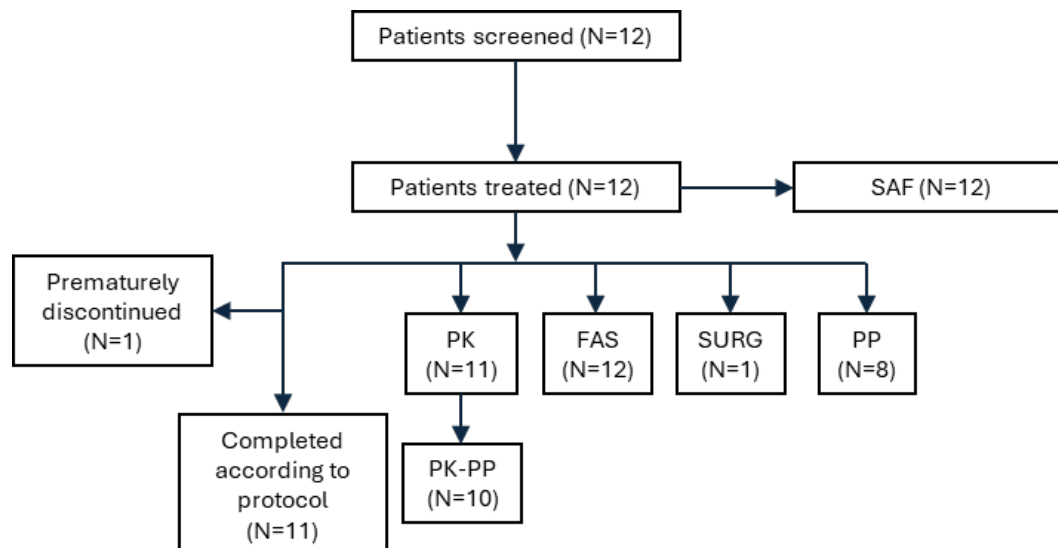


## Participant flow



FAS = full analysis set, PK = pharmacokinetics analysis set, PP = per-protocol set, SAF = safety analysis set, SURG = surgery set.

## Baseline characteristics

### Demographic and Baseline Characteristics of the Study Population (FAS/SAF, N= 12)

Parameter	Mean (SD)	Median (range)
Age (years)	2.5 (1.2)	2.0 (1.0–5.0)
Height (cm)	98.7 (10.5)	99.5 (81.0–123.0)
Weight (kg)	15.5 (3.4)	14.5 (12.0–24.3)
Body mass index (kg/m <sup>2</sup> )	15.9 (1.9)	15.8 (13.4–19.2)
Number (%)		
Sex		
Male	6 (50.0)	
Female	6 (50.0)	
Race		
White	10 (83.3)	
Arabian	2 (16.7)	
Blood group		
A	4 (33.3)	
B	2 (16.7)	
AB	2 (16.7)	
O	3 (25.0)	
Unknown	1 (8.3)	
VWD type		
Type 2	4 (33.3)	
Type 3	8 (66.7)	
VWF inhibitor history		
No	12 (100)	
FVIII inhibitor history		
No	12 (100)	
Family history of VWD		
Yes	6 (50.0)	
No	6 (50.0)	

FAS = full analysis set, FVIII = factor VIII, SAF = safety analysis set, SD = standard deviation, VWD = von Willebrand disease, VWF = von Willebrand factor.

## Outcome measures

### TABR during Prophylaxis with *Wilate*

Analysis set	All BEs			Treated BEs		
	n	TABR		n	TABR	
<b>FAS (N=12)</b>	56	4.6 (6.1)	3.0 (0–22.6)	45	3.7 (5.1)	2.4 (0–18.7)
<b>PP (N=8)</b>	24	3.0 (1.8)	3.0 (0–5.9)	17	2.1 (1.4)	2.4 (0–4)

BE = bleeding episode, FAS = full analysis set, N = number of patients; n = number of BEs, PP = per-protocol set, SD = standard deviation, TABR = total annualised bleeding rate.

TABRs are given as mean (SD) and median (range).

TABR is defined as the total number of BEs (i.e., spontaneous, traumatic, and other bleeds) occurring during the period from the first prophylactic dose of *Wilate* to the study completion visit, divided by the duration (in years) between these two time points. BEs that occurred during surgery periods were excluded from the calculation of TABR.

