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THE HAEMOPHILIA SOCIETY

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NEWS BULLETIN

February 1975

TREATMENT OF HAEMOPHILIA

The correspondence in "The Lancet" regarding the shortage of Factor VIII concentrates, which we printed in a previous News Bulletin, has lead to a great deal of coverage by the Press, Radio and Television.

An excellent series of articles by the medical correspondent of "The Yorkshire Post", Mr. Angus King, covering all aspects of the problem, appeared during January and these together with some of the resulting letters are reproduced in this Bulletin with grateful acknowledgments to the Editor.

Interviews with doctors, members of the Haemophilia Society, haemophiliacs and parents, were featured on Radio 4 "World atOne", Yorkshire TV "Calender", Radio 2 "Jimmy Young Show", Harlech TV "Report West", Radio Leeds "In Town Tonight", Radio Newcastle "First Thing", London BC "Newsday", and other programmes.

Articles in the "Sunday Times" on 16th February and 2nd March gave a comprehensive picture of the situation and aroused so much interest that some readers sent sums of money to purchase Factor VIII. It has long been a principle of the Haemophilia Society not to use funds for the provision of treatment within the National Health Service but in these new circumstances we have opened a "Home Treatment Fund" which will be used in accordance with recommendations made by our Medical Advisory Panel.

As suggested in our previous Bulletins a number of members have written to their M.P.'s; also questions have been asked in the House of Commons. Unfortunately, it appears from the answers given by the Minister responsible that it will prove extremely difficult for us to convince the Department of Health that apart from the moral right that haemophiliacs have to the most effective treatment available it must be an economic advantage to provide such treatment.

We had a similar experience when, over a period of nearly six years, we endeavoured to persuade the Department of Health of the medical, social and economic reasons for providing severely affected haemophiliacs with motor cars instead of invalid tricycles. We were in the end successful and we can only hope that those at present in authority will not maintain a similar dogmatic attitude but will accept that those now expressing their views are concerned only with the future lives of haemophiliacs.

We fully realise that there are economic problems in the National Health Service but it must be obvious that <u>now</u> is the time to provide the necessary money for the production or purchase of Factor VIII concentrates. The extension of home treatment programmes, at present restricted

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MEMBER OF THE WORLD FEDERATION OF HEMOPHILIA

by the lack of funds, will surely prevent demands, both now and in the future, upon the health and social services.

Perhaps the most important aspect is the well-being of the present generation of young haemophiliacs. We must do all we can to help them live normal lives, free from pain, and not allow them to grow up with crippled joints and limbs. Surely the amount of money required to achieve this cannot be too large a price to pay?

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We quote: "When as a result of years of research life--saving therapeutic materials suddenly become available to a population of patients, previously under-treated, there should surely be some means of assimilating this welcome advance. Otherwise it is stupid to undertake the research in the first place".

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RESEARCH APPEAL

Our North-West (Manchester) Group has just realised an aim which must be a landmark in the activities of our Groups and of the Society. Thanks to the hard work put in by individual members a target of £5,000 has been reached from which £3,500 has been donated to the Haemophilia Centre at Manchester Royal Infirmary for the purchase of equipment. The remaining £1,500 has been given to our Research Fund to be used to support the work of other Haemophilia Centres.

Conratulations and grateful thanks go to all concerned!

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ANNUAL SUBSCRIPTIONS

These are now due and we would be grateful if all members will forward their subscriptions together with the enclosed membership form duly completed.

It will be appreciated that our subscription is ridiculously low and does not even cover the postage costs of our News Bulletins. Some members do, of course, send a donation to help with our increasing costs. May we hope that many more will do likewise.

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ANNUAL GENERAL MEETING

Please note that the Society's Annual General Meeting will take place on the afternoon of Sunday, 27th April, 1975, at the Hospital for Sick Children, Great Ormond Street, London. A formal notice will be sent to all members in due course**********

NATIONAL SURVEY

All members will receive in the near future a questionnaire from which it is hoped to obtain information which will be invaluable in helping us to assess and pursue the needs of haemophiliacs in relation to present day circumstances and conditions.

The co-operation of all in completing and returning the questionnaire is earnestly sought.

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THE YORKSHIRE POST 17th Jan.1975

In the first of five articles on haemophilia, ANGUS KING, Medical Correspondent, writes about a costly drug which could revolutionise treatment of the disease.

Lives too expensive for Britain to save

MORE THAN 3,000 children and adults who need regular treatment to save them from severe pain and permanent crippling, cannot get the drugs they need — although there are ample supplies — because it is too expensive to be freely prescribed on the National Health Service.

Two firms producing and supplying the drug — freeze dried Factor VIII concentrate — originally brought large quantities of it into Britain from abroad at the request of the Department of Health.

Now they have been forced to ship out the same supplies to foreign markets because sales have not materialised and there was a danger that the drug would overrun its two-year shelf life. Stocks of the drug have been run down and the level of importation cut back — and sick children in Britain go short.

Now doctors in Britain are trying to put a stop to the scandal which threatens the lives of the country's 3,000 plus haemophiliacs. They are children and adults who suffer from a lifelong and incurable blood disease. Their blood lacks one of the ingredients essential for normal clotting.

The recent developments of Factor VIII concentrate was a major advance in the treatment of haemophiliacs, who in the past could look little further than hospitalisation and early death.

The concentrate has important advantages over the Factor VIII drug produced by the NHS — known as Cryoprecipitate and dubbed "Cryo" by younger patients.

Ironically, much of the successful research in the development of the concentrate, and its use in the treatment of patients, was first carried out in this country.

But chiefly through lack of resources, its large-scale production here has still not been achieved. Now Britain trails the modern world in the treatment of, and responsibility to, haemophilic patients.

The results of the British advances have been made internationally available — and they have been exploited by other countries. Foreign firms which seized the initiative, perfected commercial production and are now supplying many countries. Britain cannot even afford to buy their supplies.

Haemophiliacs are almost exclusively male. The substance missing from their blood can only be safely provided from human blood. The inability to manufacture it is inherited. The defect can be carried by females but it only shows up in males, with a few exceptions. It can be passed from mother to son or from a father through his daughter to the grandchildren.

The public picture of the disease is one of someone bleeding to death from a minor scratch within seconds, but haemophiliacs bleed at a rate no quicker than any ordinary person.

