HEMOSTASIS 2.0

Rethinking Hemophilia Management with Novel Agents and Shared Decision Making

CME OUTFITTERS

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Learning Objectives

- Assess the clinical efficacy, durability in restoring hemostasis, and safety of new approaches for the management of hemophilia
- Develop a clinical and laboratory monitoring plan of the hemostatic status in patients receiving new therapies
- Implement shared decision making (SDM) strategies to better engage patients/caregivers with hemophilia in their treatment plan

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Transformational Care in Today's Therapeutic Landscape

Mark W. Skinner, JD

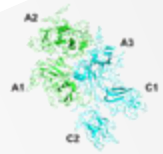
Therapeutic Evolution in a Nutshell

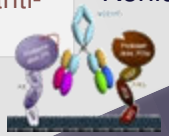
Factor Replacement
- Missing protein identified, purified, returned to PwH
- Viral inactivation
- Recombinant factor products
- Reduced volume
- Better storage/portability
- [FVIII, FIX concentrates]

Non-replacement, Rebalancing Therapies
- Metabolic manipulation
- Small molecules; SC dose
- Use with or without inhibitors
- [FVIII mimetics, anti-TFPI, anti-APC, AT-siRNA]

Gene Therapy
- Provides functional gene or edits abnormal gene
- Potential long-term cure or remission
- [FVIII and FIX products FDA approved]

Extended Half-life (EHL)
- Less frequent infusions
- Improved adherence
- Higher trough activity
- Better bleed protection
- [EHL rFVIII, EHL rFIX]





1900–1940s
Whole blood

1950s–1960s
Fresh frozen plasma

1964
Cryoprecipitate

1968
Commercially available FVIII

1985
Viral inactivation

1990s
Recombinant FVIII/FIX

2014
EHL factors

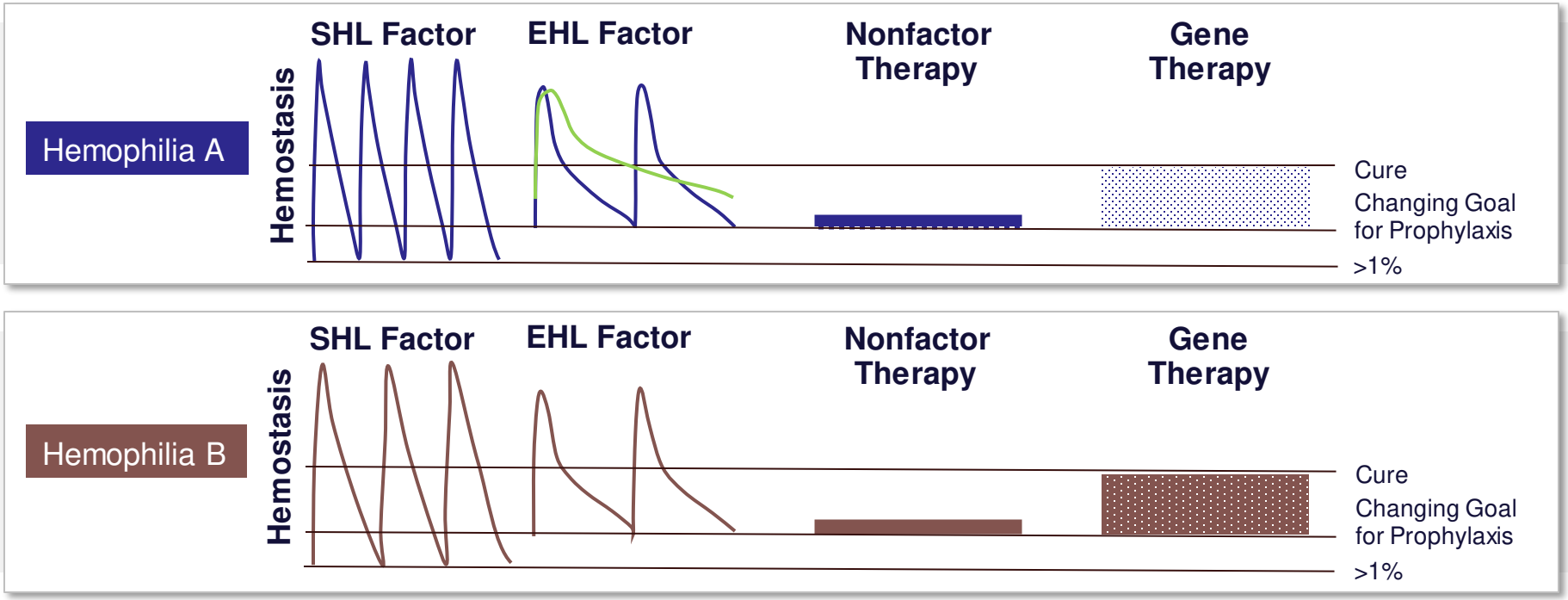
2017–2020s
Nonfactor treatment

2010s–2020s
Gene treatment

APC, activated protein C; AT, antithrombin; FIX, factor IX; FVIII, factor VIII; PwH, person with hemophilia; r, recombinant; RNA, ribonucleic acid; SC, subcutaneous; si, small interfering; TFPI, tissue factor pathway inhibitor.

Ozelo MC, Yamaguti-Hayakawa GG. *Res Pract Thromb Haemost*. 2022;6:e12695.

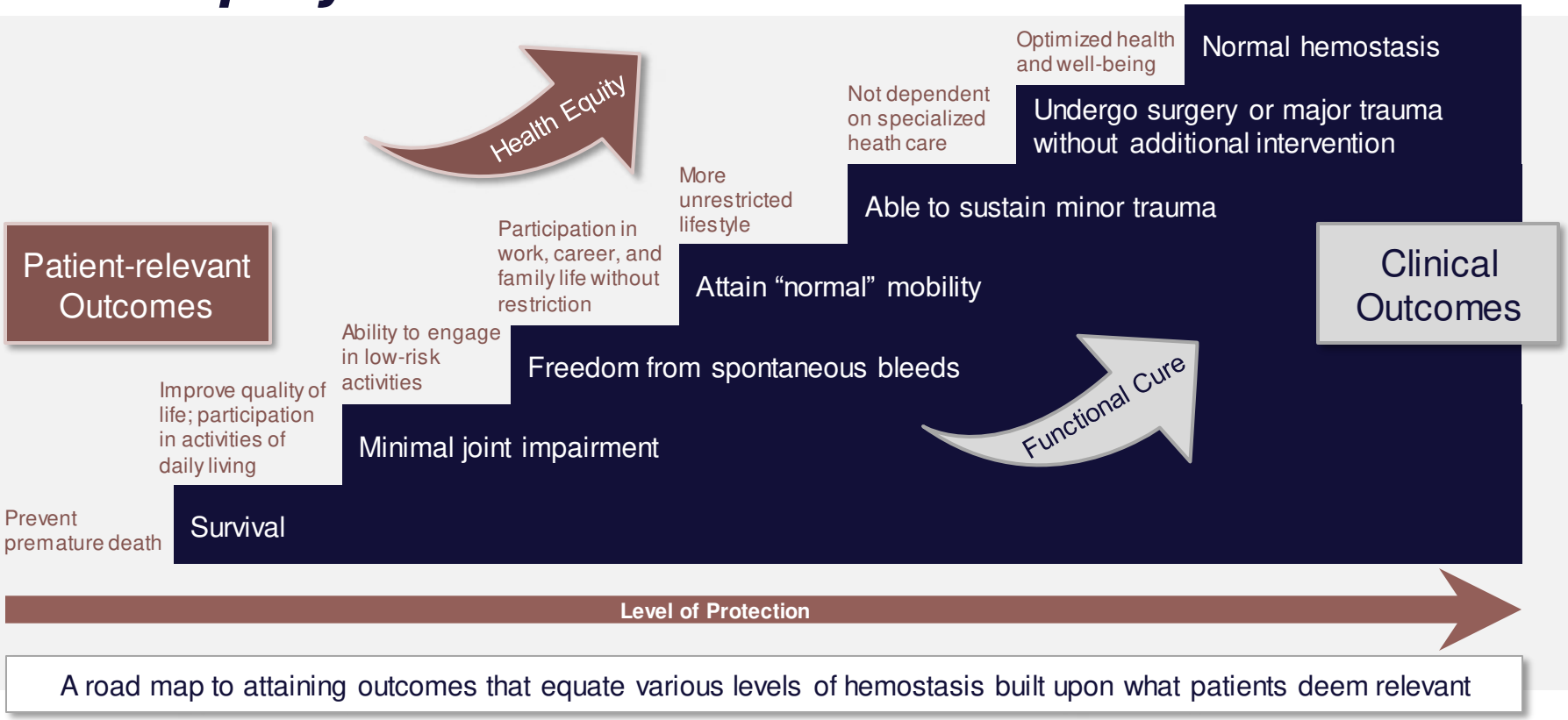
Goal of Therapy
Stable Hemostatic Levels



EHL, extended half-life; SHL, standard half-life.

Adapted from Arruda VR, et al. *Blood.* 2017;130:2251–2256.

Achieving the Unimaginable
Health Equity



A road map to attaining outcomes that equate various levels of hemostasis built upon what patients deem relevant

Skinner MW, et al. *Haemophilia*. 2020;26:17–24.

PART 1

Mechanism of Action and Efficacy of Novel Agents

Amy D. Shapiro, MD

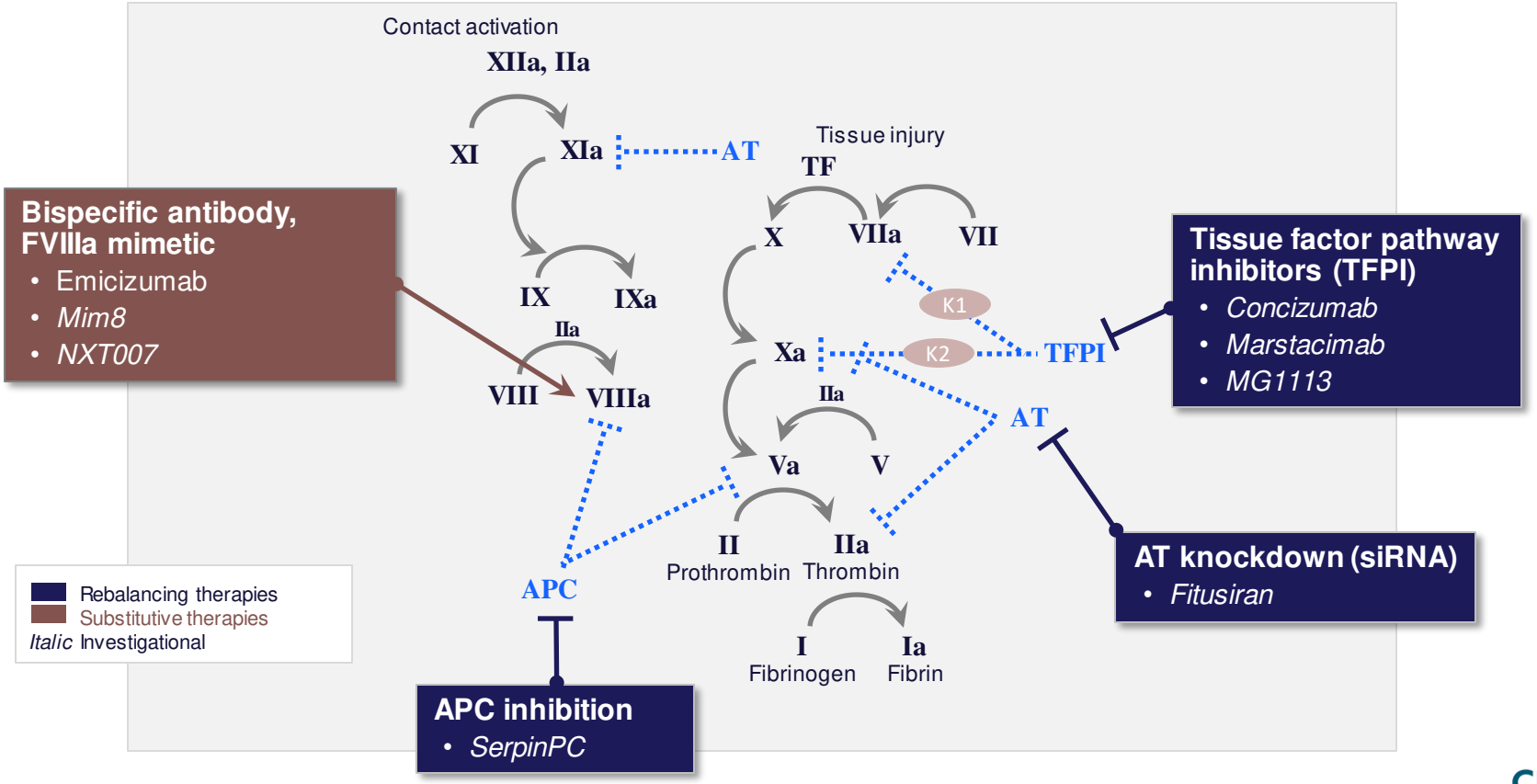
Mechanism of Action

Mimetics, Anti-TFPI, siRNA-AT

Which of the following novel therapeutics has reported a 15-fold increased potency compared to emicizumab, which may allow for lower dosing volumes?

