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Sutacimig - A Novel Prophylactic Treatment for Glanzmann Thrombasthenia: Interim Analysis of a Phase 1/2 Study

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Disclosures

In compliance with COI policy, ISTH requires the following disclosures to the session audience:

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Consultant	Guidepoint, CSL Behring, Hemab Therapeutics
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Other	No relevant conflicts of interest to declare

Glanzmann Thrombasthenia: platelet aggregation defect causing frequent bleeding events

Severe platelet function disorder

Characterized by deficient or dysfunctional GPIIb/IIIa expression on platelets impairing platelet aggregation and platelet-fibrinogen binding during primary hemostasis

Frequent bleeding events from low-volume bleeding to life-threatening hemorrhages[1-2]

88% of patients bleed weekly

> 50% bleed 3 x per week

80% miss school or work

No approved therapies for primary prophylaxis

30% develop alloimmunization to platelets

Current treatment options are limited by short half-lives, high costs, and complications with IV administration

Frequent bleeding events accumulate to create a significant clinical impact, including iron deficiency, and impaired quality of life.

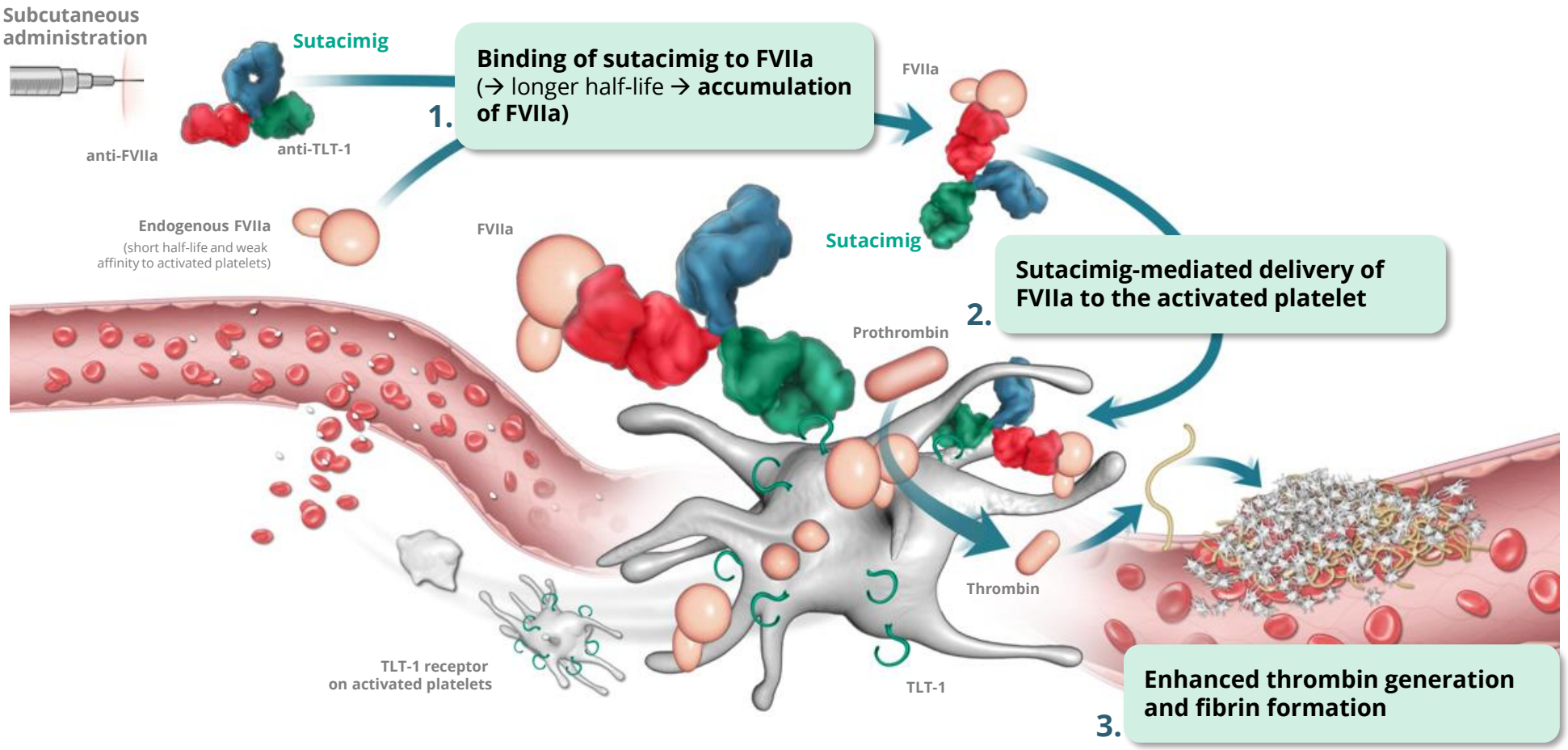
Abbreviations: GP, glycoprotein; GPIIb/IIIa, glycoprotein IIb/IIIa receptor; IV, intravenous; References: 1. Khair K, et al. Res Pract Thromb Haemost. 2024; 2. Rhea C. UK Blood Diary. ISTH 2024

