

# On-Demand Treatments for Hereditary Angioedema and Healthcare Resource Utilization in Pediatric (2-11 Years) Patients: A US Claims Database Analysis

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## Background

- Approximately 50%-75% of patients with hereditary angioedema (HAE) experience their first attack by the age of 12.<sup>1,2</sup>
  - Earlier onset is associated with more severe course of disease and increased attack burden.<sup>3,4</sup>
- Children with HAE attacks may experience anxiety, social isolation, and academic disruption, and there is a multifaceted psychosocial impact on their caregivers.<sup>5-7</sup>
- In the US, intravenous (IV) plasma-derived C1 inhibitor (pdC1INH; Berinert) is the only FDA-approved on-demand treatment for children aged <12 years.<sup>8,9</sup>
- Real-world data report that pediatric patients in the US utilize emergency services and require hospitalization more frequently than adults.<sup>10</sup>
- Injectable HAE treatments may be particularly challenging to administer in pediatric patients due to needle phobia and anticipation of pain; the smaller vein diameter may also lead to difficulties in administering IV treatment and may lead to poor adherence to treatment guidelines.<sup>1,4</sup>
- There are limited real-world data on on-demand treatment patterns and associated healthcare resource utilization outcomes in pediatric patients

## Objective

- This retrospective longitudinal analysis characterized real-world utilization of on-demand treatments among pediatric patients with HAE in the US using a national administrative claims database

## Methods

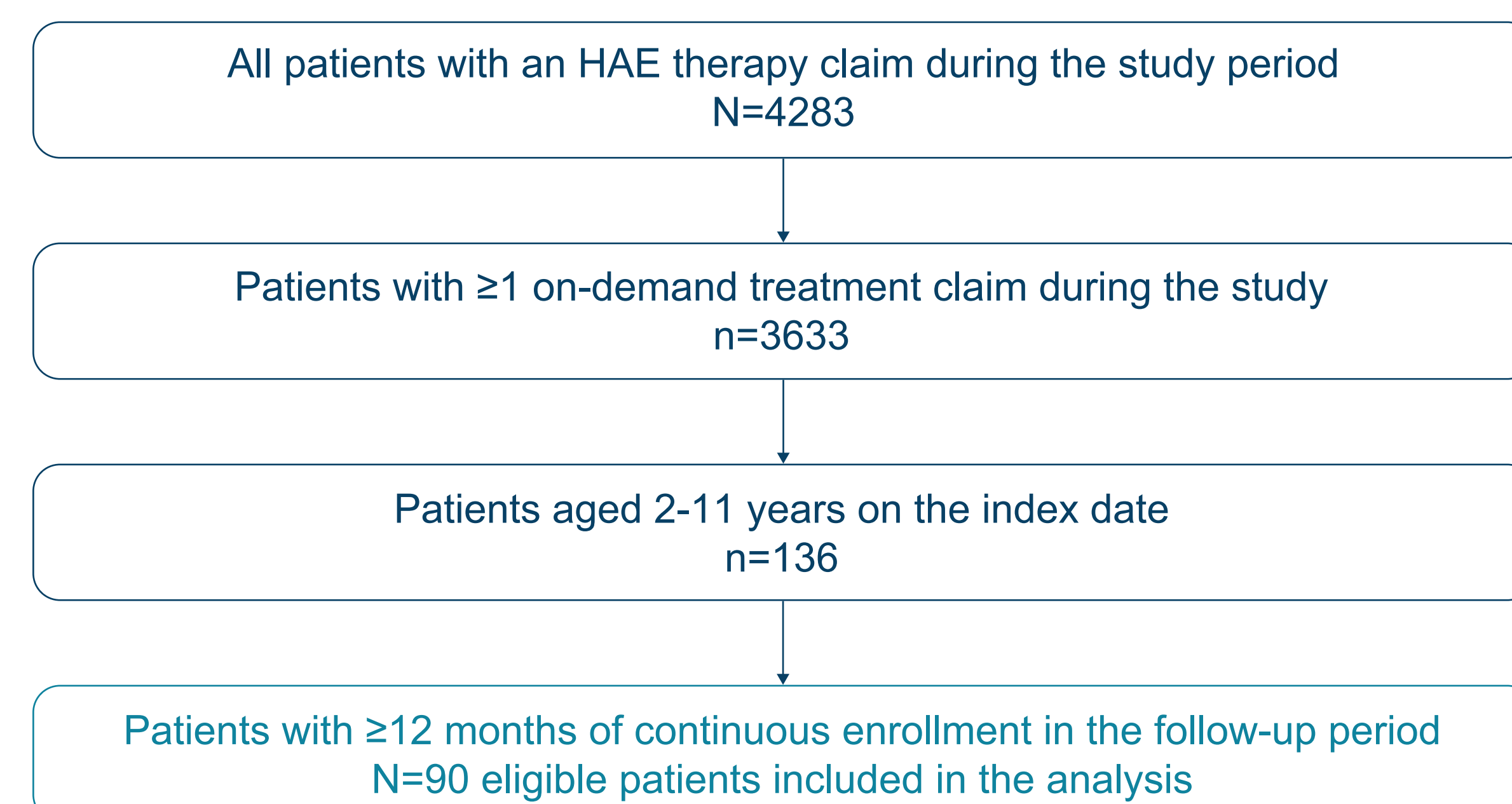
- Using the IQVIA PharMetrics® Plus Closed Health Plan Claims Database, this study analyzed US claims data from January 1, 2019, to December 31, 2024
  - Pediatric patients with HAE aged 2-11 years with ≥1 claim for an on-demand treatment and ≥12 months of continuous enrollment from the index date (the date of the first on-demand treatment claim) were included
- The mean number of doses of on-demand treatment with FDA-approved IV pdC1INH (Berinert) and off-label treatment with icatibant (Firazyr or generic), IV recombinant human C1 inhibitor (rhC1INH; Ruconest), or ecallantide (Kalbitor) was estimated across the 12-month follow-up period
  - A standardized on-demand treatment dose was defined as 2 pdC1INH vials, 1 icatibant syringe, 1 rhC1INH vial, or 3 ecallantide vials
- Outcomes assessed at baseline and follow-up included on-demand treatment doses, HAE-related healthcare utilization (emergency department, inpatient, home health, and outpatient), and location of on-demand treatment dispensing
- Statistical analyses included descriptive measures