The true picture of their lives is far more harrowing. The chief problem is internal bleeding into joints and muscles — which resembles deep bruising or severe sprains — caused easily in haemophiliacs sometimes by the slightest knock, or even spontaneously.

These bleeds result rapidly in severe swelling, excruciating pain, and, without quick treatment with a Factor VIII drug, repeated bleeds lead to permanent damage to limb joints resulting in ultimate crippling. Time is a major factor in preserving normal life especially in children.

Apart from the psychological effects of the disease, which can be severe, haemophilia savagely disrupts normal life for families. It means caution, constant anxiety, and regular, urgent trips to hospital. It means interrupted family lives, lost working hours, sleepless nights, disrupted education, severe pain and suffering, and financial problems. Mild cases can lead normal lives.

Patients suffer much delay, having to attend a haemophilia centre for each treatment. The longer the delay the more severe the pain and the greater the risk of ultimate crippling. It can still mean weeks in hospital, or joints encased in plaster to help healing.

"Cryo" in this country is supplied free of charge to hospitals by the Blood Transfusion Services.

But the Transfusion Service, while praised for effort by doctors, cannot cope. Nationally it lacks the resources, staff and organisation to increase output of separate blood products. All too often "whole" blood is pumped into hospital patients who may require only one or two components from it.

So far as Cryo is concerned, supply cannot meet demand. There has never been enough to go round, and little is being done to reorganise the service.

"Factor VIII to a haemophilia patient is literally his expectation of life" says Dr. Rosemary Biggs, director of the world's largest haemophilia centre at Churchill Hospital, Oxford, where much of Britain's successful pioneering work was done.

The greatest collective advantage of Factor VIII concentrate over Cryo is that it is ideal for simple home treatment. That single fact means that the concentrate holds the power to revolutionise the lives of thousands of families in Britain.

Cryo, kept in a bulky plastic bag, requires deep freeze storage and quickly thaws on exposure to air. It cannot be re-frozen. Its preparation for use is described as messy.

The quickly effective concentrate is stored in an ordinary refrigerator as a white powder in a small rubber topped glass bottle.

For injection the powder is dissolved in a special solution contained in a similar bottle. It is easily prepared and injected. It can be kept outside the fridge for several hours.

In addition to the home treatment revolution, the concentrate could quickly supplement the shortfall of NHS Cryo thus ensuring adequate treatment. But the Department of Health has steadfastly refused to make available additional money for this purpose.

At first glance the cost of the concentrate seems high. Depending on the amount required, a single injection costs between £25 and £50 — a sum which the drug companies claim is far less than that charged for the same thing in Europe and not far short of the cost to the NHS of producing Cryo, taking into account the cost of out-patient treatment facilities for haemophiliacs.

Since blood for the commercial concentrate is obtained from paid donors and all blood products require the most stringent controls on donors and in processing, it is not difficult to assess why the price is high.

Sufficient concentrate for, say, an ulcer operation for a haemophiliac costs in the region of £3,000.

In the meantime, doctors and specialists concerned with haemophilia, unable to carry out their duties properly, have reached and gone beyond the point of inactive frustration, and are now engaged in presenting a concerted front to do battle with the Government to secure additional funds.

A consultant physician at one of Britain's largest haemophilia centres, says: "This is the great concentrate scandal — there is a mountain of it — unused. Patients, many of them kids, need the stuff in the hope of partly reducing the severity of crippling and to delay the onset of deformity.

"It is a simple story. We cannot produce enough of it and the Health Department will not give us money to buy it from the drugs firms. That amounts to calculated cruelty."

Specific questions arising from the Yorkshire Post investigation into the concentrate scandal were put to the Department of Health, in London. These were collectively answered by referring to two written answers given in the House of Commons in July by Dr. David Owen, a Minister of State for Health.

These blandly recognised the shortage, hoped supplies would be increased and pointed out that two firms had received licences to market commercial Factor VIII in Britain.

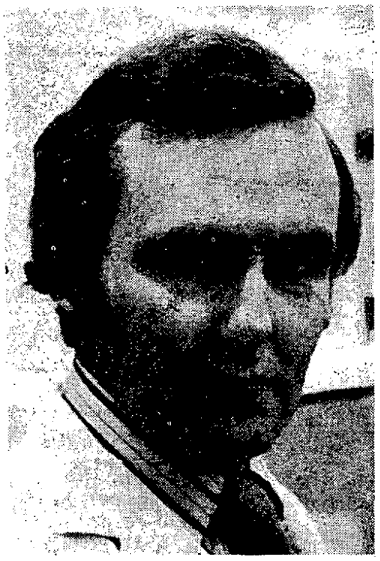
Dr. Owen said he recognised the desirability of home treatment but that progress in this direction would depend on the extent to which the drug could be produced within the NHS.

Any hopes for extra Government funds were shattered when he said: "Further increases will depend on the extent to which regional health authorities are able to expand facilities in transfusion centres for the production of plasma from which Factor VIII is derived."

Pressed for an answer as to why the Ministry had asked drug firms to make available large supplies of concentrate and then failed to allocate additional funds for purchase of the expensive drug, a spokesman for the Department said: "The Department takes the view that it is up to the various health authorities to decide what allocation they will make within the confines of their budget to which project or treatment."

Part two of a special investigation by Angus King, our Medical Correspondent

Angry doctors speak out



● **Dr. Peter Jones: "The majority of haemophilic patients only become physically handicapped because of inadequate treatment over the years."**

● **Dr. Liyanka Swinburne: "Until we have the money, the question of which patients most urgently require home treatment, is academic."**

FOR YEARS doctors in Britain have been forced to economise on supplies of an expensive drug — Factor VIII — by giving patients minimum doses. This means that children with haemophilia have been unnecessarily subjected to extra pain and suffering, and greater risk of permanent crippling.

Essential but non-urgent operations have been, and still are being, postponed and home treatment is withheld to make the drug supplies go as far as possible.

Even with dire economy some centres have been hard pressed to maintain minimum treatment. At the Lord Mayor Treloar College, at Alton, Hampshire, a residential school for physically handicapped children with special problems, the treatment of boys in recent years has been carried on 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 would go short.

