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Abstract

Introduction: Types I and II hereditary angioedema (HAE) comprise a rare genetic disorder which results in low levels of C1 inhibitor (C1-INH) or its function and unpredictable attacks of angioedema. Over the last several years, newer treatments with easier delivery have been approved for prophylactic treatment of these patients. We explored the use and impact of these newer treatments.

Methods: Online surveys were sent to Canadian HAE patients in 2017 and 2020 to better understand their treatment and health burden. We extracted the responses of patients who reported having Type I or II HAE to evaluate treatment use and attack frequency. Data was analyzed as the percent of responses to a given question.

Results: There were 56 respondents who self-identified as Type I/II in 2017 (75% female, 46±16 years of age) and 106 in 2020 (76% female, 54±16 years of age).

In 2017, 28 of 56 used prophylaxis with 24/28 (86%) taking IV C1-INH and 4/28 (14%) oral medications (androgen or tranexamic acid). In 2020, 71 of 106 used prophylaxis: C1-INH, IV: 27/98 (28%) and subcutaneous: 31/98 (32%); lanadelumab: 10/98 (10%); and oral medication (androgen) 2/98 (2%). Consequently, the percent of patients without attacks increased from 7.8% in 2017 to 22% in 2020 and those who reported having no attacks for which they would have gone for treatment were it available rose from 6.7% to 61%. In 2020, treatment costs were borne either by a government plan (80%), private insurance (11%), a clinical trial (7.6%) or the patient (1.1%), (n=92). This information was not obtained in 2017. Visits to the hospital, emergency room and clinic were unchanged.

Conclusion: The addition of newer prophylactic treatments which can be administered at home subcutaneously has benefited HAE patients by reducing attacks and attacks requiring treatment between 2017 and 2020.

Introduction

HAE results from rare genetic disorders leading to low and/or dysfunctional C1 inhibitor (C1-INH). It is characterized by painful, unpredictable episodes of edema of the face, larynx, abdomen, genitals and extremities and can be fatal if not treated quickly. Over the last several years, newer treatments with easier delivery have been approved for prophylactic treatment of HAE. We explored the use and impact of these newer treatments through two surveys conducted 3 years apart.

Objective

To evaluate the impact of newer approved treatments on the health burden of Canadian HAE patients by comparing data from surveys in 2017 and 2020.

Methods

Online surveys were sent to Canadian HAE patients in 2017 and 2020 to better understand their treatment and health burden. From these surveys, we extracted the responses of patients who reported having Type I or II HAE to evaluate treatment use and attack frequency. Data was analyzed as the percent of responses to a given question.

Results

	2017 (n=56)	2020 (n=106)
Age (years)		
mean (range)	46 (17-79)	53 (23-91)
Gender n (%)		
Female	42 (75%)	81 (76.4%)
Male	14 (25%)	24 (22.6%)
Using medication to treat HAE n (%)		
Yes	54 (98%)	94 (89%)
No	1 (2%)	12 (11%)

Table 1. Demographics and baseline characteristics. There were twice as many survey respondents with Type I/II HAE in 2020. In both years, the majority of patients were female and taking medication to treat their HAE.

