Towards Better Diagnosis and Care for HAE and Rare Disease Patients in Europe

The new EU mandate presents a unique opportunity to boldly address rare diseases by dismantling the barriers patients face in disease management. This document outlines recommendations to enhance diagnosis, treatment, and care, and to accelerate access to life-saving and life-changing therapies for people with HAE and other rare diseases.

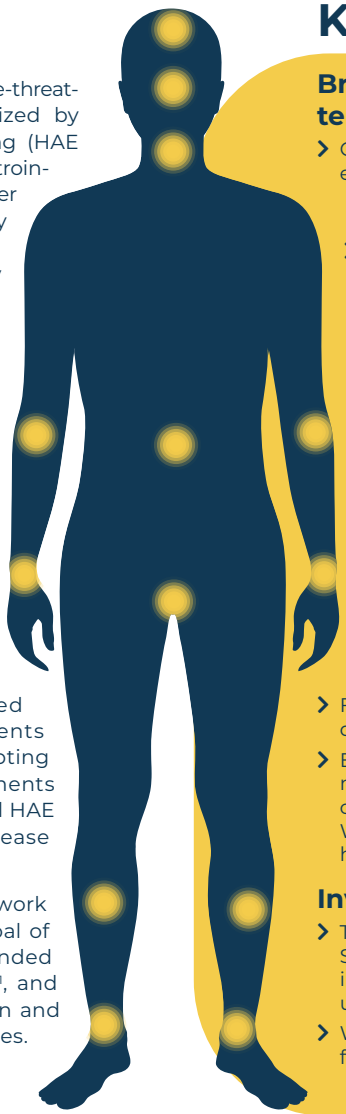
Understanding Hereditary Angioedema (HAE)

HAE is a debilitating and potentially life-threatening genetic disorder characterized by episodic and unpredictable swelling (HAE attacks) in the hands, feet, face, gastrointestinal tract and airway, among other locations. This condition significantly impacts patients' quality of life, with painful attacks that can disrupt daily activities and increase anxiety and depression.

Current Challenges

The journey to diagnosis of HAE can often last close to a decade, as it is frequently misdiagnosed. While three types of treatments exist: on-demand treatment (to manage HAE attacks when they occur), short-term prophylaxis (taken before surgery or dental procedures), and long-term prophylaxis (regular treatment to prevent swelling attacks), access to these treatment options is fragmented across the EU and many patients continue to suffer from life-disrupting attacks. While a variety of treatments are currently available, there are still HAE patients experiencing a high disease burden.

All relevant stakeholders need to work together towards achieving the goal of total control of HAE, as recommended by international expert guidelines[1], and to support broad access to modern and next-generation prophylaxis therapies.



Key Recommendations

Broaden Access to a choice of modern long term-prophylaxis treatments:

- Governments need to recognise the physical, psychological, economic, and social burden of living with HAE and support broad access and reimbursement for modern long-term prophylaxis therapies against HAE therapies.
- Cooperation and dialogue between regulators and policymakers, industry, payers and patients should be supported, with the aim to achieve an increasingly aligned and predictable pathway for the development, approval, reimbursement, and access of new therapies that allow patients to live a normal life.

On-demand therapies should be available in all countries:

- Governments must ensure that all patients, whether using modern long-term prophylaxis or not, have access to on-demand therapy in case an attack occurs.

Empower Patient Voices:

- Prioritize shared decision-making between patients and their physicians where patients' choices and lifestyles are reflected in the management of the disease.

Enhance Expert Networks:

- Foster continuous education for healthcare professionals to expedite diagnosis and early referral to specialised care.
- Enhance European Reference Networks by linking them with existing networks of medical excellence (including Angioedema Centers of Reference and Excellence (ACARE) and the EAACI Angioedema Working Group) and boost cross-border knowledge sharing to ensure high-quality, and consistent care for all patients.

Invest in Research and Development:

- The upcoming Pharmaceutical Legislation review, and Life Science Strategy offer a rare chance to ensure that patients can benefit from incremental innovations that address their complex and evolving unmet needs.
- We urge policymakers to support predictable and fit for purpose frameworks to incentivize, evaluate and reward innovation and drive equitable access to the life-saving treatments for patients.

Call TO Action

- Keeping rare diseases a top health priority of the EU institutions in the 2024-2029 political term is crucial to closing knowledge gaps, improving diagnosis and care, and ensuring equal access to innovative treatments for European patients.
- We join the rare diseases community in calling for a comprehensive EU Action Plan on Rare Diseases to drive coordinated, continued and lasting progress for those affected.
- Through enhanced multi-stakeholder collaboration, we can holistically address the challenges faced by HAE and rare disease patients to inform decision-making in health policy.

About this document

This document was initiated and funded by CSL Behring with input from Henrik Balle Boysen (President, HAEi the global HAE patient organisation) and Dr Mauro Cancian (Head of Allergology Unit, Padua University Hospital) and is based on the outcomes of the policy event 'Towards Better Diagnosis and Care for HAE Patients in Europe' supported by CSL Behring.

MEP Endorsers

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1. WAO/EEACI treatment guidelines (2021)