

Characterising the Patient Experience With Current Treatment of Hereditary Angioedema Attacks in the United Kingdom

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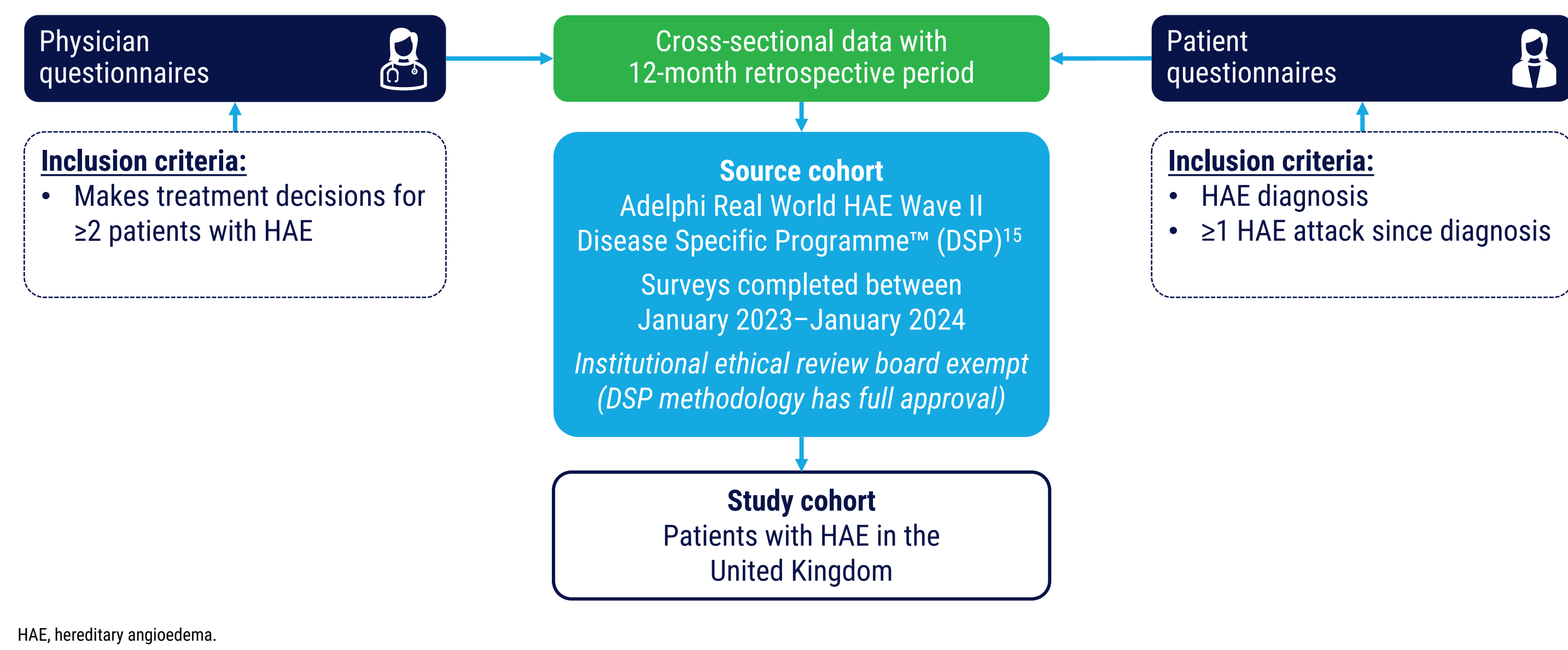
Rationale

- Hereditary angioedema (HAE) is a rare genetic condition characterised by painful, often debilitating swelling attacks affecting the skin, gastrointestinal tract, and airways.^{1,2}
 - Attack signs and symptoms may include swelling, pain, nausea, vomiting, difficulty swallowing, and airway obstruction.^{1,2}
- HAE treatment may be taken:
 - Prophylactically to prevent an attack, termed long-term prophylaxis (LTP).^{1,2}
 - On demand after an attack has begun, termed on-demand treatment (ODT).^{1,2}
- Most currently approved LTP medications and all currently approved ODT medications are administered by injection, which may lead to discomfort, pain, inconvenience, and reduced compliance.¹⁻¹⁰
 - An unmet need remains for additional treatments combining injectable-like efficacy, a well-tolerated profile, and ease of administration.¹¹⁻¹⁴
- The objective of this analysis was to characterise the patient experience with current and previous treatment of HAE attacks in the United Kingdom.

