Quality of Life and Burden of Disease in Patients With Hereditary Angioedema and Their Caregivers

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OBJECTIVES

- To characterize the clinical and psychological burden of hereditary angioedema (HAE) by
  - Identifying key clinical and psychological evidence to inform messages around unmet needs in HAE for patients, HCPs, and caregivers
  - Generating content to support scientific communications on HAE disease burden, treatment burden, and healthcare resource utilization
  - Providing sources of input and references for an economic model to influence the financial viability and impact of prophylactic therapy on healthcare budgets

SUMMARY

1. **Hereditary angioedema (HAE) is a rare and unpredictable disease where the quality of life (QoL) of patients is lower than that of the general population. Currently available prophylactic treatments have provided improvements in patients' QoL and mental health; however, patient dissatisfaction persists.**
2. **Therefore, an analysis of the existing literature on the current burden and unmet needs for HAE patients and their caregivers was undertaken.**
3. **Current management focuses on addressing acute attacks and implementing short- and long-term prophylaxis to prevent future attacks.**

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INTRODUCTION

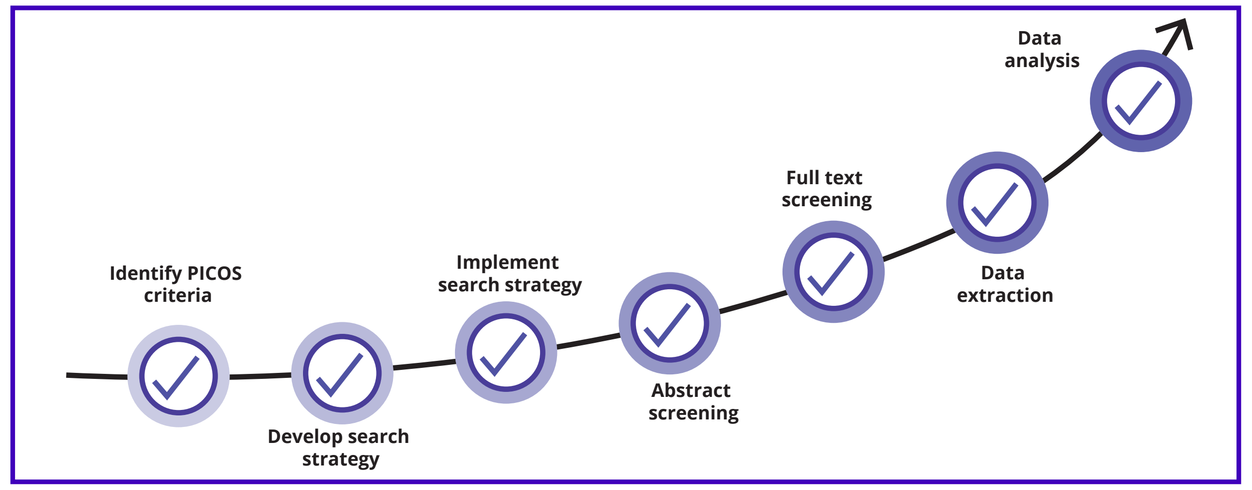
- Hereditary angioedema (HAE) is a rare genetic condition characterized by recurrent and unpredictable episodes of angioedema, commonly affecting the extremities and upper respiratory and gastrointestinal tracts[1]
- With no cure available, management focuses on addressing acute attacks and implementing short- and long-term prophylaxis to prevent future attacks[2]
- To support the development of a comprehensive evidence package for communication with payers and decision-makers, an analysis of the existing literature on the current burden and unmet needs for HAE patients and their caregivers was undertaken

METHODS

TARGETED LITERATURE REVIEW

- A targeted literature review (TLR) was conducted on January 10, 2024, through a review of Medline and Embase via Ovid and EconLit (**Figure 1**)
- Summary tables were developed along with a narrative description of the data and methods used in the eligible studies
- Methodological limitations for this TLR include:
  - Evidence was identified and analyzed in a non-systematic way
  - The TLR was limited to studies involving adults with HAE types 1 or 2 in North America and Europe

