

# Assessing the Burden of Injection with Parenteral Hereditary Angioedema 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<sup>1</sup>
- Currently, most prophylactic HAE treatments and all on-demand HAE treatments are parenteral, requiring intravenous (IV) or subcutaneous (SC) injection, and have been associated with significant burden and adherence challenges<sup>1</sup>
- Drug route of administration can impact patient preference, quality of life, disease outcomes, and costs of care<sup>2</sup>
- As the HAE therapeutic landscape evolves and new treatments emerge, gaining a better understanding of the patient-reported clinical, humanistic, and economic burden of parenteral HAE therapies is critical to facilitating optimal, patient-centered disease management

## Methods

- We conducted a targeted literature review (TLR) to assess the clinical, humanistic, and economic burden of on-demand and prophylactic parenteral HAE therapies on individuals living with HAE
- We conducted searches in PubMed and Google Scholar
- We prioritized peer-reviewed articles and conference proceedings published in English from January 1, 2017-November 1, 2022 for inclusion
- We considered older articles, if relevant
- No study design restrictions were applied

## Results

- We identified 9 publications.<sup>1, 3-10</sup> Most studies were observational (78%), peer-reviewed (78%), and conducted in the United States (67%) (Table 1)

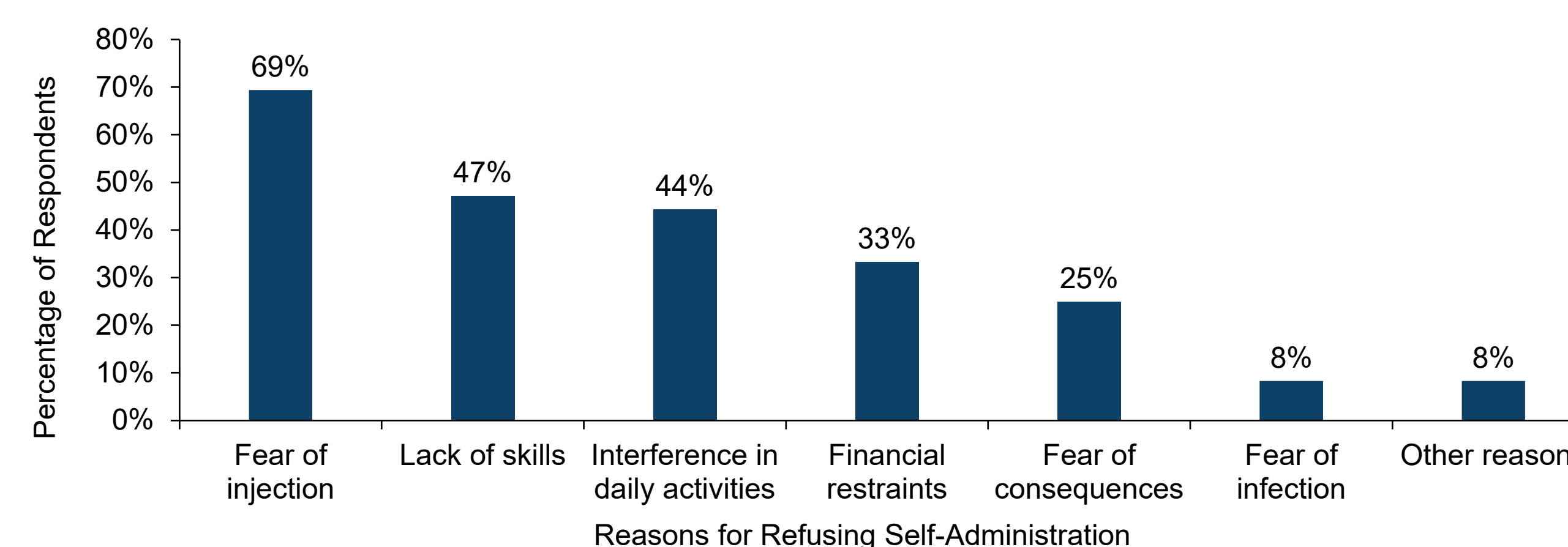
Table 1. TLR Study and Sample Characteristics

| Characteristic                          | n (%)    |
|-----------------------------------------|----------|
| <b>Total Number of Publications</b>     | 9 (100%) |
| <b>Sample Size, Number of Patients*</b> |          |
| Minimum                                 | 30       |
| Maximum                                 | 130      |
| <b>Publication Type</b>                 |          |
| Peer-Reviewed                           | 7 (78%)  |
| Conference Proceeding                   | 2 (22%)  |
| <b>Study Location</b>                   |          |
| United States                           | 6 (67%)  |
| Multiple                                | 3 (33%)  |
| <b>Study Design</b>                     |          |
| Observational                           | 7 (78%)  |
| Randomized controlled trial             | 2 (22%)  |

\*All studies reported outcomes among adult patients only

- Patients reported multifaceted challenges associated with a parenteral HAE therapeutic regimen, which impacted treatment adherence
- Patients taking on-demand HAE therapy reported refusing self-administration most commonly due to fear of injection (69%) and lack of skills (47%)<sup>3</sup> (Figure 1)
- Nearly one-fifth of patients (19%) reported skipping their prophylaxis HAE medication altogether because injections or infusions were inconvenient<sup>1</sup>
- The majority of patients (62%) who used a peripheral vein to administer HAE treatment reported difficulty finding a usable vein, and more than half of intravenous prophylaxis users (51%) were dissatisfied with the length of time required to prepare and administer their medication<sup>8</sup>

Figure 1. Commonly Cited Reasons for Refusing On-Demand Self-Administration<sup>3</sup>