There is evidence that 90 per cent. of haemophilic patients in the United Kingdom receive less — and in some cases much less — than the best treatment for their complaint. The consequences of under-treatment in this particular disease mean unnecessary, painful, and destructive bleeding into joints and muscles.

Dr. Rosemary Biggs, a dedicated specialist in this field, has made the development of haemophilia treatment her life's work. She is director of the world's largest haemophilia centre, at Oxford,

Misery and anxiety

where so much successful research work was carried out — and from which Britain steered world research efforts along the right path.

Her views reflect the feelings of the majority of specialists we spoke to during our inquiries.

She says: "Those who treat haemophilia patients in the United Kingdom have of necessity in the past 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 at least damaging. Essential but non-urgent operations have been, and still are being, postponed."

The bulk of the queue is for operations to correct damage caused by undertreatment.

An extension of home therapy —for injections to be given by the patient himself or by his family, friend or GP — could not only significantly reduce suffering but also relieve the anxiety under which patients and their families now live. It would be expensive.

"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," says Dr. Biggs.

"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."

But while haemophiliacs go on suffering, large supplies of commercially produced Factor VIII concentrate lie unused and unsold.

Dr. Biggs knows the situation but, like so many other doctors, she is powerless to act.

"Whatever solutions there may be for problems of this sort in general, some immediate solution should be found for the ridiculous impasse of, on the one hand, large available stocks of therapeutic materials locked up in stores because no one will buy them and,

Scraping the barrel

on the other, patients in dire need of this material."

To avoid similar situations support among doctors is now growing for a special research fund in the NHS to be set aside every year for the practical use of research discoveries so that British advances are not lost.

Dr. Biggs speaks from a special position. She has seen so much achieved — and lost, in years of research. She sums it up: "When, as a direct outcome of years of research, life-saving therapeutic material suddenly becomes available to patients previously chronically undertreated, there should surely be some means of assimilating this welcome advance — otherwise it is stupid to undertake the research in the first place.

Prof. E. K. Blackburn, of Sheffield Royal Infirmary, who is ad-hoc chairman of Haemophilia Centre Directors of the United Kingdom, says: "Those of us in charge of haemophilic patients no longer feel that we can tolerate the undertreatment of these patients. Also galling is the fact that major research in the United Kingdom, resulting in the acquisition of knowledge to treat haemophilic patients properly by modern standards cannot be fully implemented for reasons outside the control of haemophilia centre directors."

Those reasons are financial. To make good the shortfall of Cryo (the drug currently used by the NHS) with concentrate would, it is estimated cost between £1m. and £3m. each year. That sum cannot be found from existing NHS allocations without cuts in spending on other necessities. Already local health authorities are scraping the bottom of the barrel.

Increased production of concentrate by the NHS would also be expensive, requiring substantial outlay on organisation of blood supplies, staff, equipment and buildings. At the same time poorly treated haemophiliacs cost a lot of money — as hospital in-patients and as recipients of social security benefits.

For years the daily struggle to obtain enough Cryo has been a therapeutic nightmare at the Newcastle Haemophilia Centre despite an excellent service from the local Blood Transfusion Service.

(continued from Page 4)

The Director, Dr. Peter Jones, puts it bluntly. "The majority of haemophilic patients only become physically handicapped because of inadequate treatment over the years. With adequate supplies of Factor VIII concentrate there can be no excuse for this. The resources must be made available," he said.

From the Haemophilia Centre at London's Royal Free Hospital 19 patients are on regular home treatment using Cryo, not the concentrate.

Although they are doing well their doctors say they are always on "a knife edge of anxiety about their supplies, despite maximum effort by the local transfusion service."

Dr. Katherine Dormandy, the centre's director, told me: "Patients, including one family in which there are two haemophilic boys, ring up continually to find our whether we have had a large enough delivery to be able to let them have another few bags.

"There seems little point in national support for research through the Medical Research Council unless the Government is prepared to develop the successful outcome of such work."

The same message is spelled out by Dr. Illsley Ingram, Director of the Haemophilia Centre at St. Thomas's Hospital, London, who describes as "cruel" the failure to make up the inadequate NHS supply of Cryo from the "large supplies of good commercial material which are now available."

He adds: "When freeze dried material is provided for immediate treatment at home or work, much suffering and the consequent need for analgesic drugs will be avoided. Therapeutic material now exists. Money must be found so that sufficient may be purchased until NHS resources are adequate for the needs of patients with haemophilia."

At the Leeds Haemophilia Unit the director, Dr. Layinka Swinburne, has asked the local health authority for an allocation to buy Factor VIII concentrate to enable her to put a small number of the most urgent cases on home treatment. The request is still going through committees.

"Until we have the money, the question of which patients most urgently require

Faced with the dilemma

home treatment, is academic," she says. "We have not decided — it will not be easy to do so.

"We are faced with a situation where because we are not producing enough of the stuff ourselves we are forced to pay out large amounts of money for it. We have held back for so long we now have a real dilemma."

Dr. Jones, at the Newcastle Centre, could no longer wait for the situation to improve within the Health Service. Faced with the dilemma he approached the Newcastle Regional Health Authority. After telling them that he felt morally and ethically obliged to provide home treatment with commercial concentrate for urgent cases, he won their backing.

Now 27 patients are on home treatment. The programme is growing and the cost is already in the region of £30,000 a year.

Dr. Jones, author of a recently published book 'Living with Haemophilia," is under no illusions about the financial side. "The Health Authority has a certain amount of money to spend," he says, "and perhaps because I am using this sum something somewhere is going short. A surgery may not be built, a doctor may not be appointed. I agree, it is a matter of priority but thank God I am not in the position of having to decide."

The Newcastle Haemophilia Centre is one of a handful buying supplies of concentrate from Travenol Laboratories Ltd., based at Thetford, and Serological Products Ltd., in Kent.

The British centres producing concentrate within the Health Service at Elstree, Oxford, and more recently Edinburgh, receive insufficient plasma from the Blood Transfusion Service to produce more than a trickle of concentrate most of which is used locally. That plasma has to be taken from fresh blood within 18 hours of collection and deep frozen before being sent to a centre for processing. The blood transfusion system lacks the facilities to do this on any large scale.

