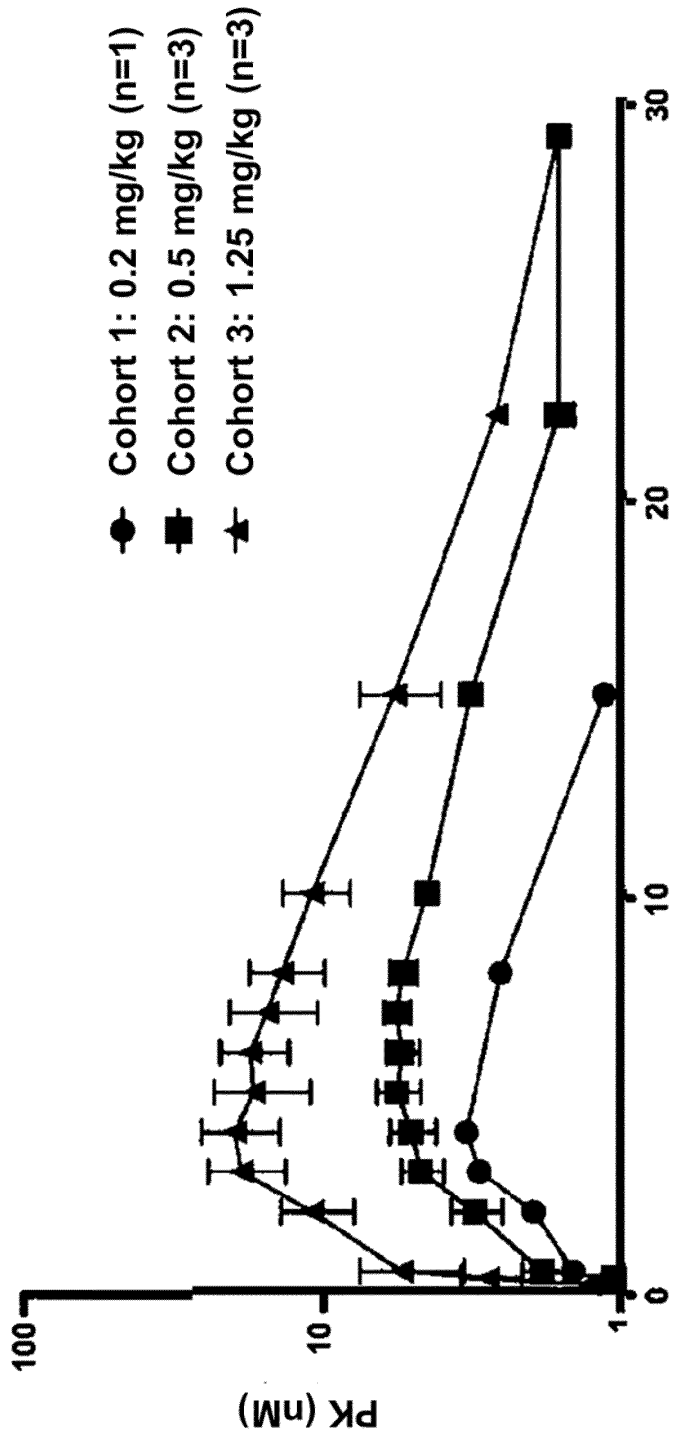

Cohort 1: 0.2 mg/kg (n=1)
Cohort 2: 0.5 mg/kg (n=3)
Cohort 3: 1.25 mg/kg (n=3)
PK (nM)
100
10
1
0
10
20
30
Days

FIG. 1

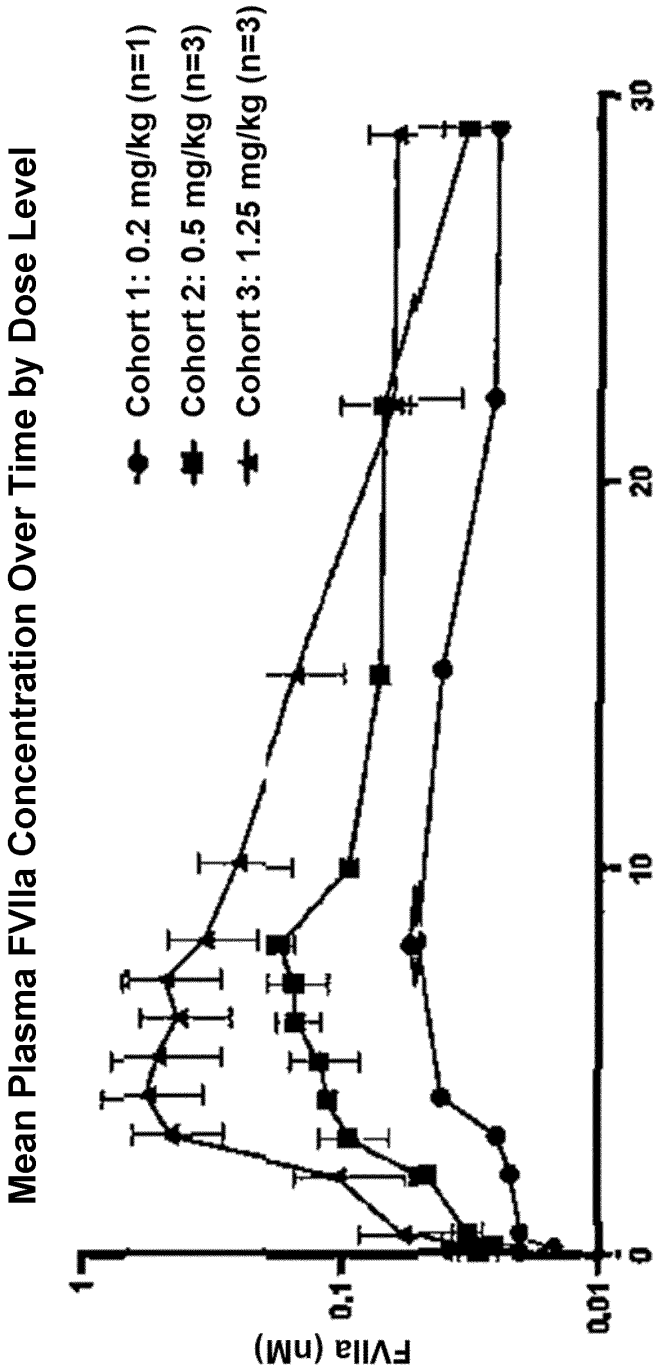

Mean Plasma FVIIa Concentration Over Time by Dose Level
Cohort 1: 0.2 mg/kg (n=1)
Cohort 2: 0.5 mg/kg (n=3)
Cohort 3: 1.25 mg/kg (n=3)
FVIIa (nM)
1
0,1
0,01
0
10
20
30
Days

