#### 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.<sup>1</sup> Swelling in the airway can restrict breathing and be fatal.<sup>2</sup>

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.<sup>2</sup>

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.<sup>1</sup>

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

Symptoms of HAE usually appear early in life, with the mean age of the first HAE attack being 10 years,<sup>3</sup> though many patients experience their first attack long before puberty.<sup>4</sup>

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.<sup>5</sup> Generally, earlier symptom onset is associated with more significant disease activity and a higher burden.<sup>6</sup> Frequency and severity also tend to increase with the onset of puberty.<sup>7</sup>

Because HAE is so rare, it can take as long as a decade to receive an accurate diagnosis after symptoms are first experienced,<sup>2</sup> and delayed diagnosis is a significant concern.<sup>8</sup> Even the presence of a family history of HAE does not guarantee children will be diagnosed earlier than others.

460%

**EXPERIENCE** their FIRST ATTACK by AGE 5\*3

ATTACKS per YEAR\*

YEARS for an ACCURATE DIAGNOSIS

of CASES are DE NOVO

\*on average

In addition, up to 25% of HAE cases are de novo, meaning there is no prior family history.<sup>9</sup>

# 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, embarrassment, etc.) and can have a significant impact on quality of life (QoL), mental health and other developmental elements.

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

#### Children with HAE are likely to:10

- miss school days; some may even miss over 100 days in a single year
- reduce or eliminate extracurricular activities such as sports, dance, scouting or joining a club due to attacks
- have difficulty traveling due to the requirements of some medications and accessibility to appropriate medical facilities.