"The answer," says Dr. Jones, "lies in the reorganisation of the British blood transfusion service. Personally I am not prepared to wait for that reorganisation or for a British product to become available in sufficient quantities.

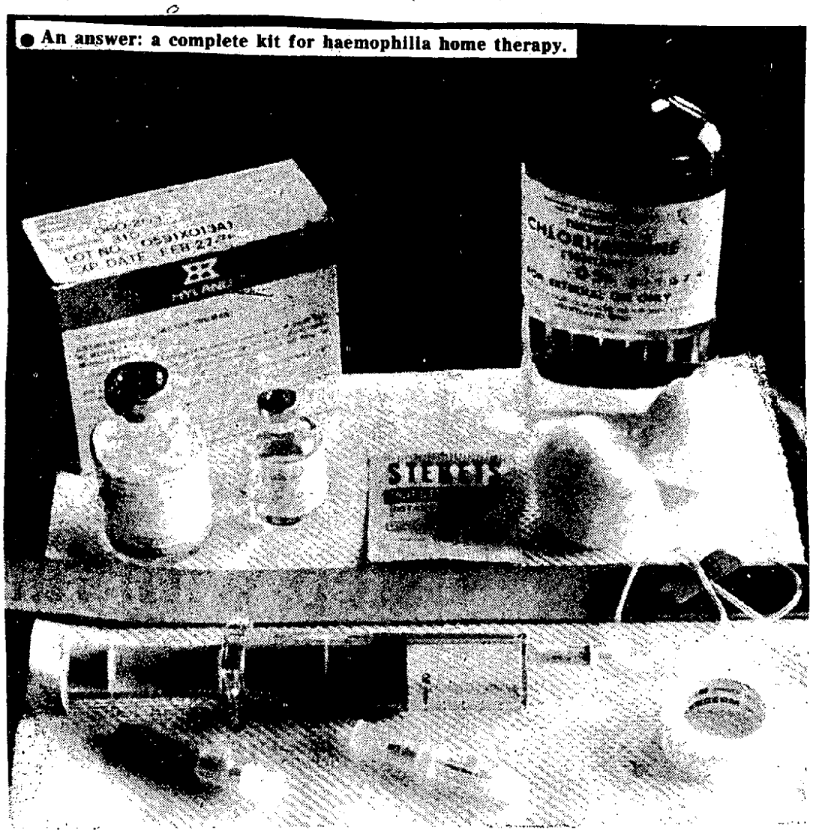
"When I see my patients growing up and suffering, I am convinced that home therapy is the only answer. The sooner a patient treats a bleed the sooner it stops and the less concentrate it requires."

The fact that British research advances have not been properly exploited is, says Dr. Jones, "the same old British story — no money to develop anything."

The major advance at Oxford in the sixties was the discovery of significant facts which meant that the amount of Factor VIII present in blood could be measured. This enabled the Factor VIII to be collected and really opened up the way to commercial production of good concentrate.

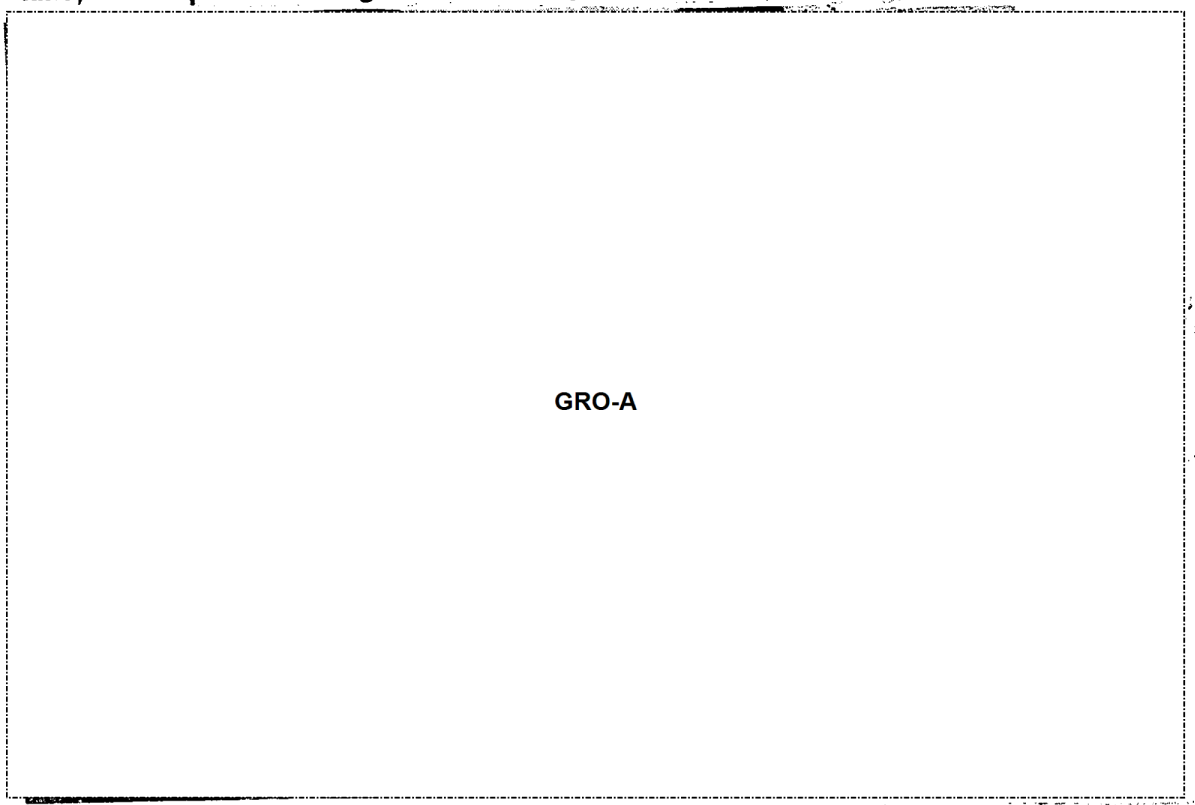
"I think it is absolutely scandalous," says Dr. Biggs. "The material should be as available for British patients as anywhere else in the world especially since we were so instrumental in advancing the treatment. Of course other people used the information, what we do is published and internationally available.

"It is terrible for doctors to have to battle for every penny like this. It is not really our job. I think the Ministry should make some move."



