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First Interim Analysis of Clinical Outcomes in Adults and Adolescents With Severe Hemophilia A Receiving Efanesoctocog Alfa Prophylaxis in XTEND-ed, a Phase 3 Long-term Extension Study

Sophie Susen,[1] Stephanie P'ng,[2] Toshko Lissitchkov,[3] Davide Matino,[4] Pratima Chowdary,[5] Angela Weyand,[6] Robert Klamroth,[7] Young-Shil Park,[8] Maria Teresa Alvarez-Román,[9] Liqi Feng,[10] Helena Palmborg,[11] Jennifer Dumont,[12] Elena Santagostino,[13] Lila-Sabrina Fetita,[14] Keiji Nogami[15]

[1]Centre Hospitalier Universitaire de Lille, Université de Lille, Lille, France; [2]The Haemophilia and Haemostasis Centre, Fiona Stanley Hospital, Murdoch, WA, Australia; [3]Specialized Hospital for Active Treatment of Hematological Diseases, Department of Chemotherapy, Hemotherapy and Hereditary Blood Diseases at Clinical Hematology Clinic, Sofia, Bulgaria; [4]Division of Hematology & Thromboembolism, Department of Medicine, McMaster University, Ontario, Canada; [5]Katharine Dormandy Haemophilia and Thrombosis Centre, Royal Free Hospital, London, UK; [6]Division of Hematology/Oncology, Department of Pediatrics, University of Michigan Medical School, Ann Arbor, MI, USA; [7]Vivantes Klinikum, Friedrichshain, Berlin, Germany; [8]Department of Pediatrics, Kyung Hee University Hospital at Gangdong, School of Medicine, Kyung Hee University, Seoul, Korea; [9]Department of Hematology, University Hospital La Paz, Autonoma University of Madrid, Madrid, Spain; [10]Sanofi, Shanghai, China; [11]Sobi, Stockholm, Sweden; [12]Sanofi, Cambridge, MA, USA; [13]Sobi, Basel, Switzerland; [14]Sanofi, Paris, France; [15]Nara Medical University, Nara, Japan

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Disclosures for Prof Sophie Susen, MD, PhD

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Presentation Learning Objectives

At the conclusion of this presentation, participants will be able to:

- Understand that efanesoctocog alfa continues to be **safe** and **well-tolerated** and remains **highly effective for bleed protection among adults and adolescents** in this first interim analysis of the XTEND-ed long-term extension study
- Recognize **efanesoctocog alfa** as a first-in-class, high-sustained factor VIII (HSF) replacement therapy for treatment of hemophilia A

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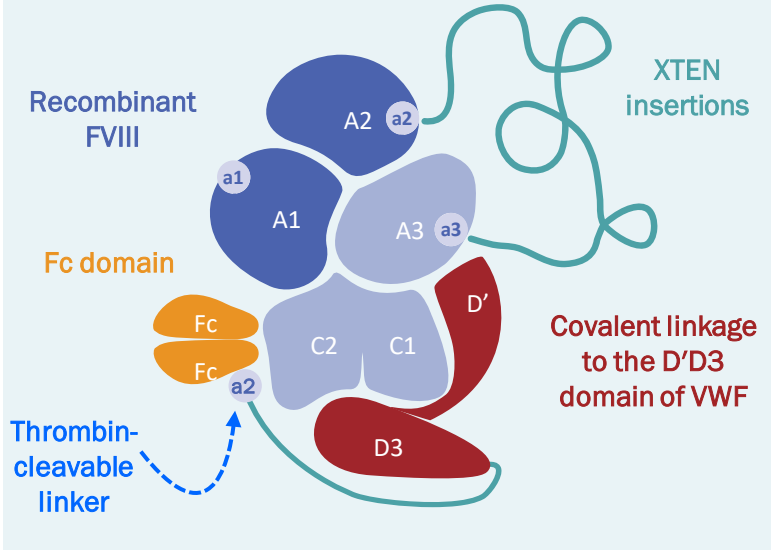
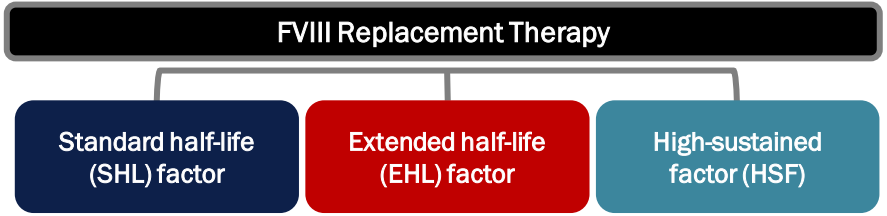
Efanesoctocog Alfa Is a First-in-Class High-Sustained FVIII Replacement Therapy

