Review Article

Current therapeutic approaches in the management of hemophilia—a consensus view by the Romanian Society of Hematology

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Abstract: Hemophilia A (HA) and hemophilia B (HB) are rare disorders, being caused by the total lack or under-expression of two factors from the coagulation cascade coded by genes of the X chromosome. Thus, in hemophilic patients, the blood does not clot properly. This results in spontaneous bleeding episodes after an injury or surgical intervention. A patient-centered regimen is considered optimal. Age, pharmacokinetics, bleeding phenotype, joint status, adherence, physical activity, personal goals are all factors that should be considered when individualizing therapy. In the past 10 years, many innovations in the diagnostic and treatment options were presented as being either approved or in development, thus helping clinicians to improve the standard-of-care for patients with hemophilia. Recombinant factors still remain the standard of care in hemophilia, however they pose a challenge to treatment adherence because they have short half-life, which where the extended half-life (EHL) factors come with the solution, increasing the half-life to 96 hours. Gene therapies have a promising future with proven beneficial effects in clinical trials. We present and critically analyse in the current manuscript the pros and cons of all the major discoveries in the diagnosis and treatment of HA and HB, as well as identify key areas of hemophilia research where improvements are needed.

Keywords: Hemophilia; diagnosis and management; consensus view; Romanian Society of Hematology