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BACKGROUND & OBJECTIVES

- Factor VIII (FVIII) prophylaxis treatment is a standard treatment for hemophilia A (HA) patients. Many protocols which were different in dosage and administration have been accepted in developed countries^{1,2}. However, no guideline prophylaxis regimens exist in Thailand.
- To compare the efficacy of 2 different regimens: 30-35 U/kg/dose once weekly (once-weekly regimen) and 15-20 U/kg/dose twice weekly (twice-weekly regimen) on treatment outcomes.

METHODS

- A prospective cohort study was conducted in moderate/severe HA patients who were followed at King Chulalongkorn Memorial hospital between March 15, 2015 and January 15, 2016.
- All the patients were treated with once-weekly regimen for 5 months, wash-out period for 4 days and continued with twice-weekly regimen for another 5 months.
- Evaluations included the number of bleeding episodes, hospital admission days, school days lost and pdFVIII used. Level of factor VIII and factor VIII inhibitor, hemophilia joint health score (HJHS), quality of life (QOL) score using EQ-5D-5L³ and appreciation score were also monitored.

Table 1: Basic characteristics of the hemophilia A patients

Characteristics	Data
Age (yr): Mean ± SD	9.6 ± 7.0
- Median (Min-Max)	7.0 (0.5-19.0)
Severity: n(%)	8 (50%)
- Moderate	8 (50%)
- Severe	
Target Joint: n (%)	
- Had Target Joint	8 (50%)
- No Target Joint	8 (50%)
Weight (Kg): mean ± SD	32.2 ± 23.0
- Median (Min-Max)	22.8 (9.3-93.0)

Fig 1: Number of total breakthrough bleeding episodes in 2 different prophylaxis regimen