Efanesoctocog alfa is a novel fusion protein that **overcomes the VWF-imposed half-life ceiling**[1,2]





In the XTEND-1 study (NCT04161495), once-weekly efanesoctocog alfa 50 IU/kg prophylaxis[3]

- Achieved **high-sustained factor levels** in the **normal to near-normal range** (>40%) for the majority of the week
- **Provided superior bleed protection compared with prior factor prophylaxis** with clinically meaningful improvements in physical health, pain, and joint health

Figure: ©2023 Bioverativ Therapeutics Inc. All rights reserved.
FVIII, factor VIII; VWF, von Willebrand factor.
1. Chhabra ES, et al. *Blood*. 2020;135(17):1484-1496. 2. Konkle BA, et al. *N Engl J Med*. 2020;383(11):1018-1027. 3. von Drygalski A, et al. *N Engl J Med*. 2023;388(4):310-318.

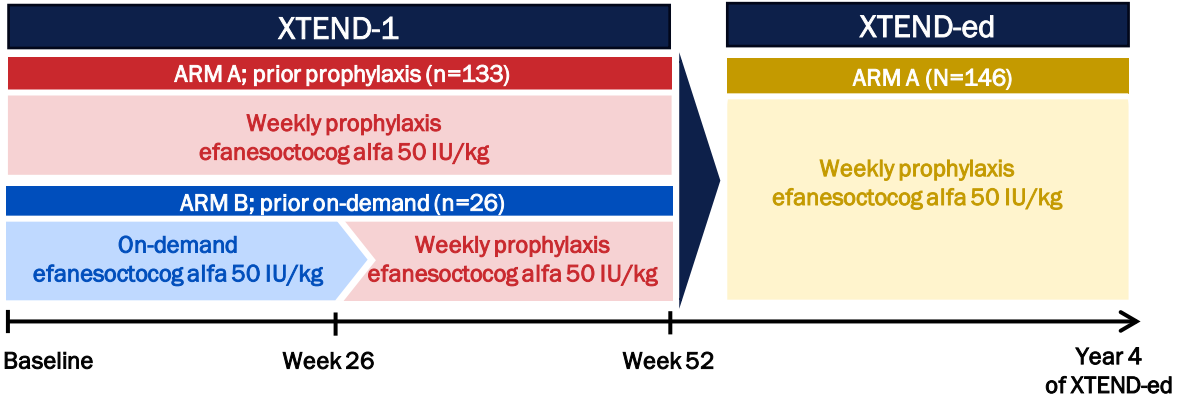
Aim

Evaluate long-term safety and efficacy of efanesoctocog alfa in adults/adolescents with severe hemophilia A who rolled over from the Phase 3 XTEND-1 study into the Phase 3 XTEND-ed long-term extension study

First interim analysis, data cut: June 8, 2023.
XTEND-ed: NCT04644575.

XTEND-ed: An Ongoing, Multicenter, Open-label Study of the Long-Term Safety and Efficacy of Efanesoctocog Alfa

- Participants from XTEND-1 who rolled over into XTEND-ed
- Severe hemophilia A (<1 IU/dL endogenous FVIII activity)[a]
- Previous treatment with any recombinant and/or plasma-derived FVIII, or cryoprecipitate



Primary endpoint

- The occurrence of inhibitor development[b]

Secondary endpoints

- Annualized bleed rates (ABRs)
- Treatment of bleeding episodes
- Safety and tolerability

This analysis: interim outcomes in adults/adolescents ≥12 years of age from XTEND-1 (Data cut: June 8, 2023)[c]

FVIII, factor VIII.
[a]Or a documented genotype known to produce severe hemophilia A. [b]Inhibitor development was evaluated using the Nijmegen-modified Bethesda assay at the central laboratory. Inhibitor development was defined as an inhibitor result of ≥0.6 BU/mL and confirmed by a second test result from a separate sample drawn 2–4 weeks following the date of the original sample. [c]XTEND-ed study commenced in February 2021.

