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Impact of Injectable HAE On-demand Treatments on Children and Caregivers: A Clinician Interview Study

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Key Takeaways

HAE and injectable on-demand treatments substantially impact children aged ≤11 years and their caregivers



- Anxiety
- Stress
- School (children) and work (caregiver) absenteeism

Difficulties administering IV on-demand treatment often necessitate admittance to the ER



- Challenges to establish venous access by caregivers

Children with HAE remain in need of an effective and more patient-friendly on-demand treatment option



Background

- In the United States, the only approved on-demand treatment for acute attacks of hereditary angioedema (HAE) in children aged ≤11 years is administered via intravenous infusion
- Although effective, injectable on-demand treatments can be painful, challenging to administer, and burdensome to both patients and their caregivers[1–3]
- Administration and emotional challenges associated with injectable on-demand therapies may contribute to delayed or forgone treatment
  - Treatment delays and nontreatment can lead to attack progression and prolonged attack duration[4,5]
- A targeted literature review (TLR) was performed, and 3 clinical experts in HAE were interviewed to understand the impact of HAE and the burden of injectable on-demand treatment on children aged ≤11 years and their caregivers

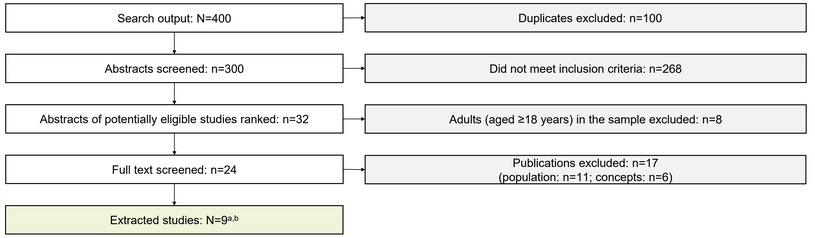
Methods

- For the TLR, Ovid (MEDLINE and Embase®) was searched (January 1, 2016–December 12, 2025) for published data on the burden of HAE type 1 or 2, treatment-related experiences, impacts, and unmet needs in children aged ≤11 years and their caregivers
  - The search was restricted to English-language primary manuscripts, congress proceedings, and reviews of primary noninterventional studies (eg, qualitative, quantitative, mixed)
- Clinical input was collected via semistructured interviews with 3 board-certified allergists or immunologists who treat HAE

Results

Targeted literature review

Figure 1. Ovid (MEDLINE and Embase) search results



[a]Patients aged ≤11 years were substantially represented in the sample. [b]Two additional publications were identified outside the Ovid search (n=1) and shared by KalVista Pharmaceuticals (n=1).

- Of the 400 publications identified, 9 satisfied all eligibility criteria **(Figure 1)**

Table 1. Overview of relevant studies identified in the Ovid searches

Author (year)	Study design	Country/region	Concepts covered	N	Patient age, years
Read (2014)[6]	Cross-sectional[a]	UK	• Treatment experience • Patient and caregiver impacts	10 patients or caregivers	Range: 3–16
Nygren (2015)[7]	Cross-sectional[b]	Sweden	• Treatment experience • Patient and caregiver impacts	31 patients or caregivers	Median (range): 9 (1–17)
Aabom (2017)[8]	Cross-sectional[a]	Denmark	• Treatment experience • Patient impacts	14 patients	Median (range): 12 (4–18)
Engel-Yeger (2017)[9]	Cross-sectional[a]	Hungary and Israel	Patient impacts	34 patients	Mean (range): 12.3 (3–18)
Kessel (2017)[10]	Cross-sectional[a]	Hungary and Israel	Patient impacts	33 patients	Mean (range): • Hungary: 12.8 (4.5–17.8) • Israel: 12.4 (5.5–18)
Farkas (2020)[11]	Prospective, longitudinal	Hungary	Treatment experience	70 patients	Range: 0–18, with 59% aged ≤12[c]
Ocak (2021)[12]	Cross-sectional[a]	Turkey	Patient impacts	59 patients	Median (IQR): 8.9 (5.4–12.6)
Martinez-Saguer (2023)[13]	Cross-sectional[d]	Argentina, Brazil, Colombia, Croatia, Denmark, Germany, Hungary, Ireland, Poland, and Portugal	Patient and caregiver impacts	55 caregivers	Mean (SD): 10.6 (3.8)
Tachdjian (2023)[14]	Cross-sectional[d]	US	Patient impacts	35 patients[e]	Range: 2–12

[a]Cross-sectional survey with patients and/or caregivers. [b]Cross-sectional survey and interview study. [c]Based on pooled n values in Table 2. [d]Cross-sectional survey with caregivers only. [e]Sample size of caregivers not reported.
IQR, interquartile range; SD, standard deviation; UK, United Kingdom; US, United States.

