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Real-world bleeding outcomes and therapy exposure in national specialty pharmacy patients receiving emicizumab: a two-year retrospective analysis

Shubha Vaidya, MS, RPh, CSP, IgCP® | Alysha Robinson, PharmD, CSP

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Background

Emicizumab, approved in 2017, is the first subcutaneous prophylactic therapy for Hemophilia A, yet real-world bleeding outcomes and treatment exposure vary across patient populations.

Understanding how therapy duration, bleeding burden, and on-demand factor use relate is essential to optimizing patient management.

Objective

To evaluate real-world bleeding outcomes, annualized bleed rate (ABR) proxies, and therapy exposure among emicizumab-treated patients, by calendar year and bleeding status.

Methods

- Retrospective observational analysis of specialty pharmacy dispensing data, January 2024–December 2025.
- Patients with ≥1 emicizumab dispense were evaluated for therapy exposure (distinct calendar months across the 24-month window) and patient-reported bleeding events (frequency, concomitant factor use, bleed duration).
- Outcomes were summarized overall, by year (2024 vs 2025), and by bleeding status (bleeders vs non-bleeders).

Results

- 121 emicizumab patients were identified, with average emicizumab exposure of 12.1 months (StDev: 8.30).
- 59 patients (49%) reported ≥1 bleed; median bleed reports across all patients was 0, reflecting a substantial bleed-free population. Overall annual bleed rate per patient-year (ABR) = 1.12 (272 bleeds over 121 patients across two years).
- Bleeders had longer therapy exposure than emicizumab patients overall (17.1 vs 13.7 months average).

Conclusion

Most emicizumab-treated patients reported no bleeds. Although bleeders had longer therapy exposure and an annual bleed rate of 1.12 per patient-year, the link between time on therapy and bleeding burden was weak. Bleeding outcomes are heterogeneous in routine practice, underscoring the need for individualized assessment beyond duration of prophylaxis alone.

** Bleeding events were defined by documented patient-reported bleed since last delivery.*
** Therapy exposure was measured as months of emicizumab delivered.*
** ABR proxy = total bleeds divided by therapy exposure in years.*

Table 1. Patient bleed reports by year

	Overall (2024-2025)	2024	2025
Total patient count	121	88	90
Patients with ≥ 1 bleed	59	43	34
Bleeds reported	272	129	143
Annual bleed rate per patient-year	1.12	1.47	1.59
Percent of patients with bleed	49%	49%	38%

Figure 1. Emicizumab-exposed patients reporting bleeding during delivery scheduling 2024-2025

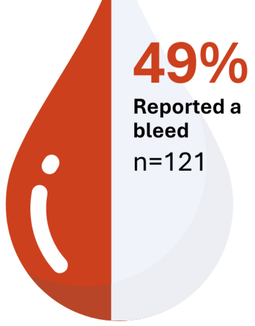


Figure 2. Annual bleed rate per patient-year overall (n=121) = 1.12

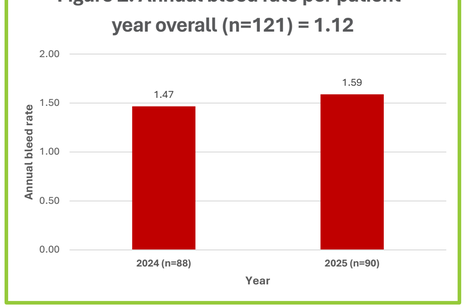


Figure 3. Concomitant therapies for emicizumab patients. (n=86) 2024-2025

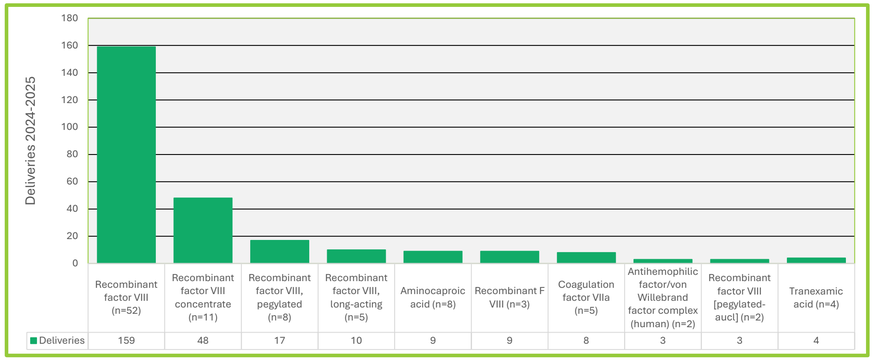
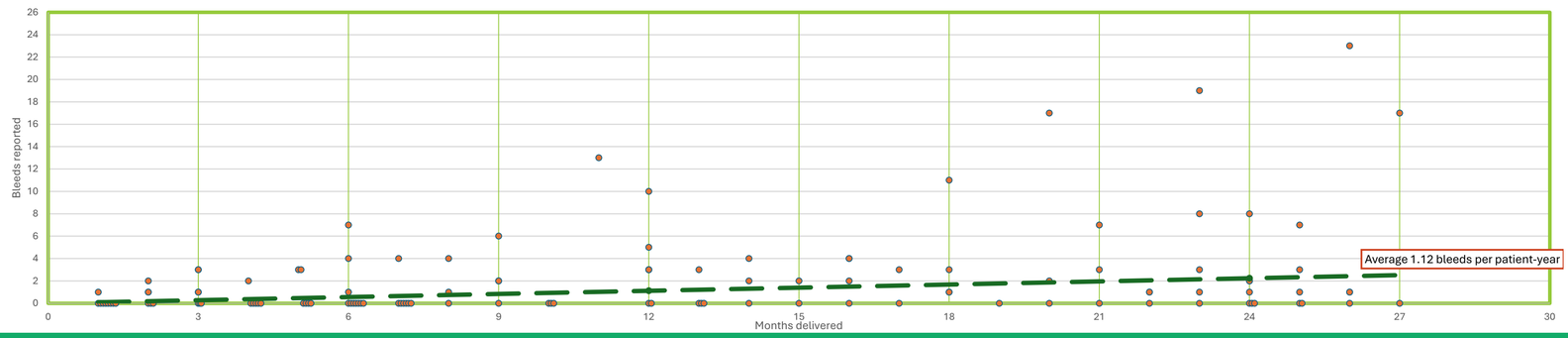


Table 2. Therapy exposure to emicizumab and concomitant therapies

	Emicizumab patients			Emicizumab patients with bleed		
	Emicizumab +/- other concomitant therapies	Emicizumab dispenses	Other concomitant therapies	Emicizumab +/- other concomitant therapies	Emicizumab dispenses	Other concomitant therapies
Unique patients	121	121	86	59	53	15
Average months of therapy delivered	13.69	12.05	3.29	17.10	15.40	2.87
StDev	9.30	8.30	3.60	9.28	7.98	1.54

Figure 4. Distribution of bleeding reports based on emicizumab exposure



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