

Informed decision-making in hereditary angioedema prophylaxis

An ultra-orphan disease affecting approximately 1 in 50,000 patients in the general population,^{1,2} hereditary angioedema (HAE) negatively impacts patients' lives because of its potentially life-threatening, recurrent episodes of swelling affecting many areas of the body. The HAE prophylaxis market continues to evolve, with a variety of treatment options for patients and physicians to choose from, and 2 new U.S. Food and Drug Administration (FDA)-approved treatments available since 2017. Current prophylactic therapies offer patients significant improvement in quality of life, providing a level of control over HAE attacks not previously experienced with on-demand medications.³ With the exception of attenuated androgens, currently available prophylactic therapies are administered by infusion or injection with different dosing frequency and preparation requirements.

As patients become more aware of the challenges of living with and treating their HAE because of the growth of social media and patient advocacy organizations, there is an interesting dynamic of patients and physicians influencing each other regarding treatment choice. Treatment decision-making is a partnership between physicians, who can bring strong clinical knowledge and experience from the patients they treat, and patients, who have individual needs.⁴

Understanding and discussing the factors that are important to patients in choosing a treatment, their preferences, and reasons for those preferences is critical in helping patients manage their HAE with an option that will work best for them. Conversations that address patients' lifestyle and treatment challenges or hesitations in starting prophylactic treatment could help patients better manage their condition. Although efficacy is important, there are other considerations as well, including route of administration. Both patients with HAE and physicians treating HAE patients participating in the 2017 FDA's Patient-Focused Drug Development Initiative Voice of the Patient Summit⁵ indicated a desire for new

treatment options, particularly those that are easier to administer. When asked about the developments they would like to see regarding HAE medications, patients voiced a desire for a noninvasive route of administration.⁶

To better understand the prophylaxis treatment experience, a survey was conducted in 2019 among 100 U.S. adult patients (age 18+ years) diagnosed with type I or II HAE either currently treating their HAE or not currently treating and experiencing at least 1 attack every 3 months. Additionally, 175 physicians (primarily allergists/immunologists and primary care practitioners) actively treating a mean of 6.3 patients with HAE in a typical year were also surveyed on similar topics. Although patients and physicians are becoming more aware of and experienced with HAE prophylactic treatments, according to the survey physicians only prescribe HAE medications for acute treatment to one-quarter of their patients, thus indicating there could be additional opportunities to offer patients a preventative treatment option to help manage their HAE.

Patients are likely to be better satisfied with and potentially more adherent to their treatment if they are involved in selecting it. Two-thirds (67%) of the patients surveyed in 2019 taking the 3 most commonly used prophylactic medications (on average) reported taking their medication as prescribed each time. Although the majority of patients and physicians (65% for both groups) were satisfied with their patients' HAE medications, understanding the reasons for this satisfaction and probing beneath the surface to reveal challenges or areas for improvement could help physicians better tailor treatments to their patients. HAE prophylactic treatment offers quality-of-life benefits and the ability to address an unmet need, resulting in high overall levels of patient satisfaction.⁷⁻⁹ However, patients are less satisfied with specific aspects of medication administration, particularly for intravenous (IV) therapies.⁹

Because learning how to successfully administer injectable treatments can be challenging for patients,

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the route of administration can be an important factor in choosing a therapy for HAE prophylaxis. Although physicians may understandably be inclined to focus on treatment efficacy, it is important to understand patients' perspectives when making treatment decisions, especially for a chronic condition requiring lifelong management and for which several treatment options are available. Benefits of shared decision-making have been demonstrated in several allergy and immunology conditions, including improving adherence and outcomes in asthma.¹⁰ Helping patients with HAE make informed choices to manage their HAE by actively listening to patients, understanding their specific situations and preferences, and having a dialogue about available treatment options could further support patients to take an active role in managing their HAE and further improving their quality of life.

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KEYWORDS

hereditary angioedema, HAE, route of administration, patient preference, prophylaxis, decision-making

REFERENCES

1. Zuraw BL, Banerji A, Bernstein JA, et al. US Hereditary Angioedema Association Medical Advisory Board 2013 recommendations for the management of hereditary angioedema due to C1 inhibitor deficiency. *J Allergy Clin Immunol Pract*. 2013;1:458-467.
2. U.S. National Library of Medicine (NLM), MedlinePlus. Hereditary angioedema. Bethesda, MD: NLM; 2020. <https://ghr.nlm.nih.gov/condition/hereditary-angioedema#statistics>. Accessed January 4, 2021.
3. Maurer M, Magerl M, Ansotegui I, et al. The international WAO/EAACI guideline for the management of hereditary angioedema—the 2017 revision and update. *Allergy*. 2018;73:1575-1596.
4. Banerji A, Baş M, Bernstein JA, et al. Expert perspectives on hereditary angioedema: key areas for advancements in care across the patient journey. *Allergy Rhinol (Providence)*. 2016;7:172-181.
5. U.S. Food and Drug Administration (FDA), Center for Biologics Evaluation and Research (CBER). The voice of the patient: hereditary angioedema. A series of reports from the U.S. Food and Drug Administration's Patient-Focused Drug Development Initiative. Report of the U.S. Food and Drug Administration Patient-Focused Drug Development Initiative public meeting September 2017. Silver Spring, MD: FDA, CBER; 2018. <https://www.fda.gov/files/about/20fda/published/The-Voice-of-the-Patient-Hereditary-Angioedema.pdf>. Accessed January 4, 2021.
6. Jose J, Lehman EB, Craig T. Evaluating satisfaction of patients with hereditary angioedema with their past and present treatments: implications for future therapies. *Allergy Asthma Proc*. 2018;39:74-80.
7. Zanichelli A, Azin GM, Cristina F, Vacchini R, Caballero T. Safety, effectiveness, and impact on quality of life of self-administration with plasma-derived nanofiltered C1 inhibitor (Berinert®) in patients with hereditary angioedema: the SABHA study. *Orphanet J Rare Dis*. 2018;13:51.
8. Wang A, Fouche A, Craig TJ. Patients perception of self-administrated medication in the treatment of hereditary angioedema. *Ann Allergy Asthma Immunol*. 2015;115:120-125.
9. Riedl MA, Banerji A, Busse PJ, et al. Patient satisfaction and experience with intravenously administered C1-inhibitor concentrates in the United States. *Ann Allergy Asthma Immunol*. 2017;119:59-64.
10. Blaiss MS, Steven GC, Bender B, Bukstein DA, Meltzer EO, Winders T. Shared decision making for the allergist. *Ann Allergy Asthma Immunol*. 2019;122:463-470.