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## Abstract

**Background:** Hereditary angioedema (HAE) is a chronic spontaneous life-threatening disease. Until recently, treatment options for HAE have been limited and required infusion. New treatment options would be beneficial to this population.

**Methods:** In 2019, HAE Canada conducted an online survey of patients and caregivers to assess the challenges patients and caregivers face as a result of hereditary angioedema and to gain insight into their experience and expectation with therapies used to treat hereditary angioedema.

**Results:** Of 73 respondents to the questions, 68 were living with HAE and 6 were providing care to a patient with HAE. Of the respondents, 43/50 (86%) indicated that access to new treatments is extremely important. Attributes for new medications that were considered extremely important were: more convenient dosing interval - 38/58, reduction in edema attacks - 47/57, easier mode of delivery 44/58, prophylactic administration - 44/56. Eight participants (13%) had received treatment with lanadelumab a newly approved medication. On a scale of 1 to 5, five participants rated its effectiveness preventing attacks at 5 and 1 each rated it a 3 or 4. Reported adverse events were headache (2/8) and pain at injection site (7/8) scored as tolerable to very tolerable. The majority (5/8) indicated their quality of life while taking lanadelumab to be comparable to normal living. Access to lanadelumab was predominantly through participation in a Canadian clinical trial (5/8), a compassionate access program (1/8) or through private insurance (1/8).

**Conclusion:** The data collected indicates that newer, more effective and more convenient treatments for are wanted by Canadian HAE patients. The newest approved treatment, lanadelumab, which is given by subcutaneous injection every 2 weeks fulfils some of these requirements according to a limited number of patients. It is important that Canadian HAE patients have access to multiple treatments to address the unpredictable nature of this disease.

## Introduction

Hereditary angioedema (HAE) is a chronic spontaneous life-threatening disease. Until recently, treatment options for HAE have been limited and required infusion. New treatment options would be beneficial to this population.

## Objective

To assess challenges HAE patients and their caregivers face and to gain insight into their experience and expectation with therapies used to treat hereditary angioedema