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

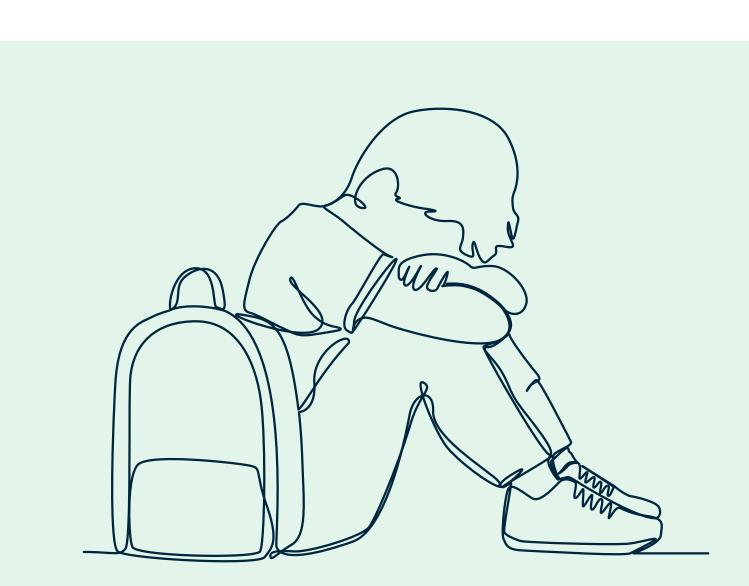




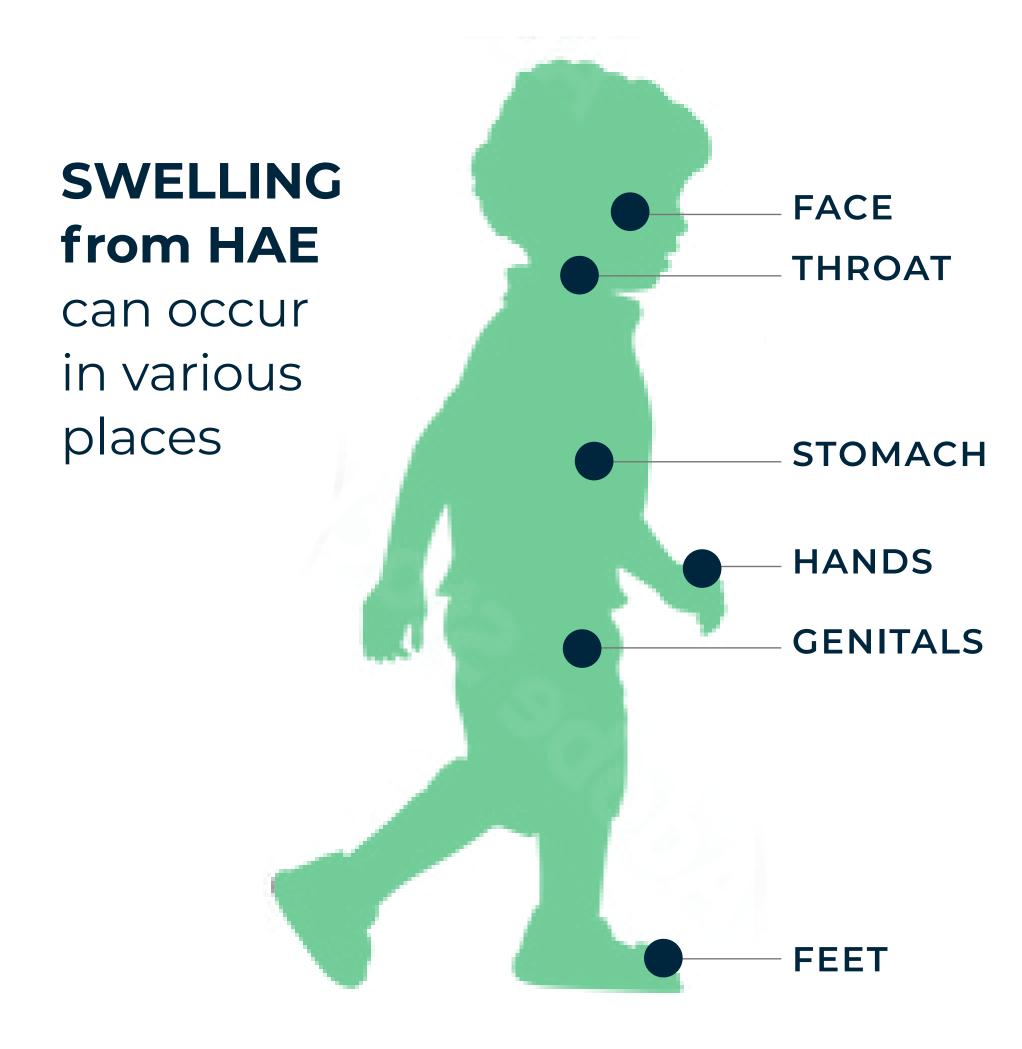


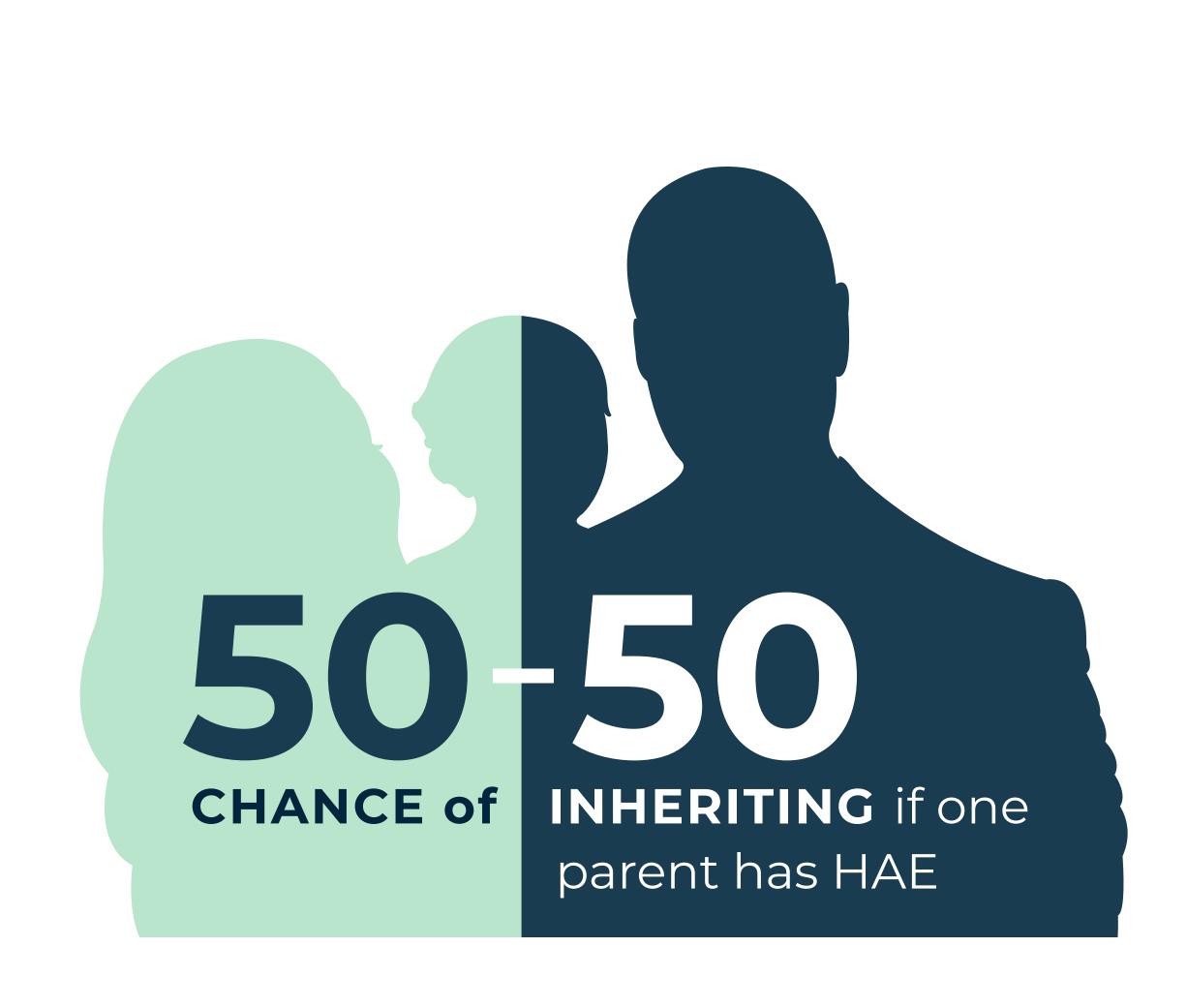


MISSED SCHOOL DAYS per YEAR



Due to a number of factors, children with HAE have been shown to experience higher levels of anxiety, stress, and exhibit lower self-esteem and greater likelihood of depression than their peers.<sup>10,12</sup>

