CLINICAL RESEARCH

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Real-world treatment patterns and outcomes of switching to efanesoctocog alfa in children with haemophilia A: A single-centre experience from the United Arab Emirates

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ABSTRACT

Background:

Haemophilia A management has advanced significantly with the introduction of extended half-life (EHL) recombinant factor VIII (FVIII) therapies, notably efmoroctocog alfa.

Aims:

This study evaluated treatment patterns and outcomes of switching from efmoroctocog alfa to efanesoctocog alfa, a new ultra-long half-life FVIII therapy, for prophylaxis in children with haemophilia A in the United Arab Emirates (UAE).

Methods:

This retrospective, observational cohort study evaluated 22 male children with haemophilia A (median age = 9.0 years [IQR: 7.3–15.5]) transitioning from efmoroctocog alfa to efanesoctocog alfa prophylaxis at Tawam Hospital, UAE. All patients received efmoroctocog alfa prophylaxis during the pre-switch period (January 2023 to July 2024) before transitioning to efanesoctocog alfa in August 2024 and were followed up until March 2025 (observation period).

Results:

Twenty-one patients (95.5%) had severe disease. During the pre-switching period, half of the cohort experienced joint bleeding episodes; eight (36.4%) experienced one joint bleed, and three (13.6%) experienced two or more bleeds. The knee was the joint most frequently affected by bleeding (54.6%), followed by the elbow (18.2%). The median prophylactic dose of efanesoctocog alfa was 57.1 IU/kg (IQR: 50.0–63.9). Patients received efanesoctocog alfa for eight months. During the observation period, 100% of patients achieved a zero annualised bleeding rate (ABR), with no reported treatment-emergent adverse events or discontinuations. All patients demonstrated 100% adherence to the prescribed once weekly efanesoctocog alfa regimen. No active inhibitors or treatment-emergent adverse events were observed.

Conclusions:

Switching from efmoroctocog alfa to once weekly efanesoctocog alfa prophylaxis demonstrated highly effective bleeding prevention and a favourable safety profile in children with haemophilia A.

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PLAIN LANGUAGE SUMMARY

Haemophilia A is an inherited bleeding disorder in which the body does not have enough working factor VIII, a protein needed for normal blood clotting. Children with haemophilia A can have repeated bleeding, especially into joints such as the knees, elbows, and ankles. Over time, these bleeds can cause pain, joint damage, reduced movement, and difficulties with daily life, school, and physical activity. Regular preventive treatment is therefore used to reduce bleeding before it happens.

This study looked at children with haemophilia A treated at Tawam Hospital in the United Arab Emirates (UAE) who changed from one long-acting factor VIII treatment, efmoroctocog alfa, to a newer once-weekly treatment, efanesoctocog alfa. The research was needed because clinical trials have shown that efanesoctocog alfa can reduce bleeding, but real-world information from children in routine care, especially in the UAE, is still limited.

The study included 22 boys with haemophilia A. Most had severe haemophilia. Before switching treatment, half of the children had experienced joint bleeding, most often in the knee. After switching to once-weekly efanesoctocog alfa and being followed for eight months, all children had no recorded treated bleeds. No child developed an active inhibitor, no treatment-related side effects were recorded, and no child stopped treatment. All children also followed the prescribed once-weekly schedule.

These findings suggest that switching to efanesoctocog alfa may provide strong bleed protection with fewer injections for children with haemophilia A. This may be important for children and families because better prevention of bleeding can help protect joints, reduce treatment burden, and support more stable daily life. Larger studies with longer follow-up are still needed to confirm long-term safety and joint-health benefits.

Keywords:

Haemophilia A, Efanesoctocog alfa, Efmoroctocog alfa, Paediatrics, Real-world evidence, Annualised bleeding rate

INTRODUCTION

Haemophilia A is a rare, X-linked bleeding disorder resulting from a deficiency or dysfunction of clotting factor VIII (FVIII) [1,2]. This genetic defect leads to recurrent bleeding episodes, primarily into joints and muscles, which over time can cause progressive, irreversible haemophilic arthropathy characterised by chronic pain, reduced mobility, and significant disability [3,4,5]. In children, haemophilia A substantially impacts daily activities, school attendance, and overall quality of life [3].