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Demographics of Adults and Adolescents in XTEND-ed Interim Analysis

Overall, **146 participants** from XTEND-1 **rolled over to XTEND-ed**

- 98% of participants (121/124) who completed XTEND-1 Arm A
- 100% of participants (25/25) who completed XTEND-1 Arm B

Mean age was **37 years**

A total of **138 participants (95%) remain in the study** and **8 have discontinued**[a]

XTEND-ed participants	From XTEND-1 Arm A (n=121)	From XTEND-1 Arm B (n=25)	Overall XTEND-ed (N=146)
Sex, n (%)			
Male	120 (99.2)	25 (100)	145 (99.3)
Female	1 (0.8)	0	1 (0.7)
Age			
Mean (SD)	35.5 (15.3)	44.2 (12.0)	37.0 (15.1)
Median (range)	36.0 (13–74)	41.0 (24–70)	37.0 (13–74)
Age group, years, n (%)			
12–17 years	23 (19.0)	0	21 (14.4)
18–64 years	95 (78.5)	24 (96.0)	120 (82.2)
≥65 years	3 (2.5)	1 (4.0)	5 (3.4)
Race, n (%)			
White	75 (62.0)	25 (100)	100 (68.5)
Black or African American	4 (3.3)	0	4 (2.7)
Asian	27 (22.3)	0	27 (18.5)
Not reported	13 (10.7)	0	13 (8.9)
Other	2 (1.7)	0	2 (1.4)
Weight, kg			
Mean (SD)	77.4 (19.7)	80.9 (18.4)	78.0 (19.4)
Median (range)	77.2 (33.9–132.8)	77.8 (50.0–119.5)	77.3 (33.9–132.8)

Data cut: June 8, 2023.
SD, standard deviation.
[a]Reasons for study discontinuation include: adverse event (n=1), consent withdrawn (n=2), use of prohibited medications due to medical needs as determined by investigator (n=4), and other (n=1).

No Inhibitors Developed During the XTEND-ed Study

- Mean (SD) **treatment duration** in XTEND-ed was **82.5 (14.3) weeks** (median [range], 84.6 [14.1–103.6] weeks)[a]
  - In XTEND-1 the mean (SD) treatment duration was 49.6 (8.3) weeks (median [range], 52.1 [1.1–55.1] weeks)[a]
- Mean (SD) **total exposure days** was **83.1 (14.8)** (median [range], 85.5 [14–108]) in XTEND-ed[b]

FVIII inhibitors did not develop[c]

- Incidence of inhibitor formation[d]: **0.0 (95% CI, 0.0–2.5)**

Data cut: June 8, 2023.
CI, confidence interval; FVIII, factor VIII.
[a]Duration of treatment refers to total duration in XTEND-1 or XTEND-ed, including periods of PK evaluation, surgery/rehabilitation (minor and major), and large injection intervals. [b]Exposure day defined as a 24-hour period in which ≥1 injections of efanesoctocog alfa are given, all injections over the study course are counted. [c]Inhibitor development was evaluated using the Nijmegen-modified Bethesda assay at the central laboratory. Inhibitor development was defined as an inhibitor result of ≥0.6 BU/mL and confirmed by a second test result from a separate sample drawn 2–4 weeks following the date of the original sample. [d]95% CI calculated using the Clopper-Pearson exact method.

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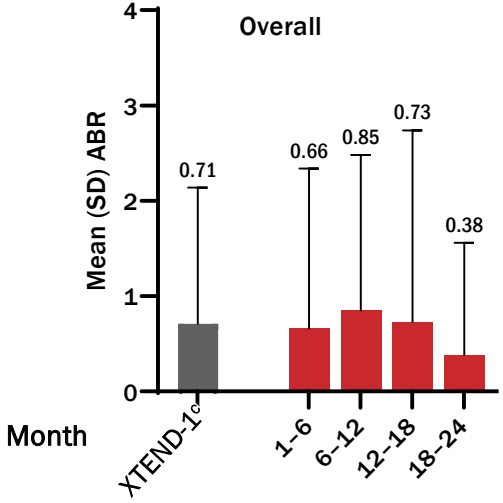
ABRs Remained Low During the First 2 Years of XTEND-ed for Participants from XTEND-1 Arm A

