

Hemophilia A Selected Publications

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Clinical	Ozelo MC, Mason J, Dunn AL, et al. Safety and efficacy of valoctocogene roxaparvovec with prophylactic glucocorticoids: 1-year results from the phase 3b, single-arm, open-label GENER8-3 study. <i>J Thromb Haemost.</i> 2025;23(5):1496-1506. DOI: 10.1016/j.jtha.2024.12.038
	Leavitt AD, Mahlangu J, Raheja P, et al. Efficacy, safety, and quality of life 4 years after valoctocogene roxaparvovec gene transfer for severe hemophilia A in the phase 3 GENER8-1 trial. <i>Res Pract Thromb Haemost.</i> 2024;8(8):102615. DOI: 10.1016/j.rpth.2024.102615
	Quon DV, Wang JD, Wang M, et al. Outcomes and management of invasive procedures in participants with hemophilia A post gene therapy: A post hoc analysis of the GENER8-1 phase III trial. <i>Ther Adv Hematol.</i> 2024; 15:20406207241304645. DOI: 10.1177/20406207241304645
	Symington E, Rangarajan S, Lester W, et al. Valoctocogene roxaparvovec gene therapy provides durable haemostatic control for up to 7 years for haemophilia A. <i>Haemophilia.</i> 2024;30(5):1138-1147. DOI: 10.1111/hae.15071
	O'Mahony B, Dunn AL, Leavitt AD, et al. Health-related quality of life following valoctocogene roxaparvovec gene therapy for severe hemophilia A in the phase 3 trial GENER8-1. <i>J Thromb Haemost.</i> 2023;21(12):3450-3462. DOI: 10.1016/j.jtha.2023.08.032
	Ozelo MC, Mahlangu J, Pasi KJ, et al. Valoctocogene roxaparvovec gene therapy for hemophilia A. <i>N Engl J Med.</i> 2022; 386:1013-1025. DOI: 10.1056/NEJMoa2113708
	Fong S, Yates B, Sihh C-R, et al. Interindividual variability in transgene mRNA and protein production following adeno-associated virus gene therapy for hemophilia A. <i>Nature Med.</i> 2022;28:789-797. DOI: 10.1038/s41591-022-01751-0
Preclinical	Pasi KJ, Laffan M, Rangarajan S, et al. Persistence of haemostatic response following gene therapy with valoctocogene roxaparvovec in severe haemophilia A. <i>Haemophilia.</i> 2021;27(6):947-956. DOI: 10.1111/hae.14391
	Russell CB, Vettermann C, Agarwal S, et al. Recombinant adeno-associated virus integration profiles in nonhuman primates and gene therapy participants after treatment with valoctocogene roxaparvovec. <i>Hum Gene Ther.</i> 2025;36(13-14):945-955. DOI: 10.1089/hum.2024.236
Other	Ismail AM, Witt E, Bouwman T, et al. The longitudinal kinetics of AAV5 vector integration profiles and evaluation of clonal expansion in mice. <i>Mol Ther Methods Clin Dev.</i> 2024;32(3):101294. DOI: 10.1016/j.omtm.2024.101294
	Santos S, Robinson T, Trueman D. Estimated long-term durability of valoctocogene roxaparvovec treatment in male patients with severe hemophilia A: An extrapolation of clinical data. <i>Adv Ther.</i> 2025; 42:5781-5793. DOI: 10.1007/s12325-025-03368-4
	Douglas TG, Santos S, Hinds DR, Hatswell AJ, Taylor K. Comparative effectiveness of valoctocogene roxaparvovec and efanesoctocog alfa in the treatment of severe hemophilia A: A matching-adjusted indirect comparison of bleeding frequency. <i>Adv Ther.</i> 2025; 42:5600-5611. DOI: 10.1007/s12325-025-03339-9
	Konkle BA, Peyvandi F, Foster GR, et al. Corticosteroid use to mitigate transaminitis-associated decline in FVIII levels following valoctocogene roxaparvovec gene therapy: clinical practice guidance. <i>J Thromb Haemost.</i> 2025;23(7):2086-2094. DOI: 10.1016/j.jtha.2025.02.042
	Agarwal S, Hermans C, Miesbach W, et al. Transitioning from emicizumab prophylaxis to valoctocogene roxaparvovec gene therapy: A simulation study for individuals with severe haemophilia A. <i>Haemophilia.</i> 2024;30(4):905-913. DOI: 10.1111/hae.15025
	Klamroth R, Gottstein S. Transitioning patients with severe haemophilia A from emicizumab prophylaxis to valoctocogene roxaparvovec gene therapy: Real-world clinical experience. <i>Haemophilia.</i> 2024;30(5):1247-1249. DOI: 10.1111/hae.15086
	Oldenburg J, Chambost H, Liu H, et al. Comparative effectiveness of valoctocogene roxaparvovec and prophylactic factor VIII replacement in severe hemophilia A. <i>Adv Ther.</i> 2024;41(6):2267-2281. DOI: 10.1007/s12325-024-02834-9
	Ragni M, Mead H, de Jong Y, et al. Optimizing liver health before and after gene therapy for hemophilia A. <i>Blood Adv.</i> 2024;19(8):5203-5212. DOI: 10.1182/bloodadvances.2024013059
	Astermark J, Buckner TW, Frenzel L, et al. Matching-adjusted indirect comparison of bleeding outcomes in severe haemophilia A: Comparing valoctocogene roxaparvovec gene therapy, emicizumab prophylaxis, and FVIII replacement prophylaxis. <i>Haemophilia.</i> 2023;29(4):1087-1094. DOI: 10.1111/hae.14818

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