FIG. 2A

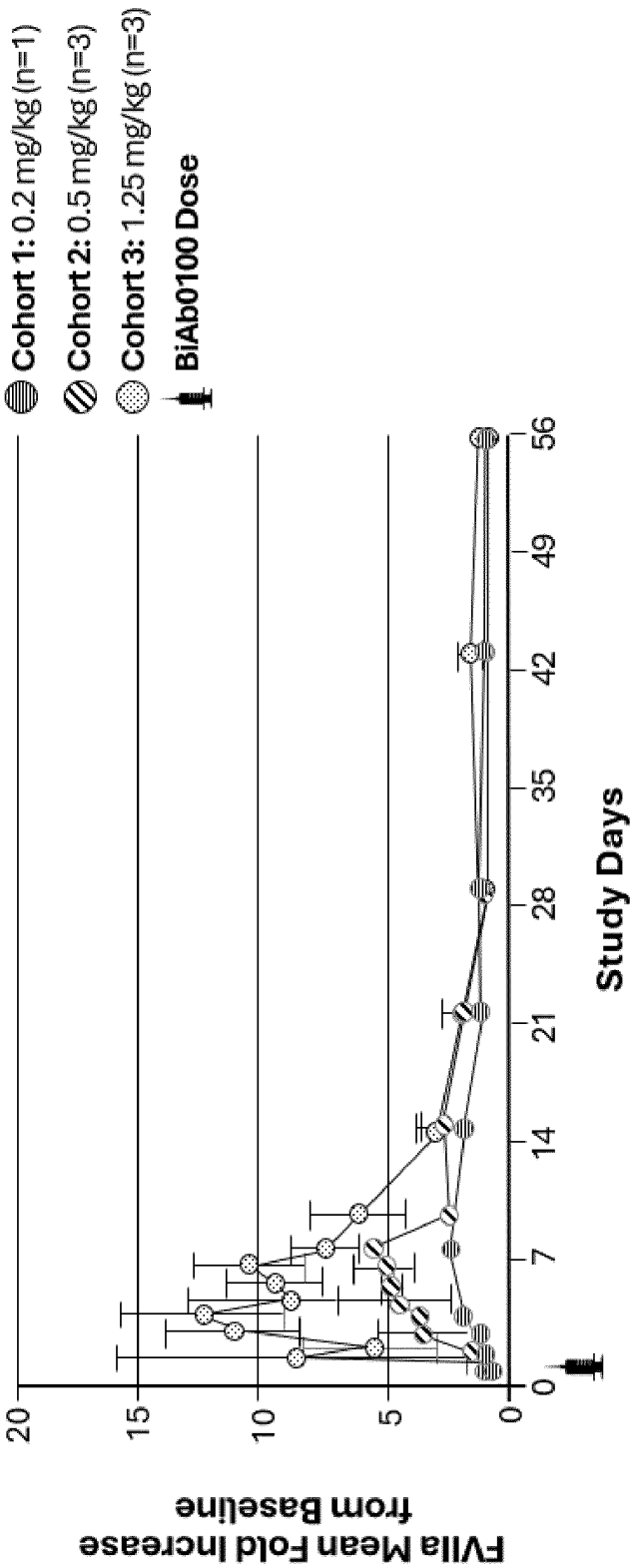

Cohort 1: 0.2 mg/kg (n=1)
Cohort 2: 0.5 mg/kg (n=3)
Cohort 3: 1.25 mg/kg (n=3)
BiAb0100 Dose
FVIIa Mean Fold Increase from Baseline
20
15
10
5
0
0
7
14
21
28
35
42
49
56
Study Days

