

# Burden of Injectable On-Demand Treatment for Hereditary Angioedema Attacks in Adolescents

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## Introduction

- Hereditary angioedema (HAE) is characterized by recurrent and unpredictable episodes of subcutaneous or submucosal swelling, which are debilitating and potentially fatal
- Treatment guidelines recommend the early use of on-demand treatment following the attack recognition to reduce morbidity and prevent mortality<sup>1-3</sup>
  - Currently available on-demand treatments for HAE attacks are administered subcutaneously or intravenously<sup>2</sup>
- The onset of HAE symptoms typically occurs during childhood and may intensify significantly during puberty<sup>1-3</sup>
  - Consequently, adolescents with HAE may face a substantial burden
- The current analysis examined the burden of HAE attacks in adolescent patients

## Methods

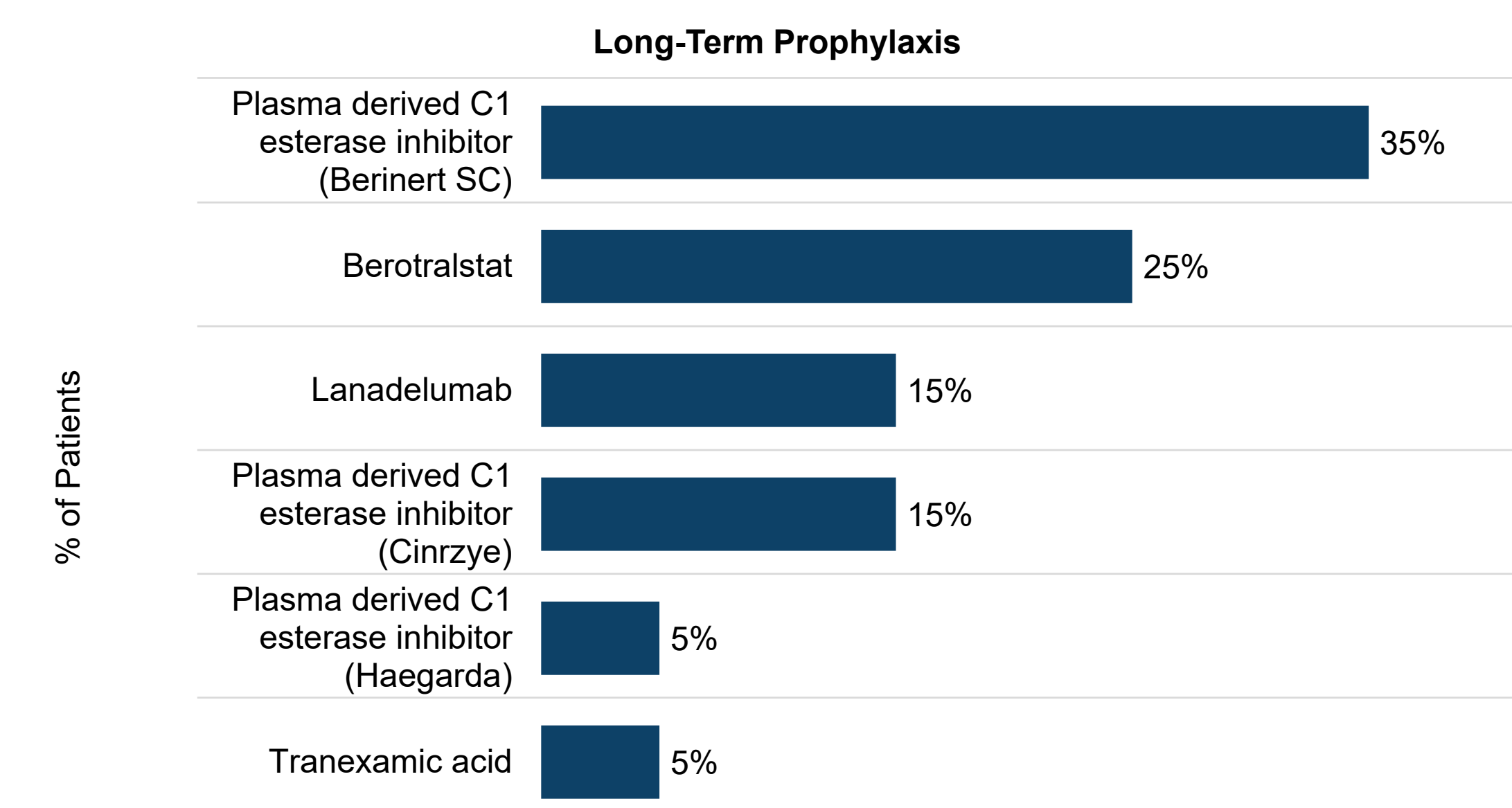
- Participants with Type 1 or 2 HAE who were 12-17 years old and had treated at least one HAE attack within the prior three months with an approved on-demand therapy were included in this analysis
- Respondents were recruited by patient advocacy groups (HAEA, HAE UK, AMSAO) from the USA, UK, and France, respectively, and a physician association (ITACA) from Italy to complete an online survey
- The survey was self-reported and took respondents approximately 20 minutes to complete
- Participants were asked to rate their anxiety about using on-demand treatment during their last attack on a scale of "0 "not anxious" to 10 "extremely anxious"
- Respondents provided consent for their data to be used anonymously or in aggregate