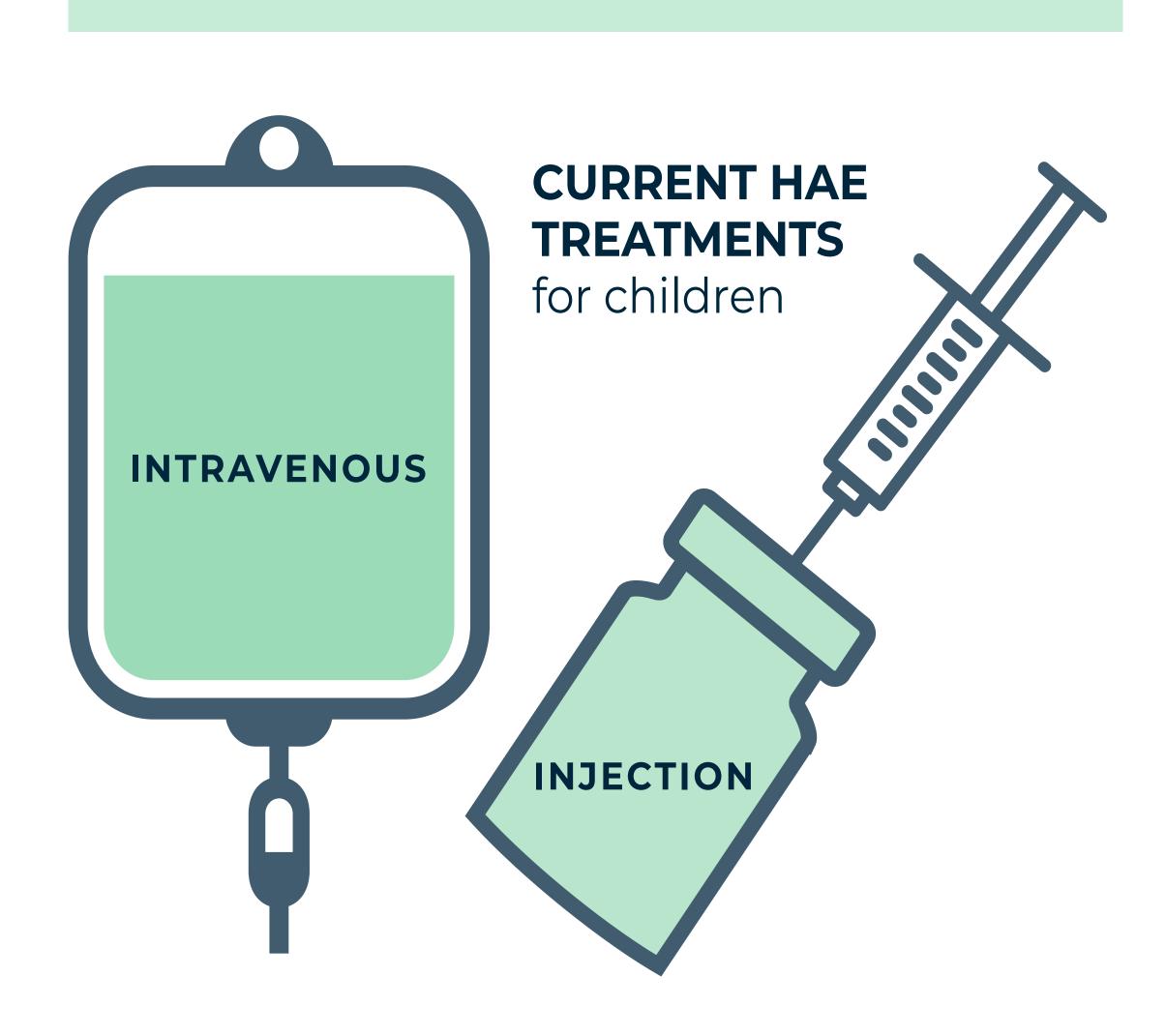




## Intraveous and injectable treatments can be burdensome for children and caregivers

Aside from the physical and mental challenges that can accompany an HAE diagnosis, treatment administration can create an additional burden.<sup>11,13</sup> Therapies currently approved for children with HAE under age 12 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.<sup>14</sup>



Injectable or IV LTP administration may cause caregivers and pediatric patients 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 for these reasons.

### 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.<sup>15</sup>

However, treatments that are currently available for children with HAE under age 12 are delivered intravenously (IV) or through subcutaneous injection (under the skin),<sup>2</sup> which can be inconvenient, burdensome and painful.<sup>14</sup> 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.

#### STRESSORS can cause an HAE ATTACK



## Effective LTP treatment options with child-friendly administration are needed

Given the significant impact of HAE, which 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. These could make a meaningful difference for younger patients and their caregivers. <sup>16</sup> 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.

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