## Limitations

- Claims data for on-demand treatment doses are a proxy measure and do not necessarily equal the number of attacks for which the on-demand treatment was administered
- It is also possible that some claims for treatment doses were utilized for reasons other than on-demand treatment of acute attacks (eg, for short-term prophylaxis)

## Results

**Figure 1. Pediatric Patient Attrition**



HAE, hereditary angioedema.

- A total of 90 pediatric patients aged 2-11 years were included in this analysis. Study population demographics are presented in **Table 1**

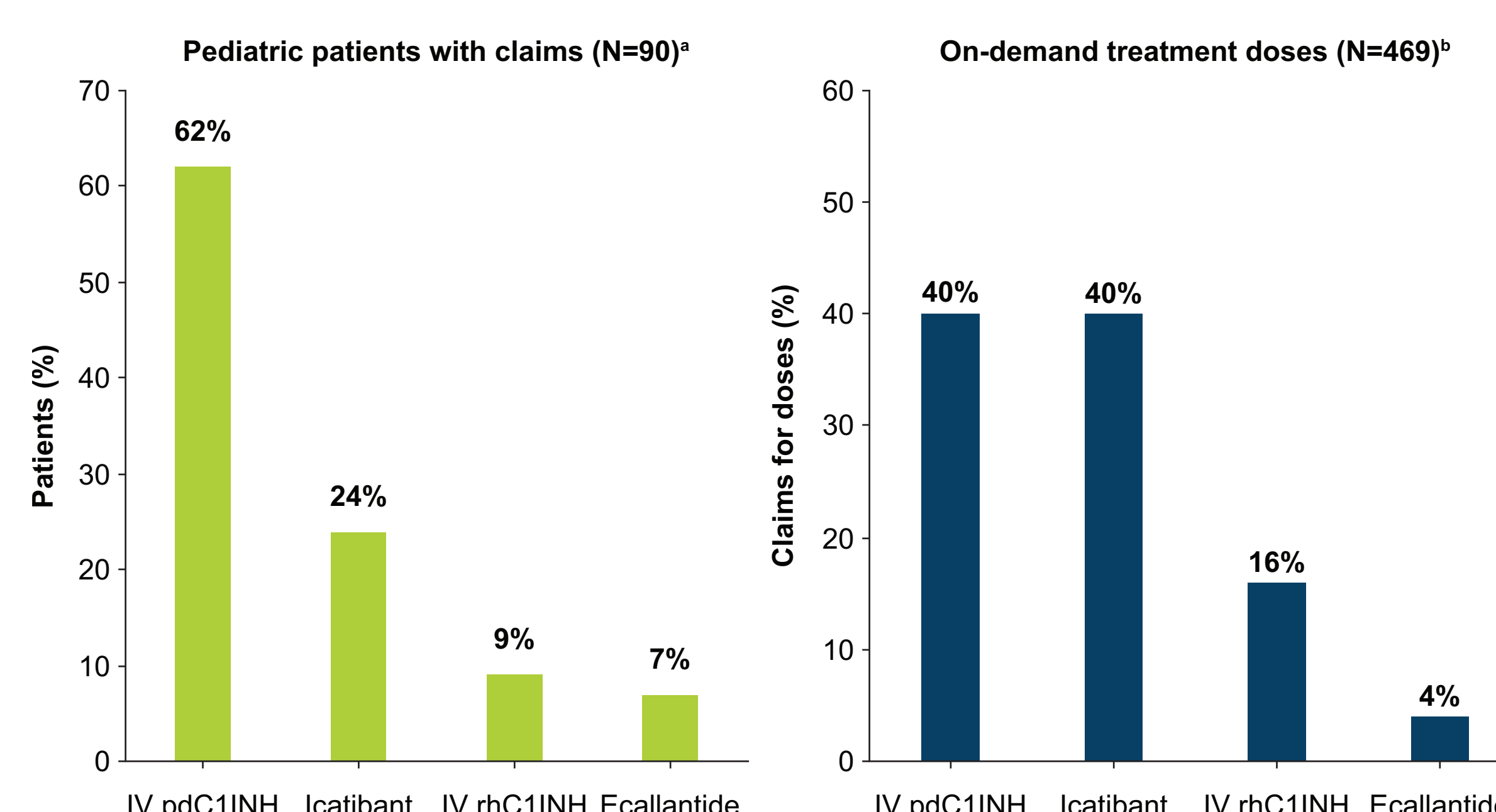
**Table 1. Study Sample Demographics**

|                          | Overall<br>N=90 |
|--------------------------|-----------------|
| Sex, male, n (%)         | 55 (61)         |
| Age at index date, years |                 |
