HAE with normal C1 esterase inhibitor (HAEnC1INH): Diagnosis, treatment and attack frequency from the 2020 Canadian patient survey

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Objective

- To use recent survey data to better understand health burden and medication use of patients with HAEnC1INH

Introduction

Hereditary angioedema with normal C1INH (HAEnC1INH) formerly known as Type III HAE is a more recently recognized type of HAE. There are presently no diagnostic tests and genetic testing may not be available or definitive. As a result, diagnosis is difficult and often delayed. In addition, there are no approved medications for HAEnC1INH with the result that treatments used in Canada are the same as for those with HAE with inadequate C1INH.

Methods

An online survey was sent to Canadian HAE patients in 2020 to better understand their health burden and treatments used. We extracted responses from patients who self-reported having HAE with normal C1INH (HAEnC1INH) and analyzed the data to capture data on demography, diagnosis, attack frequency and medication use.

Results

Demographic Data	
Age: Mean (range)	54 (23-91)
Female	84%
Male	16%
Age at symptom onset: Mean (range)	26 (1-58)
Age at diagnosis: Mean (range)	39 (16-68)

Table 1. HAEnC1INH was self-reported by 27% of respondents (45/164). The majority were female with an average age of 54 years. The mean reported age at symptom onset was 26 and at diagnosis it was 39. This gives an average gap of 13 years between onset and diagnosis.

- 87% of patients reported using either prophylactic or on demand medication to treat their HAEnC1INH.
- Most respondents used pdC1INH to treat acute attacks (IV 30%, SQ 11%) (Figure 1) and for prophylaxis (IV 33%, SQ 24%) (Figure 2).
- Icatibant was used by 30% to treat acute attacks.
- Access to treatment was provided by a government plan (74%) or by private insurance (21%). Five percent paid out of pocket.

Conclusions

- A significant proportion (27%) of Canadian HAE patients who responded to the survey report having hereditary angioedema with normal C1INH (HAEnC1INH).
- For HAEnC1INH there is a long delay between symptom onset and diagnosis. Diagnosed patients have access to and use medication approved for HAE Type I/II
- There is a need for more awareness as well as targeted treatment for those with HAEnC1INH.

Results (continued)

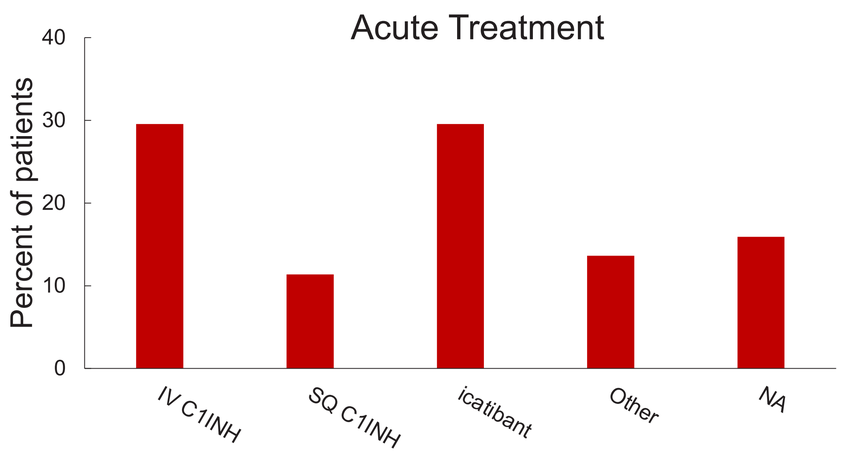

Figure 1. For acute treatment of attacks most patients used either pdC1INH (IV or SQ) or icatibant in the past year. Others (14%) included pain medication, tranexamic acid and prednisone. 16% did not use acute treatment. (n=44)

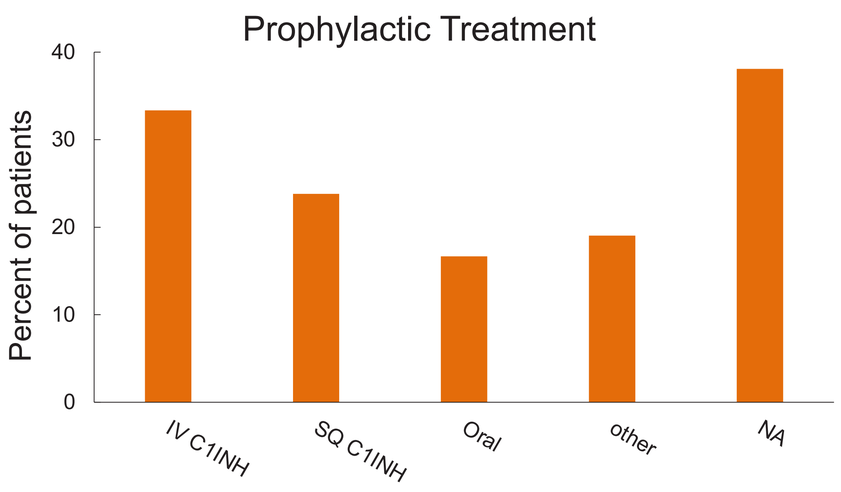

Figure 2. The most commonly used prophylactic treatment in the past year was pdC1INH. Oral medications (androgen & tranexamic acid) were used by 17% and other medications (CBD, montelukast, prednisone & immune globulin) were used by 19%. 38% were not using prophylactic treatment. (n=42)

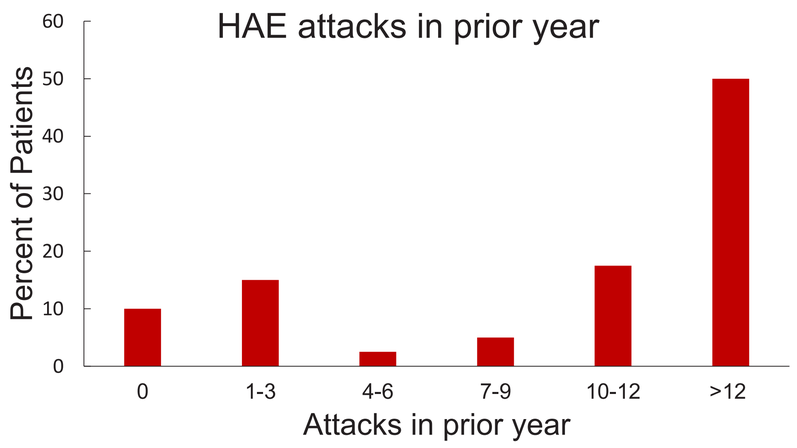

Figure 3. Despite a high proportion taking some form of medication, half of the respondents had more than 12 HAE attacks in the prior year. Only 25% had 3 or fewer attacks. (n=40)

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