

## SSC Subcommittee Project/Collaborative Project

- Name of the Project: **CLINICAL TRIAL DESIGN FOR HEMOPHILIA**
- Person responsible (Chair / Principal Investigator): Dr. Donna DiMichele (USA)
- Members: Dr. Nisha Jain (USA)  
Dr. Anneliese Hilger (Germany)  
Dr. Alok Srivastava (India)  
Dr. Flora Peyvandi (Italy)  
Dr. Sebastien Lacroix-Desmasez (France)  
Dr. Frits Rosendaal (The Netherlands)  
Dr. John Scott (USA)
- Aim / Mandate of the project: The mandate of the project will be to determine the optimal prospective pre- licensure and observational post- licensure trial designs for new clotting factor concentrates (CFCs) for hemophilia on the basis of 1) the harmonized safety and efficacy data required by regulators for registration; 2) the anticipated available study population; and 3) innovative clinical trial design suitable for rare diseases as hemophilia.
- Methodology:

There are varying recommendations for the assessment of the clinical safety and efficacy of CFCs between different regulatory agencies in the world. While the underlying philosophy of ensuring the safety and efficacy of these drugs can be well appreciated, the scientific basis of some of these requirements is sometime unclear. It would be helpful if the basis of some of these recommendations could be discussed and harmonized between the major regulators in the world.

The project members will review the current recommendations with guidance from regulators in the US and Europe, industry, scientific and methodological experts, as well as clinical investigators

It will discuss, among others, the following issues:

1. consensus definitions on hemophilia:
  - severity
  - prophylaxis related issues
  - inhibitors
  - bleeds
  - response to treatment
2. the ideal population to be studied with respect to numbers and types of subjects
3. duration of the study

4. Dosing and number of exposures
  5. Assay methodology
  6. Surgical studies: types and number needed.
- Year of starting: 2011
  - Annual report of project: The Design of Clinical Trials in Hemophilia Project Group (PG) began its deliberations in February 2011 and has continued its work through a series of 7 teleconferences to date. No formal recommendations have yet emerged but the plan of action , as outlined above, has been initiated.
  - Year of completion (expected): 2013