PROPHYLACTIC TREATMENT IN HAEMOPHILIA

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The administration of prophylactic infusions of coagulation factor concentrates is now generally accepted as representing the best approach to the modern management of haemophilia. Data from a recent prospective randomised controlled trial have confirmed the longstanding impression that such treatment reduces the incidence of joint bleeds and also protects against the development of joint damage. The cartilage of young children is particularly vulnerable to destruction mediated primarily by iron. The conventional treatment regime involves administration of 20-40 iu/kg three times weekly in the case of haemophilia A and twice weekly in haemophilia B. However, there are many variants in treatment approach and a Canadian study has suggested that once weekly treatment is a reasonable initial alternative, with escalation of the dosage if breakthrough bleeding occurs. Periodic measurement of trough levels of factor VIII is recommended as there is a clear correlation between the probability of spontaneous bleeding and time spent with a baseline level below 1%. Firm data are also available to show that prophylaxis is associated with enhanced quality of life in children. There is also some evidence that the incidence of extra-articular bleeding, including intracranial haemorrhage, may be reduced in children on prophylaxis. Data also suggest that early adoption of prophylaxis may confer protection against inhibitor development. One of the contentious issues concerns the age at which infusions should begin. The use of implantable venous devices certainly makes early treatment of children easier although complications such as thrombosis and bacterial infection are not infrequent. Another area of active debate is whether prophylaxis should be continued in adults. Factor VIII preparations with extended duration of action are now under development and clinical trials of prophylaxis of bypassing agents in patients with inhibitors have been conducted.