## Results

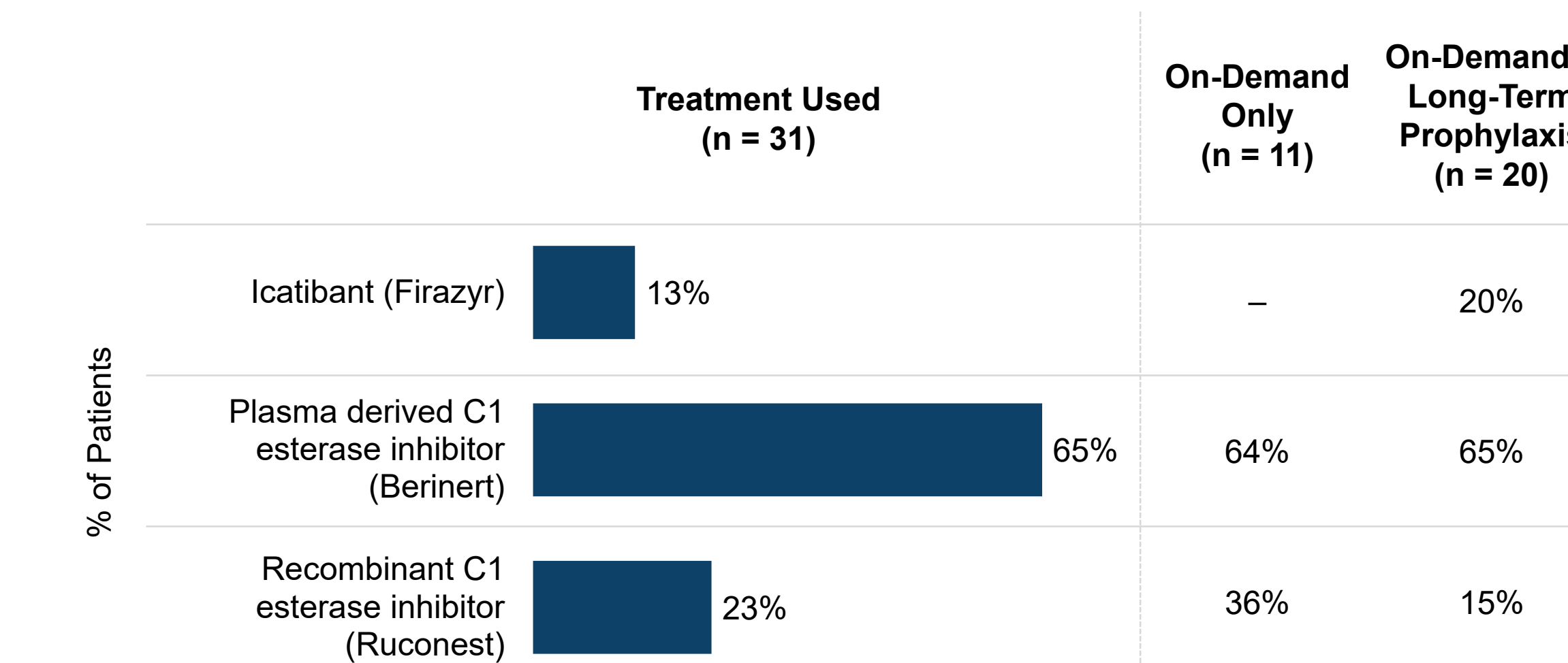
**Table 1. Respondent Characteristics**

	Total (n = 31)	On-Demand Only (n = 11)	On-Demand + Long-Term Prophylaxis (n = 20)
Current Mean Age, Years (SD)	14.3 (1.6)	14.4 (1.4)	14.3 (1.7)
Gender			
Male	61.3%	54.5%	65.0%
Female	38.7%	45.5%	35.0%
HAE Type			
Type 1	87.1%	90.9%	85.0%
Type 2	12.9%	9.1%	15.0%
Comorbidities			
Anxiety	22.6%	27.3%	20.0%
Asthma	16.1%	18.2%	15.0%
Depression	6.5%	9.1%	5.0%
Liver Disease	6.5%	0%	10.0%
None of the above	64.5%	72.7%	60.0%
Country			
United States	45.2%	54.5%	40.0%
Italy	45.2%	45.5%	45.0%
France	3.2%	0%	5.0%
United Kingdom	6.5%	0%	10.0%

