

Real-world Bleeding Rates on Emicizumab using Digital Treatment Diary data

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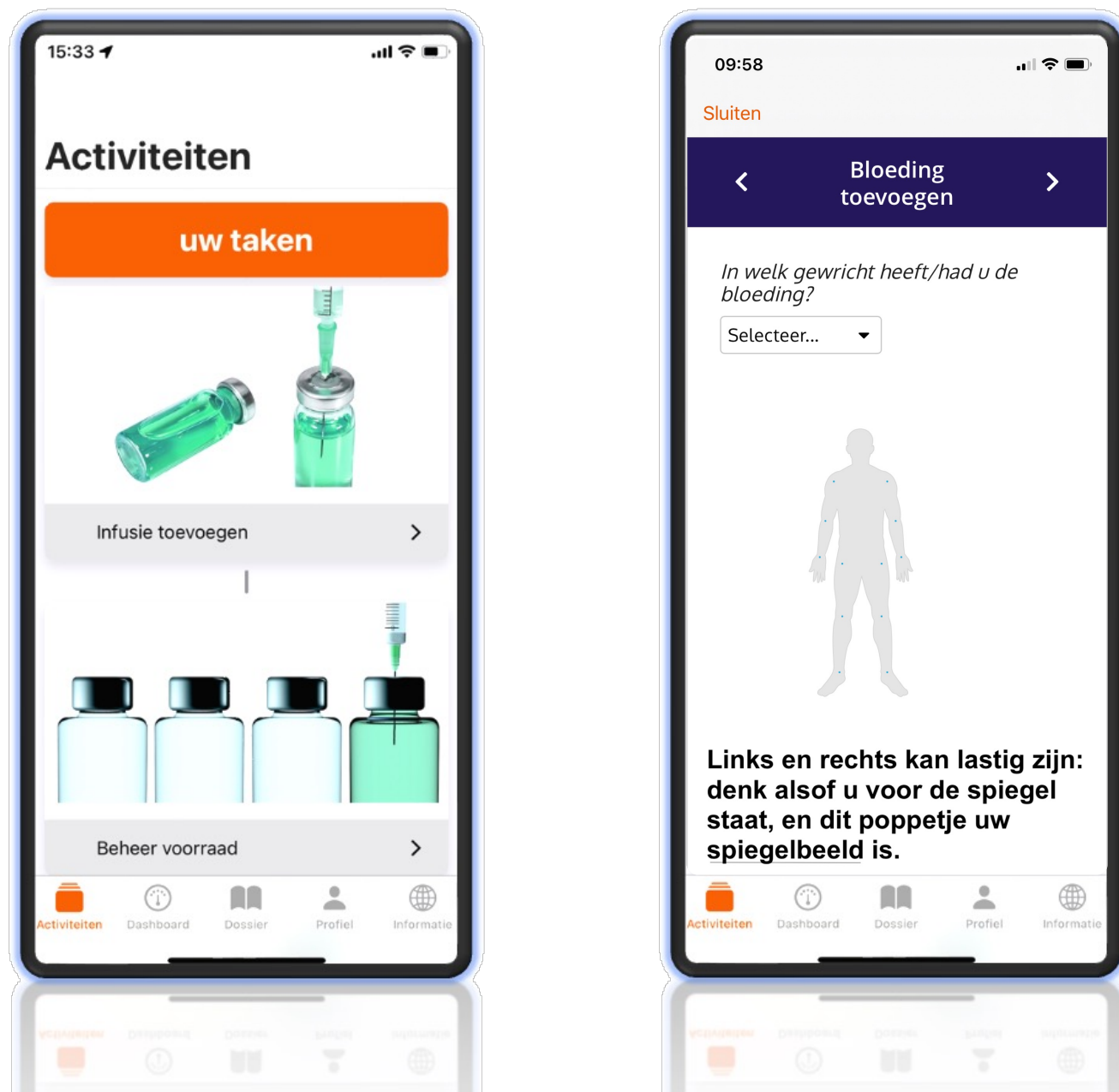
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INTRODUCTION

