

Unmet Needs Associated with Non-androgen Injectable Long-term Prophylaxis (LTP) Therapies for HAE

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Background

- Hereditary angioedema (HAE) is a rare genetic disease, most commonly caused by deficiency (type I) or dysfunction (type II) of the C1-inhibitor protein and subsequent uncontrolled activation of the kallikrein-kinin system, resulting in attacks of tissue swelling [1,2,3]
- Despite the increasing availability and utilization of non-androgen long-term prophylaxis (LTP) therapies, physicians still express unmet needs with currently available treatment options [4]

Objective

- To describe the most common physician-reported unmet needs among patients with HAE receiving non-androgen LTP

Methods

- Data were derived from the Adelphi HAE Wave II Disease Specific Programme (DSP)[™], a real-world, cross-sectional study with retrospective data collection in the US (January-November 2023)
- HAE treating physicians utilized patients' medical charts and their diagnostic and clinical judgement to provide data on patient demographics, clinical characteristics, and treatment
- This analysis included HAE patients aged ≥12 with HAE type 1 or 2 and receiving a non-androgen LTP at the time of data collection. All analyses were descriptive
- The survey was conducted according to relevant guidelines and legislation, and the methodology has been previously published and validated [5-6]

Table 1. Physician demographics

	(n=56)
Speciality, n (%)	
Allergist	7 (12.5)
Allergist-immunologist	26 (46.4)
Dermatologist	9 (16.1)
ENT specialist	4 (7.1)
Haematologist	4 (7.1)
Pulmonologist	6 (10.7)
Total number of HAE patients currently managed, mean ± SD [median, IQR]	50.0 ± 123.59 [15.5, 10.0-40.0]

Acknowledgments

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This poster is intended for healthcare professionals

Results

- Overall, 56 physicians (Allergists [12.5%], Allergist-Immunologists [46.4%], Hematologists [7.1%], Pulmonologists [10.7%], Dermatologists [16.1%] and ENT specialists [7.1%]) provided data for 180 patients treated with non-androgen LTPs (Table 1)
- Patient baseline demographic and disease characteristics are displayed in Table 2
- Mean ± standard deviation (SD) age of patients receiving injectable LTP was 35.0±12.20 years; the mean age of patients receiving oral LTP was 33.9±14.27 years. The majority of both groups were female
- The mean ± SD duration of non-androgen injectable LTP treatment was 2.5±2.0 years and 1.5±1.02 years for oral LTP treatment
- Years since first HAE attack, years since HAE diagnosis and the proportion of patients receiving LTP only were higher for patients receiving oral LTP (Table 2).

Table 2. Patient baseline demographic and disease characteristics

	Injectable LTP (n=133)	Oral LTP (n=48)
Age, years, mean ± SD [median, IQR]	35.0 ± 12.20 [34.0, 26.0-34.0]	33.9 ± 14.27 [30.5, 23.0-42.2]
Sex, n (%)		
Female	67 (50.4)	25 (52.1)
Male	66 (49.6)	23 (47.9)
Years since first attack, mean ± SD [median, IQR] (n)	7.9 ± 9.12 [4.1, 2.3-9.1] (105)	11.7 ± 12.72 [7.9, 3.0-14.0] (34)
Years since diagnosis, mean ± SD [median, IQR] (n)	7.8 ± 8.83 [4.0, 2.1-9.8] (122)	10.6 ± 12.80 [6.7, 1.7-13.2] (38)
HAE type, n (%)		
Type I	110 (82.7%)	36 (75.0%)
Type II	23 (17.3%)	12 (25.0%)
Current treatment, n (%)		
LTP only	32 (24.1)	16 (33.3%)
LTP and OD	101 (75.9)	32 (66.7%)
Years receiving current LTP, mean ± SD [median, IQR] (n)	2.5 ± 2.02 [1.9, 1.0-4.0] (123)	1.5 ± 1.02 [1.3, 0.8-2.2] (45)

