Joint Health in Patients with Severe Haemophilia A Treated with Efanesoctocog Alfa: 12-Month Interim Results from the FREEDOM Study

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Θεματική ενότητα : Αιματολογία

Background/Aims:

Efanesoctocog alfa is a first-in-class, high-sustained factor VIII replacement therapy (ultra-long half-life FVIII). We report 12-month interim joint health and efficacy data from FREEDOM (NCT05817812), a prospective, 24-month, Phase 3b study in patients with severe haemophilia A receiving efanesoctocog alfa prophylaxis.

Methods:

Previously treated patients (≥12 years) with severe haemophilia A received once-weekly 50 IU/kg efanesoctocog alfa. Joint health was assessed using the Haemophilia Joint Health Score (HJHS) and Haemophilia Early Arthropathy Detection with Ultrasound (HEAD-US). Synovial hypertrophy (SH) was defined at the patient level (≥1 joint with HEAD-US SH score of 1–2) and joint level (SH scores of 1–2). SH changes are reported as reduction (≥1 joint with ≥1-point HEAD-US SH score reduction at Month 12) or resolution (all SH-affected joints at baseline reaching scores of 0 at Month 12).

Patients:

FREEDOM enrolled 93 patients; 91 remained at Month 12.

Results:

Mean (SD) total ABR/joint ABR was 0.3 (0.9)/0.2 (0.8).

Mean (SD) HJHS total scores were 13.0 (17.6) at baseline (N=83) and 11.5 (16.7) at Month 12 (N=85) (mean change: −1.1 [4.4]; N=78). Mean (SD) HEAD-US total scores were 9.8 (11.4) at baseline (N=88) and 8.6 (10.5) at Month 12 (N=85) (mean change: 0.0 [2.8]; N=82).

At baseline, 53.8% of patients had HEAD-US SH in ≥1 joint, versus 45.1% at Month 12. Among 47 patients with ≥1 SH-affected joint at baseline and

assessment at Month 12, 53.2% had reduction or resolution; 19.1% achieved full resolution. Of 108 joints with SH at baseline, 44.4% had reduction or resolution at Month 12.

Conclusion:

Efanesoctocog alfa prophylaxis was highly effective in preventing joint bleeds; most patients had no treated joint bleeds over 12 months. Despite previous FVIII prophylaxis, ~half of patients entered FREEDOM with SH in ≥1 joint; of these, ~half had SH reduction or resolution after 12 months of efanesoctocog alfa.

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Conflicts of interest:

CK: Personal fees from Bayer, CSL Behring, Novo Nordisk, Pfizer, Roche/Chugai, Sanofi/Sobi and Takeda; speaker/advisory boards, research and/or clinical trial support for Bayer, Biotest, CSL Behring, EU H2020 ITN, FUSE e.V., Haemophilia Foundation, Novo Nordisk, Pfizer, Roche/Chugai, Sanofi/Sobi, State Hesse and Takeda.

HP: Speaker/advisory boards, honoraria, research and/or clinical trial support from Novo Nordisk, Roche, Pfizer, Sanofi, Sobi, CSL-Behring.

DD: Grant, honoraria or invitation as speaker in symposia from CSL Behring, Novo Nordisk, Roche, Sobi and Takeda.

MTÁR: Advisory boards and speakers bureau for Amgen, Bayer, BioMarin, Bioverativ, CSL Behring, Grifols, LFB, Novartis, Novo Nordisk, Octapharma, Pfizer, Roche and Takeda; research funding from Takeda; honoraria for speaking/participating in advisory boards from Amgen, Bayer, Biomarin, CSL Behring, LFB, Novartis, Novo Nordisk, Pfizer, Roche, Sobi and Takeda.

CS: Consultant for Bayer, Biomarin, CSL Behring, Novo Nordisk, Pfizer, Roche, Sobi and Takeda; speaker bureau for Bayer, Biomarin, CSL Behring, Novo Nordisk, Pfizer, Roche, Sobi and Takeda.

JBl, JBj, MF: Employees of Sobi and may hold shares and/or stock options in the company.

JA: Received grant/research support from CSL Behring and Sobi; honorarium as member of advisory boards and speaker for Bayer, BioMarin, CSL Behring, Novo Nordisk, Octapharma, Pfizer, Roche, Sanofi, Sobi, and Takeda

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