Sutacimig Binds FVIIa and TLT-1 to Enhance Thrombin Generation

Bispecific antibody

Low-volume (<1 mL) SQ dose

TLT-1 potentiated FVIIa-mediated thrombin generation



Sutacimig binds and accumulates endogenous FVIIa and, following vessel lesion, localizes FVIIa to the surface of activated platelets via TLT-1 potentiation[1]

Abbreviations: FVIIa, activated factor VII; SQ, subcutaneous; TLT-1, TREM-like transcript-1.
References: Gandhi PS, et al. Nat Cardiovasc Res. 2024.

Multiple Ascending Dose Evaluation with Long-term Extension
Enrollment complete

Study objectives
Primary:
- Safety/tolerability
- PK/PD

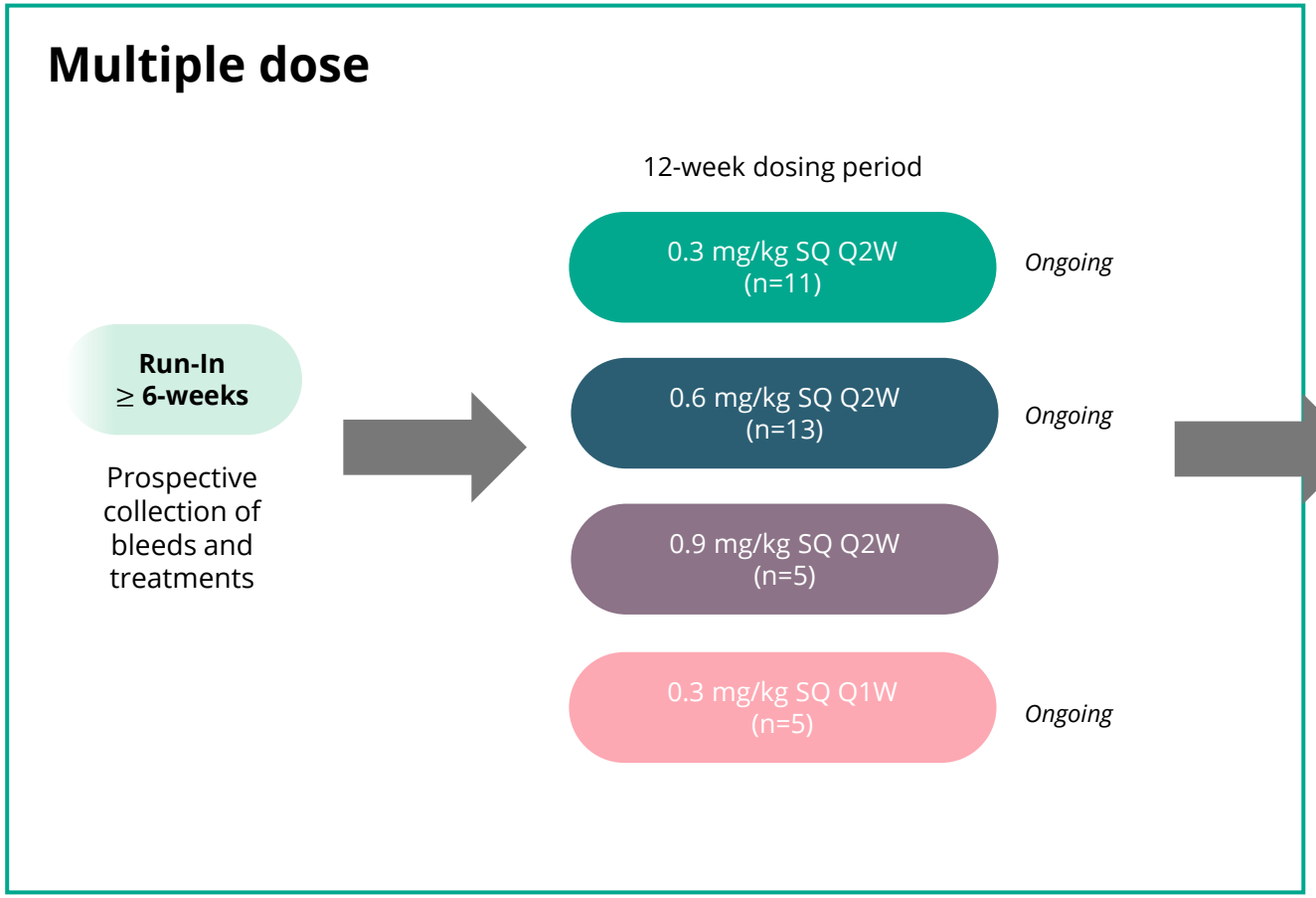
Secondary:
- Efficacy

