Concizumab Explorer 7 Phase 3 Trial

- Enrolled males ≥12 years old with hemophilia A or B with inhibitors
- Treatment:
  - Arms 1/2: randomized 1:2 to placebo (≥24 wks; n=19) or concizumab (≥32 wks; n=33)
  - Arms 3/4: assigned to concizumab (n=81)
- Primary outcome: treated spontaneous and traumatic bleeding episodes in arm 1 vs arm 2
- Results
  - Completers: arm 1 (14/19); arm 2 (28/33)
  - Estimated mean ABR: arm 1 (11.8) vs arm 2 (1.7) (86% reduction)
  - No treated bleed at wk 24: arm 1 (2/19, 10.5%) vs arm 2 (21/33, 63.6%)
  - Serious AEs: arm 1 (15.8%); arm 2 (18.2%)
    - Thromboembolic event after treatment restart*: arm 1 (0%) vs arm 2 (0%)

*Treatment paused due to thromboembolic events to institute added safety measures
ABR, annualized bleeding rate

Jiménez Yuste V, Angchaisuksiri P, Castaman G, et al. Concizumab prophylaxis in patients with haemophilia A or B with inhibitors: Efficacy and safety results from the primary analysis of the phase 3 explorer7 trial [abstract]. International Society on Thrombosis and Haemostasis, July 9-13, 2022. Accessed July 13, 2022. https://abstracts.isth.org/abstract/concizumab-prophylaxis-in-patients-with-haemophilia-a-or-b-with-inhibitors-efficacy-and-safety-results-from-the-primary-analysis-of-the-phase-3-explorer7-trial/.
ClinicalTrials.gov. Updated March 21, 2022. https://clinicaltrials.gov/ct2/show/NCT04083781.

Efanesoctocog Alfa (BIVV001) Phase 3 XTEND-1 Trial

- Enrolled patients ≥12 years old with severe hemophilia A
- Current regimen
  - Arm A (n=133): prophylaxis with FVIII or emicizumab x 6 mos during previous 12 mos, or
  - Arm B (n=26): on-demand FVIII with ≥12 bleeds in previous 12 mos or ≥6 bleeds in previous 6 mos
- Treatment
  - Arm A: once-weekly efanesoctocog alfa x 52 weeks
  - Arm B: on demand efanesoctocog alfa x 26 weeks followed by 26 weeks of prophylaxis
- Primary outcome: ABR in Arm A
- Results
  - ABR in Arm A: 0.71 (mean); 0.00 (median)
  - Compared with prior FVIII prophylaxis, prophylactic efanesoctocog alfa significantly reduced bleeding events ($P<0.001$)
  - Most common AEs (>5%): headache, arthralgia, fall, back pain

ABR, annualized bleeding rate, AE, adverse event

von Drygalski A, Chowdary P, Kulkarni R, et al. Efficacy, safety, and pharmacokinetics of once-weekly efanesoctocog alfa (BIVV001) prophylaxis in previously treated patients with severe hemophilia A: results from the phase 3 XTEND-1 Study [abstract]. International Society on Thrombosis and Haemostasis, July 9-13, 2022. Accessed July 13, 2022. https://abstracts.isth.org/abstract/efficacy-safety-and-pharmacokinetics-of-once-weekly-efanesoctocog-alfa-bivv001-prophylaxis-in-previously-treated-patients-with-severe-hemophilia-a-results-from-the-phase-3-xtend-1-study/.

ClinicalTrials.gov. Updated March 7, 2022. https://clinicaltrials.gov/ct2/show/NCT04161495.