Methods

- Data were collected in the United Kingdom from January 2023–January 2024 through the Adelphi HAE Wave II Disease Specific Programme™, a real-world cross-sectional survey of physicians and their consulting patients (Figure 1).
- Physicians used clinical judgement and patients' medical chart data to report demographics, disease characteristics, and current treatment.
- Descriptive statistics are reported.

Figure 1. Disease Specific Programme™ (DSP) methodology



Results

- 5 physicians reported data for 23 patients with HAE in the United Kingdom (Table 1).
 - Most patients were female (57%, n=13), White (96%, n=22), and had HAE type 1 (83%, n=19).
 - Mean age ± standard deviation (SD) (range) was 41.8 ± 16.6 (17–73) years.
- Mean time ± SD (range) since diagnosis was 11.0 ± 9.0 (1–32) years.
- Mean number of attacks in the year prior to survey ± SD (range) were 6.1 ± 7.1 (0–27; Table 1).

Table 1. Patient demographics and clinical characteristics

	All patients (n=23)	Patients currently prescribed LTP and ODT (n=10)	Patients currently prescribed ODT only (n=12)	Patients not currently prescribed treatment (n=1)
Age (years), mean ± SD [range]	41.8 ± 16.6 [17–73]	41.3 ± 14.3 [20–71]	40.8 ± 18.7 [17–73]	60 ± 0 [60–60]
Female, n (%)	13 (57%)	6 (60%)	6 (50%)	1 (100%)
Race, White, n (%)	22 (96%)	10 (100%)	11 (92%)	1 (100%)
HAE type, n (%)				
Type 1	19 (83%)	9 (90%)	9 (75%)	1 (100%)
Type 2	2 (9%)	0 (0%)	2 (17%)	0 (0%)
HAE with normal C1-INH	2 (9%)	1 (10%)	1 (8%)	0 (0%)
Current treatment, n (%)				
Prescribed LTP and ODT	10 (43%)	10 (100%)	0 (0%)	0 (0%)
Prescribed ODT only	12 (52%)	12 (100%)	0 (0%)	0 (0%)
Not prescribed treatment	1 (4%)	0 (0%)	0 (0%)	1 (100%)
Number of HAE attacks in 12 months prior to data collection, mean ± SD [range]	6.1 ± 7.1 [0–27]	8.0 ± 8.6 [0–27]	5.1 ± 5.7 [0–20]	0 ± 0 [0–0]
	(n=16)	(n=6)	(n=10)	(n=0)
Time since diagnosis (years), mean ± SD [range]	11.0 ± 9.0 [1–32]	14.7 ± 11.4 [1–32]	8.9 ± 7.1 [1–19]	-

CHNH, C1 esterase inhibitor; HAE, hereditary angioedema; LTP, long-term prophylaxis; n, number of patients; ODT, on-demand therapy; SD, standard deviation.

- 10 patients (43%) were currently prescribed LTP for a mean 3.3 ± 4.5 (0–13, n=7) years, including danazol (n=5), lanadelumab (n=3), and berotralstat (n=2) (Table 2).
- 22 patients (96%) were currently prescribed ODT for a mean 4.2 ± 4.5 (0–13; n=17) years, including icatibant (n=20) and C1 inhibitors (n=9) (Table 2).

Table 2. Current and previous medications for long-term prophylaxis and on-demand treatment

Medication, n (%)	Current treatment ^a	Previous treatment ^{a,b}
LTP (n=10)		(n=9)
Danazol	5 (50%)	4 (44%)
Lanadelumab	3 (30%)	-
Berotrastat	2 (20%)	-
Tranexamic acid	1 (10%)	5 (56%)
C1 inhibitor	1 (10%)	1 (11%)
ODT (n=22)		(n=13)
Icatibant	20 (91%)	5 (38%)
C1 inhibitor	9 (41%)	6 (46%)
Other	-	1 (8%)
Don't know	-	1 (8%)

LTP, long-term prophylaxis; n, number of patients; ODT, on-demand treatment.

^aCurrent and previous treatment questions were multiple choice, so the sum of the categories can be greater than the base. ^bPrevious treatment included any LTP or ODT medication that was prescribed for HAE and subsequently discontinued. For previous LTP and ODT treatment, data shown here are for the most recent previous treatment regimen prior to the current treatment regimen.

Results (continued)

- The most common unmet need reported for patients by their physicians was a desire for treatment with a different administration route for LTP (30%, n=3; Figure 2) and ODT (77%, n=17; Figure 3).
- All LTP and ODT prescribed for these patients were injectable medications.