**Figure 1. Long-Term Prophylaxis at Time of Last Treated Attack (n = 20)**

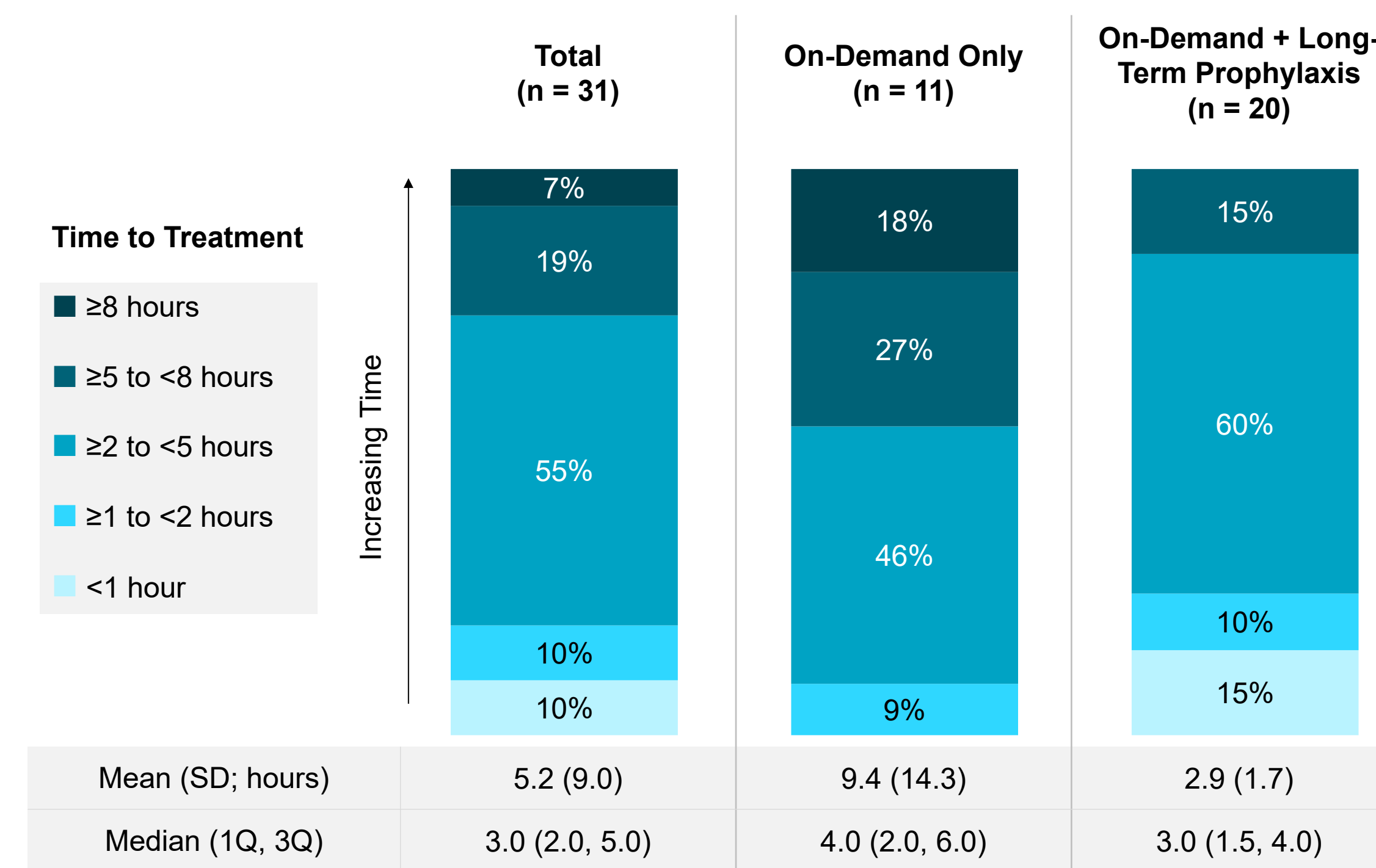


**Figure 2. On-Demand Therapy Used for Last Treated Attack**



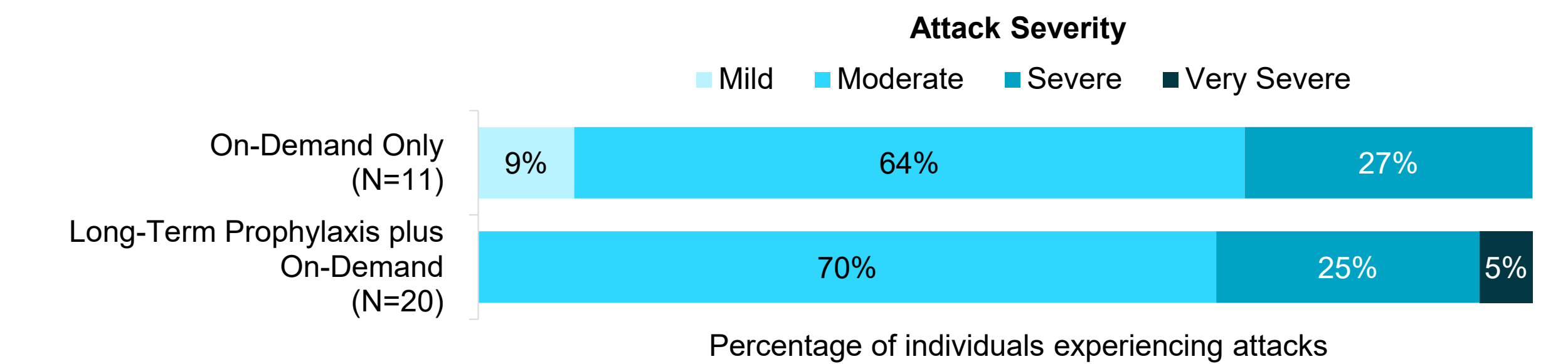
- The most commonly used on-demand treatment was plasma derived C1 esterase inhibitor (Berinert) for both the on-demand-only group (64%) and the on-demand plus long-term prophylaxis group (65%) (Figure 2)
- IV therapies were more common than SC in adolescents

**Figure 3. Time to On-Demand Treatment**



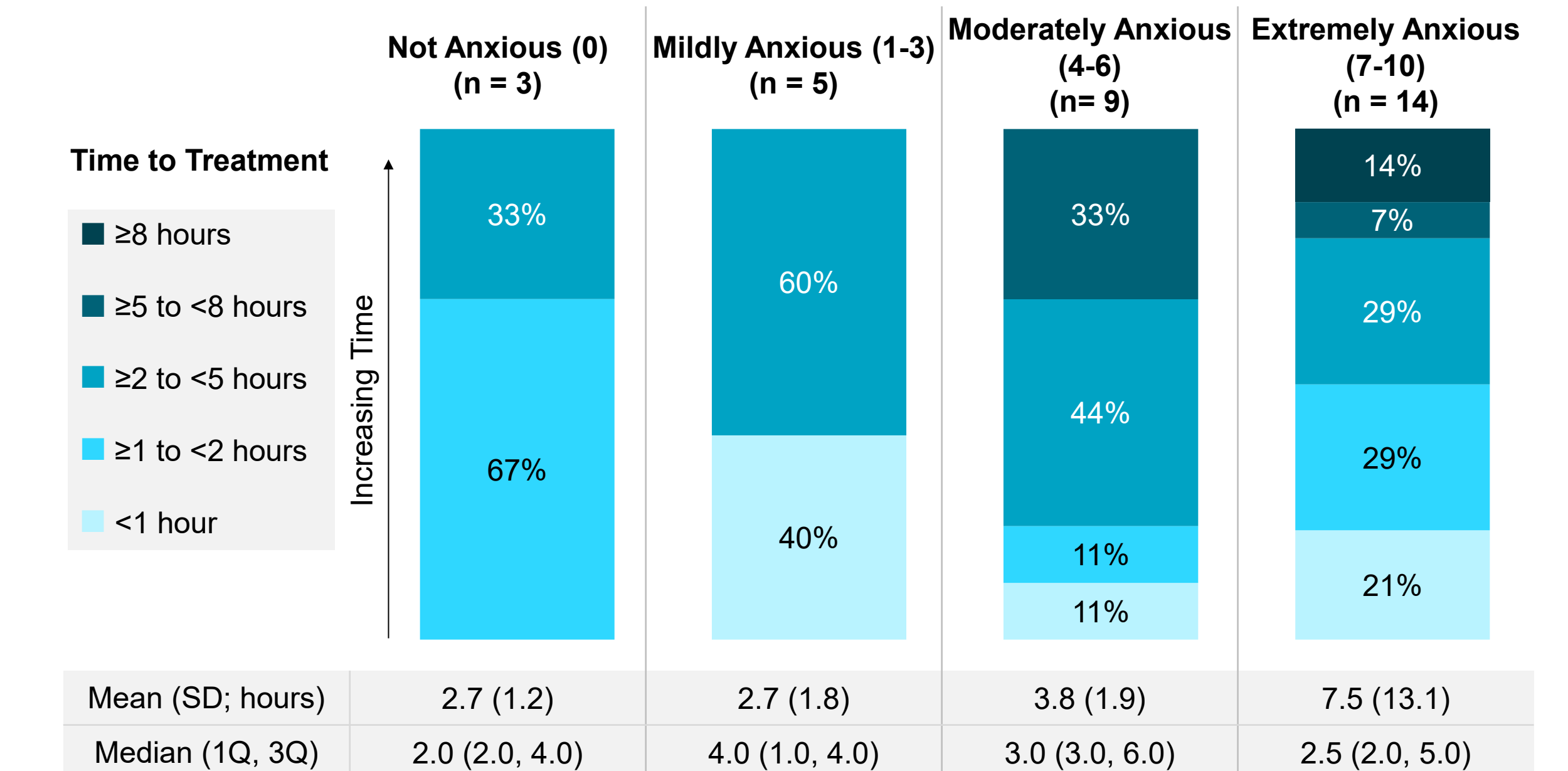
- Mean time to on-demand treatment was less for On-Demand + LTP respondents than On-Demand Only (Figure 3)
- 10% of respondents treated in <1 hour

