Summary

Hemophilia is a rare disorder that is complex to diagnose and to manage. These evidence-based guidelines offer practical recommendations on the diagnosis and general management of hemophilia, as well as the management of complications including musculoskeletal issues, inhibitors, and transfusion-transmitted infections. By compiling these guidelines, the World Federation of Hemophilia (WFH) aims to assist healthcare providers seeking to initiate and/or maintain hemophilia care programs, encourage practice harmonization around the world and, where recommendations lack adequate evidence, stimulate appropriate studies.

Introduction

The first edition of these guidelines, published in 2005 by the WFH, served its purpose of being a useful document for those looking for basic information on the comprehensive management of hemophilia. The need for revision has arisen for several reasons. The most significant of these was to incorporate the best existing evidence on which recommendations were based. There is recent high quality data from randomized controlled trials establishing the efficacy and superiority of prophylactic factor replacement over episodic treatment – though the optimal dose and schedule for prophylaxis continue to be subjects of further research. There is also greater recognition of the need for better assessment of outcomes of hemophilia care using newly developed, validated, disease-specific clinimetric instruments. This revised version addresses these issues in addition to updating all sections.

These guidelines contain several recommendations regarding the clinical management of people with hemophilia (**practice statements**, in **bold).** All such statements are supported by the best available evidence in the literature, which were graded as per the 2011 Oxford Centre for Evidence-Based Medicine (see Appendix I). Where possible, references for recommendations that fell outside the selection for practice statements were also included. These references have not been graded.

A question often raised when developing a guideline document such as this is its universal applicability given the diversity of health services and economic systems around the world. Our strongly held view is that the principles of management of hemophilia are the same all over the world. The differences are mainly in the doses of clotting factor concentrates (CFC) used to treat or prevent bleeding, given that the costs of replacement products comprise the major expense of hemophilia care programs. Recognizing this reality, these guidelines continue to include a dual set of dose recommendations for CFC replacement therapy. These are based on published literature and practices in major centres around the world. It should be appreciated, however, that the lower doses recommended may not achieve the best results possible and should serve as the starting point for care to be initiated in resource-limited situations, with the aim of gradually moving towards more optimal doses, based on data and greater availability of CFC.

One of the reasons for the wide acceptance of the first edition of these guidelines was its easy reading format. While enhancing the content and scope of the document, we have ensured that the format has remained the same. We hope that it will continue to be useful to those initiating and maintaining hemophilia care programs. Furthermore, the extensive review of the literature and the wide consensus on which practice statements have been made may encourage practice harmonization around the world. More importantly, in areas where practice recommendations lack adequate evidence, we hope that this document will stimulate appropriate studies.