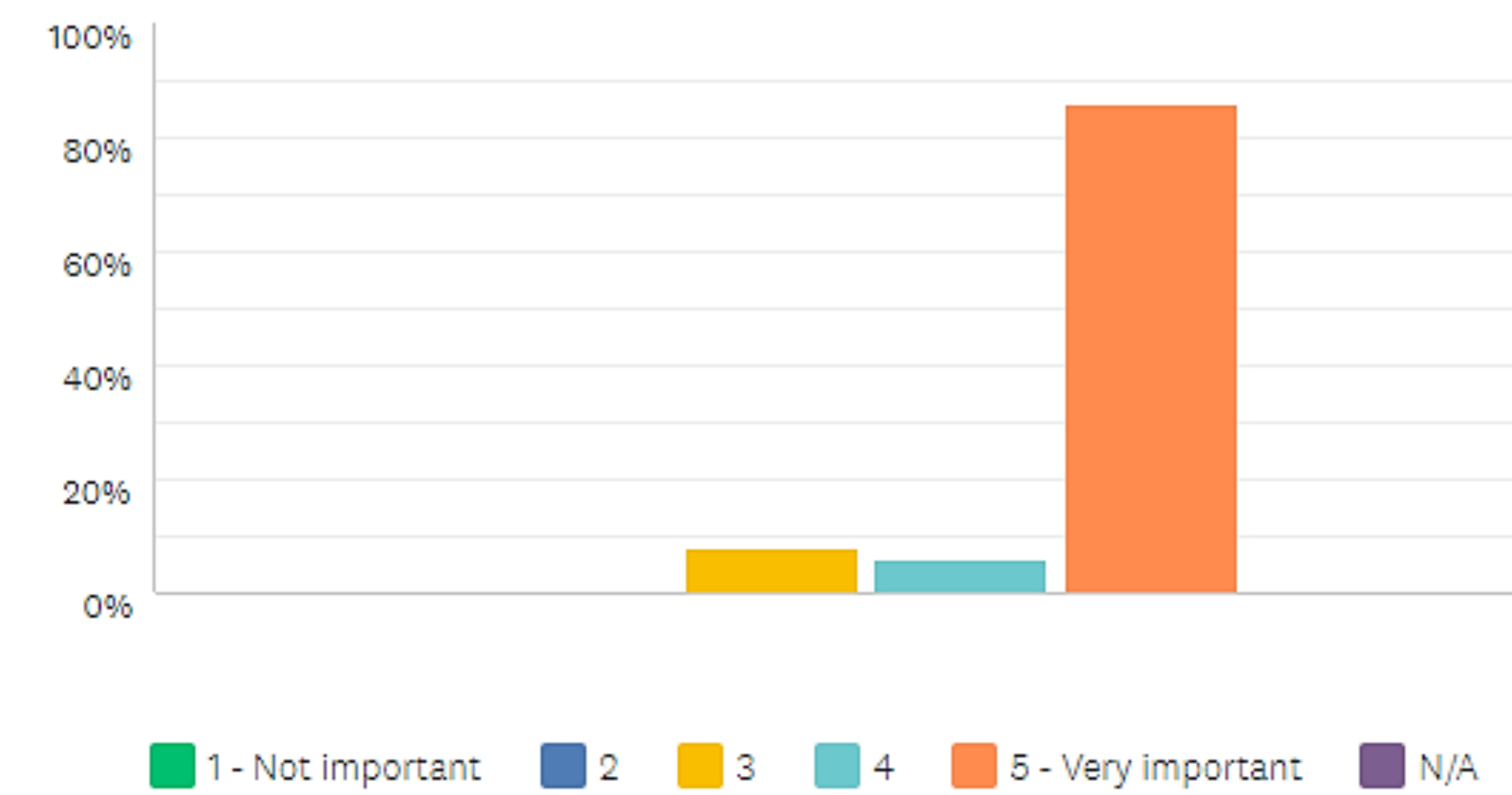
## Methods

In 2019, HAE Canada conducted an online survey of patients and caregivers to assess the challenges patients and caregivers face as a result of hereditary angioedema and to gain insight into their experience and expectation with therapies used to treat hereditary angioedema.

Responses to 6 of the survey questions were analyzed and are reported here.

## Results

Of 73 respondents to the questions, 68 were living with HAE and 6 were providing care to a patient with HAE.

