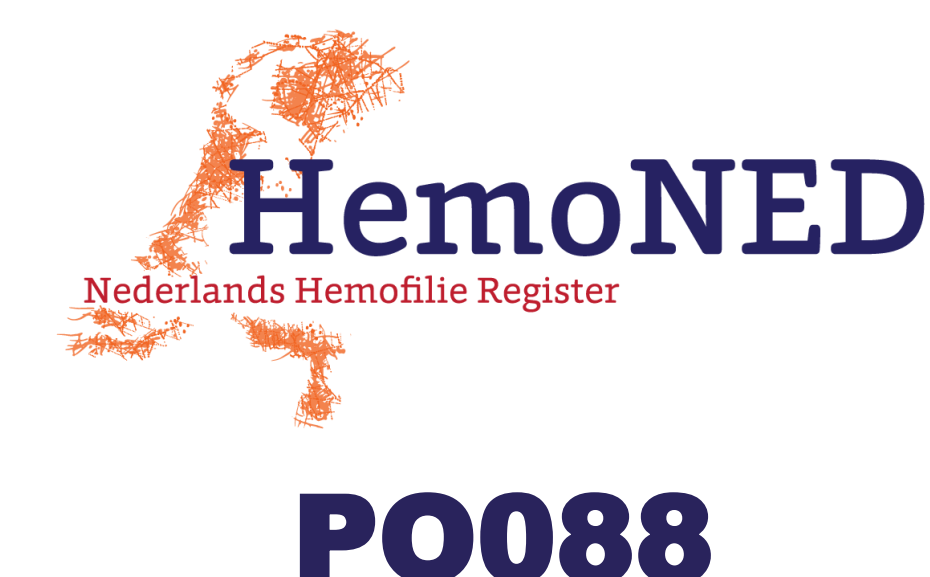


# Real-world bleeding rates of people with severe hemophilia A on emicizumab treatment in the Netherlands with and without a history of an inhibitor

Elisabeth M. Taal<sup>1</sup>, Caroline M. van Veen<sup>1</sup>, Martijn R. Brands<sup>2,3</sup>, Marlène Beijlevelt-van der Zande<sup>3</sup>, Mariette H. Driessens<sup>4</sup>, Corien L. Eckhardt<sup>5</sup>, Paul L. den Exter<sup>6</sup>, Marieke J. Kruijff<sup>7</sup>, Britta A. Laros<sup>8,9</sup>, Stephan Meijer<sup>4</sup>, Marjet Stein-Wit<sup>10</sup>, Karina Meijer<sup>11</sup>, Samantha C. Gouw<sup>1,3</sup>

<sup>1</sup>HemoNed Foundation, Leiden, <sup>2</sup>Erasmus MC, Erasmus University Medical Center Rotterdam, Rotterdam, <sup>3</sup>Department of Pediatric Hematology, Amsterdam UMC location University of Amsterdam, Amsterdam, <sup>4</sup>Netherlands Hemophilia Patient Society (NVHP), Nijkerk, <sup>5</sup>Center for Benign Haematology, Thrombosis and Haemostasis, Van Crevelkliniek, University Medical Center Utrecht, Utrecht University, Utrecht, <sup>6</sup>Department of Medicine, Thrombosis and Hemostasis, Leids Universitair Medisch Centrum, Leiden, <sup>7</sup>Department of Hematology, Erasmus University Medical Center Rotterdam, Rotterdam, <sup>8</sup>Department of Hematology, Radboud University Medical Center, <sup>9</sup>Hemophilia Treatment Center Nijmegen-Eindhoven-Maastricht, Nijmegen, <sup>10</sup>Department of Pediatrics, University Medical Center Groningen, <sup>11</sup>Department of Hematology, University Medical Center Groningen, University of Groningen, Groningen, The Netherlands



## INTRODUCTION

Emicizumab has been shown to be effective both in patients with and without inhibitors.

People with severe hemophilia A with a history of inhibitors may have more arthropathy and therefore bleed more frequently than those without an inhibitor history.

