

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

Bjoern Schwander, PhD¹; Betsy J. Lahue, MPH²; Kristen A. Cribbs, PhD MPH²; Shawn Czado³

¹ Agency for Health Economic Assessment & Dissemination (AHEAD), Bietigheim-Bissingen, Germany; ² Alkemi LLC, Manchester Center, VT, USA; ³ KalVista Pharmaceuticals, Inc., Cambridge, MA, USA

Background

- Hereditary angioedema (HAE) is a rare, genetic disease characterized by debilitating swelling episodes in various parts of the body¹
- 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 resources²
- 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:
 - Disease modeling approach
 - Impact of simulated interventions on disease progression
 - 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 complete reporting³

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Results

- We identified 10 HAE disease models; 5 were reported in peer-reviewed publications, and 5 were reported in HTA evaluations (Table 1)⁴⁻¹³

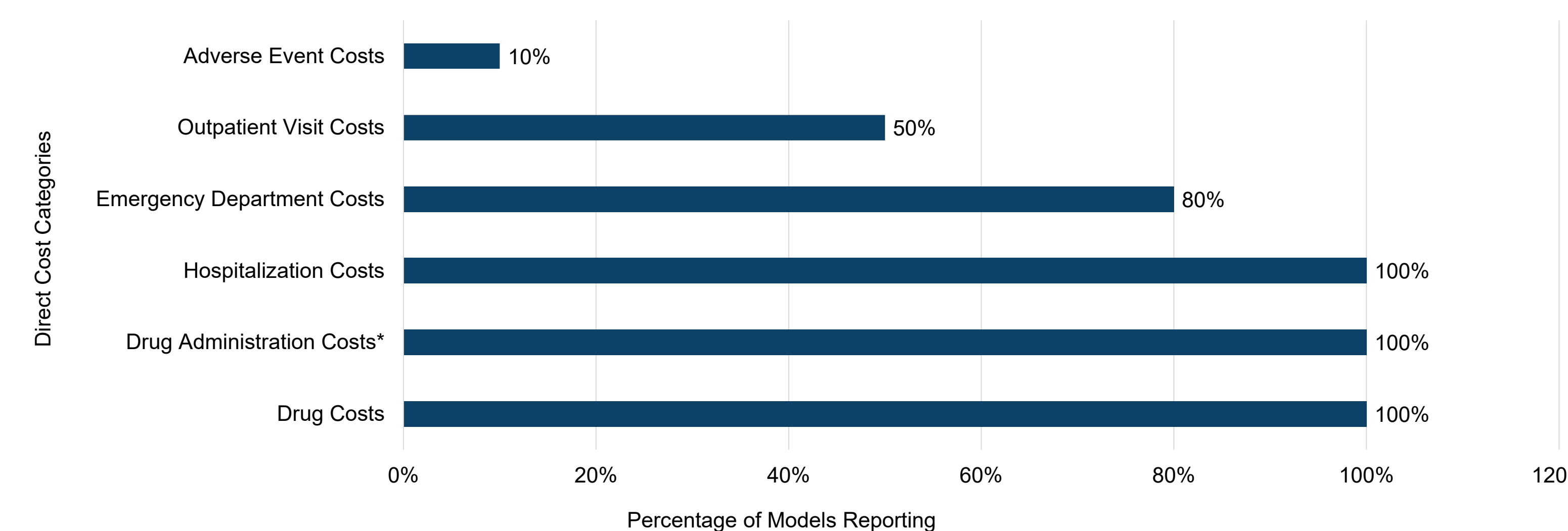
Table 1. Overview of Select Model Characteristics and Specifications