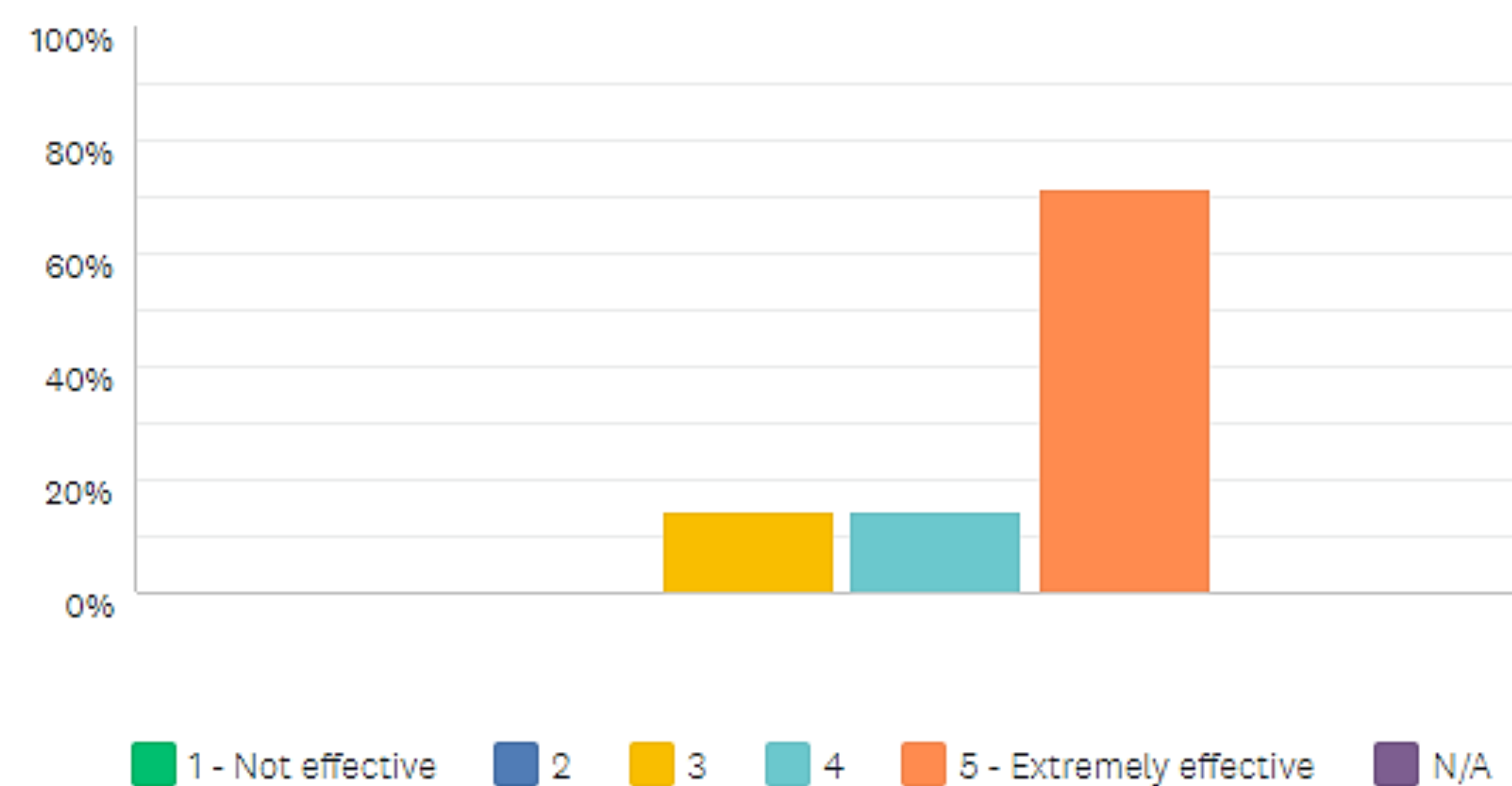


**Figure 1** Most (43/50) respondents indicated that access to new treatments was extremely important to them.

### Level of importance

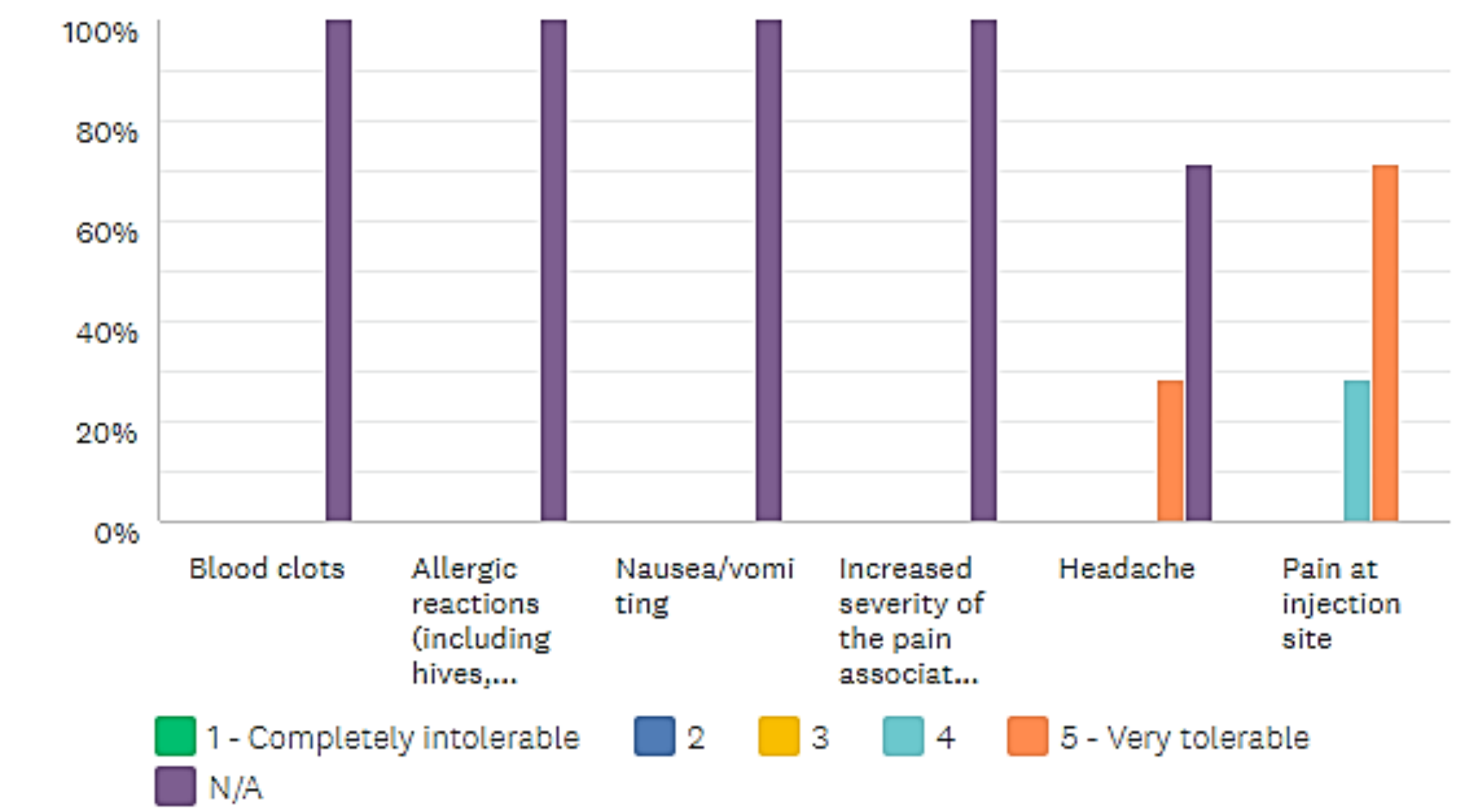
	1 Not important	2	3	4	5 Extremely important
A more convenient dosing interval/less frequent dosing	9.52%	2.38%	9.52%	14.29%	64.3%
Improved management/reduction in attacks of edema (swelling)	0.00%	0.00%	4.76%	9.52%	85.71
Easier mode of delivery as a subcutaneous option (vs. IV)	2.38%	2.38%	2.38%	14.29%	76.19
Option to administer prophylactically before known triggers (eg. traveling, dental procedures).	2.44%	0.00%	2.44%	12.20%	80.49

