Sutacimig Prophylaxis in Glanzmann Thrombasthenia: Results from a Phase 2 Long-Term Extension

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Glanzmann Thrombasthenia

Platelet Aggregation Defect Causing Frequent Bleeding Events

Severe platelet function disorder

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

No approved therapies for primary prophylaxis

Frequent bleeding events accumulate to create a significant clinical impact.

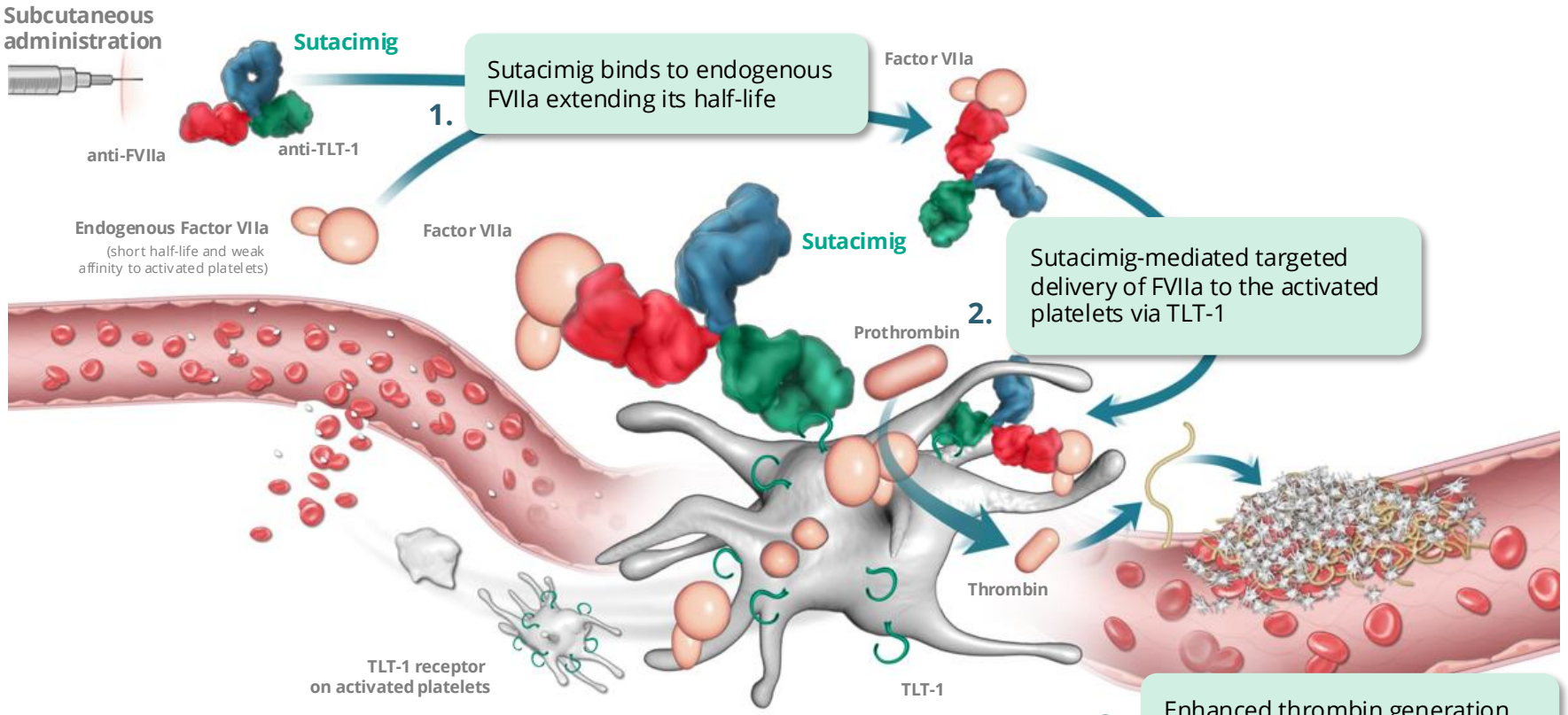
Abbreviations: GP, glycoprotein; GPIIb/IIIa, glycoprotein IIb/IIIa receptor; IV, intravenous; References: 1. Khair K, et al. Res Pract Thromb Haemost. 2024

Sutacimig targets endogenous FVIIa to activated platelet surface, leading to potentiation of FVIIa activity

