Record Status
This is a critical abstract of an economic evaluation that meets the criteria for inclusion on NHS EED. Each abstract contains a brief summary of the methods, the results and conclusions followed by a detailed critical assessment on the reliability of the study and the conclusions drawn.

Health technology
A modified prophylactic regimen versus on-demand therapy in the treatment of patients with haemophilia.

Type of intervention
Secondary prevention.

Economic study type
Cost-effectiveness analysis and cost-utility analysis.

Study population
Patients with haemophilia.

Setting
Medical clinic. The economic study was carried out in Munich, Germany.

Dates to which data relate
The effectiveness analysis and resources used were taken from 1995. The price year was not stated.

Source of effectiveness data
The effectiveness data were derived from a single study.

Link between effectiveness and cost data
The costing was undertaken retrospectively on the same patient sample as that used in the effectiveness study.

Study sample
Power calculations were not used to determine the sample size. Overall, 50 patients were included in the sample; 39 patients were treated on an on-demand therapy basis and 11 patients were treated on a modified prophylactic regimen basis. The age range was from 18 to 60 years with a mean of 35.14 years.

Study design
This was a cohort study, carried out in a single centre. The follow-up period was 6 months. No loss to follow-up was stated.

Analysis of effectiveness
It was not explicitly stated whether the effectiveness analysis was based on intention to treat or treatment completers only. Quality of life and joint bleeding were used as primary health outcomes. Quality of life (physical function, physical role function, emotional role function, physiological well-being, pain, vitality, social function and general health) was measured using the Short Form 36 (SF-36). The quality of life of haemophilic patients was compared with a normal population and also with patients suffering from hypertension, peripheral arterial diseases, chronic back pain or patients who received kidney transplantation. The health state classification system assessing four domains including physical function, role function, social emotional function and health problems was used to obtain the utility values for all patients. Joint bleeding was measured by the bleeding history, a physical examination score and an X-ray evaluation score. The comparability of the groups was not stated.

**Effectiveness results**

Quality of life (SF-36): patients treated on demand and on prophylaxis versus normal population showed significant differences on the average values in the assessment of their limitation of physical activities, limiting pain and general health. The comparison of the quality of life of haemophilic patients with patients suffering from hypertension, peripheral arterial diseases, chronic back pain or patients who received kidney transplantation showed differences in physical activities, physical role function, vitality and social function. The average level of joint bleeds was 9.84 (median, 6) per patient across all patients whilst the level of joint bleeds in the on-demand group (10.74) was greater than in the prophylactic group (6.64). The difference was not statistically significant. The on-demand group had a mean utility value of 0.59 (median:0.53; range: 0.28 - 0.87) versus 0.6 (median:0.53; range: 0.43 - 0.87).

**Clinical conclusions**

The study revealed no statistically significant differences between the types of substitution chosen in terms of health outcomes adopted.

**Measure of benefits used in the economic analysis**

The benefit measure adopted in the economic study was joint bleeds.

**Direct costs**

Quantities were not reported separately from the costs. Cost items were reported separately. Direct costs included outpatient visits, admittance to wards and factor VIII or IX clotting factor. Charge data (as a substitute for true costs) were taken from tariff lists and statistical sources. The cost analysis was performed from the perspective of a third-party payer (the German statutory sick funds). The date of the price data was not specified.

**Indirect Costs**

Quantities and costs were analysed separately. Work force participation, actual earnings and the number of days off work were measured. The method used was the human capital approach. The date of the price data was not specified.

**Currency**

German marks (DM).

**Sensitivity analysis**

No sensitivity analysis was carried out.

**Estimated benefits used in the economic analysis**

The average level of joint bleeds was 9.84 per patient across all patients whilst the level of joint bleeds in the on-demand group (10.74) was greater than in the prophylactic group (6.64). The difference was not statistically significant. The duration of the observation was 6 months.
Cost results
The total cost per patient was DM24,601 in all patients, DM17,253 for those in an on-demand group and DM28,245 in the modified prophylactic group. The duration of the observation was 6 months.

Synthesis of costs and benefits
The cost per avoided joint bleed in a patient in the prophylactic regimen (DM4,228) was greater than in the on-demand regimen (DM1,680). The incremental cost-effectiveness of the prophylactic treatment was DM2,536.

Authors' conclusions
The results suggest that the costs of haemophilia care are not only related to the high costs of clotting factors but also to treatment modality. Patients receiving prophylactic treatment with clotting factor require less additional health care resources, mainly due to the reduction of joint bleeds.

CRD COMMENTARY - Selection of comparators
No justification was given for the choice of the comparator.

Validity of estimate of measure of benefit
The internal validity of the estimates of the benefit measure may be weakened by the lack of a randomised controlled design and the sample size.

Validity of estimate of costs
Quantities were not fully reported separately from the costs and adequate details of the methods of cost estimation were not given.

Other issues
In view of the lack of randomisation, sensitivity analysis, and statistical analysis of the costs, the results need to be treated with some caution. The issue of generalisability to other settings/countries was not addressed.

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Partially funded by a grant from the Allgemeine Ortskrankenkasse, Munich (Mr J Fahn).

Bibliographic details

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Subject indexing assigned by CRD

MeSH
Cost-Benefit Analysis; Factor IX /therapeutic use /economics; Factor VIII /therapeutic use /economics; Germany; Health Care Costs; Hemarthrosis /prevention & control; Hemophilia A /prevention & control /drug therapy; Quality of Life; Recombinant Proteins /therapeutic use; Treatment Outcome

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