


# Trends in Prescribing Practices for Management of Hemophilia



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# Introduction

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- ❑ Over the past two decades, treatment options for hemophilia have evolved significantly
- ❑ The specific dosing regimens prescribed by the specialists who treat and care for people with hemophilia have not been widely studied
- ❑ The study objective is to describe the trends observed in clinician prescribing practices for management of hemophilia A (HA) and B (HB) in the United States via three surveys taken in 1999, 2015 and 2021

# Methods

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- ❑ Three surveys
  - Members of the Hemostasis & Thrombosis Research Society (HTRS) were invited to take in-person surveys at its annual symposia in 1999 and 2015
  - An online survey of HTRS members was conducted in 2021
- ❑ Survey participants included physicians, physician assistants, and nurse practitioners who manage the care of hemophilia patients at Hemophilia Treatment Centers (HTCs) in the US
- ❑ Surveys collected information regarding
  - Characteristics of the clinician's practices
  - Clotting factor products prescribed and dosages used for routine bleeds or major life-threatening bleeding, total joint replacement, and port placement
  - Frequency of recommendation for prophylaxis and inhibitor treatment for associated factor and non-factor products
  - Gene therapy (only in 2021 survey)

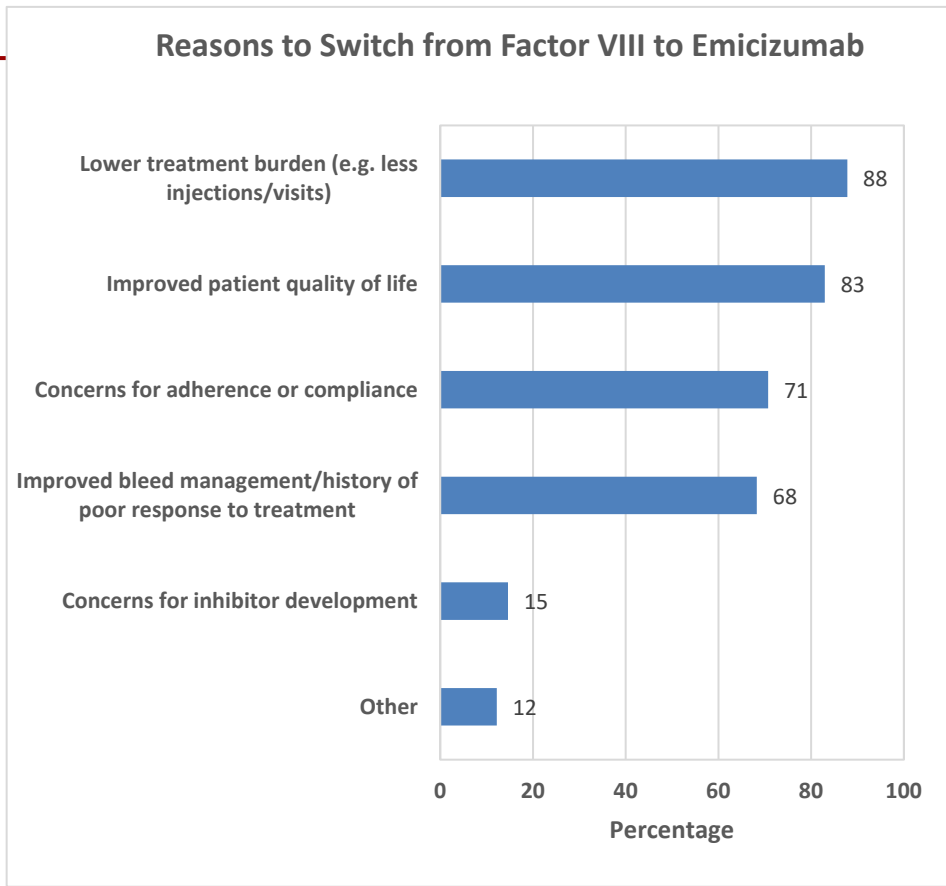
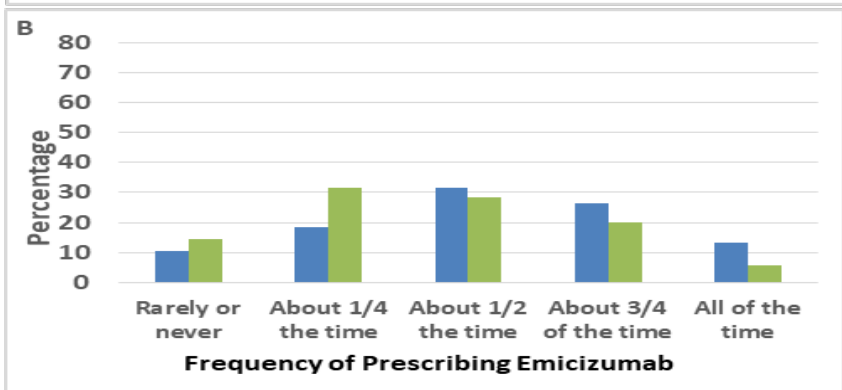
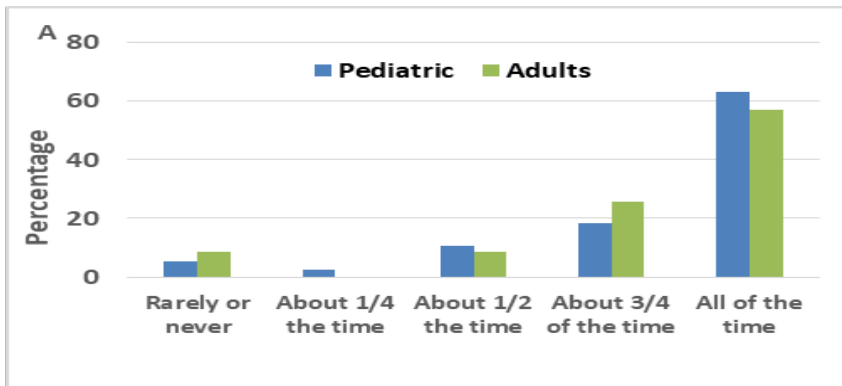
# Results: Dose for routine & major life-threatening bleeding