FIG. 2B

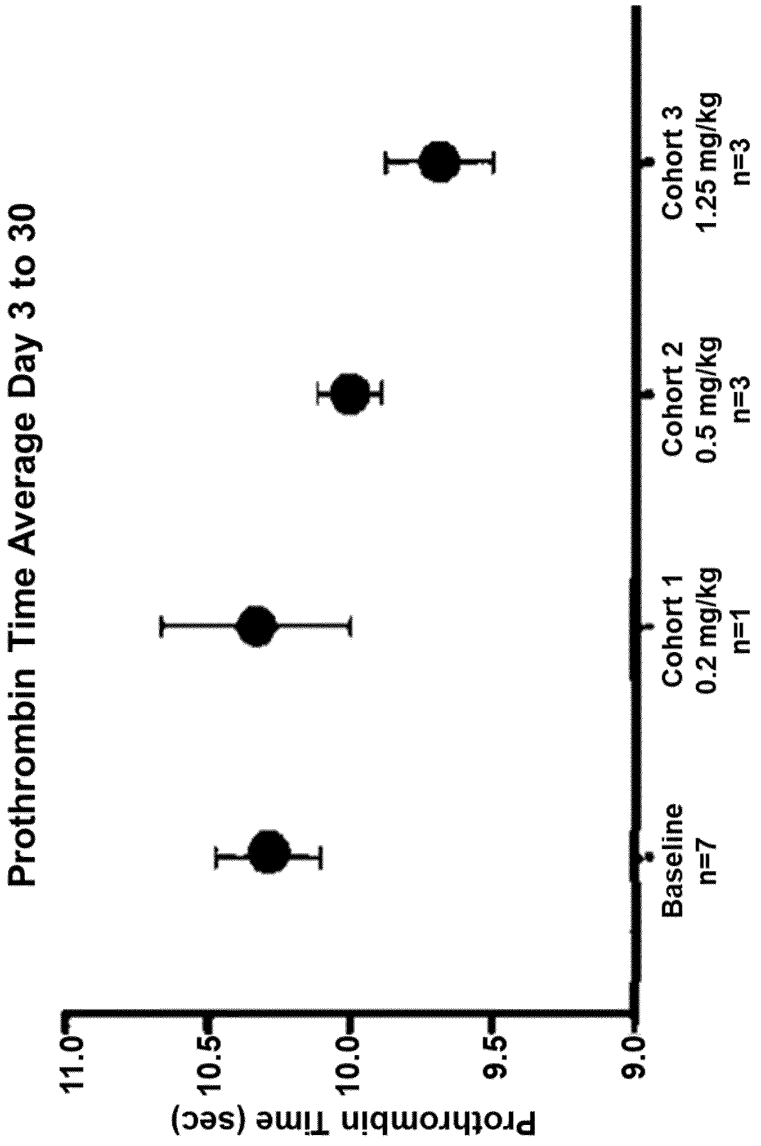

Prothrombin Time Average Day 3 to 30
Prothrombin Time (sec)
11.0
10.5
10.0
9.5
9.0
Baseline
n=7
Cohort 1
0.2 mg/kg
n=1
Cohort 2
0.5 mg/kg
n=3
Cohort 3
1.25 mg/kg
n=3

FIG. 3

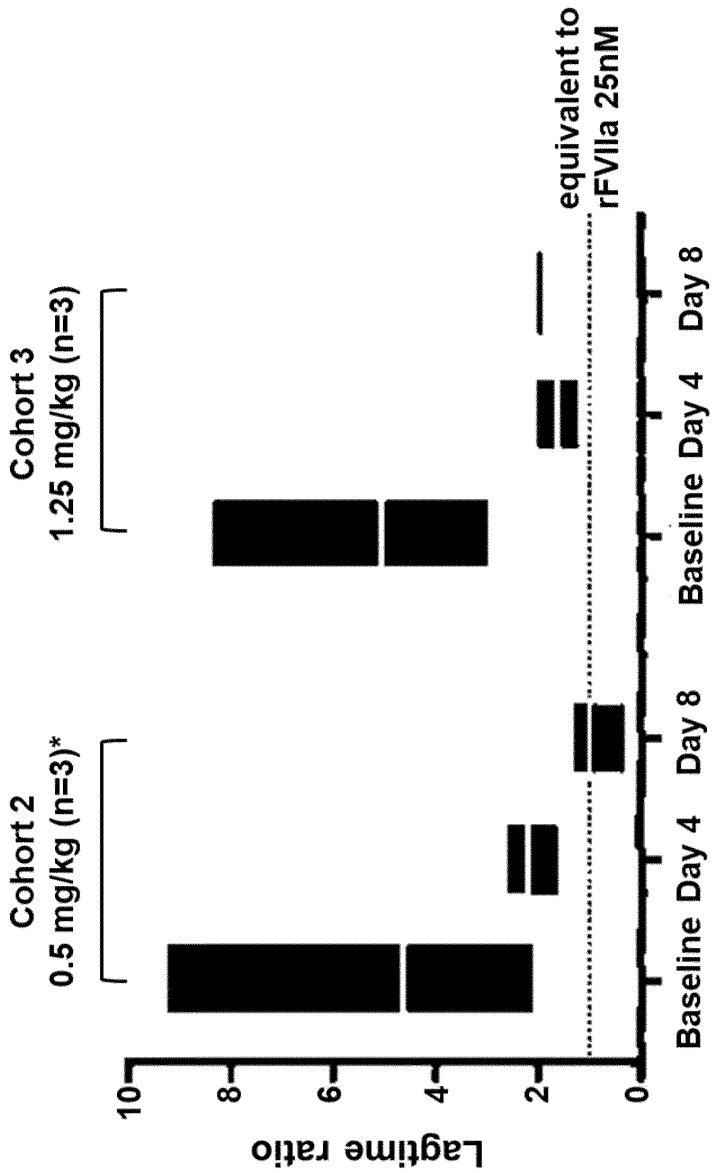

Lagtime ratio
10
8
6
4
2
0
Cohort 2
0.5 mg/kg (n=3)*
Cohort 3
1.25 mg/kg (n=3)
equivalent to
rFVIIa 25nM
Baseline Day 4
Day 8
Baseline Day 4
Day 8

