# Long-Term Prophylaxis Compliance and Healthcare Resource Utilization in Hereditary Angioedema: A Claims Database Analysis

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# Introduction

- The goals of treatment in hereditary angioedema (HAE) are to achieve full control of the disease and to normalize patients' lives. To achieve this, patients must have access to effective on-demand therapy, utilize short-term prophylaxis, and consider long-term prophylaxis (LTP) when appropriate<sup>1</sup>
- Most patients (~70%) with HAE in the United States (US) are treated with LTP therapies, primarily non-androgens, which require parenteral regimens or daily oral dosing<sup>2,3</sup>
- Frequent dosing schedule was reported as one of the primary issues/unmet needs associated with LTPs,4 which can contribute to adherence
- Multiple LTP therapies have become available in the past decade, however; patients with HAE still experience attacks requiring access to on-demand treatments and extensive medical care<sup>3,5</sup>
- There are limited real-world data on LTP refill patterns, on-demand treatment use, and associated healthcare resource utilization (HRU) and costs

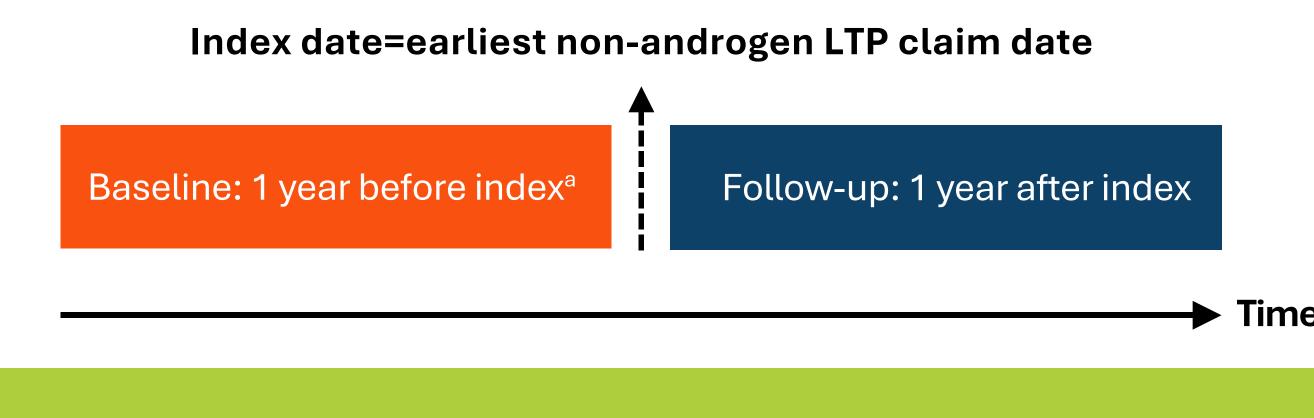
# Objective

To assess patterns in LTP refills and on-demand treatment, and associated HRU and costs in patients with HAE in the US using a national administrative claims database

## Methods

- Eligible commercially insured patients from the IQVIA PharMetrics® Plus Database (January 2016–September 2023) with ≥1 claim for non-androgen LTP, with ≥6 months of continuous enrollment before and ≥12 months after the index date (first non-androgen LTP claim) were included (**Figure 1**)
- Patients with multiple LTP claims on index date or with annualized claim amount more than mean ±3 times the standard deviation (SD; ie, outliers) were excluded

### Figure 1. Longitudinal retrospective study design



Continuous enrollment: 6 months prior and 12 months post index date

#### Outcomes (assessed at baseline and follow-up)

- HRU
- Healthcare costs
- Pharmacy On-demand doses Utilization of inpatient/outpatient/ Inpatient/outpatient/ER/ home health visits ER/home health visits
- <sup>a</sup>For patients with a baseline period <364 days, these data are annualized; for patients with a baseline period ≥364 days, the entire 12-month period is considered without annualization. ER, emergency room; HRU, healthcare resource utilization; LTP, long-term prophylaxis.
- Annualized mean on-demand therapy claims, inpatient visits, outpatient visits, ER visits, home health visits, and HAE-related healthcare costs were evaluated 1 year before and after the index date

Medical Officer of the Consortium of Independent Immunology Clinics (CIIC).

