Surveillance of colony-stimulating factor use in US academic health centers

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
Colony-stimulating factor (CSF) use.

Type of intervention
Treatment, and primary prevention.

Economic study type
Cost-effectiveness analysis.

Study population
The study population were aged 1 to 93 years: 52% were male and 48% were female. 83% received CSF as inpatients, 13% as outpatients and 4% as both.

Setting
Hospital institutions (academic health centres). The economic study was carried out in the USA.

Dates to which data relate
All data were collected between September 1 and October 15 1993 (a 45 day period).

Source of effectiveness data
Evidence for final outcomes was based on a single study.

Link between effectiveness and cost data
Costing was carried out on the sample patient sample and at the same time as the effectiveness data.

Study sample
30 institutions were asked to enroll sequential patients meeting certain data criteria. Sequential patient selection was not always practicable in all institutions. 581 patients were enrolled to the study, 16 were excluded through missing data, so the sample size was 565 patients. This sample was appropriate for the clinical study question. The authors highlighted that the sample could easily be compared against University Hospital Consortium consensus-derived guidelines. Sample size was not determined by power calculation.

Study design
This was a case-series study, carried out at multi-centres.
Analysis of effectiveness
Analysis was based on treatment completers only. The primary health outcome used was the appropriateness of the use of CSF as indicated by the guidelines. This was classified as either appropriate, promising but unproven or inappropriate. Adverse effects were also recorded. The characteristics of the sample may be broadly comparable with the population receiving CSF. The authors highlighted some limitations such as the lack of outpatient data, and also that patients who should have received CSF but did not, were excluded from the study.

Effectiveness results
71% of CSF use was deemed appropriate, 7% was inappropriate and 22% was unproven. No confidence intervals were presented around these results.

Clinical conclusions
A large proportion of CSF use was for unproven or inappropriate uses (29%). The use of CSFs for indications other than those considered appropriate by the guidelines should be discouraged until further research is carried out.

Measure of benefits used in the economic analysis
The appropriateness of the use of CSFs was the main outcome used in the economic analysis. The indication recorded for each of the 565 patients receiving CSFs was compared by the authors with a list of categorised indications contained in the consensus-derived guidelines. This was carried out in 1993.

Direct costs
Costs were not discounted in this study. Total microgram amounts of CSFs were quantified based on actual data and then costed using an estimated cost per microgram. Costs were calculated from the hospital perspective, although pharmacy preparation and nursing administration costs were not included. These data are from 1993.

Currency
US dollars ($).

Sensitivity analysis
No sensitivity analysis was performed in this study.

Estimated benefits used in the economic analysis
It was found that 29% of CSF use was not used for appropriate indications. Side-effects were considered but not as part of the economic analysis.

Cost results
The total drug cost for CSFs in the 565 patients studied was $791,000, which represented $1,400 per patient. The total cost of CSFs used was:

for appropriate indications: $561,018

for promising but unproven indications: $124,241

for other unproven indications $46,292

and for inappropriate indications: $59,286.
Synthesis of costs and benefits
Costs and benefits were only combined by summing costs observed for each category of appropriateness/proven effectiveness. Thus an amount spent effectively can be compared with that spent on indication of questionable effectiveness.

Authors' conclusions
Substantial costs were incurred currently for CSF therapy without adequate literature support.

CRD Commentary
The authors justified setting up the study through evidence which suggested that CSFs were currently being used for many indications. The study was needed to attempt to quantify the extent to which CSFs were being prescribed for indications for which their effectiveness was unproven. The study design could at best only serve as a snapshot stocktake of what was occurring: it was observational. It did not attempt to prove which indications were most effectively treated with CSFs. Adverse effects such as the occurrence of skeletal pain, nausea, headache and fever were reported in the study but the actual outcome for each patient was not followed up at all. Because guidelines say, for example, that CSF use for AIDS drug-induced neutropenia is appropriate the study simply categorised it thus, and inferred a successful outcome. Whether the drug actually worked in each case was ignored and the authors did raise this issue in their discussion. More details about the effectiveness of CSF would have strengthened the study.

The economic study was simply a costing exercise once cases had been categorised according to the guidelines based on effectiveness criteria. The perspective used for costing was also very narrow, simply costing the micrograms of CSF used. Other costs associated with administering and preparing the drug were excluded as were the costs of side effects. This study served to catalogue and cost the current extent of effective use of CSF. It does not provide information on cost-effectiveness, but implies that cases using CSF for indications not on the appropriate list are a waste of resources. This inference is not based on any evidence, but rather the lack of it, to prove effectiveness. However, the study did fulfill its limited objectives. The authors did not claim this was a comprehensive study and they highlighted the need for further evaluation of CSFs. Economic issues were not properly dealt with in this study.

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