For Haem Society Newsletter

APPENDIX C1

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1991 UPDATE

This being the New Year, I thought that you would like to know of some of the developments that are taking place towards the better treatment, and eventual cure, of haemophilia.

Firstly, treatment. The United Kingdom is now rapidly moving towards self-sufficiency in factor VIII, and the manufacturers are taking advantage of the most up- to- date technology to make their products both more appealing and safe to use. There will of course still be an option for doctors to prescribe products other than those prepared by the National Health Service. This choice will depend both on what they and particular patients consider to be best in a particular situation and, in our reorganising Health Service, on cost. All the blood products are becoming increasingly expensive and every four months the prices given to us by the different manufacturers are reviewed, the best buys being chosen from a list of products licensed for use within the United Kingdom.

Presently this list only contains products made from human plasma. However, the trials of the artificial recombinant factor VIII, which is made by genetic engineering, are producing very promising results, and it could be that within the next year or two we will be able to offer products made like this to our patients. Like everything else, cost will play a part because by the time the recombinant products come on the market, human products might be just as safe and cheaper.

Secondly, what about cure? Even ten years ago thoughts of curing haemophilia were purely speculative, but now there is real hope that a cure will be

available within the next decade. Even if we only convert people with severe haemophilia to a situation in which they are able to produce 5% or so of factor VIII or IX within their own bodies, this would mean that they were no longer subjected to spontaneous bleeding, only needing treatment in the event of a major accident or the need for surgery. So the first goal is to find a way of making the body produce some active clotting factor instead of none. The second goal is of course to achieve a complete cure, with the body making normal levels of all the clotting factors.

We are very near to achieving the first of these goals, and already have much of the technology needed to achieve the second. If we can persuade the body to accept transplanted factor VIII or IX producing cells from somebody without haemophilia, and ensure that these cells remain alive for long periods of time whilst they continue to secrete the clotting factor, we will at the very least be able to convert severe into mild haemophilia. Experiments are presently under way to find a coating for the very delicate liver cells (called hepatocytes) needed, so that they are not rejected by the recipient and can continue to survive in a healthy state. The hope is that a way can eventually be found of transplanting cells that reproduce themselves and thus continue the production of the necessary clotting factor throughout the life of the patient, a situation very similar to that presently possible in people undergoing bone marrow transplant for some forms of leukaemia.

The longer term goal of complete cure will be met by gene therapy. This technique will cure many other genetic disorders and the earliest experiments have already taken place in two human conditions (other than haemophilia) in America. In order to cure haemophilia genetic instructions to product normal factor VIII or IX would be inserted into the body cells of the person with

haemophilia. These new instructions would then provide the body with a blueprint for making normal clotting factor. This would cure the person concerned, but would still mean that haemophilia could be passed on to future generations because the implantation of instructions only into the body cells could not affect sperm or eggs. The eradication of the future threat of haemophilia to families could only come if what is called "germ line gene therapy" became acceptable, and that is many years away.

As to our future, those of us working in the Haemophilia Centre know only too well of the concerns of some of our patients that we might have been unable to keep up to date with the modern treatment of haemophilia in these difficult days. I hope that they will be assured that, despite everything that is happening to all of us, we continue to take an active part in the development of services to people with haemophilia and their families throughout the world. A vital part of this is the active participation of the Haemophilia Society and I was delighted recently to meet with the new Chairman, John Pepper, so that we could talk over our mutual concerns. We look forward to a continuing happy and fruitful association with all the Society members and their families in the North of England.

GRO-C

PETER JONES