



HAEA
US HEREDITARY ANGIOEDEMA
ASSOCIATION

Expert Physicians Discuss **GUIDELINE FOCUSED CARE IN HEREDITARY ANGIOEDEMA**

US HAEA Medical Advisory Board 2020 Guidelines for the Management of Hereditary Angioedema
Available at: <https://www.sciencedirect.com/science/article/pii/S2213219820308783?via%3Dihub>

Jointly provided by the Postgraduate Institute for Medicine, US Hereditary Angioedema Association (HAEA), and RMEI Medical Education, LLC.

 Postgraduate Institute for Medicine  **HAEA** US HEREDITARY ANGIOEDEMA ASSOCIATION  **RMEI** MEDICAL EDUCATION FOR BETTER OUTCOMES

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Learning Objectives

-  Summarize important considerations in prophylactic treatment for HAE-C1INH, including recent therapeutic advances and patient specific factors
-  Discuss the diagnostic and treatment approaches for patients with HAE-nl-C1INH
-  Develop individualized management plans, including logistics, education, and monitoring for HAE special patient populations

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Long-term Prophylaxis for HAE: When and How to Use in the Management Plan



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Hereditary Angioedema: Long-term Prophylaxis

? Who should be on long-term prophylaxis (LTP)?

Based on the needs of individual patients':

-  Treatment preferences
-  Attack frequency and severity
-  Comorbid conditions
-  Access to emergent treatment
-  Quality of life



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Hereditary Angioedema: Long-term Prophylaxis

- LTP decisions cannot be made based on rigid criteria
- Initiation or continuation of LTP periodically reviewed/discussed due to changes in condition, life circumstances, and patient preference



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Hereditary Angioedema: Long-term Prophylaxis

Treatment options for LTP in HAE-C1INH



First-line Medications

- C1INH Concentrates
 - SQ pdC1INH (Haegarda®)
 - IV pdC1INH (Cinryze®)
- Lanadelumab
 - SQ plasma kallikrein inhibitor (Takhzyro®)
- Berotralstat
 - Oral plasma kallikrein inhibitor (Orladeyo™)



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Hereditary Angioedema: Long-term Prophylaxis

Treatment options for LTP in HAE-C1INH



Second-line Medications

- Attenuated Androgens
 - Danazol, stanozolol, oxandrolone
- Antifibrinolytics
 - Tranexamic acid, aminocaproic acid



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Hereditary Angioedema: Long-term Prophylaxis



Treatment considerations when developing LTP management plan

- Efficacy
- Safety including potential long-term effects
- Tolerability, burden of treatment, adherence
- Special considerations (pregnancy, lactation)



Shared Decision Making



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HAE-nI-C1INH: How to Diagnose and Treat



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HAE-nI-C1INH: Diagnosis

- Lack of available diagnostic tests
 - Genetic: FXII, PLG, KNG, ANGPT, MYOF
 - Biochemical: Investigational assays
- Challenge of avoiding overdiagnosis or underdiagnosis
 - Similarities to HAE-C1INH, some differences (female predominance, variable penetrance, and estrogen sensitivity)



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HAE-nI-C1INH: Diagnosis

- Clinical criteria
 - Recurrent angioedema in absence of urticaria
 - Exclusion of causative medication
 - Normal C1INH testing
 - Identified HAE-nI-C1INH mutation OR documented resistance to mast-cell targeted preventative therapy plus family history



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HAE-nI-C1INH: Diagnosis

- Other supportive data
 -  A history of rapid, durable response to a bradykinin-targeted medication
 -  Predominant documented visible angioedema; or with predominant abdominal symptoms, evidence of bowel wall edema documented by CT or MRI
- Requires close clinical follow-up and assessment with treatment
- Evaluation/diagnosis by an experienced angioedema specialist is recommended

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HAE-nI-C1INH: Treatment

- Less treatment data compared to HAE-C1INH
- Acute treatment
 - Similar approach to HAE-C1INH; assess for reliable, consistent benefit of medication
- Prophylactic treatment
 - Differences in approach based on available data
 - Tranexamic acid
 - Progestins



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Enhancing QoL in HAE: Developing a Multidisciplinary Approach

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Enhancing QoL in HAE



Patient Empowerment

- Shared decision making
- Frequent communication
- What is important to the patient? (treatment goals, work, school, family, leisure activities, and/or travel)



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Enhancing QoL in HAE



Monitoring Outcomes

- Clinical follow-up with an expert HAE physician
- Patients record HAE symptoms and medication use
- Monitoring efficacy and safety of treatment plan
- Assessing impairment in daily activities from HAE symptoms or treatment burden
- Quality of life assessment (validated tools available)



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Enhancing QoL in HAE

- Pharmacoeconomic considerations
- Economic costs
 - Direct and indirect
 - Immediate and long-term
- Cost and impact on family
- Self-administration management plans to reduce treatment costs
- Information and valuable resources available at www.HAEA.org
 - A template of a general letter of medical necessity developed at the UCSD US HAEA Angioedema Center is available in the Guidelines located here: <https://www.sciencedirect.com/science/article/pii/S2213219820308783?via%3Dihub>



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Enhancing QoL in HAE

Recent study data on modern LTP treatments for HAE

 Clinically significant improvements in QoL

 Reduce direct and indirect costs related to on-demand only treatment

Castaldo AJ, et al. *Allergy Asthma Proc.* 2021 Feb 13. Online ahead of print.



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Thank You for Participating

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