Bispecific antibody

Low-volume (<1 ml) SQ dose

TLT-1 potentiated FVIIa-mediated thrombin generation

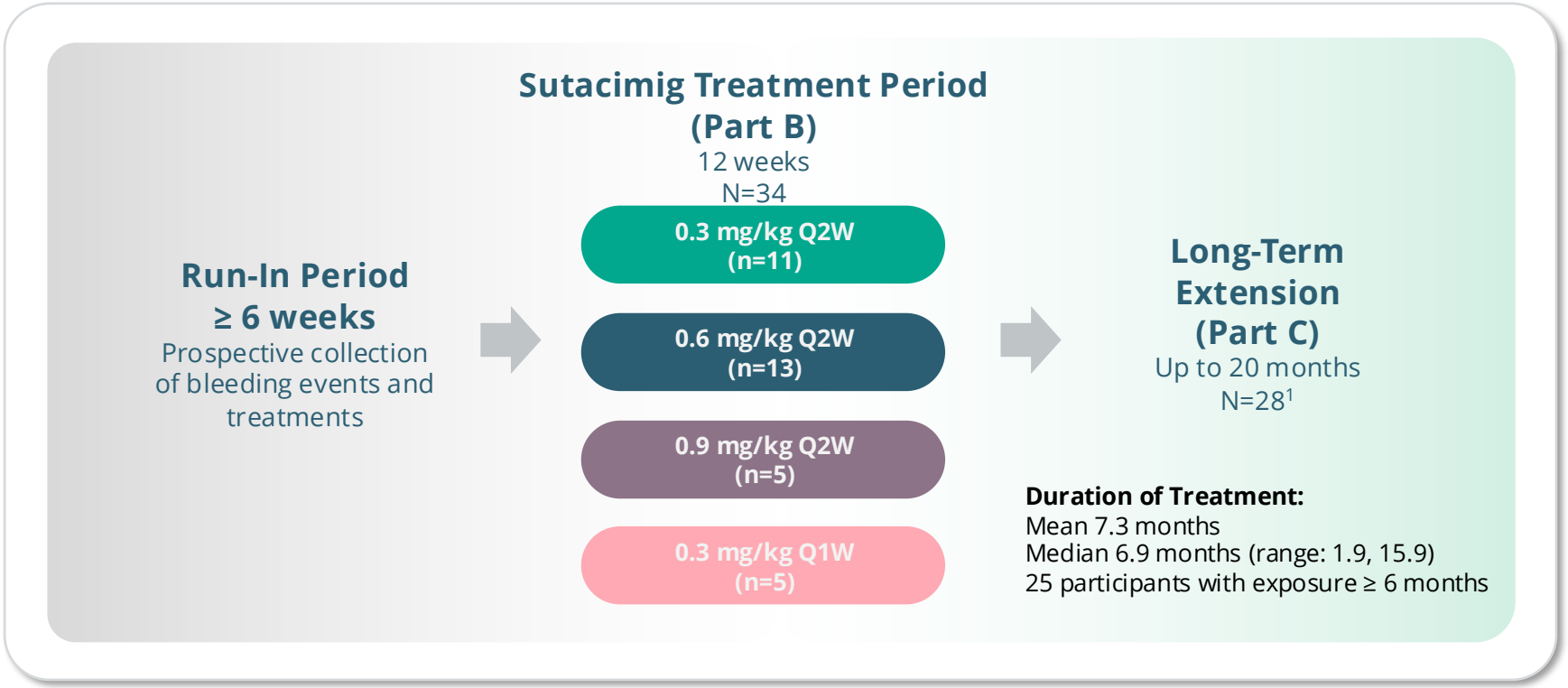


Sutacimig binds endogenous FVIIa and, following vessel lesion, localizes FVIIa to the surface of activated platelets via TLT-1, potentiating activity at the site of bleeding. TLT-1 is expressed only on activated platelets targeting FVIIa delivery to sites of injury and potentiating its activity.

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

Phase 1/2 Study Evaluating Sutacimig in Glanzmann Thrombasthenia
Multiple Ascending Dose (MAD) Evaluation with Long-Term Extension (Part B + Part C)

- **Primary Objective:**
  - Safety and tolerability
- **Other Objectives:**
  - PK/PD
  - Efficacy based on annualized treated bleed rate (ATBR) reduction
- **Key Entry Criteria:**
  - Confirmed GT diagnosis
  - History of bleeding requiring treatment
  - Adults 18-67 years



Preceded by SAD (Part A)

0.2 mg/kg, 0.5 mg/kg and 1.25 mg/kg

Data available as of interim data cut-off of October 14, 2025. Additional study details available at ClinicalTrials.gov NCT06211634.
[1]Two participants terminated the 12-week treatment period prior to completion, 1 each due to adverse event and physician decision due to participant noncompliance. Four participants did not consent to the long-term extension, 3 for logistical reasons (travel to site, scheduling) and 1 had ongoing medical issues that interfered with trial participation.

Demographics and Baseline Characteristics

Characteristic		N=34
Age (years)	Median (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)
Weight (kg)	Median (range)	75 (52-134)
Total FVII(a) (%)	Median (range)	99.5 (51.1, 164.5)

Data available as of interim data cut-off of October 14, 2025.

Enrolled Study Population with High Baseline Bleeding Burden

Characteristic		N=34
Baseline ATBR during run-in[1]	Mean[2] (SEM); range Median (Q1, Q3)	40.2 (11.8); range: 0-324.7 17.7 (4.0, 47.6)
Cause of treated bleeding events during run-in (%)[1]	Spontaneous Traumatic Iatrogenic Not categorized	75.0 19.3 4.7 1.0

[1]Efficacy set excludes 3 participants: 2 due to run-in period <6 weeks and 1 due to noncompliance with bleed diary guidelines.
[2]Weighted by number of evaluable days during the run-in period
Data available as of interim data cut-off of October 14, 2025.