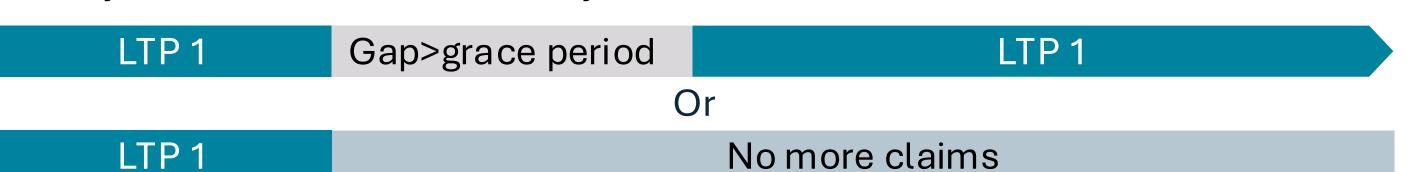
 Patients were classified into the following cohorts: no/minimal refill gaps, with refill gaps, or switchers (**Figure 2**)

### Figure 2. LTP patient cohort definitions

**No/minimal refill gaps:** Patients with no prescription gap >60 days for lanadelumab or >30 days for other LTPs



With refill gaps: Patients who discontinued their LTP or had ≥1 gap between refills >60 days for lanadelumab or >30 days for other LTPs



**Switchers:** Patients with ≥1 non-index LTP claim during the 12-month follow-up, regardless of gaps between treatments or whether patients return to index treatment



LTP 1 is the LTP at index date; LTP 2 is any non-index LTP. LTP, long-term prophylaxis

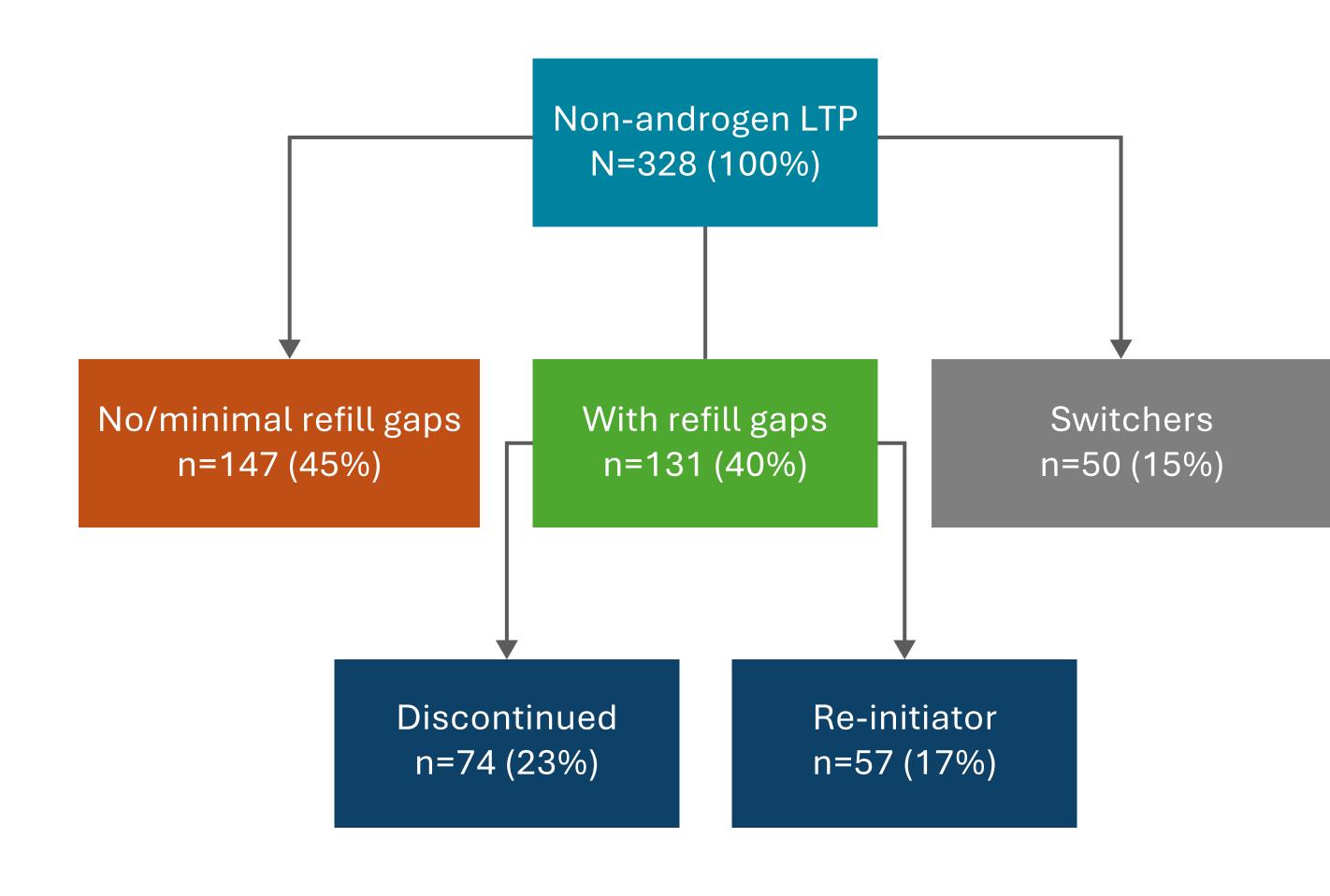
 Proportion of days covered (PDC) was calculated as the percentage of days covered by index LTP prescription fills during follow-up for both the cohorts with refill gaps and without (ie, no/minimal refill gaps). A high PDC percentage signifies good adherence to chronic treatment regimens, commonly accepted with a threshold of 80%<sup>6</sup>