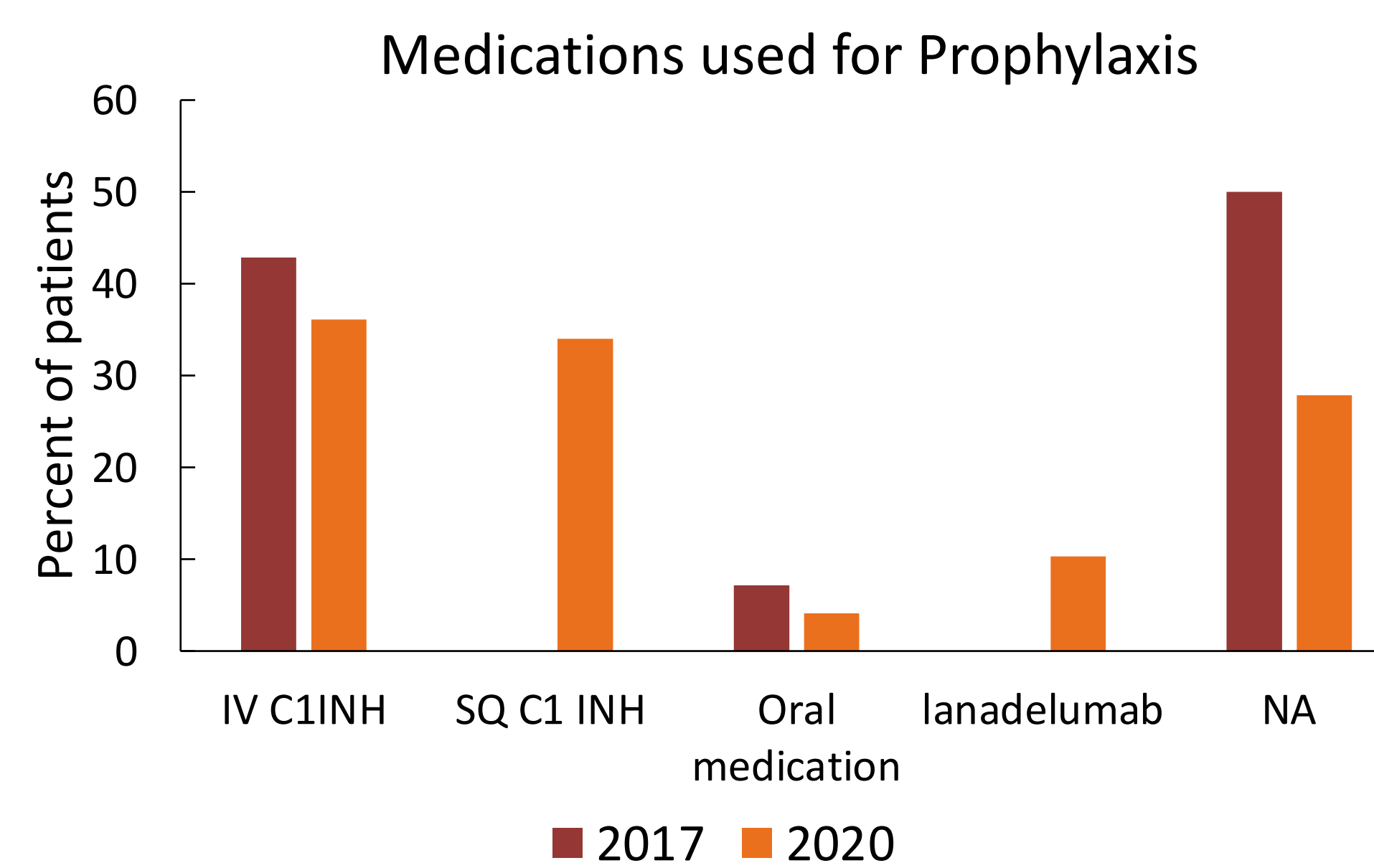


Figure 1. In 2017, 50% (28/56) of respondents indicated using prophylactic HAE treatments; in 2020 prophylactic treatment increased to 67% (71/106) of respondents. There was also a shift in medication used for prophylaxis in 2020. Lanadelumab and subcutaneous (SQ) pdC1 INH were added and the proportion of patients indicating Not Applicable (NA) was reduced to nearly half. It is likely that the majority of those choosing NA were receiving on-demand treatment for their HAE.

Results (continued)

In 2020, treatment costs were borne either by a government plan (80%), private insurance (11%), a clinical trial (7.6%) or the patient (1.1%), (n=92). This information was not obtained in 2017.

