Letters to the Editor

SUPPLY OF BLOOD-CLOTTING-FACTOR VIII FOR TREATMENT OF HÆMOPHILIA

SIR,—The treatment of hæmophilic patients involves the replacement in their blood of an essential substance which they lack. In this respect, the disease resembles diabetes or pernicious anæmia. Factor VIII to a hæmophilic patient is literally his expectation of life. Hæmophilia differs from diabetes or pernicious anæmia in that the missing factor VIII can only safely be provided from human blood. The hæmophilic patient is thus indebted to society and in turn is the responsibility of society in rather a special sense.

Without treatment, before the middle of this century, few patients reached adult life and those who did were helpless cripples. Over the past 12 years, blood products containing factor VIII have gradually increased in amount. In the early part of this time, medical attention was centred on the cure of life-endangering bleeding and on protection during essential major surgery. As more material became available, patients were treated for particularly dangerous muscle hæmatomas and hæmarthroses in the hope of reducing somewhat the severity of crippling and delaying the age of onset of deformity. The present, but still modest, objective is to treat all developing musculoskeletal bleeds as early as possible, hopefully to prevent the occurrence of severe deformity in all patients. This form of therapy is called " on demand " treatment. Very many of the patients treated on demand arrive at the hospital on crutches, in ambulances, and with painful swollen joints. Most such episodes of musculoskeletal hæmorrhage resolve with treatment, but there can be no doubt at all that in the long term these patients will have arthritic joints long before those in the normal population. An extension of on-demand hospital therapy to the home (home therapy) so that treatment is given by the patient to himself, by a relative, or by a general practitioner would undoubtedly reduce the damage and also the anxiety under which patients and their families now live. It should be noted that even home therapy is a modest objective when comparison is made with prophylaxis. In prophylaxis, treatment would be given to prevent the occurrence of bleeding altogether. Prophylaxis is, of course, the rule for patients with diabetes or pernicious anæmia; its application to hæmophilic patients would treble the present estimated requirements of factor VIII.

Those who treat hæmophilic patients in the United Kingdom have in the past of necessity tolerated the chronic undertreatment of their patients and have put much time and effort into spreading the inadequate amounts of therapeutic material thinly so that deprivation should be least damaging. Essential but non-urgent operations have been postponed and are still being postponed. Economy has also been achieved by calculating the dose for each lesion for every patient to give the absolute minimum dose. In addition, patients have not been put onto home therapy who would greatly benefit by this treatment. Even with dire economy, some centres have been hard pressed to maintain minimum treatment. For example, the treatment of the boys at the Lord Mayor Treloar College at Alton in recent years has been maintained against a background of begging and borrowing from other centres from one week to the next. Were the school not supplemented in this way, it is calculated that there would be a deficit of about 260,000 factor-VIII units annually. There is, in fact, evidence that 90% of hæmophilic patients in the United Kingdom receive less (and in some cases much less) than optimum treatment for their complaint. The consequences of this undertreatment include subjecting the patients to unnecessary, painful, and destructive bleeding into joints and muscles. Ancillary effects of undertreatment include loss of educational time and inability to hold continuous employment.

The question that arises is for how long should this shortage of factor VIII be considered to be a reasonable feature of hæmophilia treatment? Two things, in my view, make continued limitation both unnecessary and unethical. The first of these is the fact that three commercial companies are now licensed to sell good-quality human factor VIII in this country and they have between them amounts of material adequate to supplement the present provisions of the National Health Service. In fact, at the time of writing, one commercial firm has over 1,000,000 units of factor VIII awaiting use.

The second consideration which renders adequate provision of factor VIII both feasible and desirable is the fact that blood can now be collected in plastic containers, which makes it possible to use the red cells for patients who are anæmic and the plasma for patients who lack some plasma components. The blood donated in the United Kingdom is freely given by responsible citizens; the best use of this valuable resource clearly lies in the best use of all parts of the blood. With regard to the provision of factor VIII by the N.H.S., we can say with certainty that we have the skill, experience, and capacity in this country to provide factor VIII of very high quality in the amounts required.

Why, then, is there still a chronic shortage of factor VIII in the clinics where patients are treated? The reason is that factor VIII is expensive, whether bought commercially or made by the N.H.S. Over the country as a whole, a supply of commercial human factor VIII sufficient adequately to supplement that made at present by the N.H.S. would cost an annual £1–2 million. It is claimed that a sum of money of this order cannot be found from current allocations to the N.H.S. without reducing money spent on other necessities. To make increased amounts of factor VIII in the N.H.S. is also likely to be expensive since it would require substantial expenditure on organisation of blood-supplies, on staff, apparatus, and buildings for fractionation. Set against this financial argument, it must be remembered that poorly treated hæmophiliacs also cost a lot of money in their role as hospital inpatients and in receipt of social-security benefits. But of course the financial argument takes no account of the misery and anxiety attached to frequent painful episodes of bleeding and inability to hold a normal place in school and society. In the long run it will probably be found cheaper to pay for these patients' treatment rather than to pay for the inevitable consequences of undertreatment.

When, as a direct outcome of years of research, lifesaving therapeutic materials suddenly become available to a population of patients previously chronically undertreated, there surely should be some means of assimilating this welcome advance, otherwise it is stupid to undertake the research in the first place. How this should be achieved is an administrative and political problem rather than a medical one. Perhaps there should be a special fund in the N.H.S. set aside every year for the practical implementation of research discoveries. Perhaps an organisation should be set up to collect money on a charitable basis to supplement the N.H.S. funds available for the introduction of new treatments. Whatever solutions there may be for problems of this sort in general, some immediate solution should be found for the ridiculous impasse of large available stocks of therapeutic materials locked up in stores because no-one will buy them and, on the other hand, patients in dire need of this same material.

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