Budget Impact of Factor VIII Treatments for Hemophilia A in the United States

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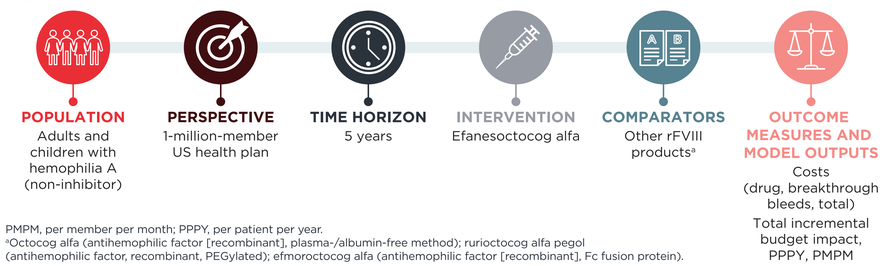
Introduction

- Hemophilia A is an inherited bleeding disorder characterized by partial/total deficiency of factor VIII (FVIII)[1]
- People with hemophilia A (PwHA) are highly susceptible to bleeding, commonly in joints and skeletal muscles,[1] which can result in irreversible damage if not stopped promptly[2]
- Prophylaxis with clotting factor concentrates or non-factor therapies is the recommended standard of care for people with severe hemophilia to prevent or reduce frequency of bleeding[3]
- US approval of efanesoctocog alfa (antihemophilic factor [recombinant] Fc-VWF-XTEN fusion protein-ehtl) in 2023 for the prophylactic treatment of PwHA[4] may impact the market share dynamics for the treatment of hemophilia A, with associated budget implications for payers
- **Objective of budget impact model:** Estimate the economic impact of efanesoctocog alfa market uptake for recombinant FVIII (rFVIII) prophylaxis in PwHA over 5 years based on a hypothetical 1-million-member US health plan

Methods

Figure 1. Model design overview



PMPM, per member per month; PPPY, per patient per year.
[a]Octocog alfa (antihemophilic factor [recombinant], plasma-/albumin-free method); rurioctocog alfa pegol (antihemophilic factor, recombinant, PEGylated); efmoroctocog alfa (antihemophilic factor [recombinant], Fc fusion protein).

- The model base case included PwHA without inhibitors (N=56; **Figure 2**)
- rFVIII product comparators
  - Octocog alfa
  - Rurioctocog alfa pegol
  - Efmoroctocog alfa
- Clinical and economic inputs included prophylaxis drug acquisition costs and management costs for breakthrough bleeds (moderate severity; **Table 1**)
  - Efanesoctocog alfa dosing based on the product label
  - Comparator rFVIII product dosing based on real-world evidence
  - Breakthrough bleeding rates during prophylaxis based on pivotal clinical trials
- Incremental drug acquisition costs for efanesoctocog alfa prophylaxis were estimated to exceed cost offsets realized by reduced annualized bleeding rate (ABR)
- Market shares were applied to estimate the number of PwHA treated with each regimen over 5 years
- Annual budget impact and incremental cost PPPY and PMPM were assessed and reported in 2022 US dollars

Figure 2. Population inputs

Hypothetical health plan population	1,000,000
Prevalence of hemophilia A[5] (estimated 12 cases per 100,000)	60
Incidence of hemophilia A (assumption of 1.1 per 1 million)	1.1
Portion of PwHA without inhibitors (~93%)[6] (literature estimate)	55
Eligible population	**56**

Assuming the hypothetical plan population is 50% male and 50% female. Eligible population for 2023 (55.8), rounded to nearest whole number.

Table 1. Clinical and economic inputs

	PROPHYLAXIS (ADULTS)		MODERATE BREAKTHROUGH BLEEDS (ADULTS)[b]			
	DOSE PER WEEK	YEARLY COST PER PATIENT[b]	DOSE PER WEEK	EVENT COST	MEDIAN ABR	YEARLY COST PER PATIENT
Efanesoctocog alfa	50.00 IU/kg[4]	**$769,723**	50.00 IU/kg[4]	$14,802	0[4]	**$0**
Efmoroctocog alfa	105.40 IU/kg[7]	**$698,543**	25.00 IU/kg[10]	$3186	1.6[10]	**$7647**
Rurioctocog alfa pegol	105.34 IU/kg[7,8]	**$643,855**	22.50 IU/kg[11]	$2645	1.9[11]	**$7538**
Octocog alfa	85.96 IU/kg[9]	**$369,865**	22.50 IU/kg[12]	$1862	1.0[12]	**$2793**

[a]Average sale price used to determine annualized acquisition cost. [b]All breakthrough bleeds were assumed to be treated with the same rFVIII product that a patient received for prophylaxis. Breakthrough bleeds were assumed to require 1.5 doses to resolve bleeding. IU, international unit.