Study population
- Confirmed GT diagnosis
- History of bleeding requiring treatment
- Adults 18–67 years

Safety population
- N=34

Efficacy population
- N=33*

Results of SAD portion previously presented^
- N=7
- No DLT. Max dose tested 1.25 mg/kg



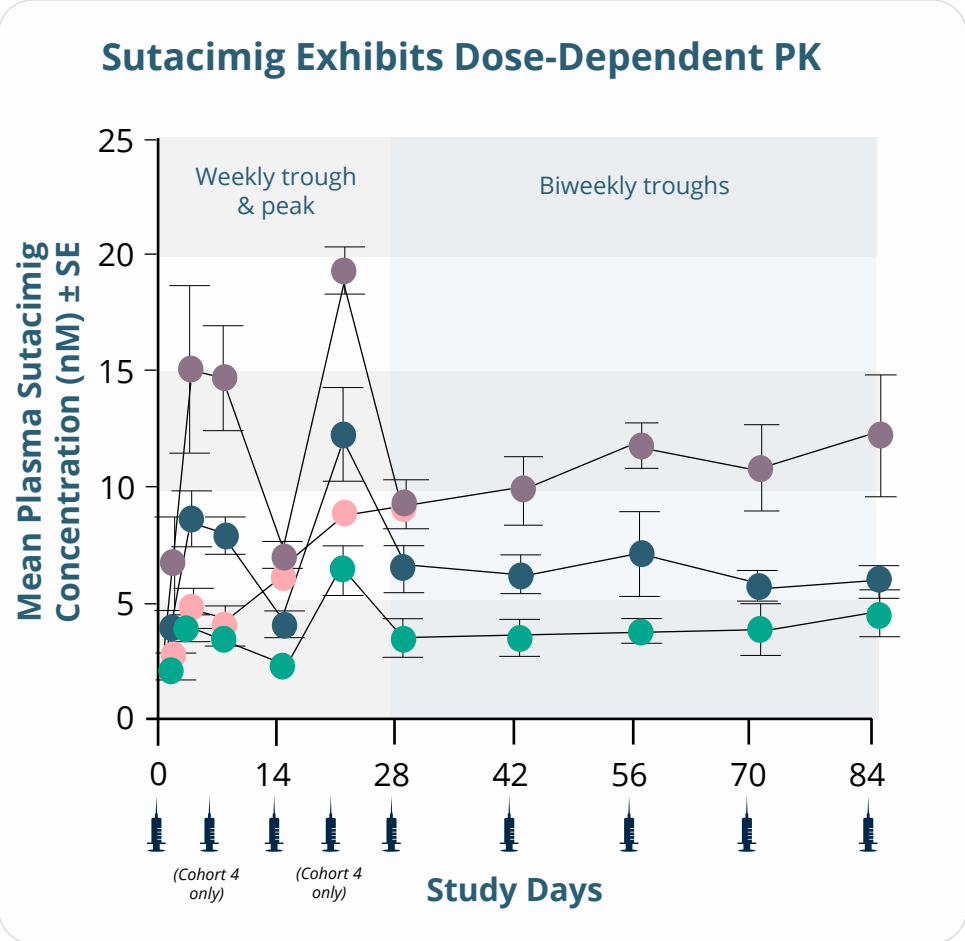
Full eligibility criteria available at ClinicalTrials.gov (NCT06211634). Interim data cutoff as of April 1, 2025; *1 patient excluded due to non-compliance with bleed diary guidelines
^Sivapalaratnam, EAHAD, 2025; 4 participants completed study at the end of Part B and did not continue to long-term extension due to: travel to site too far (2); upcoming surgery (1); mild bleeding severity