Prophylactic FVIII replacement therapy represents the cornerstone of modern haemophilia A management, significantly reducing bleeding episodes and preventing or delaying joint damage, especially when initiated early [4,6]. The development and adoption of extended half-life (EHL) recombinant FVIII products, such as efmoroctocog alfa, using Fc fusion technology, have improved treatment convenience by reducing infusion frequency compared to standard half-life (SHL) products [7,8,9]. Clinical trials like A-LONG, Kids A-LONG, and their extension, ASPIRE, demonstrated efmoroctocog alfa's effectiveness in reducing annualised bleeding rates (ABRs) and improving joint health across age groups, particularly in children, with a favourable safety profile [9,10,11].

Despite the benefits of current EHL therapies, maintaining sufficient FVIII activity between doses can be challenging, especially for patients with rapid FVIII clearance, due to factor VIII's natural binding to von Willebrand Factor (VWF) [12]. Efanesoctocog alfa (ALTUVIIIO®, ALTUVOCT®) is an ultra-long half-life FVIII replacement therapy designed to decouple rFVIII from endogenous VWF and overcome the VWF-imposed half-life ceiling. It consists of a single rFVIII molecule fused to the Fc domain of human immunoglobulin G1, D'D3 domain of VWF (FVIII-binding domain), and two XTEN polypeptides. These components result in a 3–4-fold half-life extension over EHL and standard half-life (SHL) FVIII therapies, enabling weekly dosing with higher and more sustained FVIII activity, including trough levels near the normal range [12,13]. This has been shown to result in superior bleed protection, as evidenced by clinical studies, most notably the pivotal phase 3 trials XTEND-1 and XTEND-Kids [12,14-18]. Efanesoctocog alfa's potential advantages include reduced dosing frequency, better adherence, high-sustained FVIII activity, improved bleed prevention, and enhanced quality of life, which is particularly beneficial in children [12,19].

While clinical trials provide robust evidence, real-world data following treatment switches are essential to assess effectiveness and safety across diverse patient populations and healthcare settings [20]. Despite efanesoctocog alfa's promising profile in international trials, there remains a need for real-world evidence, especially regarding outcomes among children transitioning from established EHL products like efmoroctocog alfa, within specific regional contexts such as the United Arab Emirates (UAE) [21]. This retrospective study evaluated treatment patterns and outcomes of switching from efmoroctocog alfa to efanesoctocog alfa prophylaxis in children with haemophilia A in the UAE.

METHODS

Study design, study, and period

This single-centre, retrospective, observational cohort study was conducted at Tawam Hospital, UAE, using electronic medical records from January 2023 to October 2024. It evaluated the clinical outcomes and safety of children with haemophilia A who switched from long acting efmoroctocog alfa (Elocta®) to efanesoctocog alfa (ALTUVIIIO®) as part of routine care. The pre-switch period spanned from January 2023 to July 2024, during which all patients received efmoroctocog alfa. The index event, defined as the treatment switch, occurred in August 2024. Patients were then observed during the follow-up period from August 2024 to March 2025 while receiving efanesoctocog alfa.

The study was non-interventional and based solely on retrospective data. It adhered to the Declaration of Helsinki and received approval from the Tawam Hospital Institutional Review Board. Given the use of de-identified data and minimal patient risk, the requirement for written informed consent was waived. All data were managed in compliance with confidentiality and privacy regulations.

Patient population

The study population consisted of children with haemophilia A who had been receiving prophylactic treatment with efmoroctocog alfa and subsequently transitioned to prophylactic treatment with efanesoctocog alfa at the study centre during the specified period. Inclusion criteria required patients to be diagnosed with haemophilia A, be within the paediatric age range, have previously received efmoroctocog alfa, and have received at least one dose of efanesoctocog alfa. All included patients were male.

Data collection

Data were collected retrospectively from the electronic medical records of eligible patients. The variables collected included: demographic characteristics (e.g., age, weight, height, body mass index, ethnicity, nationality), baseline clinical characteristics (disease severity, history of inhibitors, history of major surgeries, venous access device status), treatment patterns (efanesoctocog alfa dose and frequency), bleeding episodes during the observation period post-switch (number and type of joint bleeds, intracranial haemorrhage, other bleeds), inhibitor status (history and current), and follow-up status.