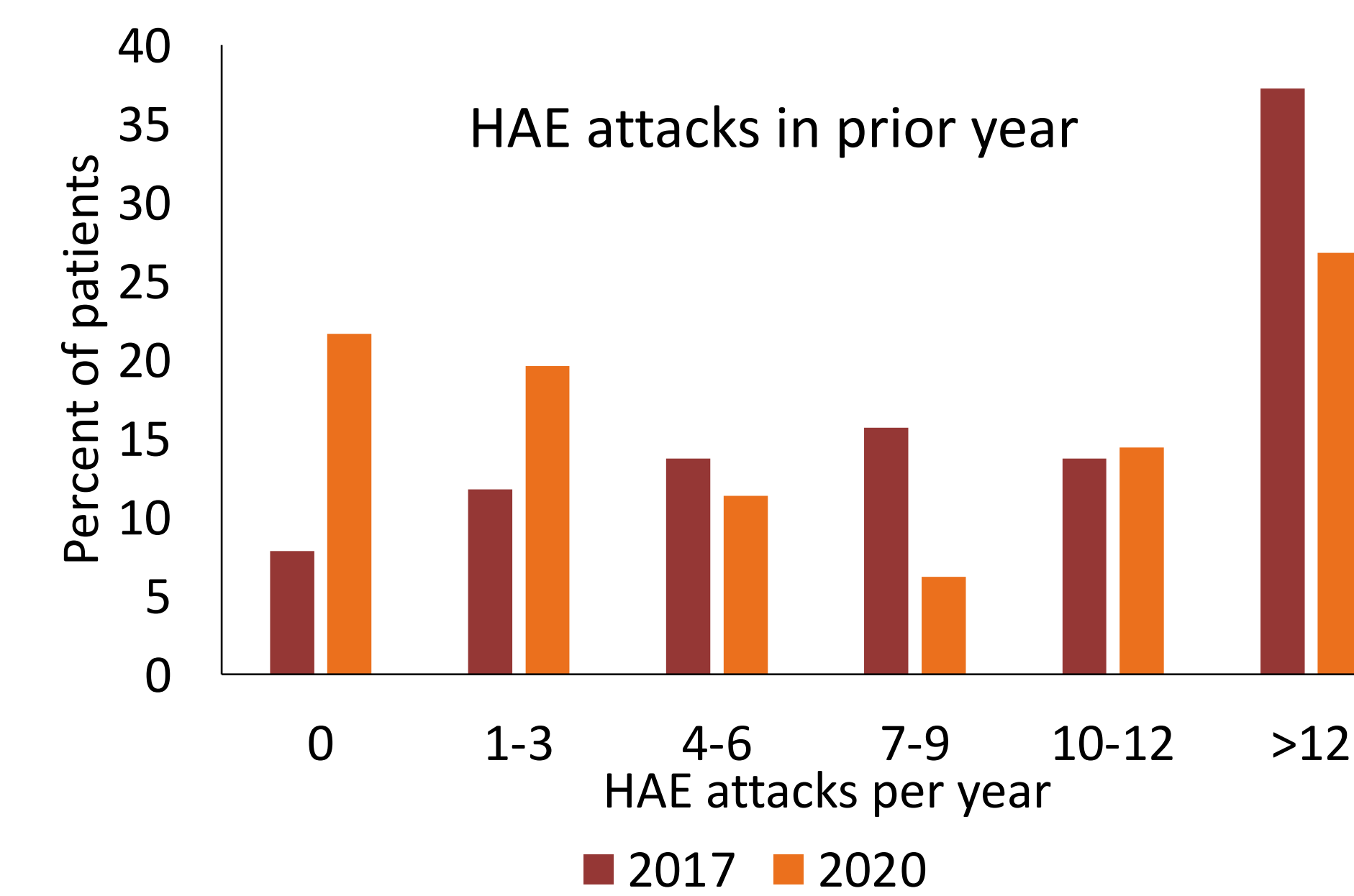


Figure 2. In conjunction with the increased use of prophylaxis, in 2020 (n=97/106) there was a significant increase in the proportion of patients reporting no attacks or 1-3 attacks in the prior year and a decrease in those reporting more than 12 compared to 2017 (n=51/56).

