



Hereditary Angioedema Attack Frequency and Severity According to Individuals Taking Berotralstat for Long-Term Prophylaxis

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INTRODUCTION

- Reducing the frequency and severity of hereditary angioedema (HAE) attacks is an important goal of long-term prophylaxis (LTP), playing a key role in improving quality of life for individuals with HAE.¹
- Berotralstat is the only targeted oral LTP approved for the treatment of HAE, recommended as one of the first-line LTP treatments for HAE by the World Allergy Organization/European Academy of Allergy and Clinical Immunology global HAE practice guidelines.¹
- This study aimed to understand real-world experience with berotralstat, specifically, the effect of berotralstat on HAE attack frequency and severity among patients naive to LTP and those switching from another LTP.

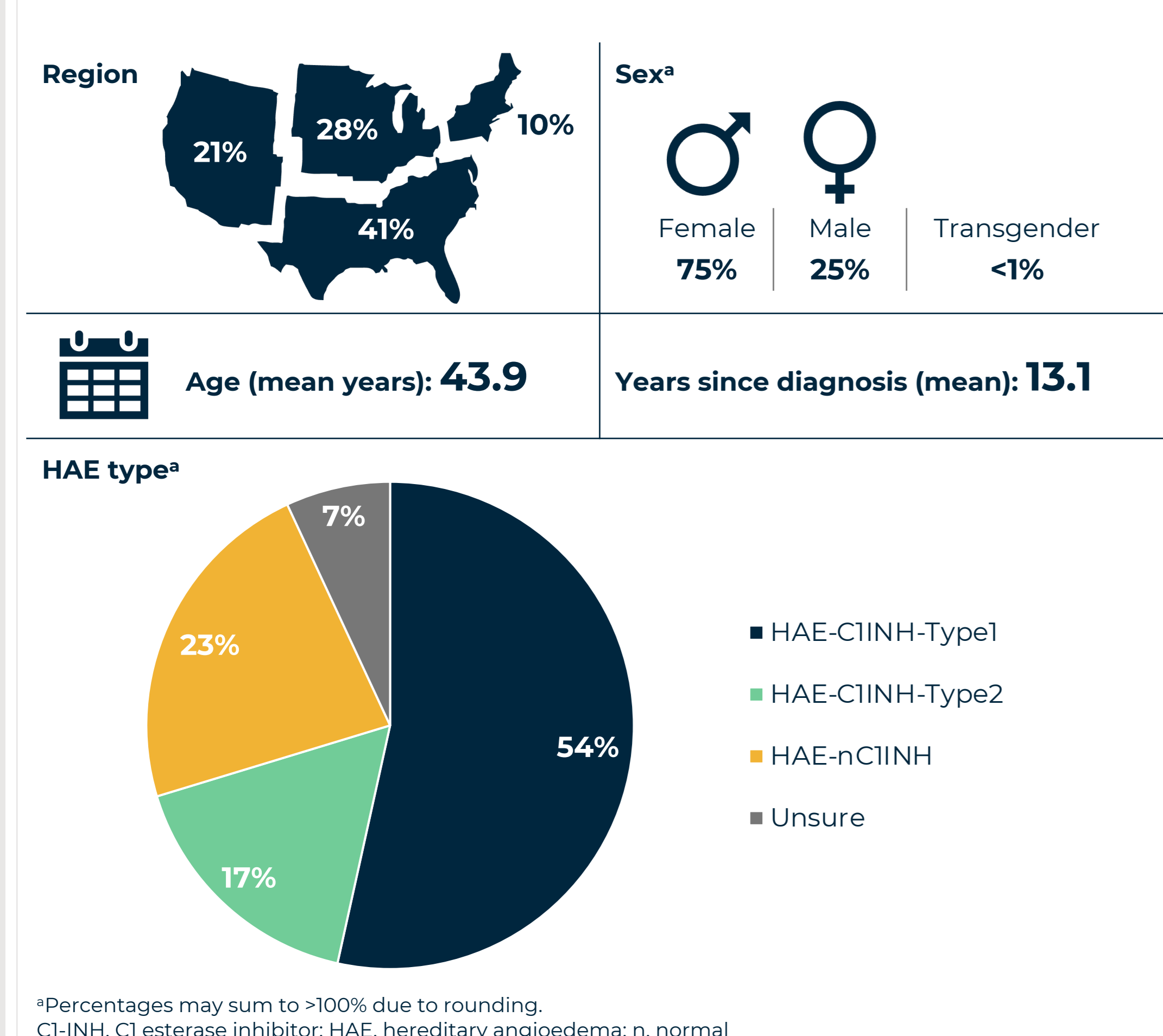
METHODS

- An online discussion and survey were conducted among individuals with HAE in the United States in January 2023, April 2024, and March 2025.