FIG. 4A

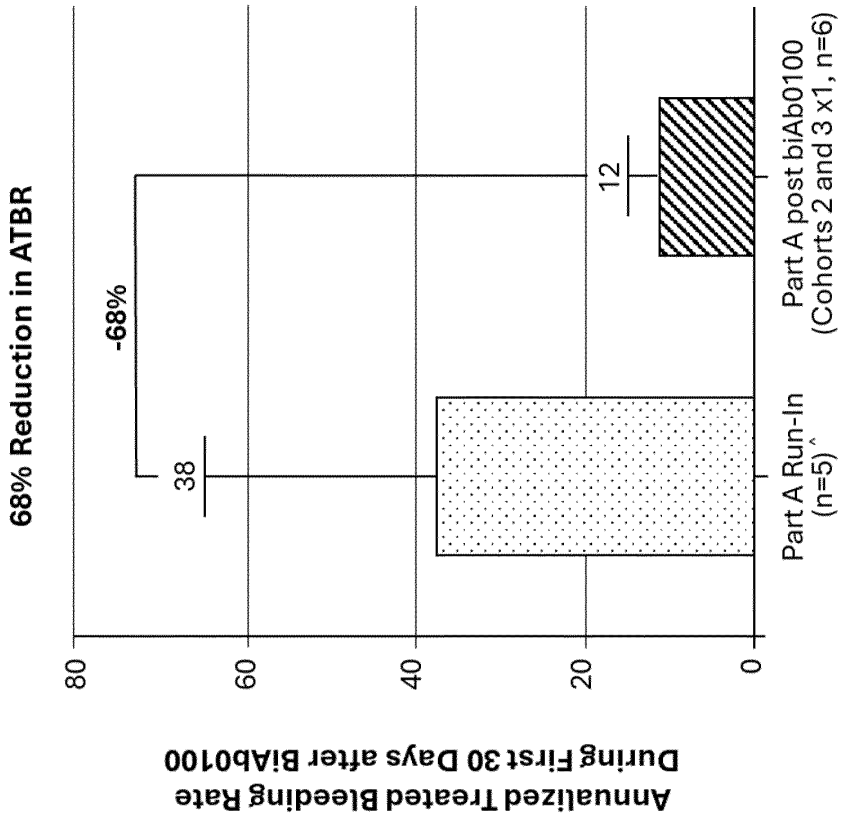

68% Reduction in ATBR
-68%
38
12
0
20
40
60
80
Annualized Treated Bleeding Rate During First 30 Days after BiAb0100
Part A Run-In (n=5)^
Part A post biAb0100 (Cohorts 2 and 3 x1, n=6)

FIG. 4B

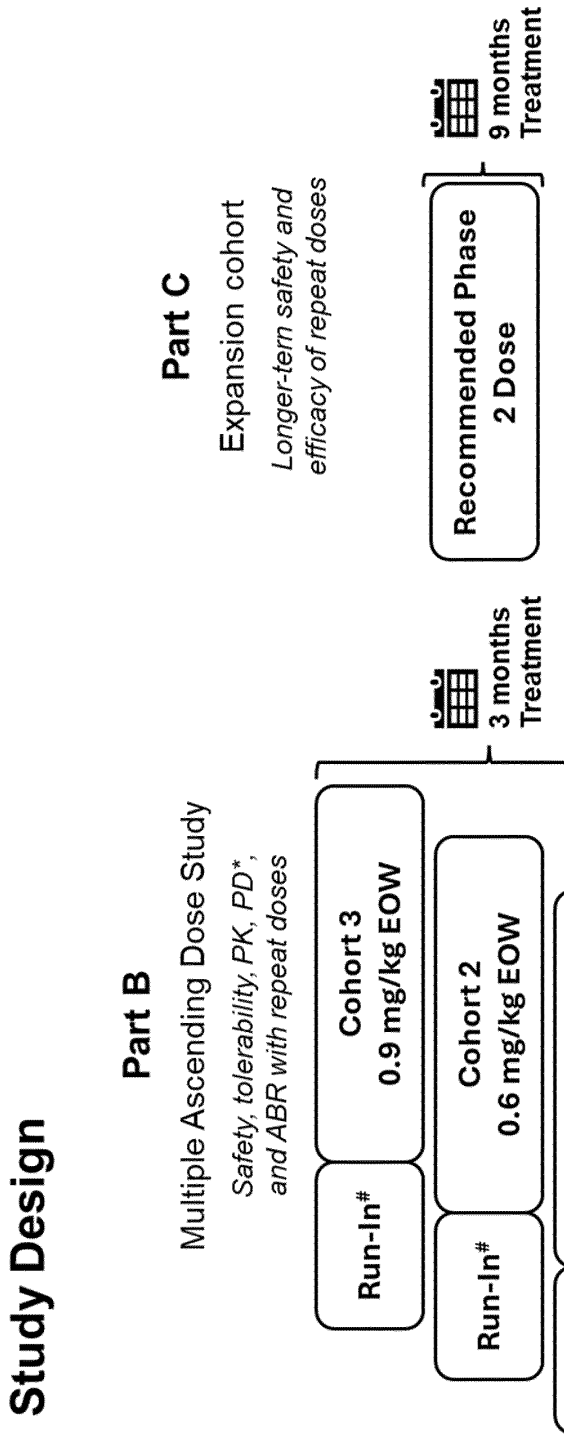

Study Design
Part B
Multiple Ascending Dose Study
Safety, tolerability, PK, PD*, and ABR with repeat doses
Run-In#
Cohort 3
0.9 mg/kg EOW
Run-In#
Cohort 2
0.6 mg/kg EOW
Run-In#
Cohort 1
0.3 mg/kg EOW
3 months Treatment
Part C
Expansion cohort
Longer-term safety and efficacy of repeat doses
Recommended Phase 2 Dose
9 months Treatment

