Poster 739

Long-Term Prophylaxis for Hereditary Angioedema: Real-World Treatment Patterns in Selected US Allergy Clinics

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BACKGROUND

- Hereditary angioedema (HAE), a rare genetic disorder, causes recurrent, potentially life-threatening swelling episodes which can be spontaneous or triggered by stress, injury, or hormones.
- An important clinical management approach for HAE is long-term prophylactic (LTP) treatment.

OBJECTIVE

- To assess real-world treatment patterns among patients with HAE receiving LTP therapy.

STUDY DESIGN

- Data Source: The Patient Important Outcomes National Data Repository for Hereditary Angioedema (PIONEER-HAE) is a secure, de-identified, and HIPAA-compliant database housing patient data from the Consortium of Independent Immunology Clinics (CIIC).[1] This extensive network, spanning all 50 states and encompassing over 150 locations, serves nearly 2 million patients.
- Eligibility Criteria: Patients selected were with C1-esterase inhibitor deficient HAE (HAE-C1INH-Type1, HAE-C1INH-Type2), receiving LTP, and in care of CIIC between Apr 2021 to Mar 2024. Patients known to be a participant in a clinical trial or with a concurrent diagnosis of any other type of recurrent angioedema, including acquired or idiopathic angioedema, were excluded.
- Observation Period: For this study, the earliest initiation of prophylactic therapy, with at least 180 days history prior to initiation, served as the index date. Note that the full observation period for any patient was greater than 360 days, with >180 days prior to and following index.
- Study Population: Data for 816 patients were in PIONEER-HAE at time of study; data for 179 patients who met all eligibility criteria were used for this study.

RESULTS

TABLE 1: Population Characteristics

63% female, 71% white, 38 years (25-53) median (IQR) age at index, 86% HAE Type 1, 78% known family history of HAE, median (IQR) observation of 6.6 (5.4-9.2) years.

CHARACTERISTIC (n=179)	n (%)
FEMALE	113 (63%)
RACE	
BLACK	12 (7%)
OTHER	4 (2%)
UNKNOWN	36 (20%)
WHITE	127 (71%)
ETHNICITY	
HISPANIC OR LATINO	10 (6%)
NOT HISPANIC OR LATINO	113 (63%)
UNKNOWN	56 (31%)
AGE AT INDEX	
<18y	15 (8%)
18-49y	113 (63%)
50y+	51 (28%)
COMORBIDITIES	
ALLERGIES	93 (52%)
ANXIETY AND/OR DEPRESSION	48 (27%)
ASTHMA AND/OR COPD	36 (20%)
CANCER	4 (2%)
DIABETES	7 (4%)
GI DISORDERS	51 (28%)
HYPERLIPIDEMIA	24 (13%)
HYPERTENSION	36 (20%)
LIVER DISEASE	5 (3%)
RENAL DISEASE	1 (1%)
OTHER	89 (50%)
HAE TYPE	
HAE-C1INH-Type1	154 (86%)
HAE-C1INH-Type2	25 (14%)
FAMILY HISTORY OF HAE	
KNOWN FAMILY HISTORY, UNSPECIFIED LINEAGE	17 (9%)
MATERNAL	63 (35%)
PATERNAL	60 (34%)
NO KNOWN HISTORY	32 (18%)
UNKNOWN	7 (4%)
PATIENT OBSERVATION YEARS – MEDIAN (IQR)	6.6 (5.4-9.2)

FIGURE 1: Index LTP

55% kallikrein inhibitors, 37% C1-esterase inhibitors, 6% androgens, 2% anti-fibrinolytic

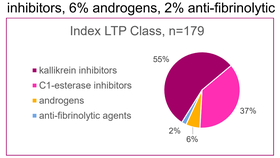


FIGURE 2: Index LTP Status

At time of analysis: 83 patients (46%) discontinued index LTP with median (IQR) duration of 12.1 (4.8-33.3) months.

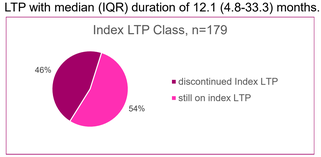


FIGURE 3: Index LTP Discontinuation Reasons

Reasons were available for 76/83 patients. More than one reason for discontinuation may have been recorded. The most common discontinuation reason was lack of efficacy.