● An answer: a complete kit for haemophilia home therapy.

Part three of a special report by Medical Correspondent, ANGUS KING, on the problems facing Britain's haemophiliacs and their families.



ANGRY BLACK bruises on the legs and hands of an eight-month-old baby heralded an unbelievable and harrowing change of life for a happy family.

That family is still happy — but its existence is dominated and disrupted by a sick child for whom the parents can never do too much.

The bruises turned out to be internal bleeds caused when the baby began to crawl on the floor; Stephen, now five years old, is a severe case of haemophilia.

The disease ended all plans for a larger family for GRO-A and GRO-A they decided against it. It brought financial stress, endless worry, sleepless nights. For Mrs. GRO-A it has turned home into something of a prison as she stands by all day, unable to go out, in case of an emergency call to rush GRO-A to hospital 15 miles away for a vital injection of the special Factor VIII drug.

Haemophilia has sentenced her to long nights working at a 24-hour transport cafe for a few extra pounds a week to make ends meet.

"We did not realise what it was going to do to our lives," says Mr. GRO-A "Many a time we sit down at night after the worries of the day seem to be over and the tension is easing, and we just have a good cry together. It gets on top of you. You have to have a release."

GRO-A is undaunted by it all. He is bright, impish, restless, mischievous and shows a bulldog determination to do everything he should not. Being such a normal five-year-old makes it all the more difficult for him to appreciate the seriousness of the disease.

Protected by his condition from the occasional slap means inevitable exploitation by any five-year-old. GRO-A is just learning that, and proving a handful. GRO-A, his sorely tormented 12-year old sister exercises superhuman patience against physical retaliation.

About twice a week — it can be daytime or middle of the night — GRO-A GRO-A with a painful internal bleed has to be taken to the Haemophilia Centre at St. James's Hospital, Leeds, from his home in Rutland Drive, Crofton, near Wakefield. The pain means more often that not that he must be carried about before and after the treatment.

Because speedy treatment is medically vital and the least inconvenience to life, the GRO-A find themselves the most reluctant two-car family in Britain — but it is essential; so is the telephone — another expensive item — which provides an important link between home, school and hospital.

The luxuries possible on the salary of a fire brigade sub-officer are limited. One car is always tied up with his work 16 miles away at the Brigade training school at Birkenshaw and irregular working hours make public transport unworkable. Mrs. GRO-A uses the second car to take GRO-A to school, ("a normal one") about a mile away, five minutes late in the morning, and pick him up five minutes early in the afternoon to avoid injury in the crush of children.

Nights at the nearby transport cafe — when her husband is home to look after the children — means hours of washing up, cleaning, serving meals, etc. when others are asleep. Depending on business, she sometimes leaves early or stays from 10 p.m. to 6 a.m. before returning home to get breakfast for the family, and take GRO-A to school before slumping into bed to snatch perhaps a few hours sleep before a telephone heralds another long trip with her son to the Haemophilia Centre at Leeds. She say: "Sometimes I am absolutely shattered but I have to keep going."

Severe and persistent nose bleeds from weak blood vessels take their toll. It is not unusual to find GRO-A crying, lying in bed in a pool of blood in the middle of the night, or in his parents' bedroom, leaving behind him a trail of blood on the carpet. The automatic washing machine — another costly luxury — is also essential and helps the family to cope.

"We try to let him lead a normal life," says his mother. "He will stay out playing all day — if he gets a knock he will not give in. It is at bedtime that we sometimes find out. Sometimes he wakes up during the night in pain and we have to set out for the hospital.

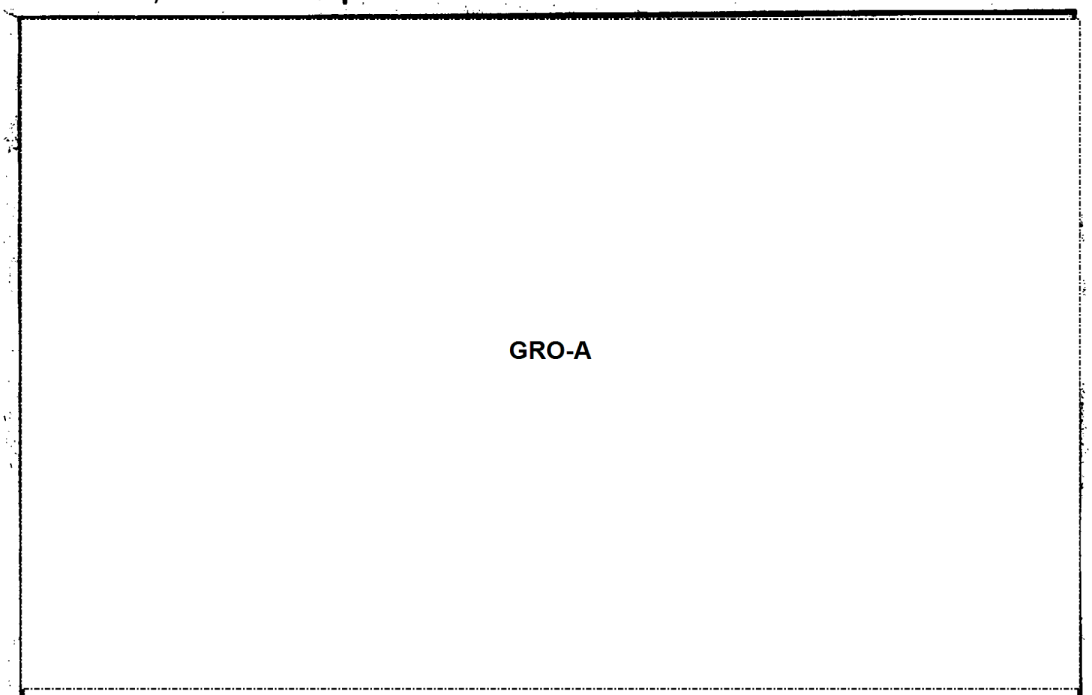
"He now knows the importance of what is being done when he is injected. He has accepted it and if he feels pain then he asks to go — but we can still see the fright in his face when his nose begins to bleed again."

It is a grim picture of the life of just one family in Britain living with haemophilia. "We don't get many full nights sleep," says Mr. GRO-A "I have gone to work sometimes without sleep for three nights."