| Mean (SD)                | 7.8 (2.5)       |
| Median (IQR)             | 8.5 (6-10)      |
| Region                   |                 |
| Northeast                | 17%             |
| South                    | 29%             |
| Midwest                  | 29%             |
| West                     | 26%             |

IQR, interquartile range; SD, standard deviation.

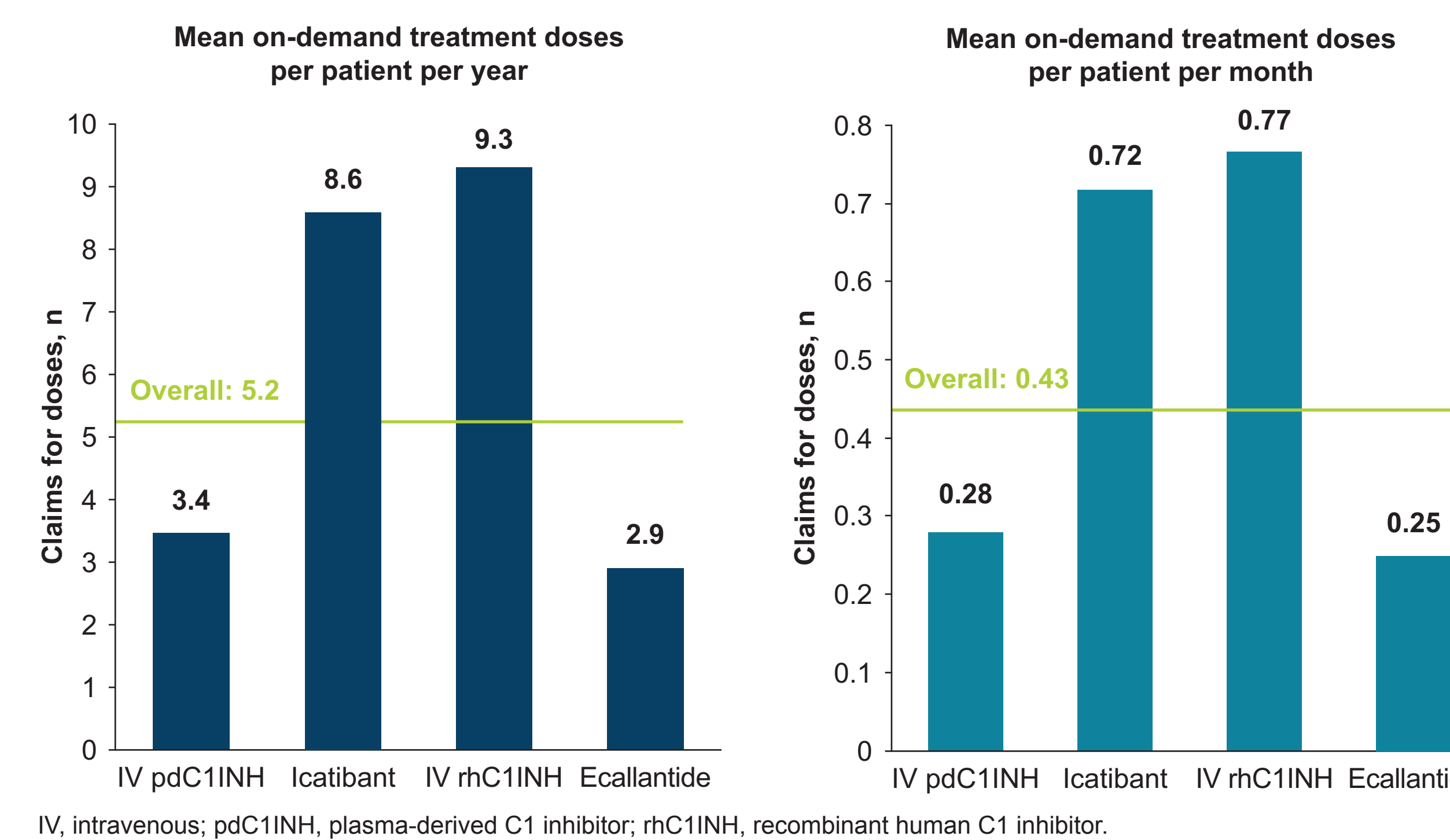
- When analyzed by patient, IV pdC1INH had the greatest number of claims, followed by icatibant. When analyzed by on-demand treatment dose claims, icatibant and IV pdC1INH each represented 40% of the total (**Figure 2**)
- Claims data for mean on-demand doses per patient suggest substantial variability: IV rhC1INH had the highest number of mean treatment doses per month (0.77), followed by icatibant (0.72), IV pdC1INH (0.28), and ecallantide (0.25; **Figure 3**)