- There were limited data describing the burden of HAE and of on-demand treatment in pediatric patients
  - No in-depth qualitative interviews were identified
- Of the 9 publications, 8 reported on the burden of HAE[6–10,12–14] **(Table 1),** and 4 discussed on-demand treatment[6–8,11]

Clinician interviews

- The 3 clinicians interviewed had 5–30 years of experience and treated 100–150 patients with HAE annually, approximately 5%–25% of whom were aged ≤11 years
- Per clinicians, attack severity and duration vary by timing of on-demand treatment administration

Table 2. Clinician-reported impacts of HAE on children aged ≤11 years

School/education • Absenteeism • Reduced academic performance
Daily activities • Reduced physical activity
Emotional function • Anxiety • Fear • Depression
Family life • Increased dependency on parents • Decreased interaction with siblings
Social function • Victim of bullying • Increased social avoidance

HAE, hereditary angioedema.

> *"There are important consequences [of mild attacks]. Especially if that attack is not treated, the child is going to end up missing school, missing activities. That compounds over time, impacting their education and social outcomes."*
>
> *Clinician quote*

- All clinicians commented on the substantial impact that HAE had on children's school/education, daily activities, emotional function, family life, and social function **(Table 2)**

Table 3. Clinician-reported impacts of HAE on caregivers of children aged ≤11 years

Work/career • Absenteeism • Loss of employment
Daily activities • Forgoing recreational activities
Emotional function • Anxiety over the child's attacks • Stress about caregiving responsibilities • Guilt over having passed HAE to their child

HAE, hereditary angioedema.

> *"I had a parent who was saying that whenever the child had a HAE attack at school, the school would call him, and he would have to go pick up the child. He would then have to stop his work, go pick up the child, look after the child, and administer the icatibant. But then, his employer was upset, and eventually he was laid off. When he was laid off, they lost their insurance to cover the icatibant. So, these things can then spiral, and the family can end up in fairly dire situations rather quickly."*
>
> *Clinician quote*

- Per clinicians, the main impacts to caregivers of children with HAE aged ≤11 years were on work/career, daily activities, and emotional function **(Table 3)**

Table 4. Clinician-reported patient/caregiver issues with injectable on-demand treatment

Preparation • Need for training • Reconstitution requirements (specific to IV C1-INH) • Drawing the correct dose (specific to off-label use of SC icatibant)
Administration • Difficulties with IV administration due to the small diameter of children's veins • Need to take children to the ER after multiple failed attempts at establishing venous access • Obligation to sometimes forcefully administer treatment because of the child's needle phobia • Lack of privacy when administering treatment in public
Side effects • Needle pain • ISRs (eg, swelling, redness, irritation, soreness), specific to off-label use of SC icatibant

C1-INH, C1 esterase inhibitor; ER, emergency room; ISR, injection-site reaction; IV, intravenous; SC, subcutaneous.

- **Caregivers typically administer on-demand treatment to children**
- **Sometimes ER staff need to intervene when attempts at establishing venous access at home are unsuccessful**

Clinician observations

- According to clinicians, patients with HAE and their caregivers have expressed dissatisfaction with injectable on-demand treatment because of cumbersome preparation processes, administration and emotional challenges, and injection-site reactions **(Table 4)**
- The 3 clinicians interviewed agreed on the need for an oral on-demand treatment option for children with HAE aged ≤11 years

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Disclosures

TJC has served as a speaker/researcher for Astria, CSL Behring, KalVista Pharmaceuticals, and Takeda; researcher for ADARx, Argo, BioMarin, Intellia, Ionis, and Pharvaris; speaker for Grifols; and consultant for ADARx, Astria, BioCryst, CSL Behring, Intellia, KalVista Pharmaceuticals, Pharvaris, and Takeda. He is the Director of the Angioedema Centers of Reference and the Excellence International Hereditary Angioedema Center and a member of the Medical Advisory Board for the US Hereditary Angioedema Association. **RT** has received grants, consulting fees, honoraria, and/or speaking honoraria from ARS, AstraZeneca, Astria, BioCryst, CSL Behring, Grifols, GSK, Intellia, Ionis, KalVista Pharmaceuticals, Lilly, Novartis, Pharming, Pharvaris, Sanofi/Regeneron, and Takeda. **DFS** has received grants, consulting fees, and/or honoraria from BioCryst, BioMarin, CSL Behring, KalVista Pharmaceuticals, Pharming, Pharvaris, and Takeda. **SCC** has participated in advisory boards for BioCryst, CSL Behring, and KalVista Pharmaceuticals and is a member of the Medical Advisory Board for the US Hereditary Angioedema Association. **SHL, EW,** and **KT** are employees of Acaster Lloyd; Acaster Lloyd were commissioned by KalVista Pharmaceuticals to conduct the study. **AW** is a salaried employee of KalVista Pharmaceuticals. **AA** has received research funding and/or consultancy fees from AstraZeneca, Astria, BioCryst, CSL Behring, GSK, Intellia, Ionis, KalVista Pharmaceuticals, Octapharma, Pharvaris, and Takeda and is a speaker for AstraZeneca, BioCryst, Covis Pharma, CSL Behring, and GSK.

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