Summary of ABRs by 6-month intervals for participants from XTEND-1 Arm A (n=121)[a,b]



Month	Number of evaluable participants, n (%)
1–6	121 (100)
6–12	120 (99.2)
12–18	116 (95.9)
18–24	87 (71.9)

Median overall ABR was 0 across all 6-month intervals

Mean (SD) **overall ABR** during **XTEND-ed** for **participants from XTEND-1 Arm A** was **0.72 (1.26),** maintaining the low ABR of 0.71 observed in XTEND-1

Data cut: June 8, 2023. ABR, annualized bleed rate; SD, standard deviation.
[a]Values are based on the number of participants with an evaluable efficacy period, defined as the treatment regimen period, from the first injection of efanesoctocog alfa in Arm A of XTEND-ed to the day of the last dose of efanesoctocog alfa or the data cutoff date of June 8, 2023 (the date of the first interim data cut), whichever was first. The efficacy period excluded periods of surgery/rehabilitation (minor and major) and large injection intervals (>28 days).[b]During XTEND-1, in Arm A, participants received once-weekly efanesoctocog alfa 50 IU/kg prophylaxis for 52 weeks. [c]Overall ABR during XTEND-1 for participants in Arm A (n=133).

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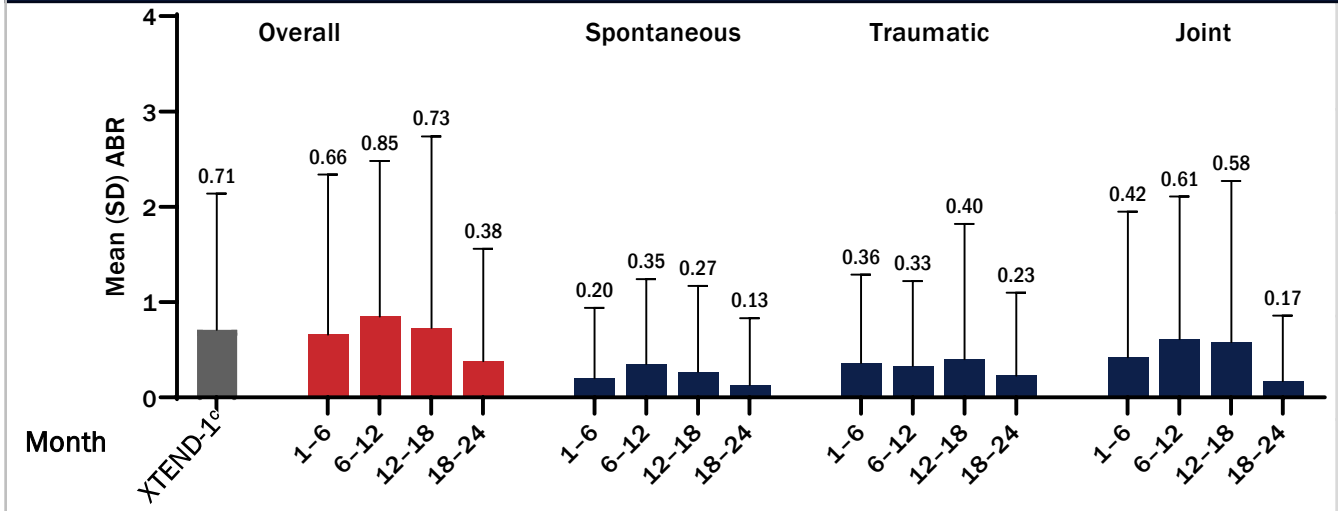

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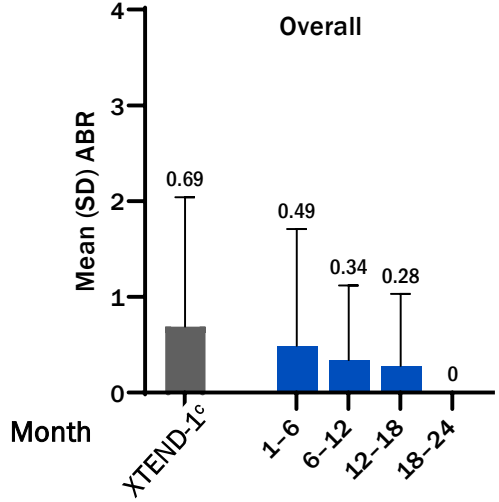
ABRs Remained Low During the First 2 Years of XTEND-ed for Participants from XTEND-1 Arm B

