Lady Hayman

From: Mike McGovern

Date: 30 November 1998

Fiona Anderson PS/SoS
Neil Townley PS/M(PH)
Sarah Crossland

PS/H.Hayman

Kirsty Jarvie PS/CE
Sue Shepherd PS/CMO
Joe McCrea Sp/Av
Simon Stevens Sp/Av

SoS: to a lay person, haemophilia B sounds much like it's more common relation. Plasma-derived factor IX is in short supply (with a poor reputation) so recombinant factor IX is going to be in demand and expensive. However we need to treat the same way as those with Haem A - but, given the law in year costs, get HAs to bear those costs.

Graham Winyard DCMO
David Jefferys MCA
David Hewlett
Frances Rotblat MCA
Keith Paley
Robert Newton
Charles Lister
Gwen Skinner
Debbie Barnes

GRO-C

GRO-C

Haemophilia B (Factor IX deficiency), Factor IX supplies and Recombinant Factor IX (BeneFIX from Baxter)

The issue

In January 1999 recombinant Factor IX will become available on the UK market for the treatment of haemophiliacs with factor IX deficiency -Haemophilia B or Christmas Disease. Like recombinant Factor VIII for use in Haemophilia A this is about twice as expensive as plasma derived products and will add to local cost pressures. An added complication is that plasma derived Factor IX is now in short supply in the UK as a major US supplier (Alpha) has recently been closed by the Food and Drug Administration (FDA) because of serious defects in the manufacturing processes. There is a good supply of recombinant Factor IX and this is likely to drive changes in prescribing practice.

Background

Earlier this year Secretary of State indicated that all children under the age of 16 and new patients with Haemophilia A should receive recombinant factor VIII. This decision was based on the fact that these patients would have been less likely to have been exposed to infectious agents than older haemophiliacs and would consequently benefit most from recombinant product. The decision was influenced by the concern of the families of these children about the theoretical risk of nvCJD from plasma derived products and representations from the Haemophilia Society. It

was agreed that funding for unplanned recombinant Factor VIII treatment in these patients in 1998/9 would be provided centrally but that from 1999 onwards this would need to come from central allocations. No specific decision was made about recombinant Factor IX, which was not available at that time.

Haemophilia B or Christmas Disease -Factor IX Deficiency

There is a total of about 6550 registered haemophiliacs in the UK of whom 1150 have haemophilia B. The effects of Haemophilia B are almost exactly the same as Haemophilia A. It is inherited through the female line on an X chromosome. As in Haemophilia A, women are carriers and may themselves in fact have low levels of Factor IX. Again as in Factor VIII deficiency in about 30 % of cases there is no family history and the condition appears sporadically. Factor IX is required to maintain the integrity of the blood clotting system and without it people get painful spontaneous bleeding into their joints which without treatment leads to crippling arthritis. In addition minor trauma and surgery can result in major episodes of bleeding which without treatment can lead to serious injury or death.

Plasma derived Factor IX

Current treatment for people with Haemophilia B is plasma derived Factor IX. There are several high quality products available from the UK fractionators -Bio Products Laboratory (BPL) in Elstree and the Protein Fractionation Centre (PFC) in Edinburgh. These are doubly virally inactivated and have an excellent safety record since the early 90s. However UK supplies of Factor IX have come under pressure by a recent ban and recall of Alpha products by the FDA. The Alpha product was widely used in the UK
-increasingly following concerns of patients and doctors about nvCJD. A large proportion of people with Haemophilia B were using it in preference to the BPL/PFC products. The move by BPL and PFC to producing non UK derived Factor IX has also compounded the supply problem which however has not resulted in patients going without treatment. From December/January the BPL and PFC products will be made from imported non UK plasma and the supply of plasma derived Factor IX products will become more comfortable.

Recombinant Factor IX -BeneFIX from Baxter

People with Haemophilia B and the professions have been expecting the marketing of recombinant Factor IX for over a year now. However licensing through the European Medicines Evaluation Agency was delayed for various reasons and a marketing authorisation will not be in place until January 1999. The manufacturer, Baxter, indicate that stocks are plentiful and there appear to be none of the supply problems associated with recombinant Factor VIII production.

The extra costs

The product is likely to cost about 70-100p/unit compared with 45p per unit of plasma derived factor IX. While there are about 1150 patients with Haemophilia B on the register only about 500 are in fact receiving treatment -those not being treated are likely to have mild disease requiring Factor IX only occasionally. Those treated require about 50,000 units a year. About 180 being treated are under 16 years of age. Likely extra costs per annum are depending on final unit costs:

All patients	*6 -14 million
Under 16	*2 -5 million

Costs in year for 1998/9 would be expected to be between *0.5 and *1.25 million for new patients and those under 16 years of age.

Representations

The Haemophilia Society and the UK Haemophilia Centre Directors Organisation have already written several times to Ministers and officials asking about the provision of recombinant Factor IX when this is licensed. They are especially concerned about provision for those with Haemophilia B under the age of 16 and new patients and that there is equity of treatment. Given the uncertainty about licensing we have given holding replies to the effect that policy for Haemophilia B would be seen in the context of Secretary of State's decision about Haemophilia A. We now need to reply to these representations definitively.

Policy and handling

There is every reason that policy for children and new patients with Haemophilia B should be exactly the same as that for those with Haemophilia A as there are no clinical differences between the two groups. In addition agreeing to those under 16 and new patients to be treated with recombinant Factor IX would take pressure of the currently low supply of plasma derived products. This would then become more available for older people with Haemophilia B who have been exposed to all the risks of plasma derived products for a longer period of time. This would help to keep policy in both groups of haemophiliacs aligned and avoid contingency treatment of older patients with recombinant Factor IX because of problems with the supply plasma derived Factor IX.

Advice

We recommend that recombinant Factor IX be used for new patients with Haemophilia B and those under 16 years of age as is the case for haemophilia A following Secretary of State's decision earlier this year. As treatment with recombinant Factor VIII was centrally funded for 1998/99 this should also apply to recombinant factor IX. However, given the much smaller sums involved (between *5k and 12.5k on average per Health Authority for the rest of the year) and the fact that no central provision has been made for recombinant factor IX the financial effects would be more easily and more appropriately managed if Health Authorities were asked to meet the costs from their existing allocations. For 1999/2000 Health Authorities have been asked to fund recombinant Factor VIII for new patients and those under 16 from their central allocations and this should apply to recombinant factor IX also.

Action

If you are content we would write to the Service in the above terms and indicate to the Haemophilia Society and the UK Haemophilia Centre Directors Organisation that the policy for treatment with recombinant Factor IX is the same as that for recombinant Factor VIII. This could also be publicised in CMO's proposed letter to

clinicians about treatment with UK and non UK derived blood products

We are happy to discuss.

Dr Mike McGovern

Health Services Directorate