



# The impact of improved hemophilia care on hospitalization rate in children with hemophilia: A single-center experience

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#### INTRODUCTION

Hemophilia is characterized by a considerable burden - both for the patient and the healthcare system. Over the last decade, treatment optimization has been achieved with the use of factor concentrates with extended pharmacokinetic properties, as well as non-factor molecules that provide prophylaxis even in the presence of an inhibitor.

Although bleeding rates have indisputably, been reduced, there is limited data regarding hospitalization rates of hemophilia patients in this new landscape of hemophilia treatment - especially, outside the clinical study setting.

### AIM

Aim of the present study was to evaluate disease-related hospitalizations in children with hemophilia before and after availability of these improved prophylactic options.

## **METHOD**

Medical records of pediatric patients with hemophilia under prophylaxis were retrospectively reviewed.

- demographics
- disease type and severity inhibitor history
- type of prophylaxis and Hospitalizations

during the periods 2012-2018 (1st study period) and 2019-2025 (2nd study period) were recorded.

#### **RESULTS**

During the 1st study period, 40 hospitalizations in 13 male patients (12/1 with hemophilia A/B, respectively) were reported. Out of 13, 9 (69.2%) patients had multiple disease-related hospital admissions during the study period. With regards to patients with documented inhibitor presence (5/13, 38.5%), 19 (47.5%) hospitalizations were recorded.

In both the inhibitor and non-inhibitor patients the main reason for hospitalization was

- head injury (7, 17.5%) central venous catheter re muscle bleeds (5, 12.5%) related complications (6, 15%) and

In terms of **treatment**, 7 (69.2%) patients received standard half-life products and 1 (7.7%) an extended half-life product. With regards to inhibitor patients, 2 out of 5 (40%) were low titer and, thus, on low dose immune tolerance treatment, while 3 (60%) were on short term periods of off label prophylaxis with bypassing agents.

During the 2nd study period, 16 hospitalizations in 13 male patients were recorded (13/0 with hemophilia A/B respectively). Only 2 (15.4%) patients had more than one hospital admissions. Two (15.4%) patients were diagnosed with inhibitor relapse during

Main reasons for admission in the whole of the study group were

- central venous catheter complications (4, 25%) and
- hematuria (4, 25%)

In terms of treatment, 8 (61.5%) patients received standard half-life products and 5 (38.5%) extended half-life products. (Table 1.)

	Study period	Study period	p-value
	2012-2018	2019-2025	
Disease related hospitalizations, n	40	16	
Rate of hospitalization per year	5.71	2.29	Poisson regression
			Wald test
			<0.01
Rate of hospitalization per patient	3.08	1.23	Poisson regression
			Wald test
			<0.01
Mean duration of hospitalization in days (range)	5.2 (1-22)	4.8 (2-11)	t-test
			0.82
Median duration of hospitalization in days (IQR)	3.0 [2.0;6.0]	4.0 [2.8;6.2]	Wilcoxon rank sum te
			0.52
Hospitalized patients, n	13	13	Fisher's exact test
			0.04
1 hospital admission	4	11	
2 hospital admissions	3	1	
3 hospital admissions	3	1	
>3 hospital admissions	3	0	
Hemophilia, n (%)*			Fisher's exact test
Α	12 (92.3)	13 (100)	0.99
В	1 (7.7)	0 (0)	
Disease severity, n (%)*		- (-)	Fisher's Exact Test
Severe	12 (92.3)	11 (84.6)	0.99
Moderate	1(7.7)	2 (15.4)	
Mild	0 (0)	0 (0)	
Reasons of hospitalization, n (%)			Fisher's Exact Test
Brain hemorrhage	1 (2.5)	0 (0)	0.99
Central venous catheter related			0.99
Loint Bleed	6 (15)	4 (25)	0.45
Joint Bleed Muscle hematoma	12 (30)	2 (12.5)	0.31
	5 (12.5)	1 (6.25)	0.66
Head injury Hematuria	7 (17.5)	2 (12.5)	0.99
Scrotum Hematoma	3 (7.5)	4 (25) 0 (0)	0.21
Enormous soft tissue hematoma	1 (2.5)		0.99
Enormous soft tissue nematoma Epistaxis	2 (5)	0 (0)	0.99
	1 (2.5)	0 (0)	0.99
Gastrointestinal bleeding Immunomodulatory treatment due to inhibitor	1 (2.5)	0 (0)	0.99
	1 (2.5)	0 (0)	0.99
presence Post-surgery intraabdominal bleeding	0 (0)	0 (0) 2 (12.5)	0.99
			0.09
Frenulum Bleeding Factor concentrates, n (%)*	0 (0)	1 (6.25)	0.29 Fisher's exact test
Factor concentrates, n (%)* Standard half life	0(60.3)	0 (61 5)	Fisher's exact test 0.05
	9(69.2)	8 (61.5)	0.05
Extended half life	1 (7.7)	5 (38.5)	
Bypassing agents	3 (23.1)	0 (0)	
Presence of Inhibitor, n (%)*	5 (38.5)	2 (15.4)	Fisher's exact test
			0.41

Percentages marked with \* refer to total number of hospitalized patients (n=13)
Table 1. Patient characteristics and hospitalizations

## **CONCLUSIONS**

- Hospitalization rate decreased by 60% in children with hemophilia followed at a single institution during the past years, as a result of newer and better treatments
- Of particular interest the documented hospitalization independency in inhibitor patients, as well as the limited recording of repeated hospitalizations in the patients on conventional prophylaxis

CONTACT INFORMATION

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