

13 March 2009

P.M. Mannucci
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Department of Medicine and Medical Specialties
Via Pace, 9
20122 Milano, Italy

RE: Inhibitor Development in Previously Untreated Patients (PUPs) or Minimally Blood Component-Treated Patients (MBCTPs) when Exposed to von Willebrand Factor-Containing Factor VIII Concentrates and to Recombinant Factor VIII Concentrates: An International, Multicentre, Prospective, Controlled, Randomized, Open Label, Clinical Trial

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Dear Professor Mannucci:

As you are familiar, the World Federation of Hemophilia is an international not-for-profit organization dedicated to improving the lives of people with hemophilia and related bleeding disorders.

As we move toward our vision of achieving *Treatment for All*, we recognize that there are several important research questions which to date have not been adequately addressed and which must be answered if we are to achieve our vision.

The WFH Strategic Plan calls upon the WFH to “*Promote Access to Safe and Improved Treatment and Cure-related Research.*” Specific to inhibitors, the strategic plan objectives state that the WFH will “*Monitor research and treatment advances relative to adverse events (inhibitors, HIV, HCV) and provide timely information on their prevention, treatment and management.*” It is therefore incumbent upon us to encourage research to address these areas as is your intention in the SIPPET study.

We also recognize that inhibitor development has replaced pathogen risk as the most common adverse event for patients with hemophilia A and that global/ multi-national collaboration is essential for a better understanding of the risk factors for inhibitors and immunogenicity of different treatment products.

Answers are needed so that better informed treatment decisions may be made based on scientific evidence. When the key research questions related to inhibitor formation and management are answered, this will lead to a dramatic improvement in the quality of treatment and care, as well as a better, safer and longer life for the thousands of patients living with an inhibitor today.

Through our various programs we are actively engaged in monitoring scientific and technological developments that can achieve successful cures or more efficacious treatment of inherited bleeding disorders. One of our roles is to foster debate and dialogue on this critical issue. We support clinicians and researchers working in the field to achieve this goal, and are most grateful to those patients who have and will participate in clinical trials designed to seek a better understanding of inhibitors.

Improving treatment is a challenge that cannot be met by the efforts of one individual, organization, company or country. A global approach and community effort is required. The best way to achieve this is through multinational clinical trials that incorporate a commitment to patient protection and careful adherence to generally accepted guidelines for testing experimental therapies in humans. As new hemophilia trials are instituted, patient safety must remain a high priority, and institutional review boards must maintain rigor in approving informed consent procedures. These trials must also involve the active participation of physicians with long-standing experience in the clinical management of hemophilia.

We believe with proper design and careful attention to the safety of the patient, the critical research questions related to inhibitor formation and management can be answered. We are eager for this field of research to proceed and we support the pursuit of research leading to a better life for those living with an inhibitor. Although it will take time and much remains to be done, we are hopeful that research to find answers in this critical research area will advance expeditiously.

We are pleased to offer our endorsement as to the critical importance of this study and look forward to hearing of its imminent approval, funding and implementation.

Thank you again for advising us of the aims and study plan.

Respectfully,



Mark W. Skinner
WFH President



Alison Street, MD
Vice President Medical