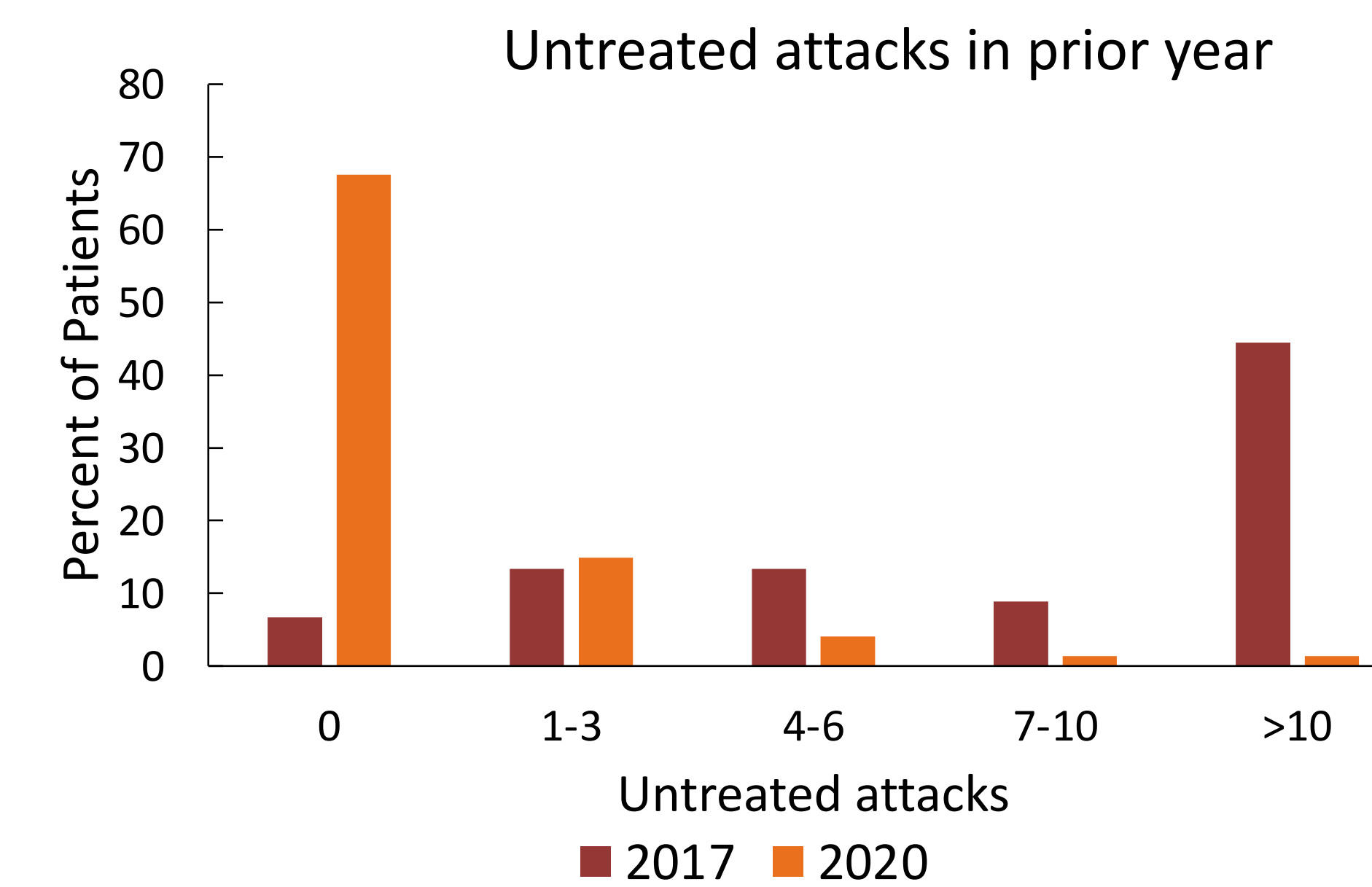


Figure 3. Those who reported having no attacks for which they would have gone for treatment were it available rose from 6.7% in 2017 to 61% in 2020. Consequently, there was a decrease in the percentage of patients reporting multiple untreated attacks.