# Results

 Data for 328 patients with HAE LTP prescriptions was analyzed; mean age was 41.2 years and 70% were female. Baseline demographics were similar across LTPs

 Mean PDC among those patients with no/minimal refill gaps was 93% compared with 42% among those with refill gaps

Figure 3. Patient cohort populations



LTP, long-term prophylaxis

- Mean (SD) annualized on-demand doses post-LTP decreased significantly for the no/minimal refill gap cohort (P=0.001), remained the same for the cohort with refill gaps (P=0.769), and increased in the switchers cohort (P=0.12) (**Table 1**)
- A reduction in on-demand doses was more likely among patients with no/minimal refill gaps than with refill gaps (odds ratio [95% CI]: 1.43 [1.24–1.65]) or those who had switched LTP therapies (odds ratio [95% CI]: 2.04 [1.60–2.60]) (**Table 1**)
- Despite initiating LTP, HAE-related HRU was still substantial (Table 2)
- Increases in total HAE-related healthcare costs were mainly driven by LTP pharmacy costs (**Table 3**)

Table 1. Summary of on-demand doses pre-and post-index LTP by LTP cohort

Number of on-demand doses per patient per year									
	Overall (N=328)		No/minimal refill gaps (n=147)		With refill gaps (n=131)		Switchers (n=50)		
	Baseline	Follow-up	Baseline	Follow-up	Baseline	Follow-up	Baseline	Follow-up	
All patients									
Mean ± SD	13.1 ± 21.5	11.8 ± 19.7	13.6 ± 22.5	8 ± 13.5	10.5 ± 17.4	11.5 ± 19.8	18.5 ± 26.8	23.9 ± 28.4	
Patients with ≥1 on-demand dose, n (%)	207 (63.1)	220 (67.1)	96 (65.3)	95 (64.6)	75 (57.3)	84 (64.1)	36 (72.0)	41 (82.0)	
Mean ± SD	20.8 ± 24.0	17.7 ± 21.8	20.8 ± 25.1	12.4 ± 15.2	18.3 ± 19.7	18.0 ± 22.3	25.7 ± 28.7	29.2 ± 28.8	
SD, standard deviation.									