Generally Manageable Safety and Tolerability Profile

12-Week Treatment + Extension Periods – Median Exposure: 6.9 months (range: 1.9, 15.9)

Majority of observed TEAEs were mild to moderate in severity, and without specific pattern

- Headache (23.5%), Fibrin D-dimer increased (23.5%), nasopharyngitis (20.6%), back pain (17.6%) most common

No related TEAE ≥ Grade 3[1]

Grade 2 VTEs[2] N=3 participants, Assessed as related

- All treated at higher dose levels and/or ≥ 3 concurrent VTE risk factors present
- Drug discontinued , received anticoagulation
- All were resolved (SSPE, IDDVT) or resolving (DVT) at last visit

6/34 participants developed ADA titers

- 2/6 received single dose in Part A prior to Part B
- 3/6 resolved/resolving at data cut off
- No associated safety events
- FVIIa did not drop below LLN

0.3 mg/kg Q2W regimen N=11 assigned pts had no TE with > 6 months exposure

	Treatment + Extension Period Part B + Part C (N=34)
All TEAE	32 (94.1)
Related TEAs	15 (44.1)
Grade 3+ TEAEs by Maximum CTCAE Grade[3]	7 (20.6)
Serious Adverse Events[4]	5 (14.7)
Deaths	0
Related Serious Adverse Events	2 (5.9)
TEAEs leading to Study Discontinuation[5]	4 (11.8)

Pt	TEAE by PT	AE Description	SAE	Grade	Related	Drug discontinued due to AE	Assigned Dose	Last Dose Prior to Event	Potential contributing risk factors
1	DVT	Proximal DVT lower extremity	Yes	2	Yes	Yes	0.9 mg/kg Q2W	0.9 mg/kg Q2W	Hypertension; Obesity; Nicotine – vaping
2	DVT	Isolated distal DVT lower extremity	No	2	Yes	Yes	0.6 mg/kg Q2W	0.6 mg/kg Q2W	Age >40 yrs; Obesity; Nicotine – smoking
3	Pulmonary embolism	Subsegmental PE	Yes	2	Yes	Yes	0.6 mg/kg Q2W	0.3 mg/kg Q1W	Age >40 yrs; Active GI bleed; Hospitalization; Platelet transfusion

[1]7 pts experienced Grade 3 AE : bleeding events due to underlying GT (3) , back pain (1), neutropenia (1), invasive ductal carcinoma (1) and 1 pt with dental carries & post procedural hemorrhage. Grade 3 GT-related bleeding events consist of GI hemorrhage (1), intra-abdominal hemorrhage (1- same pt as SSPE), epistaxis (1). [2]Venous thrombotic events of DVT or PE (3) , [3] If a patient experienced more than one adverse event, the participant was counted once at the most severe or most related event . [4]Total SAE (5): 2 VTE above and the 3 GT bleeding events above. [5]AE leading to study drug discontinuation: 3 VTE above and invasive ductal carcinoma (1).

SSPE: subsegmental pulmonary embolism ; IDDVT: Isolated distal deep vein thrombosis; DVT: Deep vein thrombosis; LLN: Lower limit of normal, Pt = participant, PT = Preferred Term, SAE = Serious Adverse Event, TEAE = Treatment-emergent adverse event, CTCAE = common terminology criteria for adverse events. Data available as of interim data cutoff of October 14, 2025.

