

Assessing the Potential Benefits of Oral Therapies in Hereditary Angioedema

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Background

- Hereditary angioedema (HAE) is a rare, chronic disease characterized by debilitating swelling episodes in various parts of the body¹
- Current on-demand treatments for HAE are limited to parenteral therapies, which have been associated with significant burden and adherence challenges¹
- Drug route of administration can impact patient preference, quality of life, disease outcomes, and costs of care²
- Novel, oral therapies are emerging for the treatment of acute HAE attacks; however, little is known about patient preference for these therapies and other benefits¹
- As on-demand HAE treatment landscape evolves, there is a need to better understand the potential humanistic and cost benefits of oral therapies, as well as drivers of patient preference, to ensure optimal disease management

Methods

- A targeted literature review (TLR) was conducted to assess the economic and patient benefits of oral therapies in HAE and in chronic disease analogues that have parenteral and oral treatment options
- Searches were conducted in PubMed and Google Scholar
- English HAE-specific peer-review articles and conference proceedings from January 1, 2017-October 15, 2022 were prioritized for inclusion
- Articles in other chronic disease areas and older articles were considered, if relevant
- No study design restrictions were applied

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Results

- We identified 8 publications. Three (37.5%) focused on HAE, 7 (87.5%) were observational, and 7 (87.5%) were conducted in the United States (**Table 1**)
- Findings in the HAE publications revealed that drug route of administration was rated as the most significant HAE treatment development desired by patients, with preference expressed for noninvasive administration modalities, especially oral and non-intravenous medications (62%, 8/13)³
- HAE patients receiving prophylaxis had a high preference for oral therapy (98%, n=47/48), and 100% (n=27/27) of patients not receiving prophylaxis stated they would try an oral option⁴ (**Figure 1**)
- The most common reason why patients receiving prophylaxis reported interest in an oral HAE therapy was ease of administration (87%, n=65/75), followed by improved "convenience" and avoiding "needle sticks" (both 67%, n=32/48) and treating the disease more discreetly (61%, n=46/75)^{1,4} (**Figure 2**)

Table 1. TLR Study and Sample Characteristics

Characteristic	n (%)
Total Number of Publications	8 (100)
Sample Size, Number of patients^a	
Minimum	38
Maximum	200,168
Patient Disease State	
Hereditary Angioedema	3 (37.5)
Multiple Sclerosis	1 (12.5)
Type 2 Diabetes	1 (12.5)
Multiple Disease States	3 (37.5)
Study Location	
United States	7 (87.5)
Multiple	1 (12.5)
Study Design	
Observational	6 (87.5)
Economic Analysis	1 (12.5)

