Recombinant Factor VIII for Treatment of Haemophilia A

1 Context

Directors of Public Health in Wales were asked by Chief Executives to review the priority for funding Recombinant Factor VIII.

Haemophilia centres have indicated that the cost of this new drug product could not be managed within existing contractual arrangements and that its funding as a development was a priority for health authorities in the 1997/98 contracting round.

Haemophilia A is a bleeding disorder whereby patients lack the clotting factor VIII. The lack of factor VIII causes persistent bleeding which leads to pain and joint deformities if not treated quickly. Haemophilia B is a much rarer disease with deficiency of clotting factor IX. This paper deals solely with the treatment of Haemophilia A, although the same principles apply to Haemophilia b.

2 Directors of Public Health considered the following issues:

- past history of care to this patient group
- effectiveness, safety and risk
- ethical and legal considerations
- equity within the UK NHS
- economic factors

2.1 Past history:

In the last 15 years this group has been identified to have been infected by at least three major blood borne viral infections (HIV, hepatitis B, hepatitis C). Though safety measures have improved, it cannot be absolutely guaranteed.

2.2 Effectiveness, Safety and Risk:

The issue of the risk of transmission of infection, not effectiveness of the treatment. The risk of future transmission of unknown agent cannot be quantified. The risk is therefore assessed by qualitative components such as avoidability, justification, acceptability and seriousness. It is the judgement of the DPHs that the purchasing of Recombinant Factor VIII improves the qualitative risk on all these parameters.

2.3 Ethical and legal:

The Haemophilia Centres suggested that use of Factor VIII could be phased, providing it initially to newly diagnosed cases, then established cases who had received other products and then those who had been infected by prior viral infection, eg persons who were HIV positive. It was considered not ethical to discriminate on grounds of age or infected status. Persons who are HIV positive or have AIDS are likely to suffer more adverse consequences of a viral infection.

2.4 Equity within the UK NHS:

In Scotland, funds have been made available by the Scottish Office. In Wales, cases in North Wales are receiving treatment from centres in northern England. Equity is assured through a consistent policy in Wales.

2.5 Economic factors:

New treatments tend to have high costs in early years as pharmaceutical companies try to recover development costs prior to the loss of patent.

Table 1

Estimated number of Haemophilia A

Estimated number of covers by Gwent Health Authority who attend tertiary units UHW

Severe	5	(2 children)
Moderate	8	(3 children)
Mild	36	(9 children)
Total	47	(14 children)

Table 2

Estimated Costs to Tertiary Centre

Gwent	Units No	Present Cost £	Recombinant Cost £	Additional Cost £
Children not infected with HIV or HCV	377,000	75,400	177,190	101,790
Adults not infected with HIV or HCV	80,000	16,000	37,600	21,600
Patients infected with HCV but not HIV	183,000	36,600	86,010	49,410
Patients with HIV	183,000	36,600	86,010	49,410
Total	**904,000**	**194,000**	**424,800**	**230,800**

3 Options

These options were considered and set aside:

3.1 The option to Health Authorities to make a stand on the issue of effectiveness. The issue is safety and it would be perceived that we were risking the lives of a patient group who had already suffered three confirmed outbreaks of disease from blood products.

3.2 Various tactical options of waiting until the price fell. This could be tenable had it been done on a UK wide basis. However the action of the Scottish Office undermines this approach.

3.3 Phased introduction by groups identified by age and prior viral infection. This would inevitably lead to a legal challenge and cannot be justified morally or ethically. Already ministers, Welsh Office and Health Authorities are receiving petitions from the various patient groups.

Recommendation

In the circumstances and taking into account relevant factors, it is recommended that the health authority should support the option to fund the conversion of all patients to Recombinant Factor VIII where this is considered appropriate by the doctor treating the patient.