A. Concizumab
B. Mim8
C. Fitusiran
D. SerpinPC
E. I'm not sure

Novel Therapeutics to Treat Hemophilia A or B ± Inhibitors

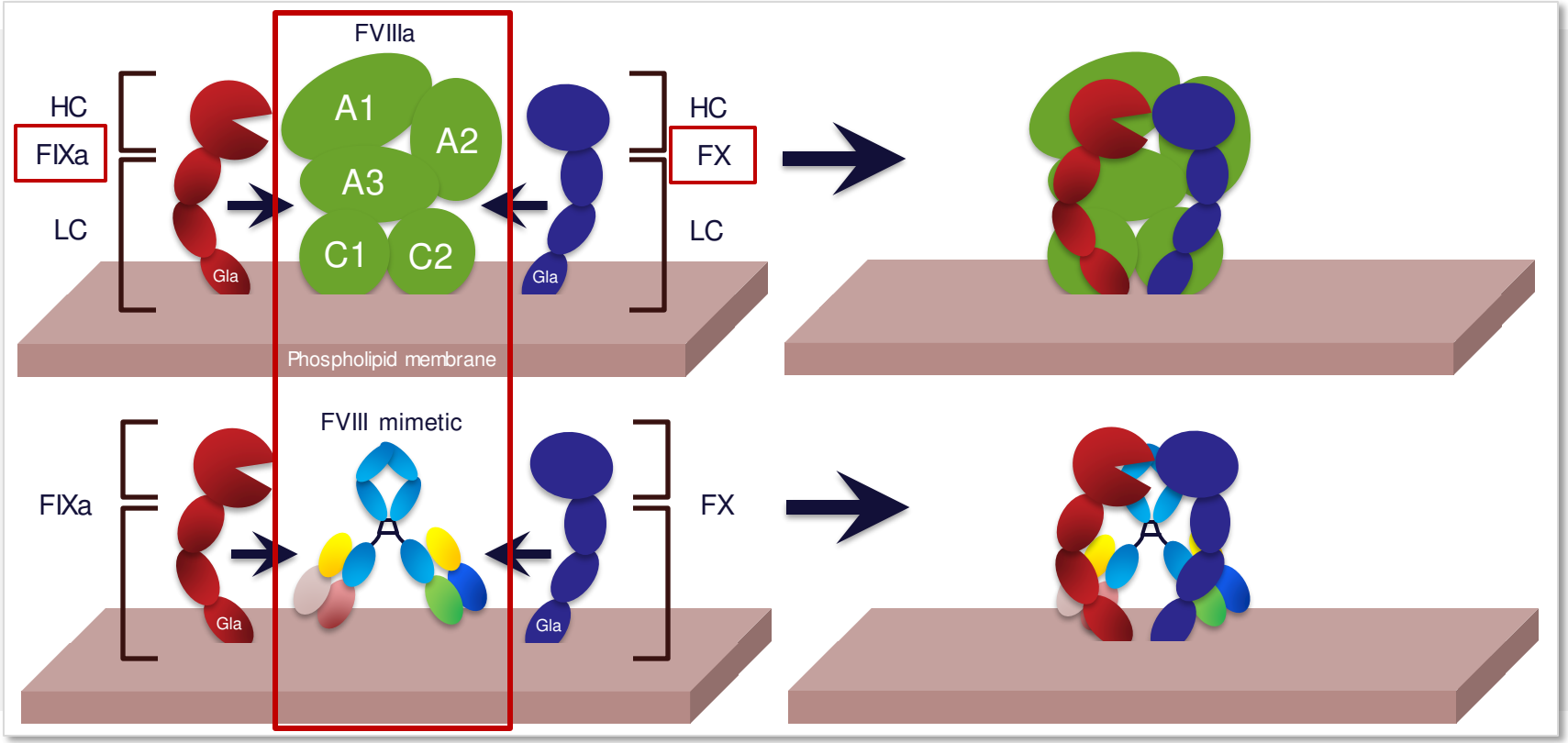


TF, tissue factor.

Croteau SE, et al. *Am J Hematol.* 2021;96(1):128–144.

Factor VIII vs FVIII Mimetics

MOA Comparison



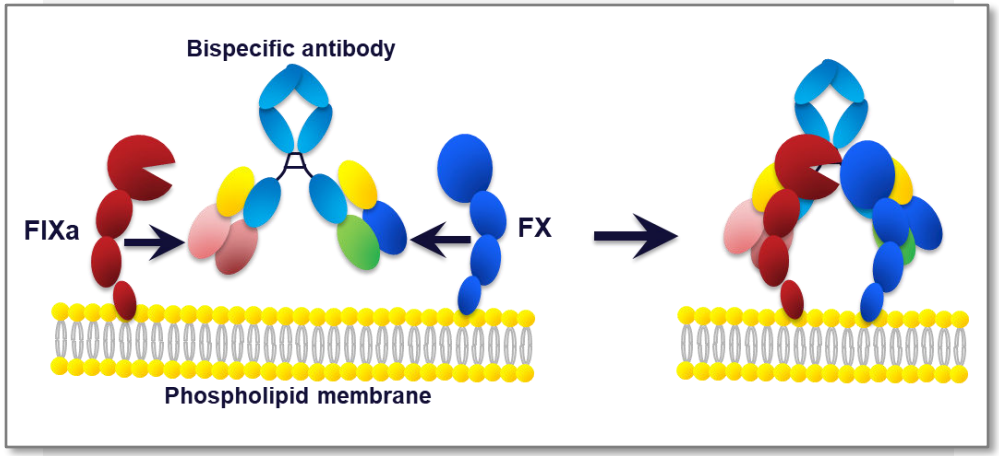
HC, high concentration; LC, low concentration; MOA, mechanism of action.

Sampei Z, et al. *PLoS One*. 2013;8(2):e57479. Lenting PJ, et al. *Blood*. 2017;130 (23):2463–2468.

FVIIIa Mimetics
Bispecific Antibodies for Hemophilia A ± Inhibitors

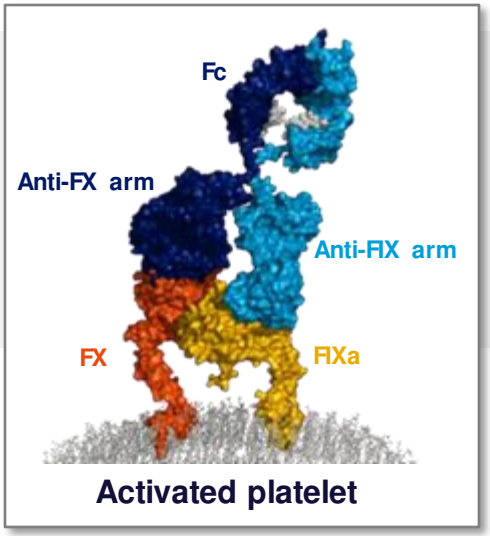
Emicizumab

- FDA approved 2017–2018
- Subcutaneous (SC) administration
- Flexible dosing regimens
- Long half-life (26.9 ± 9.1 days)
- Decreased treatment burden, especially with inhibitors



Sampei Z, et al. *PLoS One.* 2013;8(2):e57479.
FDA-approved drug: emicizumab-kxwh. Revised January 2024. https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/761083s018lbl.pdf.
Zhou Z-Y, et al. *J Manag Care Spec Pharm.* 2020;26(9):1109–1120.
Skinner MW, et al. *Haemophilia.* 2021;27:854–865.

FVIIIa Mimetics
Bispecific Antibodies for Hemophilia A ± Inhibitors

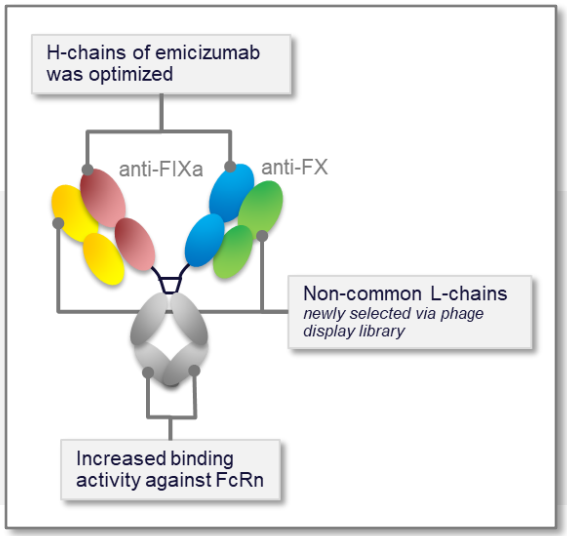


Mim8

- Currently in phase 3 trials
- Preclinical models: potency ~15-fold higher than emicizumab analog

NXT007

- Phase 1 clinical trial showed 10-week half-life
- Engineered and optimized based on emicizumab



Fc, fragment crystallizable; FcRn, neonatal crystallizable fragment receptor.

Sampei Z, et al. *PLoS One*. 2013;8(2):e57479. Lentz SR, et al. *J Thromb Haemost*. 2024;22:990–1000. Teranishi-Ikawa Y, et al. *J Thromb Haemost*. 2024;22:430–440.