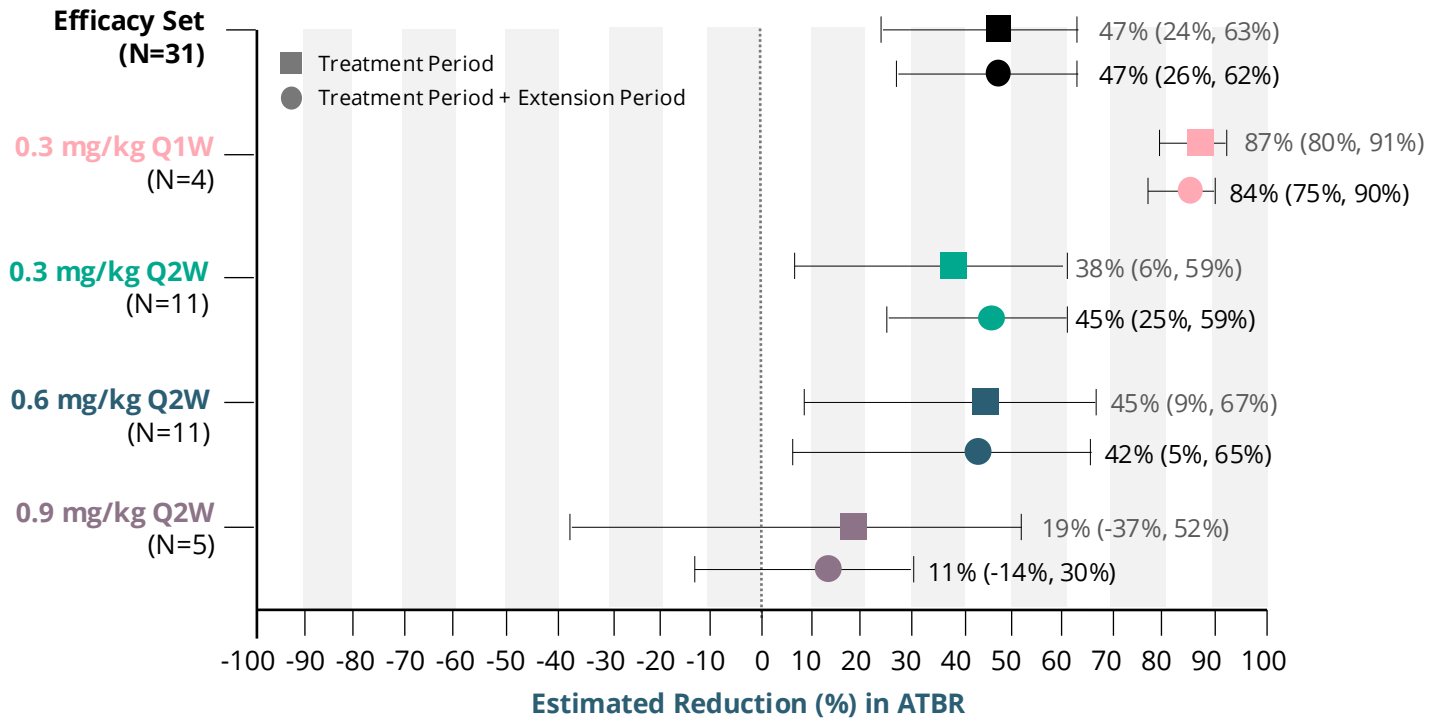
Reduction in Mean ATBR Across Multiple Dose Cohorts

12-Week Treatment Period vs Treatment + Extension Periods (median exposure 6.9 months)

- Persistent and clinically meaningful ATBR reduction observed, including over extended treatment
- ATBR reduction across all dose levels
- Enhanced response with weekly dosing

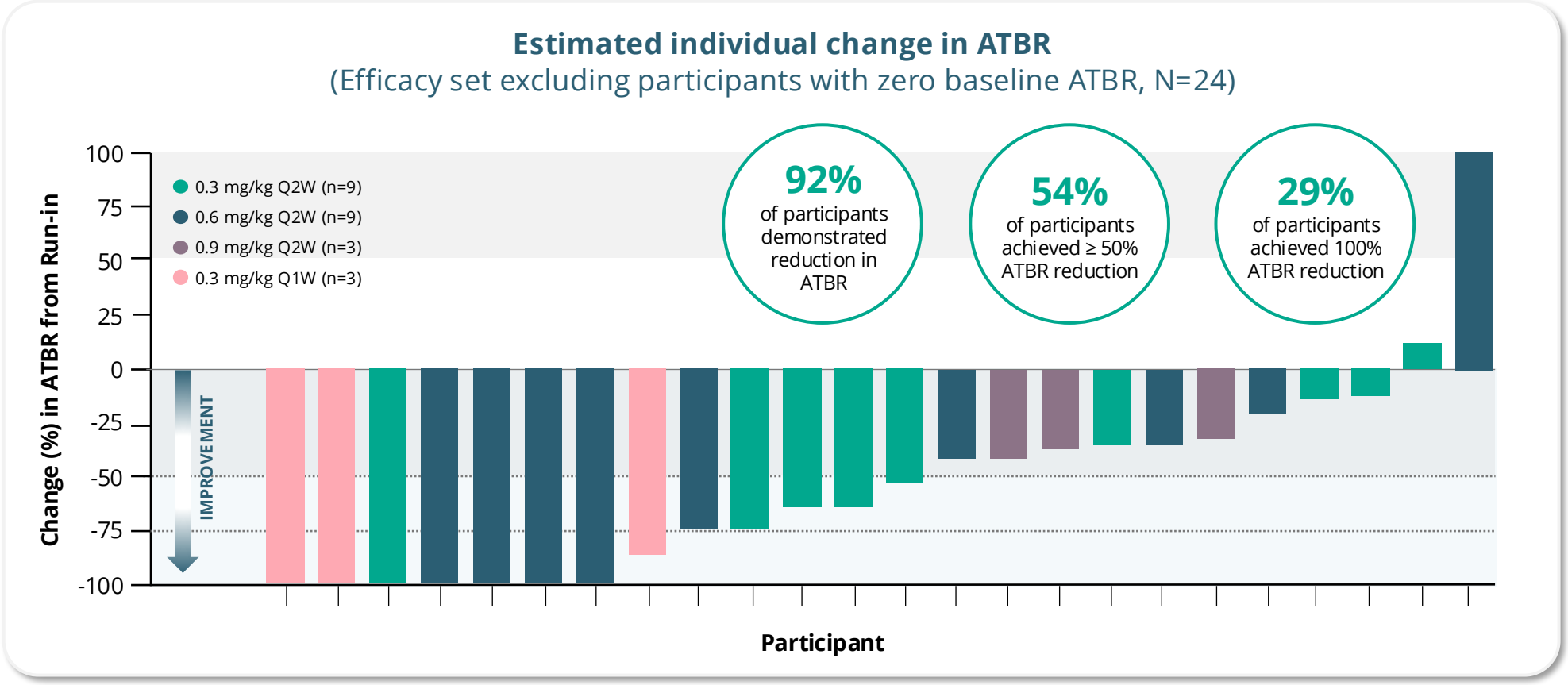
Estimated reduction in ATBR with 95% confidence interval (Efficacy set, N=31)