Table 2. Annualized HAE-related HRU by LTP patient cohort

	Overall No/minimal refill gaps		ıl refill gaps	With refill gaps		Switchers		
Parameter, all patients	Baseline (n=300)	Follow-up (n=328)	Baseline (n=138)	Follow-up (n=147)	Baseline (n=116)	Follow-up (n=131)	Baseline (n=46)	Follow-up (n=50)
ER visits								
% of patients with ≥1 visit	21%	17%	18%	12%	22%	17%	26%	30%
No. visits <sup>a</sup>	3.1	3.4	2.1	1.8	4.4	4.4	2.6	3.9
Inpatient visits								
% of patients with ≥1 visit	12%	8%	9%	5%	13%	9%	20%	10%
No. visits <sup>a</sup>	1.8	2.2	1.3	2.3	1.9	1.8	2.2	3.0
Length of stay, days Mean ± SD	3.1 ± 2.6	4.3 ± 4.8	2.3 ± 1.7	5.3 ± 5.5	4.2 ± 3.3	4 ± 5.1	2.4 ± 1.6	3.5 ± 3.3
Home health visits								
% of patients with ≥1 visit	3%	9%	1%	5%	4%	8%	7%	20%
No. visits <sup>a</sup>	9.9	15.8	20.3	31.6	4.9	6	11.5	15.6
Outpatient visits								
% of patients with ≥1 visit	88%	81%	88%	84%	86%	73%	89%	96%
No. visits <sup>a</sup>	3.2	3.9	2.9	3.3	3.7	3.7	3.3	5.8

<sup>a</sup>Mean number of visits per patient per year among patients with ≥1 visit. ER, emergency room; HAE, hereditary angioedema; IQR, interquartile range; LTP, long-term prophylaxis; SD, standard deviation.

#### Table 3. Annualized mean cost per patient by cost type and by LTP patient cohort

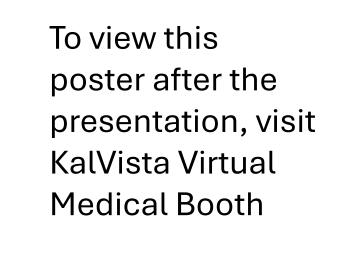
	Overall		No/minimal refill gaps		With refill gaps		Switchers	
Parameter, all patients	Baseline (n=300)	Follow-up (n=328)	Baseline (n=138)	Follow-up (n=147)	Baseline (n=116)	Follow-up (n=131)	Baseline (n=46)	Follow-up (n=50)
Medical costs								
ER/IP <sup>a</sup>	\$23,060	\$14,716	\$10,284	\$12,230	\$31,159	\$15,646	\$31,385	\$16,253
OP/HH/other <sup>a</sup>	\$2255	\$1668	\$808	\$934	\$4552	\$1869	\$970	\$3214
Pharmacy costs								
On-demand <sup>a</sup>	\$217,857	\$167,462	\$217,740	\$109,821	\$202,768	\$186,616	\$247,543	\$258,622
Other <sup>a</sup>	\$14,214	\$4193	\$26,935	\$653	\$4075	\$7928	\$2340	\$1327
LTP	\$0	\$395,845	\$0	\$524,191	\$0	\$219,900	\$0	\$479,487
Total healthcare costs	\$165,348	\$515,333	\$165,937	\$597,851	\$143,843	\$350,098	\$217,812	\$705,647
	\$165,348	\$515,333	•	,	·	•	·	

ER, emergency room; HH, home health; HRU, healthcare resource utilization; IP, inpatient; LTP, long-term prophylaxis; OP, outpatient.

# Conclusions

- This study found 55% of patients treated with LTP had substantial refill gaps in their LTP claims, discontinued, or switched within a year from initiation
- Substantial increases in total HAE-related healthcare costs were observed across patients with and without refill gaps and who switched LTP, driven by LTP pharmacy costs without significant reductions in healthcare resource utilization

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