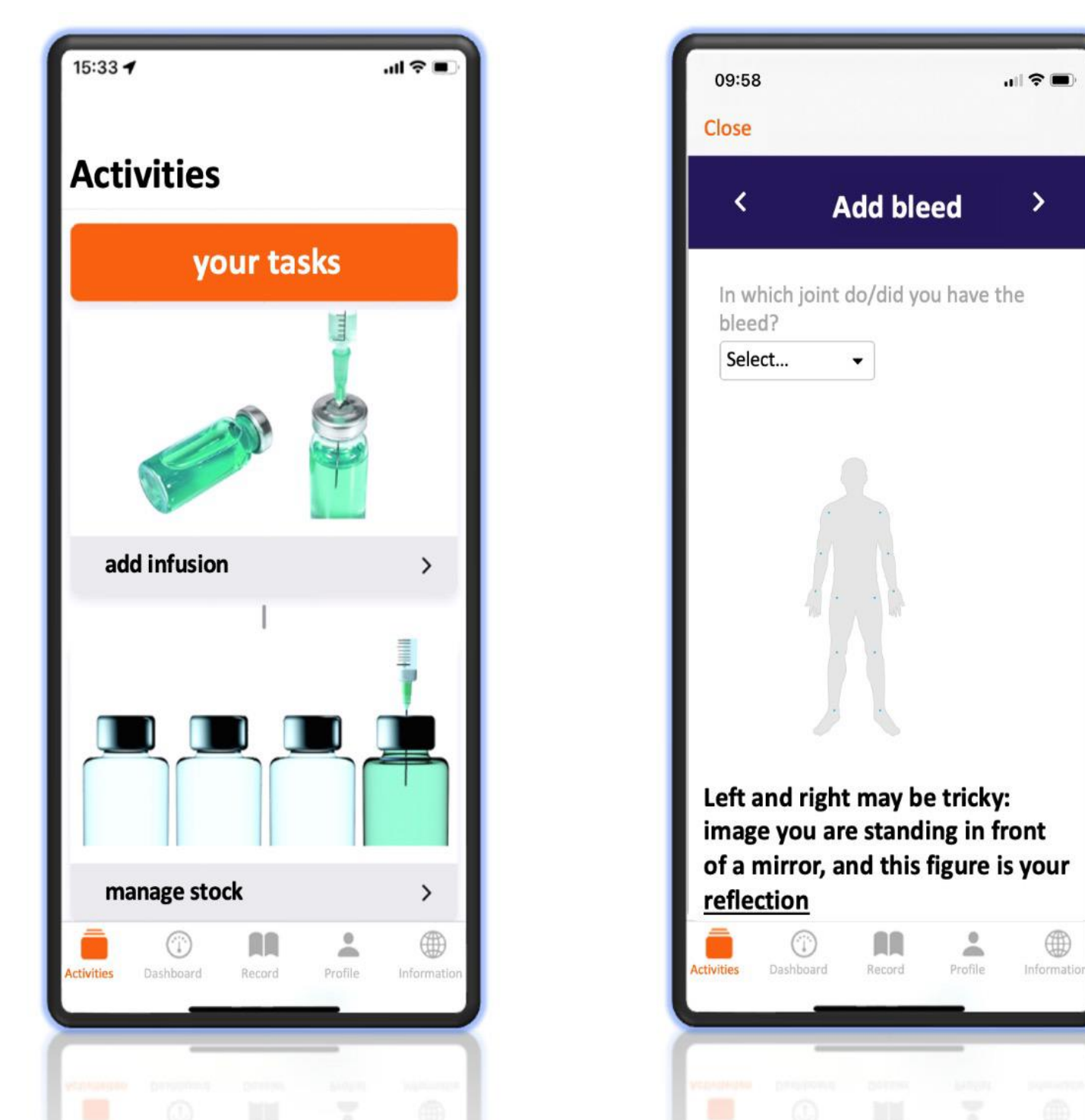
## AIM

This study explores whether emicizumab treatment is equally effective in people with severe hemophilia A on emicizumab prophylaxis, with and without a history of inhibitors by assessing real-world bleeding rates.

