

Real-World Treatment Patterns Among People With Moderate-to-Severe Hemophilia A and B in China: A Retrospective Database Study

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BACKGROUND

- Hemophilia is an X-linked inherited bleeding disorder characterized by the deficiency of coagulation factor VIII (FVIII) or factor IX (FIX), affecting approximately 5.5 per 100,000 males in China.^{1,2}
- The World Federation of Hemophilia recommends prophylactic treatment as a standard of care (SoC) for severe hemophilia.^{1,3} However, the high factor consumption and cost hinder the use of standard and intermediate dose prophylaxis in many developing countries. In China, the SoC consists of low-dose prophylaxis with standard half-life (SHL) FVIII and FIX replacement therapies and immune tolerance induction (ITI) regimens.^{2,4}
- Currently, there is a scarcity of evidence describing the hemophilia treatment landscape in China.

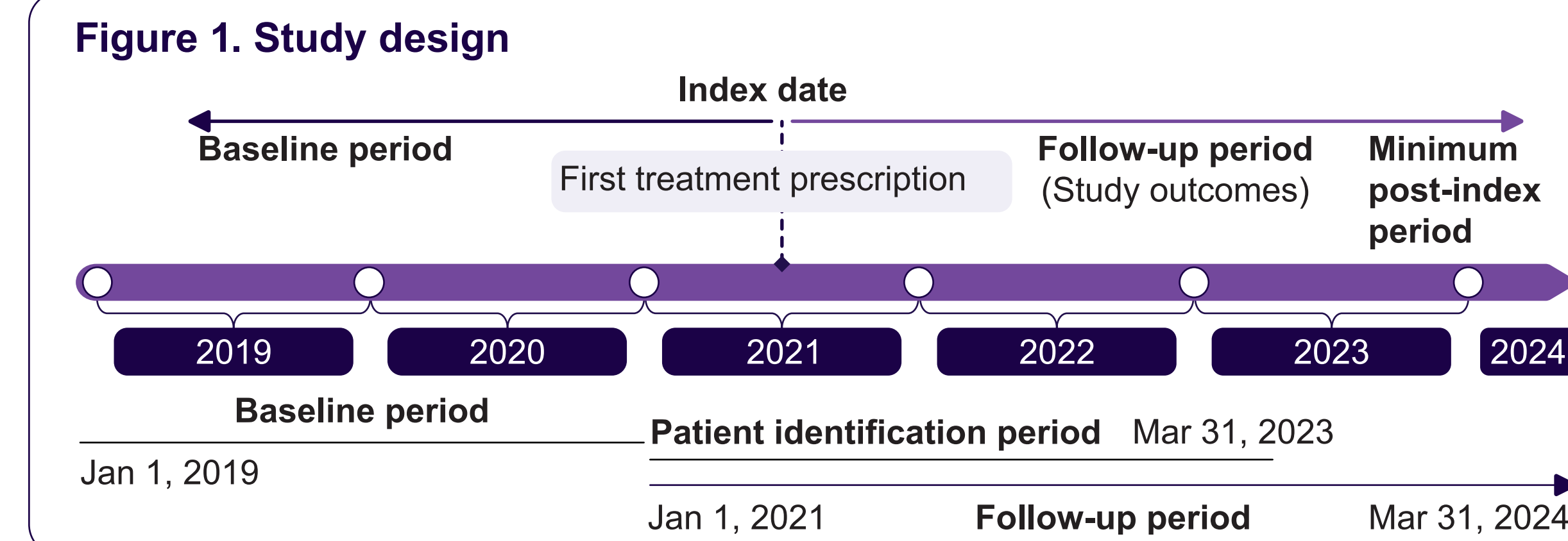
OBJECTIVE

To assess clinical burden and real-world treatment patterns among people with moderate-to-severe hemophilia A or B in China.

METHODS

Study design

- This retrospective, observational study utilized China's National Population Health Data Center's (NPHDC) Chronic Disease Prevention and Control Database.
- Males aged ≥ 12 years diagnosed with moderate-to-severe hemophilia A or B in China were identified retrospectively during patient identification period between January 1, 2021, and March 31, 2023.
- Patients were indexed on the first identified hemophilia prescription during the patient identification period and followed up until death or study end (March 31, 2024) (Figure 1).
- A minimum baseline period of 6 months and a post-index period of 12 months were required for inclusion in the study, except in case of death.



