Assessing the Utility of Existing Hereditary Angioedema Disease Models for Health Economic Evaluation of Future Therapies

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Background

• Hereditary angioedema (HAE) is a rare, genetic disease characterized by debilitating swelling episodes in various parts of the body
• The chronic and unpredictable nature of HAE results in substantial burden for patients, caregivers, and health systems
• Over the last two decades, acute and long-term prophylactic treatments have become available for patients; however, these formats and requirements can be burdensome
• As new HAE treatments, such as oral therapies, emerge, it is important to consider whether existing HAE modeling frameworks are fit for assessing their potential value impact
• Payers and health technology assessment (HTA) bodies use economic models to help inform their decisions on population interventions and to make the best use of limited healthcare resources
• This study identified existing HAE disease models and assessed their utility for evaluating the health economic value of future HAE therapies

Methods

• We conducted a prespecified literature search in PubMed and HTA databases to identify pertinent peer-review literature, congress proceedings, and agency assessments on HAE disease models
• We included studies involving patients with any type of HAE (type 1, 2, and HAE with normal C1-INH) reporting a disease model relating to HAE pharmaceutical treatment
• For each study, we extracted detailed information on:
  1. Disease modeling approach
  2. Impact of simulated interventions on disease progression
  3. Health economic analysis specifications and outcomes
• We assessed the quality of reporting using the Consolidated Health Economic Evaluation Reporting Standards (CHEERS) II criteria—a 28-item checklist assessing the robustness of health economic evaluation reporting, where a higher score indicates more comprehensive reporting

Results

• We identified 10 HAE disease models. 5 were reported in peer-reviewed publications, and 5 were reported in HTA evaluations (Table 1)1-7

Table 1. Overview of Select Model Characteristics and Specifications

<table>
<thead>
<tr>
<th>Economic Analyses Used</th>
<th>N Earnings</th>
<th>N Studies</th>
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<tr>
<td>Economic Analyses Used</td>
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*Some models simulated multiple attack characteristics
**Some models simulated both direct and indirect costs

• All models simulated direct costs, with drug costs, drug administration costs, and hospitalization costs most frequently included (Figure 1); 3 models simulated indirect costs—all in scenario or exploratory analyses

Figure 1. Categories of Direct Costs Included in Models

- Attack characteristic simulation varied across models: attack location and severity were each simulated in half of reviewed models, while duration was simulated in nearly all (n=9), with varying operational definitions used
• Two reviewed models simulated disease-related mortality
• Economic outcomes were measured using different metrics across reviewed models: Health Utility (Quality-Adjusted Life Years, QALY) (n=6); Health Utility (QALY) and Life Years (n=2); HAE Attacks Avoided (n=1); Time to Onset of Symptom Relief (n=1); and Time to Complete Resolution of Attack Symptoms (n=1)
• Quality of reporting was higher in HAE reports compared to peer-reviewed publications (average CHEERS score of 21.4 versus 10.9, respectively) (Figure 2)

Figure 2. Quality of Reporting as Assessed by CHEERS II

Conclusions

• We found that existing HAE disease models do not fully capture relevant variables required to evaluate emerging HAE therapy options, including all attack characteristics and both direct costs and indirect economic impact
• The development of a new HAE disease model that incorporates a societal perspective is important to capture the true burden of disease and support health economic evaluations for future HAE therapies

References


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