Figure 2. Unmet needs and other issues associated with current long-term prophylaxis (n=10)

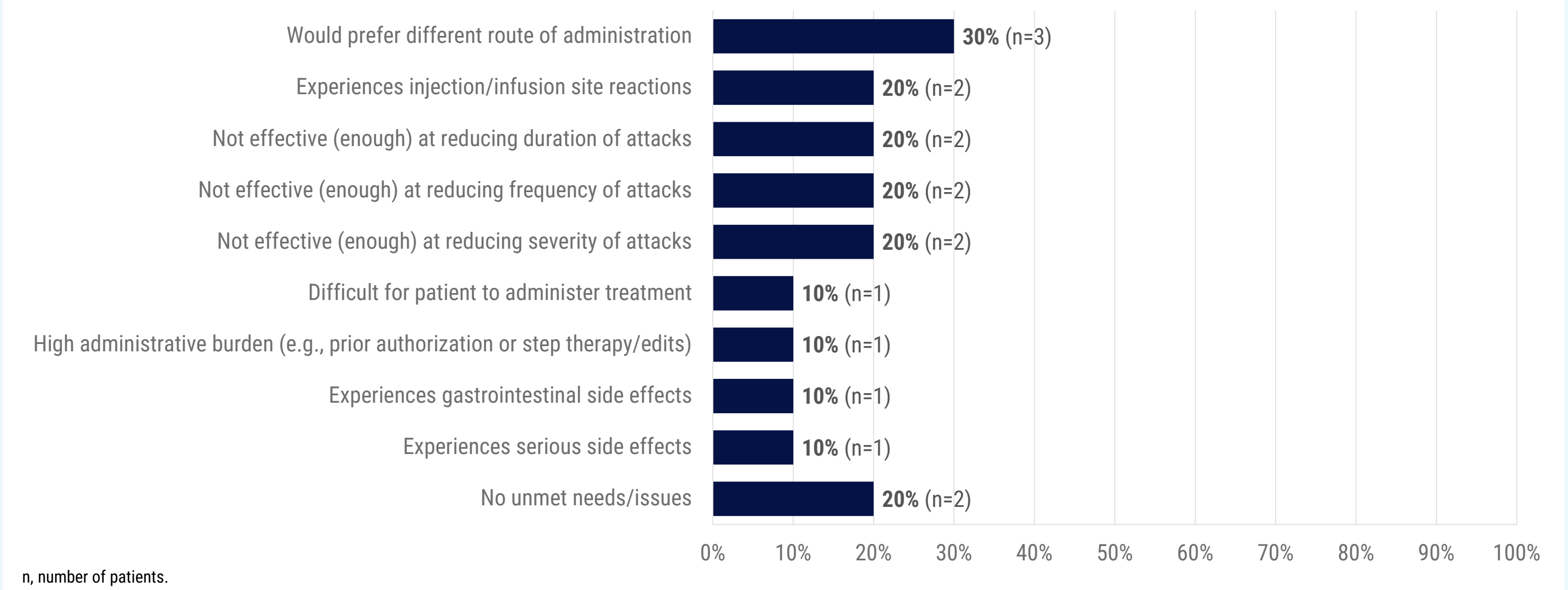
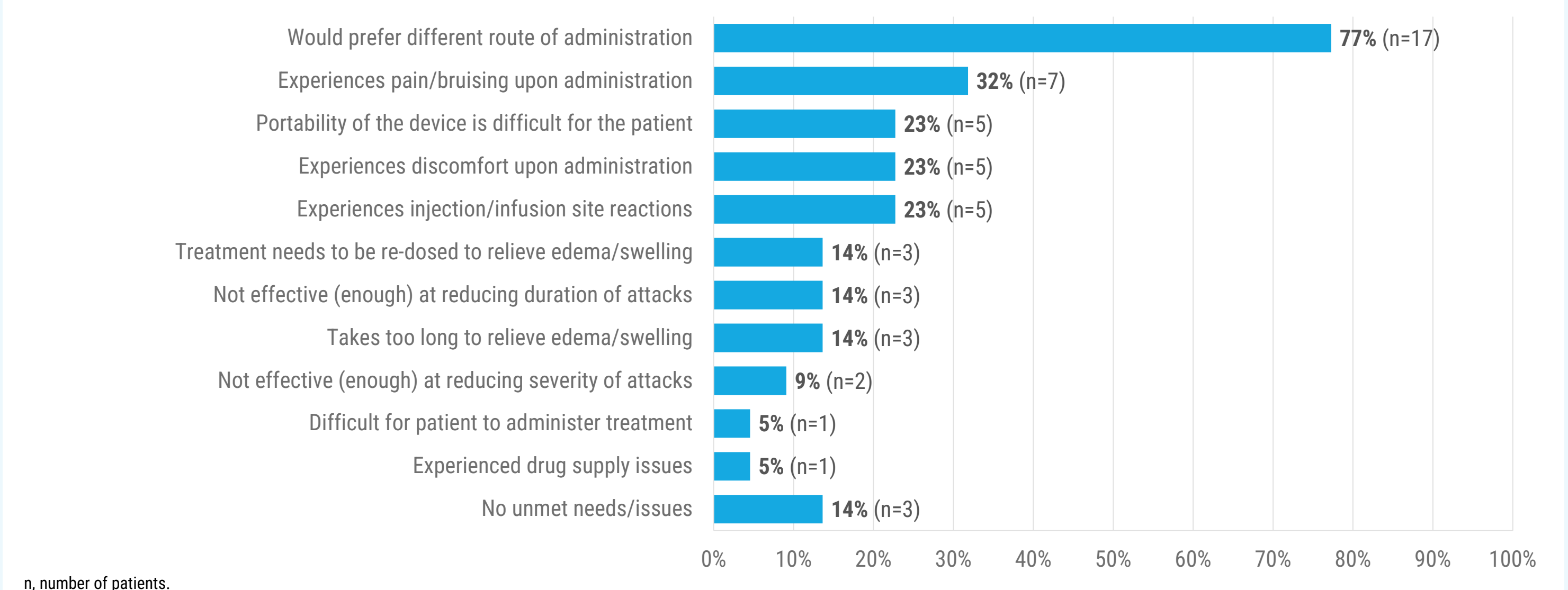