 - The WCG Institutional Review Board determined that the study was exempt from review.
- Study participants were US residents, aged ≥18 years, diagnosed with HAE by a health care professional, and taking berotralstat for long-term prophylaxis at the time of study recruitment.
 - Individuals who discontinued berotralstat during the study were permitted to continue participating in study activities.
- Changes in attack frequency and severity after berotralstat initiation were stratified by prior LTP use and berotralstat treatment duration.
- Treatment satisfaction was also assessed and stratified by attack frequency and severity.

RESULTS

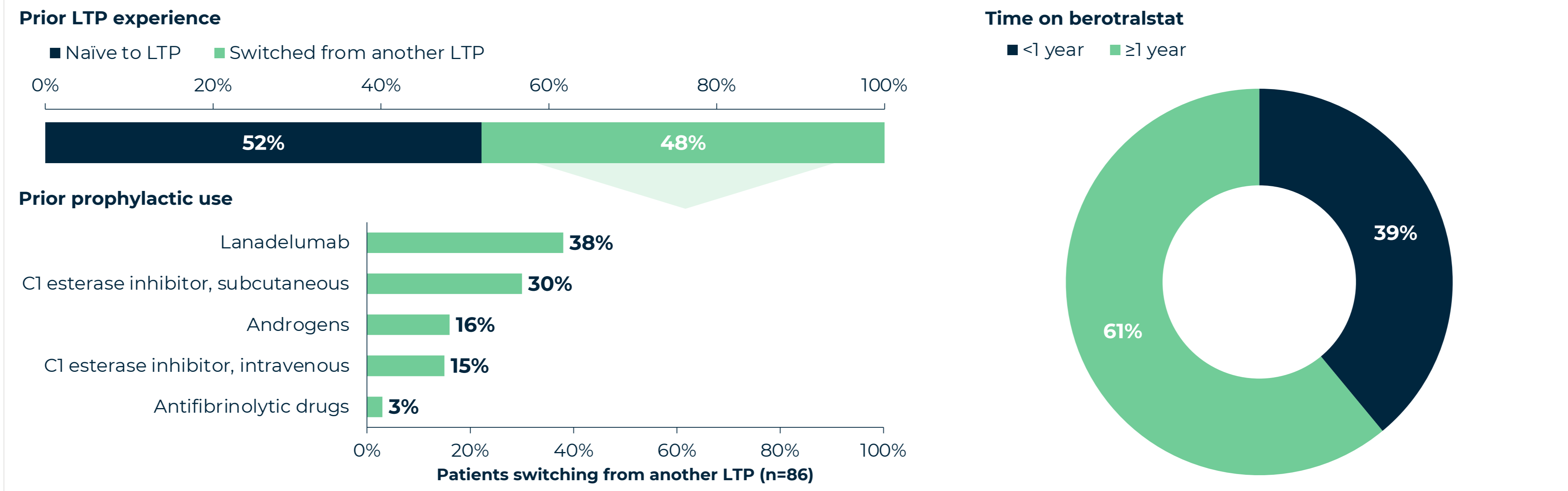
- A total of 181 individuals with HAE participated in research activities over the 3-year period (Figure 1).
 - Approximately half were naive to LTP prior to starting berotralstat, and the majority had been taking the medication for more than 1 year (Figure 2).

