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ECONOMIC ASPECTS OF HAEMOPHILIA

1. At our meeting to talk about the above topic you suggested that it would be useful to you if I were to set out the sort of questions to which economists, and particularly the Economic Adviser's Office, might address themselves.

2. It emerged very clearly from our conversation that there were major uncertainties concerning the cost of producing anti-haemophilia agents. The cost of commercial Factor VIII concentrates are obviously known - the price of 10p per clotting factor unit is normally quoted - but beyond this little appears to be certain.

3. Carter, Forbes et al. estimated the cost of preparing cryoprecipitate in the West of Scotland at £1.69 per pack or just over 2p per unit. This is, as far as we can locate, the sole published estimate. There are no published estimates of the cost of preparing non-commercial Factor VIII concentrates. The cost of having Factor VIII concentrate produced for the NHS by a commercial contractor might be expected to be less than 10p a unit.

4. In order to compare the costs of various products it is necessary to compare like with like. Thus there is little point in comparing the cost of producing cryoprecipitate with the cost of purchasing a Factor VIII concentrate since the processes of reconstitution and administration of these two products are quite different and much more costly in the case of cryoprecipitate. The comparison in the BMJ article by Carter et al. of cryoprecipitate at about 2p per unit with a concentrate price of 10p is therefore misleading.

5. It would represent an improvement to compare the cost of delivering a unit of AHF product to a patient. This would include the cost of the product and the cost of the staff, materials and equipment to administer the product. The difficulty with this approach is that the purpose of administering AHF products is to raise the level of Factor VIII activity in the patient. Products differ in their yield of Factor VIII, in the extent to which this is recovered in the patient and in the certainty with which this effect is achieved. These differences need to be allowed for in order to produce a comparable unit for costing purposes. Indeed even a formulation in these terms is incomplete; for the production of Factor VIII can be combined to a greater or lesser extent with the production of other blood products. The incorporation of these incremental costs and benefits into the analysis adds another dimension of complexity.

6. It could be maintained that the relevant unit for costing purposes is not the "input" (the unit of product administered, for example) but the "output" - the bleeding episode controlled. This point of view would lead us to compare the average costs of bringing a bleeding episode under control using the various products. However even the bleeding episode, while it has advantages over the input-orientated measures, is not completely satisfactory as a unit for costing purposes. For the purpose of treatment is not to control bleeding episodes but to relieve pain, reduce the possibility of crippling injuries, iatrogenic diseases or death and enable patients to live as normal a life as possible. Ideally one would wish to know how the cost of the products compared in achieving these aims. In practice one must admit that any comprehensive and definitive analysis of the cost of treating haemophilia is beyond our present knowledge despite the interesting attempt by Carter and her colleagues. Nevertheless there would appear to be a number of studies which could reasonably be carried out and

which would be useful even though they might fall short of this sort of comprehensive analysis.

7. The first study would examine the costs of producing and administering cryoprecipitate in a hospital setting. Biggs et al. found large differences in the assayed Factor VIII values of multi-donor samples of cryoprecipitate between five Haemophilia Centres and even for samples from the same centre. Apart from differences in Factor VIII concentrations between donors the differences appear to be due to different methods of manufacture and, more importantly, different methods of making up cryoprecipitate for administration to patients. In particular it would seem that if the pooling of the cryoprecipitate were carried out by the Blood Transfusion Service rather than at the hospital the level of Factor VIII per unit would be higher. Given that hospitals do not pay for the cryoprecipitate, however, the Blood Transfusion Service has an incentive to leave the pooling, a laborious process, to the hospital.

8. The questions which the study would ask are:

a) How do the cost of producing and administering cryoprecipitate vary from centre to centre? Are there economies of scale, for example?

b) Is there any relationship between the level of Factor VIII activity in samples of cryoprecipitate and the costs of production and preparation?

c) What is the most cost-effective method of producing and administering cryoprecipitate?

d) What does it cost to produce other products in association with cryoprecipitate?

9. The advantage of beginning with cryoprecipitate is that it should be somewhat easier to obtain data than for concentrate products. Even here, however, it may prove difficult to answer questions about Factor VIII activity without obtaining the active support of the centres involved, nor is it clear whether the expected level of Factor VIII activity (both mean and variance) affects the amount of cryoprecipitate given.

10. If the cryoprecipitate study were successful the logical next step would be a corresponding study of concentrate production and delivery, focussing on the cost to the UK public sector of producing the concentrate equivalent (or some fraction thereof) of current UK cryoprecipitate output, how such costs might be affected by the scale of production, the price of greater purification and the costs of extracting other blood by-products. It seems unlikely that detailed information of this sort could be obtained from commercial sources. The possibility of obtaining data from the Lister Institute, Edinburgh and Oxford would need to be investigated.

11. It is envisaged that the EAO could carry out studies of the cost of cryoprecipitate and concentrates. Wider studies on the costs and benefits of alternative treatment regimens for haemophilia would be likely to require outside research resources. If such wider research studies seemed appropriate the EAO would be happy to collaborate in formulating the economic aspects of a research study of this kind and in locating economists who would be interested in such work.

References

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