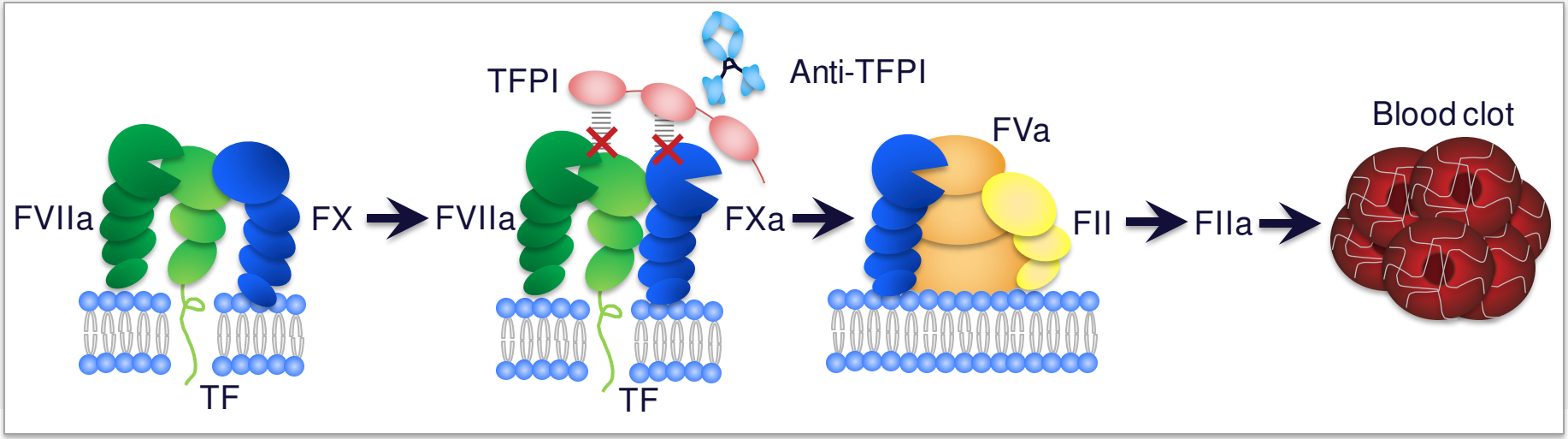
Anti-TFPIs in Development for Hemophilia ± Inhibitors

Concizumab

- Assessed in explorer trials
- Approved in Canada (FIX with an inhibitor)
- Approved in Japan (FVIII or FIX with inhibitors); under FDA review in the United States
- SC, once-daily, custom pen

Marstacimab

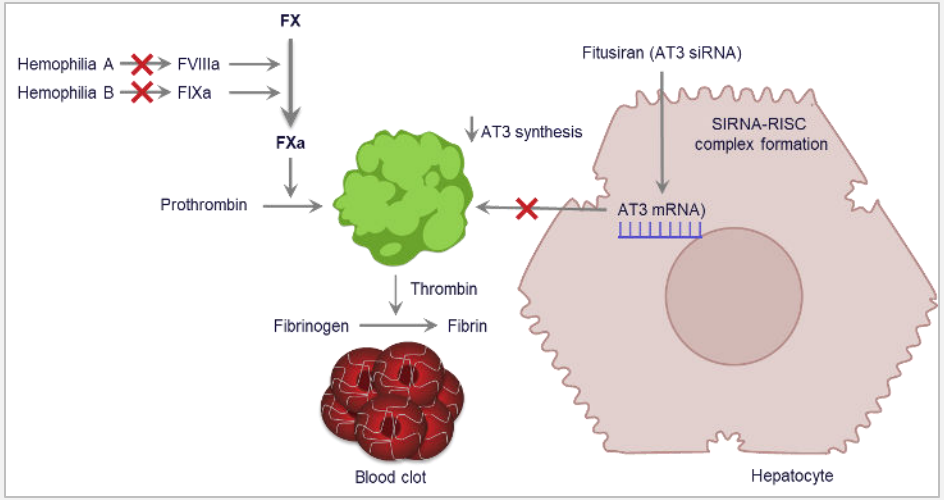
- Phase 3 BASIS trial
- Under regulatory review in the United States and the European Union (EU)
- Once weekly SC dosing



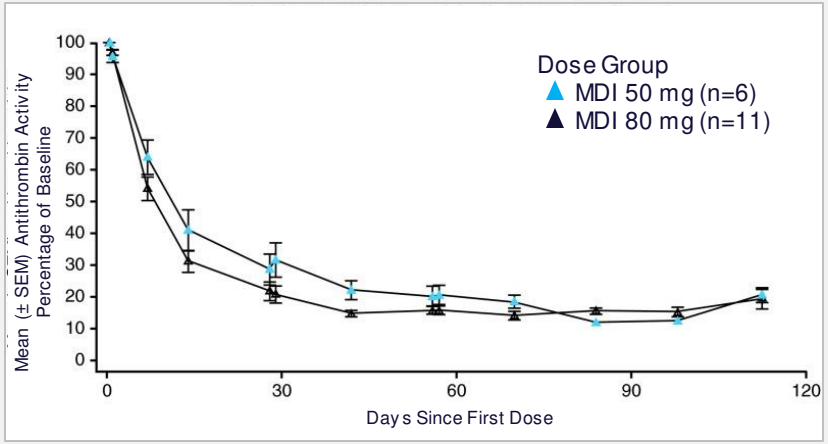
Matsushita T, et al. *N Engl J Med.* 2023;389:783–794. Keam SJ. *Drugs.* 2023;83(11):1053–1059.
Matino D, et al. *Blood.* 2023;142(Suppl 1):285. Chowdary P. *Drugs.* 2018;78(9):881–890.

Fitusiran
SC siRNA Targeting Antithrombin

MOA of Fitusiran

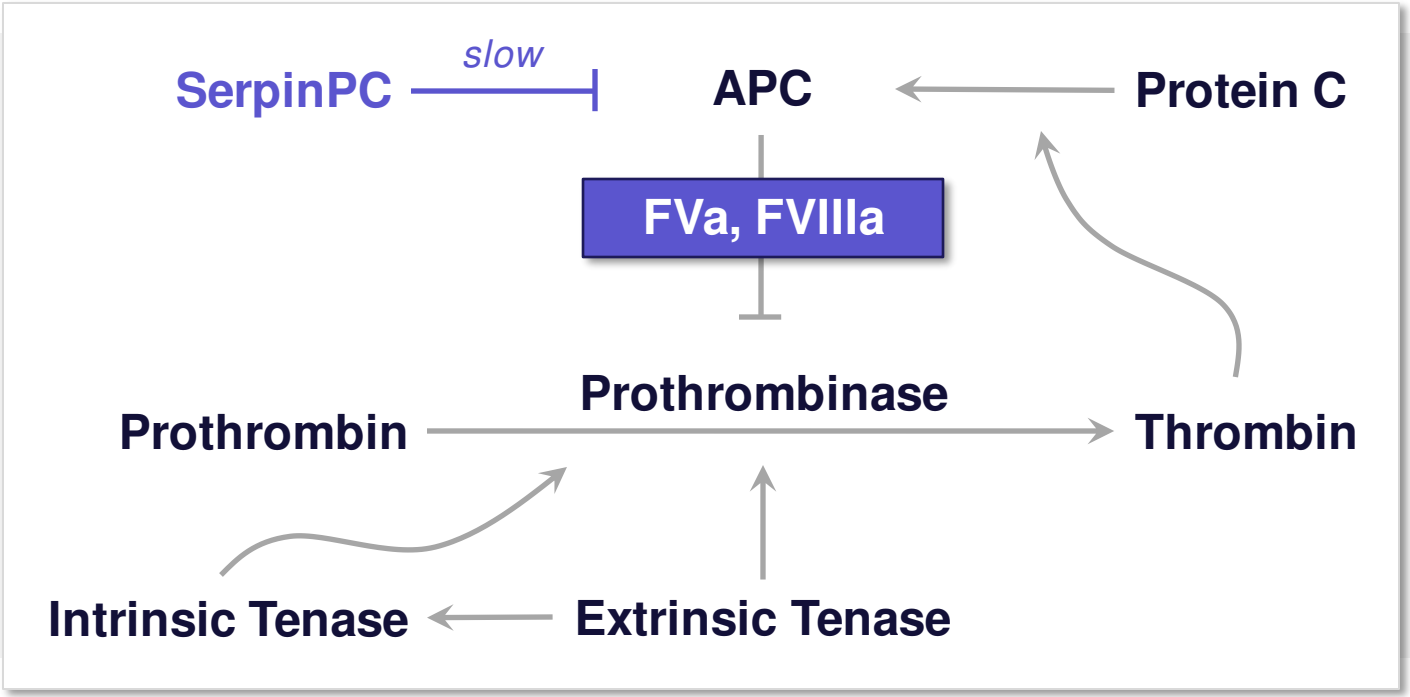


Effect of Fitusiran on AT Activity



Boyce S, Rangarajan S. *J Blood Med.* 2023;14:317–327. Pasi JK, et al. *J Thromb Haemost*. 2021;19(6):1436–1446.

SerpinPC
(Recombinant Serine Protease Inhibitor)



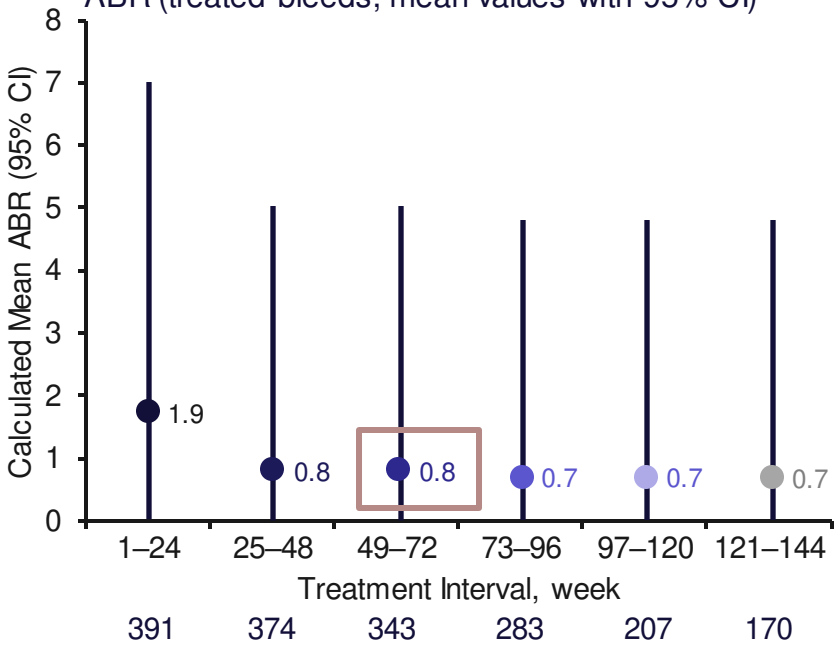
Baglin T, et al. *Blood.* 2023;142(Suppl 1):2619. Polderdijk S, et al. *Curr Opin Hematol.* 2017;24:446–452.