Outcome measures

The primary outcome measure was the post-switch ABR during prophylaxis with efanesoctocog alfa. Efficacy was primarily assessed by the proportion of patients achieving a zero ABR. ABR was calculated for each patient as the total number of treated bleeds during the observation period/duration of the observation period in years. Secondary outcomes included the frequency and type of joint bleeding episodes, the occurrence of inhibitors, and dosing patterns of efanesoctocog alfa. Treatment adherence was assessed using infusion logs and pharmacy refill records. Patients were considered fully adherent if they received all scheduled doses throughout the observation period. Post-switch monitoring included clinical review, assessment of bleeding events, FVIII activity testing, and FVIII inhibitor screening according to the institutional haemophilia product-switching protocol. FVIII activity assays and inhibitor screening were performed during the post-switch observation period as part of routine clinical follow-up after product switching.

Statistical analysis

All statistical analyses were performed using Jamovi Software (version 2.6.23.0). Descriptive statistics were used to summarise the study population's demographic and clinical characteristics. Continuous variables were presented as means and standard deviations (SD) for normally distributed data and as medians with interquartile ranges (IQRs) for skewed data. Categorical variables were summarised using frequencies and percentages. Cohort-level ABR was summarised using both mean ± SD and median (IQR), and efficacy was also assessed by the proportion of patients achieving zero ABR. For comparisons between patient subgroups (specifically, those with versus without joint bleeding episodes during the observation period), the Mann-Whitney U test was used for continuous variables due to the small sample size and potential non-normal distribution, while Fisher's Exact Test was applied for categorical variables, particularly suitable for small cell counts. A two-sided p-value of <0.05 was considered statistically significant. No formal imputation methods were required as there were no missing data for the variables analysed in this cohort. Subgroup analysis focused on exploring associations between baseline characteristics and the occurrence of joint bleeding.

RESULTS

A total of 22 male children with haemophilia A who transitioned from prophylactic treatment with efmoroctocog alfa to efanesoctocog alfa were included. The median age of the cohort was 9.0 years (IQR: 7.3–15.5), with a mean (SD) age of 13.1 ± 11.0 years. All participants were male. The cohort represented diverse nationalities, the most frequent being Sudanese (18.2%), Jordanian (13.6%), Indian (13.6%), and Egyptian (13.6%). Ethnicity was predominantly Arab (45.5%) and Arab/African (36.4%). The median weight was 34 kg (IQR: 22.5–54.3), the median height was 135 cm (IQR: 120.3–163.5), and the median body mass index (BMI) was 18.0 kg/m² (IQR: 15.0–21.0; **Table 1**).

Disease characteristics and treatment patterns

Most patients had severe haemophilia A (n=21, 95.5%); one (4.5%) had moderate haemophilia A. Twenty patients (90.9%) had no history of major surgery. However, one had undergone a knee synovectomy (4.6%), and another had had multiple synovectomies (4.6%). Venous access was primarily achieved without an indwelling device (n=16, 72.7%). Five patients (22.7%) utilised a Port-a-Cath prior to switching, and one (4.6%) had a Port-a-Cath that was subsequently removed after switching.

Regarding bleeding episodes experienced during the pre-switching period, half of the cohort (n=11, 50%) reported joint bleeding episodes. Of these, eight patients experienced one episode (36.4% of the total cohort), and three experienced two or more episodes (13.6%). For those with reported joint bleeds (n=11), the knee was the most frequently affected joint (six occurrences, 54.6%), followed by the elbow (two occurrences, 18.2%), and the ankle, hip, and shoulder (each one occurrence, 9.1%). Intracranial haemorrhage was reported in the history of three patients (13.6%). Other bleeding events during the pre-switch period were reported in three patients (13.6%), including isolated muscle and joint bleeds, haematuria, and one case described as haemophilic arthropathy. A history of FVIII inhibitors was present in seven patients (31.8%), as shown in **Table 2**.