**Figure 2. Pediatric Patients With Claims and Treatment Doses per Individual On-demand Therapy**

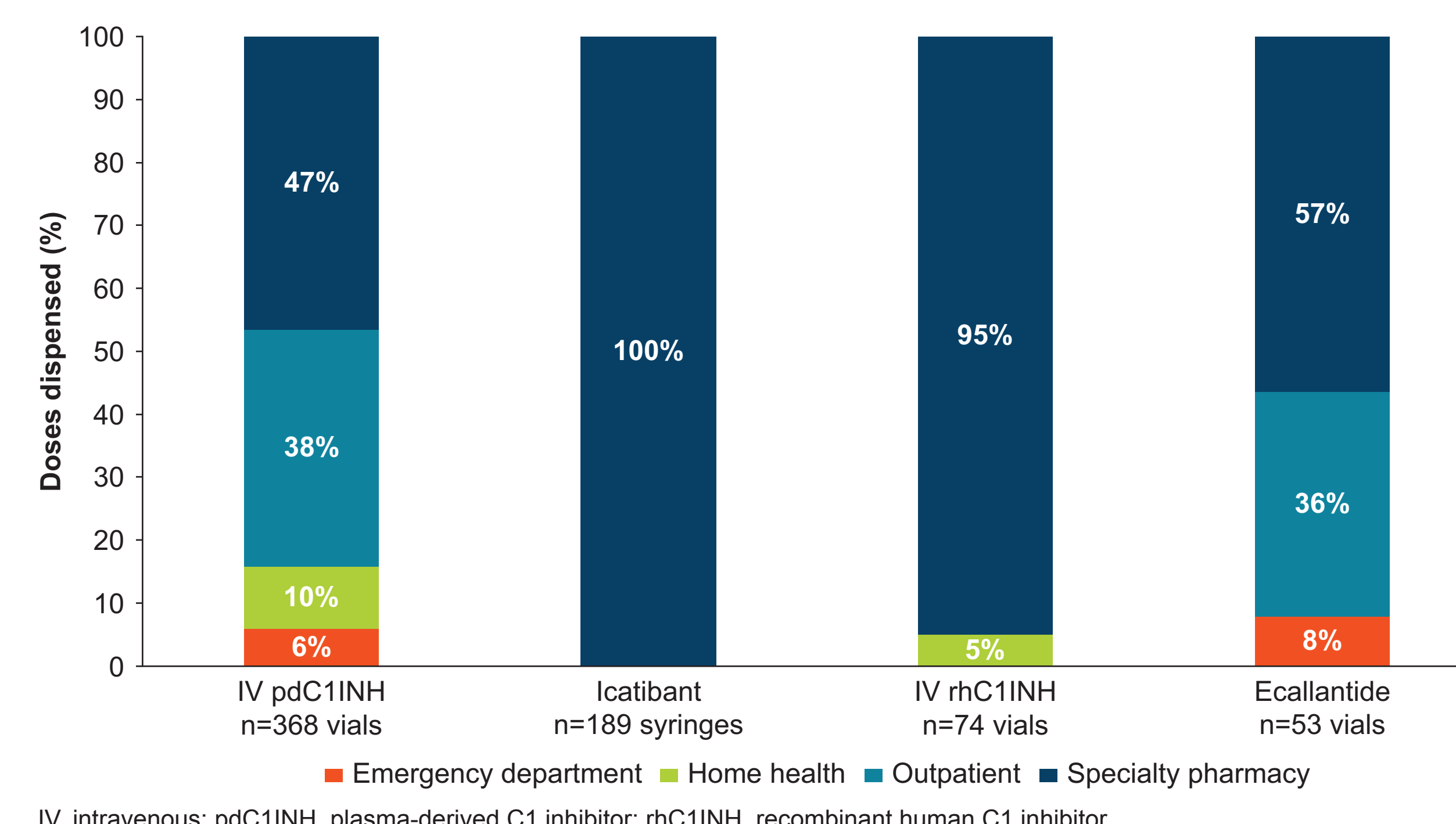


\*Two pediatric patients had claims for >1 on-demand treatment during follow-up: one had IV pdC1INH and ecallantide; the other had IV pdC1INH and icatibant.  
\*Patient share may total >100% as patients may have had multiple on-demand treatments.  
IV, intravenous; pdC1INH, plasma-derived C1 inhibitor; rhC1INH, recombinant human C1 inhibitor.

**Figure 3. Mean Claims for Doses per Patient**



**Figure 4. Location Where On-demand Treatment Was Dispensed**



- Over half of IV pdC1INH doses were dispensed in outpatient, home health, and emergency department settings (**Figure 4**)
- Almost half of patients (47%) utilized the emergency department, with the highest use in patients with claims for IV pdC1INH or ecallantide (**Table 2**)

**Table 2. HAE-Related Healthcare Visits**

|  | Overall<br>N=90 | IV pdC1INH<br>n=56 | Icatibant<br>n=22 | IV rhC1INH<br>n=8 | Ecallantide<br>n=6 |
|--|-----------------|--------------------|-------------------|-------------------|--------------------|
| Patients with ≥1 healthcare visit, n (%)     |                 |                    |                   |                   |                    |
| Emergency department                         | 42 (47)         | 30 (54)            | 5 (23)            | 4 (50)            | 4 (67)             |
| Inpatient                                    | 3 (3)           | 2 (4)              | 1 (5)             | 0                 | 0                  |
| Home health                                  | 15 (17)         | 14 (25)            | 0                 | 1 (13)            | 0                  |
| Outpatient                                   | 73 (81)         | 52 (93)            | 16 (73)           | 3 (38)            | 4 (67)             |
