Real-World Persistence with and Adherence to Emicizumab Prophylaxis in Persons with Hemophilia A: A Secondary Claims Database Analysis

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Key takeaways
• Little is known about patterns of emicizumab use in the real-world setting
• The vast majority of individuals treated with emicizumab had high rates of persistence with and adherence to treatment

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Disclosures
AM: employee of CHOC Children’s Hospital, and has received speaker fees from Spark Therapeutics, Alexion, and Genentech, Inc.; RK, CSM, IMA, ET, and KR: employees of and holders of stocks/shares in Genentech, Inc. and holders of stocks/shares in F. Hoffmann-La Roche Ltd; RW: employee of and holder of stocks/shares in Genentech, Inc. and holder of stocks/shares in F. Hoffmann-La Roche Ltd, and previous employee of Acumen, LLC; LYL: employee of Genentech, Inc. and holder of stocks/shares in F. Hoffmann-La Roche Ltd.
Little is known about emicizumab persistence and adherence in the real-world setting

Background

- Preliminary evidence suggests that PwHA are more adherent to prophylaxis with emicizumab than FVIII or bypassing agents, but further evidence is needed*.
  - A single-center study involving 34 individuals taking emicizumab for a mean duration of 321 days found:
    - 82.0% were adherent to emicizumab, with a mean (SD) PDC of 92.4% (10.6)
    - Of those with dispensing records for FVIII (n=18), 44% were adherent to FVIII prophylaxis which increased to 72% when the same individuals switched to emicizumab

Definitions

PDC: the proportion of days covered by emicizumab during post-index persistence period (minimum 3 months)
The PDC threshold is the level above which the medication has a reasonable likelihood of achieving the most clinical benefit; clinical evidence provides support for a standard PDC threshold of 80%.

Adherence: individuals meeting the ≥80% PDC threshold were considered adherent.

Aim: To evaluate real-world persistence with and adherence to emicizumab prophylaxis in PwHA through analysis of insurance claims databases

* A PubMed search for ‘emicizumab real world adherence’ returned no results.
FVIII, factor VIII; PDC, proportion of days covered; PwHA, persons with hemophilia A; SD, standard deviation.
Emicizumab persistence and adherence data were collected from insurance claims databases

Study design

Commercial insurance claims databases

- IBM® MarketScan® Commercial Research
- IQVIA PharMetrics® Plus

Deletion of potential duplicate claims

Inclusion criteria

- Evidence of emicizumab use (≥2 prescription refills)
- Continuous enrolment for ≥3 months pre-emicizumab initiation

Data collected on emicizumab:

- Persistence
- Discontinuation
- Adherence

Follow-up† until end of study period or continuous enrollment

Follow-up‡ until end of study period or continuous enrollment

Pre-index period

Nov 16, 2017:* US approval of emicizumab for PwHA with FVIII inhibitors

Post-index persistence period‡

Index date†

Oct 4, 2018: US approval of emicizumab for PwHA without FVIII inhibitors

Dec 31, 2019*: US approval of emicizumab for PwHA without FVIII inhibitors

Definitions:
- Persistence—proportion of individuals continuing emicizumab prophylaxis during the study period.
- Discontinuation—60 days without a prescription refill; smaller lapses in adherence are not considered true discontinuation.
- Adherence—proportion of individuals meeting a threshold of ≥80% days covered by emicizumab.

*Emicizumab claims data were identified across these dates. †Date of first prescription of emicizumab. ‡Follow-up time varied.

FVIII, factor VIII; PwHA, persons with hemophilia A; US, United States of America.

References:
A total of 328 individuals initiating emicizumab were included in this retrospective study.

### Characteristic

<table>
<thead>
<tr>
<th>PwHA with evidence of emicizumab use (n=328)</th>
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<tbody>
<tr>
<td><strong>Male, n (%)</strong></td>
</tr>
<tr>
<td>328 (100)</td>
</tr>
<tr>
<td><strong>Mean age, years (SD)</strong></td>
</tr>
<tr>
<td>22.8 (16.4)</td>
</tr>
<tr>
<td><strong>Median age, years (range)</strong></td>
</tr>
<tr>
<td>19 (0–64)</td>
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<tr>
<td><strong>Mean follow-up post-index, days (SD)</strong></td>
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<tr>
<td>245 (147)</td>
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</tbody>
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### Age range (%)

- <5 years: 24.7%
- 6–17 years: 16.2%
- 18–34 years: 30.8%
- 35–64 years: 28.4%

### Region of US (%)

- East/Northeast: 43.6%
- Midwest: 23.8%
- South: 18.3%
- West: 14.3%

### Plan type (%)

- PPO: 39.9%
- POS: 3.4%
- Other: 56.7%

Note: Percentages may not add exactly to 100% due to rounding.

POS, point of service; PPO, preferred provider organization; PwHA, persons with hemophilia A; SD, standard deviation; US, United States of America.
The vast majority of individuals persisted with emicizumab prophylaxis during the study period.

328 individuals initiated emicizumab prophylaxis:

- **n=303 (92.4%)** Persistent through study period

- **n=25 (7.6%)** Discontinued emicizumab
  - Mean time to discontinuation (SD): 84 (124) days
  - n=10* (40.0%) Restarted emicizumab
  - Mean time to restart after discontinuation (SD): 126 (56) days

- **Mean persistence (SD): 240.7 (146.4) days**

**Definitions**

- **Persistence**: proportion of individuals continuing emicizumab prophylaxis during the study period
- **Discontinuation**: 60 days without a prescription refill; smaller lapses in adherence are not considered true discontinuation

The emicizumab discontinuation rate was low (n=25, 7.6%), with n=10 (40%) of those who discontinued, subsequently restarting treatment.

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*nOne person with hemophilia A restarted emicizumab twice.
PwHA, persons with hemophilia A; SD, standard deviation.

The majority of individuals were adherent to emicizumab during the post-index persistence period.

The majority of PwHA taking emicizumab met the ≥80% PDC threshold, suggesting they had sufficient adherence to have a reasonable likelihood of emicizumab achieving the expected effects.

*Those with at least 1-year post-index enrollment.

The PDC threshold is the level above which the medication has a reasonable likelihood of achieving the most clinical benefit; clinical evidence provides support for a standard PDC threshold of 80%. PDC, proportion of days covered; PwHA, persons with hemophilia A.

Conclusions

This is one of the first studies to report real-world persistence with and adherence to emicizumab prophylaxis in PwHA.

The vast majority of individuals treated with emicizumab had high rates of persistence to treatment; this was consistent throughout follow-up.

The mean PDC (81%) and proportion of adherent PwHA (≥80% PDC: 70%) observed in this study (n=328) are similar to those in a previous study of individuals from a single center (n=34)\(^1\).

High adherence to prophylaxis is associated with lower bleeding rates and better long-term joint health in PwHA\(^2\); future evaluations should examine the association of emicizumab persistence and adherence with health outcomes in this population.

PDC, proportion of days covered; PwHA, persons with hemophilia A.

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