

# COAGULATION FACTOR VIII: PHARMACOECONOMIC EVALUATION AND QUALITY OF LIFE AS MEASURING TOOLS IN CLINICAL PRACTICE

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**Introduction:** Hemophilia A is an inherited, X-linked, recessive disorder caused by deficiency of functional plasma clotting factor VIII (FVIII). The disease, which has an incidence of 1 in 6000 male births, can be defined as mild, moderate, or severe depending on the level of FVIII deficiency. Severe hemophilia A is characterized by spontaneous bleeding episodes localized mainly in muscles (hematomas) and joints (hemarthroses). The therapy relies upon the occasional administration of the deficient factor in the case of bleeding (“on demand” treatment), or regular factor VIII administration in order to prevent bleedings (“prophylaxis”). End-stage chronic arthropathy and chronic liver disease are frequent complications of most patients treated “on demand”. Clinical and pharmacoeconomic studies underline the economic gap existing between the two different types of treatment of severe hemophilia A; however, the prophylaxis strategy started in Italy less than 20 years ago, so few studies are so far available, which compare the actual costs of both treatment modalities. Such studies anyway are based on pharmacoeconomic evaluations of the prophylactic treatment as related to improvement of the patients’ quality of life (QoL), but do not compare the costs of prophylaxis with the real ones of the “on demand” treatment and its long-term complications and HIV infections.

**Methods:** Our observational study aims to evaluate the cost utility of the two strategies, through an EQ-5D questionnaire administered to 100 severe hemophilia A patients, divided into:

- adults (25-70 years) treated “on demand”, who, as a result of the disease and the type of treatment, developed chronic arthropathy and hemophilia-related comorbidities;
- children and young adults (<25 years), who started replacement prophylaxis when still children, and did not develop such comorbidities.

The evaluation of patients’ data from clinical charts allowed us to calculate the total costs of primary prophylaxis and of “on demand” treatment.

By using the Markov model we were able to compare the hypothetical costs of both strategies based on the assumption that patients on prophylactic treatment will not develop hemophilia-related complications, as patients treated on demand do.

**Results and Conclusions:** It seems likely that primary prophylaxis, initially more expensive, after some years improves the Quality of Life of patients; in the long distance primary prophylaxis might result a cost-effective strategy due to a significant reduction of hemophilia-related comorbidities. Therefore, primary prophylaxis in Hemophiliacs might exert even a reduction of the economic impact on regional health costs.