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ORAL ABSTRACTS

904.OUTCOMES RESEARCH-NON-MALIGNANT CONDITIONS

Equitable Care for Severe Hemophilia_A: Distributional Cost-Effectiveness of Prophylactic Weekly Efanesoctocog Alfa Versus Standard-Care Factor VIII in Patients with Severe Hemophilia_A in the United States

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Introduction:
Prophylactic coagulation factor replacement is crucial to decrease bleeding episodes and improve quality-adjusted life expectancy (QALE) for patients with severe hemophilia A. Even with prophylactic treatment (i.e., at three to four times weekly) patients remain at risk of bleeding events and still develop chronic arthropathy across their lifetimes. The additional economic burden associated with lifelong prophylactic treatment is a major equity concern for patients with hemophilia A and their families. Efanesoctocog alfa is a recently developed extended half-life agent for hemophilia A prophylaxis and management, supporting the possibility of once-weekly prophylaxis. An open-label multicenter study (XTEND-1) showed that once-weekly dosing of efanesoctocog alfa improves the annualized bleeding rate (ABR) by 77% versus prophylaxis with standard-care factor VIII in patients with severe hemophilia A. We sought to evaluate, both for the first time, the conventional and distributional cost-effectiveness of prophylaxis with efanesoctocog alfa versus with standard-care factor VIII for patients with severe hemophilia A in the United States.
Methods:
For this independent analysis free of industry influence, we built a Markov model to examine the conventional cost-effectiveness of prophylaxis with efanesoctocog alfa versus standard-care factor VIII for patients with severe hemophilia A. Transition probabilities for ABRs and product utilization were informed by phase 3 study data and supplemented with FDA package insert information. Within ABRs, we accounted for treated bleeding severity and bleeding event types, inclusive of hemarthrosis, intracranial hemorrhage, in addition to assuming the prevalence of chronic arthropathy with SHL (i.e., due to silent bleed burden) is eliminated by efanesoctocog alfa. Costs for the base-case were sourced from the Centers for Medicare & Medicaid Services with negotiated (i.e., lower) cost of efanesoctocog alfa supplied from the Veterans Affairs Federal Supply Schedule Service (VA-FSS). Age-, sex- and disease-specific background mortality were employed. Utilities for severe hemophilia A and disutilities for bleed events and hospitalizations were informed by extensive literature specific to severe hemophilia A. The primary outcome was the incremental cost-effectiveness ratio (ICER) in USD/QALY, at a willingness-to-pay threshold of $150,000/quality-adjusted life-year (QALY). The secondary outcome was a threshold analysis for the maximum efanesoctocog alfa price that renders efanesoctocog alfa prophylaxis cost-effective. To account for health inequities in the care of patients with hemophilia A we then conducted a distributional cost-effectiveness analysis (DCEA) to derive an equity weight threshold (i.e., inequality aversion parameter) for the decision of prophylaxis with efanesoctocog alfa versus with standard-care factor VIII. This threshold was then compared to prior estimates for commonly used equity weights in the United States (range 0.5-3.0).
Results:
In the base-case, prophylaxis with efanesoctocog alfa versus with standard-care factor VIII accrued 19.4 and 15.4 QALYs at mean costs of $17.3 and $9.7 million. The ICER for efanesoctocog alfa was $1,890,000/QALY (95% credible interval 1,190,000-3,330,000). Threshold analysis showed that prophylaxis with efanesoctocog alfa became cost-effective when the price of efanesoctocog alfa decreased by at least 59%. In deterministic sensitivity analysis, the model was most sensitive to the costs of efanesoctocog alfa and factor VIII. In probabilistic sensitivity analysis, standard-care factor VIII was favored over efanesoc-

tocog alfa in 100% of 10,000 Monte Carlo simulations. The equity weight threshold for the treatment decision of prophylaxis with efanesoctocotog alfa versus with standard-care factor VIII was 7.4, higher than commonly used equity weights in the US. This implies a threshold that is higher than what has previously been used in the United States, per DCEA standards.
Conclusion:
As compared to standard-care factor VIII and at VA-FSS pricing, efanesoctocog alfa prophylaxis is conventionally cost-ineffective for patients with severe hemophilia A. Prophylaxis with efanesoctocog alfa does not meet commonly used distributive equity standards in the United States at this time.