Treatment Period vs Treatment + Extension Periods



A GEE negative binomial regression model with an offset for duration of study period was fitted to estimate the mean ATBR during each study period and reduction in mean ATBR along with corresponding 95% CIs. Efficacy analyses included comparison of the run-in and 12-week treatment + extension periods (data cut-off October 14, 2025).

ATBR Reduction Observed Across All Dose Cohorts and in the Majority of Participants

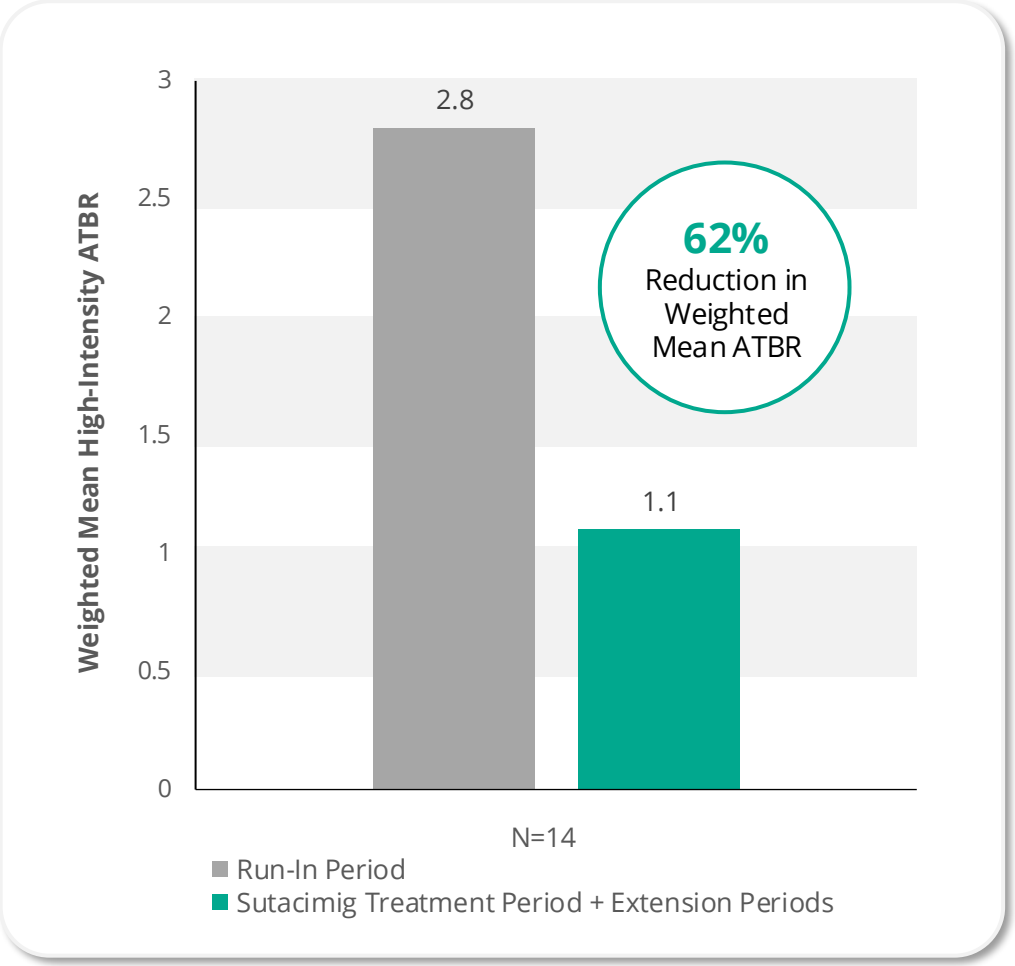


A GEE negative binomial regression model with an offset for duration of study period was fitted to estimate the mean ATBR during each study period and reduction in mean ATBR along with corresponding 95% CIs.
Efficacy analyses included comparison of the run-in and 12-week treatment period for Efficacy Set excluding participants with zero ATBR during run-in period (N=24, data cut-off October 14, 2025).