Before switching, all patients were receiving stable prophylaxis with efmoroctocog alfa for at least six months. Efmoroctocog alfa was prescribed twice weekly in 16 patients (69.6%), three times weekly in six patients (26.1%), and once weekly in one patient (4.3%). The median prescribed dose per infusion was 1,000 IU (IQR: 500–1,500), with a mean ± SD dose of 1,608.7 ± 1,514.7 IU and a range of 500–6,000 IU. The median total weekly dose was 2,000 IU/week (IQR: 1,250–3,750), with a mean ± SD weekly dose of 3891.3 ± 4335.3 IU/week and a range of 500–18,000 IU/week. The median weight-adjusted weekly prophylactic dose was 69.0 IU/kg/week (IQR: 52.8–113.2), with a mean ± SD of 86.8 ± 62.5 IU/kg/week and a range of 22.5–315.8 IU/kg/week.

Post-switch outcomes

All patients received efanesoctocog alfa as once-weekly intravenous prophylaxis. The median (IQR) dose administered was 57.1 IU/Kg (50.0–63.9), with a mean (SD) dose of 60.7 ± 16.3 IU/Kg. Following the transition to efanesoctocog alfa, all patients achieved a zero ABR during the observation period. The mean post-switch ABR was 0.0 ± 0.0, and the median post-switch ABR was 0.0 (IQR: 0.0–0.0). None of the 22 patients had active inhibitors during the observation period on efanesoctocog alfa. No treatment-emergent adverse events were reported in the medical records, and no patients discontinued efanesoctocog alfa prophylaxis during the study period.

Comparison of patients with and without joint bleeding pre-switching to efanesoctocog alfa

An exploratory analysis compared the demographic and clinical characteristics pre-switching to efanesoctocog alfa between patients who experienced any joint bleeding episode (n=11) and those who did not (n=11) (**Table 3**). Patients with joint bleeding were older (16.6 ± 12.8 years vs. 9.6 ± 8.0 years), had higher mean (SD) weight (49.6 ± 33.2 kg vs. 34.8 ± 23.3 kg), height (146.3 ± 24.0 cm vs. 128.8 ± 33.3 cm), and BMI (20.8 ± 8.0 kg/m² vs. 18.4 ± 3.6 kg/m²) compared to those without joint bleeding. However, these differences were not statistically significant (P=0.166 for age, P=0.324 for weight and height, P=1.00 for BMI).

TABLE 1. DEMOGRAPHIC AND BASELINE CHARACTERISTICS (N=22)

VARIABLES		VALUE
Age, years	Median (IQR)	9.0 (7.3–15.5)
	Mean ± SD	13.1 ± 11.0
Gender, n (%)	Male	22 (100%)
	Female	0 (0.0%)
Nationality, n (%)	Sudanese	4 (18.2%)
	Emirati	1 (4.6%)
	Jordanian	3 (13.6%)
	Indian	3 (13.6%)
	Syrian	2 (9.1%)
	Egyptian	3 (13.6%)
	Somali	2 (9.1%)
	South African	1 (4.6%)
	Lebanese	1 (4.6%)
	Comoran	2 (9.1%)
Ethnicity, n (%)	Arab/African	8 (36.4%)
	Arab	10 (45.5%)
	South Asian	3 (13.6%)
	White South African	1 (4.6%)
Weight, Kg	Median (IQR)	34 (22.5–54.3)
	Mean ± SD	42.2 ± 29.0
Height, cm	Median (IQR)	135 (120.3–163.5)
	Mean ± SD	137.6 ± 29.7
Body Mass Index, kg/m2	Median (IQR)	18 (15.0 – 21.0)
	Mean ± SD	19.6 ± 6.2

TABLE 2. PRE-SWITCH CLINICAL CHARACTERISTICS, BLEEDING PROFILE, AND TREATMENT DETAILS (N=22)

