

Hemophilia A & Hemophilia B

ARE UNIQUE BLEEDING DISORDERS

Management strategies should be individualized

Hemophilia A^{1,2}

Prevalence	1:5,000 males
Patients with severe factor VIII deficiency	~50%
Annual bleed rate in moderate to severe patients	14-16

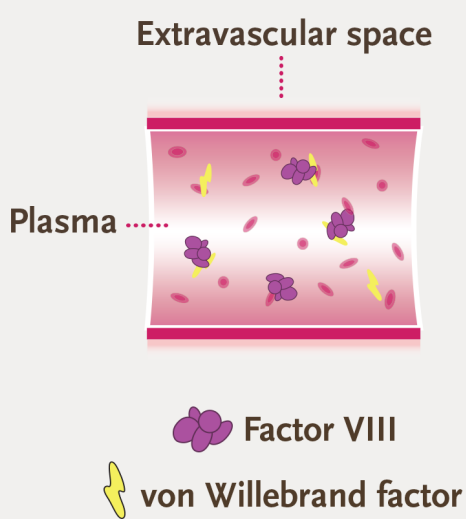


Hemophilia B^{1,2}

Prevalence	1:30,000 males
Patients with severe factor IX deficiency	~30%
Annual bleed rate in moderate to severe patients	9-11

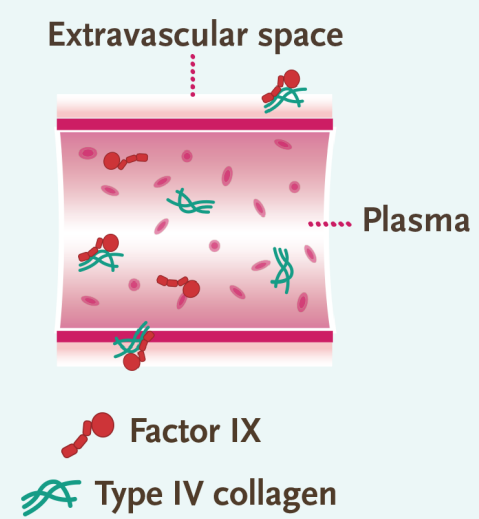
FACTOR VIII

Distribution is largely limited to the plasma and binds to von Willebrand factor⁴⁻⁶



FACTOR IX

Has a widespread distribution in the extravascular space and binds to type IV collagen^{4,7,8}



Based on a high Volume of distribution, THE MAJORITY OF FACTOR IX SPENDS ITS TIME IN THE EXTRAVASCULAR SPACE⁴

Due to the complexity of **FACTOR IX**, evaluation of FIX replacement therapies should **INCLUDE MULTIPLE PK PARAMETERS**⁴
FIX=factor IX.

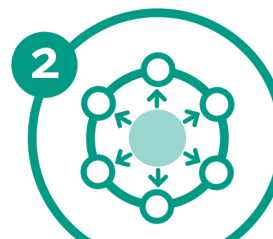
1 PK PARAMETER ≠ ALL FACTOR IX ACTIVITY^{9,10}
PK=pharmacokinetic.

THE **ISTH** RECOMMENDS that PK assessments include at least **6 PARAMETERS**³

ISTH=International Society on Thrombosis and Haemostasis.



1 Peak
Maximum concentration observed after infusion



2 Volume of distribution
Drug distribution in the plasma and rest of the body



3 Area under the curve
Total concentration in the body in a given period of time



4 Clearance
Amount of plasma free of drug in a given period of time



5 Half-life
Time to half of initial concentration



6 Trough
Minimum concentration

When assessing your patients with hemophilia B, consider a patient-centered approach that includes^{11,12}:



Bleed rates



Joint bleed prevention



Adherence



Quality of life

References: 1. Castaman G, et al. *Haematologica*. 2019;104(9):1702-1709. 2. American Thrombosis and Hemostasis Network. ATHN research report—ATHNdataset. Published June 30, 2019. 3. Ragni MV, et al. *J Thromb Haemost*. 2018;16(7):1437-1441. 4. Iorio A, et al. *Thromb Haemost*. 2017;117(6):1023-1030. 5. Lenting PJ. *J Thromb Haemost*. 2007;57:1353-1360. 6. Morfini M. *J Clin Med*. 2017;6(3):E35. 7. Gui T, et al. *Blood*. 2002;100(1):153-158. 8. Björkman S, et al. *Haemophilia*. 2013;196:882-886. 9. Dolan G. *Blood Rev*. 2018;32(1). doi: 10.1111/hae.14046. 10. Mann DM, et al. *Haemophilia*. 2021;27(3):332-339. 11. McLaughlin JM, et al. *Haemophilia*. 2014;20(4):506-512. 12. Srivastava A, et al. *Haemophilia*. 2020;26(suppl 6):1-158.