Marked Reduction in Bleeding Events Treated with High-Intensity Treatments

Subpopulation with Prior High-Intensity Treatment Use – Run-In vs Treatment + Extension Periods

- **Mean reduction of high-intensity ATBR of 62%**
- High-intensity treatment definition:
  - Systemic hemostatic intervention (rFVIIa, platelet transfusion, plasma, cryoprecipitate)
  - Medical, surgical, or radiologic procedures for hemostasis



A subset of participants were identified as experiencing bleeding events treated with high-intensity treatments within 12-month period prior to first dose of sutacimig.
Efficacy analyses included comparison of the run-in and 12-week treatment + extension periods (data cut-off October 14, 2025).

Participants with Procedures

Successful hemostatic outcomes with minimal intervention

Gingival Plaque Removal

0.6 mg/kg Q2W

rFVIIa **5 mcg/kg IV** + topical rFVIIa

✓ **Successful hemostasis**

Moh's Surgery, Basal Cell Carcinoma, Scalp

0.9 mg/kg Q2W

Oral tranexamic acid (peri-procedural)

✓ **Successful hemostasis**

Partial Mastectomy & Lymph Node Biopsy

0.3 mg/kg Q1W

IV TXA 1 g + rFVIIa **7 mcg/kg** (pre-procedure)
Oral TXA 1300 mg TID × 10 days (discharge)

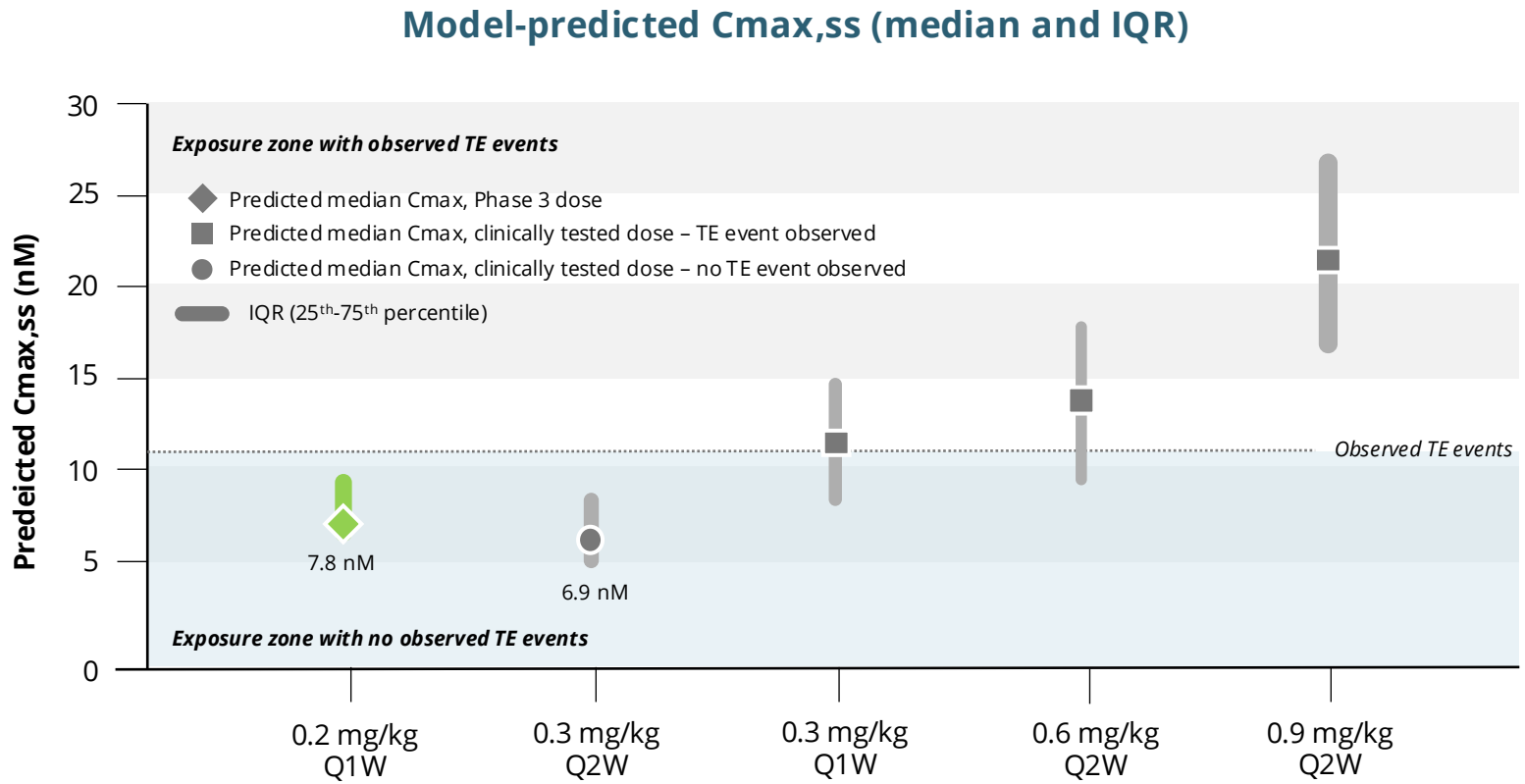
✓ **Successful hemostasis**

Individualized bleed management plans were followed during sutacimig treatment, including administration of lower than standard dose of rFVIIa, if needed

Data available as of interim data cut-off of October 14, 2025.