Summary of ABRs by 6-month intervals for participants from XTEND-1 Arm B (n=25)[a,b]



Month	Number of evaluable participants, n (%)
1–6	25 (100)
6–12	24 (96.0)
12–18	23 (92.0)
18–24	10 (40.0)

Median overall ABR was 0 across all 6-month intervals

Mean (SD) **overall ABR** during **XTEND-ed** for **participants from XTEND-1 Arm B** was **0.42 (0.89)**, during XTEND-1 prophylaxis period overall ABR was 0.69 in this cohort

Data cut: June 8, 2023. ABR, annualized bleed rate; SD, standard deviation.
[a]Values are based on the number of participants with an evaluable efficacy period, defined as the treatment regimen period, from the first injection of efanesoctocog alfa in Arm A of XTEND-ed to the day of the last dose of efanesoctocog alfa or the data cutoff date of June 8, 2023 (the date of the first interim data cut), whichever was first. The efficacy period excluded periods of surgery/rehabilitation (minor and major) and large injection intervals (>28 days).[b]During XTEND-1, in Arm B, participants received on-demand treatment with efanesoctocog alfa 50 IU/kg for 26 weeks followed by once-weekly efanesoctocog alfa 50 IU/kg prophylaxis for 26 weeks. [c]Overall ABR during XTEND-1 prophylaxis period for participants in Arm B (n=26).

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ABRs Remained Low During the First 2 Years of XTEND-ed for Participants from XTEND-1 Arm B

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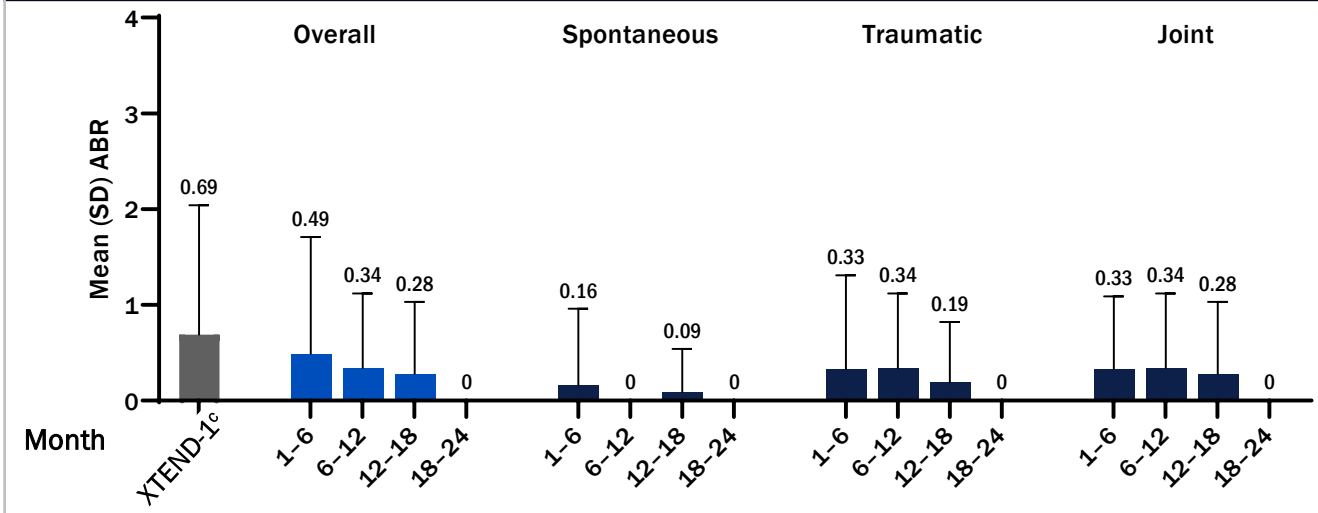