Efficacy Summary

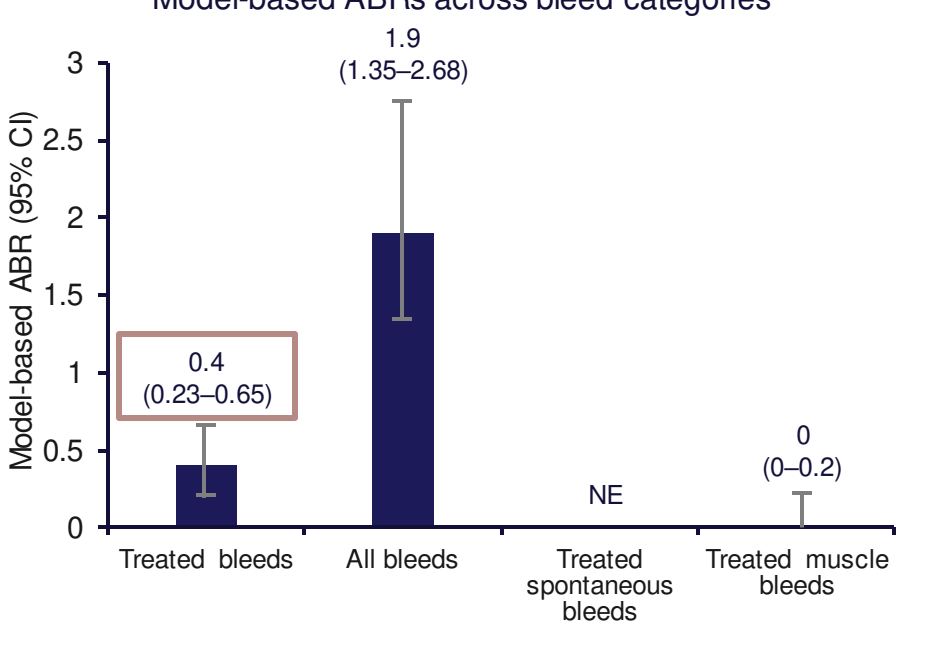
Mimetics, Anti-TFPI, siRNA-AT

Emicizumab Phase 3

HAVEN 1–4[1]: Pooled analysis of long-term results in PwHA with or without inhibitors



HAVEN 7[2]: Model-based ABRs across bleed categories in infants with HA



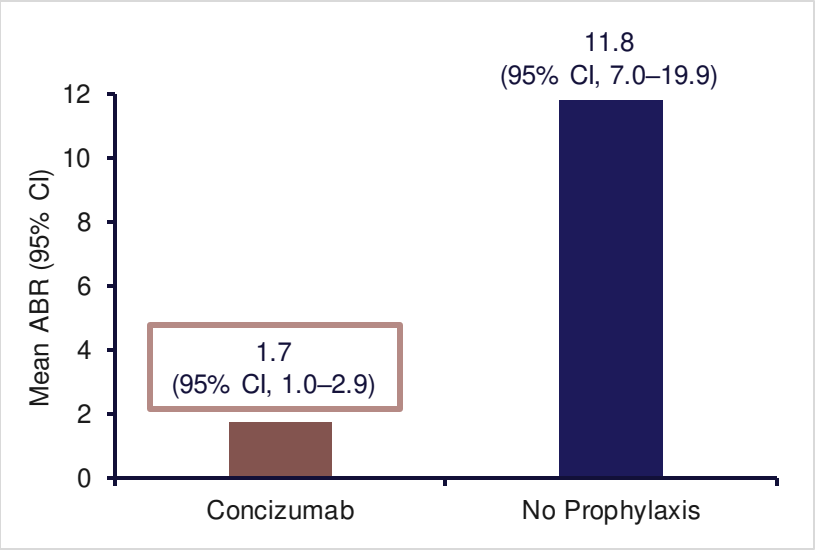
[1]Callaghan M, et al. *Blood.* 2021;137:2231–2242. [2]Pipe SW, et al. *Blood.* 2023;202321832.

ABR, annualized bleeding rate; CI, confidence interval; HA, hemophilia A.

Concizumab Phase 3

explorer7[1]: Patients with HA or HB with inhibitors

Estimated mean ABR
Rate ratio, 0.14 (95% CI, 0.07–0.29); *P*<0.001



explorer8[2]: Spontaneous and traumatic bleeding episodes by HA/HB at the 56-week cut-off

		Concizumab Prophylaxis (arms 1–4)	
		Hemophilia A	Hemophilia B
N in full analysis set		80	64
Patient years of exposure in analysis data set		111.9	71.7
Treated spontaneous and traumatic bleeding episodes			
Number of bleeding episodes		349	302
ABR	Median (interquartile range)	1.7 (0.0–4.5)	2.8 (0.0–6.4)
	Mean (standard deviation)	3.9 (6.6)	6.4 (14.2)
	Min; max	0.0; 37.1	0.0; 91.3

[1]Matsushita T, et al. *N Engl J Med*. 2023;389:783–794. [2]Astermark J, et al. *Blood*. 2023;142(Suppl 1):2609.

HB, hemophilia B.

Marstacimab Phase 3

BASIS[1]: Severe HA or moderately severe to severe HB, with or without inhibitors

Treatment Group	Factor Replacement Treatment Received during OP (n=116)	Marstacimab Prophylaxis during ATP (n=116)	Marstacimab Prophylaxis during LTE (n=87)
OD	OD	Marstacimab	Marstacimab
Mean ABR[a] (95% CI)	**(n=33)** 38.00 (31.03–46.54)	**(n=33)** 3.18 (2.09–4.85)	**(n=29)** 3.86 (2.02–7.37)
Rate estimate (95% CI), *P*-value[b]	0.084 (0.059, 0.119), *P*<0.0001		—
RP	RP	Marstacimab	Marstacimab
Mean ABR[a] (95% CI)	**(n=83)** 7.85 (5.09–10.61)	**(n=83)** 5.08 (3.40–6.77)	**(n=58)** 2.27 (1.40–3.67)
Rate estimate (95% CI), *P*-value[c]	-2.77 (-5.37, -0.16), *P*=0.0376		—

[a]Model-derived ABR
[b]*P*-values for the null hypothesis that the ration = ½ for all bleed related parameters
[c]*P*-value if superiority met

ATP, 12-month active treatment phase; LTE, long-term extension study; OD, on demand; OP, 6-month observation phase; RP, routine prophylaxis.

Matino D, et al. *Blood.* 2023;142(Suppl 1):285.

Fitusiran Phase 3

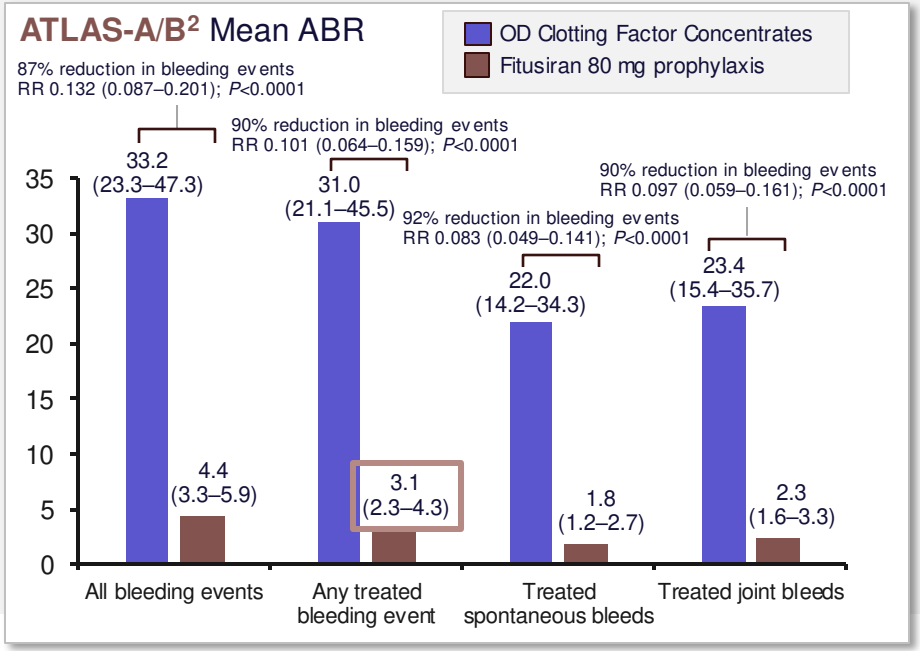
ATLAS-INH[1]	Bypassing Agent On-demand Group (n=19)	Fitusiran Prophylaxis Group (n=38)	*P*-value
Primary efficacy outcome			
Mean ABR estimated by negative binomial model	18.1 (10.6–30.8)	1.7 (1.0–2.7)	*P*<0.0001
Observed median ABR	16.8 (6.7–23.5)	0.0 (0.0–1.7)	NR
Participants with zero bleeds	1 (5%)	25 (66%)	NR

ATLAS-INH[1]: HA or HB with inhibitors

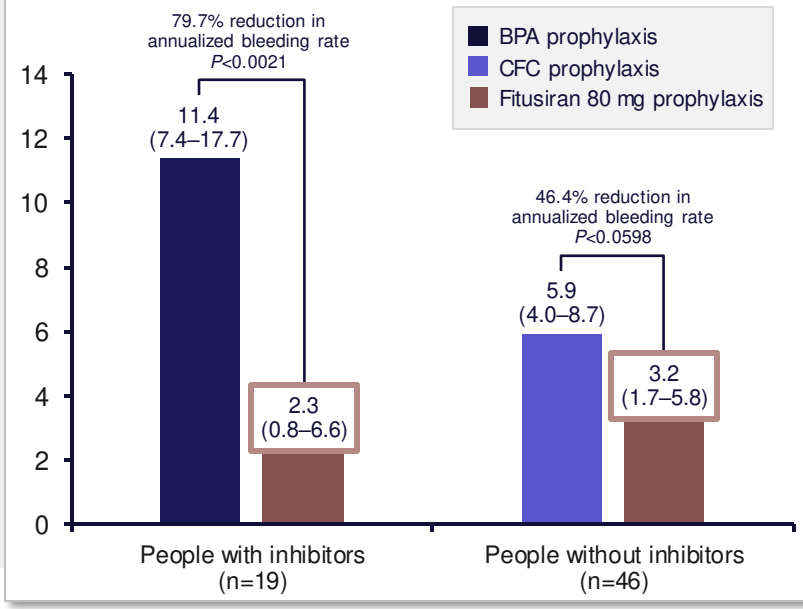
ATLAS-A/B[2]: HA or HB without inhibitors

ATLAS-PPX[3]: HA or HB with or without inhibitors who have switched from prior clotting factor concentrate (CFC) or bypassing agent (BPA) prophylaxis

ATLAS-A/B[2] Mean ABR



ATLAS-PPX[3] Mean ABR



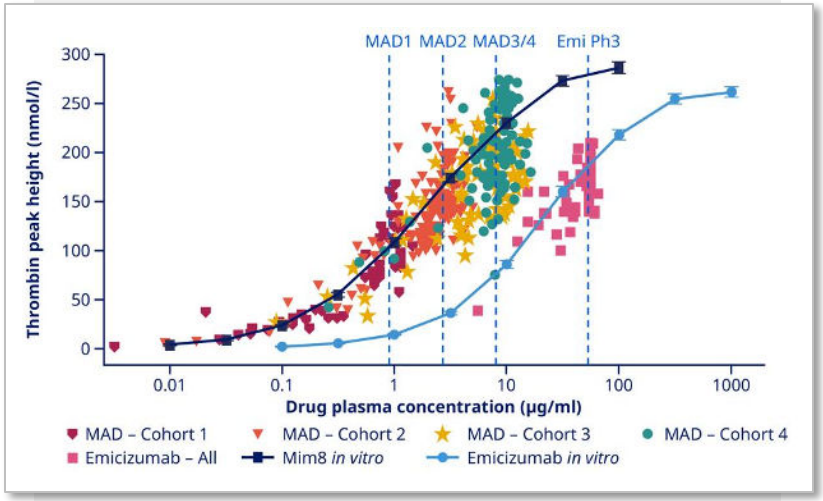
[1]Young G, et al. *Lancet.* 2023;401(10386):1427–1437.
[2]Srivastava A, et al. *Lancet Haematol.* 2023;10(5):e322–e332. [3]Kenet G, et al. *HemaSphere.* 2023;7(S3):e643526e.