Phase 3 Dose: Potential to Optimize for Safety and Efficacy

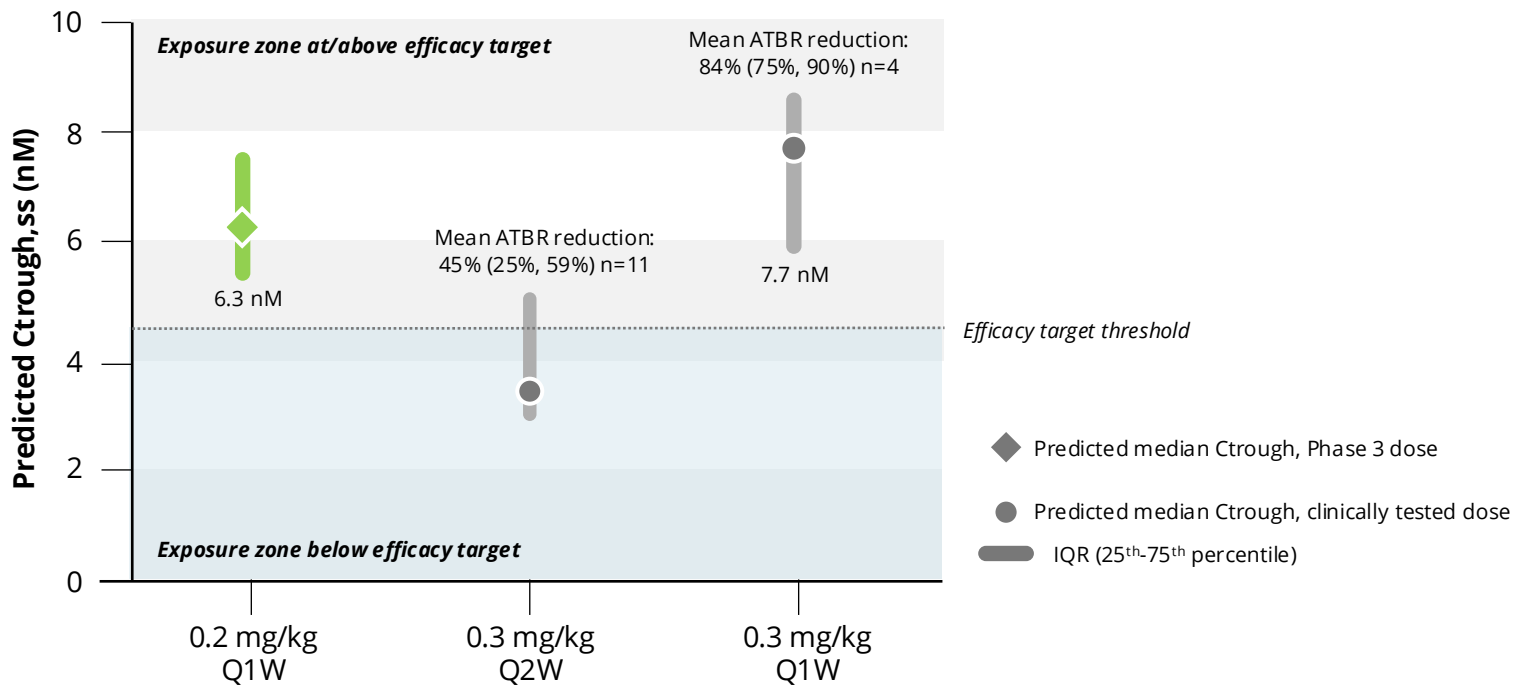
- 0.2 mg/kg Q1W Cmax comparable to 0.3 mg/kg Q2W



Ctrough, ss values are model-predicted medians with IQR band from exposure-response modeling and simulation (N=1500 simulated patients).
Abbreviations: IQR, interquartile range; ss, steady state

Phase 3 Dose: Potential to Optimize for Safety and Efficacy

- Substantial elevation of trough with weekly dosing
- 0.2 mg/kg Q1W predicted to achieve exposure levels at or above efficacy target threshold



Patients with predicted Ctrough,ss > 4.5nM (N=23) showed a 2-fold greater mean ATBR reduction than those below this threshold

Ctrough, ss values are model-predicted medians with IQR band from exposure-response modeling and simulation (N=1500 simulated patients).
Abbreviations: IQR, interquartile range; ss, steady state

