

THE UNIQUE NEEDS OF CHILDREN WITH HAE

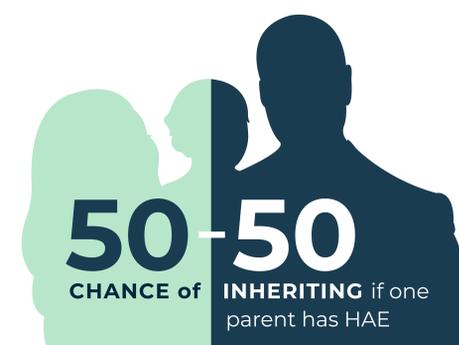
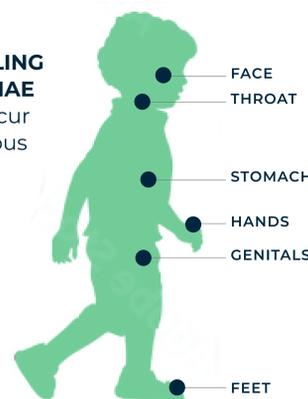


Hereditary angioedema (HAE) is a rare, inherited disease characterized by repeated painful and unpredictable episodes (or attacks) of severe swelling in various parts of the body.¹ Swelling in the airway can restrict breathing and be fatal,² and asphyxia can develop more rapidly in children due to the smaller diameter of their airway.³

Episodes may occur in response to a concurrent illness, physical trauma or stress (positive or negative), however, attacks can be unpredictable, and swelling often occurs without a known trigger.²

HAE affects about 1 in 50,000 people in the United States and, in most cases, is autosomal-dominant, meaning children of a parent diagnosed with HAE have a 50% chance of inheriting it.⁴

SWELLING from HAE can occur in various places

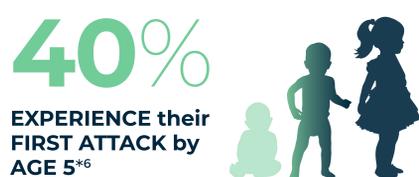


HAE frequently presents in childhood, and delays in diagnosis are common

Symptoms of HAE usually appear early in life, and many patients experience their first attack long before puberty.⁵

Children and adolescents with HAE between the ages of 4-15 can experience 30+ attacks per year on average, according to findings of one study.⁷ Generally, earlier symptom onset is associated with more significant disease activity and a higher burden.⁸ Frequency and severity also tend to increase with the onset of puberty.⁹

Because HAE is so rare, it can take as long as a decade to receive an accurate diagnosis after symptoms are first experienced,² and delayed diagnosis is a significant concern. Even the presence of a family history of HAE does not guarantee children will be diagnosed earlier than others.



In addition, up to 25% of HAE cases are de novo, meaning there is no prior family history.¹⁰

Therapies for HAE fall into two categories

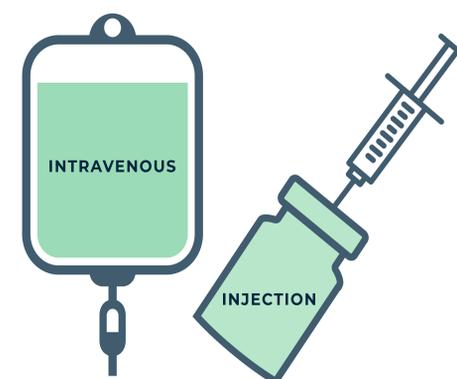
On-demand therapy is used for acute HAE attacks and focuses on reducing the severity and duration of an ongoing attack, with the goal of resolving symptoms as quickly as possible.¹⁴

Long-term preventative, or prophylactic, therapy (LTP) focuses on reducing the number and severity of HAE attacks and associated burden of disease, and restoring normal QoL.¹⁵

The burden of injectable HAE treatments for children and caregivers is multifactorial

Treatments that are delivered intravenously (IV) or through subcutaneous injection (under the skin) can be inconvenient, burdensome and painful.¹⁴ In some instances, administration of acute treatment must also be done in an HCP setting, such as when venous access cannot be obtained or in the case of laryngeal attacks.

Aside from the physical and mental challenges that can accompany an HAE diagnosis, treatment administration can create an additional burden.¹⁶



Injectable or IV LTP administration may cause caregivers and children with HAE to feel nervous, overwhelmed and stressed, and such emotions and stressors can actually trigger HAE attacks.¹⁴ This can result in suboptimal use of LTP, as some HCPs are hesitant to prescribe LTP to children, and some caregivers are hesitant to pursue LTP for their child.

HAE can negatively impact children's quality of life, mental health & development

Regardless of age, HAE can be burdensome both physically (pain, swelling, other attack symptoms) and psychologically (fear of an attack, isolation, etc.) and can have a significant impact on quality of life (QoL), mental health and other developmental elements.

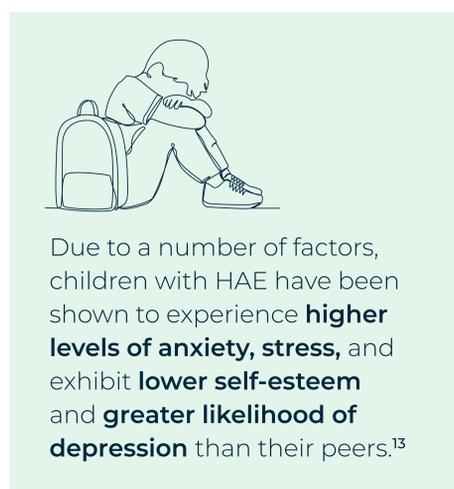
Uncontrolled HAE can significantly interfere with the ability to live a "normal" life.

Children with HAE are likely to:

- **miss school days;** on average, 20 days are missed annually¹¹
- **reduce or eliminate extra-curricular activities** such as sports, dance, scouting or joining a club¹²
- **have difficulty traveling** due to the requirements of some medications and accessibility to appropriate medical facilities¹²

The perceived impact of HAE on QoL may be altered or diminished as caregivers and patients grow increasingly accustomed to the burden of living with HAE – some may even begin to "normalize" the challenges.

THE BURDEN of HAE can be both physical & psychological



Oral delivery of LTP therapy provides a more child-friendly administration option

The significant impact of HAE can include medical trauma and long-term psychosocial impacts from the condition and/or treatments.

Less burdensome, more convenient administration of effective LTP treatments is highly desirable and could make a meaningful difference for younger patients and their caregivers.¹⁷

The availability of this type of therapy may also have a positive impact on overall patient QoL by allowing them to live a more normal life.

Recently, treatment options have expanded to include **ORAL ADMINISTRATION.**



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