Successful Treatment of Glanzmann's Thrombasthenia with Recombinant Activated Factor VII "NovoSeven

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OBJECTIVES

Glanzmann thrombasthenia (GT) is an extremely rare autosomal recessive coagulopathy. It is characterized by significant prolonged bleeding time and normal platelet count. The cause of disease is the quantitative or qualitative defect of the platelet glycoprotein IIb–IIIa. As a result, the aggregation processes via fibrinogen bridging of platelets to other platelets cannot occur properly. The disease characteristically presents as mucosal bleeding such as epistaxis, gum, dental extraction bleeding, menorrhagia, increased bleeding post-operatively and rarely muscle hematomas, central nervous system bleeding and hemarthrosis. Recombinant activated factor VII (rFVIIa, NovoSeven, Novo Nordisk, Bagsvaerd, Denmark) as a universal hemostatic agent has been used in many bleeding disorders including GT.

Purpose:

The present study aims to review the use of NovoSeven in treatment of 53 patients with GT from south west Iran.

METHODS

A total of 53(27 male, 26 female) patients with GT in the range of one to 50 years old were enrolled in this study. During two years 106 bleeding episodes and 43 surgical procedures (minor and major), including epistaxis; gum bleeding, menorrhagia, muscle hematomas, post minor trauma bleeding , hemarthrosis , circumcision, tooth extraction, cesarean section, dilation and curettage, ovarian cyst removal, and fracture fixation were recorded and analysed.

NovoSeven at a dose of 90µg/kg (1-42 injections), along with tranexamic acid (15-25 mg/kg) with or without local measures were used for 91.5 % of bleeding episodes and 95% of surgical procedures. This is a descriptive cross sectional study that was conducted at Ahvaz Jundishapur University of Medical Sciences in Khuzestan province. Data were collected by a questionnaire form. Statistical analysis was done using Statistical Package for the Social Sciences version 17.

RESULTS

NovoSeven was effective in complete cessation of bleeding in 95 % of bleeding episodes and 93% of surgical procedures. There were five partial failures in bleeding episodes and two partial failures for major surgical procedures. No serious adverse events such as thrombo embolisms were seen. Blood transfusion was used for three major surgical interventions and two bleeding episodes.

CONCLUSIONS

Tengborn and Petruson first reported the use of rFVIIa in the treatment of a GT patient with epistaxis. Following this report, several other authors supported the efficacy of NovoSeven in the management of bleeding in patients with GT. The present study shows that NovoSeven is an effective treatment for GT patients with protean bleeding presentations. However, alloimmunization is a major complication of repeated platelet transfusion. NovoSeven can be a good alternative or the first treatment of GT patients in conditions where access to platelets is difficult.

References

Text

DOI: 10.3252/pso.eu.WFH2014.2014