

Adherence to prophylaxis and bleeding outcome in children and adolescents with severe haemophilia

ABSTRACT

Background: Severe haemophilia can lead to spontaneous bleeds which can be prevented by the use of prophylaxis. In children and adolescents, several factors can influence adherence to the prescribed prophylaxis regime. They experience different kind of challenges, depending on their age. Now, children and adolescents receive comparable care although the need for guidance is likely to vary. To be able to provide tailored care, the relationship between adherence, age and bleeds should first be investigated.

Aim: The aim of this study was to explore adherence to prophylactic treatment and its association with age and frequency of bleeding events in children and adolescents with severe haemophilia.

Methods: A quantitative, cross-sectional survey was conducted, using existing data that was collected between 2011 and 2016 during outpatient visits in the Netherlands (Utrecht). The focus was on children and adolescents with severe haemophilia. During this study, demographic data and data of bleeding events were extracted from electronic patient medical records. A Spearman's rank correlation was conducted to analyze adherence to prophylaxis and its association with age and number of bleeds.

Results: In total, 68 patients (children and adolescents) were included whereafter data was analyzed. An adherence was found of median 84.9%. A significant correlation was found between the VERITAS-pro total score and an increasing age ($r_s = 0.37$; $P = 0.002$). No significant correlation was found between the VERITAS-pro total score and total bleeds or joint bleeds.

Conclusion and Recommendation: This study showed that patients were less adherent as they increased in age. No association was found between adherence and bleeds. Future research should focus on optimizing care, depending on age, to enable tailored care from physicians and nurses fitted to patients age.

Keywords: *adherence, prophylaxis, bleeds, severe haemophilia, minors*