

Discussion: The positive long-term consequences of an early start of prophylaxis were illustrated by the lower costs in adult years for the Swedish 1960s cohort that also reported no haemophilia-related sick leave or early retirement. The difference in the level of cost in Norway and Sweden was attributed to the higher use of factor-concentrate in the Swedish prophylaxis regime compared to the Norwegian on-demand regime.

06 PO 07

Barriers to the provision of haemophilia care in the Limpopo Province of South Africa

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The Limpopo Province of South Africa is home to almost 6 million South Africans and is a historically underdeveloped area with a large rural population and high levels of unemployment. Since 1994 the new South African government has actively promoted a more equitable distribution of resources including health expenditure. Specialist health services were developed at the major urban hospital in the Province and in 2000 a haemophilia treatment centre was established. The centre has access to a full range of support services and funding for factor concentrates and other medication has been unrestricted. As at the end of 2003 the centre had 52 registered clients compared with an expected 300 in the Province. The ratio of clients under 13 years to adults is 1:0.3 and the per capita use of factor is 0.15 units. These figures are disappointing and compare unfavourably with other similarly financially resourced provinces in the country. This is a descriptive study of informal and formal patient interviews conducted at the haemophilia centre as well as of reports received from district hospitals. A simple model is used to identify barriers in access to haemophilia care. Possible strategies to improve access to care for all people with haemophilia are discussed.

06 PO 08

Cost-effectiveness of FEIBA vs. NovoSeven as initial therapy for the treatment of mild-to-moderate bleeds in haemophilia patients with inhibitors

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Background: FEIBA and NovoSeven are both indicated as initial therapy for mild-to-moderate bleeds in hemophilia patients with inhibitors. Their comparative cost-effectiveness is not well understood.

Methods: We used a decision-analytic model to estimate outcomes and costs of initial treatment of mild-to-moderate bleeds in patients with hemophilia and inhibitors (mean weight = 70 kg) with either FEIBA (75 U/kg/12 h) or NovoSeven (90 µg/kg/2 h). Bleeds were assumed to resolve after the first, second, or third infusions, or not to resolve with initial therapy. The efficacy of FEIBA and NovoSeven was estimated using data from published studies; per these studies, all bleeds that resolved with NovoSeven were assumed to receive an additional 'maintenance dose' (90 µg/kg). Second-line therapy for unresolved bleeds was not considered in base case analyses due to significant variation in clinical practice. The costs included in the base case analyses were those of FEIBA and NovoSeven. **Results:** The percentage of bleeds controlled with the first, second, and third FEIBA infusions was 52.0, 13.0 and 13.0%, respectively; corresponding percentages for NovoSeven infusions were 25.6, 21.2 and 40.5%. The average estimated cost per treated bleed was \$15,033 for FEIBA and \$30,639 for NovoSeven. The average cost per controlled bleed was \$19,294 for FEIBA and \$35,091 for NovoSeven. Findings were robust in sensitivity analyses.

Conclusions: Average costs per treated and controlled bleed with FEIBA are approximately one-half those with NovoSeven. FEIBA therefore may represent a more cost-effective treatment for mild-to-moderate bleeds in hemophilia patients with inhibitors.

06 PO 09

The plight of haemophiliacs in Cambodia

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Governments and aid agencies spend millions of dollars on research and treatment for infectious diseases such as TB, malaria and HIV. Research on genetic disorders also receive considerable funding in the developed West; not so in developing countries. Two factors inform this situation. The first is financial - where money is scarce the pressing needs of the many necessarily outweigh the needs of a few. The second is that the so-called few are routinely underestimated as the diagnosis of genetic disorders is ill-informed. At Angkor Hospital for Children, the introduction of coagulation testing has made it possible to increase awareness of hereditary coagulation factor disorders. Prior to this the Cambodian doctors were unaware, even when faced by the obvious, that hereditary factor deficiencies existed among their patients. The diagnosis, only a month after these tests were introduced, of the first haemophilia patient, and six subsequent cases in the next 2 years - three of whom had incidental findings of a haemoglobinopathy - has had a positive impact on the Cambodian doctors. By presenting these seven haemophilia case studies - four of which were previously misdiagnosed as TB of the joints, septic arthritis or osteomyelitis, in spite of obvious evidence of bleeding problems - the aim of this talk is to issue a challenge. This challenge invites specialist knowledge, innovations and practical suggestions as to how a much needed haemophilia education centre for Cambodian healthworkers could be established with associated spin-offs for those working in developing countries. To make this activity more than a hypothetical task, pertinent features of each of the seven cases will be discussed as a way of giving insight into the reasons for misdiagnosis, the problems of making cryoprecipitate vs. the level of demand for supplies of FFP from patients with acquired coagulopathies. Further, these cases show the inevitable dilemma of managing and treating in a resource-poor setting where the urgent needs of patients with haemorrhagic dengue fever, for example, are pitted against the needs of a young haemophilic with acute haemarthrosis, who without adequate treatment, can only be sent home to a slow, and probable agonizing death.

