Changes in Healthcare Resource Use and Costs after Emicizumab Initiation for Hemophilia A

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Summary

Hemophilia A is a rare, bleeding disorder in which blood does not clot normally, due to a deficiency in coagulation factor VIII[1]

Persons with Hemophilia A are prescribed prophylactic treatment, taken to prevent a bleed from occurring, or treatment to take on-demand when needed

Emicizumab is a bispecific, humanized monoclonal antibody that restores the function of missing activated FVIII, recently approved for routine prophylaxis in persons with hemophilia A

Real-world healthcare resource use and costs in patients initiating emicizumab are not well-documented

The results of this study show significant reduction in healthcare resource use with no increase in total healthcare costs

INTERACTIVE

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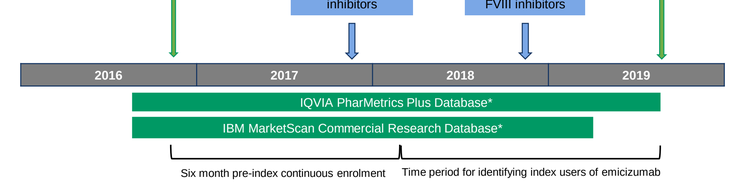
Background

- Hemophilia A (HA) is a rare, congenital disorder caused by deficiency in coagulation factor VIII (FVIII) activity and is characterized by spontaneous and traumatic bleeding.[1]
- Treatment has traditionally included FVIII replacement, either prescribed prophylactically to prevent bleed from occurring, or to take on demand to treat bleeds.
- Around 30% of persons with severe HA develop inhibitors against FVIII upon exposure to FVIII, making treatment ineffective and leaving these patients with few treatment options.[2]
- Emicizumab is a subcutaneously administered treatment for persons with HA (PwHA) that replaces the function of missing activated FVIII (FVIIIa); it was first approved in the United States (US) for prophylaxis in PwHa with FVIII inhibitors in 2017, and the label was extended to all PwHA in 2018.[3-5]
- Data on real-world effectiveness of emicizumab beyond bleed control are sparse.
- This study describes the changes in real-world healthcare resource use (HCRU) and costs in PwHA after initiating emicizumab prophylaxis.

Methods

- This retrospective study used pooled secondary claims data from IQVIA PharMetrics Plus and IBM MarketScan Commercial Research databases (See Figure 1 for study design).

Figure 1: Study Design and Timeline



Inclusion Criteria:

- **Patients with ≥1 emicizumab claim (identified using National Drug Code [NDC] or Healthcare Common Procedure Coding System [HCPCS] codes [Q9995]) between Nov 2017 and Aug 2019**
- **≥6 months of pre-index and ≥1 month post-index continuous enrolment was required for inclusion (index date was defined as the date of first prescription claim for emicizumab)**

- De-duplication was performed using age, index date, state, and region.
- NDC or HCPCS codes were used to identify use of emicizumab, FVIII and bypassing agents (BPA).
- Baseline patient demographics and pre-index clinical characteristics for the study cohort (major bleeds, arthropathy, pain and comorbidities) were summarised.
- Major bleeds were defined using an algorithm by Shrestha et al.[6;] Arthropathy and pain were identified using ICD-9-CM/ICD-10-CM diagnosis codes.
- Patients were followed until end of study period or continuous enrolment. Per patient per month (PPPM) HCRU and costs were calculated during the pre- and post-index period and the estimates were annualized.
- Mean annualized HCRU and costs were compared in the pre- and post-index periods using paired t-tests and Wilcoxon signed rank tests.

Results

- A total of 163 unique individuals who met the inclusion criteria were identified.
- All patients were male (100%); average age was 23 years (standard deviation [SD] ±17; median=19, range=1-64), with a mean Charlson Comorbidity Index (CCI) of 0.6 (SD±1.5).
- Median follow-up post emicizumab initiation was 5 months (interquartile range=3-8, range=1-20).
- In the pre-index period, 15% (n=25) had evidence of major bleeds, with an average of 3.2 bleeds (SD±1.7, range=2-8); 28% (n=46) had evidence of arthropathy or related disorders, 22% (n=36) had any pain diagnosis, and 7% (n=11) had evidence of inhibitors.
- After emicizumab initiation, there were fewer annualized inpatient hospitalizations (0.14 vs. 0.04, p=0.07) and significantly shorter lengths of inpatient hospitalizations (0.98 vs. 0.47 days, p<0.05) compared to the pre-index period (Figure 2).
- Individuals also experienced significantly fewer outpatient hospital visits (5.49 vs. 3.89, p=0.005) and physician office visits (9.08 vs. 5.67, p<0.001), while fewer emergency room visits were observed (0.65 vs.0.49, p=0.10) (Figure 2).
- Compared to the pre-index period, the mean annualized total cost of care was lower in the post-index period, but the difference was not statistically significant ($585,400±1,033,065 vs. $557,815±443,389, p=0.74) (Figure 3).

Figure 2: Mean Annualized Health Care Resource Use in Persons with Hemophilia A Before and After Emicizumab Initiation

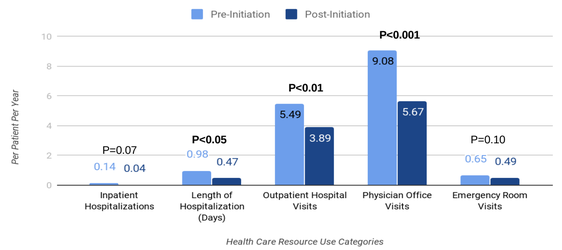
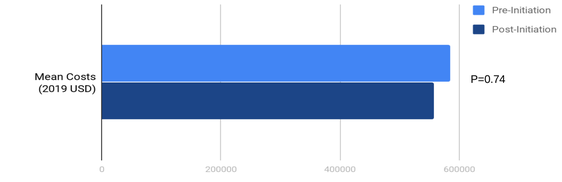


Figure 3: Mean Annualized Health Care Costs in Persons with Hemophilia A Before and After Emicizumab Initiation



Limitations

- This study relied on a relatively small sample size and did not examine hemophilia specific costs.
- The follow-up period was relatively short (median follow-up of 5 months).
- Annualized estimates were derived from variable follow-up data post-index.
- Pharmacy dispensing claims may not reflect patients' actual compliance to the medications.

Conclusions

- This is one of the first studies to evaluate HCRU and costs after initiating emicizumab prophylaxis based on early outcomes data.
- Shortly after initiation of emicizumab prophylaxis, individuals experienced reductions in various HCRU categories, without an increase in total costs of care.
- Longer follow-up data will help further examine these real-world outcomes.

Presented at the Academy of Managed Care Pharmacy (AMCP) Nexus 2020

References

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Disclosures

AM: Alexion, Genentech, Inc., Kedrion, and Spark; **RK, CSM, IA, LL, and KR:** Employment and shareholder of stock with F. Hoffmann-La Roche Ltd./Genentech, Inc.