

school or work days missed, and enhancement of the child's self-esteem and independence.

Prophylactic therapy is periodic factor replacement for children with severe hemophilia to prevent bleeding complications, including arthropathy and spontaneous life-threatening bleeding events (Coppola, Tagliaferri, Di Capua, et al, 2012; Montgomery, Gill, and DiPaola, 2009; Scott and Montgomery, 2011; Zimmerman and Valentino, 2013). Primary prophylaxis involves the infusion of factor VIII concentrate on a regular basis before the onset of joint damage. Secondary prophylaxis involves the infusion of factor VIII concentrate on a regular basis after the child experiences his or her first joint bleed. The administration of infusions differs among treatment centers and may range from every other day to three times a week for several weeks to promote healing. On-demand factor replacement may be a cost-effective alternative to primary prophylaxis, but prophylaxis decreases the development of joint disease and preserves joint function compared with on-demand factor replacement treatment (Iorio, Marchesini, Marcucci, et al, 2011; Manco-Johnson, Abshire, Shapiro, et al, 2007). Prompt appropriate treatment of hemorrhage and prophylactic therapy are key to excellent care and prevention of long-term morbidity in patients with hemophilia (Lillicrap, 2013; Montgomery, Gill, and DiPaola, 2009).

Prognosis

Although there is no cure for hemophilia, its symptoms can be controlled and its potentially crippling deformities greatly reduced or even avoided. Today many children with hemophilia function with minimal or no joint damage. They have an average life expectancy and are normal in every respect but one—they have a tendency to bleed, which is a significant inconvenience but not necessarily a life-threatening event.

Gene therapy may prove to be a treatment option in the future. Techniques are under development to introduce factor VIII and factor IX genes into hepatocytes, fibroblast, endothelial cells using adeno-associated viral vectors, and other novel ideas for genetic correction (Branchford, Monahan, and Di Paola, 2013; Nienhuis, 2008; Walsh and Batt, 2013). Problems exist with appropriate selection of the vector, identification of the cell for gene expression,