VARIABLES		N (%)
Disease severity	Moderate	1 (4.6%)
	Severe	21 (95.5%)
Pre-switch joint bleeding	0	11 (50.0%)
	1	8 (36.4%)
	≥2	3 (13.6%)
Type of joint*	Knee	6 (54.6%)
	Elbow	2 (18.2%)
	Ankle	1 (9.1%)
	Hip	1 (9.1%)
	Shoulder	1 (9.1%)
Intracranial haemorrhage	Yes	3 (13.6%)
	No	19 (86.4%)
Other bleeds	None	19 (86.4%)
	Muscle and other joints	1 (4.6%)
	Elbow, orbital, haematuria	1 (4.6%)
	Haemophilic arthropathy	1 (4.6%)
Major surgeries	None	20 (90.9%)
	Knee synovectomy	1 (4.6%)
	Multiple synovectomies	1 (4.6%)
Access device	None	16 (72.7%)
	Port-a-Cath	5 (22.7%)
	Port-a-Cath, then removed	1 (4.6%)
Inhibitory history	Yes	7 (31.8%)
	No	15 (68.2%)

*The percentage was calculated out of the 11 patients who reported joint bleeding

TABLE 3. COMPARISON OF CLINICAL AND DEMOGRAPHIC VARIABLES BETWEEN PATIENTS WITH AND WITHOUT JOINT BLEEDING PRE-SWITCHING TO EFANESOCTOCOG ALFA

VARIABLES				
Age, years, mean±SD		9.6 ± 8.0	16.6 ± 12.8	0.166**
Weight (kg), mean±SD		34.8 ± 23.3	49.6 ± 33.2	0.324**
Height (cm), mean±SD		128.8 ± 33.3	146.3 ± 24.0	0.324**
Body Mass Index (kg/m2), mean±SD		18.4 ±3.6	20.8 ± 8.0	1.00**
Ethnicity, n (%)	Arab/African	4 (36.4%)	4 (36.4%)	0.298*
	Arab	6 (54.6%)	4 (36.4%)	
	South Asian	0 (0.0%)	3 (27.3%)	
	White South African	1 (9.1%)	0 (0.0%)	
Severity, n (%)	Moderate	1 (9.1%)	0 (0.0%)	1.00*
	Severe	10 (90.9%)	11 (100%)	
Intracranial haemorrhage [Yes], n (%)		2 (18.2%)	1 (9.1%)	1.00*
Other bleeds [Yes], n (%)		0 (0.0%)	3 (27.3%)	0.214*
Major surgeries [Yes], n (%)		0 (0.0%)	2 (18.2%)	0.476*
Inhibitory history [Yes], n (%)		2 (18.2%)	5 (45.5%)	0.361*
Weekly dose of efanesoctocog alfa, IU/Kg, mean±SD		65.2 ± 21.4	56.1 ± 9.1	0.323*

*Fisher Exact Test; **Mann-Whitney U test

Ethnicity distribution showed a higher proportion of South Asian patients in the joint bleeding group (27.3% vs. 0.0%) and a lower proportion of Arab patients (36.4% vs. 54.6%), but the overall ethnic distribution difference between the groups was not significant (P=0.298). All patients with joint bleeding had severe haemophilia A, while one patient in the no-bleed group had moderate disease (P=1.00). A history of inhibitors was more prevalent in the joint bleeding group (45.5%) compared to the no-bleed group (18.2%), but this difference was also not statistically significant (P=0.361).

Patients with joint bleeding received a slightly lower mean dose of efanesoctocog alfa (56.1 ± 9.1 IU/Kg) compared to those without bleeding (65.2 ± 21.4 IU/Kg), but this difference did not reach statistical significance (P=0.323). Rates of intracranial haemorrhage history were slightly higher in the no-bleed group (18.2% vs. 9.1%). In comparison, other bleeding events during the observation period occurred only in the joint bleeding group (27.3%), neither reaching statistical significance (P=1.00 and 0.214, respectively). Major surgeries (history) were also more frequent in the joint bleeding group (18.2% vs. 0.0%), though not statistically significant (P=0.476).