Assessments

- Bleeding events were captured and analyzed during the follow-up period.
- Treatment regimen was evaluated based on the yearly factor consumption per kilogram for individuals with available weight data or treatment frequency in case weight data is not available: on-demand: < 1000 international unit (IU)/kg/year or ≥ 2 prescription/year with frequency "when necessary"; prophylaxis: ≥ 1000 IU/kg/year and ≤ 4000 IU/kg/year or ≥ 2 prescription/year with frequency "twice/week or once/week"; ITI: > 4000 IU/kg/year or ≥ 2 prescription/year with frequency "3 times/week or once/2 days".
- Treatment regimen patterns were analyzed during the follow-up in consecutive 12-month observation periods.
- Treatment regimen switch was defined as a change in the treatment regimen (prophylaxis or on-demand or ITI) between two consecutive 12-month treatment periods and was analyzed during the follow-up duration. A treatment switch was defined as the absence of further prescriptions of initial hemophilia treatment, followed by a prescription of another treatment and was analyzed over the follow-up period.
- Prophylactic treatment persistence was defined as the period from prophylaxis initiation (date of the first prescription of factor replacement during the 12-month treatment period) to discontinuation and analyzed yearly during the follow-up period.
- Data were summarized using descriptive statistics. Kaplan–Meier analysis was used to estimate prophylactic treatment persistence.

RESULTS

Patients

- This study included 602 male people with hemophilia (PwH); of these, 94.0% ($n = 566$) had hemophilia A and 6.0% ($n = 36$) had hemophilia B.
- Mean (SD) age of the overall population at index was 34.3 (15.9) years. Patient demographics and clinical characteristics are presented in Table 1.

Table 1. Patient demographics and clinical characteristics

Characteristic	Value (N = 602)
Age at index (years), Mean (SD)	34.3 (15.9)
Age class at index (years), n (%)	
12–17	108 (18.0)
18–64	471 (78.2)
≥ 65	23 (3.8)
Weight (kg), Mean (SD)	67.2 (13.1)
Hemophilia type, n (%)	
Hemophilia A	566 (94.0)
Hemophilia B	36 (6.0)
Disease severity, n (%)	
Moderate hemophilia	123 (20.4)
Severe hemophilia	479 (79.6)
Inhibitor status, n (%)	
With inhibitors	129 (21.4)
Without inhibitors	473 (78.6)
Charlson comorbidity index, Mean (SD)	0.37 (0.9)
Most common comorbidities, n (%)	
Hypertension	107 (17.8)
Hepatitis C	64 (10.6)
Osteoporosis	50 (8.3)
Anemia	45 (7.5)
Type 2 diabetes	36 (6.0)
Intracranial hemorrhage	23 (3.8)
Chronic ischemic cardiomyopathy	17 (2.8)
Deep venous thrombosis	15 (2.5)
Thromboembolic events	15 (2.5)
Depression	12 (2.0)
Hepatitis B	11 (1.8)

N, total number of patients; n, number of patients with data for the specified category.

