

Key Publication List

Title	Author	Year
Multiyear Follow-up of AAV5-hFVIII-SQ Gene Therapy for Hemophilia A	Pasi KJ, et al	2020
AAV5-Factor VIII Gene Transfer in Severe Hemophilia A	Rangarajan S, et al	2017
Strategy to detect pre-existing immunity to AAV Gene Therapy	Falese L, et al	2017
Gene Therapy with BMN 270 Results in Therapeutic Levels of FVIII in Mice and Primates and Normalization of Bleeding in Hemophilic Mice	Bunting S, et al	2017
The impact on pre-existing immunity on the non-clinical pharmacodynamics of AAV5-based gene therapy	Long, BR, et al	2019
Assessing the potential cost-effectiveness of a gene therapy for the treatment of hemophilia A	Cook K, et al	2020
Activity of Transgene-Produced B-Domain Deleted Factor VIII in Human Plasma Following AAV5 Gene Therapy	Rosen S, et al	2020
Induction of ER Stress by an AAV5 BDD FVIII Construct Is Dependent on the Strength of the Hepatic-Specific Promoter	Fong S, et al	2020

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