Results (continued)

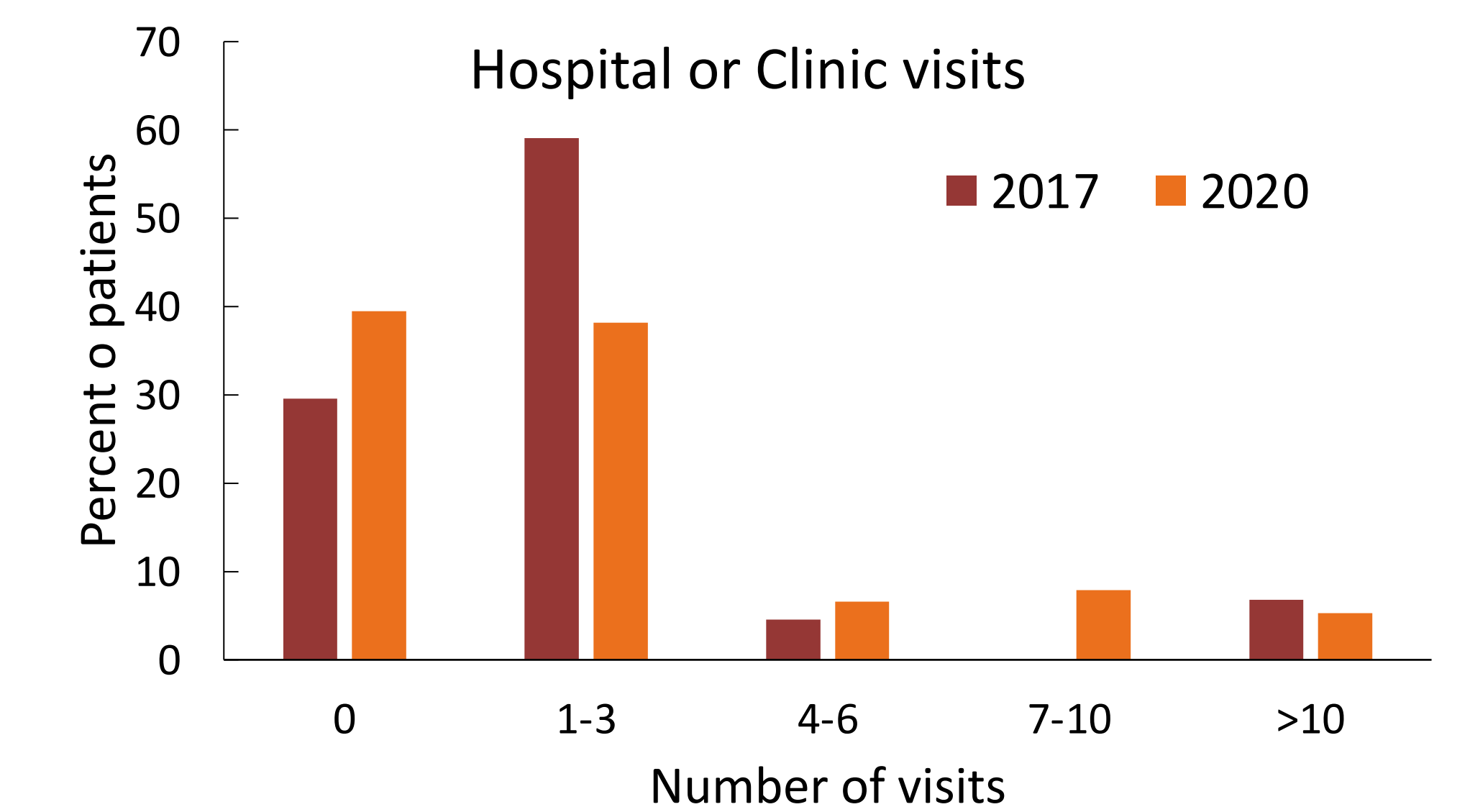
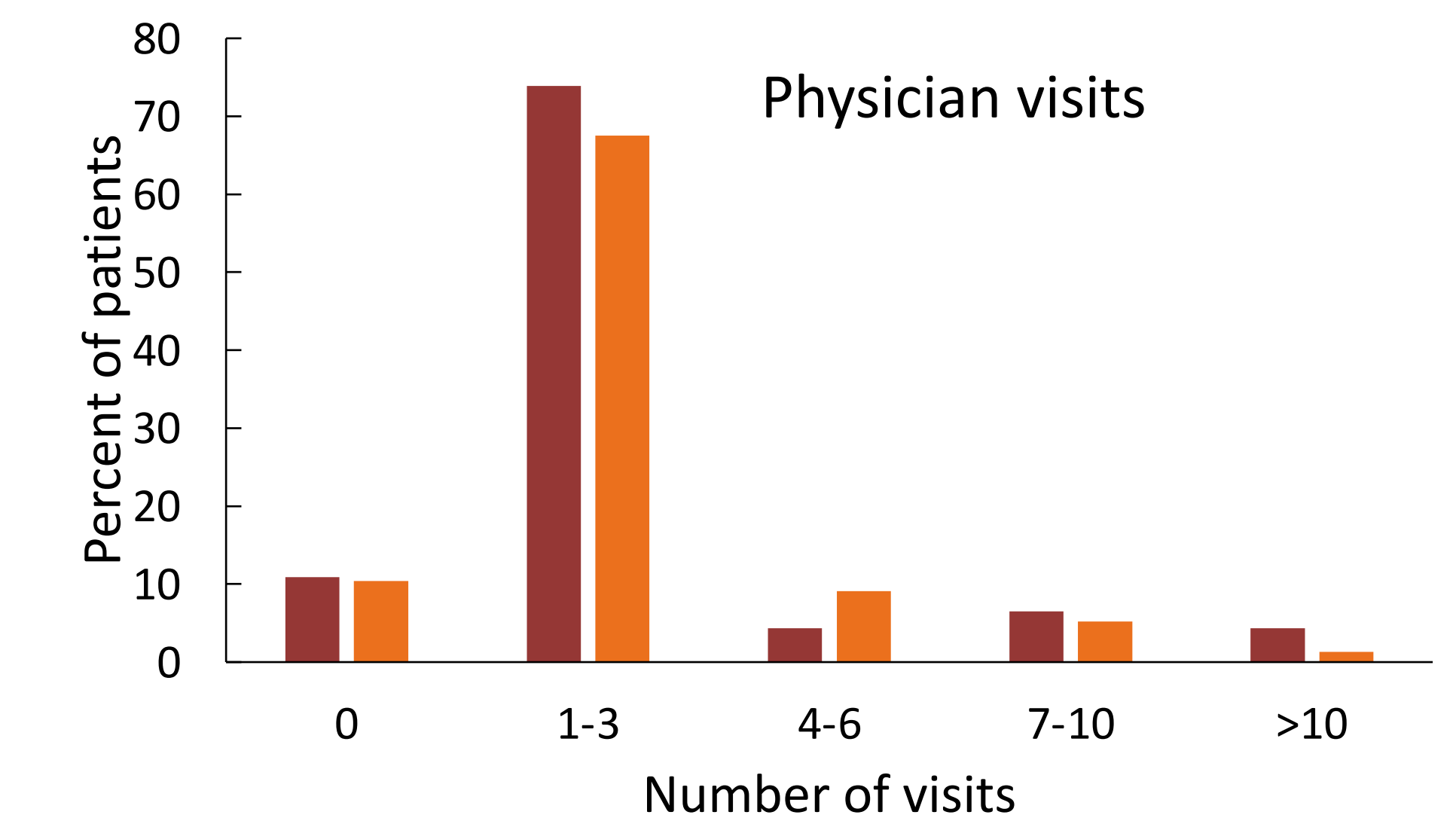


Figure 4. Despite a reduction in attacks, physician visits were similar in 2017 and 2020. However, there was a shift to fewer hospital/clinic visits.

Conclusions

Between 2017 and 2020, new prophylactic treatments administered subcutaneously became available or HAE patients.

As a result, there was a decrease in HAE attacks and in untreated attacks. This did not impact physician visits, but hospital/clinic visits were reduced.

Acknowledgements

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