## METHOD

- In the Netherlands, people with hemophilia A on prophylactic treatment report their infusions and bleeds in a digital treatment diary
- Data from **inhibitor negative people with severe hemophilia A**, both with and without a history of an inhibitor in the past, on emicizumab treatment who use this diary were collected from 2018 to 2023
- Treated bleeds** documented in the digital treatment diary **were validated and supplemented** with data from Electronic Health Records from the Hemophilia Treatment Centers
- Mean annualized (joint) bleeding rates (A(J)BR)** for treated bleeds from the start of emicizumab treatment were calculated using a negative binomial regression model
- The proportions of participants with zero treated (joint) bleeds at 24 weeks and 1 year were assessed by Kaplan-Meier survival analysis

Figure 1. Digital Treatment Diary



## RESULTS

A total of 214 male patients with severe hemophilia A were included, of whom 177 never had an inhibitor and 37 had a history of an inhibitor in the past.

Real world bleeding rates, ABR, AJBR for all and major bleeds were similar for both groups.

Zero treated bleed rates were similar at 24 weeks and 1 year.

Table 2. Number of bleeds, model-based bleeding rates and % zero treated bleeds

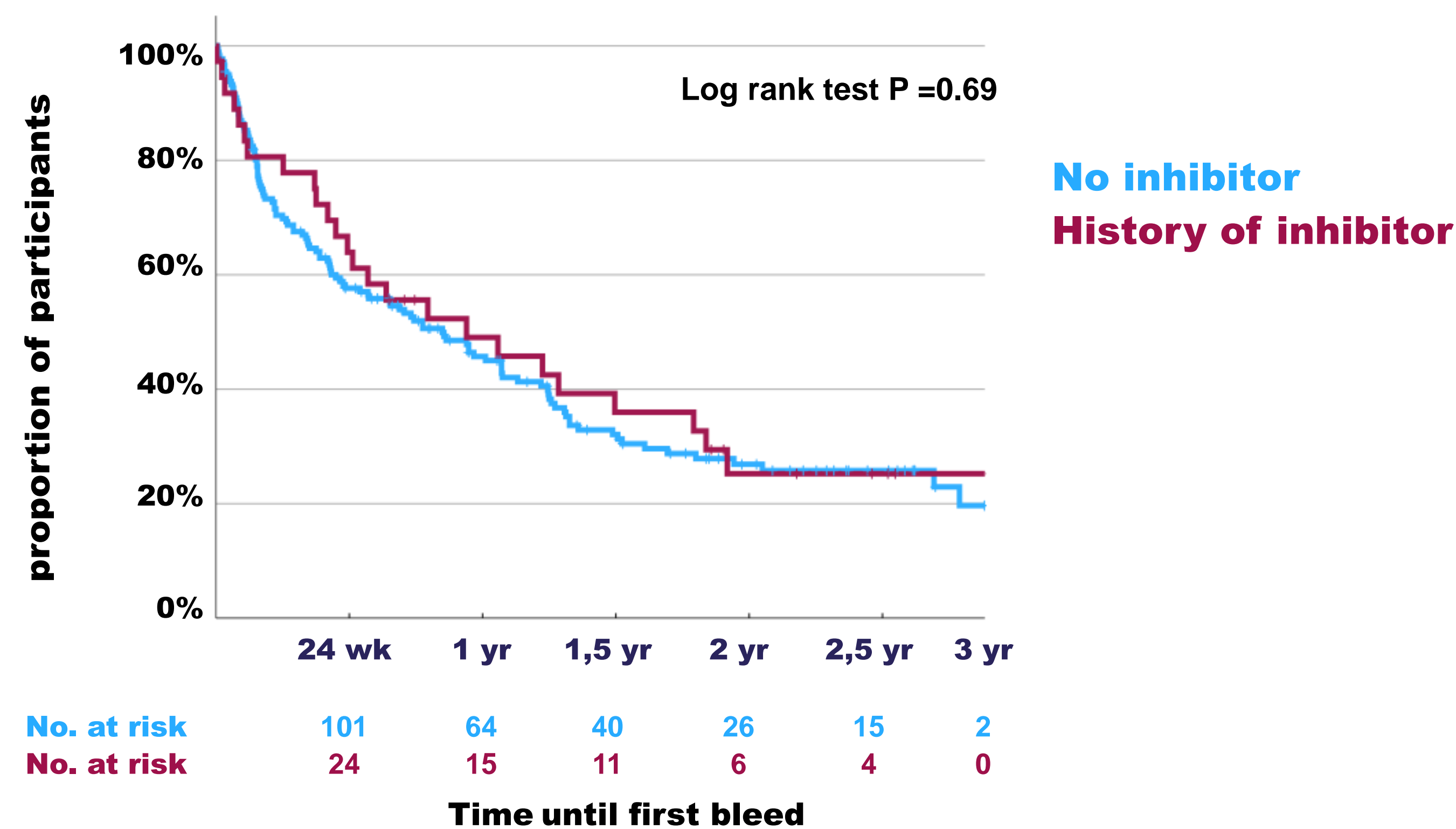
	Never had an inhibitor N=177	History of an inhibitor in the past N=37
N Bleeds*	367	76
ABR, mean (95% CI)	1.6 (1.3-1.9)	1.6 (1.2-2.1)
N Joint bleeds*	199	35
AJBR, mean (95% CI)	0.9 (0.7-1.1)	0.7 (0.5-1.0)
N Major bleeds**	181	43
ABR, mean (95% CI)	0.8 (0.7-1.0)	0.9 (0.6-1.2)
N Major joint bleeds**	102	18
AJBR, mean (95% CI)	0.4 (0.3-0.6)	0.4 (0.2-0.6)
% zero treated bleeds 24 weeks	59%	67%
% zero treated bleeds 1 year	46%	49%