DISCUSSION

The optimal management of haemophilia A in children centres on prophylaxis to prevent bleeding and subsequent arthropathy, thereby preserving long-term musculoskeletal health and improving quality of life [3,4]. EHL FVIII products have emerged as standard prophylactic options, offering reduced infusion frequency compared to SHL products[6,7]. Efmoroctocog alfa, an EHL FVIII product utilising Fc fusion, has demonstrated effectiveness and a favourable safety profile in children, allowing for less frequent dosing [8,9]. However, the inherent limitation imposed by VWF on FVIII half-life still necessitates dosing intervals that may not consistently maintain FVIII levels sufficient to prevent all bleeding, particularly in individuals with faster clearance or during periods of increased activity [12,22].

Efanesoctocog alfa represents a significant advance in FVIII replacement therapy, designed to overcome the VWF half-life constraint through a unique molecular structure, resulting in a markedly extended FVIII half-life, independent from the half-life of VWD [12,13,15]. This permits once-weekly dosing while maintaining mean FVIII

levels in the normal to near-normal range (>40 IU/dL) for up to three days post-injection, with sustained levels above 10 IU/dL for nearly seven days, as demonstrated in the XTEND-Kids trial [12]. Pivotal clinical trials, including XTEND-Kids in children, have demonstrated superior bleed prevention with efanesoctocog alfa prophylaxis compared to prior FVIII therapies, characterised by very low ABRs [15,17]. Our real-world retrospective study in a cohort of children in the UAE who switched from efmoroctocog alfa to once weekly efanesoctocog alfa prophylaxis provides valuable local evidence complementing these trial findings.

The principal finding of this study is the remarkable prophylactic efficacy demonstrated by efanesoctocog alfa in this cohort post-switch, with all 22 patients (100%) achieving a zero ABR during the observation period. This outcome is consistent with the high efficacy reported in the XTEND-Kids trial, where the model-based mean ABR was 0.61, and a high proportion of children experienced zero bleeds [18]. The observed 100% zero ABR in our cohort is particularly notable and strongly supports the effectiveness of efanesoctocog alfa in preventing treated bleeding episodes in routine clinical practice in the UAE, even if the relatively short, retrospective follow-up period or the specific characteristics of this cohort may contribute to this high rate.

The clinical characteristics of our cohort reflect typical challenges in managing severe haemophilia A in children. The majority had severe disease (95.45%), and a notable proportion (31.82%) had a history of FVIII inhibitors; however, all had achieved immune tolerance induction (ITI) prior to switching, and none had active inhibitors at baseline or developed inhibitors following the switch. This is reassuring and aligns with clinical trial data showing no inhibitor development with efanesoctocog alfa in previously treated patients [12,15]. The absence of new inhibitor development following the switch is consistent with findings from broader literature, which report a low incidence of inhibitor formation in previously treated patients transitioning between FVIII products [20].

A point of interest is that, although all patients achieved a zero ABR during the observation period on efanesoctocog alfa, 11 patients (50%) had experienced joint bleeding episodes prior to the switch, during their previous prophylaxis with efanesoctocog alfa. This highlights the clinical impact of switching, as the same patients reported no treated bleeds following the transition. An exploratory analysis comparing patients with and without prior joint bleeds, though limited by sample size and not statistically significant, revealed that those with bleeding episodes were numerically older, heavier, and more likely to have a history of FVIII inhibitors. These characteristics may indicate a higher baseline bleeding risk or greater joint vulnerability, which is consistent with known predictors of bleed susceptibility. This aligns with clinical understanding that age, joint status (often correlated with age and prior bleeding), and inhibitor history can influence bleeding risk and potentially reflect a higher baseline risk or greater joint vulnerability in these patients, even on effective prophylaxis [23,24].

The observed benefits of efanesoctocog alfa, particularly the once-weekly dosing schedule, offer significant practical advantages for children and their families, potentially enhancing adherence compared to more frequent infusions required with efmoroctocog alfa [19]. Improved adherence and more sustained high FVIII levels are expected to translate into better long-term outcomes, including reduced joint damage [25]. These clinical implications are particularly relevant in the UAE, where regional expert consensus endorses individualised prophylaxis and consideration of newer therapies based on patient outcomes [20].