FIG. 5

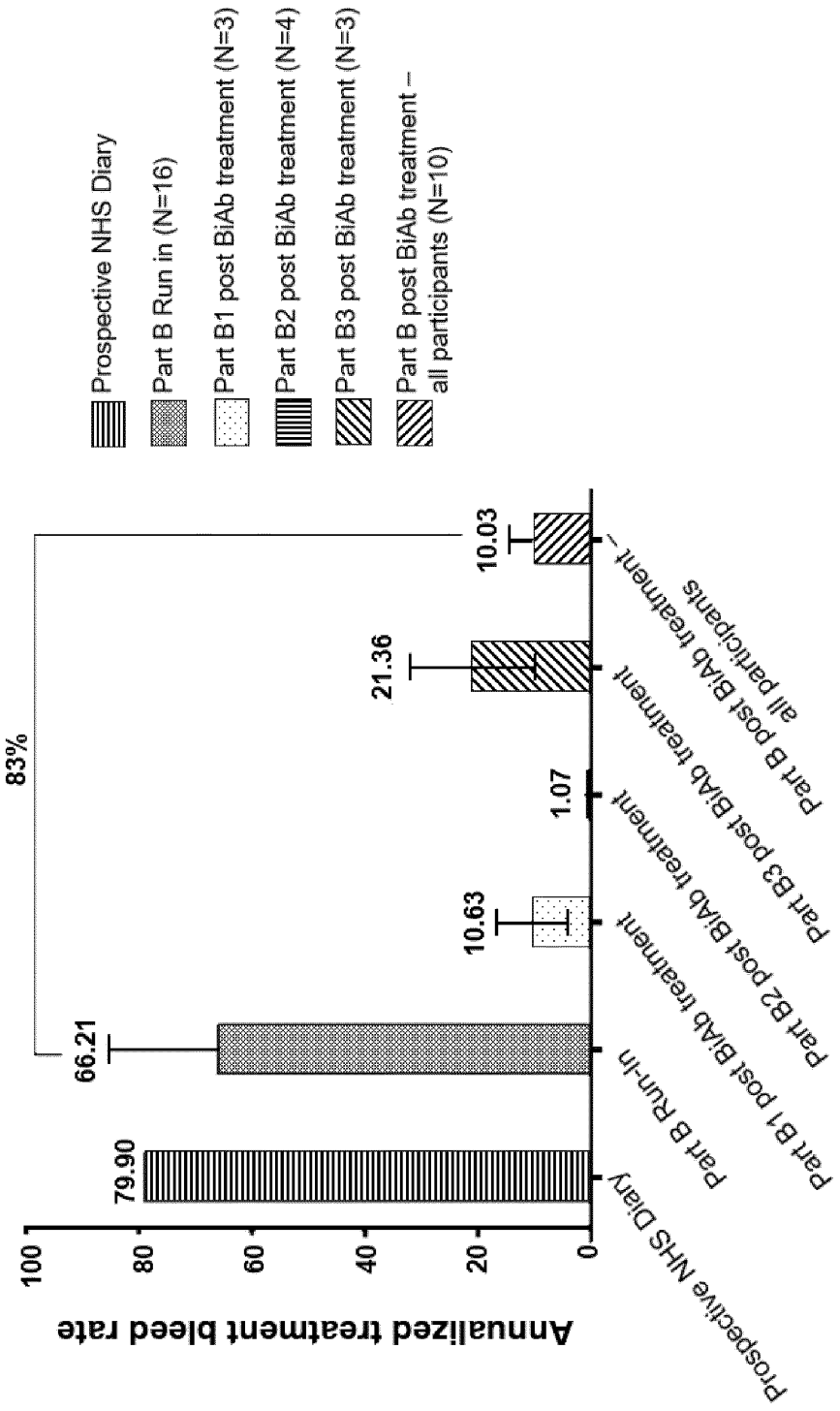

Prospective NHS Diary
Part B Run in (N=16)
Part B1 post BiAb treatment (N=3)
Part B2 post BiAb treatment (N=4)
Part B3 post BiAb treatment (N=3)
Part B post BiAb treatment – all participants (N=10)
Annualized treatment bleed rate
100
80
60
40
20
0
79.90
66.21
83%
10.63
1.07
21.36
10.03
Prospective NHS Diary
Part B Run-in
Part B1 post BiAb treatment
Part B2 post BiAb treatment
Part B3 post BiAb treatment
Part B post BiAb treatment – all participants

