

Background

- Hereditary angioedema (HAE) is a rare genetic disease resulting in deficiency (type I) or dysfunction (type II) in the C1-inhibitor protein and subsequent uncontrolled activation of the kallikrein-kinin system
- People living with HAE experience unpredictable, painful and debilitating attacks of tissue swelling in various locations of the body that can be life-threatening depending on the location affected
- While self-administered parenteral on-demand treatments are available for HAE attack management, future oral on-demand therapy may provide patients with a new option for treating attacks

Objective

- The objective of this analysis was to assess anticipated behavior related to potential oral on-demand treatment versus current parenteral on-demand treatment for patients with HAE

Methods

- The US Hereditary Angioedema Association (HAEA) recruited people living with HAE to complete an online survey
 - Recruitment was stratified to include 50% of patients taking on-demand only and 50% receiving LTP plus on-demand
- The survey was self-reported, and took respondents approximately 20 minutes to complete
- The survey was completed by 107 individuals between September 6 and October 19, 2022
- Data collected on anticipated behavior related to potential oral on-demand treatments were assessed
- Respondents provided consent for their data to be used anonymously or in aggregate
- Analysis was performed using descriptive statistics

Disclosures
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Results

- Respondents included 107 Type I or II HAE patients, 80.4% female, mean age 41 years (**Table 1**); response rate 69% (107/155)

Table 1. Respondent characteristics	
Characteristic	Total (N=107)
Age, mean (range), years	41 (16-83)
Gender, n (%)	
Female	86 (80.4)
Male	21 (19.6)
Type of therapy, n (%)	
On-demand only	53 (49.5)
Prophylaxis with on-demand	54 (50.5)
On-demand treatments used, n (%)	
Icatibant	84 (78.5)
C1 esterase inhibitor (recombinant)	13 (12.1)
C1 esterase inhibitor (human)	9 (8.4)
Ecallantide	1 (0.9)
Time to administration of on-demand treatment, n (%)	
<1 hour	46 (43.0)
≥1 hour	61 (57.0)
Prophylactic treatments used of those using prophylaxis, n (%)	
Lanadelumab	31 (57.4)
Berotralstat	7 (13.0)
C1 esterase inhibitor (subcutaneous)	7 (13.0)
Androgens/steroids	5 (9.3)
C1 esterase inhibitor (intravenous)	4 (7.4)

Conclusion

- Based on this analysis, people living with HAE currently using parenteral on-demand treatment may be more likely to carry on-demand treatment, treat more of their attacks, and treat attacks earlier if they had an approved oral on-demand treatment option available

Table 2. Comparison of anticipated behavior related to potential oral on-demand treatment versus current parenteral on-demand treatment		
Question	Percent, %	Behavior with current parenteral on-demand treatment, %
What percent of the time do you think you would carry an effective HAE on-demand pill/tablet with you when traveling outside your home?	95.1	63.9
What percent of attacks do you think you would treat with an HAE on-demand pill/tablet?	88.5	80.3
Would you treat your attacks faster/earlier with an HAE on-demand pill/tablet versus your current on-demand treatment? (“Yes”)	75.7	(mean time to treat is 2.4 hrs.)

- Patients reported that they anticipated carrying an oral on-demand treatment 95.1% (96.8% for patients ≤24 years of age) of the time on average versus 63.9% for patients with current parenteral on-demand treatment (**Table 2**)
- Patients reported that they would treat 88.5% of their attacks on average with an oral on-demand treatment (96.1% of attacks for patients ≤24 years of age) versus 80.3% for patients with current parenteral on-demand treatment (72.5% of attacks for patients ≤24 years of age) (**Table 2**)
- 75.7% of patients reported that they would plan to treat their attacks earlier with an oral on-demand treatment versus parenteral, including 100% (n=14) of patients ≤24 years of age (**Table 2**)
- Of these patients, 82.9% were extremely anxious about parenteral on-demand treatment, with 80% of those patients reporting that they would have less anxiety when anticipating using an oral on-demand treatment