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06 PO 10

Cost of care and quality of life in hemophiliacs developing inhibitors against products of coagulation: The Cocis Study

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Background: Bleeding and its complications cause pain, disability and lead to impairment of hemophiliacs' quality of life (QoL). Problems become extreme when hemophiliacs develop antibodies compromising effectiveness of treatment: modern strategies are likely to have improved this situation, with a sensitive increase of health care cost.

Aim: To estimate the economic burden and QoL of hemophiliacs with inhibitors.

Methods: We conducted a longitudinal, prospective, multicentre Cost of Care Inhibitors Study (COCIS), observing inhibitory patients for 18 months. Costs were evaluated from the point of view of the Italian National Health Service (NHS). QoL was measured by means of EQ-5D and SF-36 questionnaires.

Results: Fifty-two hemophiliacs (median age: 34.8; range:15-64) were enrolled, almost all high responders. The orthopedic functioning was impaired in 98% of patients, 81% had at least one bleeding event, with an average of 0.60/patient/month. Eleven surgical procedures (six for joint replacements), 30 hospitalizations, 712 outpatient visits and 702 physiotherapy sessions occurred. Cost of care to the NHS was 18,000 €/patient/month, 99% due to treatment products. Recombinant activated factor VII represented 50% of total medical cost, mostly used for surgery. Patients showed an important impairment of physical health perception, while psychological QoL was comparable to that of the Italian general male population. QoL of inhibitor patients was not different from that in other moderate and severe hemophiliacs as reported by other authors.

Conclusions: Hemophilia with inhibitors represents an example of a rare disease that requires high cost. Effective and expensive care of hemophilia with inhibitors provides a satisfactory QoL.

06 PO 11

Development of the haemophilia age-group specific quality of life questionnaire

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The aim is to develop a Quality of Life (QoL) questionnaire specific to patients suffering from haemophilia, adapted to age-groups (from young children to adults). The development of the questionnaire went through a rigorous process: literature review, qualitative interviews with clinical experts, adults suffering from haemophilia and parents of haemophilia children. An 'adult' version (18-35 years old) was first developed with 89 items covering seven domains. Three other versions were developed from this adult version: an 'adolescent' version (12-17 years old), a 'child' version (8-11 years old) and a 'parents of children' version (3-11 years old). These versions include 77, 22 and 45 items respectively and cover the same domains, except for the 'professional life' domain. This questionnaire was then administered in a cross-sectional pilot study run in France, in order to assess the acceptability among haemophilia patients aged 3-35 years. In total, 23 adults, 17 adolescents and 11 parents completed the QoL questionnaire. The questionnaire acceptability was revealed to be good among all ages. The next step is the validation of this questionnaire in order to have a QoL measurement tool adapted to haemophilia patients of any age.

06 PO 12

Health Economics of Haemophilia Care in Europe (ESCHQoL-Study) costs of haemophilia care

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Background: Treatment of patients with haemophilia requires a large amount of economical and human resources. Despite a high level of biotechnological research in developed countries, health economic data are scarce.

Objective: Identifying improvement opportunities by documenting the current state of health economic research and determining the cost of hemophilia care in Europe.

Methods: For structured literature review, publications concerning health economics in haemophilia from 1/1994 to 3/2004 in the databases of Medline, Embase, Econlit and EBM Reviews of the Cochrane Society were searched. The keywords were: cost, cost-benefit, cost-utility, cost-effectiveness and haemophilia. The next step includes prospective bottom-up data collection in haemophilia care centres in 22 European countries with medical documentation and patient diaries. These questionnaires were developed, translated in the project languages and feasibility tested in a pilot testing.

Results: A total of 268 publications concerning health economics were identified. Incremental cost-effectiveness ratios vary from \$900 (UK) to \$1200 (Germany) per avoided joint bleed in prophylactically treated patients. Cost per quality adjusted life year (QALY) ranges from £55,922 to £244,409 in inhibitor patients treated within different immune tolerance protocols (UK). Utilities vary from 0.85 in mild/moderate haemophilia to 0.59 in patients with on demand treatment. In patients with inhibitors the monthly cost of care amounted to €18,000 with rFVIIa representing 50% of the expenses (Italy).

Conclusion: Since differences in results of health economic studies reflect variable methodology, most of the existing studies are not comparable. The ESCHQoL study will be the first study providing comparable results with a consistent method.

1. We used a decision-analytic model to estimate outcomes and costs of initial treatment of mild-to-mo...

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2. The efficacy of FEIBA and NovoSeven was estimated using data from published studies; per these studi...

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