Figure 1. Participant Characteristics (N=181)



RESULTS, CONTINUED

Figure 2. Prior LTP Experience and Berotralstat Treatment Duration



- Nearly all participants who were naive to LTP and approximately two-thirds of those who switched to berotralstat from another LTP reported that their attacks were less frequent since starting berotralstat (Figure 3).
 - Reports of changes in attack frequency were similar regardless of time on berotralstat (Figure 3).
 - Change in attack frequency was consistent across HAE types: 78%, 90%, and 76% of individuals with CIINH-Type 1, CIINH-Type 2, and nCIINH, respectively, reported less frequent attacks since starting berotralstat (data not shown).
- The majority of participants reported less severe attacks; none of the individuals who were naive to LTP, and only 7% who switched to berotralstat, reported that their attacks were more severe (Figure 4).
 - Approximately two-thirds of participants taking berotralstat for less than 1 year, and 7 in 10 of those taking the medication for at least 1 year, reported less severe attacks (Figure 4).
 - The change in attack severity since starting berotralstat was also similar across HAE types. At least two-thirds of participants with CIINH-Type 1, CIINH-Type 2, and nCIINH (66%, 65%, and 78%, respectively) reported less severe attacks (data not shown).

Figure 3. Change in Attack Frequency Since Starting Berotralstat

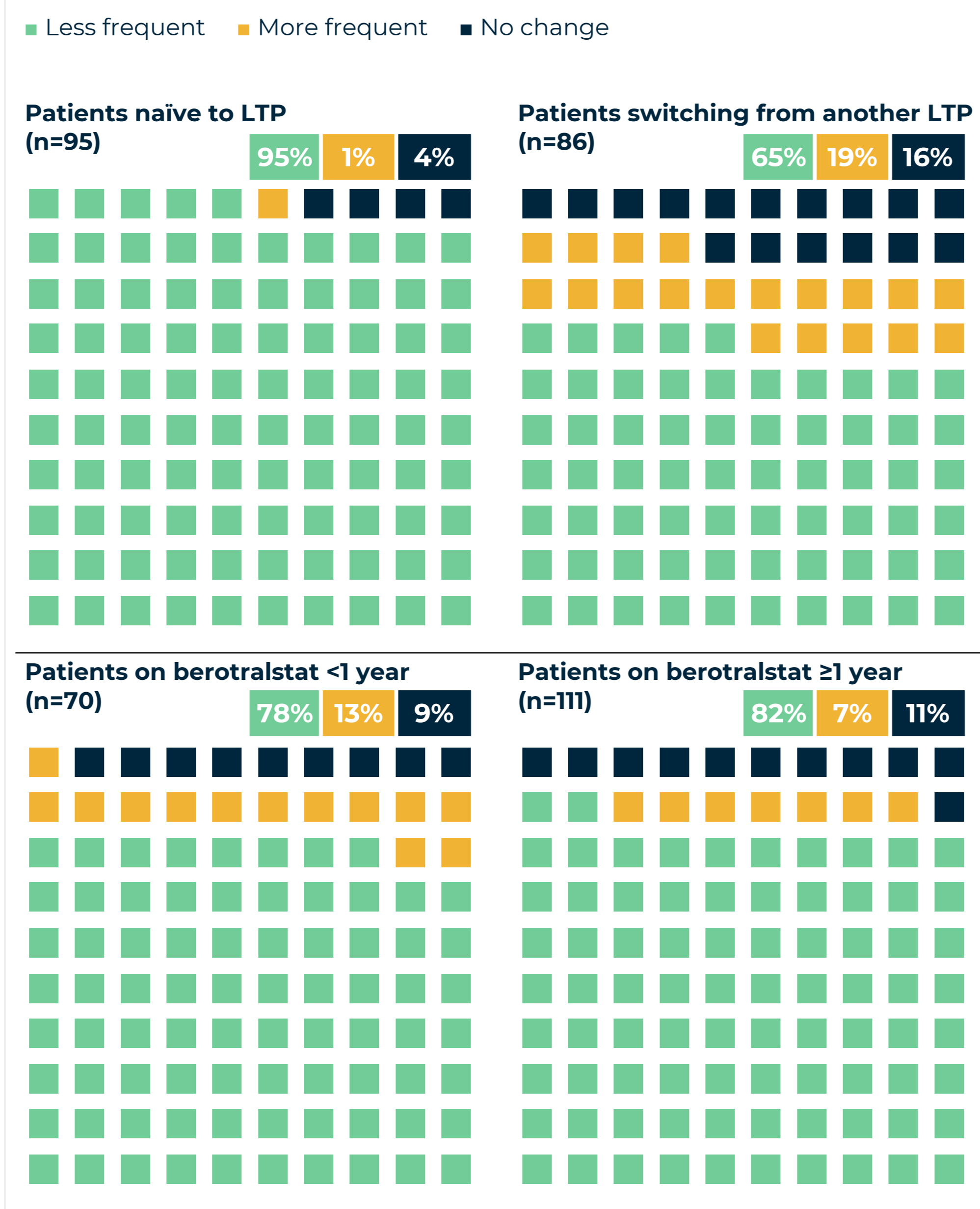
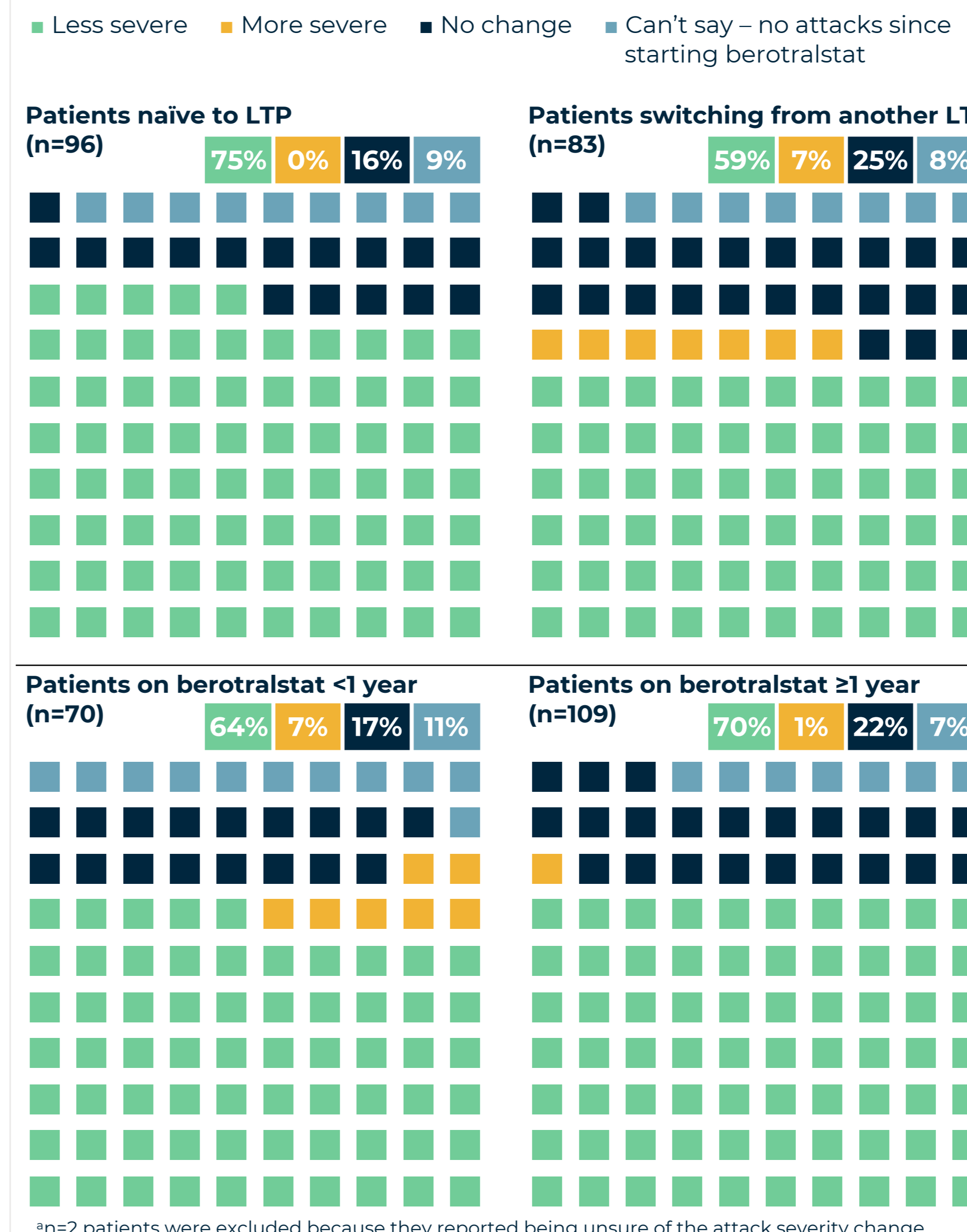