Abbreviations: HAE; hereditary angioedema, IQR; interquartile range, LTP; long-term prophylaxis, OD; on demand, SD; standard deviation

- 133 were prescribed injectable LTPs and 48 were prescribed oral LTP, with one patient receiving both. The non-androgen LTP types are shown in Figure 1
- Most frequently, patients were prescribed lanadelumab (n=76; 42%), followed by berotralstat (n=48, 27%), subcutaneous C1-INH (n=33; 18%), and intravenous C1-INH (n=24; 13%)

Figure 1. Current long-term prophylaxis treatment in patients with HAE

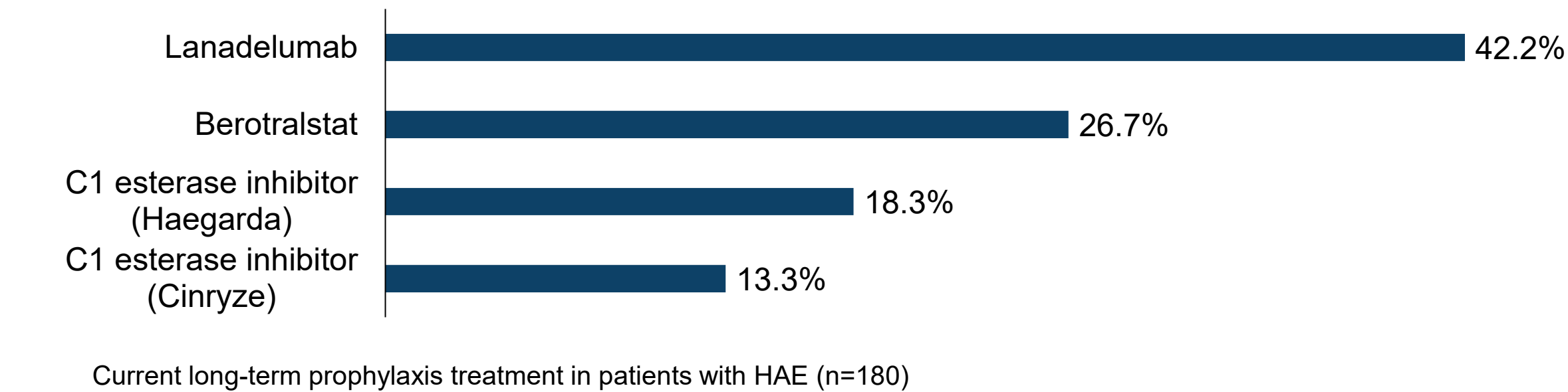
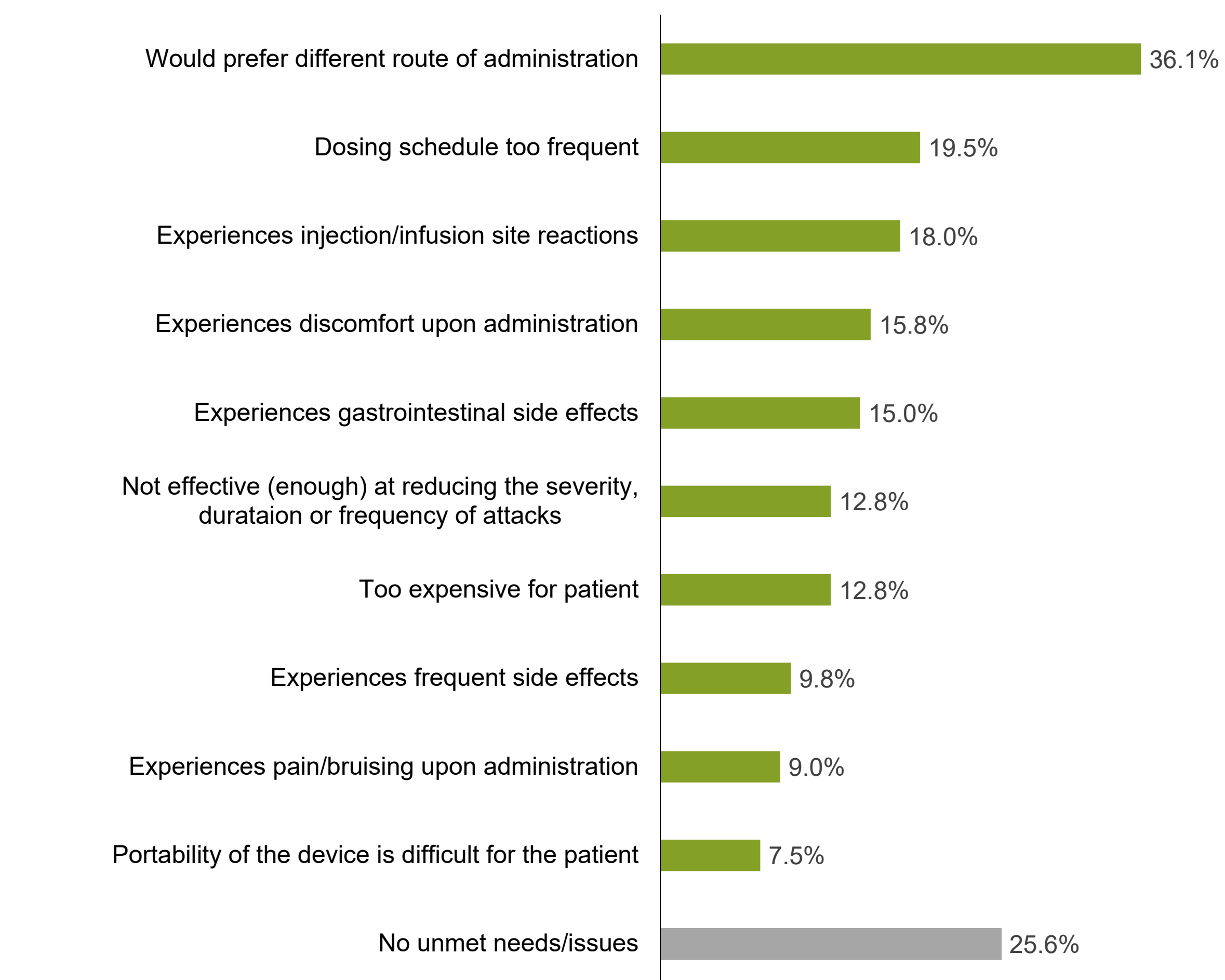