Figure 1. Literature review study design



INCLUSION CRITERIA FOR STUDIES

- Studies were prioritized at the data extraction stage according to the following criteria:
  - Studies conducted in North America or Europe
  - Adults with HAE types 1 or 2
  - Studies presenting data on
    - Quality of life (QoL) & impact on daily life, including pain
    - Psychological distress
    - Frequency & severity of attacks
    - Burden & preferences/satisfaction associated with long-term prophylactic treatments
    - Caregiver burden and QoL

RESULTS

OVERALL DESCRIPTION OF INCLUDED STUDIES

- Out of 1,975 abstracts identified, a total of 48 studies were included in this analysis (**Figure 2**)
- Most studies were cross-sectional, primarily conducted in North America, notably in the United States (n=21; 44%; **Figure 3**)

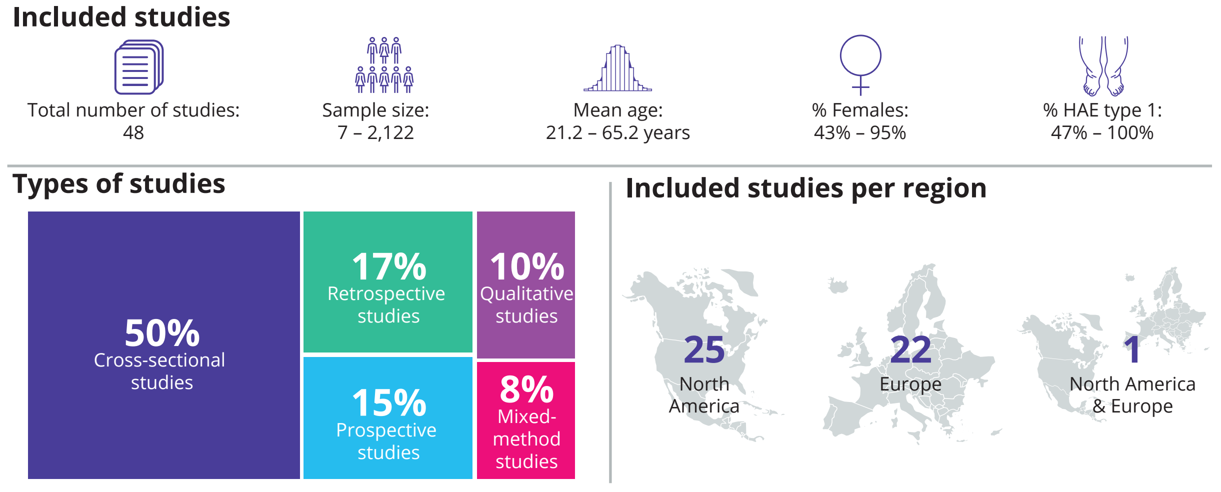
Figure 2. Identification of included studies

1975
Abstracts identified from search strategy & conference searching

270
Included studies from abstract screening

48
Included studies from full text review & prioritization of outcomes of interest

Figure 3. Overall description of included studies



HAE, hereditary angioedema.

EVIDENCE BASE

Quality of Life

- 23 studies reported outcomes on QoL and pain (**Figure 4A**)
- Factors associated with QoL included
  - Psychological distress: Severity of anxiety and depression were associated with worse QoL[3]
  - Severity of attack: Pain visual analog scale scores increased with severity of attack[4]
- After conducting an ad hoc exploratory search, a total of 282 trials were retrieved from clinicaltrials.gov, the International Clinical Trials Registry Platform, and the European Clinical Trials Register. After removal of duplicated or observational studies, 120 HAE trials were identified, 20 of which reported measuring QoL (**Figure 4B**)

Psychological Distress

- 9 studies reported outcomes on psychological distress: 1 study provided data on stress, 8 studies provided data on anxiety, and 8 studies provided data on depression
- Trends noted included
  - Attack severity: Higher anxiety and depression among those experiencing more severe attacks[4]
  - HAE treatment: Long-term prophylaxis reduced anxiety and depression[5]
  - Age: As patients aged, their acceptance of the disease increased[6]