| Mean number of healthcare visits per year, n |                 |                    |                   |                   |                    |
| Emergency department                         | 3.2             | 3.7                | 1.6               | 1                 | 4.5                |
| Inpatient                                    | 1.3             | 1.5                | 1                 | 0                 | 0                  |
| Home health                                  | 2.3             | 2.4                | 0                 | 1                 | 0                  |
| Outpatient                                   | 4.1             | 4.2                | 3.1               | 2.6               | 7.2                |

IV, intravenous; pdC1INH, plasma-derived C1 inhibitor; rhC1INH, recombinant human C1 inhibitor

## Conclusions

- Even though most pediatric patients are being prescribed IV pdC1INH, icatibant is used off-label at the same rate as IV pdC1INH
- Although about two-thirds of patients were prescribed IV pdC1INH, overall utilization is lower than IV rhC1INH and icatibant, suggesting potential underutilization of IV pdC1INH in pediatric patients
- Overall, emergency department utilization among pediatric patients with HAE in the US was high, and was highest among those who used on-demand IV pdC1INH and ecallantide
  - Outpatient and home health visits were also high for those using IV pdC1INH
- Undertreatment with prescribed on-demand therapy, substantial off-label use of on-demand treatments, and high emergency department utilization in pediatric patients with HAE suggest that new, less invasive on-demand treatment options are needed

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## Disclosures

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