

## Efficacy and Safety of Recombinant Factor IX Fusion Protein (rIX-FP) in Previously Untreated Patients with Hemophilia B

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### HIGHLIGHTS



12 PUPs with a median age of 0 years were treated with rIX-FP for a median of 50 exposure days (EDs)



rIX-FP was safe and effective for both on-demand and prophylaxis use



11/12 patients did not develop FIX inhibitors

### INTRODUCTION

rIX-FP is effective and well tolerated in previously treated patients (PTPs) with hemophilia B; however, previously untreated patients (PUPs) are often young pediatric patients who are more vulnerable to severe bleeding episodes due to increased activity

This post hoc analysis reports **rIX-FP efficacy and safety in PUPs**

### STUDY CHARACTERISTICS



Objective  
**Evaluate rIX-FP safety and efficacy in 12 PUPs with hemophilia B**



Study design  
**Phase 3b, prospective, multicenter study**



Patient population  
**PUPs aged 0 to 11 years with FIX levels ≤2%**

#### Start of study

On-demand  
n=6

Prophylaxis  
(7 days)  
n=5

Prophylaxis  
(10 days)  
n=1

#### End of study

Prophylaxis  
(7 days)\*  
n=11

Prophylaxis  
(10 days)  
n=1

#### Surgery substudy

Prophylaxis  
(7 days)  
n=1

\*All patients ended the study on the prophylactic regimen, but an 11-year-old patient who developed an inhibitor in the 7-day cohort was switched to an off-protocol intensified regimen, and the dose was increased from 50 to 100 IU/kg.

### RESULTS – EFFICACY

Table 1. Summary of total annualized bleeding rates for patients on prophylactic treatment for >6 months (>183 days)

		ABRs based on treated bleeding episodes	ABRs based on all bleeding episodes
7-day regimen (n=9)	Mean (SD)	0.6 (0.7)	1.2 (1.1)
	Median	0.0	1.2
	Min, max	0.0, 1.5	0.0, 3.1
10-day regimen (n=1)	Mean (SD)	1.0 (NC)	1.0 (NC)
	Median	1.0	1.0
	Min, max	1.0, 1.0	1.0, 1.0
Total (n=10)	Mean (SD)	0.6 (0.7)	1.2 (1.0)
	Median	0.5	1.1
	Min, max	0.0, 1.5	0.0, 3.1



~94% of spontaneous bleeding events were controlled with 1 or 2 rIX-FP infusions



9 PUPs had an AsBR of 0



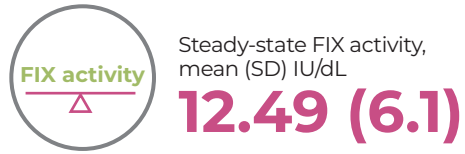
ABR range of 0 to 3.9  
3.9 ABR occurred in a patient who developed FIX inhibitors and was placed on intensified FIX treatment



No major bleeding episodes were reported

# RESULTS – PHARMACOKINETICS AND SAFETY

## Steady-state FIX activity on 7-day prophylaxis regimen (n=7)\*



## Single rIX-FP dose (50 IU/kg) PK parameters (mean, n=8)†



11/12 patients experienced 135 treatment-emergent adverse events (TEAEs), most of which were mild and most resolved\*



Most common TEAE was infection or infestation (57/135)



2 participants had 5 TEAEs related to rIX-FP

- An 11-year-old patient developed an inhibitor against FIX after 8 EDs
  - Patient was switched from a 7-day prophylaxis regimen to an intensified treatment with rIX-FP and was ultimately withdrawn following 2 mild hypersensitivity reactions
  - Genetic mutation data showed large deletions in exons 7 and 8 of the F9 gene
- Another patient had a mild rash that resolved during study

**Table 2. Overview of TEAEs occurring during prophylaxis**

Category	Prophylaxis regimen (n=12)	
	PUPs, n (%)	Events
<b>Any TEAEs</b>	11 (91.7)	109
Intensity		
Mild	11 (91.7)	86
Moderate	5 (41.7)	21
Severe	2 (16.7)	2
TEAE related to rIX-FP	2 (16.7)†	2
Any SAEs	3 (25)‡	3

\*This excludes adverse events that occurred during the surgery period.

†The 2 related TEAEs were recorded as hypersensitivity in the 11-year-old patient who later developed an inhibitor and development of a rash on lower legs and forearms in a second patient. The rash was considered mild in intensity and resolved within 4 days, and prophylaxis continued in this patient.

‡Includes the patient who developed an inhibitor (who also had large gene deletions) and discontinued the study.

### Abbreviations

ABR: annualized bleeding rate; AsBR: annualized spontaneous bleeding rate;  $C_{max}$ : maximum concentration; ED: exposure day; FIX: factor IX; IR: incremental recovery; IU: international unit; NC: not calculable; PK: pharmacokinetic; PTP: previously treated patient; PUP: previously untreated patient; rIX-FP: recombinant factor IX fusion protein; SAE: serious adverse event; TEAE: treatment-emergent adverse event.

### Reference

Lemons R, Wang M, Curtin J, et al. *TH Open*. 2024;8(1):e155-e163.



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