

Reimagining Outcomes in Hemophilia:

Improving Efficacy and Reducing Treatment Burden With High-Sustained Factor and NonFactor Replacement Therapies

Suggested Readings

Hemophilia A

Chhabra ES, Liu T, Kulman J, et al. BIVV001, a new class of factor VIII replacement for hemophilia A that is independent of von Willebrand factor in primates and mice. *Blood*. 2020;135:1484-1496. doi:10.1182/blood.2019001292

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Frei-Jones M, Cepo K, d'Oiron R, et al. Subcutaneous concizumab prophylaxis in patients with hemophilia A or B with inhibitors: efficacy and safety results by hemophilia subtype from the phase 3 explorer7 trial. *Blood*. 2022;140(suppl 1):466-468. doi:10.1182/blood-2022-166522

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. *Res Pract Thromb Haemost*. 2022;6(suppl 1):LB 01.2.

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Lissitchkov T, Willemze A, Jan C, et al. Pharmacokinetics of recombinant factor VIII in adults with severe hemophilia A: fixed-sequence single-dose study of octocog alfa, ruriococtocog alfa pegol, and efanesoctocog alfa. *Res Pract Thromb Haemost*. 2023;7:100176. doi:10.1016/j.rpth.2023.100176

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Mahlangu J, Luis Lamas J, Cristobal Morales J, et al. Long-term safety and efficacy of the anti-tissue factor pathway inhibitor marstacimab in participants with severe haemophilia: phase II study results. *Br J Haematol*. 2023;200:240-248. doi:10.1111/bjh.18495

Malec L, Peyvandi F, Chan AKC, et al. Efanesoctocog alfa prophylaxis for previously treated patients <12 years of age with severe hemophilia A. Presented at: International Society on Thrombosis and Haemostasis (ISTH) 2023 Congress; June 24-28, 2023; Montreal, Canada. Abstract LB01.1.

Matsushita T, Shapiro A, Abraham A, et al. Phase 3 trial of concizumab in hemophilia with inhibitors. *N Engl J Med*. 2023;389:783-794. doi:10.1056/NEJMoa2216455

Miesbach W, Klamroth R, Oldenburg J, et al. Gene therapy for hemophilia—opportunities and risks. *Dtsch Arztebl Int*. 2022;119:887-894. doi:10.3238/arztebl.m2022.0353

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Skinner MW, Nugent D, Wilton P, et al. Achieving the unimaginable: health equity in haemophilia. *Haemophilia*. 2020;26:17-24. doi:10.1111/hae.13862

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Tieu P, Chan A, Matino D. Molecular mechanisms of inhibitor development in hemophilia. *Mediterr J Hematol Infect Dis.* 2020;12:e2020001. doi:10.4084/MJHID.2020.001

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Young G, Srivastava A, Kavakli K, et al. Efficacy and safety of fitusiran prophylaxis in people with haemophilia A or haemophilia B with inhibitors (ATLAS-INH): a multicentre, open-label, randomised phase 3 trial. *Lancet.* 2023;401:1427-1437. doi:10.1016/S0140-6736(23)00284-2

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Sehgal A, Barros S, Ivanciu L, et al. An RNAi therapeutic targeting antithrombin to rebalance the coagulation system and promote hemostasis in hemophilia. *Nat Med.* 2015;21:492-497. doi:10.1038/nm.3847

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