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POSTER ABSTRACTS

901.HEALTH SERVICES AND QUALITY IMPROVEMENT - NON-MALIGNANT CONDITIONS

Cost-Effectiveness of Emicizumab Vs Efanesoctocog Alfa, Standard Half Life (SHL) and Other Extended Half Life (EHL) FVIII Products for Prophylaxis in People with Severe Hemophilia a without Inhibitors

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Background: Hemophilia A is a bleeding disorder characterized by a deficiency in factor VIII (FVIII). Routine prophylaxis with FVIII products or emicizumab is the recommended approach to management for people with hemophilia A (PwHA). The burden of lifelong prophylaxis treatments to prevent bleeding events for PwHA is substantial, impacting both the patients and the healthcare system. The objective of this study is to estimate the relative cost-effectiveness of these prophylactic therapies for PwHA, including the newly approved efanesoctocog alfa.
Methods: A Markov model was developed with four FVIII-based health states (normal clotting function, mild hemophilia, moderate hemophilia, severe hemophilia) and death to compare emicizumab, efanesoctocog alfa, standard half-life (SHL) and other extended half-life (EHL) FVIII products as prophylaxis for PwHA in the United States (US). Advate and Eloctate data were used to represent SHL and EHL, respectively. The model considered a cohort of 38 years old hemophilia A patients with average weight of 94.4 kg treated with prophylaxis. Probability of experiencing each health state was based on each product's pharmacokinetic data, where FVIII activity levels were used to determine disease severity. Other clinical inputs, including annualized bleed rates and adverse event risks, were estimated from clinical trial data. Wholesale acquisition cost (WAC) of drugs and healthcare costs of bleed management and adverse events were included and were estimated from published literature. Utilities by disease severity were derived from published real-world health-related quality of life studies for hemophilia patients. Disutility of infusion was obtained from the published literature. Costs (in 2023 US dollars), quality-adjusted life-year (QALY), and total bleeds were estimated over a lifetime horizon. Cost-effectiveness was estimated as incremental cost per QALY gained. One-way and probabilistic sensitivity analyses were conducted.
Results: Over a lifetime, emicizumab was estimated to be less costly (total cost at $17.0 million for emicizumab vs $24.0 million, $19.3 million, and $18.2 million for efanesoctocog alfa, SHL, and EHL) and more effective (18.61 vs 18.58, 16.91, and 17.33 QALY) compared with efanesoctocog alfa, SHL, and EHL FVIII, respectively. Although efanesoctocog led to fewer bleeds (26.58 vs 59.91 bleeds over a lifetime), PwHA on emicizumab were expected to incur fewer bleeds (59.91 vs 198.45, and 108.58 bleeds) compared with EHL and SHL. Results were robust to one-way and probabilistic sensitivity analyses.
Conclusions: Findings in this cost-effectiveness analysis suggest that emicizumab is less costly and more effective (i.e., dominant) over a lifetime compared with available FVIII prophylaxis in pediatrics and adult PwHA in the US.

Disclosures Curtis: *University of Southern California:* Consultancy; *Bayer AG and Novo Nordisk:* Membership on an entity's Board of Directors or advisory committees. **Decker-Palmer:** *Genentech, Inc. - A Member of the Roche Group:* Current Employment, Current equity holder in publicly-traded company. **Wilson:** *RTI Health Solutions:* Current Employment. **McDade:** *RTI Health Solutions:* Current Employment. **Lin:** *Genentech- Roche group:* Current Employment, Current equity holder in publicly-traded company. **Wallick:** *Genentech, Inc.:* Ended employment in the past 24 months. **Ko:** *Genentech, Inc.:* Current Employment, Current equity holder in publicly-traded company.

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Table. Cost-Effectiveness Analysis of Emicizumab vs FVIII products for Prophylaxis in Hemophilia A Patients: Base Case Results

	Emicizumab	Efanesoctocog Alfa	Standard Half-life FVIII (Advate)	Extended Half-life FVIII (Eloctate)
Costs				
Prophylaxis cost (drug)	$16,225,511	$23,694,719	$16,630,239	$16,811,002
Bleed management	$793,172	$351,970	$2,627,380	$1,437,623
Total	$17,019,384	$24,046,689	$19,257,715	$18,248,625
Outcomes				
Number of all bleeds	59.91	26.58	198.45	108.58
QALY	18.61	18.58	16.91	17.33
ICER (vs. emicizumab)	**N/A**	**Emicizumab dominant**	**Emicizumab dominant**	**Emicizumab dominant**

FVIII = factor VIII; ICER = incremental cost-effectiveness ratio; N/A = not applicable; QALY = quality-adjusted life-years.

Figure 1