

## Selected Publications List BMN270

### Open Access

Clinical	<a href="#">Two-Year Outcomes of Valoctocogene Roxaparovec Therapy for Hemophilia A</a> Mahlangu J, Kaczmarek R, von Drygalski A, et al. <i>N Engl J Med</i> . 2023.
	<a href="#">Valoctocogene Roxaparovec Gene Therapy for Hemophilia A</a> Ozelo MC, Mahlangu J, Pasi KJ, et al. <i>New England Journal of Medicine</i> . 2022. <i>Phase 3</i>
	<a href="#">Persistence of haemostatic response following gene therapy with valoctocogene roxaparovec in severe haemophilia A</a> Pasi KJ, Laffan M, Rangarajan S, et al. <i>Haemophilia</i> . 2021. <i>Phase 1/2</i>
	<a href="#">Interindividual variability in transgene mRNA and protein production following adeno-associated virus gene therapy for hemophilia A</a> Fong S, Yates B, Choong-Ryoul Sihm, et al. <i>Nature Medicine</i> . 2022.
	<a href="#">Real-world rates of bleeding, factor VIII use, and quality of life in individuals with severe haemophilia A receiving prophylaxis in a prospective, noninterventional study</a> Kenet G, Chen Y-C, Lowe G, et al. <i>Journal of Clinical Medicine</i> . 2021.
Preclinical	<a href="#">Molecular analysis of AAV5-hFVIII-SQ vector-genome-processing kinetics in transduced mouse and nonhuman primate livers</a> Shin C-R, Handyside B, Liu S, et al. <i>Molecular Therapy: Methods &amp; Clinical Development</i> . 2022.
	<a href="#">Vector genome loss and epigenetic modifications impact long-term transgene expression of AAV5 vectors produced in mammalian HEK293 and insect Sf cells</a> Handyside B, Ismail AM, Zhang L, et al. <i>Mol Ther</i> . 2022.
	<a href="#">The Impact of Pre-existing Immunity on the Non-clinical Pharmacodynamics of AAV5-Based Gene Therapy</a> Long BR, Sandza K, Holcomb J, et al. <i>Molecular Therapy: Methods &amp; Clinical Development</i> . 2019.
	<a href="#">Gene Therapy with BMN 270 Results in Therapeutic Levels of FVIII in Mice and Primates and Normalization of Bleeding in Hemophilic Mice</a> Bunting S, Zhang L, Xie L, et al. <i>Molecular Therapy</i> . 2018.
	<a href="#">Validation of an IFN-gamma ELISpot assay to measure cellular immune responses against viral antigens in non-human primates</a> Yang F, Patton K, Kasprzyk T, et al. <i>Gene Therapy</i> . 2021.
Other	<a href="#">Activity of Transgene-Produced B-Domain Deleted Factor VIII in Human Plasma Following AAV5 Gene Therapy</a> Rosen S, Tiefenbacher S, Robinson M, et al. <i>Blood</i> . 2020.
	<a href="#">Assessing the potential cost-effectiveness of a gene therapy for the treatment of hemophilia A</a> Cook K, Forbes SP, Adamski K, et al. <i>Journal of Medical Economics</i> . 2020.
	<a href="#">Psychometric Validation of the Haemo-QOL-A in Participants with Hemophilia A Treated with Gene Therapy</a> Quinn J, Delaney KA, Wong WY, et al. <i>Patient Relat Outcome Meas</i> . 2022

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