Figure 4. Change in Attack Severity Since Starting Berotralstat (n=179)^{a,b}



- Nearly all individuals were at least somewhat satisfied with berotralstat.
 - More than 8 in 10 participants who experienced less frequent (Figure 5) and less severe attacks (Figure 6) were extremely satisfied with berotralstat; satisfaction was also high for individuals with the same number of attacks and severity of attacks.
 - Satisfaction was lower for individuals who experienced more frequent/more severe attacks since starting berotralstat; of the 17 who experienced more frequent attacks, 1 was dissatisfied (Figure 5), and of the 6 who had more severe attacks, 1 was dissatisfied (Figure 6).

Figure 5. Patient Satisfaction With Berotralstat by Attack Frequency Since Starting Berotralstat

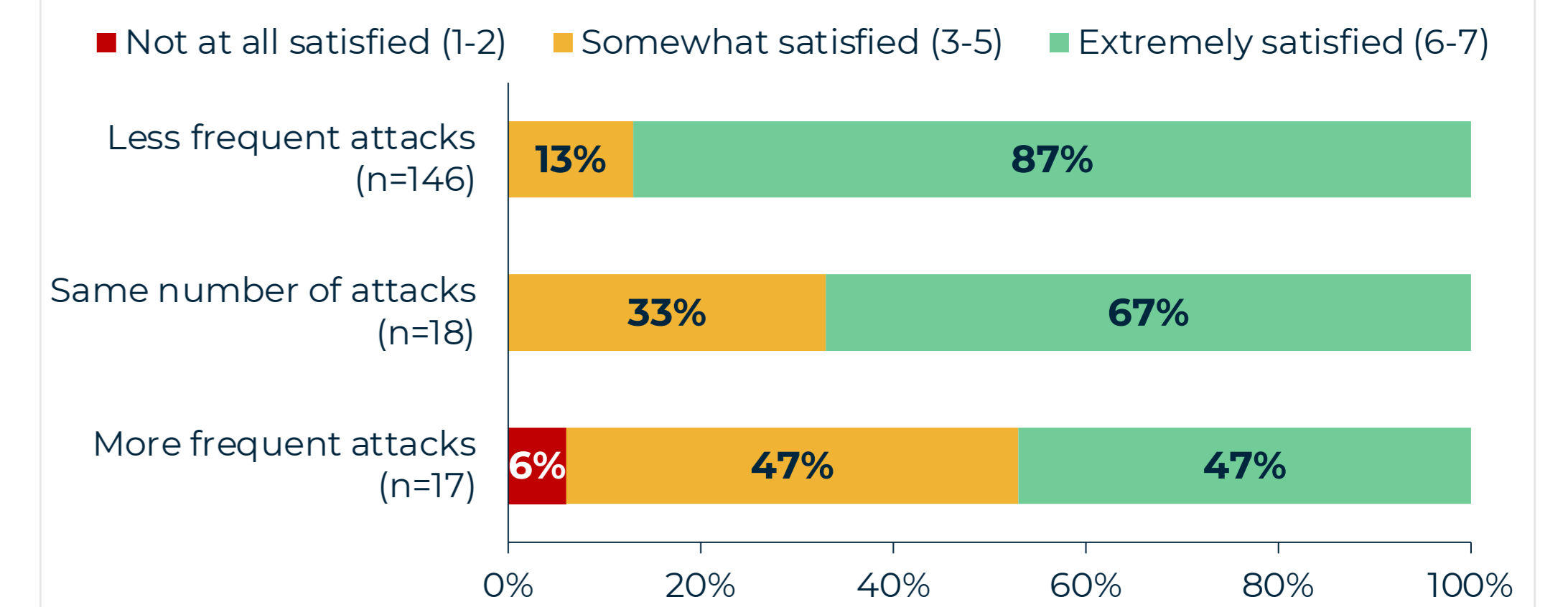
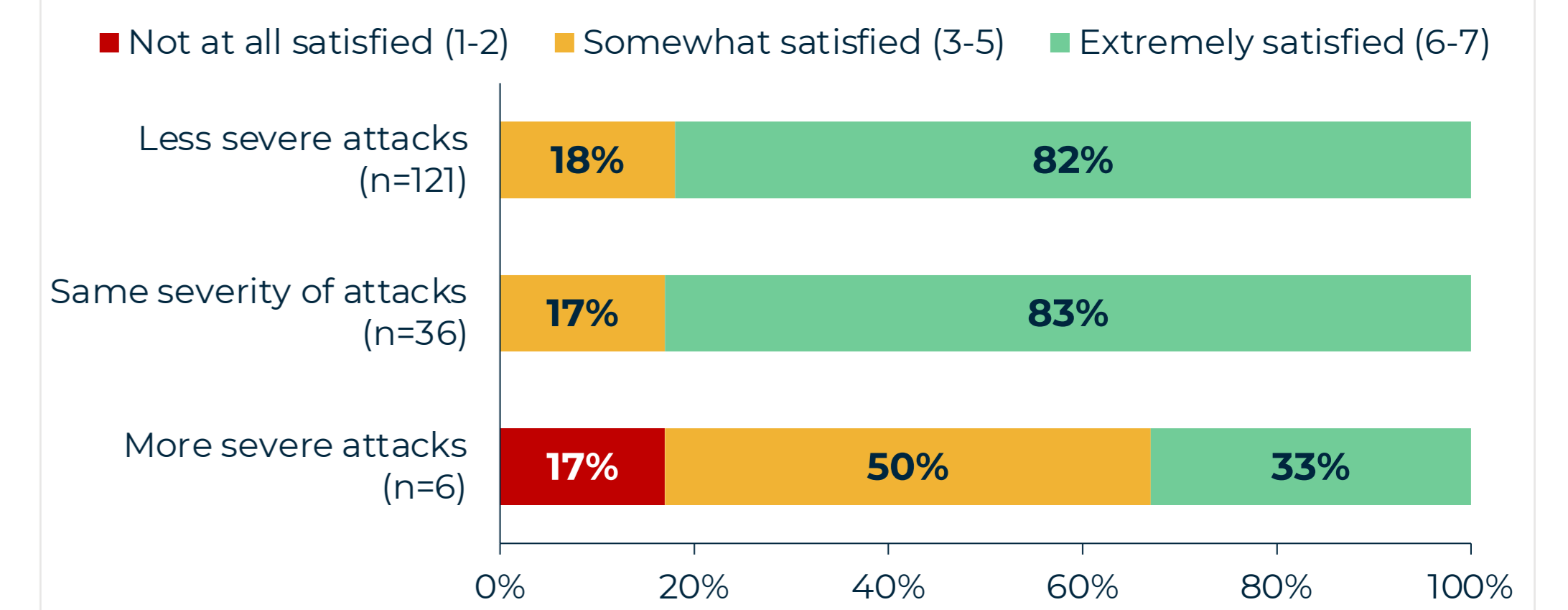


Figure 6. Patient Satisfaction With Berotralstat by Attack Severity Since Starting Berotralstat (n=163)^a



CONCLUSIONS

- Most individuals with HAE had fewer and less severe attacks after starting berotralstat, regardless of whether they were naive to LTP or had switched from another LTP, or the length of time they were on berotralstat.
- Most participants were highly satisfied with berotralstat, particularly those with less frequent or less severe attacks, as well as those whose frequency or severity remained unchanged.

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1. Maurer M, et al. *Allergy*. 2022;77(7):1961-1990.

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