FIG. 6

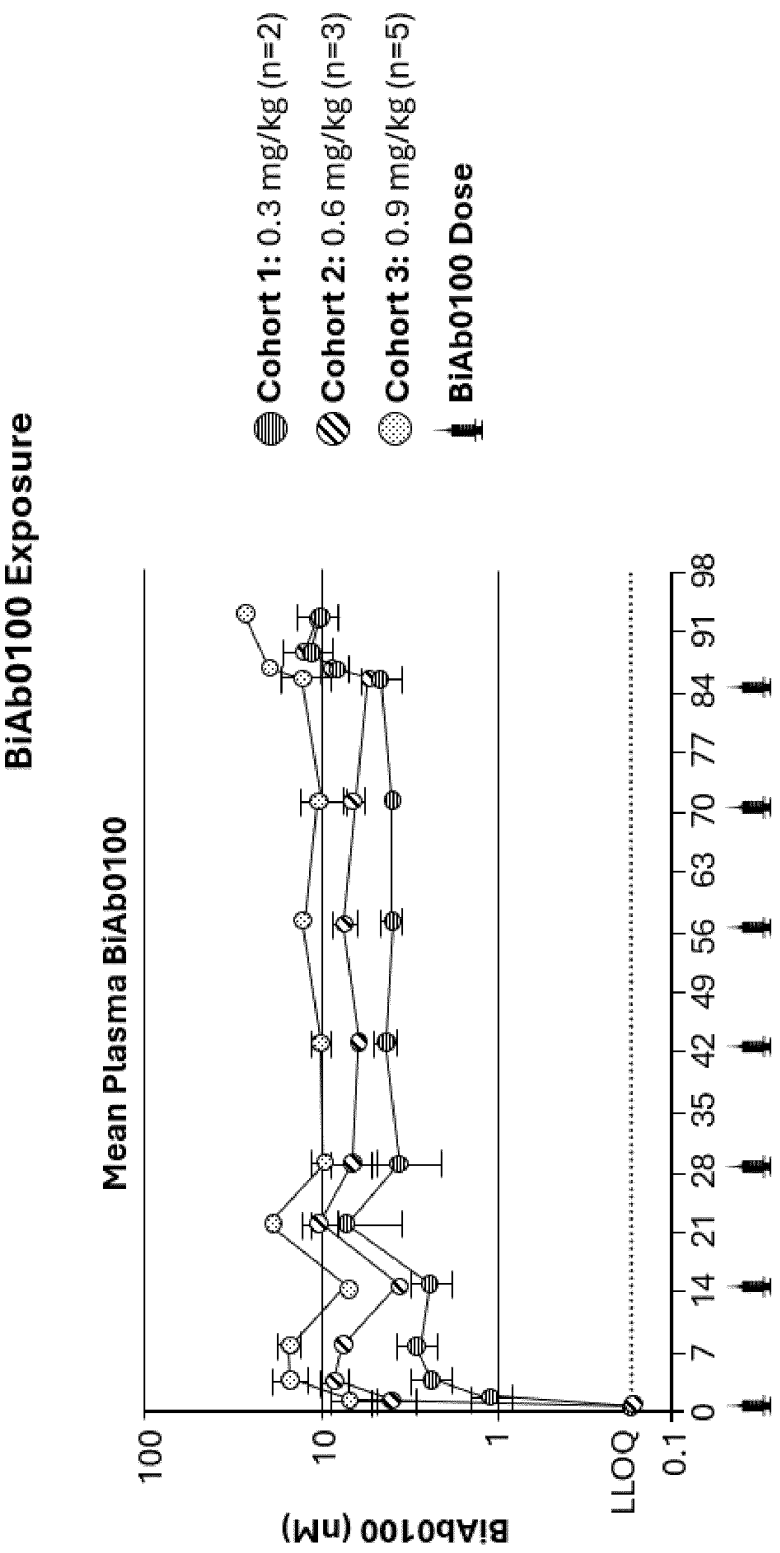

BiAb0100 Exposure
Mean Plasma BiAb0100
Cohort 1: 0.3 mg/kg (n=2)
Cohort 2: 0.6 mg/kg (n=3)
Cohort 3: 0.9 mg/kg (n=5)
BiAb0100 Dose
BiAb0100 (nM)
100
10
1
LLOQ
0.1
0
7
14
21
28
35
42
49
56
63
70
77
84
91
98
Study Days

FIG. 7A

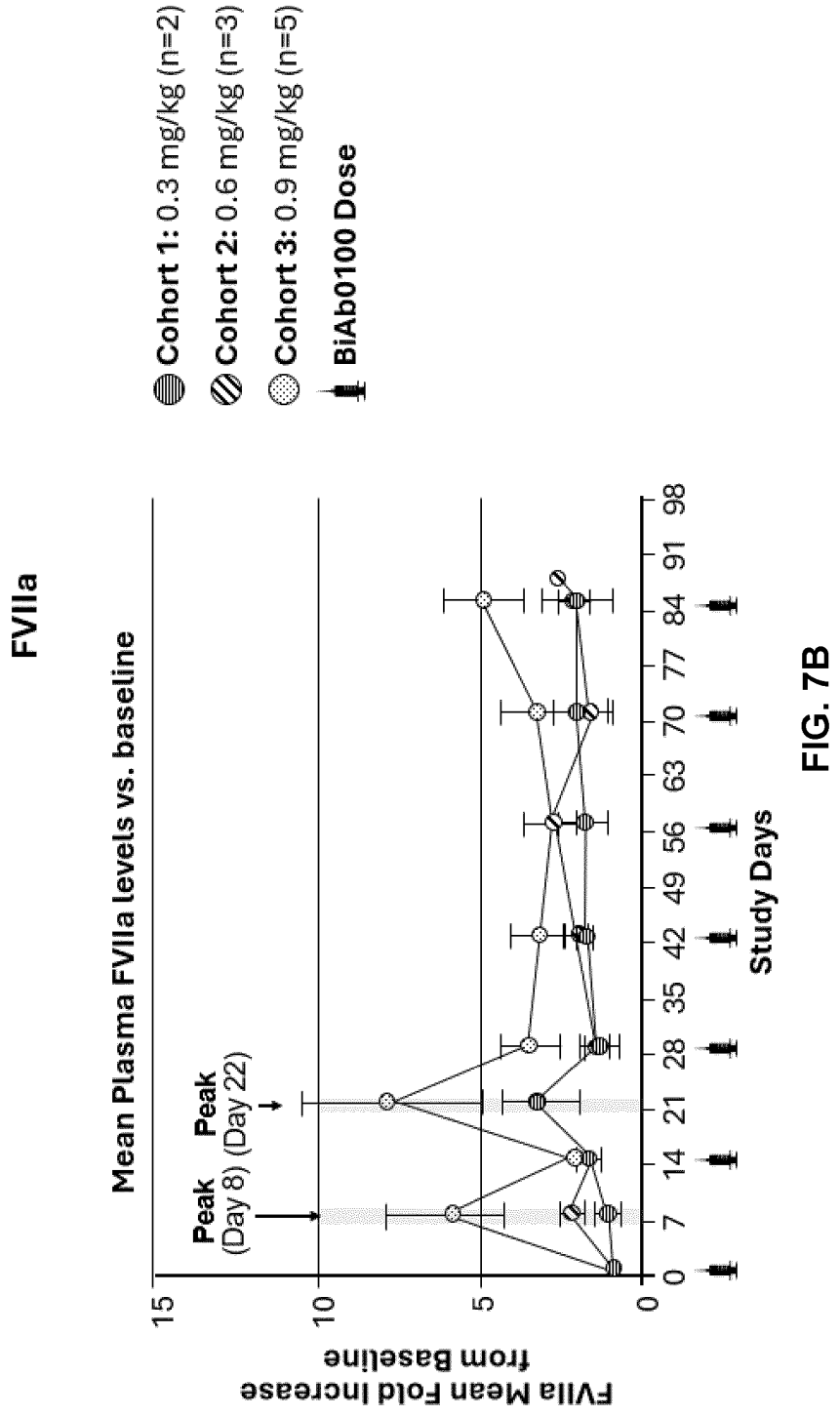

FVIIa
Mean Plasma FVIIa levels vs. baseline
Cohort 1: 0.3 mg/kg (n=2)
Cohort 2: 0.6 mg/kg (n=3)
Cohort 3: 0.9 mg/kg (n=5)
BiAb0100 Dose
Peak (Day 8)
Peak (Day 22)
FVIIa Mean Fold Increase from Baseline
Study Days
0
5
10
15
0
7
14
21
28
35
42
49
56
63
70
77
84
91
98

