER visits, missed days of work and a negative psychologic impact.
- A potential bias exists as patients with more active disease may have chosen to participate in the survey, while those with less active disease did not.
- Further research and targeted treatments are needed to better manage all forms of HAE. A registry to collect real-world data and genetic testing would be highly beneficial.

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