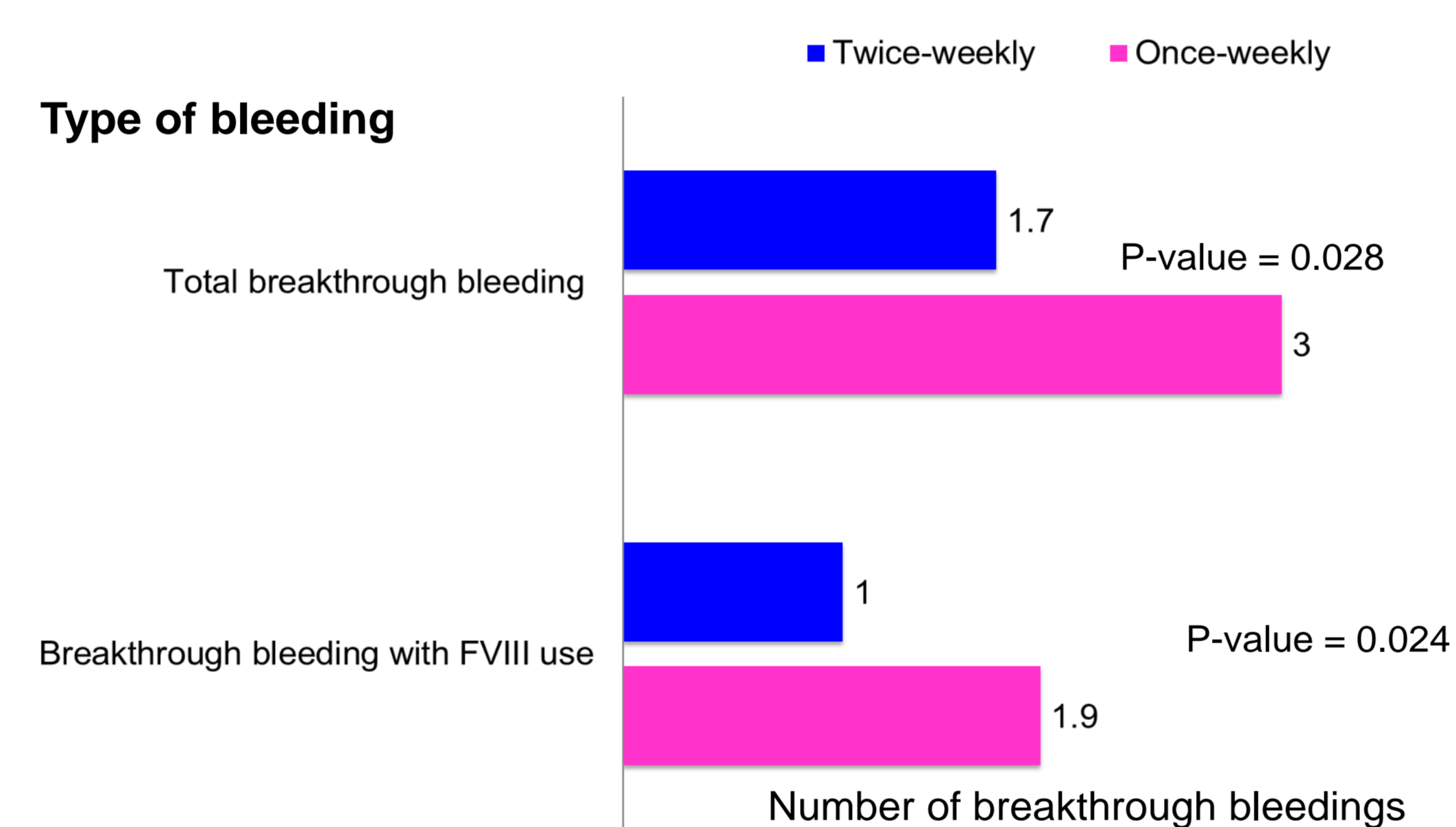


Table 2: Sites of bleeding in 2 different prophylaxis regimen

Variables	Treatment				p-value
	Twice-weekly	total	Once-weekly	total	
Sites of bleeding median (min-max)					
Joint					
Elbow	0 (0-3)	5	0 (0-7)	15	
Knee	0 (0-1)	4	0 (0-3)	8	
Ankle	0 (0-2)	3	0 (0-4)	6	
Hip	0 (0-1)	1	0 (0-1)	1	
(Total)	0.5 (0-3)	13	1 (0-7)	30	0.02
Non-joint bleeding	0 (0-3)	15	1 (0-5)	26	0.12

Table 3: Quality of life and appreciation score in 2 different prophylaxis regimen

Variables	Treatment		p-value
	Twice-weekly	Once-weekly	
Quality of life (100 points) Mean ± SD. Median (min-max)	91.56 ± 7.68 92.5 (80-100)	84.06 ± 9.69 82.5 (65-100)	0.005
Appreciation score (100 points) Mean ± SD. Median (min-max)	90.69 ± 6.80 90 (80-100)	72.19 ± 12.51 70 (50-100)	0.001

RESULTS

Sixteen patients were enrolled. Eleven and five were severe and moderate hemophilia A, respectively (Table 1). The median numbers of breakthrough bleeding and dosage of FVIII requirement for treatment of breakthrough bleeding in twice-weekly regimen were significantly lower than once-weekly regimen. (1.7 vs. 3.0, p=0.03 and 250 units vs. 1,500 units, p=0.01) (Fig 1). The patients who on once-weekly regimen had a significantly higher total joint bleeding than twice-weekly regimen but there was no significant difference in soft tissue bleeding (Table 2). There was no significant difference in number of hospital admission days, school days lost and HJHS. The median QoL score and appreciation score in twice-weekly regimen was significantly better than once-weekly regimen, respectively (95 vs. 82.5, p=0.005 and 90 vs 70, p=0.001) (Table 3).

CONCLUSIONS

Twice-weekly prophylaxis regimen was better than once-weekly regimen regarding breakthrough bleeding prevention, increased QoL and economically suited for Thai children with moderate/severe HA, despite similar total dosage of FVIII use.

References:

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3. Devlin NJ, Krabbe PF. The development of new research methods for the valuation of EQ-5D-5L. Eur J Health Econ 2013; 14 Suppl 1 : S 1-3

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Clotting Factor Concentrates
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13-PP-M
910ZHM