Emergency Department Experiences of Young Patients With Hereditary Angioedema and Their Caregivers

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Patricia Stewart, MD[1]; Tamara Ray, MBA[2]; Diane Ramsey-Paige, BSN, PNP, CCRC[2]; Daniel Petroni, MD, PhD[2]; Clare Murphy, BA[3]; Mike Lionetti, BA[3]; Nicole Garberg, MS[3]

[1]Mississippi Asthma and Allergy Clinic P.A., Jackson, MS; [2]BioCryst Pharmaceuticals, Inc., Durham, NC; [3]KJT Group, Inc., Rochester, NY

INTRODUCTION

- Hereditary angioedema (HAE) is a rare disease that often presents in early childhood and causes spontaneous episodic swelling that can be life-threatening.[1,2]
  - Potentially fatal laryngeal swelling and painful abdominal swelling can occur in young children and typically require an emergency department (ED) visit.[2]
- Lack of convenient oral prophylactic treatment for children under 12 years of age may necessitate ED/hospital visits for injection treatments during HAE attacks.
- This study sought to understand young patients' experiences with HAE attacks, including ED and hospital experiences.

METHODS

- Participants residing in the United States (US) were invited via email to participate in research activities:
  - Caregivers of young children with HAE, along with their children aged 2-11 years
  - Adolescents with HAE (reporting on their experiences before age 12)
  - Healthcare professionals (HCPs) treating young children with HAE
- Caregivers and adolescents were recruited by the US Hereditary Angioedema Association; HCPs were recruited from online research panels.
- Inclusion criteria:

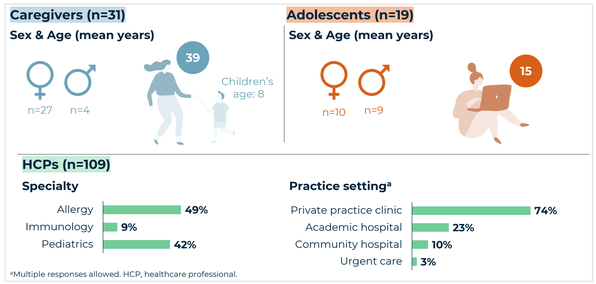
Caregivers & children	Adolescents	HCPs
• Caregivers: ▫ Aged ≥18 years ▫ Has at least 1 child aged 2-11 years diagnosed with HAE ▫ Aware of child's HAE type or child has family history of HAE • Children: ▫ Aged 2-11 years diagnosed with HAE	• Aged 12-17 years • Diagnosed with HAE • Received diagnosis before age 12 • Aware of HAE type or has family history of HAE	• Allergist, immunologist, pediatrician, nurse practitioner, or physician assistant • In practice ≥2 to ≤35 years • Sees ≥2 patients with HAE per year • Has ≥1 patient with HAE aged 2-11 years • Actively treats patients with HAE/ makes treatment decisions

- Caregivers consented to participate and provided consent for their child/adolescent. HCPs also consented to participate.
- Caregivers and adolescents participated in online discussions (bulletin boards) or video in-depth interviews (VIDIs). Children (aged 2-11 years) participated in 1 day of the online discussions with their caregivers. HCPs participated in VIDIs or online surveys.
  - The online discussions took place over 5 days between December 2024 and January 2025.
  - The 60-minute VIDIs were conducted from March to April 2025 (caregivers/adolescents) and from December 2024 to January 2025 (HCPs).
  - The HCP survey was conducted from March to April 2025.
- Adolescents were asked to reflect on having HAE as a child (before age 12), including ED/hospital visits. Caregivers, together with their children, were asked to reflect on ED/hospital visit experiences.
- In VIDIs, HCPs were asked to reflect on ways that HAE impacted their young patients and their caregivers. HCPs rated agreement or disagreement with statements regarding psychosocial impacts of ED/hospital visits on their pediatric patients.
- The study was approved by the WCG Institutional Review Board.