- Dutch people with a congenital bleeding disorder use a **mobile-based treatment diary app**
 - To log bleeds and infusions
 - The diary is linked to the Dutch **Hemophilia Patient Registry HemoNED**
- Many people with severe hemophilia A have switched to emicizumab for prophylaxis
- Yet, the efficacy of emicizumab has not been evaluated in a real-world setting using **nationwide treatment diary data**



RESULTS

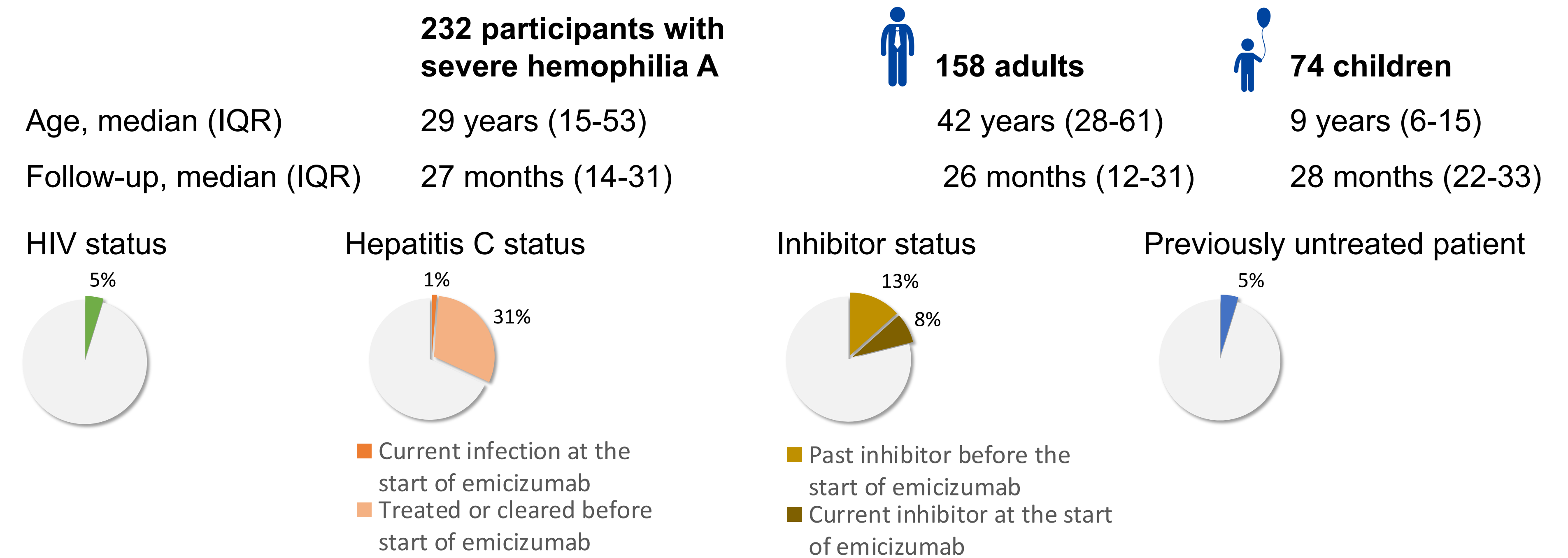


Table 1. Bleeding rates on emicizumab

	All participants (n=232)	All adults (n=158)	18-30yr (n=50)	31-60yr (n=68)	>60yr (n=40)	All children (n=74)	0-10yr (n=44)	11-17yr (n=30)
ABR, median (IQR)								
Bleeds treated ≥ 1 times	0.6 (0.0-1.4)	0.8 (0.0-1.5)	0.7 (0.0-1.4)	0.8 (0.0-1.5)	0.8 (0.0-2.6)	0.6 (0.0-1.2)	0.4 (0.0-1.1)	0.9 (0.3-2.0)
Bleeds treated ≥ 2 times	0.3 (0.0-0.8)	0.4 (0.0-0.9)	0.4 (0.0-0.9)	0.4 (0.0-0.8)	0.0 (0.2-1.1)	0.1 (0.0-0.5)	0.0 (0.0-0.4)	0.5 (0.0-1.3)
AJBR, median (IQR)								
Joint bleeds treated ≥ 1 times	0.0 (0.0-0.8)	0.0 (0.0-0.8)	0.0 (0.0-0.7)	0.2 (0.0-0.9)	0.0 (0.0-1.1)	0.0 (0.0-0.5)	0.0 (0.0-0.4)	0.4 (0.0-1.2)
Joint bleeds treated ≥ 2 times	0.0 (0.0-0.4)	0.0 (0.0-0.4)	0.0 (0.0-0.4)	0.0 (0.0-0.3)	0.0 (0.0-0.4)	0.0 (0.0-0.4)	0.0 (0.0-0.2)	0.4 (0.0-0.5)

All bleeds treated with either at least 1 FVIII concentrate administration or at least 2 FVIII concentrate administrations were included. ABR, annual bleeding rate; AJBR, annual joint bleeding rate; IQR, interquartile range

Fig 1. Proportion of participants with zero bleeds treated with FVIII concentrate ≥ 2 times

