

Poster No. 866

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

Arash Mahajerin,¹ Rahul Khairnar,² Craig S. Meyer,² Ibrahim M. Abbass,² Rongrong Wang,² Lucy Y. Lee,² Eunice Tzeng,² and Karina Raimundo²

¹CHOC Children's Hospital, Orange, CA, USA;

²Genentech, Inc., South San Francisco, CA, USA

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**,^{1,2} but further evidence is needed*
 - A single-center study¹ 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%³

Adherence: individuals meeting the $\geq 80\%$ PDC threshold were considered adherent³

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

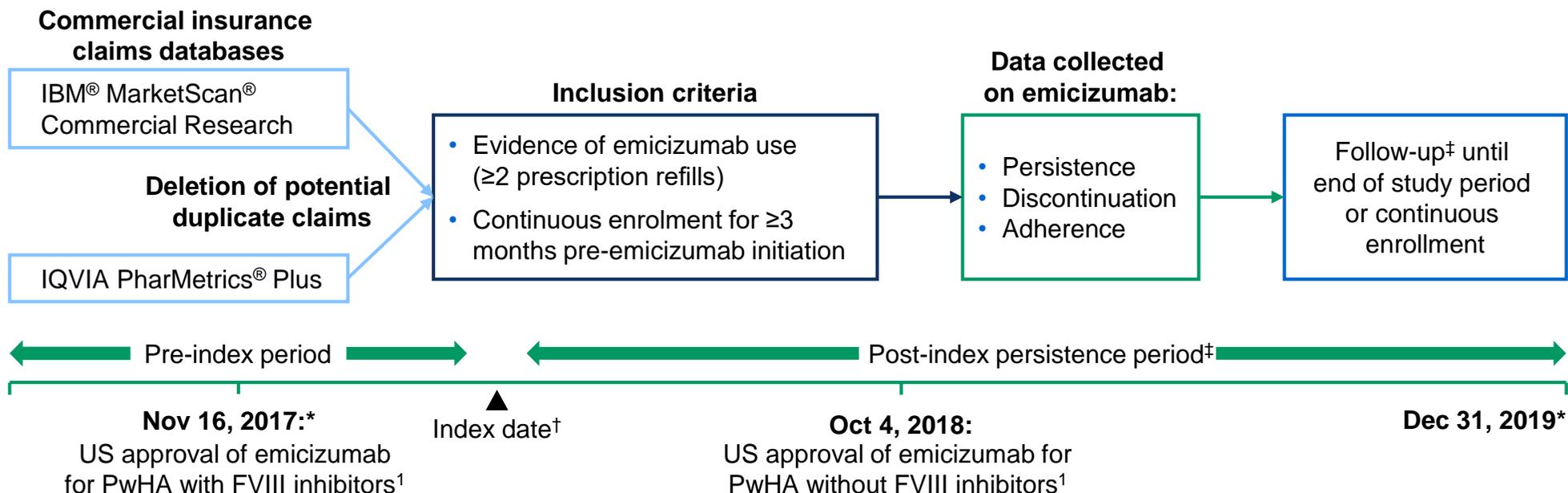


*A PubMed search for 'emicizumab real world adherence' returned no results.
FVIII, factor VIII; PDC, proportion of days covered; PwHA, persons with hemophilia A; SD, standard deviation.

1. Buckner TW, et al. Intl Soc Thromb Haemost (ISTH) Congress 2020 (PB0937). Accessed Oct 23, 2020;
2. Li A, et al. *Blood* 2019;134:2178; 3. Pharmacy Quality Alliance. PQA Adherence Measures.
<https://www.pqaalliance.org/adherence-measures>. Accessed Oct 19, 2020.

Emicizumab persistence and adherence data were collected from insurance claims databases

Study design



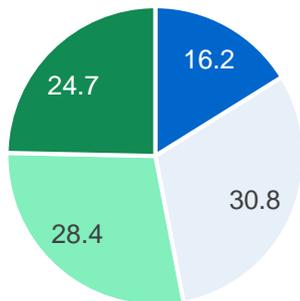
Definitions: *Persistence*—proportion of individuals continuing emicizumab prophylaxis during the study period; *Discontinuation*—60 days without a prescription refill;² smaller lapses in adherence are not considered true discontinuation. *Adherence*—proportion of individuals meeting a threshold of $\geq 80\%$ days covered by emicizumab.³
^{*}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.

1. US FDA. [FDA approves emicizumab](#). Accessed Oct 10, 2020;
2. Raebel MA, et al. *Med Care* 2013;51:S11–21.
3. Pharmacy Quality Alliance. PQA Adherence Measures. <https://www.pqaalliance.org/adherence-measures>. Accessed Oct 19, 2020.

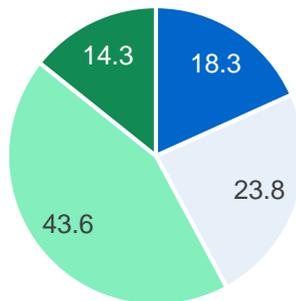
A total of 328 individuals initiating emicizumab were included in this retrospective study

 Characteristic	 PwHA with evidence of emicizumab use (n=328)
Male, n (%)	328 (100)
Mean age, years (SD)	22.8 (16.4)
Median age, years (range)	19 (0–64)
Mean follow-up post-index, days (SD)	245 (147)

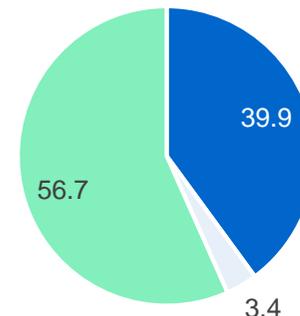
Age range (%)