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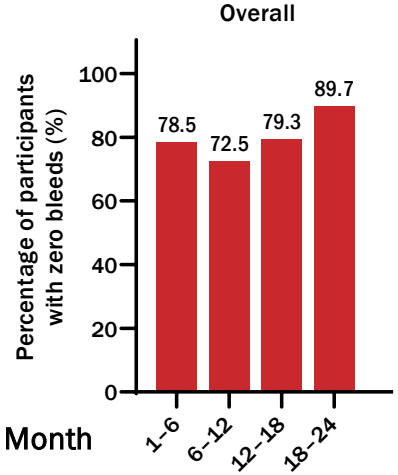
Percentage of Participants from XTEND-1 Arm A with Zero Bleeds Was Maintained During the First 2 Years of XTEND-ed

Summary of percentage of participants from XTEND-1 Arm A (n=121) with zero bleeds by 6-month intervals[a,b]



Month	Number of evaluable participants, n (%)
1–6	121 (100)
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In participants in XTEND-ed from XTEND-1 Arm A, the **percentage of patients with zero bleeds overall remained approximately 80% through the first 2 years** of XTEND-ed

- In XTEND-1 Arm A, 65% of evaluable patients had zero bleeds

Data cut: June 8, 2023. ABR, annualized bleed rate; SD, standard deviation.
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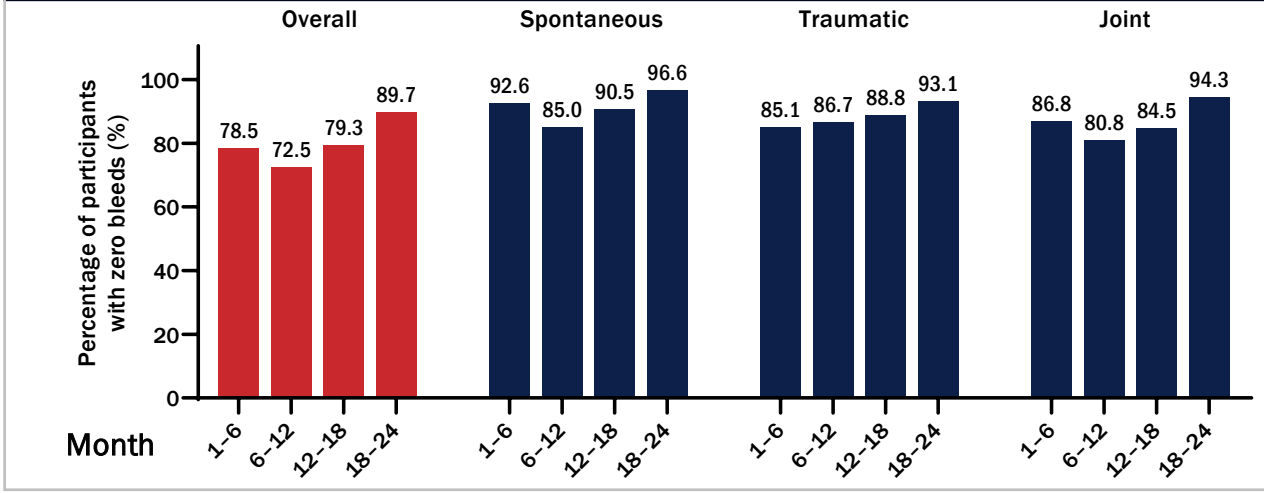

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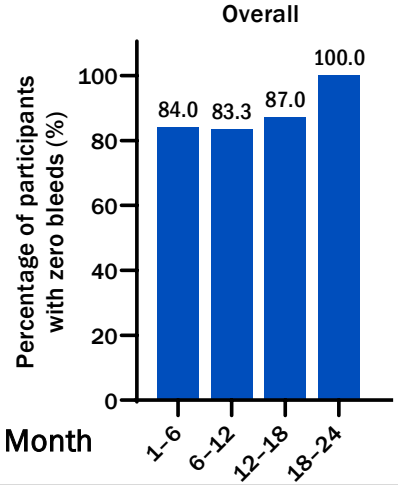
Percentage of Participants from XTEND-1 Arm B with Zero Bleeds Was Maintained During the First 2 Years of XTEND-ed

Summary of percentage of participants from XTEND-1 Arm B (n=25) with zero bleeds by 6-month intervals[a,b]



