

Real world data of Canadians living with Hereditary Angioedema (HAE): Need for Innovative, Newer Prophylactic and Subcutaneous Treatments.

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Background: Hereditary angioedema (HAE) is a rare inherited disorder affecting the production and/or function of C1-Esterase Inhibitor (C1INH) and leading to acute attacks of swelling which can be fatal if the oropharynx is affected. The timing and severity of an attack are unpredictable, potentially leading to stress, anxiety and depression.

Methods: In 2019, HAE Canada conducted two online surveys to assess the challenges faced by HAE patients and their caregivers and to gain insight into their experience and expectations with new therapies, HAEGARDA (human plasma-derived, pasturized, nanofiltered C1 inhibitor, given subcutaneously) and Takhzyro (lanadelumab - fully human monoclonal antibody-plasma kallikrein inhibitor, subcutaneous) in particular.

Results: The first survey had 73 respondents: 68 (92%) were individuals with HAE, and 6 (8%) were caregivers. In response to the question “Do you have regular fear of unpredictable, debilitating attacks?” 50 of 68 respondents answered yes. Of these, 30% rated their fear as mild, 62% as moderate and 8% as severe. Sixty-three percent indicated that fear of attacks led to generalized anxiety. The majority of patients (91%) are treated with an IV medication indicated for acute treatment. When asked to rate attributes of new medications for HAE, 79% rated prophylactic administration and 76% rated subcutaneous delivery as extremely important. Eight participants (13%) had received treatment predominantly through participation in a clinical trial with Takhzyro, a newly approved medication for prophylaxis of HAE. Five considered it extremely effective in preventing attacks. Reported adverse events were headache (2/8) and pain at injection site (7/8). Both were scored as tolerable to very tolerable.

The second survey, intended to capture information about HAEGARDA, had 19 respondents, 3 of whom had received treatment with HAEGARDA. It was found to be “extremely effective” by all 3 respondents and significantly reduced attacks for 2 of them. Reported adverse events headache (1/3) and injection site reaction (2/3) scored as tolerable to very tolerable.

Conclusion: Prophylactic treatment which can reduce attacks and subcutaneous delivery are considered important by Canadian HAE patients. Two newly approved medications, HAEGARDA and Takhzyro, have these attributes.

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