

## INTRODUCTION

- On-demand or prophylactic factor IX (FIX) replacement therapy is the recommended treatment for hemophilia B patients.
- Little evidence exists on the effectiveness of FIX in managing bleeding rates when used prophylactically.

## OBJECTIVE

The aim of this study was to examine real-world evidence on bleeding rates (annualized bleeding rate, ABR) among patients undergoing prophylactic FIX therapy in the U.S.

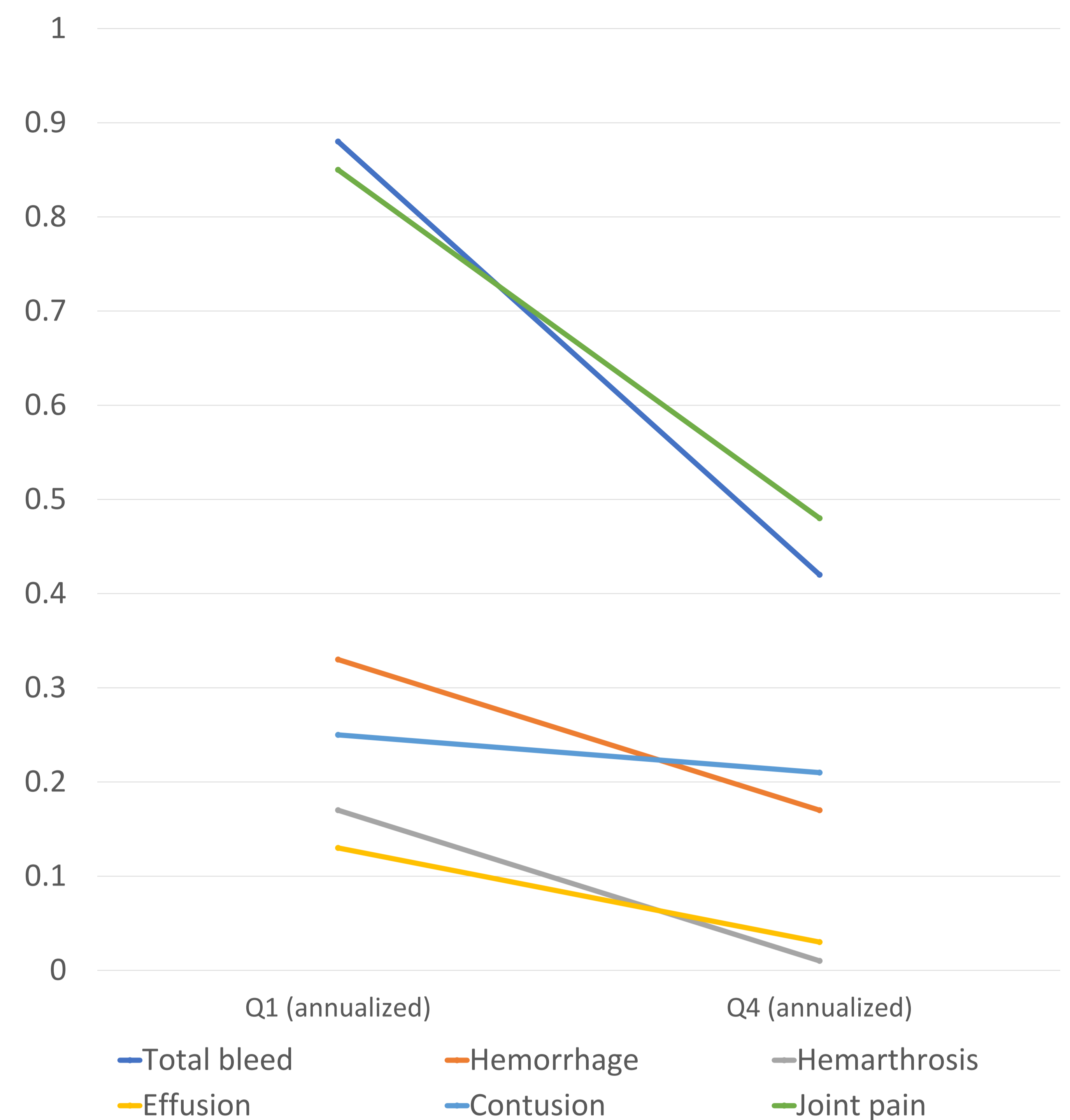
## METHODS

- Using IBM MarketScan Commercial Claims and Medicare data from 2010 to 2018, FIX therapy initiators were identified.
- Male patients, with at least 6 months of baseline and 1 year of follow-up enrollment were included.
- Those having  $\geq 2$  FIX prescriptions or procedures with no bleeding episodes (hemorrhages, hemarthrosis, effusion, or contusion) in the first 3 months after treatment initiation were defined as prophylactic users.
- Similar types of bleeding events in similar body parts within 7 days were considered as single episodes.
- After a 6 month wash-out period, ABR and annualized joint pain were estimated using observations in the final 3 months follow-up (months 10 to 12 after therapy initiation).