RESULTS

- The study included 31 caregivers, together with their children (n=23), 19 adolescents with HAE (reporting on experiences when aged <12 years), and 109 HCPs (n=24 VIDIs, n=85 surveys) (**Figure 1**).
  - Most caregivers and half of adolescents were female (**Figure 1**). Most HCPs were physicians. Most HCPs were based in private practice and had been in practice an average of 18 years.

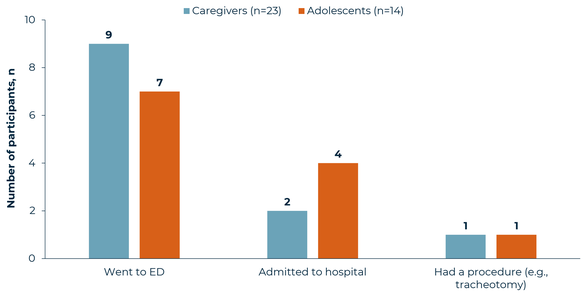
Figure 1. Participant Demographics



[a]Multiple responses allowed. HCP, healthcare professional.

- About half of adolescents and caregivers (with their children) reported going to the ED/hospital at least once before age 12 (**Figure 2**).
  - Caregivers reported an average of 3 hospital visits (range = 1 to 8) within their child's lifetime.
  - Adolescents reported an average of 6 hospital visits before age 12. Two adolescents with particularly severe symptoms estimated having between 50 and 100 hospital visits.
  - Most ED/hospital visits lasted a few hours; a few involved multiple overnight stays.

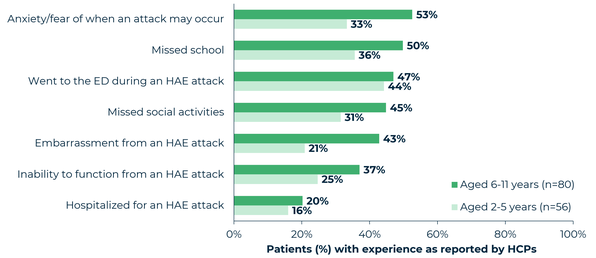
Figure 2. Hospital Experiences[a] Since HAE Diagnosis Before Age 12



[a]Responses are not mutually exclusive. ED, emergency department; HAE, hereditary angioedema.

- Pediatricians (50%) and allergists (39%) reported that their patients experience ED visits, and 16-20% are hospitalized after an attack (**Figure 3**). HCPs noted that their young patients experienced fear and anxiety about future attacks, missed school and social activities, and embarrassment.
- Pediatricians (44%) and allergists (33%) reported that patients aged 6-11 years are more likely than patients aged 2-5 years to have anxiety about future attacks and embarrassment from attacks.
- ED visits and hospitalizations are more common before diagnosis, when the family does not yet know the cause of the symptoms.

Figure 3. HCP Perspectives on Pediatric Patient Medical Experiences



ED, emergency department; HAE, hereditary angioedema; HCP, healthcare professional.

- Caregivers and adolescents reported having negative experiences in the ED (**Figure 4**).

Figure 4. Caregiver and Adolescent ED/Hospital Experiences

Negative ED experiences are associated with long-term fear of hospitals or needles for some adolescents and children (as reported by caregivers).

Negative experiences motivated prophylactic treatment use because the ED visit emphasized the severity of HAE and established a goal to avoid future ED visits.

Most have experienced negative or challenging ED situations:

- **Unfamiliar or unwilling HCPs**
  Caregivers may have had to fight for their child to be treated.
  Some caregivers and adolescents go to the ED prepared with HAE educational resources and treatment plans to mitigate challenges.
- **Delay of treatment and general time-consuming nature of the ED**
  Treatment may be delayed if HCPs are unfamiliar with HAE.
  Some HCPs do not have sense of urgency, prefer to administer other treatments (e.g., antihistamines) or wait to see if condition improves.
  The experience results in lost time at work/school and shifting plans for the entire family.
- **Lack of medication availability**
  May have to travel for ED treatment.
- **Overall fear and anxiety**
  Stress and anxiety are driven by the chaotic ED environment.