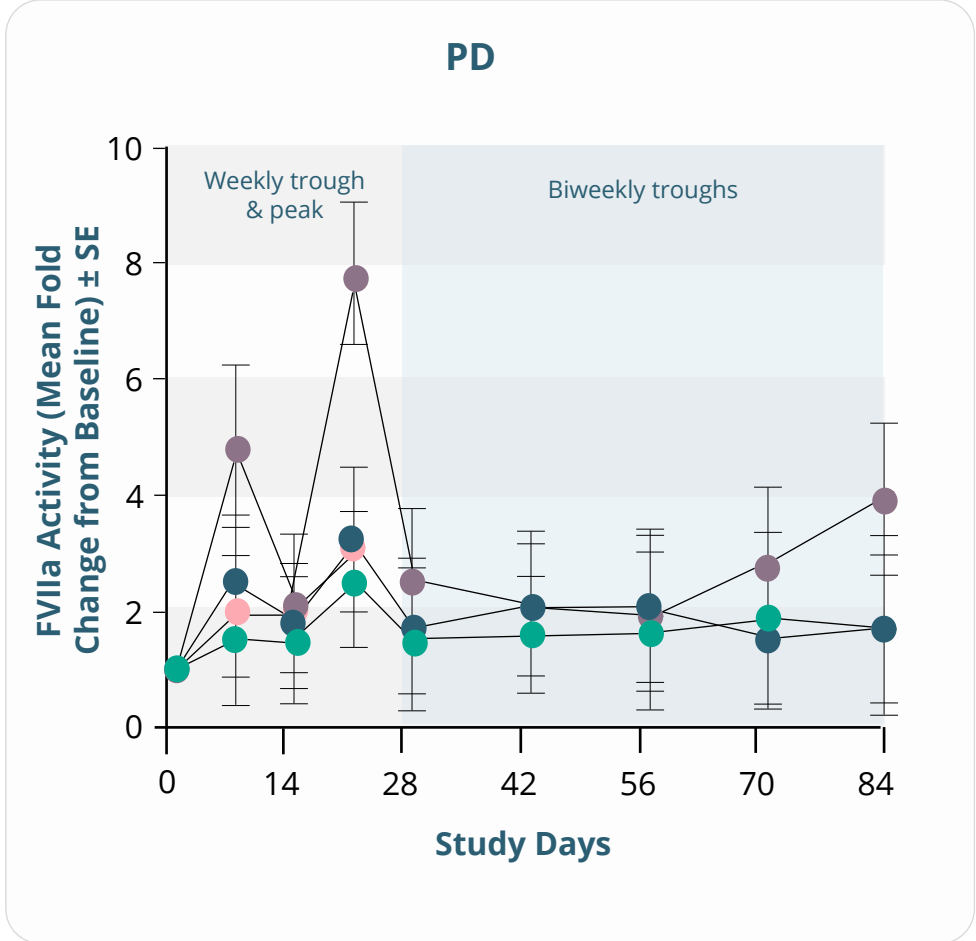
Baseline Characteristics and Demographics

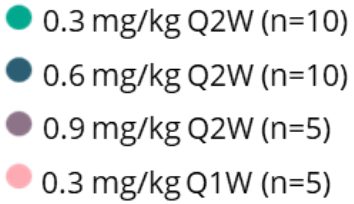
Demographics		**N=34**
Age	Median years (range)	40.5 (18-66)
Sex – N (%)	Female	16 (47)
	Male	18 (53)
Race – N (%)	Asian	6 (18)
	Black or African American	2 (6)
	White	16 (47)
	Other	2 (6)
	Not Reported	8 (23)
Baseline ATBR during run-in	Mean (Range) Median (Q1, Q3)	54.9 (0, 372.0) 23.6 (5.5, 59.6)
Cause of bleed (%)	Spontaneous Traumatic Iatrogenic	68 25 7

ATBR, annualized treated bleed rate. Baseline ATBR calculated from 6-week run-in. Interim data cutoff as of April 1, 2025.

