Exploring hemophilia and its ramifications involves a myriad of medical disciplines—hematology, immunology, genetics, infectious disease, radiology, nursing, and pharmacology, to name a few. During two recent professional gatherings of hematologists and other health professionals, an international cadre of experts on hemophilia reviewed our current understanding of this difficult disease and shared insights on its management and impact on society.

Some 3,000 healthcare professionals attended or participated in over 40 educational sessions offered during the 65th Annual Meeting of the National Hemophilia Foundation, held in Anaheim, California, October 3–5, 2013, to better understand this disease and associated issues. During the 55th Annual Meeting of the American Society of Hematology, which took place in New Orleans, Louisiana, December 7–10, 2013, over 18,000 hematologists and other healthcare professionals took part in scientific sessions and presentations, many of which involved the etiology, diagnosis, and treatment of bleeding disorders. This premiere edition of The Hemophilia Report delves into subjects pertinent to the practices of hematologists and other healthcare professionals involved in managing hemophilia.

### HEMOPHILIA THERAPY

Duc Q. Tran, MD, from Emory University in Atlanta, Georgia, provides a historic perspective on the treatment of hemophilia. Among the subjects he covers are the successes and challenges of prophylactic replacement factor therapy in children; in particular, the results of the Joint Outcome Study and the Evaluation Study on Prophylaxis: a Randomized Italian Trial (ESPRIT) are compared and contrasted with data collected during other clinical trials of prophylactic regimens. Dr. Tran also reviews the major studies on prophylaxis versus episodic, on-demand therapy in adults and the current options for managing the development of alloimmune inhibitory antibodies to factor VIII (FVIII).

Anthony Sung, MD, from Duke University Medical Center in Durham, North Carolina, summarizes the history of hemophilia therapy and a number of discoveries that are advancing treatment of the disease. He writes about strategies toward longer-acting clotting factors through the use of PEGylation, the attachment of polyethylene glycol (PEG) molecules to recombinant factor proteins, and fusion protein technologies, among others, that promise to improve the efficacy of treatment and reduce the burden of infusions. The latest safety and efficacy data from clinical trials with these novel agents are provided. Dr. Sung then reviews challenges to reversal and inhibition of bleeding presented by the use of older and newer oral anticoagulants. The efficacy of prothrombin complex concentrates and recombinant activated factor VII for traumatic or surgical bleeding and reversal of bleeding complications resulting from oral anticoagulation are detailed.

### CHALLENGING DECISIONS

Anne Chalmers, MD, from Rush University Medical Center in Chicago, Illinois, compares hemophilia A with hemophilia B and confronts challenging treatment decisions. When treatment should be started, what the optimal dose of certain therapeutic agents should be, and how long prophylactic therapy should continue remain controversial. A historic view of hemophilia management has led to greater regard for prophylaxis rather than symptomatic treatment, even though optimal prophylactic regimens and schedules continue to be explored.

In a separate article, Dr. Chalmers reviews our current understanding of the genetics of hemophilia A and B. She addresses the spectrum of genetic mutations that lead to development of each disease, strategies for genetic testing, and how data from such tests could be used to guide treatment.

### NOVEL THERAPEUTICS

Holleh D. Husseinzadeh, MD, from the Hospital of the University of Pennsylvania in Philadelphia, covers recent advances in novel therapeutics for bleeding disorders,
including gene-transfer options. Also discussed are new insights into the application of prophylaxis for hemophilia complicated by inhibitors; issues that affect compliance with therapy; and outcomes of total joint replacement in patients with congenital bleeding disorders.

MEETING THE CHALLENGES AHEAD

Kerry Hege, MD, from the Indiana University School of Medicine in Indianapolis, takes a closer look at the improved outcomes and remaining challenges with prophylactic therapies for hemophilia and the results of clinical trials with current state-of-the-art agents. Dr. Hege describes efforts to increase patient adherence to prophylactic regimens by prolonging the half-life of recombinant factor products (and thereby reducing the need for frequent dosing), prevent bleeding episodes, and improve patient quality of life. Among the exciting subjects she covers are PEGylation, polysialylation, fusion protein technology, and gene therapy.

In recent years, many advances in preventing bleeding, reducing the risk of inhibitor formation, and managing acute bleeds in hemophilia have been reported. Noa Biran, MD, from the Mount Sinai School of Medicine in New York, reports on studies investigating the use of recombinant FVIII and factor IX crystallizable fragment fusion protein in patients with severe hemophilia A or B, respectively. Dr. Biran also reviews the results of studies comparing early prophylactic measures with episodic therapy, noting that prophylaxis has resulted in superior results. He also reports on the usefulness of magnetic resonance imaging to monitor hemophilic joint disease, covers strategies to reduce the incidence of inhibitors, and touches upon methods to limit acute bleeding.

By sharing the information imparted by hemophilia experts at these two important medical meetings, the authors of this report greatly enhance our understanding of many complicated issues involved in hemophilia prophylaxis and management. Future editions of The Hemophilia Report certainly will add to these insights and further assist busy practitioners and nurses in keeping abreast of novel developments in the optimal management of hemophilia.