Factor VIII Mimetics in Development

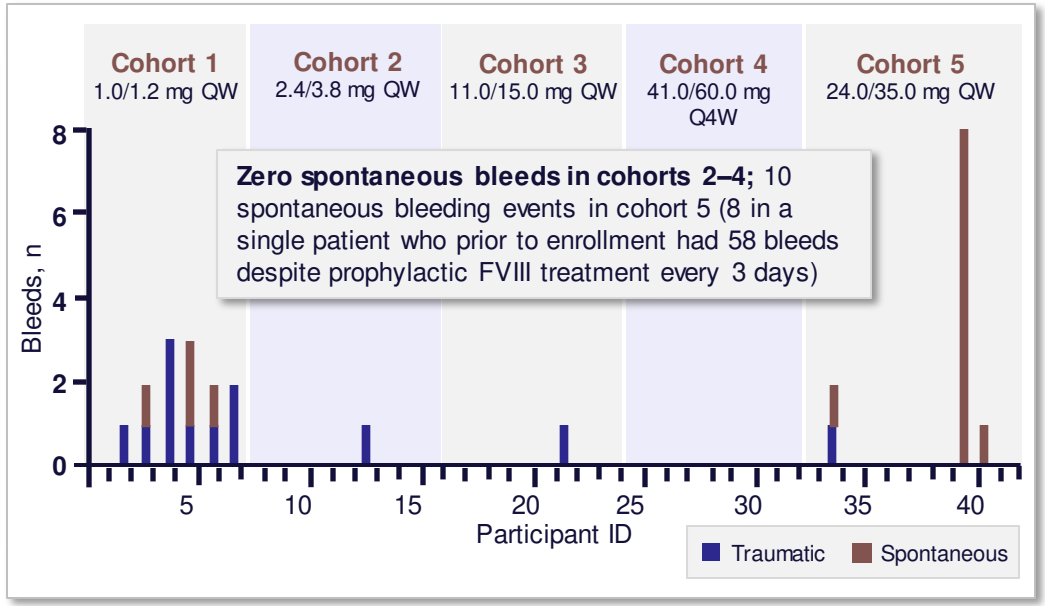
Mim8 and NXT007

Mim8 *(FRONTIER 1/2)*

Thrombin Peak Height vs Drug Plasma Concentration



Observed Treated Bleeds from the Multiple Ascending Dose (MAD) Cohorts

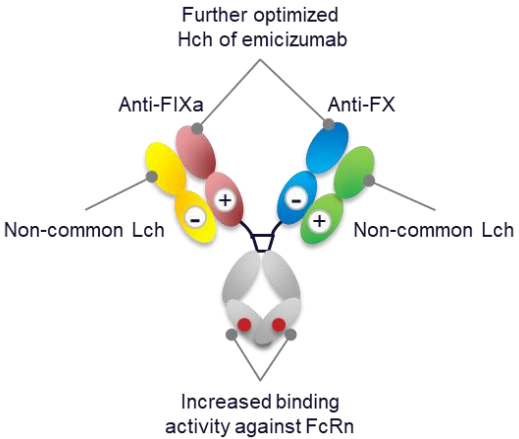


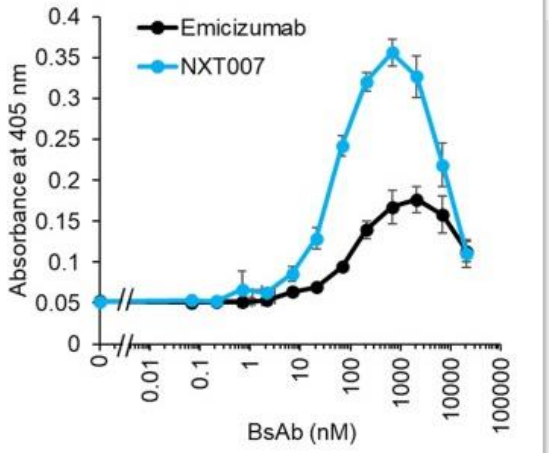
- In vitro, Mim8 was 15× more potent than emicizumab

Lentz S, et al. EAHAD 2023. Abstract PO-072. Lentz SR, et al. *J Thromb Haemost*. 2024;22:990–1000.

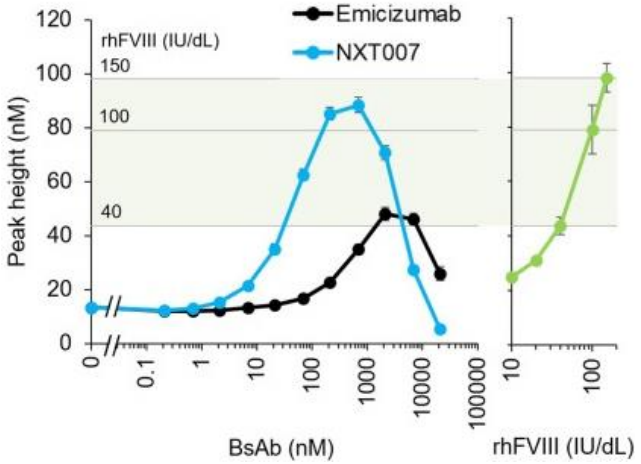
NXT007
A Bispecific Antibody That Mimics the Cofactor Function of FVIIIa

Molecular Features of NXT007





Effect of NXT007 or emicizumab on FIXa-catalyzed FX activation in an enzymatic assay using purified coagulation factors



Effect of NXT007, emcizumab, or rhFVIII on the peak height of thrombin generation using FVIII-deficient patient plasma

Teranishi-Ikawa Y, et al. *J Thromb Haemost*. 2024;22(2):430–440.

PART 2

Thrombotic Risk Mitigation and Coagulation Assays

Allison D. Wheeler, MD, MSCI

Thrombotic Risk Mitigation

Thromboembolic Events Reported during Trials

- Emicizumab (HAVEN)
- Concizumab (explorer)
- Fitusiran (ATLAS)

Risk mitigation strategies put in place: dosing adjustments and guidance for management of mild/moderate bleeds

Concizumab
Thrombotic Events (3) in 3 Patients Resulting in Trial Pause

PwH	Age Range (years)	Time on Concizumab	Thrombotic Event (all non-fatal)	Baseline Thrombotic Risk?*	Concomitant Hemostatic Medication on Day of or Days up to Event Onset?
HA	45–50	2 months	Acute myocardial infarction	Yes	Yes
HBwI	25–30	3 weeks	Renal infarction	Yes	Yes
HA	40–45	3 months	DVT, PE, superficial thrombosis of vein (left elbow region at site of FVIII injection)	Yes	Yes

*One patient (in explorer7) had obesity, hypercholesterolemia, and multiple removals and replacements of a central venous access device. One patient (in explorer8) had obesity, lower leg edema, and hypertension. A second patient in explorer8 had a history of smoking, hypertension with occasional use of ACE inhibitors, increased BP at screening, chronic tooth inflammation followed by extraction, and occasional chest pain for the month preceding the thromboembolism in the other patient.

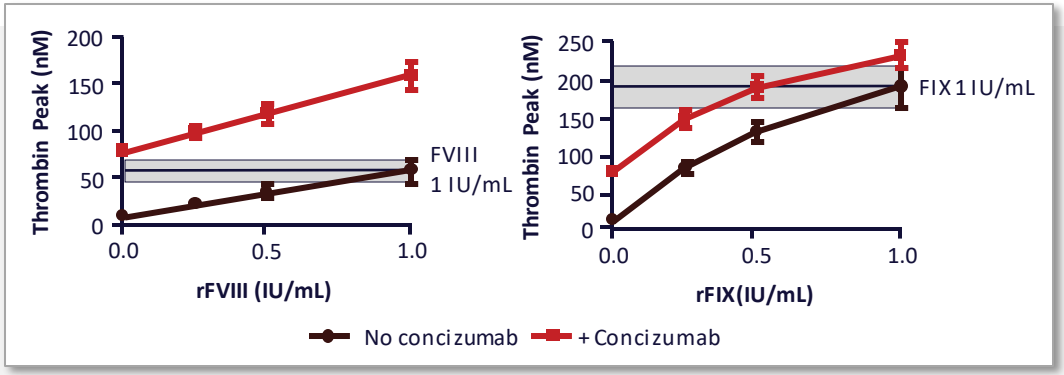
- In March 2020, study was paused for evaluation of trial data and development of mitigation strategy

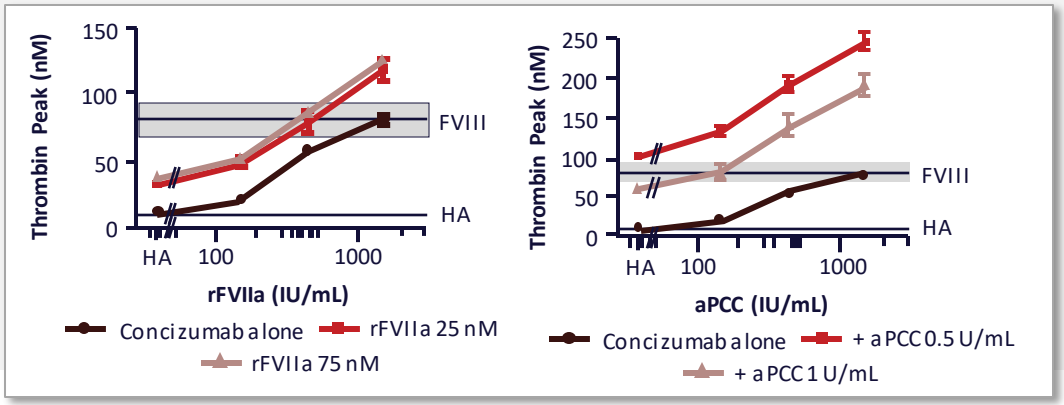
DVT, deep vein thrombosis; PE, pulmonary embolism.

Seremetis S, et al. *Blood.* 2020;136:40. Shapiro AD, et al. *Blood Adv.* 2022;6(11)a;3422–3432.
Matsushita T, et al. *N Engl J Med.* 2023;389(9):783–794.

Concizumab Phase 3 Trials
Risk Mitigation

- Assessment included clinical review and nonclinical data
  - Pharmacokinetic profile of patients based on population PK modeling
  - Thrombin generation studies with concomitant FVIII, FIX, FVIIa, and aPCC
- Risk mitigation
  - ELISA-based concizumab dose adjustments
    - Therapeutic: 200–4,000 ng/mL
  - Decreased factor dosing to the lowest approved dose for each product when treating mild/moderate bleeds





aPCC, activated prothrombin complex concentrate; PK, pharmacokinetics.

Kjalke M, et al. *J Thromb Haemost*. 2021;19(7):1687–1696.

Fitusiran
Thrombotic Events Resulting in Trial Pause

- Evaluation of thrombotic events as of October 2020 leading to trial pause and subsequent mitigation strategy

PwH	Age Range (y)	Medical History/Comments	AT Category	Thrombotic Event
HA	30–40	DVT (not identified at enrollment), T2D, obesity, HCV, tobacco use	<10%	CVA
HA	>60	Well-controlled HIV, HCV, and prostate cancer status post-radical prostatectomy (recent PSA WNL)	<10%	Cerebral infarct
HAwI	20–30	Suspected thrombosis involving a spinal injury	<10%	Spinal vascular disorder
HBwI	20–30	Concomitant use of BPA (rFVIIa) in excess of current bleed management guidelines in fitusiran studies	10%–20%	Atrial thrombosis
HA	20–30	Concomitant use of factor concentrate in excess of current bleed management guidelines (event initially misdiagnosed and treated as a subarachnoid hemorrhage resulting in fatal outcome)	10%–20%	Cerebral venous sinus thrombosis

HAwI/HBwI, hemophilia A/B with inhibitors; HCV, hepatitis C virus; HIV, human immunodeficiency virus; PSA, prostate-specific antigen; T2D, type 2 diabetes; WNL, within normal limits.