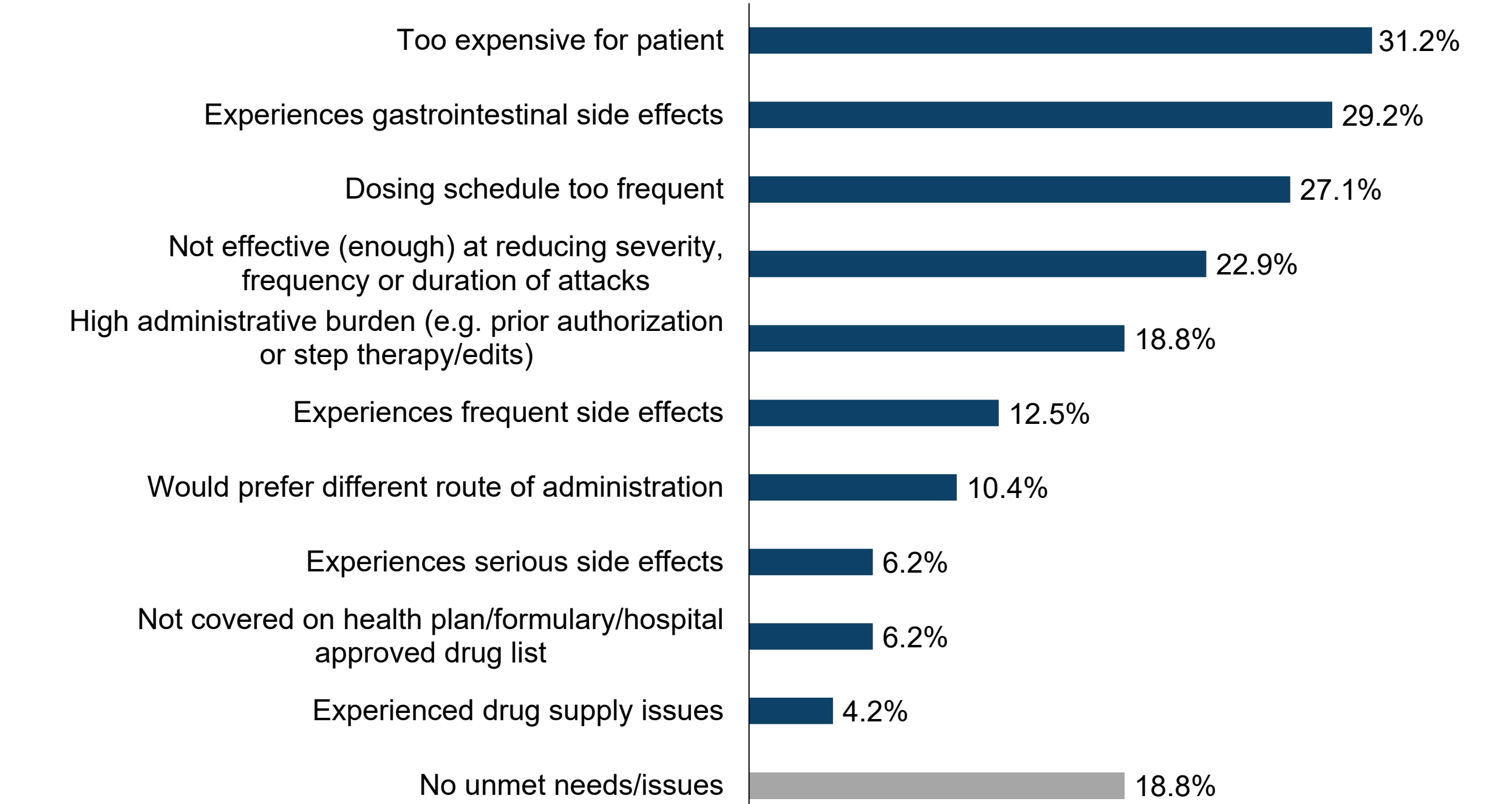
Figure 2. Unmet needs of injectable non-androgen long-term prophylaxis among patients with HAE