Month	Number of evaluable participants, n (%)
1–6	25 (100)
6–12	24 (96.0)
12–18	23 (92.0)
18–24	10 (40.0)

In participants from XTEND-1 Arm B, the **percentage of patients with zero bleeds** was **84% during the first 6 months of XTEND-ed**

- During the prophylaxis period of XTEND-1, 77% of participants in Arm B had zero bleeds

Data cut: June 8, 2023. ABR, annualized bleed rate; SD, standard deviation.
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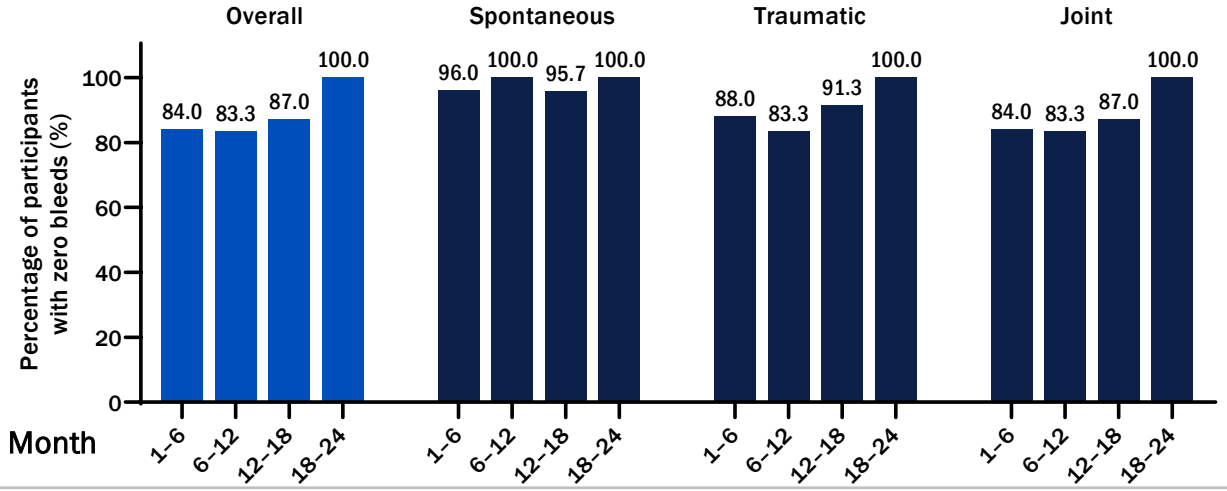

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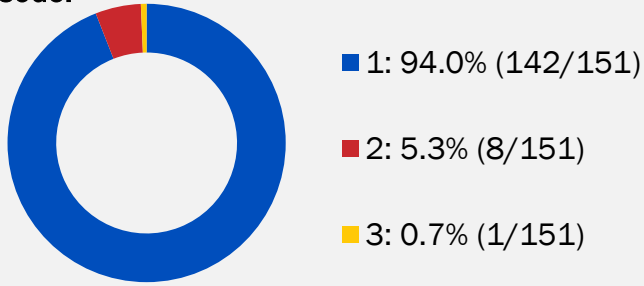
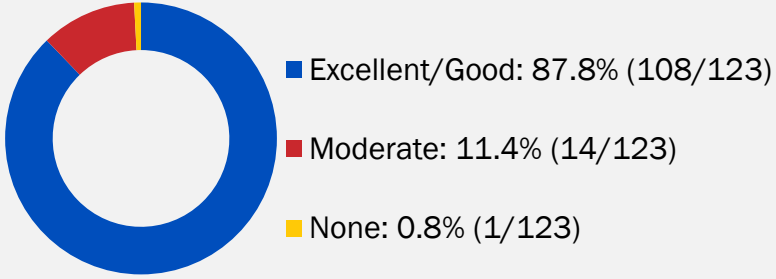
Efanesoctocog Alfa Remains Highly Effective for the Treatment of Bleeding Episodes in XTEND-ed

Number of injections required to resolve a bleeding episode:



94% of bleeding episodes (142/151) resolved with a single injection of efanesoctocog alfa

Participant assessment of response to efanesoctocog alfa treatment of bleeding episodes[a]:



Of the **123 injections with an evaluation, 88% (108/123)** were assessed as excellent or good