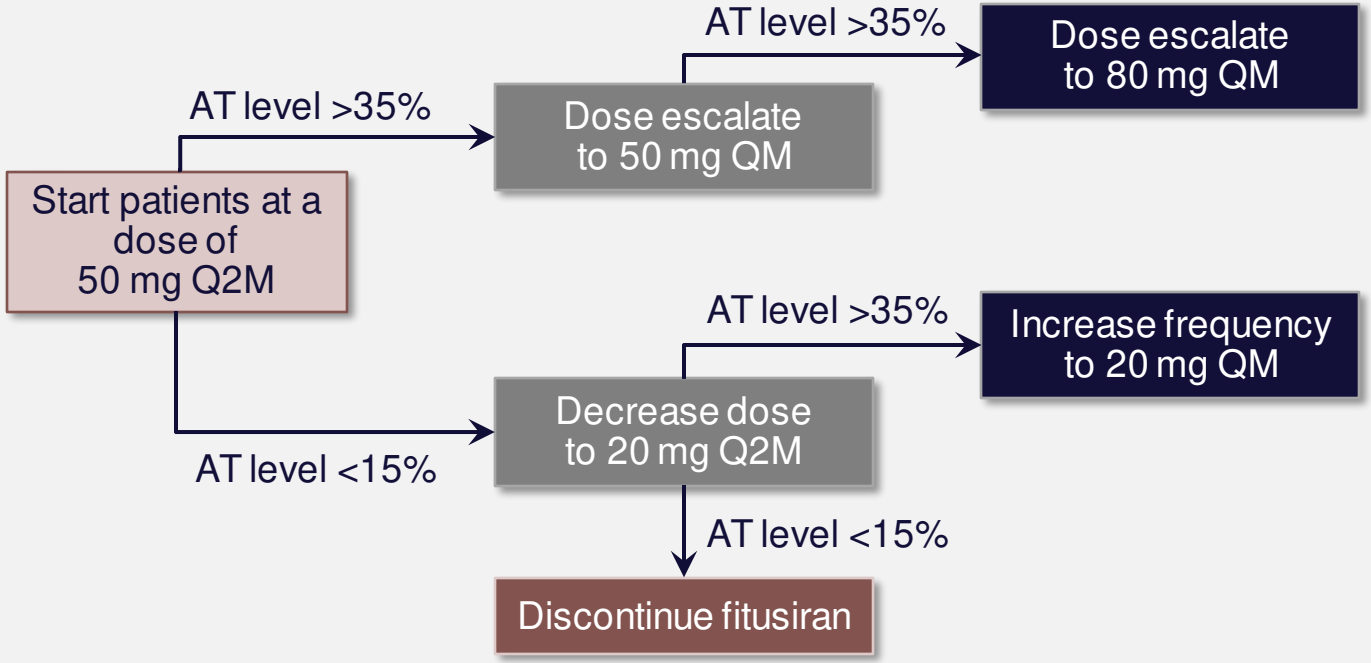
Young G, et al. *Res Pract Thromb Haemost*. 2023;7(4):100179.

Fitusiran Revised Dosing

Targeting AT Range from ≥15% to ≤35%

Based on fitusiran's MOA and observed AT activity <10% in clinical trial participants with reported vascular thrombotic events, AT activity was evaluated as a potential modifiable target for risk mitigation.

A simulation based on PK/PD modeling identified a dose and regimen targeting AT activity between 15% and 35%.



PD, pharmacodynamic.

Young G, et al. *Res Pract Thromb Haemost*. 2023;7(4):100179.

Coagulation Assays and Non-Factor Products

Which clinically-available, standard coagulation tests measure anti-TFPI hemostatic activity?

A. PTT and PT
B. D-dimer
C. Fibrinogen
D. Standard tests not applicable
E. I’m not sure

PT, prothrombin time; PTT, partial thromboplastin time.

Assays to Assess FVIII Mimetics

Assay to Determine Drug Is Present

- aPTT normalized
  - FVIII activity is ↑↑↑
- Human chromogenic FVIII provides some measure of equivalence
- Bovine chromogenic assays used to
  - Determine level of exogenous FVIII administered
  - Measure FVIII inhibitor
- Drug level

Evaluation of Efficacy

- Clinical monitoring of bleeding events used to assess efficacy
- aPTT prolonged determine if
  - Patient taking drug ($t_{1/2}$ is long)
  - Drug is functional
- Human chromogenic FVIII activity and inhibitor to assess for neutralizing antibody

aPTT, activated partial thromboplastin time; $t_{1/2}$, half-life.

Jenkins PV, et al. *Haemophilia.* 2020;26(1):151–155.

Assays to Assess Anti-TFPI Antibodies

Assay to Determine Drug Is Present

- Drug levels
  - Concizumab level will be available to direct drug dosing at 1 month
  - Marstacimab level reported in the trial manuscripts

Evaluation of Efficacy

- Clinical monitoring of bleeding events used to assess efficacy
- Assays to determine activity of agent are not standard
  - TFPI measurements
    - Concizumab: ↓ free TFPI
    - Marstacimab: ↑ total TFPI
  - ↑ Thrombin generation
  - ↑ D-dimers/PF 1.2

Lenting PJ. *Blood Adv.* 2020;4(9):2111–2118. Anandani G, et al. *Cureus*. 2022;14(10):e30212.

Assays to Assess Fitusiran

Assay to Determine Drug Is Present

- ↓ AT level demonstrates drug activity

Evaluation of Efficacy

- Clinical monitoring of bleeding events used to assess efficacy
- Assays to determine activity of agent are not standard
  - ↑ Thrombin generation

Lenting PJ. *Blood Adv.* 2020;4(9):2111–2118. Anandani G, et al. *Cureus*. 2022;14(10):e30212.

Assays to Assess SerpinPC

Assay to Determine Drug Is Present

- No standard assay, SerpinPC concentration in clinical trial

Evaluation of Efficacy

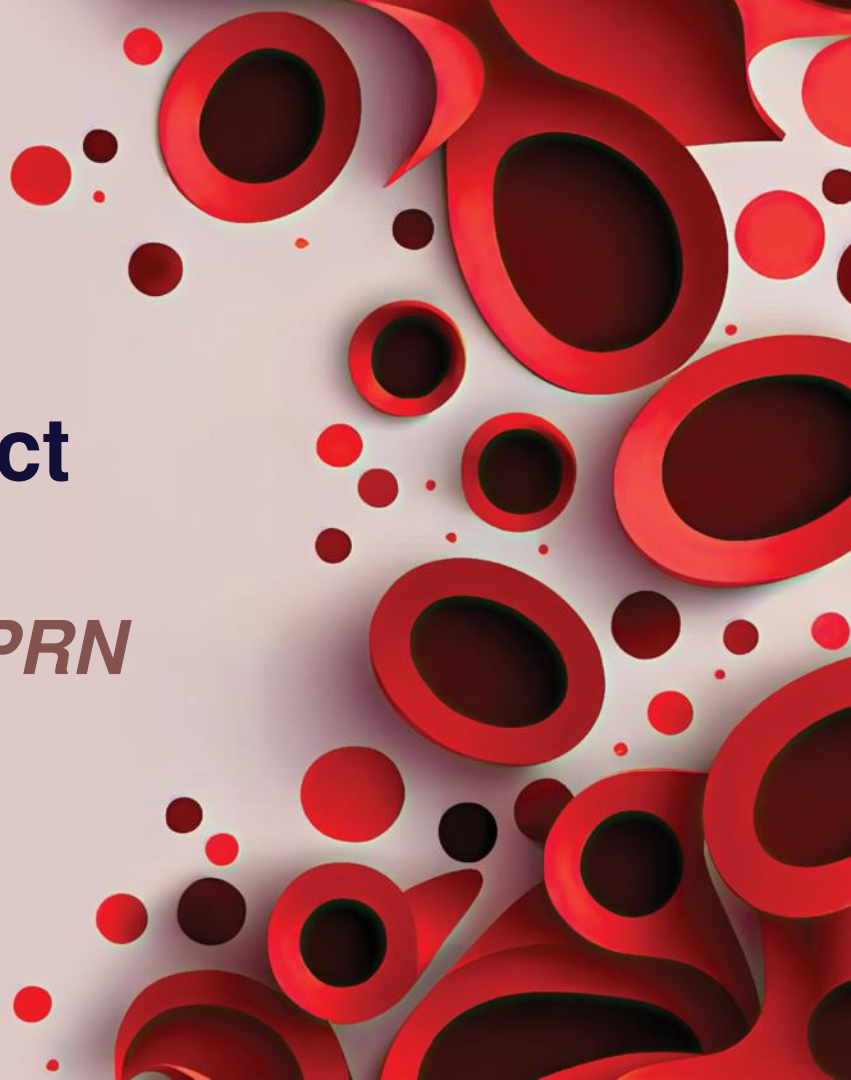
- Clinical monitoring of bleeding events used to assess efficacy
- Assays to determine activity of agent are not standard
  - ↑ Thrombin generation

Baglin T, et al. *Blood.* 2022;140(Suppl 1):460–461.

PART 3

Choosing the Best Product For and With the Patient

Maya C. Bloomberg, MSN, APRN
Mark W. Skinner, JD



According to the World Federation of Hemophilia (WFH) Shared Decision Making Guide, what is the recommended first step for patients?

A. Learn about the treatment options
B. Have an open and meaningful conversation with the healthcare team
C. Reflect on life goals and current treatment
D. Assess side effects of available treatments
E. I'm not sure

What Is Shared Decision Making?

A process wherein:

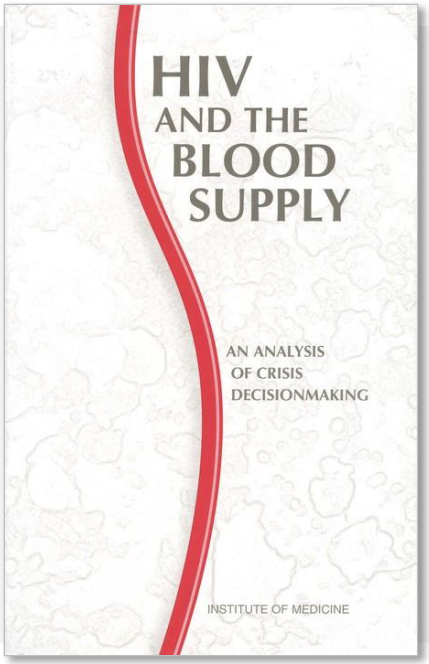
A patient shares with the provider all their aspirations, relevant values, preferences, and goals.

A health care provider shares with a patient all relevant information and best scientific evidence on the pros and cons of all potential treatment options.

With this mutual understanding, the **patient and provider decide** the best course of action.

Hemophilia Foundation Australia. 2023. https://www.haemophilia.org.au/national-haemophilia/no-223-september-2023/wfh-shared-decision-making-tool/.
Velentino LA, et al. *J Haem Pract.* 2021;8(1):69–79.

SDM Adopted in Hemophilia in 1980s



Blood safety is a **shared responsibility** of many diverse organizations, including manufacturers, **groups such as the NBDF** (formerly NHF), and others.

How is medical decision-making shared? The case of haemophilia patients and doctors: the aftermath of the infected blood affair in France

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2 July 2003

Keywords: AIDS, clinical relationship, decision-making, haemophilia, prosecution, sociology

Abstract

Objective This article looks at how users and doctors in France have rethought the question of shared decision-making in the clinical field of haemophilia following a major crisis – that of the infected blood affair.

Design We did a qualitative survey based on semi-structured interviews in three regions of France.

Setting and participants The interviews covered 31 clinical doctors of haemophilia and 31 users: 21 adult males with severe haemophilia (21/31), infected (14/21) or not (7/21) with HIV, the infected wife of one of the latter (1/31) and nine parents of young patients with severe haemophilia (9/31), either HIV positive (6/9) or negative (3/9).