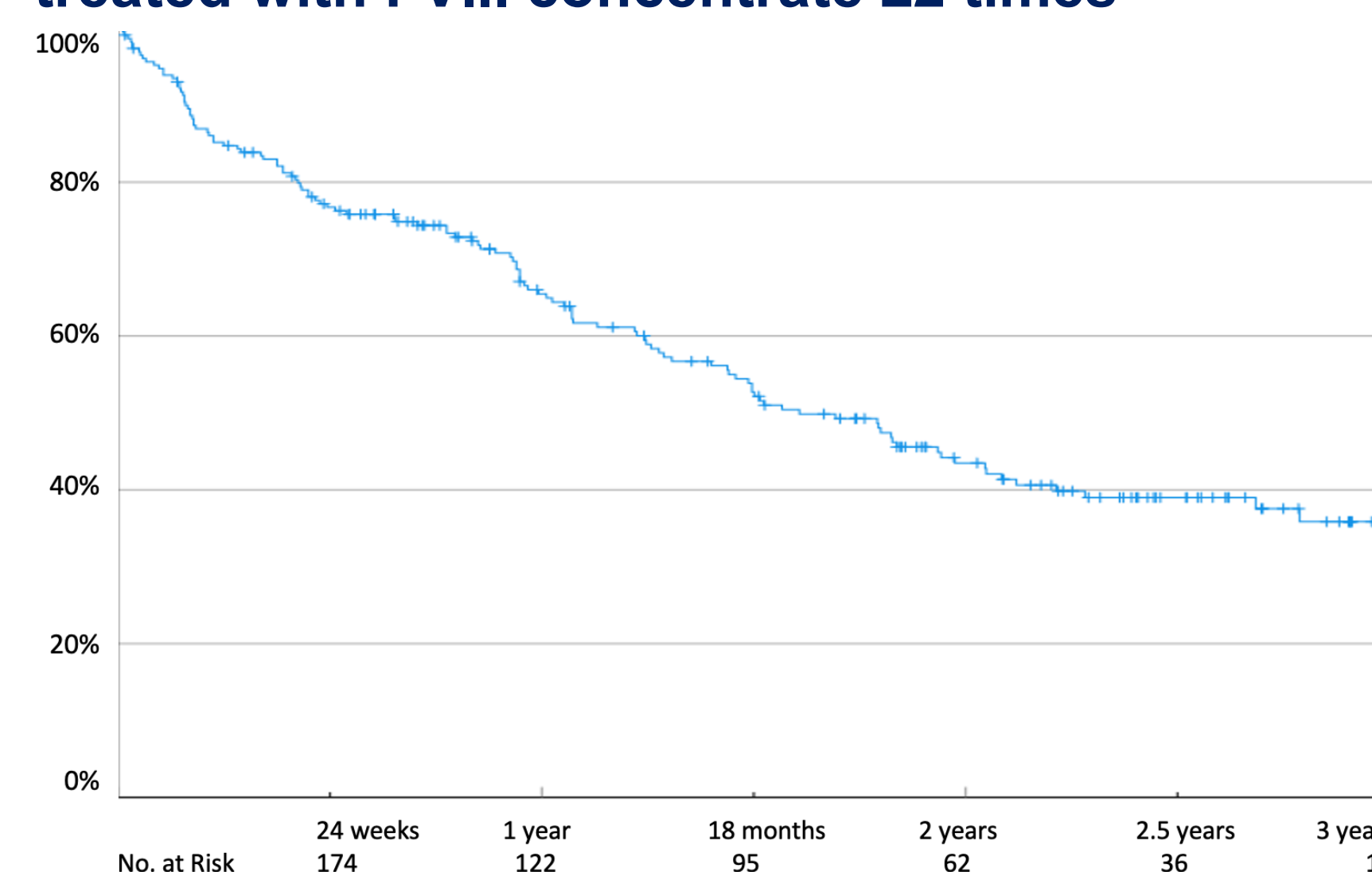


Fig 2. Proportion of participants with zero joint bleeds treated with FVIII concentrate ≥ 2 times