ABR Annual Bleeding Rate, AJBR Annual Joint Bleeding Rate, CI Confidence Interval  
\*Bleeds were defined as a bleeding event for which coagulation factor concentrate was administered  
\*\*Major bleeds are defined as bleeds treated with factor concentrate at least 2 days

Table 1. Characteristics of patients

	Never had an inhibitor N=177	History of an inhibitor in the past N=37
Age, median (IQR)	29 years (13-53)	26 years (16-36)
Follow up time, duration of emicizumab use median (IQR)	26 months (13-30)	27 months (23-31)
HIV positive	9	1
Hepatitis C infection		
Never	119	27
Treated or cleared	56	10
Current	2	0

Figure 1. Kaplan-Meier survival curve for proportion of participants with zero treated bleeds



## CONCLUSIONS

Among Dutch people with severe hemophilia A on emicizumab prophylaxis, from our real-world data, emicizumab appeared to be equally effective in people who had an inhibitor in the past compared to people who never had an inhibitor.

## DISCUSSION

We could not evaluate the effect of arthropathy on the bleeding rates on emicizumab because of lack of data on arthropathy.

We continue to strive for optimal data quality by checking and completing bleeding data reported by patients with data from Electronic Health Records.

This is important in order to be able to draw valid conclusions on real-world data studies.

## ACKNOWLEDGEMENT

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

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

The HemoNED Foundation received grants and research support from:

CSL Behring, Novo Nordisk, Octopharma, Pfizer, Roche, Sobi and Stichting Haemophilia.

## REFERENCES

Real-world bleeding rates on emicizumab using digital treatment diary data, preliminary results

Poster presentation EAHAD 2024, Martijn Brands et al.

## CONTACT INFORMATION

[www.hemoned.nl/en/](http://www.hemoned.nl/en/)

[info@hemoned.nl](mailto:info@hemoned.nl)

Ms. Caroline van Veen, projectcoordinator  
Ms. Liesbeth Taal, datamanager