FIG. 7B

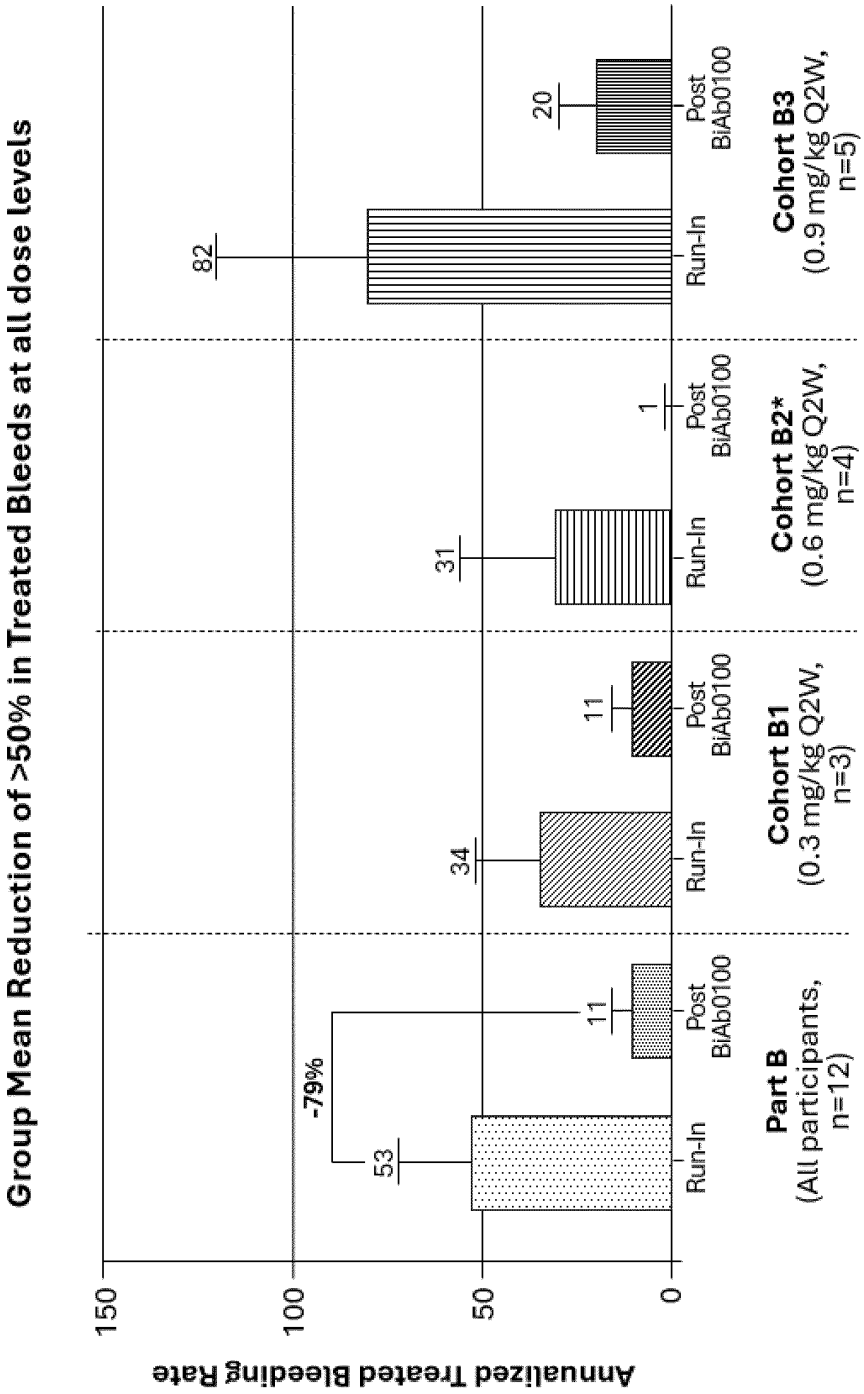

Group Mean Reduction of >50% in Treated Bleeds at all dose levels
Annualized Treated Bleeding Rate
150
100
50
0
-79%
53
11
34
11
31
1
82
20
Run-In
Post BiAb0100
Part B
(All participants, n=12)
Cohort B1
(0.3 mg/kg Q2W, n=3)
Cohort B2*
(0.6 mg/kg Q2W, n=4)
Cohort B3
(0.9 mg/kg Q2W, n=5)