Dose-Dependent Increase in Exposure and FVIIa Levels







- Dose-proportional exposure and PD (n= 30)
- FVIIa elevation in all dose cohorts
- 0.9 mg/kg peak PD approximately double that of 0.3 mg/kg and 0.6 mg/kg cohorts
- 0.3 mg/kg Q1W peak below 0.6 mg/kg cohort with trough levels similar to 0.9 mg/kg cohort

Interim Safety Summary

Median exposure: 2 months (range: 0.03–10.15)

	N (%)
Safety population	34 (100)
Any grade AE (all causality)	26 (77)
AE leading to discontinuation	1 (3)
AE in ≥ 10%	
Headache	6 (18)
Nasopharyngitis	6 (18)
D-dimer elevation	6 (18)
≥ Grade 3 AE (non serious)	5(15)
Dental caries	1 (3)
Iron deficiency anemia	1 (3)
Post procedural hemorrhage	1 (3)
Back pain	1 (3)
SAE	
DVT (Gr 2)	1 (3)
Melena (Gr 3)	1 (3)

- **Majority AEs mild to moderate in severity**
- **Notable AEs:**
  - DVT (0.9 mg/kg Q2W) on Day 46 at peak exposure (previously reported; patient with multiple potential risk factors, managed outpatient; patient discontinued due to AE)
  - Grade 3 melena Day 2, assessed as unrelated and resolved
  - Grade 3 post-procedural hemorrhage (following dental procedure, resolved with 5 mcg/kg administration of rFVIIa); iron deficiency anemia (D1-3, resolved)
  - D-dimer elevation in 6 participants (all low grade); 1 associated with previously reported DVT; all others associated with treated bleeding events; otherwise asymptomatic; no associated clinical thrombocytopenia (all platelet values > 120), or hypofibrinogenemia
- **4/34 participants developed neutralizing ADA; 2/4 resolved with continued dosing; no associated safety events**

Abbreviations: AE, adverse event; ADA, anti-drug antibody; DVT, deep vein thrombosis; rFVIIa, recombinant FVIIa SAE, serious adverse event; All AEs summarized are treatment emergent. Adverse event grouping based on MedDRA-coded terms, excluding bleed events. Interim data cutoff of April 1, 2025.

Improvement in Thrombin Generation (Lag Time) Demonstrates TLT-1 Potentiation of FVIIa by Sutacimig

Baseline and post-sutacimig lag time compared to rFVIIa-spiked baseline sample lag time