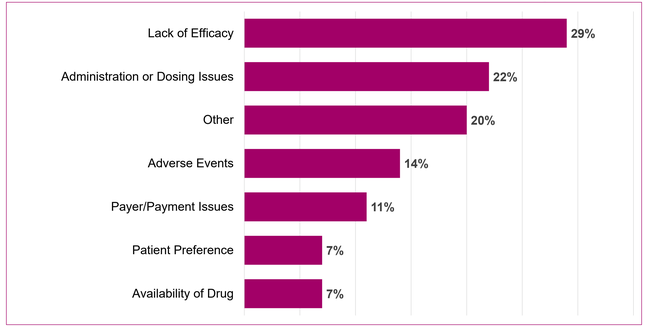
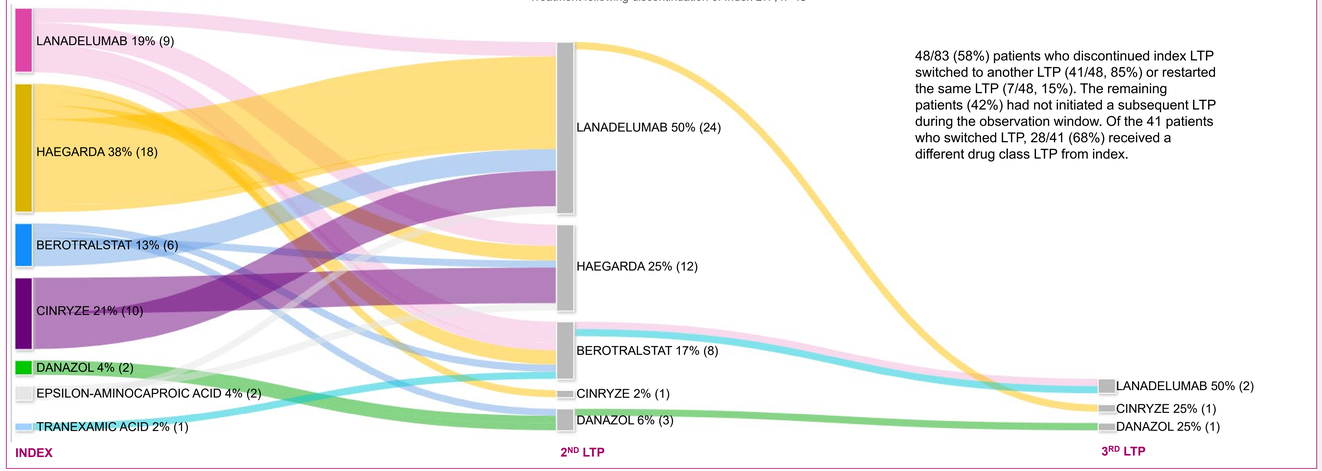


FIGURE 4: LTP Following Index Discontinuation

Treatment following discontinuation of Index LTP, n=48



48/83 (58%) patients who discontinued index LTP switched to another LTP (41/48, 85%) or restarted the same LTP (7/48, 15%). The remaining patients (42%) had not initiated a subsequent LTP during the observation window. Of the 41 patients who switched LTP, 28/41 (68%) received a different drug class LTP from index.

CONCLUSIONS

- This study demonstrated that 46% of patients discontinued index LTP for a variety of clinical and non-clinical factors with the most common being lack of efficacy.
- When patients switched to a different LTP, most switches led to a different drug class.
- Despite the availability of several LTP drug classes, over 40% of HAE patients continue to experience variable efficacy and treatment burden leading to changes in therapy.

DISCLOSURES

This study was funded by Ionis Pharmaceuticals (Carlsbad, CA, US). J Ruggles, CS, KV, and MM are employees and shareholders of Ionis Pharmaceuticals (Carlsbad, CA, US). HL and J Raasch served on the DSMB for the Ionis phase 2 and phase 3 trials. MR has received research support from Astria, Biocryst, Biomarin, CSL Behring, Ionis, Kalvista, Pharvaris, Takeda and was a paid consultant for Astria, Biocryst, Biomarin, Celldex, CSL Behring, Cycle Pharma, Grifols, Intellia, Kalvista, Pfizer, Pharming, Pharvaris, Sanofi-Regeneron, and Takeda. SM is an employee of Trio Health which has received research funding from Ionis and KalVista.

REFERENCE

1. W. Lumry et al. 2022. Annals of Allergy, Asthma & Immunology, 129 (5):S30

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