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RECOMBINANT FACTOR VIII

1. The purpose of this submission is to seek Ministers' agreement to the arrangements proposed for the purchasing of recombinant Factor VIII for the treatment of haemophiliacs.

Timing

2. There is a meeting of the Haemophilia Directors with the Department on 6 June and - if possible - it would be helpful if we could let them know at the meeting that the necessary arrangements for the contracting and purchasing of recombinant Factor VIII will be put in place.

Background

3. Haemophilia is a rare bleeding disorder resulting from lack of either clotting factor VIII or IX in the blood. Affected patients bleed spontaneously (or following minor injury), usually into muscles or joints. Early and appropriate treatment is essential to avoid disability and prolonged hospitalisation. There are over 800 patients in Scotland suffering from congenital bleeding disorders, most of whom are haemophiliacs. Treatment is provided in 5 centres - Glasgow (2), Edinburgh, Aberdeen and Dundee. Medical management of haemophiliacs is complex due to the variable severity and life long nature of the condition.

4. The Department has been approached by the Scottish Haemophilia Directors seeking funding for the purchase of recombinant Factor VIII. At present Factor VIII concentrate - which is used to treat or prevent bleeding - is manufactured in Scotland from plasma collected from blood donors, and provided free of charge to the Haemophilia Centres by the Scottish National Blood Transfusion Service (SNBTS). Recombinant Factor VIII is a synthetic blood coagulant produced in the commercial sector. Several recombinant Factor VIII concentrates are now licensed in the UK and are being used in significant amounts in England, Wales and Northern Ireland. A few patients in Scotland are also receiving the

recombinant product, and Haemophilia Directors are keen to extend its use to avoid further episodes of virus transmission in the haemophiliac population.

5. Ministers will be aware of the problems which have arisen in relation to virus contamination of plasma derived products particularly HIV/AIDS, Hepatitis B (HBV) and Hepatitis C (HCV). Many haemophiliacs have already died as a result of being treated with plasma derived Factor VIII contaminated with HIV, and many more are known to be infected with HIV, HBV and HCV. While we can be confident that the plasma derived Factor VIII now in use is not contaminated with HIV, HBV or HCV, the possibility of contamination with other, as yet unidentified, viruses is impossible to discount. Recombinant Factor VIII is therefore perceived as a safer and more suitable product for many patients.

Funding

6. At present plasma derived Factor VIII concentrate is produced by the SNBTS at its Protein Fractionation Centre and supplied to the Haemophilia Centres across Scotland. The Haemophilia Directors estimate that 3.9m units of recombinant Factor VIII would be required in the first year to treat more vulnerable patients, such as children and those newly diagnosed as haemophiliac, with demand rising rapidly as more patients are provided with the product - eventually reaching the stage where virtually all haemophiliacs would be treated with recombinant Factor VIII. The cost of the recombinant product is likely to be in the range of 40p - 48p per unit. The costs in the first full year will probably be around £1.3m - £1.5m rising to around £4m - £5m by the time the near complete transition is made to recombinant Factor VIII, at current demand levels.

Proposed Arrangements

7. Following discussions with the Common Services Agency (CSA), including officials from the SNBTS, National Services Division (NSD) and Scottish Healthcare Supplies (SHS), it is proposed that the National Services Division set up central contracts for the provision of recombinant Factor VIII to the NHS Trusts in which the Haemophilia Centres are located. The Coagulation Factor Working Party, which is a group comprising Haemophilia Directors and SNBTS personnel, augmented by representatives from NSD, SHS and DoH will be responsible for managing and monitoring the new arrangements. This Working Group will advise Scottish Healthcare Supplies on the purchase and distribution of the product. The contract negotiated by SHS with the private sector manufacturers will be as flexible as possible and will not be locked into one supplier.

8. As the take-up of recombinant Factor VIII increases - initially focusing on young or new patients but in due course encompassing most if not all existing haemophiliacs - the demand for the SNBTS's plasma derived Factor VIII will decline. This was recognised by the SNBTS in its recent Strategic Review which acknowledged that the future focus for the SNBTS would be on meeting red cell demand. There will, nevertheless continue to be an ongoing demand for plasma based Factor VIII for some patients, at least in the interim. Furthermore, even if production of plasma derived Factor VIII were to be discontinued completely, savings of only around £1m per annum would be achieved by the SNBTS since the fixed costs at the Protein Fractionation Centre, necessary for production of other important blood products would remain. However, there was an underspend of £1m last year

in one of the NSD programmes which we have carried forward and earmarked for funding the new arrangements for the purchase of recombinant Factor VIII in the current financial year. While there will be a reducing demand over time for plasma derived Factor VIII concentrate and the SNBTS should be able to make a small contribution towards the future costs of recombinant Factor VIII the main funding ie £2m - £4m for recombinant Factor VIII over the next 2-3 years will have to come from the HCS budget. If Ministers agree to proceed it should be noted that this will be a pre-emption of PES.

Recommendation

9. There is no doubt that haemophiliacs have special health problems and their treatment and care has to be carefully managed. They are also a very powerful lobby group with considerable public sympathy for their plight and they have the ability to highlight very effectively issues affecting their treatment. The argument for moving to recombinant Factor VIII on safety grounds, given the experience with blood-borne viruses such as HIV, HBVand HCV, is compelling. A commitment by Ministers to provide central funding for the move from plasma derived Factor VIII to recombinant Factor VIII will be well received by haemophiliacs and their families and by the Haemophilia Directors in Scotland. Accordingly we recommend that Ministers should agree to the arrangements proposed in paragraphs 7 and 8 above.

10. If Ministers accept this recommendation we will inform the Haemophilia Directors and identify a suitable opportunity for some positive media publicity for the announcement.

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