NBDF, National Bleeding Disorders Foundation; NHF, National Hemophilia Foundation.

Institute of Medicine Committee to Study HIV Transmission through Blood and Blood Products. Leveton LB, et al, eds. HIV and the Blood Supply: An Analysis of Crisis Decisionmaking. National Academies Press (U.S.). 1995. https://www.ncbi.nlm.nih.gov/books/NBK232417/.
Fillion, M. *Health Expect.* 2003;6(3):228–241.

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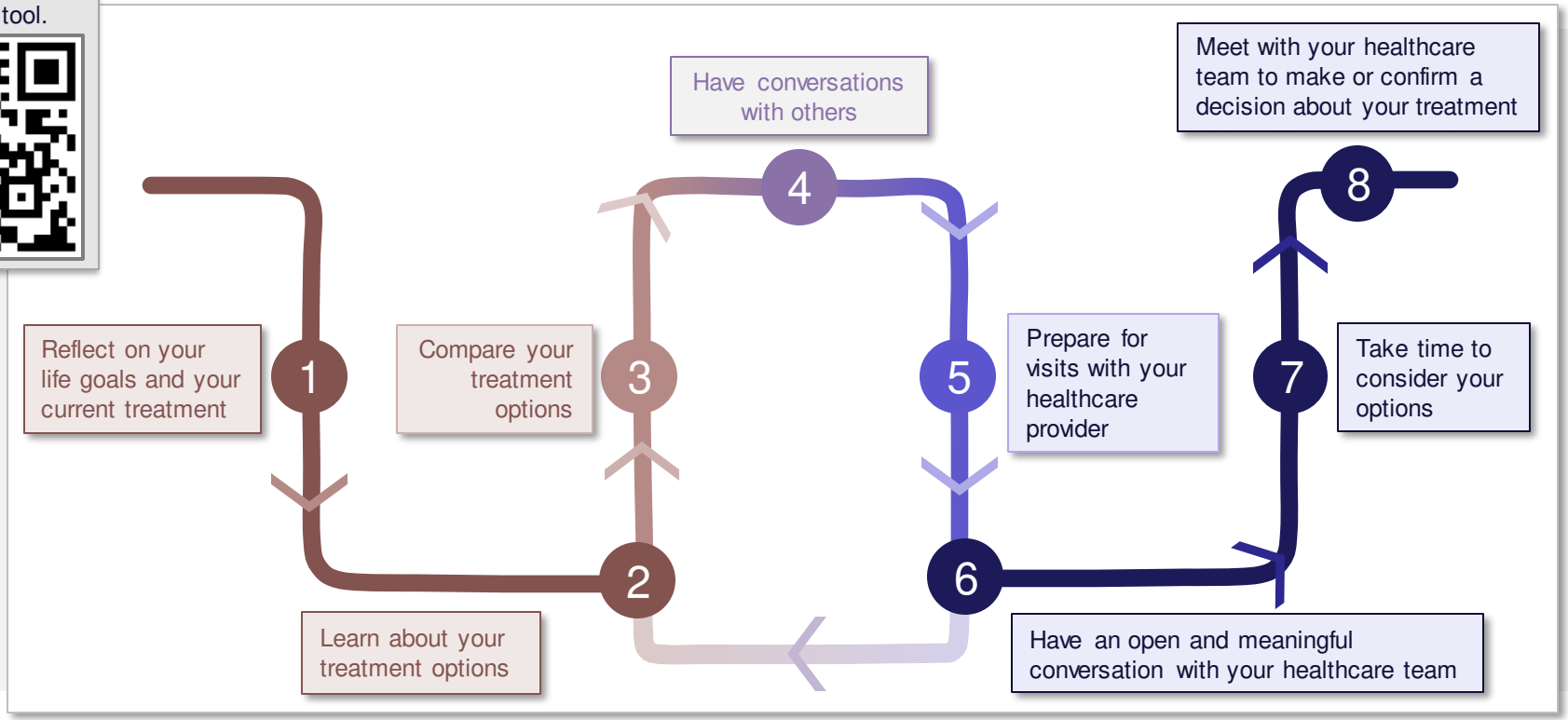
Step-by-Step Guide to SDM

World Federation of Hemophilia (WFH) Decision Making Tool

Scan QR code for WFH tool.

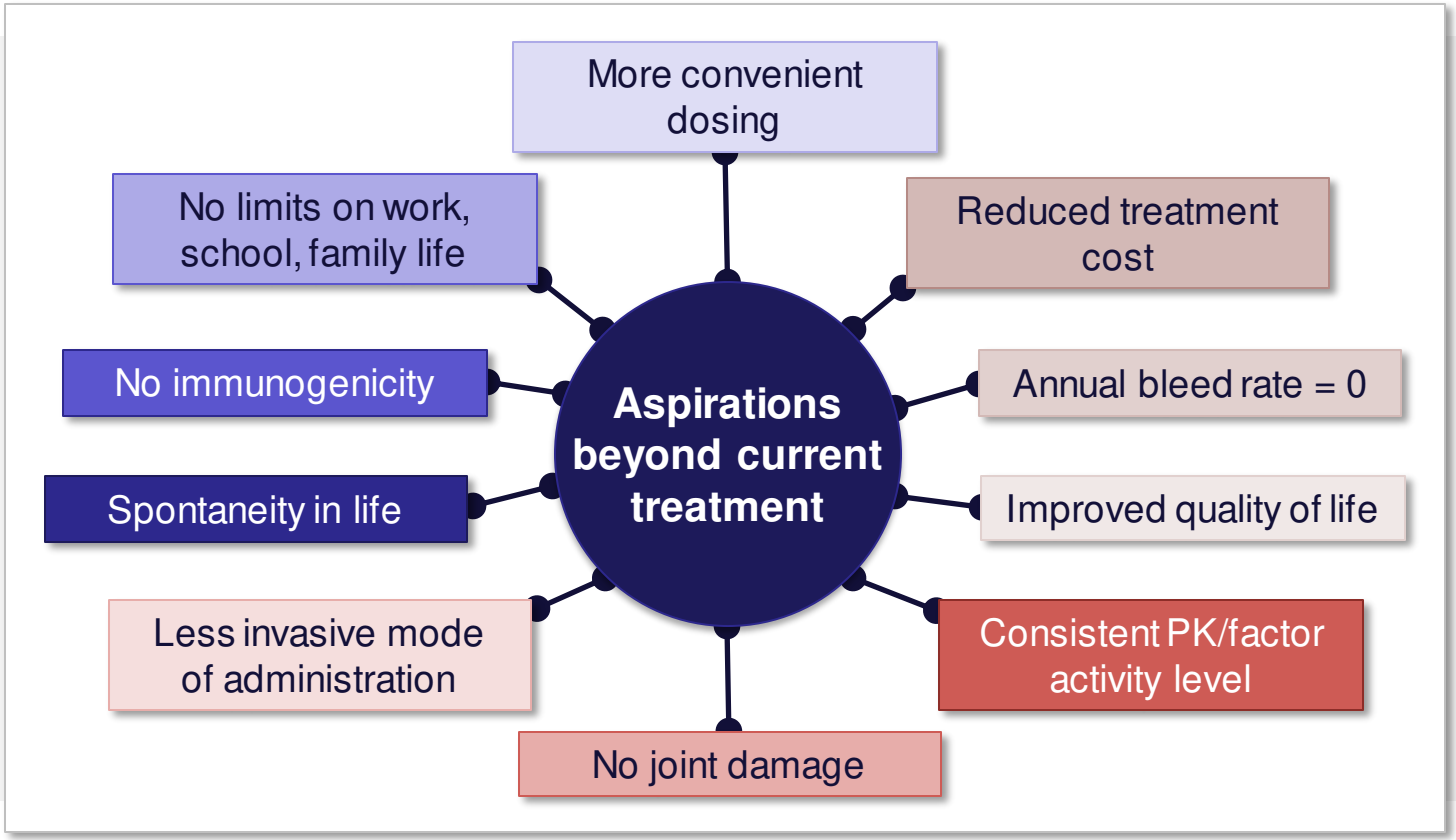


Adapted from WFH. https://sdm.wfh.org.

Assess Your Goals and Aspirations

How would you describe the impact of your hemophilia on obtaining your life goals (goals related to work, education, family, hobbies, etc.)?

Why are you considering a change to your therapy?

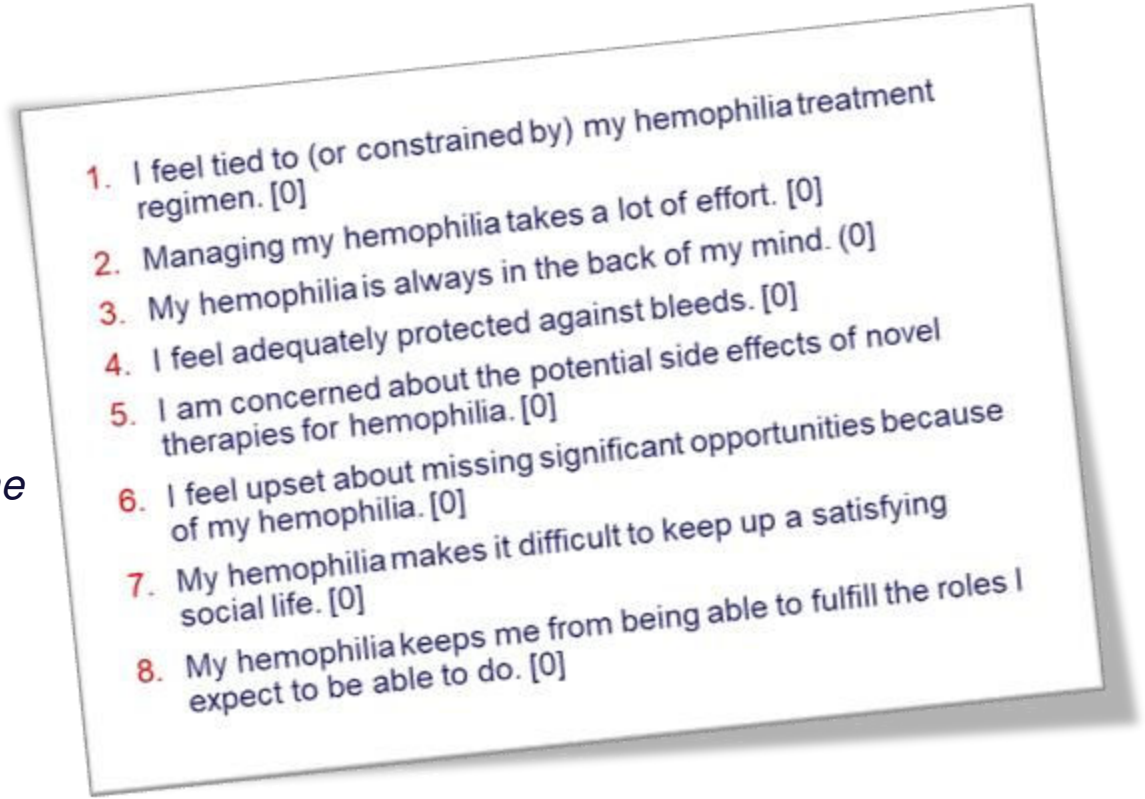


CME OUTFITTERS

Adapted from WFH. https://sdm.wfh.org. And NBDF. https://www.hemophilia.org/bleeding-disorders-a-z/treatment/shared-decision-making.

