

The efficacy of low-dose prophylaxis in haemophilia A children

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Objectives: Prophylactic treatment for severe hemophilia A is likely to be more effective than on demand treatment, however, prophylaxis is costly. Limited by economy and inadequate treatment, developing countries face huge challenge to reduce disability and improve quality of life (QoL) of haemophilic children. The aim of this study was to investigate the efficacy of low dose prophylaxis in haemophilia A children in China.

Methods: We assigned 33 children with moderate or severe hemophilia A to receive low dose factor VIII prophylaxis (Kogenate® FS, 10–15 IU kg⁻¹ per time, twice weekly), three months for a course, two consecutive courses. Bleeding frequency, self-care ability, inhibitor development and treatment costs before and after prophylaxis were analyzed. Bethesda method is used to detect factor VIII inhibitor.

Results: After prophylaxis, the frequency of joint bleeding significantly decreased either in children with moderate (3.5 vs. 0.3) or severe hemophilia A (8.7 vs. 1.15); Joint X-ray assessment was carried out in eight children (5 cases above 4 years old, 3 cases under 4 years old), the results show that the children who start prophylaxis before 4 years old had no changes in synovial, and original synovial lesions eased in those start prophylaxis after 4 years old. Self-care ability improved in all children, and the percentage of children who enter school increased more significantly; none of them developed factor VIII inhibitor. For the patients who have more than 1.5 bleeds monthly, the costs of prophylaxis is less than on demand treatment.

Conclusion: Low-dose prophylaxis for haemophilia A children is safe and efficacy; and compared with on demand treatment, the costs of prophylaxis did not increased significantly.