All BEs is defined as the totality of BEs that occurred during the study period, regardless of whether they were treated with *Wilate*. Treated BEs refers to the subset of BEs treated with *Wilate*.

### ABRs during Prophylaxis with *Wilate* by Bleeding Type

Analysis set Bleeding type	All BEs			Treated BEs		
	n	ABR		n	ABR	
<b>FAS (N=12)</b>	56			45		
Spontaneous	11	0.9 (1.2)	0.4 (0–3.0)	9	0.7 (1.1)	0 (0–3.0)
Traumatic	17	1.4 (1.7)	1.0 (0–5.9)	15	1.2 (1.7)	0.9 (0–5.9)
Other	28	2.3 (6.6)	0 (0–22.6)	21	1.7 (5.4)	0 (0–18.7)
<b>PP (N=8)</b>	24			17		
Spontaneous	9	1.1 (1.4)	0.5 (0–3.0)	7	0.9 (1.4)	0 (0–3.0)
Traumatic	10	1.2 (1.0)	1.0 (0–2.9)	8	1.0 (1.0)	1.0 (0–2.9)
Other	5	0.6 (1.8)	0 (0–4.9)	2	0.3 (0.7)	0 (0–2.0)

ABR = annualised bleeding rate, BE = bleeding episode, FAS = full analysis set, N = number of patients, n = number of BEs, PP = per-protocol set, SD = standard deviation.

ABRs are given as mean (SD) and median (range).

## Adverse events

### Display of TEAEs by System Organ Class and Preferred Term (SAF, N=12)

System Organ Class Preferred Term	Number (%) of patients	Number of TEAEs
<b>Any TEAE</b>	<b>10 (83.3)</b>	<b>46</b>
<b>Infections and infestations</b>	<b>7 (58.3)</b>	<b>16</b>
Bronchitis	1 (8.3)	1
Conjunctivitis	1 (8.3)	1
Ear infection	1 (8.3)	1
Gastroenteritis	1 (8.3)	1
Nasopharyngitis	2 (16.7)	2
Pharyngitis	1 (8.3)	1
Pneumonia	1 (8.3)	1
Pulpitis dental	1 (8.3)	1
Respiratory tract infection	1 (8.3)	1
Respiratory tract infection viral	1 (8.3)	1
Rhinitis	1 (8.3)	1
Scarlet fever	1 (8.3)	1
Soft tissue infection	1 (8.3)	2
Upper respiratory tract infection	1 (8.3)	1
<b>Blood and lymphatic system disorders</b>	<b>3 (25.0)</b>	<b>4</b>
Iron deficiency anaemia	3 (25.0)	4
<b>Metabolism and nutrition disorders</b>	<b>1 (8.3)</b>	<b>1</b>
Vitamin D deficiency	1 (8.3)	1
<b>Psychiatric disorder</b>	<b>1 (8.3)</b>	<b>1</b>
Attention deficit hyperactivity disorder	1 (8.3)	1
<b>Vascular disorder</b>	<b>1 (8.3)</b>	<b>1</b>
Hypertension	1 (8.3)	1
<b>Respiratory, thoracic and mediastinal disorders</b>	<b>2 (16.7)</b>	<b>2</b>
Cough	2 (16.7)	2
<b>Gastrointestinal disorders</b>	<b>2 (16.7)</b>	<b>10</b>
Dental caries	1 (8.3)	1
Mouth swelling	1 (8.3)	3
Toothache	1 (8.3)	6

<b>System Organ Class</b> Preferred Term	<b>Number (%) of patients</b>	<b>Number of TEAEs</b>
<b>Skin and subcutaneous tissue disorders</b>	<b>1 (8.3)</b>	<b>1</b>
Dermatitis contact	1 (8.3)	1
<b>General disorders and administration site conditions</b>	<b>3 (25.0)</b>	<b>7</b>
Pain	1 (8.3)	1
Pyrexia	2 (16.7)*	6*
<b>Injury, poisoning and procedural complications</b>	<b>2 (16.7)</b>	<b>3</b>
Limb injury	1 (8.3)	1
Lip injury	1 (8.3)	1
Skin laceration	1 (8.3)	1

SAF = safety analysis set, TEAE = treatment-emergent adverse event.

\*One mild instance of pyrexia was considered possibly related to study drug by the Sponsor and unrelated by the Investigator.