Reflect on Your Life with Hemophilia

Reflect on your life with hemophilia. Your answers will be included in your personalized summary at the end of the tool for you to print and bring to your healthcare team. On a scale of 0 to 100, rate how much you agree with these statements.



WFH. https://sdm.wfh.org. NBFD. coreHEM MHO PROM instrument. https://www.hemophilia.org/research/research-projects/corehem-mental-health-tool.

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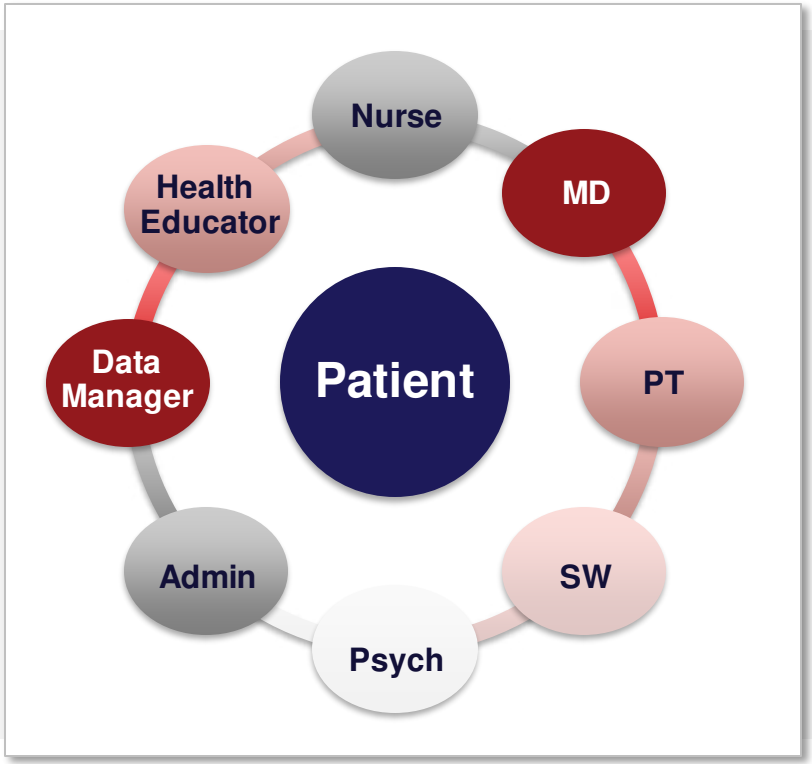
Velentino LA, et al. *J Haem Pract.* 2021;8(1):69–79.

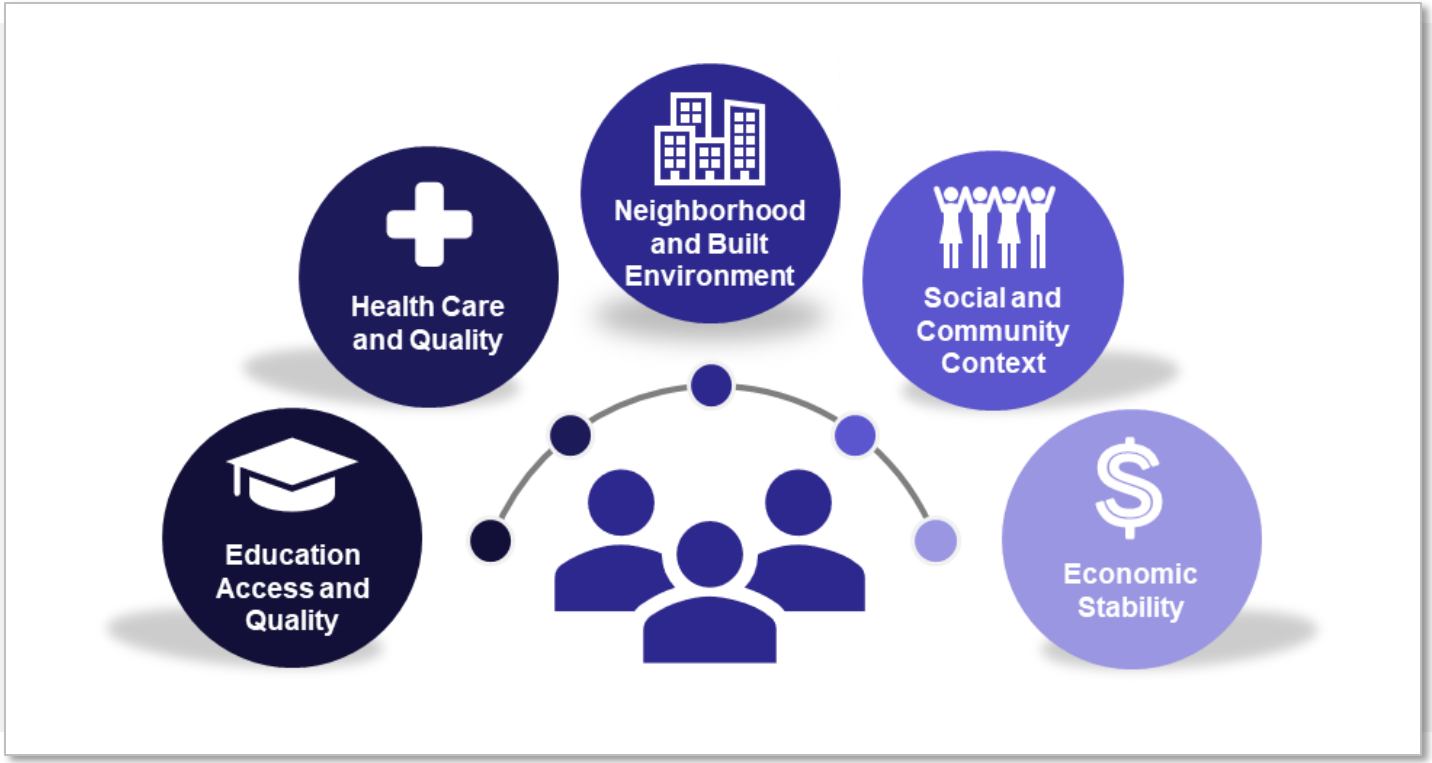
Importance of Patient Education

- Involve the multidisciplinary team
- Take into account patient's
  - Development stage
  - Health literacy
  - Cultural background
  - Other social determinants of health (SDoH)



PT, physical therapist; SW, social worker.

Understand Social Determinants of Health



U.S. Department of Health and Human Services. Healthy People 2030. https://health.gov/healthypeople/priority-areas/social-determinants-health.

SDoH (…cont'd)

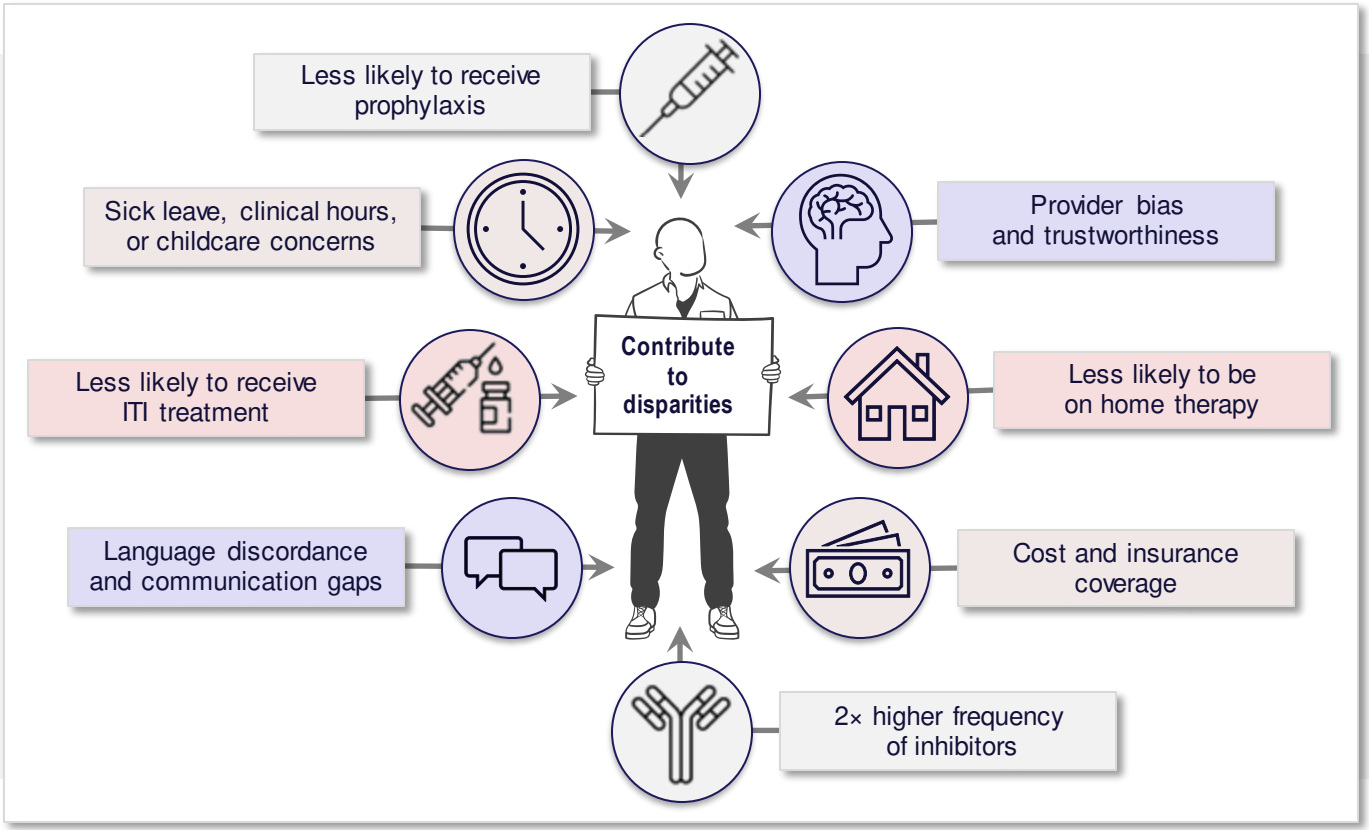
Economic Stability	Neighborhood and Physical Environment	Education	Food	Community and Social Context	Health Care System
Employment	Housing	Literacy	Hunger	Social integration	Health coverage
Income	Transportation	Language	Access to healthy options	Support systems	Provider availability
Expenses	Safety	Early childhood education		Community engagement	Provider linguistic and cultural competency
Debt	Parks	Vocational training		Discrimination	Quality of care
Medical bills	Playgrounds	Higher education		Stress	
Support	Walkability				
	Zip code/ geography				

Health Outcomes

Mortality, morbidity, life expectancy, health care expenditures, health status, functional limitations

U.S. Department of Health and Human Services. Healthy People 2030. https://health.gov/healthypeople/priority-areas/social-determinants-health.

Contributors to Racial and Ethnic Disparities in Hemophilia Care and Outcomes



Merz LE, et al. *Res Pract Thromb Haemost*. 2024;8(1):102290.

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Questions & Answers

Summary

- Stay current with transformational changes in hemophilia management, including FVIIIa mimetics, TFPI inhibitors, AT-siRNA, and APC inhibition
- Where applicable, follow risk mitigation strategies to ensure safe use of novel therapies
- Assess and implement emerging monitoring strategies for nonfactor therapies
- Implement shared decision making with patients to improve quality of care, adherence to therapies, and outcomes

To receive CME/CE credit

Complete the post-test and evaluation

HEMOSTASIS 2.0

Rethinking Hemophilia Management with Novel Agents and Shared Decision Making

CME OUTFITTERS

Supported by an educational grant from **Novo Nordisk, Inc.**