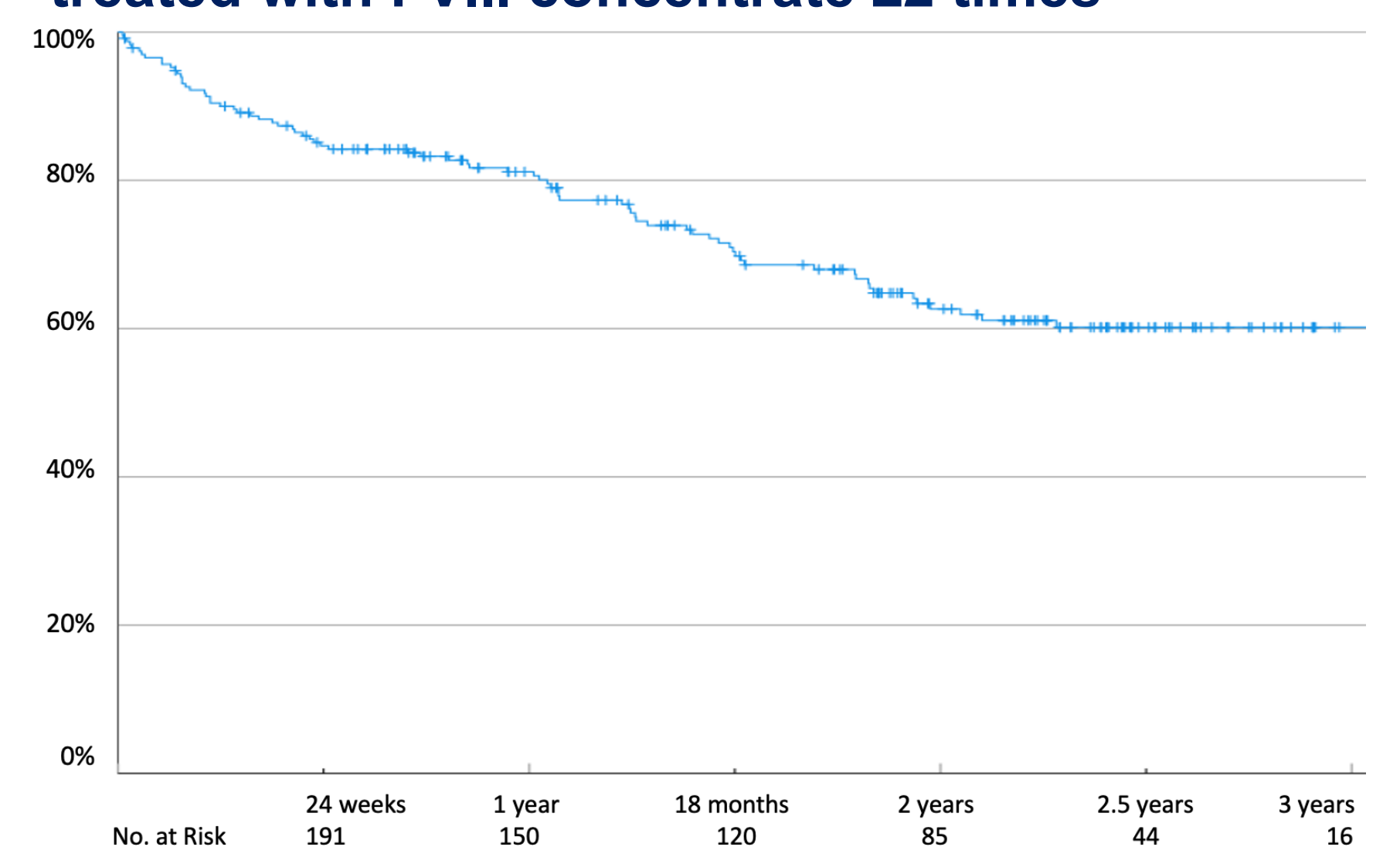


Table 2. Data source of reported bleeds

	Treated bleeds, n (%)	Bleeds treated with FVIII ≥ 2 times, n (%)	Treated joint bleeds, n (%)	Joint bleeds treated with FVIII ≥ 2 times, n (%)
Number of bleeds, n	456	235	240	125
Bleed is reported in:				
Both treatment diary & EHR, n (%)	200 (44%)	116 (49%)	101 (42%)	59 (47%)
Treatment diary only, n (%)	108 (24%)	27 (12%)	66 (28%)	49 (39%)
EHR only, n (%)	148 (32%)	92 (39%)	73 (30%)	17 (14%)
Proportion treatment diary-reported bleeds / all bleeds	308/456 (68%)	143/235 (61%)	167/240 (70%)	108/125 (86%)

EHR, electronic health record

AIMS

- Assess **bleeding rates** on emicizumab among people with severe hemophilia A
- Assess the reliability of the **secondary use** of treatment diary data for clinical research

METHODS

- Eligible:**
 - Dutch people with severe hemophilia A of all ages
 - using emicizumab
 - who have used the digital treatment diary
- Data sources:** digital treatment diaries verified and complemented with electronic health records (EHR)
 - Included bleeds: treated with ≥ 1 FVIII concentrate administrations (document in diaries and/or EHRs)
 - Subgroup: bleeds treated with ≥ 2 FVIII concentrate administrations
- Outcomes:**
 - Annual bleeding rates (ABR)
 - Annual joint bleeding rates (AJBR)
 - Proportions of people with zero treated bleed

CONCLUSIONS

Real-world bleeding rates in Dutch people with severe hemophilia A using emicizumab, determined using digital treatment diary and electronic health record data, are comparable to other real-world studies.

Most of the **joint bleeds** were documented in the digital treatment diary.

This study showcases the **value and limitations of the treatment diary** to evaluate treatment efficacy.

REFERENCES

Visit the VastePrik treatment diary website

Treatment diary video 1: Register prophylaxis

Treatment diary video 2: Register a bleed

ACKNOWLEDGEMENT

HemoNED is the **Dutch Hemophilia Registry** for people with hemophilia & associated disorders in the Netherlands.

The **HemoNED Foundation** is an initiative of the Dutch Hemophilia Treaters Society & Netherlands Hemophilia Patient Society

The HemoNED Foundation received grants & research support from Biomarin, CSL Behring, Pfizer, Roche & Sobi.

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