Other ED experiences are relatively smooth if ED staff are trained or caregivers prepare ahead to avoid negative experiences:

- **Understanding and friendly HCPs**
  Some HCPs joke around, provide stuffed animals, and make the child feel special. These behaviors can be helpful.
- **Some HCPs familiar with HAE**
  Some HCPS are familiar with HAE or willing to follow the caregiver or allergist/ immunologist treatment plan.
- **Providing treatment quickly, without delay**
  Caregivers may call ahead to help ensure quick attention.
- **Symptom relief**
  Relief knowing that the pain will go away.

"…I have an emergency notebook. It has articles of what HAE is and we bring our [IV medication]. We would be like, "Here, this is what she has," and we would have to educate them a little bit. It was definitely nerve-racking. A lot of times I remember my mom yelling at the emergency workers."
– Adolescent

"They hate that to get relief they have to go through the anxiety, fear, and discomfort of an [ED visit] and needles." – Caregiver

"Our pediatrician called ahead to let them [ED] know we were coming for throat swell and would bring our own medication…the doctor wanted to ask several more questions and acted like she was very familiar with HAE. She didn't think he was having a throat attack because his oxygen levels weren't very low. We [had to] call the HAEA [US Hereditary Angioedema Association] to get them to call the ER and explain the urgency of the situation. After ~1 hour…[the doctor] finally let the nurse administer his medication. After the medication, she insisted he have an x-ray of his throat. The x-ray showed narrowing of the airway." – **Caregiver**

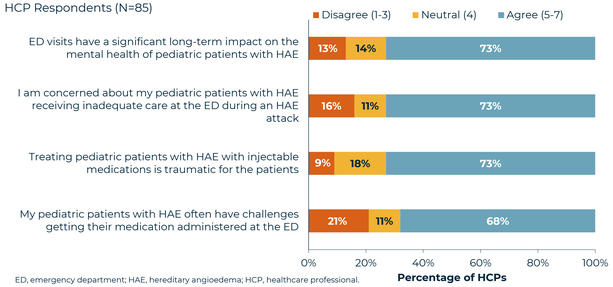
"They [ED] treat us like we were there for no reason. I would have to explain that he's been vomiting for days and needs fluids and pain medication. We would be treated awful because 'why would you ask for narcotics?' Even though he was just a little boy, they still treated him that way, which was really hard for me to watch because I knew exactly what he needed." – **Caregiver**

ED, emergency department; HAE, hereditary angioedema; HCP, healthcare professional.

- Approximately 75% of HCPs reported concern about their patients receiving inadequate care, treatment-related trauma, and the long-term impact of ED/hospital visits on patients' mental health (**Figure 5**).
- HCPs noted that ED visits/hospitalizations (especially if frequent) underscore the seriousness of HAE and prompt caregivers to seek treatment for their child if they weren't previously treated.
- To help prevent negative experiences, most HCPs provide emergency resources to patients and caregivers (acute medication administration instructions, care letters, or their office phone number for emergency contact).

Figure 5. HCP Concerns Related to Pediatric Patient Visits to the ED/Hospital

Rated on a 7-pt Likert scale where 1 is "Strongly disagree" and 7 is "Strongly agree"
HCP Respondents (N=85)



ED, emergency department; HAE, hereditary angioedema; HCP, healthcare professional.

Overall, HCPs agreed that ED visits are not positive experiences for patients and caregivers.

Most HCPs indicated ED visits are stressful, anxiety-inducing, and scary due to the uncertainty of the child's prognosis and the chaos of ongoing tests, blood draws, treatments, etc.

A few HCPs also mentioned ED visit disrupts caregiver work schedules and the child's school attendance.

CONCLUSIONS

1. ED/hospital visits are common for young patients with HAE. Negative ED/hospital experiences can be traumatic with lasting impacts.
2. HCPs are concerned about the adequacy of care, treatment-related trauma, and long-term psychosocial impact of these experiences on young patients.
3. Negative experiences and anxiety related to HAE attacks are important concepts for HCPs and caregivers to discuss during shared decision-making regarding initiation of prophylactic treatment.

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