Variable	Factor VIII				Factor IX			
	1999	2015	2021 SHL	2021 EHL†	1999	2015	2021 SHL	2021 EHL†
<b>Routine bleed*</b>								
10 – 15 (IU/KG)	2.50	1.89	0.00	0.00	0.00	0.00	0.00	0.00
16 – 20 (IU/KG)	17.50	3.77	2.44	0.00	10.00	0.00	0.00	0.00
21 – 25 (IU/KG)	50.00	22.64	19.51	17.07	10.00	5.66	0.00	0.00
26 – 30 (IU/KG)	17.50	32.08	29.27	24.39	10.00	5.66	2.44	0.00
31 – 35 (IU/KG)	5.00	9.43	9.76	7.32	15.00	3.77	0.00	7.32
36 – 40 (IU/KG)	7.50	18.87	9.76	17.07	32.50	30.19	9.76	2.44
>40 (IU/KG)	0.00	11.32	29.26	31.71	22.50	50.95	87.80	85.36
Other	0.00	0.00	0.00	2.44	0.00	3.77	0.00	4.88
<b>Major life-threatening bleed*</b>								
36 – 40 (IU/KG)	5.14	0.00	0.00	0.00	0.00	0.00	0.00	0.00
41 – 45 (IU/KG)	2.56	1.92	0.00	0.00	2.50	0.00	0.00	0.00
46 – 50 (IU/KG)	46.15	15.38	29.27	19.51	5.00	0.00	2.44	2.44
51 – 55 (IU/KG)	41.03	59.62	51.22	51.22	7.50	0.00	0.00	2.44
56 – 60 (IU/KG)	2.56	5.77	12.20	12.20	15.00	5.79	4.88	17.07
> 60 (IU/KG)	2.56	17.31	7.31	7.31	67.50	86.54	90.24	65.85
Other	0.00	0.00	0.00	9.76	2.50	7.69	2.44	12.20

Note: Data was presented as the proportion of respondents who reported factor dose ranges that they prescribed for treating routine bleeds or major life-threatening bleeds. Abbreviations: SHL, standard half-life; EHL, extended half-life. \*Clotting factor units were presented in units/kg body weight. †Only 2021 survey asked about dosages for prescribing extended half-life product.