Social outings and holidays lead to obvious difficulties — even a quick visit to the local pub means that a telephone number must be left with the baby sitter. Without the introduction of home treatment the GRO-A can expect no change in their existence. "The doctors say in time to come they hope to give it to everyone," says Mrs. GRO-A "We can only go on hoping too."

There is no hope of GRO-A being put on home treatment in the near future. Despite his condition he is not, in fact, one of the most urgent cases.

Most haemophiliacs have to rely upon hospitals for their life support, often travelling many miles in great discomfort and at any time of day or night. What difference does it make to be treated at home? — Part Four of a special investigation by ANGUS KING, Medical Correspondent



New life at home

ANDREW was quicker with the answer than his parents. What was life like before you began home treatment? "Terrible." His reply was like a bullet. His big eyes told me he meant it and the nodding heads of his parents confirmed it.

The memory of it is still not far away. Life was much like that of the GRO-A at Crofton until February his year when GRO-A and GRO-A began to give injections to their eight-year-old son themselves at home.

With precious and expensive supplies of Factor VIII concentrate nestling in the family fridge the GRO-A have managed to put behind them a long and bitter nightmare. It is not easy to watch your own child suffer — especially when it is unnecessary.

The number of haemophilic bleeds, mainly affecting the ankles, was constantly increasing. For GRO-A the 14-mile journeys from his bungalow home at GRO-A to the Newcastle Haemophilia Centre were becoming more painful, more frequent, and longer. The tally was 78 for 1973 — pain killing drugs, used to kill discomfort until the treatment takes effect, were ineffective, and the Cryo injections themselves were taking longer to work.

GRO-A is one of the few lucky ones — one of those "rescued" by the goodwill of the Newcastle Regional Health Authority which has made money available to combat suffering and the shortage of the NHS-produced Factor VIII drug.

Yet less than 100 miles away, five-year-old GRO-A cannot command the same treatment. All over Britain thousands of haemophiliacs cannot command the same treatment. They and their families must soldier on, continue suffering, and waiting in vain.

"As far as we are concerned it is the best thing God gave us," says GRO-A 's father of the Factor VIII concentrate.

Dr. Peter Jones, director of a Newcastle haemophilia centre, explains: "We put GRO-A on home treatment because he has good veins, sensible parents, who are able to give him injections, and because he lives in a geographically remote place.

"He was suffering recurrent bleeds which were interfering with family life and his education. We hope that home therapy will prevent physical and social crippling."

When home treatment became a possibility the GRO-A decided they would tackle it as a family. They went together to the haemophilia centre for a couple of days training in injection procedure.

"My wife and I were determined we should both go together with GRO-A and share the responsibility," says Mr. GRO-A. "We felt it was no good one without the other."

As a former farm worker who had injected thousands of cows Mr. GRO-A faced few problems but for his wife every trip to hospital with GRO-A had been an ordeal. She was afraid of injections — even watching them — and had to wait outside. Once she fainted.

Now she has overcome the fear and injects her son. "When it was first mentioned I never thought I could do it," she says. "We talked it over and I realised it had to be done — I would have to accept it."

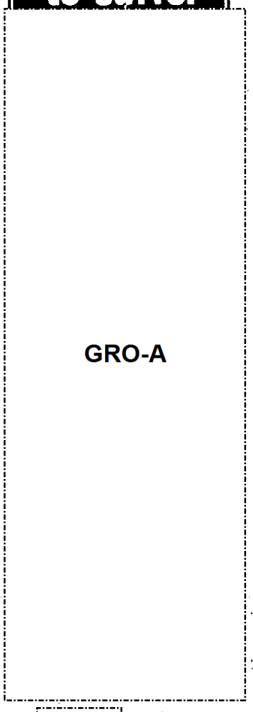
The concentrate takes about two minutes to inject and takes effect almost immediately.

"We did not realise what a difference it would make to our life," said Mr. GRO-A. "We can now live almost like anyone else and if we want to go away we can take the stuff with us.

"Like most haemophilic children, Andrew does everything he really should not, but he is a good patient. He just sticks his arm out — that has helped us enormously.

"Since we started home treatment he has hardly been off school at all and we have all changed. The strain was beginning to tell on all three of us. Now we have more confidence generally. Before this happened we had no intention of ever giving him anything as dangerous as a two-wheel bike — but now he has one."

Condemned to suffer



GRO-A on the steps of a happier childhood?

Britain, the pioneer in treatment of haemophilia, now struggles hopelessly to pay for vital drugs, while European countries which once looked to her for leadership are able to give treatment freely. Why does Britain lag so far behind and why have some drug firms overestimated the amount of drugs which they can sell to the State? ANGUS KING, Medical Correspondent, concludes his special report

Patient pawns in a political power game

WHILE BRITISH doctors continue to struggle against the chronic shortage of the vital drug needed by haemophiliac patients the rest of Europe looks on in bewilderment.

Britain is regarded by European doctors as the nation where the modern principles of haemophilia diagnosis and management have been laid down and effectively carried out since the early 1960s; the nation which has trained many specialists now dealing with haemophilia all over the world; the nation where the national health system created the first, and still the only, network of specialised haemophilia centres.

Despite this, British doctors have to suffer the ignominy of being the church mice of the Western world because progress, dedication and innovation have been strangled by the lack of money.

Haemophiliacs in European countries are receiving substantial supplies of commercially produced Factor VIII concentrate. Recently it was estimated that each haemophiliac in Austria, Germany and Italy was getting respectively 23, 19, and seven times more concentrate than a haemophiliac in Britain. The drug companies also claim that the European countries are paying a far higher price for the drug than is being asked here.

Even in Italy — a country facing bankruptcy — and where the price of Factor VIII concentrate is inflated by heavy import taxes, the concentrate is free to all haemophiliacs covered by a state-supported health system. It is expected that new legislation will, in the near future, extend this scheme to cover all haemophiliacs in the country.

A survey, as yet unpublished, on the quality of haemophilia care in various European countries, was recently completed by the European Committee of the World Federation of Haemophilia.

Reliable sources say that the survey will show that where the blood bank production of concentrates is not sufficient to cope with the national demand (i.e. Spain, West Germany), commercial concentrate is supplied free of charge to haemophiliacs by governmental health organisations.