**Figure 4. Attack Severity at the Time of Treatment**



- At the time of treatment, 68% (21/31) of attacks had progressed to moderate and 29% (9/31) to severe/very severe
- 26% (8/31) required a hospital/ER visit for treatment of their last attack

**Figure 5. Time to On-Demand Treatment by Level of Treatment-Related Anxiety**



- 45% (14/31) of respondents were extremely anxious about treating their last attack with on-demand treatment (Figure 5)
- Median time to treatment was 2 hours for those who were not anxious and 2.5 to 4 hours for those who were anxious

## Conclusions

- Most adolescents delayed treatment of their last HAE attack (mean 5.2 hours), after attacks had progressed in severity
- Treatment-related anxiety was correlated with increased time to treatment
- Effective alternatives to parenteral on-demand treatments may reduce attack morbidity in adolescents with HAE

## Acknowledgments

The authors wish to thank Jason Allaire, PhD of Generativity Health Outcomes Research for his assistance with this poster. Funding for Dr. Allaire was provided by KalVista Pharmaceuticals.

## Disclosures

Paul Audhya is an employee of and owns stock in KalVista. Busse: reports consulting fees: Takeda, KalVista, CVS Specialty, BioCryst, CSL, Behring, ADARx, Astria, Pharvaris. Mauro Cancian received honoraria and/or meeting/travel support paid to the institution from KalVista Pharmaceuticals, BioCryst, CSL Behring, Pharvaris, and Takeda. Sandra Christiansen participated in advisory boards for KalVista, BioCryst, and US HAEA Medical Advisory Board. Sherry Danese received consulting fees from KalVista. Vibha Desai is an employee of and owns stock in KalVista. Tariq El-Shanawany received educational support, research support, speaker fees and/or consultant fees from ALK-Abello, Allergy Therapeutics, CSL, KalVista, Octapharma, Novartis, Takeda and Viatrix. Maeve O'Connor is a speaker/consultant/advisor or research for KalVista, Pharming, CSL, GSK, Blueprint, TEVA, AZ, Sanofi, Grifols, Abbvie and is the Chief Medical Officer of the CMC. Sinisa Savic received consulting fees and/or honoraria from CSL Behring, BioCryst, KalVista, Pharvaris, Novartis, and Astra Zeneca. Julie Ulloa received consulting fees from KalVista. Paola Triggianese report no disclosures. Patrick Yong received consulting fees, honoraria and/or support for attending meetings from BioCryst, CSL Behring, KalVista, Pharming, Pharvaris and Takeda. Andrea Zanichelli received honoraria, meeting/travel support, and/or served on advisory boards for KalVista, Astria, BioCryst, CSL Behring, Pharming, Pharvaris, and Takeda.

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## Presented

AAAAI Annual Meeting 2025. February 28 to March 3, 2025 in San Diego, CA.

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