Disclosures No relevant conflicts of interest to declare.

Table 1. Base-case and probabilistic sensitivity analysis for efanesoctocog alfa versus standard-care factor VIII prophylaxis. All point estimates rounded to maximum 3 significant digits. Legend: USD, United States dollar; QALY, quality-adjusted life-year; CI, credible interval

Prophylaxis	Cost (million USD)	Incremental Cost (million USD)	Effectiveness (QALYs)	Incremental Effectiveness (QALYs)	Threshold inequality aversion parameter
Standard-care factor VIII	9.70	--	15.4	--	
Efanesoctocog alfa	17.3	7.62	19.4	4.03	7.4
Incremental Cost-Effectiveness Ratio (ICER) =1,890,000 USD/QALY [95%CI: 1,190,000-3,330,000]					

Figure 1. Cost-effectiveness acceptability curve for efanesoctocog alfa versus standard-care factor VIII prophylaxis

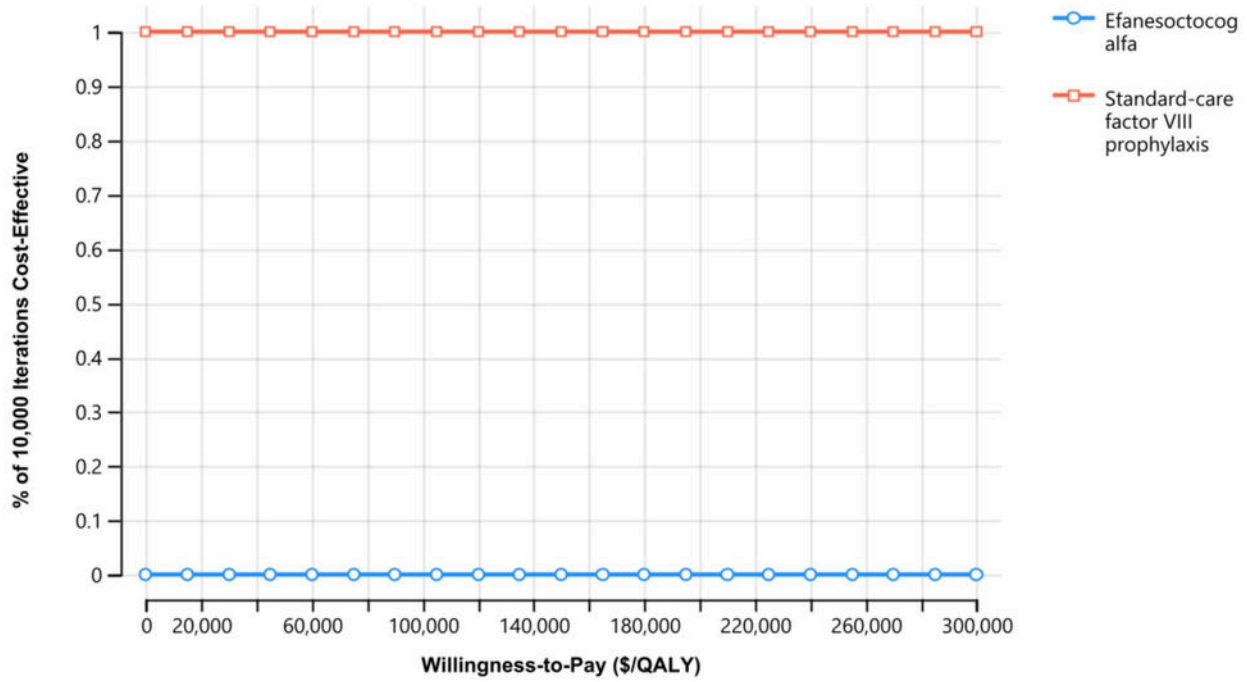

Figure 1

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