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Abstract

Introduction: HAE is a genetic disease leading to intermittent attacks of angioedema of the face, the extremities, genitalia and the abdomen. Access to treatment may be impacted by the mode of delivery. Many approved medications are delivered intravenously. Newer approved medications are delivered subcutaneously. The oral medications used to treat HAE (androgen and tranexamic acid) are older, not approved for HAE (androgen), have unwanted side effects and are less effective. Newer, effective oral medications are desired by patients with HAE.

Methods: Using data from the HAE Canada patient surveys performed in 2017 and 2020, we analyzed and compared responses to questions on the type of medications used to treat HAE and number of attacks not treated because of access to medication.

Results: IV medication use was similar in 2017 (on-demand: 58.8%, prophylaxis 41.3%) and 2020 (on-demand: 41.3%, short-term (44.0%), long-term (32.5%) prophylaxis). Similarly, there was little change in oral medication use. In 2017: 3.8% for on-demand and prophylaxis; in 2020: 2.5%, on demand, 9.2% short-term and 3.4% long-term prophylaxis. By contrast, subcutaneous medication use has increased from in 2017: 10.0% on-demand, 1.25% prophylaxis to, in 2020, 60.4% on-demand, 28.1% short-term and 38.4% long-term prophylaxis. Percentages may exceed 100% because of multiple categories for use. In 2017, the number of attacks not treated because of medication access was zero for 4.35% of patients while in 2020 it was zero for 59.7%.

Conclusion: The use of newer, self-administered HAE treatments has significantly increased between 2017 and 2020. In parallel, the percent of patients with attacks not treated because of lack of access to medication has decreased significantly. Medications taken at home which are non-invasive and accessible (subcutaneous or oral) are desired by HAE patients and will likely have a significant impact on their quality of life.

Introduction

Hereditary angioedema (HAE) is a rare inherited disorder characterized by recurrent painful episodes of severe swelling in different parts of the body. On-demand treatment is used to control attacks; prophylactic treatment is used short-term to prevent attacks if there is a known risk (e.g., dental procedures) and long-term to eliminate or reduce attacks. Oral, intravenous and, more recently, subcutaneous treatments are available for treatment. Many approved medications are delivered intravenously but the availability of subcutaneous medication has increased in recent years.

Objective

With the advent of newer, more convenient forms of delivery we wished to understand the evolution of medication use by patients with HAE and the impact of these changes on the rate of attacks.

Methods

In 2017 and again in 2020, a comprehensive email survey was sent to all members of HAE Canada to gather information on multiple aspects of HAE. Responses to questions on the type of medications used to treat HAE and number of attacks not treated because of access to medication were analyzed for this report. Data was expressed as the percent of respondents.

Results

	2017	2020
Gender:		
Female	77.0	79.6
Male	23.9	19.6
HAE Diagnosis		
Type 1/2	59.6	64.6
HAEnC1-INH	26.8	27.4
Acquired	3.8	2.4
Don't know	10.6	5.5

Table 1. Demographic information. Data given is percent of respondents; n=100 for 2017 and 164 for 2020.

In both surveys, the majority of respondents were female. The proportion with a given diagnosis is similar although, in 2020, slightly more indicated having Type1/2 HAE and fewer didn't know their diagnosis.

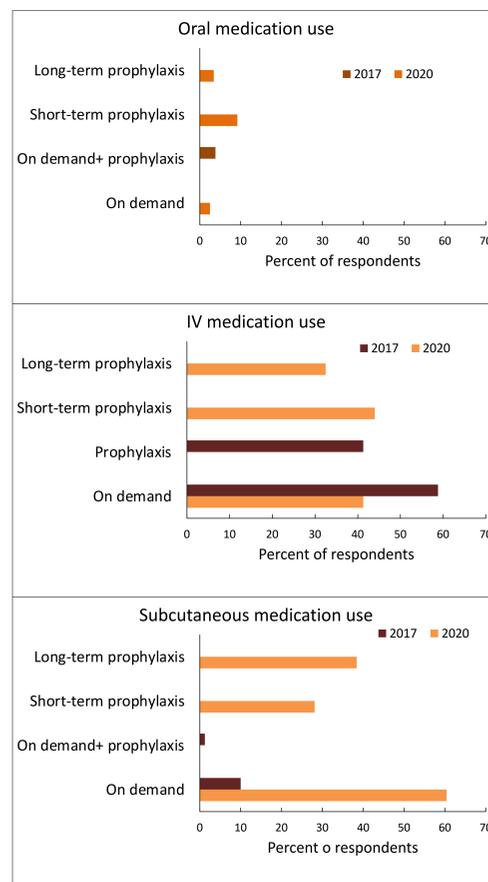


Figure 1. Comparison of oral, IV and subcutaneous medication use between 2017 and 2020. In the 2017 survey, long- and short-term prophylaxis were not differentiated. Percentages may exceed 100% because of multiple categories for use.

Results (continued)

The results in Figure 1 suggest that:

- Oral medications (androgens and tranexamic acid) are used by less than 10% of respondents and this has not changed significantly.
- On demand use of IV medications appears to have declined in 2020 compared to 2017.
- The use of subcutaneous medication has increased significantly in 2020 particularly for on-demand treatment but also for prophylaxis. Note that in 2017 icatibant was the only subcutaneous medication and was approved for on-demand use. In 2020, additional medications were approved for both on-demand and prophylactic use.

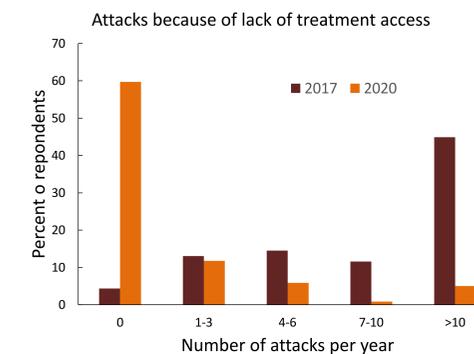


Figure 2 Number of attacks per year attributed to lack of access to treatment (n=69 in 2017 & 162 in 2020). In 2017, the number of attacks due to lack of treatment access was zero for 4.35% of respondents while in 2020 it was zero for 59.7%.

Conclusions

- The use of newer, self-administered subcutaneous medications for HAE has significantly increased between 2017 and 2020 in particular for on-demand treatment.
- In parallel, the percent of patients with attacks not treated because of lack of access to medication has decreased significantly.
- Medications which are easy for patients to use are immediately accessible, and can be taken at home, can reduce attacks and treat breakthrough attacks.
- Easy to use treatments (subcutaneous or oral) are desired by HAE patients and will likely have a significant impact on their quality of life.

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