	N Studies		N Studies
Economic Analyses Used		Time Horizons Used	
Cost utility	6	Lifetime	4
Cost-effectiveness	3	1-year	3
Cost-minimization	1	1 HAE attack	3
HAE Therapies Studied		HAE Attack Characteristics Simulated*	
On-demand only	5	Location	5
Long-term prophylaxis (LTP) only	3	Severity	5
LTP with on-demand	2	Duration	9
Primary Modeling Approach Used		Key Intervention Effects Simulated	
State transition	5	Reduction of HAE attacks	5
Decision tree	4	Time to HAE attack resolution	5
Cost-effectiveness	1	Cost Types Simulated**	
		Direct	10
		Indirect	3

*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

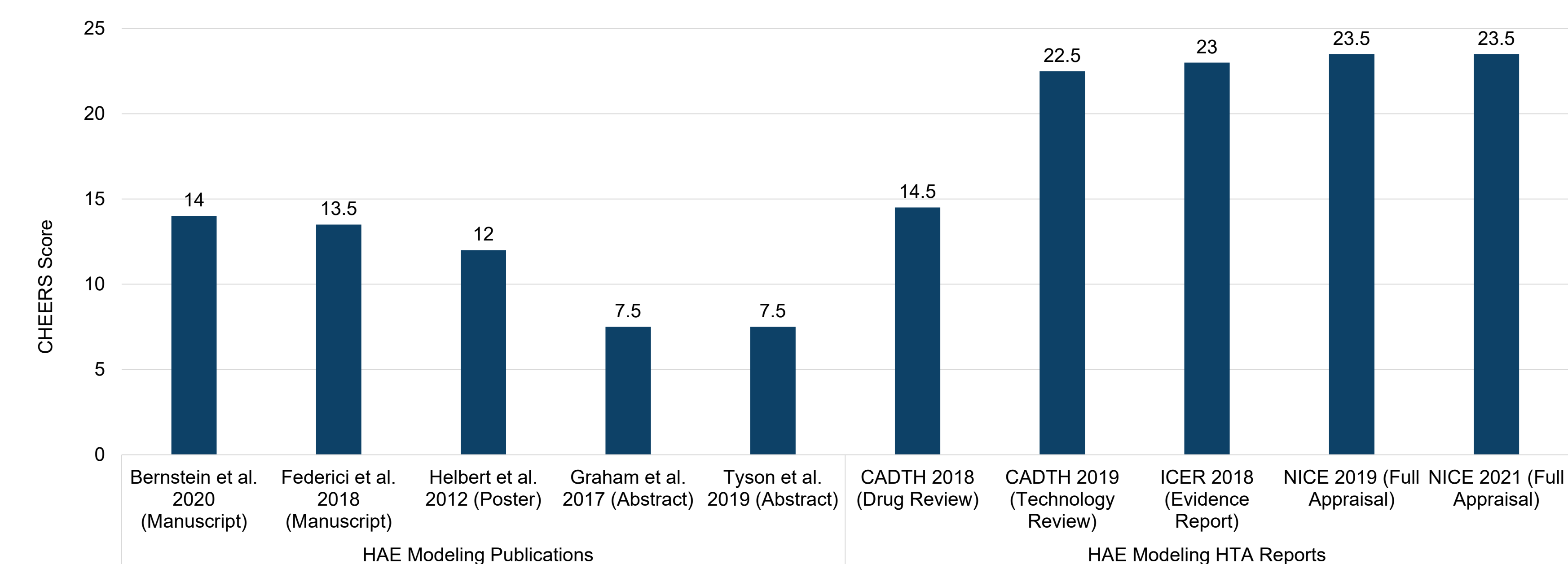
Figure 1. Categories of Direct Costs Included in Models



*Monitoring & training costs, nurse costs, etc. included as part of drug administration costs

- 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=5); 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 HTA 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

Acknowledgements

The authors thank Alkemi LLC contributor Da-In K. Fang, who assisted with writing and editing.

Disclosures

This study was sponsored by KalVista Pharmaceuticals, Inc. SC is an employee of KalVista Pharmaceuticals, Inc. No authors received compensation for their involvement in this research.



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