Conclusions

- **At > 6 months of treatment in participants with GT, sutacimig data support advancing to Phase 3**
  - Safety profile remained stable with continued dosing and was overall generally well tolerated
  - Exposure range expected to mitigate the risk of occurrence of TE events identified
  - Sutacimig proof of concept as prophylactic treatment for GT confirmed
    - Clinically meaningful mean ATBR reduction across dose levels, marked reduction of bleeding events requiring high-intensity treatments, successful management of surgeries with minimal concurrent hemostatic agent use
  - Phase 3 dose identified, with potential to attain clinically-derived safety and efficacy targets
- **FDA alignment reached to enter Phase 3 with 0.2 mg/kg Q1 Week**
- **Next steps: Proceeding to a global Phase 3 study in 2026**

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Acknowledgements: Lin Zhang, Tara Parsons, Shea Golden*

*Sponsor: Hemab Therapeutics

ENROLLMENT COMPLETE: Glanzmann thrombasthenia

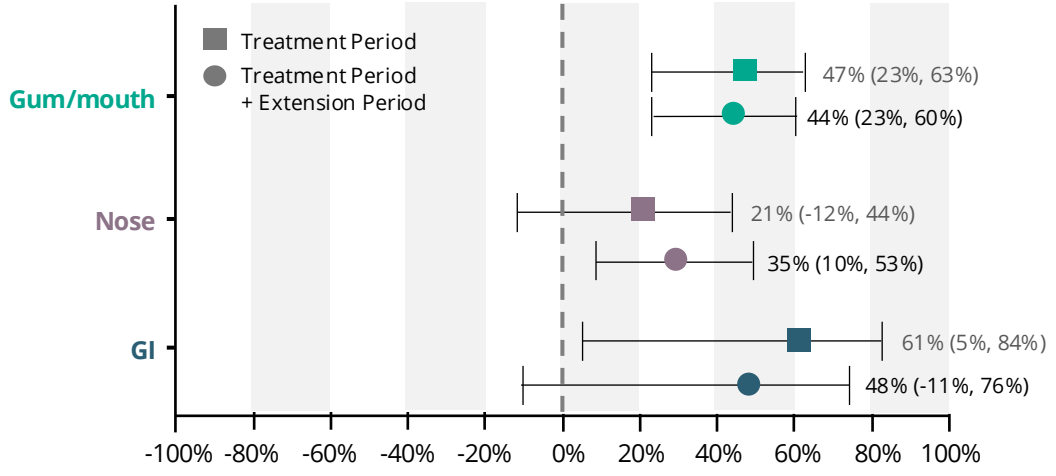
Country	Phase 1/2 sites
Belgium	University Hospital Leuven
France	AP-HP Hôpital Bicêtre
	AP-HP Hôpital Necker
	AP-HM - Hôpital de la Timone
Italy	IRCCS Ca' Granda Maggiore Hospital
Netherlands	University Medical Centre Utrecht
United Kingdom	Leeds Teaching Hospitals
	The Royal London Hospital
	Richmond 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

Mean ATBR Reduction Observed across Bleeding Event Type and Cause

12-Week Treatment Period vs Treatment + Extension Periods (median exposure 6.9 months)

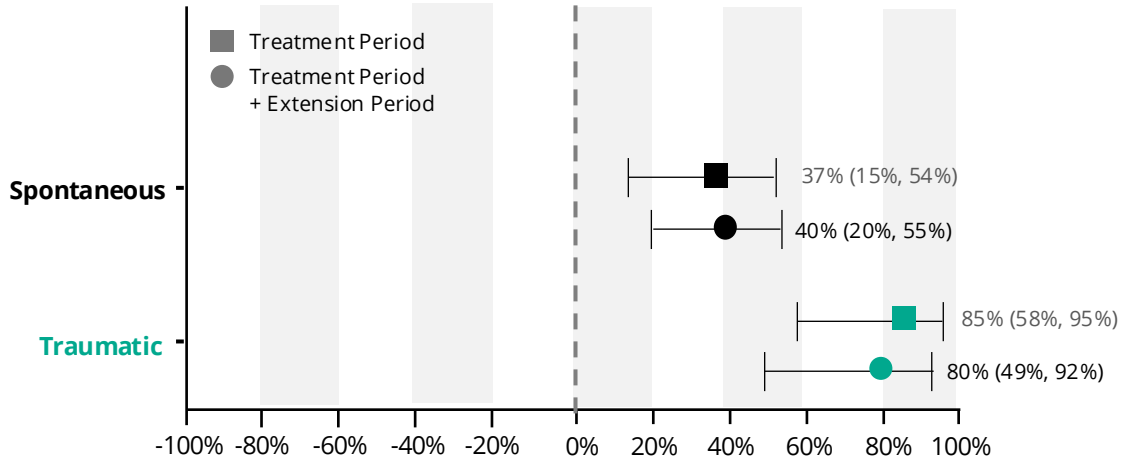
Estimated reduction in ATBR by type of bleeding event (Efficacy set, N=31)

Treatment Period vs Treatment + Extension Periods



Estimated reduction in ATBR by cause of bleeding event (Efficacy set, N=31)

Treatment Period vs Treatment + Extension Periods



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Efficacy analyses included comparison of the run-in and 12-week treatment + extension periods (data cut-off October 14, 2025).