## RESULTS

**TABLE 1. Characteristics of Hemophilia B patients receiving factor IX therapy between 2010-2019**

	Prophylactic users	On-demand users	Total	p value
<b>N, (%)</b>	197 (49%)	205 (51%)	402	
<b>Age, median (q1, q3), years</b>	30 (14, 56)	26 (15, 52)	28 (15, 54)	
<b>Age category, n (%), years</b>				
<12	33 (16.75)	34 (16.59)	67 (16.67)	0.99
12-17	25 (12.69)	25 (12.20)	50 (12.44)	
$\geq 18$	139 (70.56)	146 (71.22)	285 (70.90)	
<b>Charlson Comorbidity Index, n (%)</b>				
0	148 (75.13)	155 (75.61)	303 (75.37)	0.27
1	36 (18.27)	29 (14.15)	65 (16.17)	
$\geq 2$	13 (6.60)	21 (10.24)	34 (8.46)	
<b>Plan type, n (%)</b>				
HMO	20 (10.15)	31 (15.12)	51 (12.61)	0.22
PPO	115 (58.38)	128 (62.44)	243 (60.45)	
CHHP	14 (7.11)	10 (4.88)	24 (5.97)	
HDHP	15 (7.61)	9 (4.39)	24 (5.97)	
Other	33 (16.75)	27 (13.17)	60 (14.93)	
<b>Year, n (%)</b>				
2010	43 (21.83)	66 (32.20)	109 (27.11)	0.38
2011	34 (17.26)	29 (14.15)	63 (15.67)	
2012	27 (13.71)	29 (14.15)	56 (13.93)	
2013	22 (11.17)	22 (10.73)	44 (10.95)	
2014	13 (6.60)	12 (5.85)	25 (6.22)	
2015	20 (10.15)	10 (4.88)	30 (7.46)	
2016	10 (5.80)	10 (4.88)	20 (4.98)	
2017	15 (7.61)	15 (7.32)	30 (7.46)	
2018	13 (6.6)	12 (5.85)	25 (6.22)	
<b>Region, n (%)</b>				
Northeast	42 (21.99)	31 (15.35)	73 (18.58)	0.36
North Central	54 (28.27)	58 (28.71)	112 (28.50)	
South	64 (33.51)	79 (39.11)	143 (36.39)	
West	31 (16.23)	34 (16.83)	65 (16.54)	



**FIGURE 1: Change in annualized bleeding events and joint pain after initiation of FIX therapy**

- 16,430 FIX claims were identified between 2010-2018 with 1,628 unique patients.
- Among them, 402 patients met the inclusion criteria and 197 were deemed to be prophylactic users.
- The median age in this group was 28 years.
- 24.6% had at least one comorbidity diagnosis (CCI).
- Prophylactic users had an average ABR of 0.28 (nine months after therapy initiation), where the mean number of hemorrhages, hemarthrosis, effusion, and contusion episodes were 0.08, <0.01, 0.04, and 0.16 respectively.
- On average these patients received 8.57 FIX doses during the entire follow-up year. (12.17 among prophylactic users)

**TABLE 2. Annualized bleed rates and joint pain among FIX users during the follow up period**

	Prophylactic group	On demand group
<b>All bleeds</b>	0.28	0.55
Hemorrhage	0.08	0.25
Hemarthrosis	<0.01	0.02
Effusion	0.04	0.02
Contusion	0.16	0.25
<b>Joint pain</b>	0.30	0.64

## DISCUSSION

- Among prophylactic FIX users, bleeding rates were well controlled. FIX replacement clearly has a protective effect in managing bleeding rates in hemophilia B patients.
- However, a small sample size and inability to follow-up patients for a longer period are limitations of this study.
- Future studies should examine long-term effect of FIX therapy on ABR in hemophilia B patients.