**Table 1** These attributes of a new medication were considered extremely important by a majority of surveyed patients.

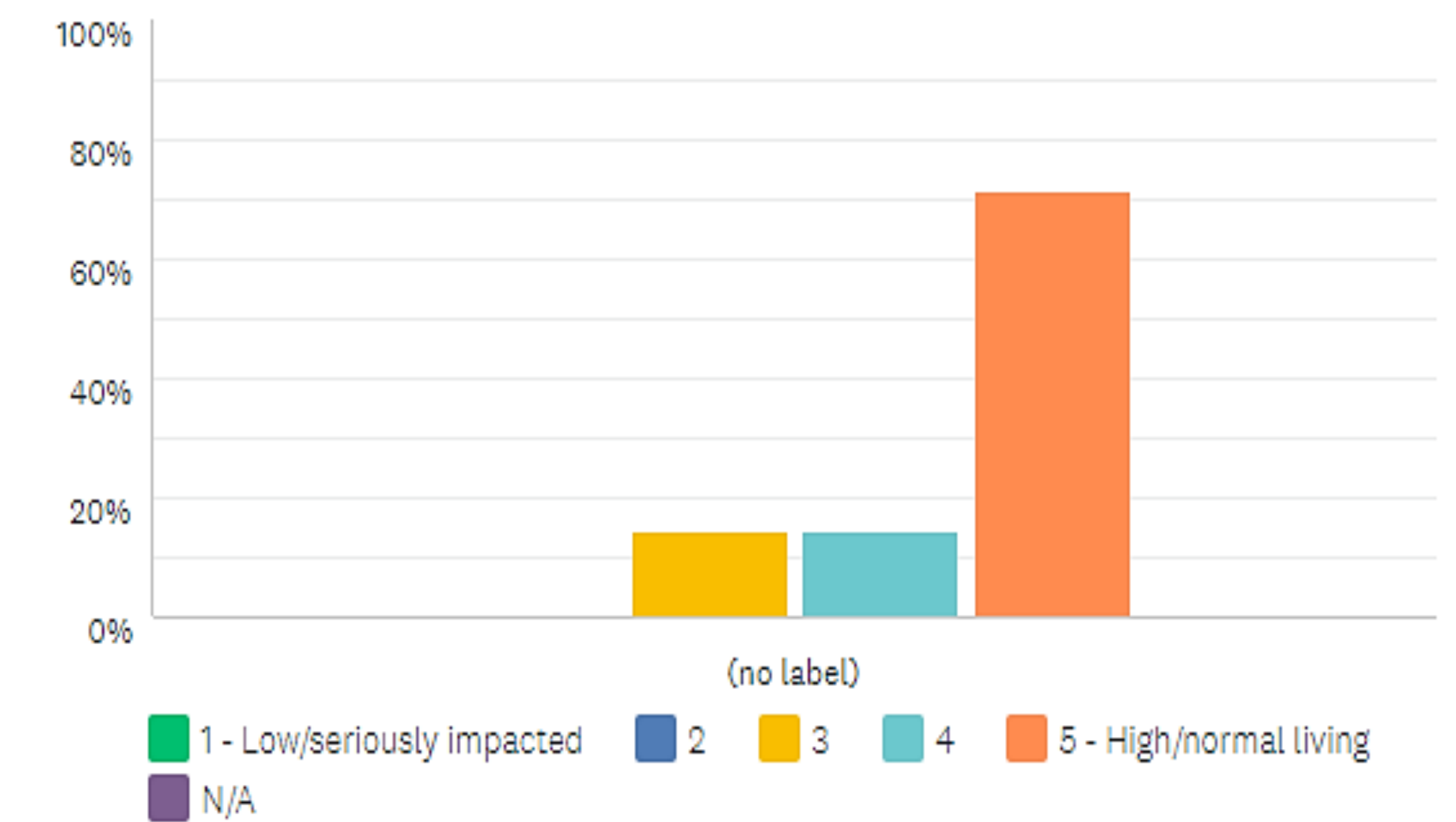


**Figure 2** Eight participants (13%) had received treatment with lanadelumab through participation in a clinical trial (6), compassionate access (1) or private insurance (1). Five participants rated it extremely effective in preventing HAE attacks. Of these, 4 received treatment in a clinical trial.

## Results (continued)



**Figure 3** Reported adverse events were headache (2/8) and pain at injection site (7/8) scored as tolerable to very tolerable.



**Figure 4** The majority of participants (5/8) indicated that their quality of life while taking lanadelumab was comparable to normal living.

## Conclusions

- The data collected indicates that newer, more effective and more convenient treatments for are wanted by Canadian HAE patients.
- The newest approved treatment, lanadelumab, which is given by subcutaneous injection every 2 weeks fulfils some of these requirements according to a limited number of patients.
- It is important that Canadian HAE patients have access to multiple treatments to address the unpredictable nature of this disease