- Nearly half of patients reported feeling nervous when starting new prophylactic treatment (47%)<sup>1</sup> (Figure 2)
- Adverse events related to parenteral HAE therapies were also a concern, with one study reporting 98% of patients using on-demand icatibant had injection site reactions.<sup>4</sup> Common reactions included: pain, bruising, hematoma, burning, numbness, edema, pruritus, and urticaria<sup>4,5</sup>
- Results also revealed the types of attack-related parenteral HAE therapy costs that burden patients and payers, including healthcare visits and associated travel to obtain treatment as well as the cost of injection supplies, such as syringes and alcohol swabs<sup>6</sup>
- Additionally, patients required significant training to be able to administer on-demand therapies, with half of patients in one study requiring 3-5 training sessions to be able to self-administer<sup>3</sup> (Table 2)

Figure 2. Prophylactic Parenteral Treatment Impact on Patient Mental Health<sup>1</sup>

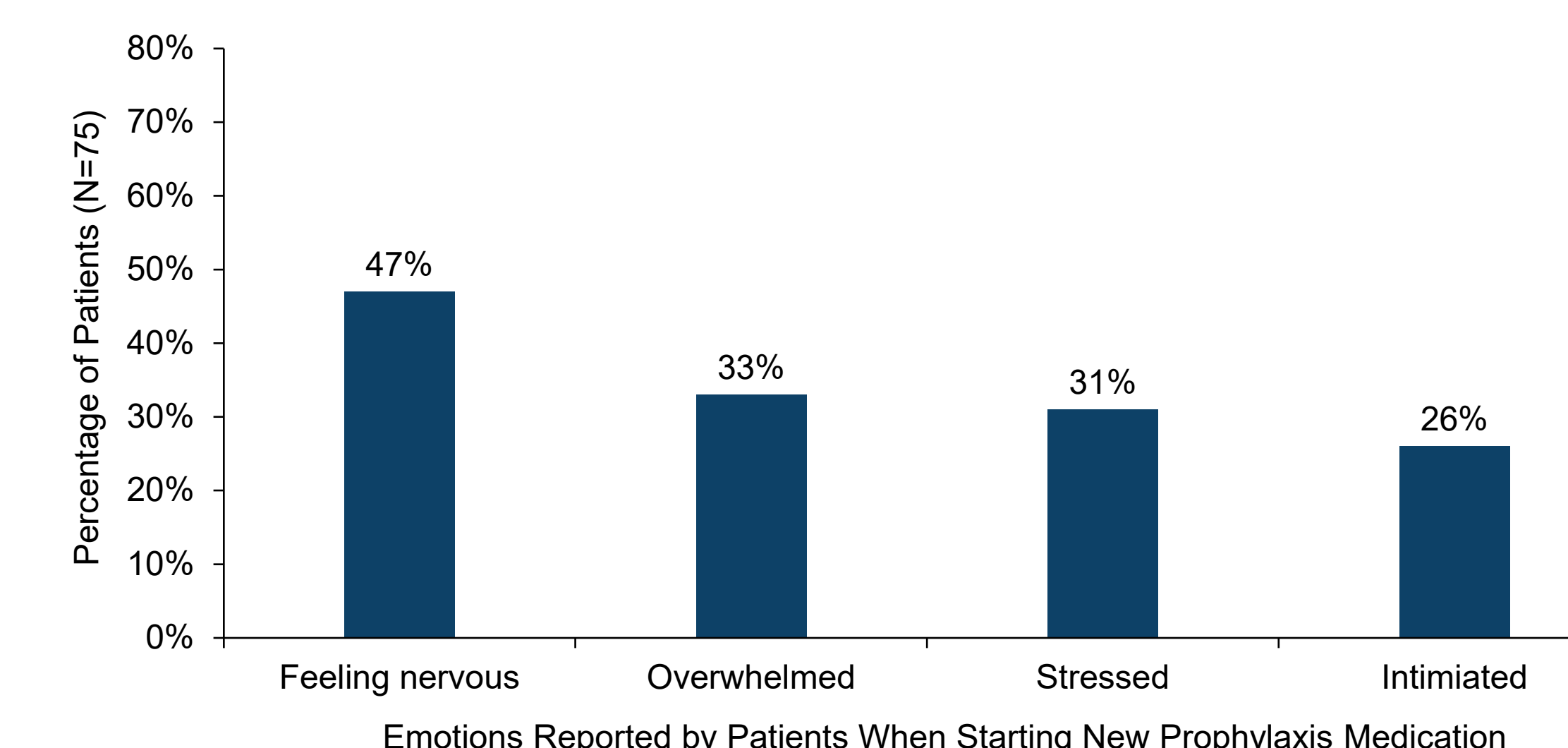


Table 2. Training Session Required to Self-Administer Parenteral On-Demand Therapies<sup>3</sup>

| Number of Training Sessions Required | Percentage of Patients |
|--------------------------------------|------------------------|
| 1-2                                  | 42.7%                  |
| 3-5                                  | 50.0%                  |
| 6-10                                 | 2.8%                   |

## Conclusions

- This TLR found that parenteral HAE therapies are burdensome for patients, yielding significant clinical and mental burdens that may impact quality of life
- Fear of injection, lack of skills, and perceived inconvenience of injection were commonly reported, leading to adherence challenges. Injection site reactions were frequently experienced
- Findings also illuminated the economic impact of parenteral HAE therapies on patients and payers, as healthcare services and supports, such as repeated medication administration trainings, were often required
- Novel, easy-to-administer HAE treatments, especially in the on-demand space – where only parenteral therapies exist – provide an opportunity to address unmet patient needs and improve treatment outcomes

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