Figure 3. Unmet needs and other issues associated with current on-demand treatment (n=22)



- The most common reason for discontinuing previous LTP was that it was not effective enough at reducing attack severity (n=5) or frequency (n=4; Figure 4); all were oral medications.
- The most common reason for discontinuing previous ODT was supply issues (n=4; Figure 5).

Figure 4. Reasons for discontinuing previous long-term prophylactic treatment (n=9)

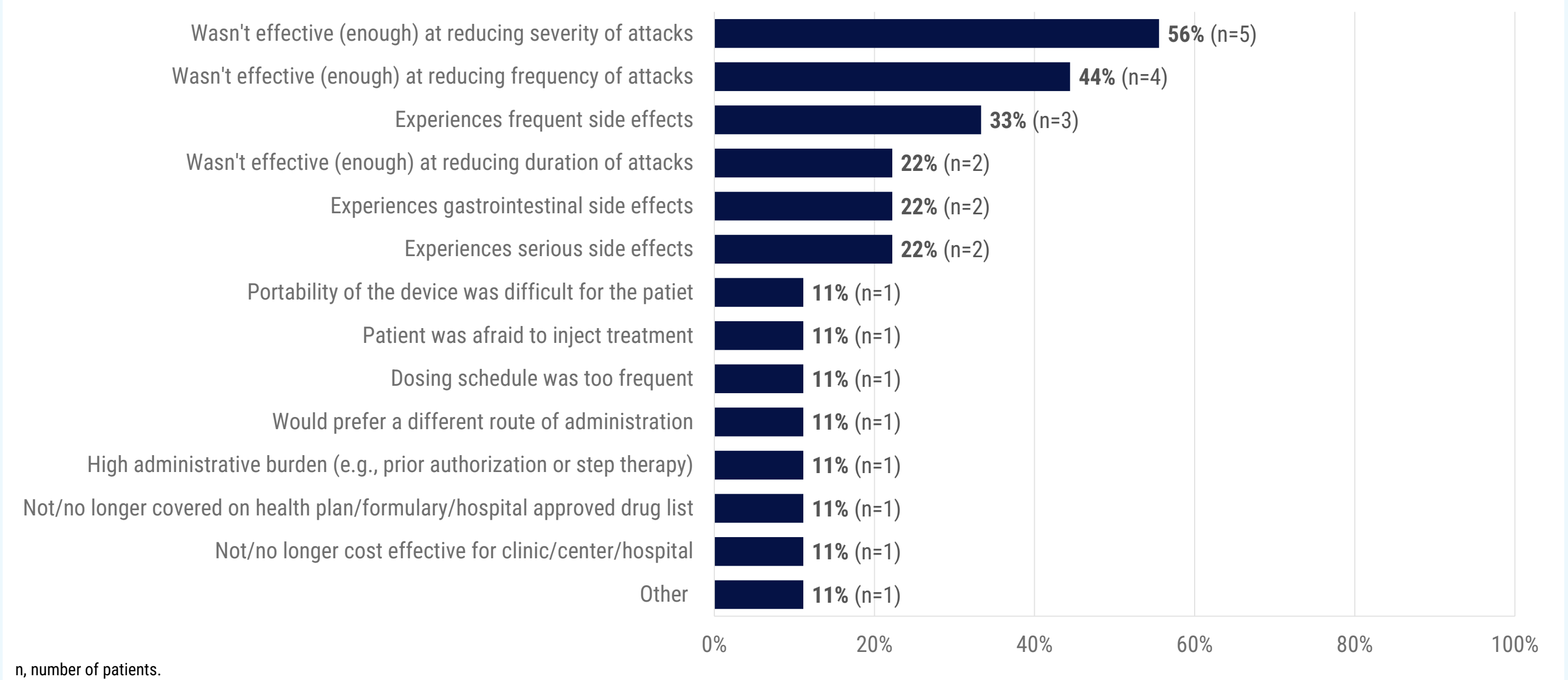
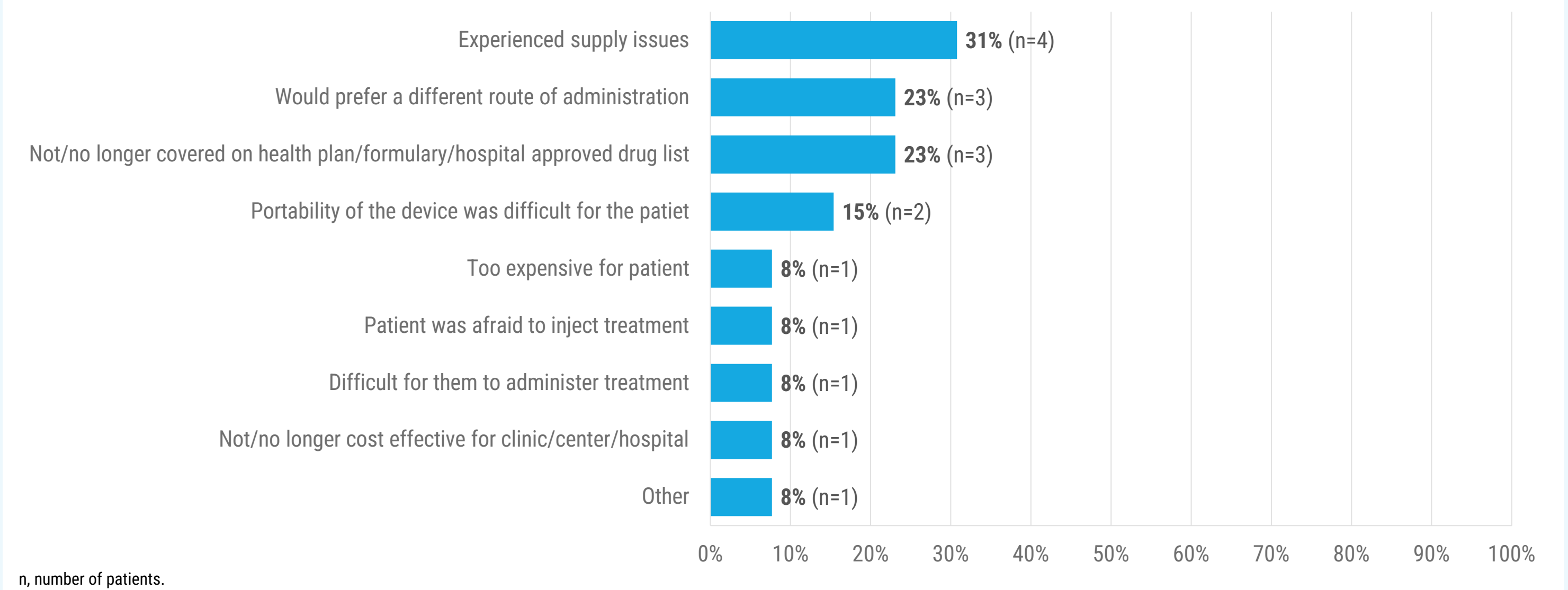


Figure 5. Reasons for discontinuing previous on-demand treatment (n=13)



Conclusions

- The most common unmet need associated with currently prescribed LTP and ODT medications was a desire for a different route of administration.
- The most common reasons for discontinuing previously prescribed LTP and ODT medications were supply issues and a lack of effectiveness.
- This analysis highlights an unmet need for people with HAE in the United Kingdom for effective, convenient, well-tolerated oral LTP and ODT options.

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