

SNH - Abstract n° 1

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THE "SUIVI THÉRAPEUTIQUE NATIONAL DES HÉMOPHILES",
COHORT STUDY OF HEMOPHILIACS IN FRANCE:
RATIONALE, ORGANIZATION AND PRELIMINARY RESULTS

Group of the "Suivi thérapeutique National des Hémophiles" - France

Following a decision of the French Ministry of Health, a prospective multicenter cohort study ("Suivi thérapeutique National des Hémophiles" or SNH) based on a voluntary participation of physicians and patients was designed to assess long term safety of treatment with plasma-derived (pd) and recombinant (r) clotting factor concentrates for patients with hemophilia.

Patients and Methods: Hemophilia A or B patients non-previously or previously treated with clotting factors are enrolled after giving written informed consent. Clinical events, biological data (inhibitor to FVIII or FIX, viral status, etc.), treatments (number of treatment episodes, product and amount used, lot number, etc.) and adverse events are collected on standardized forms. Samples of blood are collected for establishing a repository of biologic specimens (frozen serum, plasma and mononuclear cells) in a central laboratory for future studies. On annual follow-up visits the same data and samples are collected. In some cases (patients non-previously treated and inhibitor development), a quarterly follow-up is introduced. This study is performed according to good clinical and epidemiological practices. All the data are recorded in a computerized database.

Results: From October 1994, 38 hemophilia centers have enrolled 835 hemophiliacs in the SNH. Data from 767 patients have been included in the present report: 660 with hemophilia A and 107 with hemophilia B (14%). Sixty six per cent of patients had a severe disease (factor level < 2%), 52 % were less than 15 years old and 33% had less than 50 cumulative exposure days (CED) at entry. In the cohort, 15% of patients are HIV positive and 54% HCV positive. The mean follow-up duration is 16.1 months, and the total is 1,030 patient-years corresponding to 42,610 CED to coagulation products. For the patients treated in 1997, the last product used was pd-FVIII in 111 (26%) and r-FVIII in 318 (74%) hemophilia A patients and pd-FIX in all hemophilia B patients. FVIII or FIX use was within 0-499 IU/Kg/year in 29% of severe hemophiliacs, 500-1,999 in 40% and 2,000 or over in 31%. Thirteen new inhibitors were detected: 2 on pd-FVIII, 7 on r-FVIII and 4 in patients who had received pd-FVIII then r-FVIII before inhibitor development; 4 of these patients are high responders (11-156 BU), while the other 9 still remain with an inhibitor titer below 10 BU (0.6-7.8 BU); the follow-up is still too short and the number of new inhibitors too low to draw any definite conclusion on this issue.

Conclusion: A wide network of health professionals has been established including more than 60 hemophilia specialist physicians, epidemiologists, Drug Agency and Health Administration representatives. It will allow together with samples stored in the Central laboratory any fast investigation in case an alert occurs.