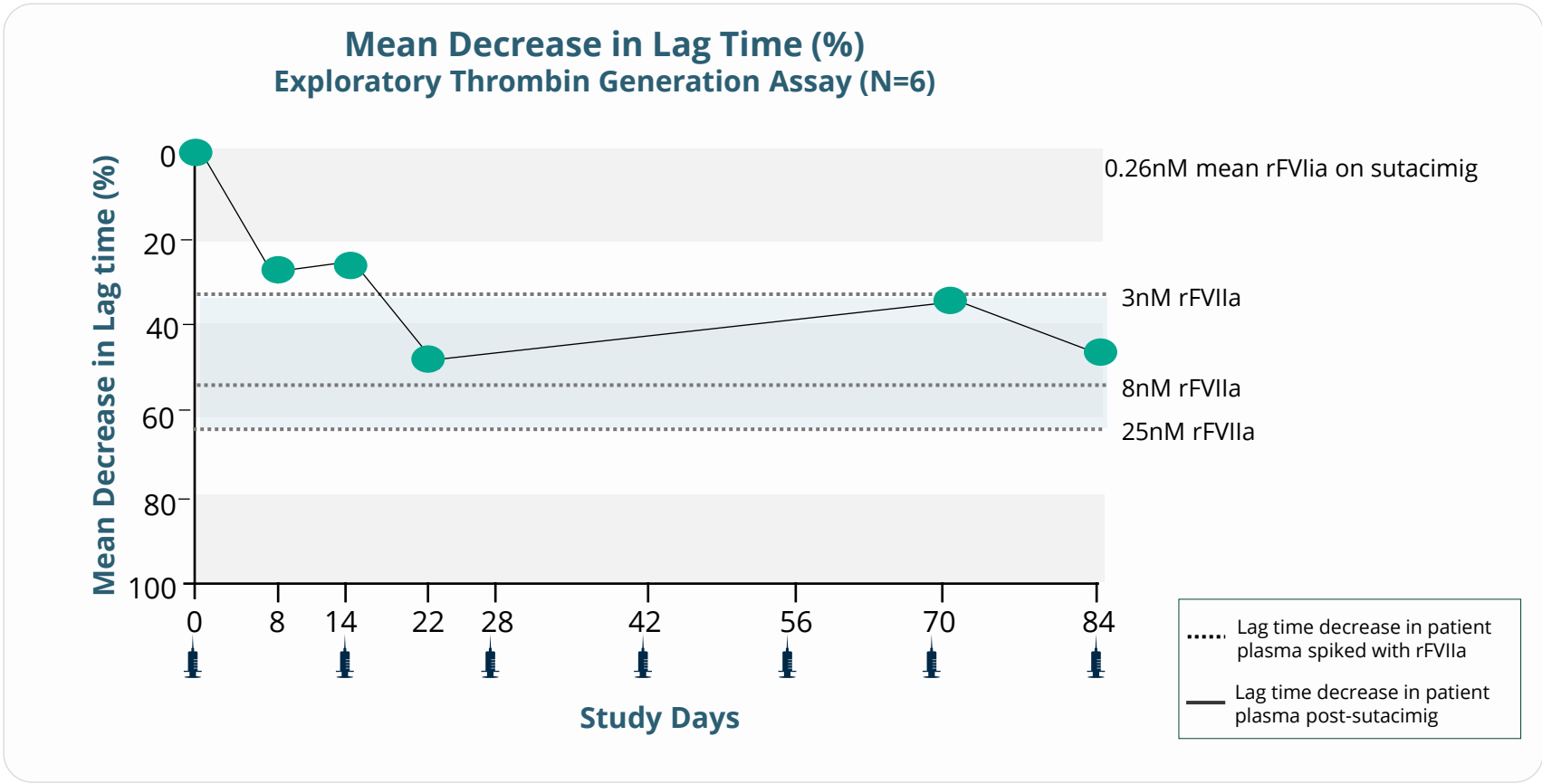
GT patients exhibit extended lag time

Lag time improvement on sutacimig comparable to improvement in baseline samples spiked with 3 nM to 8 nM rFVIIa

Mean FVIIa post-sutacimig for these patients was 0.26 nM

Demonstrates TLT-1 potentiation of FVIIa

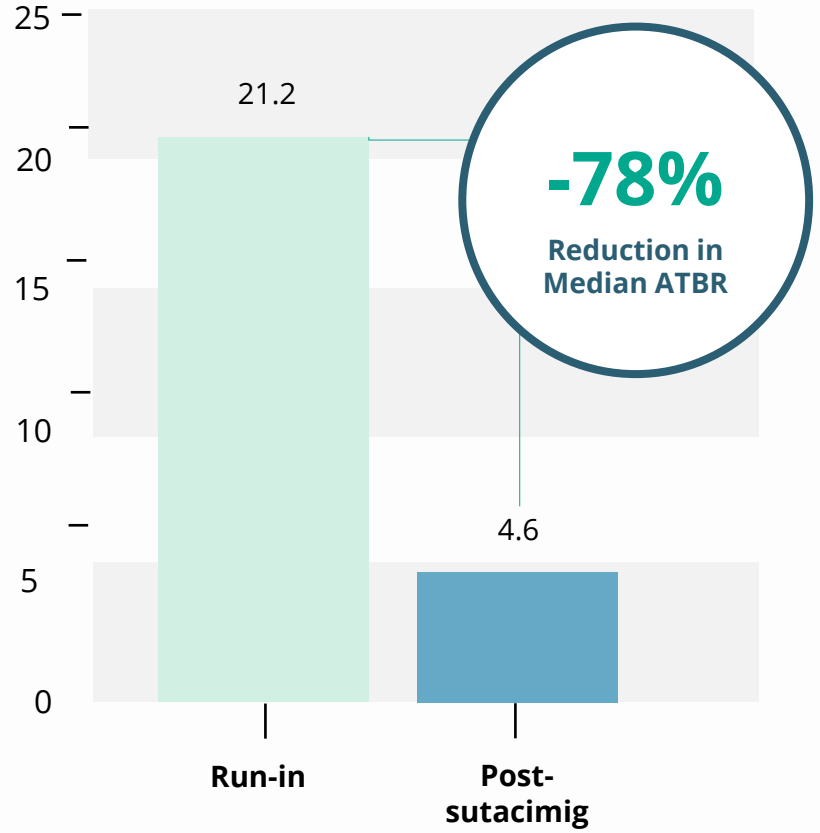
Lag time improved ~50% in sutacimig-treated patients, comparable to clinically relevant rFVIIa



