Comparison of Different Prophylaxis Regimens for Moderate/severe Hemophilia A Children

Polpong na Songkhla, Pattaramon Aungbumnet, Darintr Sosothikul*
Division of Hematology and Oncology, Department of Pediatrics, Faculty of Medicine, King Chulalongkorn Memorial Hospital, Chulalongkorn University, Bangkok, Thailand 10330

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. 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 quality of life (QOL).
- Level of factor VIII and factor VIII inhibitor, hemophilia joint health score (HJHS), quality of life (QOL) score using EQ-5D-5L and appreciation score were also monitored.
- The development of new research methods for the valuation of EQ-5D-5L.

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 HIJS. 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:

Acknowledgements: This study was supported by, the Ratchadapiseksompotch Fund, Faculty of medicine, Chulalongkorn University and Thai Society of Hematology.