Region of US (%)



Plan type (%)



■ <5 years ■ 6–17 years ■ 18–34 years ■ 35–64 years

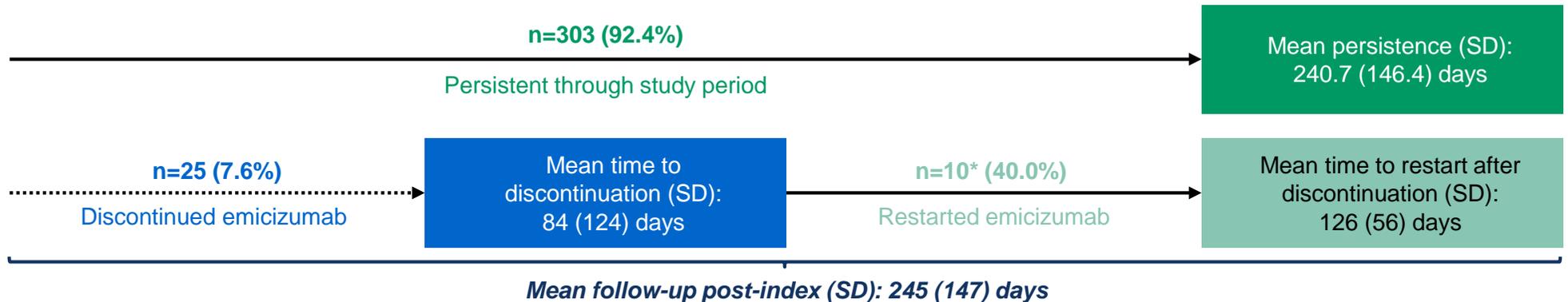
■ East/Northeast ■ Midwest ■ South ■ West

■ PPO ■ POS ■ Other

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:



Definitions

Persistence: proportion of individuals continuing emicizumab prophylaxis during the study period

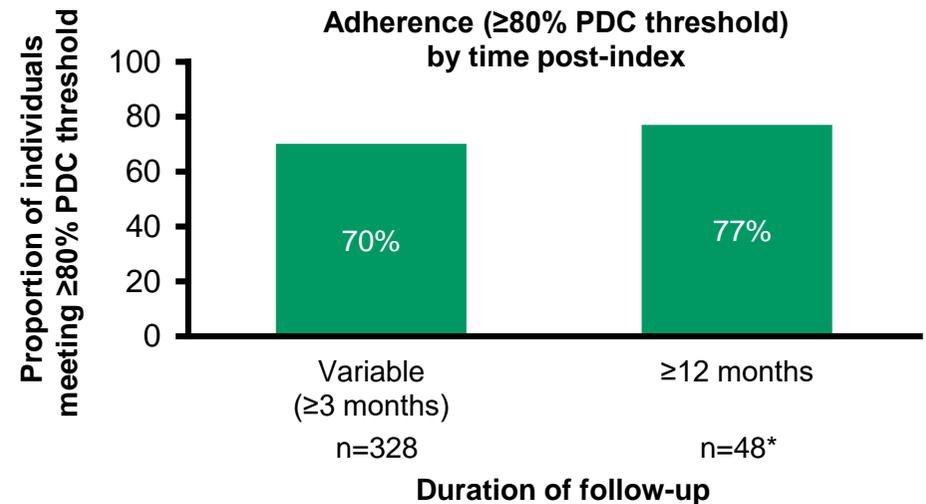
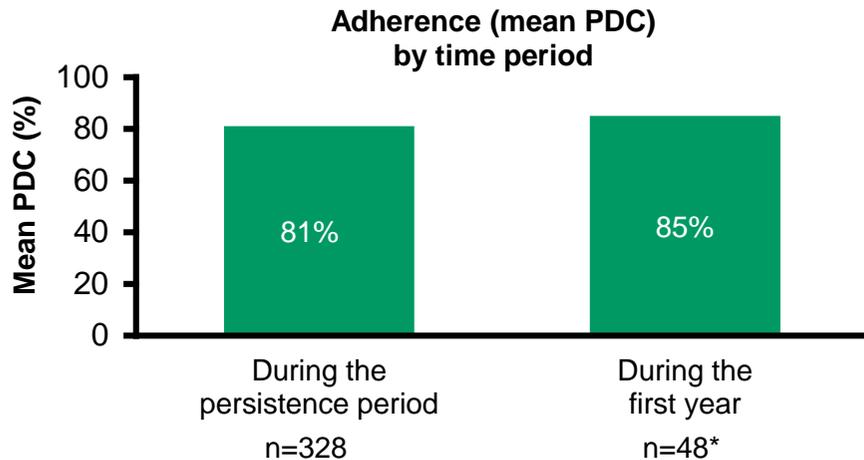
Discontinuation: 60 days without a prescription refill;¹ smaller lapses in adherence are not considered true discontinuation

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



*One 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 $\geq 80\%$ PDC threshold,¹ 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%.¹ PDC, proportion of days covered; PwHA, persons with hemophilia A.

1. Pharmacy Quality Alliance. PQA Adherence Measures. <https://www.pqaalliance.org/adherence-measures>. Accessed Oct 19, 2020.

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 ($\geq 80\%$ 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 PwHA²; 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.

1. Buckner TW, et al. ISTH 2020 (PB0937). Accessed Oct 23, 2020;

2. Dover S, et al. *Res Pract Thromb Haemost* 2020;4:318–25.



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