

showed that prophylaxis in adult patients with haemophilia is effective in reducing the bleeding rate. Despite the higher cost of prophylactic treatment compared to on-demand treatment prophylaxis showed to have a good cost-effectiveness ratio.

PHM2

THE COST OF CARE OF HEMOPHILIC PATIENTS WITHOUT INHIBITORS: THE COCHE STUDY

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OBJECTIVE: Hemophilia is a rare but very expensive disease. Available treatment strategies have prolonged patients' life expectancy and are now focused on improving their quality of life. We evaluated cost of care of hemophilic patients without inhibitors. **METHODS:** The Cost Of Care of Hemophilia (COCHE) is a naturalistic, multicenter, longitudinal study (time horizon 8 months) involving moderate and severe patients with hemophilia A or B. Patients, aged >18 years, without inhibitors, were sequentially enrolled at 23 Italian Hemophilia Centers. Information on socio-demographic, clinical, resource absorption, quality of life and treatment satisfaction was collected. This analysis pertains on estimate of cost of care with clotting factor concentrates (perspective of Italian National Health Service, data expressed as € of 2004). **RESULTS:** A total of 232 patients were enrolled (median age = 34.3 years, 18–74), 86.6% had hemophilia A, 72.4% were severely affected. At the time of enrolment 81.0% of patients had chronic hepatic C, 25.0% hepatitis B, 15.9% HIV infection. Patients reporting orthopedic problems were 87.8% and those with target joints were 57.0%. Bleeding occurred with a mean frequency of 2. Ten hemorrhages/patient/month (median 1.44, 0–26). At enrolment, 58.5% of the treated patients were administered recombinant products, with an increase of 11.4% in 4 months of follow-up. At the enrolment 67.4% of patients were treated on demand and 32.6% on prophylaxis regimen. Patients on demand reported on average 2.26 hemorrhages/patient/month (median = 1.87, 0–8.5), those on prophylaxis 1.56 hemorrhages/patient/month (median = 0.5, 0–26). Overall, patients cost €8341/patient/month, €4200 those on demand, €16,473 those on prophylaxis regimen. The incremental cost per avoided hemorrhage in patients on prophylaxis vs. those on demand was €2123. **CONCLUSIONS:** Hemophilia is one of the most expensive diseases: clotting factor concentrates used to manage hemorrhages and surgery contributes to the most part of the cost, providing patients with relatively good levels of quality of life.

PHM3

COSTS OF HAEMOPHILIA ASSISTANCE IN ROMANIA

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OBJECTIVES: Economic factors are very important in limiting therapy options for haemophilia, but inadequate treatment lead to costly consequences having a negative impact on patients' social integration **AIM OF THE STUDY:** to evaluate direct medical costs (therapy and hospitalization) of bleedings, secondary prophylaxis and surgical interventions; direct non-medical costs (home-hospital travel costs) and indirect costs (morbidity costs, loss of income of family members, average

number of days off at school/work). **METHOD:** A total of 224 haemophiliacs registered and treated in Haemophilia Centre Timisoara and in Clinical Centre for Evaluation and rehabilitation "Christian Serban" Buzias, followed-up during a seven-years period. 84.38% of the patients had haemophilia A and 15.62%-haemophilia B. Data was obtained from medical charts and from questionnaires administered to patients. Because in a developing country an economic analysis is difficult to ascertain, unitary costs were expressed in €, at average exchange rate communicated by the National Romanian Bank for the last year of the study period. **RESULTS:** Therapy costs represented 54.56% of direct medical costs in haemophilia A patients without inhibitors, 67.13% in haemophilia B- and 87.63% in patients with high-titer inhibitors. Pseudotumour consumed the highest financial resources in haemophilia A patients and complicated haematoma was the most costly complication in haemophilia B patients. Direct non-medical costs represented important percents of mean patient and family income. Mean monthly morbidity cost was €108.28 and loss of income of family members who forfeid employment in order to offer home care for haemophilia patients was €81.88/month. Average number of days off at school/work was 46.64/year, varying according to haemophilia severity. **CONCLUSIONS:** Inadequate resource allocation for haemophilia treatment lead to costly complications, affecting social integration and leading to important loss of income, which is responsible for a poor treatment compliance, all these factors having a strong interactions.

PHM4

UK COST COMPARISON OF BUCY2 CONDITIONING IN ALLOGENEIC HSCT: ORAL VERSUS IV BUSULFAN (BUSILVEX®)

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Allogeneic HSCT is a cost-intensive procedure. Oral busulfan (Bu) as part of BuCy2 is a commonly used conditioning regimen but is associated with high plasma variability. IV Bu has more predictable pK parameters allowing better targeting of plasma exposure and reducing hepatic veno-occlusive disease (HVD) occurrence, related to blood over-exposure. **OBJECTIVES:** To estimate costs of IV versus Oral Bu-based BuCy2 conditioning in the UK NHS system. **METHODS:** A simulation based on the strong correlation established between Bu blood over-exposure and the occurrence of HVD (Kashyap A, BB&MT 2002) included costs of drugs and local HVD management. **RESULTS:** The cost of a full course of oral Bu (1 mg/kg × 16) and IV Bu (0.8 mg/kg × 16) is £116.5 and £3220 respectively. Patients receiving oral Bu have a greater risk of developing HVD (20% vs. 5%, p = 0.03, Kashyap 2002). In Southampton the cost of managing HVD was estimated to be £11,050 per case (based on additional in-patient stay, drug treatment and medical management), generating an extra cost per patient of £2210 (£11,050 × 0.2) and £552.50 (£11,050 × 0.05) respectively. Therefore the total estimated cost using oral Bu compared to IV Bu in BuCy2 is £2326.50 and £3772.50 respectively. From an initial cost ratio of 1/27.6 in favor of oral Bu (drug costs only) the ratio dropped to 1/1.6 when the cost of HVD management was included in the simulation. **CONCLUSIONS:** The additional cost of £1446 with IV Bu is relatively modest in the context of better targeting of the therapeutic window, potential superior transplant outcome, and better time allocation of health care personnel (easier administration, patient monitoring and treatment compliance) seen with Busilvex®.