Top 10 unmet needs of patients receiving injectable non-androgen LTP (n=133). Multiple choice question

- The most frequent physician-selected unmet need of patients receiving injectable LTP was different route of administration (n=48; 36%) (Figure 2)
- The most frequent physician-selected unmet need of patients receiving oral LTP was that the treatment was too expensive (n=15; 31%) (Figure 3)

Figure 3. Unmet needs of oral non-androgen long-term prophylaxis among patients with HAE



Top 10 unmet needs of patients receiving oral non-androgen LTP (n=48). Multiple choice question

Limitations

- The proportion of patients with unmet needs may potentially have been underestimated, as the current non-androgen LTP users included in the study had a mean duration of their current LTP of 2.5 years (survivor bias) and also because the unmet needs were reported by physicians

Conclusions

- This study provides real-world insights into physicians' perceptions of unmet needs associated with oral and injectable non-androgen LTP treatments
- Current route of administration, frequent dosing schedules, side effects, and discomfort were the most common unmet needs/issues reported for patients using injectable non-androgen LTPs
- Lack of efficacy and gastrointestinal issues were the most common unmet needs/issues reported for patients using oral LTPs
- Despite the availability of LTPs, including non-androgen LTPs, their use is associated with a high treatment burden, including route of administration for injectables, dosing frequency, cost and side effects

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