In Britain no such arrangement exists — a fact which makes even more curious the antics of the Department of Health which resulted in drugs firms bringing in large supplies of commercial concentrate from abroad only to find that sales did not materialise because no funds were made available.

It was at the request of the department that two drug firms first made available large quantities of Factor VIII concentrate in Britain and they were granted licences to sell it.

One was an American firm — Travenol Ltd. based in the United Kingdom at Thetford, in Norfolk — which produces concentrate in both America and Belgium and markets it under the name Hemofil. The other was Serological Products Ltd., a United Kingdom distributing subsidiary of Immuno AG Ltd., of Vienna. It handles solely Immuno products including Factor VIII concentrate marketed under the name Kryobulin.

Travenol brought in supplies from America. Serological Products imported from Vienna. But the firms soon realised that they were caught up in a high powered game of medico-politics of which there could be no winner. The haemophilia centres had clamoured for the concentrate, the department had made it available with a "there it is, go and buy" manoeuvre, but no-one had the cash to go ahead.

"We got quite dizzy with the reps. coming round asking why we were not buying it. They had calculated the need, imported the right amount, and, surprise, there it was unsold," said Dr. Layinka Swinburne, at Leeds.

Before long the desperately needed stocks of concentrate were being shipped out for sale to other countries as they threatened to over-run their shelf life. These stocks were replaced by fresh supplies, but at a far lower level, and the size of reserves held in this country were run down.

For a time Travenol halted imports to reduce reserves to match the disappointingly small demand.

Not unnaturally both firms expected the original supply request from the Department of Health to be followed by reasonable initial sales, and later by regular orders for substantial supplies.

With broadly based interests in this country the firms are reluctant to make an issue of the affair with the department. But clearly both feel they were misled.

"We were," says a spokesman for Travenol, "put in a very difficult position — but we overcame it." The firm eventually sold only about 60 per cent. of the original amount it was asked to make available.

"We acted in good faith and it exploded in our face really. We feel the haemophilia centres have been let down. Our annoyance was not knowing where we stood."

Until now Hemofil has been brought in from America but soon Britain will take supplies from the company's five million dollar plant in Belgium.

Sales are reported to be going along "at the same old rate." "We only came into this because we were asked," says the Travenol spokesman. "We have been let down but we are not despondent."

Immuno's Kryobulin is manufactured in Vienna from blood from paid donors in Austria and Germany.

In Austria the Red Cross provides a service of whole blood from unpaid donors while Immuno, as a commercial enterprise, provides a service of all other blood products from plasma obtained from paid donors. Advertising for donors is not permitted — it has to be done by word of mouth.

A spokesman for Serological Products says: "We make no secret of the fact that Kryobulin has been sold at a level far less than was expected. We have a supply contract with the Department of Health. They put in an estimate nearer what was actually needed rather than for an amount for which money was provided.

"It certainly appeared to us that this whole scheme was going to be got under way very much earlier than it has — or has not."

He added: "When it became obvious that demand was not going to be in line with supplies, in order that it would not be wasted we sent batches out and brought in new ones — at the same time gradually reducing stocks."

The firm now holds stocks of between only 300,000 and 1 million units at any one time, compared with over 2 million units initially.

Italy imports most of its concentrate from Immuno in Austria and from Travenol Ltd. The price is high — about £100 for 500 units (about double that in the United Kingdom) an amount barely enough for one injection. The high cost is due to heavy import taxes.

At this price Italy's 3,000 haemophiliacs, needing an average 6,000 units of concentrate each annually, are costing £3 million a year to treat. But the patient pays nothing.

About 80 per cent. of the Italian population are covered by a social security health scheme supported by the State, the workers, and employers, and operated by the Ministry of Labour. Soon everyone will be included.

Dr. P. M. Mannucci, director of the Milan Haemophilia Foundation, says: "It is simply astonishing to hear that many British haemophiliacs are undertreated or have no access to the most modern form of therapy (home treatment) for lack of availability through the NHS of the commercial freeze dried concentrates.

"To illustrate the absurdity of the situation, in my country which started no earlier than 1968 a modern programme of haemophilia care basically derived from that existing in the United Kingdom, the commercial freeze dried concentrates are available free of charge to all haemophiliacs covered by the social security system.

"The concentrates, which can be obtained at the chemists with a prescription from the haematologist, make home treatment very simple and easy for GPs, the patients or relatives."

The Italian system is something of a vicious circle — although there is nothing vicious about the end result for haemophiliacs.

The concentrate is imported from Immuno Vienna by Immuno Italy. The state reaps in heavy import duties part of which goes towards its contribution to the health scheme. Immuno, allowing for its own profits, distributes the concentrate to chemists who supply the drug over the counter on prescription to haemophiliacs. They are then reimbursed by the state health scheme.

"These facts," says Dr. Mannucci, "are not meant to magnify the recent achievement of other countries but to display the absurdity of the situation in the country which has been, for a long time, pioneer in the field and example of other countries."

LETTERS TO THE EDITOR

THE YORKSHIRE POST
23rd Jan.1975

Critical points

SIR, — May I be allowed to make three major criticisms of Mr. Angus King's series of articles on Haemophilia.

Firstly, although he states that haemophilia can be treated by the preparation "cryoprecipitate" (cryo), he does give the impression by using such phrases as "children with haemophilia have been unnecessarily subjected to extra pain and suffering," that cryoprecipitate is unsatisfactory for managing these cases. I must emphasise therefore, that although "cryo" is less convenient to administer in that it must be stored in a deep freeze, and the amount of "AHG" can vary a little from batch to batch, it does stop bleeding as many haemophiliacs will confirm. At the Transfusion Centre in Leeds, we have produced more "cryo" than has been requested from doctors caring for haemophiliac children and I can recall no occasion when a request for "cryo" has been refused.

Secondly, the articles give the impression that almost all haemophiliacs are suitable for home treatment. Cases vary considerably in severity and what may be more important, some children's veins are so small or inaccessible that it can be a very difficult task to give the intravenous injections of "AHG" necessary.

Even doctors experienced in this work have considerable difficulty in giving the injection to some of these children. Therefore, it might well be an intolerable burden to expect the parents of these children to be responsible for these intravenous injections.