The absence of active inhibitors after switching should be interpreted cautiously. Although no treatment-emergent inhibitors were detected in this cohort, inhibitors directed against efanesoctocog alfa have recently been described in the post-marketing literature. Tokugawa et al. reported three Japanese patients with haemophilia A in whom FVIII inhibitors were observed during prophylactic treatment with efanesoctocog alfa [26], and Dumont et al. subsequently highlighted challenges and opportunities in post-marketing reporting of FVIII inhibitors with efanesoctocog alfa [27]. Therefore, the present findings do not exclude the possibility of rare inhibitor development after switching, and structured FVIII inhibitor surveillance remains clinically warranted, particularly during the early post-switch period and in patients with prior inhibitor history.

This study benefits from providing valuable real-world evidence from a specific, diverse population in the UAE, a region with unique healthcare dynamics [20]. It documents the clinical experience following a switch from an established EHL (efmoroctocog alfa) to a high-sustained FVIII therapy (efanesoctocog alfa) in routine clinical care, complementing controlled trial

data. However, the study is not without limitations. Its retrospective, single-centre observational design limits the ability to establish causality and generalise findings beyond the specific institution and patient cohort. The small sample size (n=22) reduces the statistical power for detecting significant associations in exploratory analyses and increases the risk of chance findings. The relatively short observation period post-switch provides limited insight into long-term efficacy and safety outcomes, particularly regarding the prevention of cumulative joint damage or the very rare risk of late inhibitor development. Reliance on retrospective data from electronic medical records means the depth and consistency of data collection may vary, although the analysis confirmed no missing data for the variables included. Furthermore, specific safety data collection was not systematically performed as part of a prospective protocol, relying solely on documentation in routine medical records, which may underestimate the incidence of adverse effects. Finally, this study did not include formal pharmacokinetic assessments or measurement of FVIII activity levels, which are central to the mechanism and proposed advantages of efanesoctocog alfa.

Based on these findings and limitations, several directions for future research are suggested. Larger, prospective, multi-centre real-world studies in diverse haemophilia A populations are needed to confirm the high efficacy and safety of efanesoctocog alfa post-switch and to improve the generalisability of findings. Longer follow-up periods are essential to assess long-term clinical outcomes, including joint health progression, and to monitor for rare adverse events or late inhibitor development. Future studies could also incorporate patient-reported outcomes and quality of life assessments to capture the broader impact of reduced infusion burden and improved bleed control. Investigations into the pharmacokinetics of efanesoctocog alfa in real-world cohorts and correlations with clinical outcomes would provide further insights into individualised dosing strategies. Comparative studies, ideally prospective head-to-head or using robust statistical methods like propensity score matching or within-patient comparisons, could more definitively evaluate the relative benefits of efanesoctocog alfa versus established EHLs like efmoroctocog alfa. Research into the cost-effectiveness of switching to efanesoctocog alfa in the UAE and other regional healthcare settings would also be valuable for informing reimbursement decisions and healthcare resource allocation.

CONCLUSIONS

This real-world study from the UAE demonstrates highly effective bleeding prevention following a switch from efmoroctocog alfa to once weekly efanesoctocog alfa prophylaxis, with all patients achieving zero ABR during the observation period and no new inhibitor development. These findings support the clinical benefits of efanesoctocog alfa and align with data from pivotal clinical trials, suggesting that its unique pharmacokinetic profile translates into excellent bleed protection in routine practice. While limited by its retrospective, single-centre nature, small sample size, and relatively short follow-up period, the study provides encouraging real-world evidence that contributes to the growing understanding of efanesoctocog alfa's utility in the management of haemophilia A in children, particularly for patients transitioning from other EHL therapies. Further research with larger cohorts and longer follow-up is warranted to confirm these durable benefits and address the identified limitations.

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Conflict of interest

The authors declare that they have no competing interests.

Data statement

The datasets analysed during the current study are not publicly available due to confidentiality and data protection policies but may be made available upon reasonable request to the corresponding author, subject to institutional data-sharing approvals.

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Informed consent

Ethics approval was obtained from the Institutional Review Board (IRB) of Tawam Hospital. As this was a retrospective study using de-identified data from standard clinical care, the requirement for individual patient consent was waived by the ethics committee. All data were handled in compliance with confidentiality and privacy regulations.

Use of AI

None.

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