Based on a hypothetical 1-million-member US private health plan, a 37% discount on efanesoctocog alfa would be required to achieve a neutral budget impact over 5 years for prophylaxis in adults vs other recombinant factor VIII products[a]

[a]Octocog alfa (antihemophilic factor [recombinant], plasma-/albumin-free method), rurioctocog alfa pegol (antihemophilic factor, recombinant, PEGylated), efmoroctocog alfa (antihemophilic factor [recombinant], Fc fusion protein).

Summary and Conclusions

- **Adding efanesoctocog alfa to a formulary to treat hemophilia A is associated with a substantial incremental budget impact on payers, totaling $7.5 million over a 5-year period based on a hypothetical plan size of 1 million**
- **The incremental drug acquisition costs for efanesoctocog alfa are estimated to far exceed the medical cost offsets that are associated with its reduced ABR when compared with other rFVIII products**

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Disclosures

Qi Fan, Ali G. Mokdad, Jorge Caicedo, and Mike Bullano are employees of Takeda Pharmaceuticals U.S.A., Inc. and holders of Takeda stock/stock options. John A. Carter is an employee of OPEN Health, a consulting firm that received fees for the conduct of this analysis.

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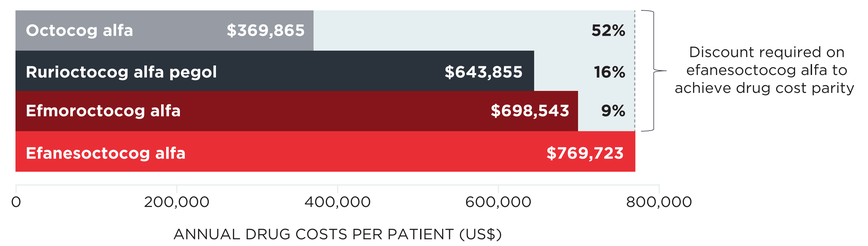
Results

Drug acquisition costs

- Annual drug acquisition costs for adult prophylaxis indicated that a 9–52% discount on efanesoctocog alfa would be required to achieve drug cost parity with other rFVIII products (**Figure 3**)

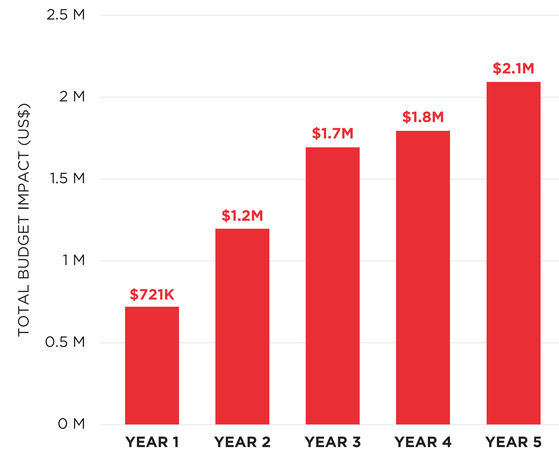
Figure 3. Prophylaxis (adults) drug acquisition costs



Incremental budget impact

- Estimated incremental cost PPPY and PMPM may increase by nearly three-fold over a 5-year period (**Figure 4**)
- Market share dynamics, drug acquisition costs, and real-world drug utilization were the main drivers of the incremental budget impact

Figure 4. Incremental budget impact of efanesoctocog alfa uptake over 5 years



	YEAR 1	YEAR 2	YEAR 3	YEAR 4	YEAR 5
IMPACT PPPY	+$12,919	+$21,333	+$28,584	+$30,553	+$34,105
IMPACT PMPM	+$0.06	+$0.10	+$0.14	+$0.15	+$0.17

BASE-CASE SCENARIO
Uptake of efanesoctocog alfa

5-year budget increase
+$7.5M
plan size of 1 million

37%
discount to efanesoctocog alfa acquisition cost required for neutral budget impact

Model assumptions and limitations

- Model assumes adult weight = 70 kg and adult population = 85%
- Model does not consider pharmacokinetics-based or individualized dosing, which may be associated with lower ABR
- The budget impact analysis is based primarily on drug acquisition costs and does not consider relatively smaller but potentially relevant sources of cost
- There are no head-to-head clinical studies of rFVIII products. Analysis is based on evaluating each product separately. Therefore, results are for illustrative purposes only and do not imply clinical outcomes or product safety or efficacy claims