Exploratory Adapted Thrombin Generation Assay: Platelet poor plasma reconstituted with pooled washed treated (activated and GT-like) platelets; Measured using calibrated automated thrombogram method. ADA+ participants excluded; recombinant FVIIa dose of 90 ug/kg; FVIIa mean D22

Median Annualized Treated Bleed Rate on Weekly or Biweekly Sutacimig

Up to 12 weeks of dosing; median exposure 8.5 weeks; all doses included



Dosing regimen	N
0.3 mg/kg Q2W	11
0.6 mg/kg Q2W	12
0.9 mg/kg Q2W	5
0.3 mg/kg Q1W	5

ATBR	Run-In	Post-sutacimig
Median	21.2	4.6
Interquartile range	4.8-55.6	0-27.4

Interim data cutoff as of April 1, 2025

Clinical Impact of Sutacimig

Participant

37 year-old male with recurrent epistaxis, oral iron intolerance, and anti-GPIIb/IIIa antibodies on sutacimig treatment for 6 months

Cessation of epistaxis-induced transfusions on sutacimig 0.6 mg/kg Q2W

Epistaxis history at baseline – in prior 3 months

- Daily epistaxis lasting >30 minutes
- Transfusions required
- Medical attention needed

6 months post-sutacimig

- Decreased epistaxis frequency
- Transfusion-free for 6 months
- No medical attention needed for 6 months

Participant

50 year-old female with history of rFVIIa use for bleeding; baseline ATBR 46; on sutacimig treatment for 9.5 months

Successful post-procedure hemostasis with reduced rFVIIa sutacimig 0.6 mg/kg Q2W

ATBR reduction 46 → 0

Successful hemostasis post dental procedure using >90% reduced dose of rFVIIa*

90 mcg/kg *(typical dose)*
→ 5 mcg/kg

Conclusions

- Interim data from N=34 participants with Glanzmann thrombasthenia and baseline ATBR mean/median of 55/24 with 0.03 to 10 months exposure
- Majority of AEs mild or moderate; one DVT at prior 0.9 mg/kg dose level
- Dose dependent peak PK, PD at ongoing dose levels with demonstration of FVIIa elevation 2-4-fold above baseline and improved thrombin generation (lag time)
- Sutacimig continues to demonstrate clinically meaningful >50% reduction in ATBR
- Safety and clinical activity at ongoing SQ weekly or biweekly doses support potential as prophylactic agent in GT

Next steps

- Identification of a Phase 3 dose and regimen following additional treatment and follow up

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***Sponsor:** Hemab Therapeutics

ENROLLMENT COMPLETE:
Glanzmann thrombasthenia

Country	Phase 1/2 sites
Belgium	University Hospital Leuven
France	AP-HP Hôpital Bicêtre
	AP-HP Hôpital Necker
	AP-HM - Hôpital de la Timone
Italy	Careggi University Hospital
	IRCCS Ca' Granda Maggiore Hospital
Netherlands	University Medical Centre Utrecht
United Kingdom	Leeds Teaching Hospitals
	The Royal London Hospital
	Richmond Pharmacology
	Royal Free London
	Queen Elizabeth Hospital Birmingham
United States	University of California, San Diego
	Tulane University Medical Centre
	Mayo Clinic - Rochester
	University of Pittsburgh
	Washington Institute for Coagulation