Clinical burden

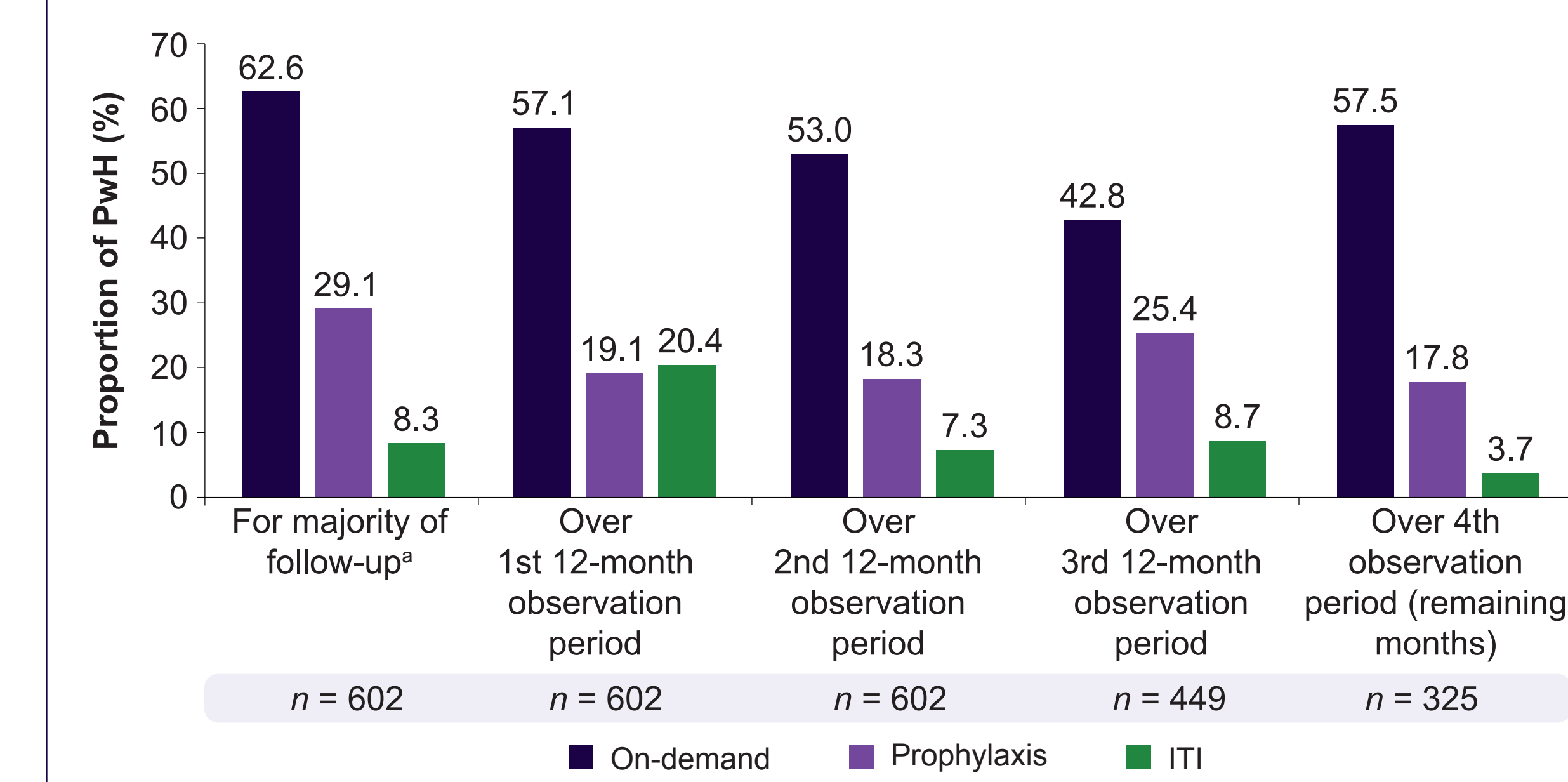
- During the follow-up period, ≥ 1 bleeding episode was documented in 77.7% ($n = 468$) of PwH.
- At least one joint bleeding episode was documented in 36.7% ($n = 221$) of PwH.

Treatment regimen pattern

- Overall, 29.1% ($n = 175$) of PwH received prophylaxis for the majority of their follow-up and were classified as patients with prophylaxis.
- About 62.6% ($n = 377$) received on-demand treatment for most of their follow-up and were classified as patients with on-demand treatment.
- About 8.3% ($n = 50$) received ITI as their predominant treatment and were classified as patients with ITI treatment (Figure 2).

- From the first-year to the third-year follow-up, the proportion of PwH receiving the on-demand treatment regimen declined from 57.1% to 42.8%, whereas the proportion of PwH receiving the prophylaxis treatment regimen increased from 19.1% to 25.4%, respectively (Figure 2).
- During the follow-up period, 87.4% ($n = 526$) of PwH experienced ≥ 1 treatment regimen switch. Mean (SD) number of treatment regimen switches during the follow-up was 1.32 (0.8).

Figure 2. Treatment regimen patterns among PwH



^aPatients were assigned to a treatment regimen subgroup based on the regimen they received for the maximum duration of their follow-up period. ITI, immune tolerance induction; n, number of patients analyzed for the specified category; PwH, people with hemophilia.