^aAmongst 7 studies reporting sample size

Figure 1. Patient Preference for Oral HAE Therapy⁴

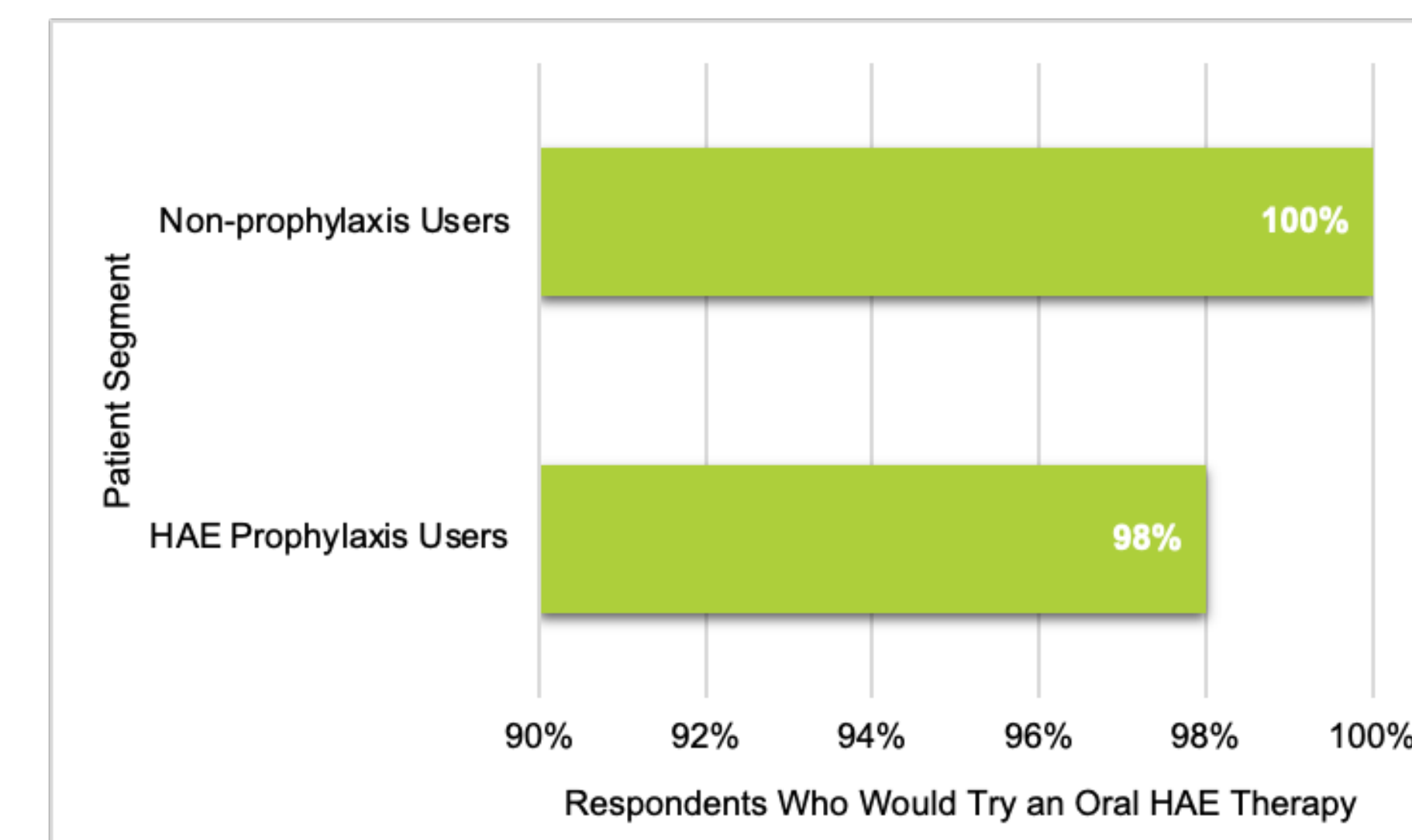


Figure 2. Primary Patient-Reported Reasons of Interest in Oral HAE Therapy^{1,4}

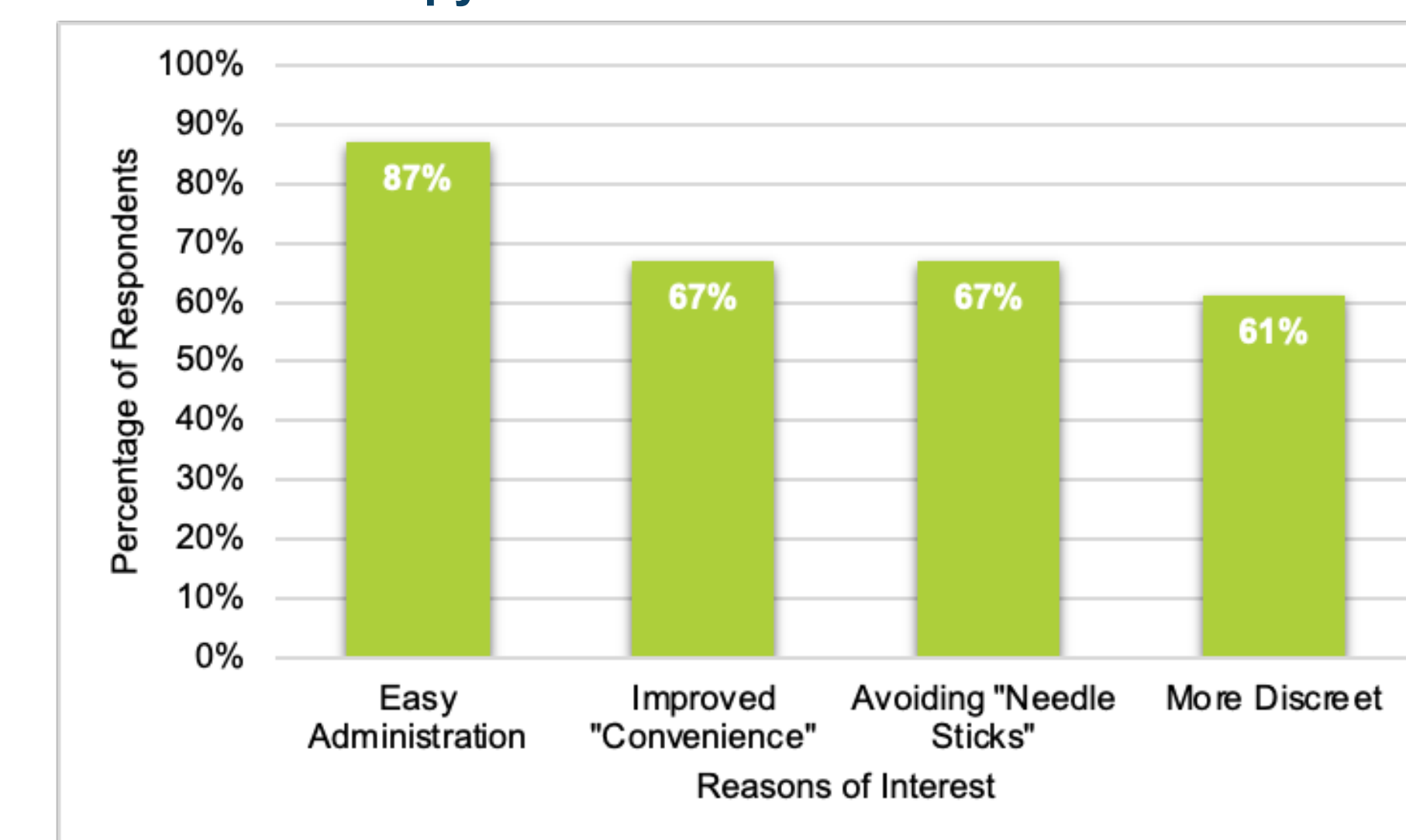
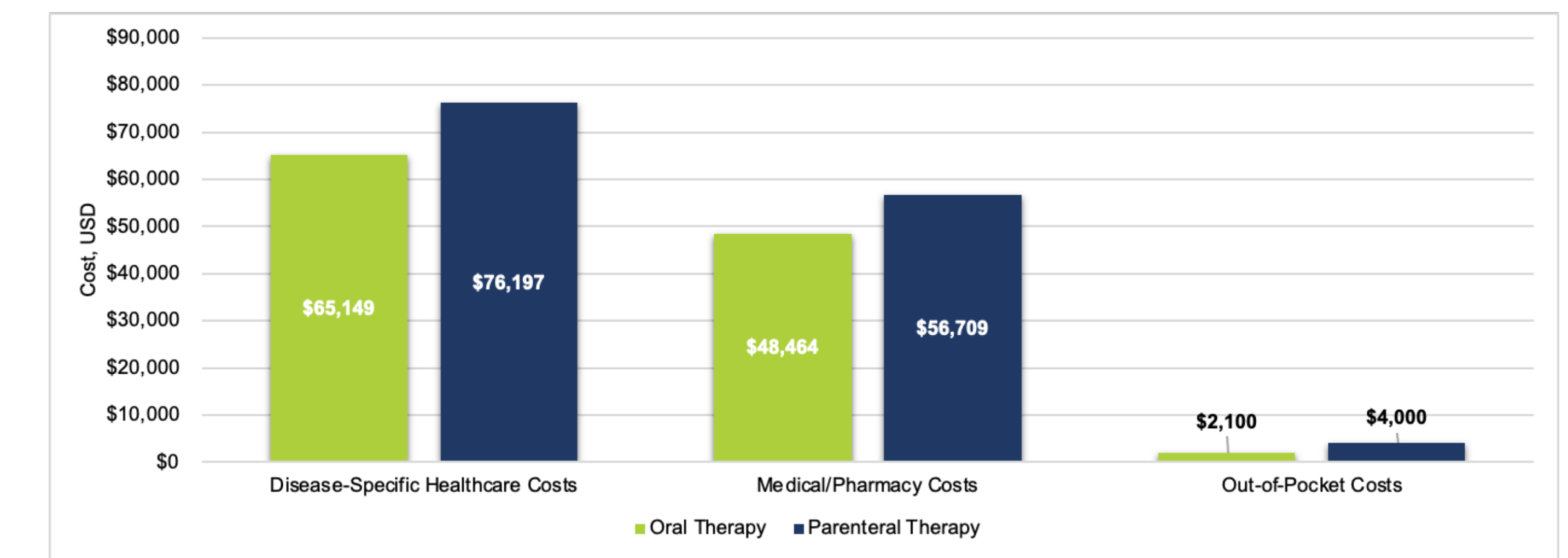


Figure 3. Annual Healthcare Costs, Oral Versus Parenteral Therapies⁶⁻⁸



- In analogous chronic disease states, oral therapies were associated with higher patient adherence rates (67% vs. 59%) than parenteral therapies⁵
- Annual healthcare costs associated with oral therapies were also lower in several domains compared to parenteral therapies: disease-specific healthcare costs (\$65,149 vs. \$76,197 12 months post-index),⁶ total medical and pharmacy costs (\$48,464 vs. \$56,709),⁷ and patient out-of-pocket costs (\$2,100 vs. \$4,000)⁸ (**Figure 3**)
- A cost-minimization analysis found that a switch from intravenous to oral formulation would generate a yearly cost-savings of \$16,000 per patient, largely due to fewer intravenous administrations²

Conclusions

- **This TLR found that patients with HAE and those with analogous disease states prefer oral therapies due to ease of administration, improved convenience, needle stick avoidance, and discreetness**
- **Additionally, results revealed that oral therapies yield lower healthcare and out-of-pocket costs than parenteral therapies, with implications for patients and payers**
- **As such, there is an opportunity for an oral, on-demand HAE therapy to support patient disease management and mitigate healthcare expenditures**

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