FIG. 8

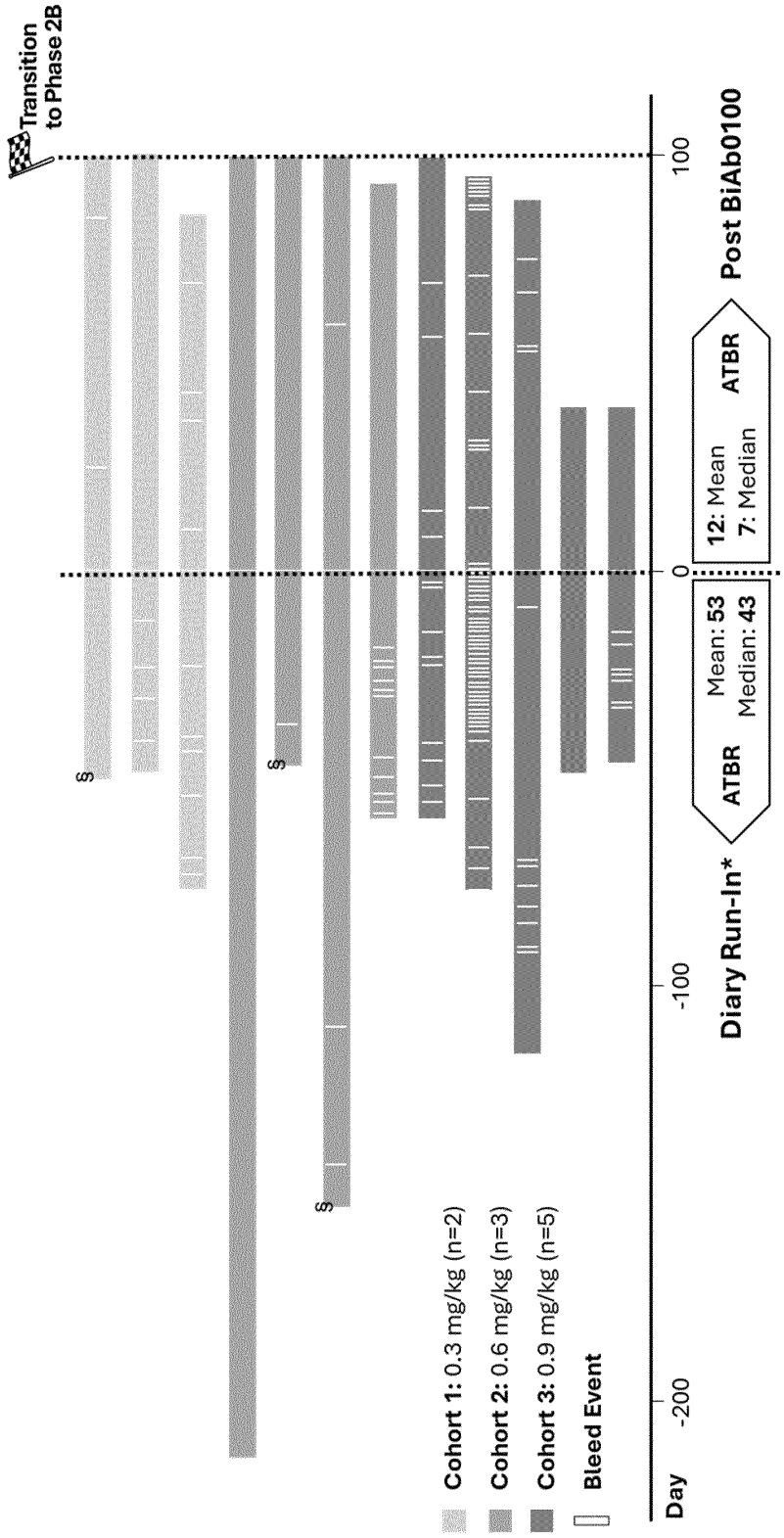

Transition to Phase 2B
Cohort 1: 0.3 mg/kg (n=2)
Cohort 2: 0.6 mg/kg (n=3)
Cohort 3: 0.9 mg/kg (n=5)
Bleed Event
Day
-200
-100
0
100
Diary Run-In*
ATBR
Mean: 53
Median: 43
12: Mean
7: Median
ATBR
Post BiAb0100

FIG. 9

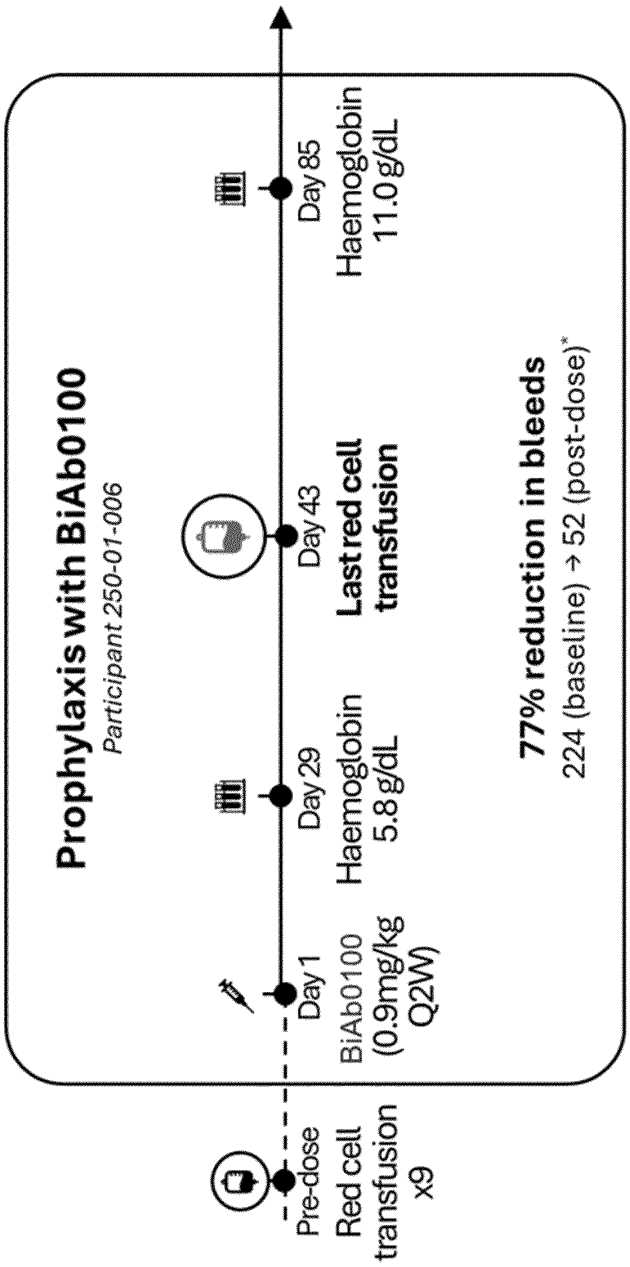

Prophylaxis with BiAb0100
Participant 250-01-006
Pre-dose
Red cell transfusion x9
Day 1
BiAb0100 (0.9mg/kg Q2W)
Day 29
Haemoglobin 5.8 g/dL
Day 43
Last red cell transfusion
Day 85
Haemoglobin 11.0 g/dL
77% reduction in bleeds
224 (baseline) → 52 (post-dose)*

FIG. 10