Hemophilia treatment among PwH receiving on-demand treatment regimen

- Standard half-life recombinant factor was the most documented treatment (94.7% of PwH, $n = 357$) among the people who received the on-demand treatment regimen; mean (SD) monthly dose and monthly frequency were 2,483.7 (3,801.4) IU and 1.2 (1.8), respectively (Table 2).
- About 10.3% ($n = 39$) of people who received the on-demand regimen had ≥ 1 treatment switch; mean (SD) number of treatment switches per person was 0.4 (1.8).
- Most PwH (88.3%, $n = 333$) had ≥ 1 extended interval without recorded treatment; mean (SD) duration of the extended interval was 597.4 (362.7) days.

Table 2. Hemophilia treatments among PwH

Treatment	PwH receiving on-demand treatment regimen (N = 377) ^a	PwH receiving prophylactic treatment regimen (N = 175) ^a
SHL recombinant factor, n (%)	357 (94.7)	175 (100)
Monthly dose, (IU), Mean (SD)	2,483.7 (3,801.4)	7,991.5 (4,457.7)
Monthly frequency, Mean (SD)	1.2 (1.8)	1.7 (1.2)
Bypassing agent (PCC), n (%)	50 (13.3)	17 (9.7)
Monthly dose, (IU), Mean (SD)	1802.4 (2536.4)	2,051.8 (2,701.0)
Monthly frequency, Mean (SD)	0.6 (0.7)	0.6 (0.8)
Desmopressin, n (%)	9 (2.4)	2 (1.1)
Monthly dose, (μ g), Mean (SD)	1.3 (0.9)	1.8 (1.0)
Monthly frequency, Mean (SD)	0.1 (0.1)	0.1 (0.0)
Tranexamic acid, n (%)	17 (4.5)	27 (4.5)
Monthly dose, (mg), Mean (SD)	199.4 (190.8)	186.3 (279.1)
Monthly frequency, Mean (SD)	0.1 (0.1)	0.1 (0.2)

^aAs PwH were receiving > 1 treatment, total percentage for treatment type is above 100%.

^aNumbers 377 and 175 represent patients who received on-demand and prophylactic treatment regimens, respectively, for the maximum duration of their follow-up period. IU, international unit; N, total number of patients; PCC, prothrombin complex concentrate; PwH, people with hemophilia; SHL, standard half-life.

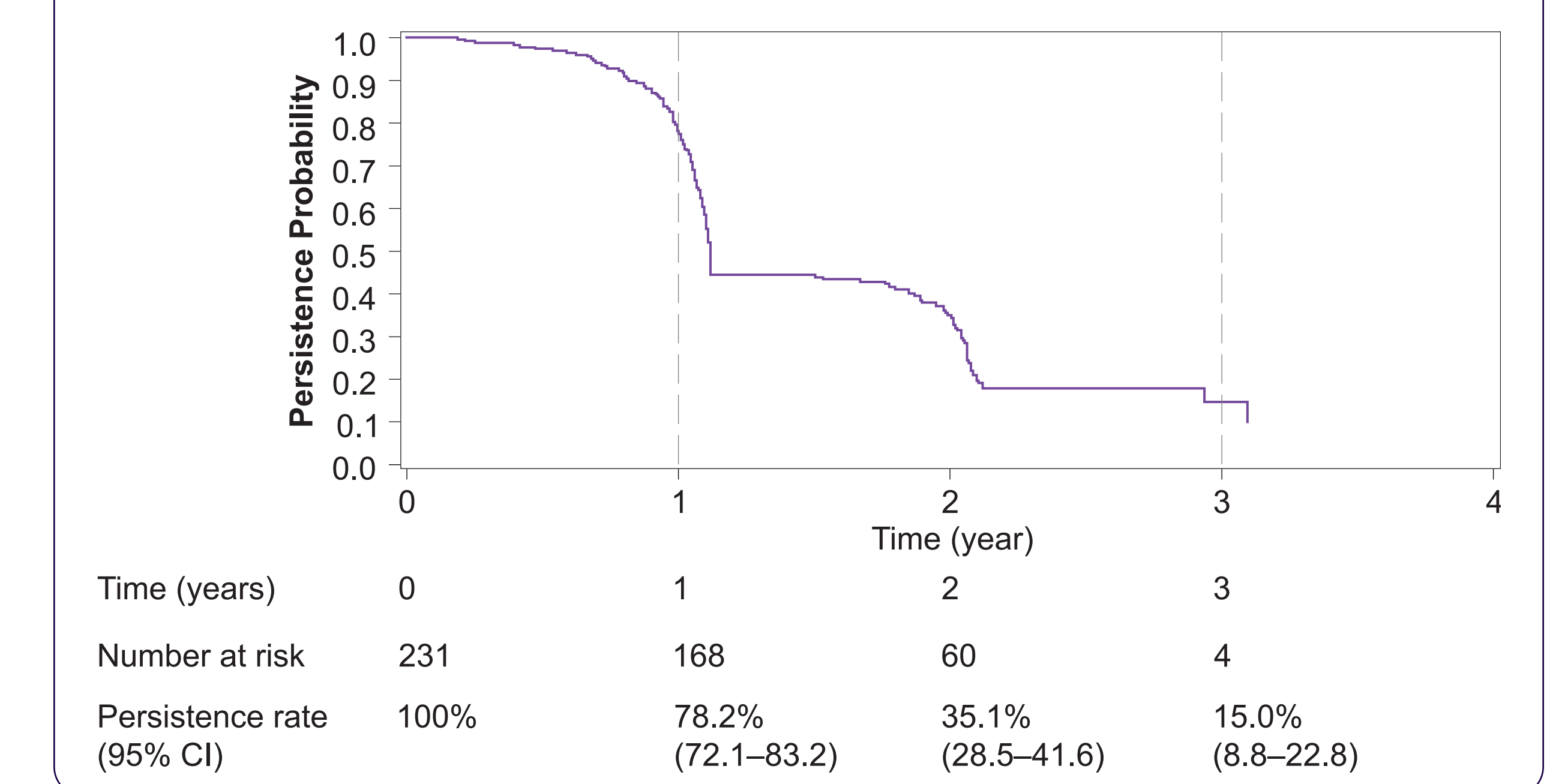
Hemophilia treatment among PwH receiving prophylactic treatment regimen

