



## First Laureate Applied Research



- **Name:** Prof. Pier Mannuccio Mannucci
- **Nationality:** Italian
- **Date of Birth:** 1939
- **Position:** Professor of internal Medicine & Chairman of the Department of Medicine & Medical Specialties Foundation University Hospital of Milan, Italy
- **Scientific Affiliation:** University of Milan, Italy

**Project Title:** 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 Randomised, Open Label, Clinical Trial.

### Abstract:

The study is a clinical trial on inhibitor frequency in patients with hemophilia previously untreated (PUPs) when exposed to plasma-derived, von Willebrand factor-containing factor VIII (VWF/FVIII) concentrates or to recombinant factor VIII (rFVIII) concentrates. Patients will be randomized to be treated exclusively with a single FVIII product, either plasma-derived and VWF containing or recombinant, and followed up until inhibitor development or until 50 exposure days (EDs) have elapsed, whichever comes first. Study objectives are: Primary: To assess whether or not VWF/FVIII are less immunogenic than rFVIII concentrates by determining the frequency of inhibitor development in the first 50EDs in PUPs. Secondary: To evaluate the anamnestic response of inhibitor patients; to evaluate the frequency of transient inhibitors; to evaluate the modality of occurrence of inhibitors (number of exposure days, titer at onset, etc); to evaluate clinical factors potentially associated to inhibitor development (age at first treatment, severity of bleeding episodes, surgery, intensity of treatment, modality of treatment, severity of bleeding episodes, surgery, intensity of treatment, modality of treatment, time of treatment in relation to vaccinations, concurrent diseases (in particular viral infections) and/or medications, type of delivery, breast feeding, type of milk formula; To evaluate laboratory factors potentially associated to inhibitor development (severity of FVIII defect, FVIII gene defect, FVIII Ag, HLA phenotype, IL-10 and TNF  $\alpha$  genotypes, epitope mapping); To evaluate the incidence of other adverse events related to the products used.

### Biography:

Pier Mannuccio Mannucci is currently Professor of Internal Medicine Chairman of the Department of Medicine and Medical Specialties at the Foundation University Hospital of Milan. He is also director of the Angelo Bianchi Bonomi Hemophilia and Thrombosis Center, a clinical unit and associated research center characterized by long lasting expertise in the field of hemorrhagic and thrombotic disorders. He received many awards and prizes such as the International Prize by the French Hemophilia Association, the Murray Thelin Award by the USA National Hemophilia Foundation, the Distinguished Career Award and the Robert Grant Medal by the International Society for Thrombosis and Haemostasis and the Ham-Wasserman Lecture and Award by the American Society of Hematology. He has also received numerous prizes and awards in Italy, from the President of the Republic, the Ministry of Health, the Milan Province, the Lombardy region and the Italian Society of Internal Medicine. From 1996-1997, he was President of the International Society for Thrombosis and Haemostasis and from 1998 to 2006 Editor-in Chief of the official journal of the Society the Journal Thrombosis and Haemostasis. He ranks among the 214 highly cited scientists in the Clinical Medicine category, in the period 1981-1999.