Experience With Long-Term Prophylaxis

- 16 studies reported experiences with long-term prophylaxis: 9 reported on treatment satisfaction, 11 reported on treatment dissatisfaction, and 6 assessed treatment preferences
- Consistent long-term prophylaxis has been shown to alleviate anxiety and depression and improve QoL, compared to only on-demand treatment[7]

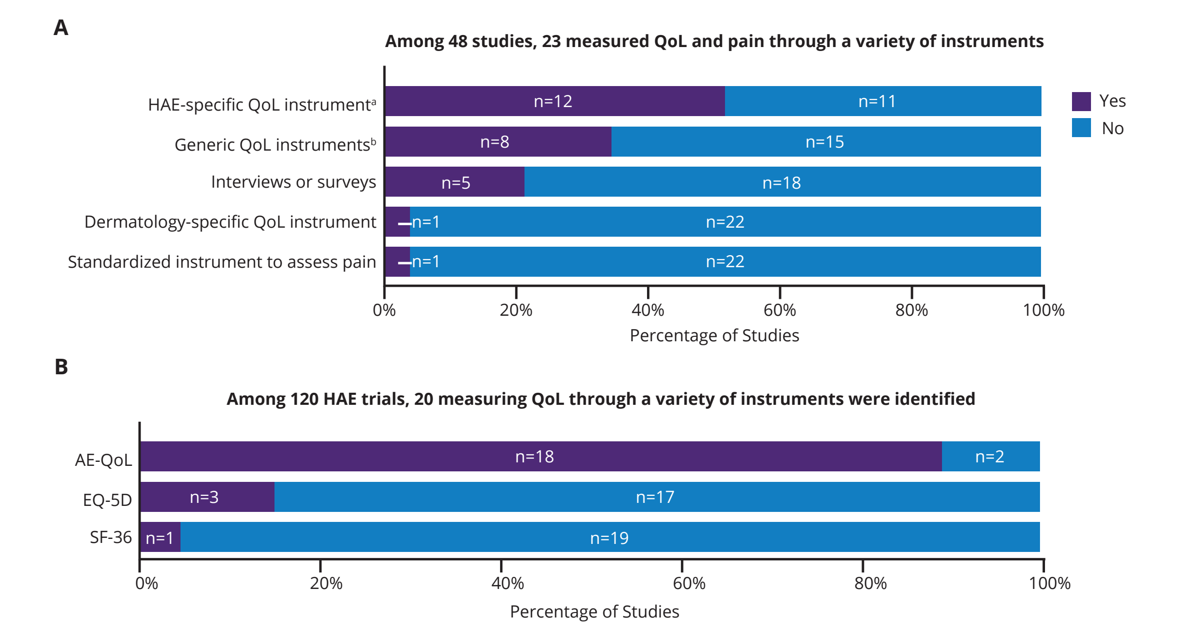
Attack Outcomes

- 36 studies reported outcomes related to HAE attacks: 33 provided data on HAE attack frequency, 22 provided data on HAE attack location, 15 provided data on attack severity, and 9 provided data on attack duration
- The 5 most reported triggers included stress, emotional distress, physical trauma, menstruation, and surgery[8-12]

Caregiver Burden

- 1 qualitative study described the burden experienced by caregivers of patients with HAE
- Major sources of caregiver burden included gravity of HAE symptoms, unpredictability of HAE attacks, and diagnosis delays

Figure 4. HAE publications and clinical trials assessing QoL



AE, angioedema; EQ-5D-5L: EuroQol 5-Dimension 5-Level; HAE, hereditary angioedema; QoL, quality of life; SF-36: short form health survey.
[a]Included: AE-QoL and HAE-QoL. [b]Included: RAND-36 and EQ-5D-5L.

CONCLUSIONS

- HAE types 1 and 2 are diseases with high patient and caregiver burden, especially driven by the severity, frequency, location, and unpredictability of attacks
- Patients experience poor QoL and high prevalence of anxiety & depression as a result of their symptoms, with psychological distress also being a trigger for HAE attacks
- Prophylactic treatments have provided improvements in patients' mental health and QoL and allowed them to regain independence; however, dissatisfaction persists with current options