- Standard half-life recombinant factor was the most documented treatment (100% of PwH, $n = 175$) among the people who received prophylactic treatment regimen; mean (SD) monthly dose and monthly frequency were 7,991.5 (4,457.7) IU and 1.7 (1.2), respectively (Table 2).
- About 13.7% ($n = 24$) of people who received the prophylactic regimen had ≥ 1 treatment switch; mean (SD) number of treatment switches per person was 1.0 (4.0).
- The majority of PwH (86.3%, $n = 151$) had ≥ 1 treatment discontinuation; mean (SD) duration of treatment discontinuation was 418.2 (303.2) days.

Prophylactic treatment persistence

- Among the 231 people who initiated the prophylaxis treatment regimen at least once during follow-up, 168 people remained on prophylaxis (16 censored) after 1 year, 60 remained (35 censored) after 2 years, and only 4 remained (61 censored) after 3 years, reflecting a declining persistence from 78.2% (95% CI: 72.1–83.2) by Year 1 to 15% (95% CI: 8.8–22.8) by Year 3 (Figure 3).

Figure 3. Kaplan–Meier curve of prophylactic treatment persistence



LIMITATIONS

- The electronic health record data only captured bleeding events and factor consumption in hospital settings, potentially underestimating bleeding events that were treated by factor replacement therapy at home.
- Available data only captured partial patient care, resulting in missing information of acute bleeding episodes managed elsewhere.
- Information loss was greater for PwH receiving the on-demand treatment due to less frequent follow-up compared to prophylaxis, leading to underestimation of bleeding events and factor consumption in this group.

CONCLUSIONS

- Majority of PwH in China receive on-demand treatments following bleeding episodes.
- Despite a low overall rate of regular prophylaxis, evolving clinical practices reflect an encouraging trend toward greater prophylaxis use.
- Ongoing challenges in hemophilia care in China include frequent treatment discontinuations and lower rate of prophylaxis, which underscore the opportunity for optimized treatment strategies in hemophilia management for PwH in China.

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COI DISCLOSURES

AH was an employee of Oracle at the time of the study. EN and MF are employees of Oracle. MG was an employee of Cerner Enviza, an Oracle company at the time of the study. AA, JC, AC, and MA are employees of Sanofi and may own stock/stock options in Sanofi. XB was an employee of Sanofi at the time of the study.

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