Dose of efanesoctocog alfa required for resolution of a bleeding episode

	Dose per injection (IU/kg)	Total dose (IU/kg)
Mean (SD)	45.5 (9.0)	48.2 (14.0)
Median (range)	50.1 (28.7–71.1)	50.3 (28.7–132.8)

Data cut: June 8, 2023. Outcomes reported here for the efficacy period in XTEND-ed, mean (SD) duration of efficacy period was 81.70 (14.30) weeks.
ISTH, International Society on Thrombosis and Hemostasis; SD, standard deviation.
[a]Based on the ISTH 4-point response scale of excellent, good, moderate, and none. "None" means there was no improvement, not that the participant did not provide a response.

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Compliance Rates in Adults/Adolescents Treated With Efanesoctocog Alfa in XTEND-ed Were High

Mean (SD) number of prophylactic injections per patient in XTEND-ed was **81.2 (14.7)**[a]

- 144 participants **(99%)** were both **dose and interval compliant**[b-d]
  - 1 participant (0.7%) was dose but not interval compliant
  - 1 participant (0.7%) was interval but not dose compliant

Data cut: June 8, 2023. PK, pharmacokinetic; rFVIIIFc, recombinant factor VIII Fc fusion protein; SD, standard deviation.
[a]Injection types are as collected in the electronic patient diary/electronic case report form. [b]Prophylactic dose compliance rate defined as % of doses taken within 80% to 125% of prescribed dose. [c]Prophylactic dosing interval compliance rate defined as % of doses taken within ±36 hours of prescribed day. [d]A participant was considered both dose and interval compliant if calculated compliance rate was ≥80%.

Efanesoctocog Alfa Was Well-tolerated

	XTEND-ed participants from XTEND-1 (N=146)[a-c]
Total number of TEAEs	460
Participants with ≥1 TEAE, n (%)	108 (74.0)
Participants with ≥1 related TEAE, n (%)	2 (1.4)
TEAEs occurring in >5% of participants, n (%)[d,e]	
COVID-19	32 (21.9)
Arthralgia	19 (13.0)
Headache	13 (8.9)
Nasopharyngitis	12 (8.2)
Influenza	9 (6.2)
Total number of TESAEs	25
Participants with ≥1 TESAE, n (%)[f]	17 (11.6)
Participants with ≥1 related TESAE, n (%)	0
TEAEs leading to death, n (%)	0
TEAEs leading to treatment discontinuation, n (%)	2 (1.4)

Related TEAEs were:

- **Facial paralysis**
- **Coagulation FVIII level decreased** (isolated incident of low FVIII activity levels observed prior to next dose of efanesoctocog alfa; FVIII activity levels were measured locally)

TEAEs leading to discontinuation included:

- **Femur fracture**
- **Deep vein thrombosis after surgical correction of a femur fracture** (in the setting of **treatment with another FVIII product**)

Both events were unrelated to efanesoctocog alfa treatment

Data cut: June 8, 2023.
AE, adverse event; FVIII, factor VIII; TEAE, treatment-emergent adverse event; TESAE, treatment-emergent serious adverse event.
[a]Percentages based on the number of participants in the safety analysis set. [b]AEs with missing causality assessment are included in the related TEAE or related TESAE category. [c]AEs that occurred during a major surgical/rehabilitation period are excluded from this table, but AEs that occurred on the day the surgical/rehabilitation period started are included. [d]Events were coded using MedDRA version 26.0. [e]Participants were counted once if they reported multiple events in the same system organ class or preferred term. [f]There were no serious allergic/anaphylactic reactions.

Conclusions

FVIII inhibitors did not develop in adult and adolescent participants in Arm A of XTEND-ed who had rolled over from XTEND-1

ABRs remained low (<1) and the percentage of patients with zero bleeding episodes remained ~80% (across 6-month intervals) **over an additional 2 years of treatment** with efanesoctocog alfa

Efanesoctocog alfa continues to be **well-tolerated**

In this interim analysis of XTEND-ed (mean treatment duration: 82.5 weeks), once-weekly efanesoctocog alfa prophylaxis (50 IU/kg) continues to be well-tolerated and highly effective in adults and adolescents

Data cut: June 8, 2023.
ABR, annualized bleed rate; FVIII, factor VIII.

Thank you

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