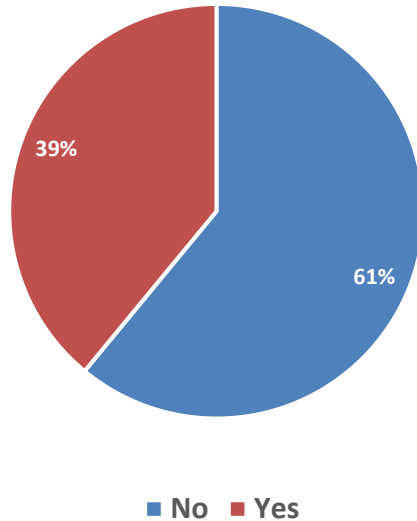
# Results: Prescribing practices for emicizumab in 2021 survey



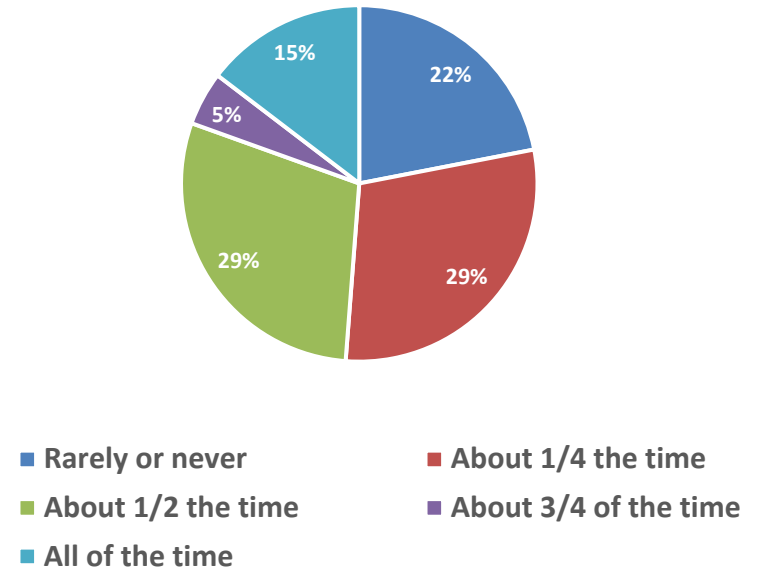
Figures A and B show the frequency of the respondents reported prescribing Efficizumab. Figure A displays Efficizumab prescribing patterns for patients with inhibitors; Figure B illustrates patterns for patients without inhibitors.

# Results: Gene therapy in 2021 survey

Clinicians with patients enrolled in clinical trial for gene therapy\*



Frequency of clinicians recommending gene therapy



\* About 28% of clinicians reported that they have patients who have completed gene therapy.

# Conclusions

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- ❑ These data indicate changes in prescribing practices among hemophilia specialists in the US over the past two decades
- ❑ Prescribing of high dose of factor (>40 units/kg) has increased, while Immune Tolerance Induction (ITI) prescribing practices have remained similar over time
- ❑ In 2021 survey, most clinicians frequently prescribed emicizumab for patients with HA inhibitors, but less frequently for those without inhibitors
- ❑ In 2021 survey, the expected uptake of gene therapy diverges widely among clinicians

# Acknowledgments

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*Emicizumab is subject to additional safety monitoring requirements in many countries. Healthcare professionals are asked to report any suspected adverse reactions to the regulatory authorities in your country according to your national requirements.*



# Conflict of Interest Disclosure

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