My third criticism is that the Yorkshire Regional Health Authority, and the Leeds Regional Hospital Board, before this, has been planning to provide sufficient "AHG" concentrate as opposed to "cryo" for at least two years. One of the last tasks of the old "RHB" was to give top priority to the £50,000 scheme of extensions to help in this matter. We had also asked for increased staff and equipment and began to plan to obtain extra donors for this task. The extraordinary thing is that, although Mr. King spoke to almost every Haemophilia Centre in the world, he never spoke to anyone at the Yorkshire Regional Health Authority or at the Yorkshire Regional Transfusion Centre!

It is therefore in my opinion, totally wrong to infer nothing has been done about "AHG" concentrate production in this area or in fact, in this country.

Lastly, when we do reach the target of around 40 per cent., of all donations being used for "AHG" concentrate it will mean we have to issue 40 per cent. of our blood supplies to hospitals as concentrated red cells and many cases requiring blood are unsuitable for this type of transfusion. Haemophilia therapy therefore cannot be isolated from the total medical services to the region.

One of our immediate problems is that "AHG" concentrate has to be manufactured from fresh plasma at the special laboratory near London and this does take some time. In this transition period therefore, we have to produce both "cryo" and "AHG" concentrate, and this was not mentioned in the articles.

Taking all these facts into account in my opinion it is unfair to use such phrases as "the archaic blood transfusion service" when in fact, it appears Mr. King never visited a centre or even consulted a member of their staff. — Yours faithfully,

DR. L. A. DERRICK TOVEY,
(Director)
Regional Transfusion Centre,
Leeds.

Angus King writes. — The Yorkshire Post investigation was a nationwide inquiry into the plight of haemophiliacs. In the series, no direct criticism was levelled at the Blood Transfusion Service in Leeds, but overall advice and criticism of the service nationally came from many experts whose opinions were reflected in the articles. In any investigation there comes a time when inquiries must end. It is surely more important to highlight the plight of haemophiliacs than to bicker about which specific experts or organisations, of which there are many, were not interviewed. It is widely recognised that those involved in the Blood Transfusion Service in Britain do a magnificent job within the facilities which exist.

Haemophilia and home treatment

3rd Feb.1975

SIR, — As one of those interviewed by Angus King for his recent series on haemophilia, I would like the opportunity to comment both on the articles and on Dr. Tovey's letter (January 27).

In my opinion Mr. King wrote about a very complicated subject truthfully and displayed a depth of knowledge which could only have come from much careful research. That this did not include an interview with the local Blood Transfusion Service or Health Authority does not detract from the essentials of his argument. The supply of factor VIII has been a national problem of major concern to haemophiliacs and their doctors for the past decade.

Before commenting on the specific criticisms made by Dr. Tovey it must be emphasised how grateful haemophiliacs and their doctors are to the Transfusion Service for their continued support. Without the unfailing generosity of blood donors and the painstaking work performed in the Regional Transfusion Centres any advance in haemophilia management would be impossible. The argument is therefore not with the Transfusion Centres but with successive government for their consistent failure to provide the funds necessary for the full developments of the service on a national basis.

Dr. Tovey is of course absolutely right to say that Cryoprecipitate stops bleeding. The advent of "cryo" in the 1960s heralded a dramatic change in the management of haemophiliacs, and quick out patient therapy became possible for patients who had previously been admitted to hospital for prolonged periods of plasma transfusion. However, in spite of the excellent service referred to in the article, the production of "cryo" in Newcastle has not always kept pace with demand and our use of other forms of factor VIII replacement material, including commercial concentrate, continues to rise.

As Dr. Tovey states concentrate is preferable to "cryo" because of easier storage and more accurate dosage. It is also simple to prepare for injection without contamination, and to administer in the dosage required because of its small volume. The incidence of immediate side effects is lower, it has a longer shelf life and it can be used for travel or work away from home as well as for home therapy.

In our experience, and that gained in Haemophilia Centres in both Europe and America, the majority of severely affected haemophiliacs are suitable for home therapy. Because recurrent bleeding into the joints does not usually begin until the age of five years the problem of the "poor or inaccessible veins" sometimes found in the pre-school child should not be used as an argument against parents treating their own children.

Although home therapy can be safely recommended from the age of six, an earlier programme is still both safe and suitable for the occasional younger child with a particular need. To suggest that home therapy might well be an intolerable burden for those parents responsible for the intravenous injections required says little for them or for the care afforded to families by their Haemophilia Centres. That the reverse is true is evident from the interview with the Atkinson family.

These though are minor quibbles in the context of the national situation. The fractionation of blood into its component parts, one of which contains factor VIII for haemophiliacs would as Dr. Tovey says, benefit many other patients requiring transfusion. At present the majority of these patients receive whole blood rather than the component they need — an example of poor economics at a time when the demand for blood is increasing.

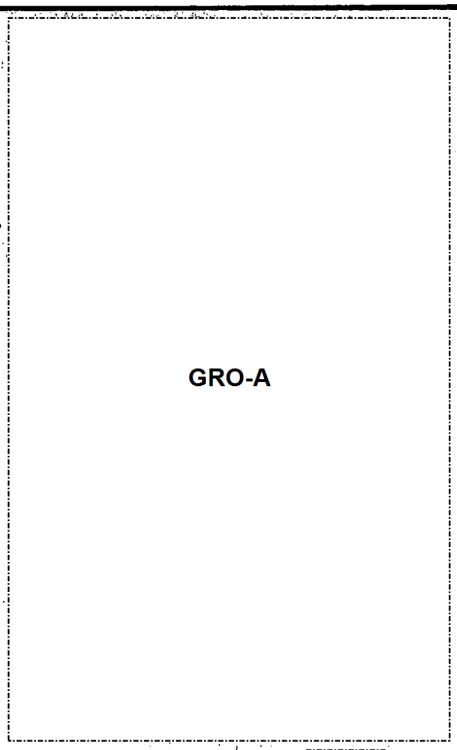
While we wait for the fractionation programme he mentions to reach fruition haemophiliacs have to rely on imported concentrate. The Government have shown their recognition of this need by licensing the commercial material, but have not produced the funds necessary to buy it. To say that factor VIII concentrate is readily available to Regional Health Authorities who are already struggling to maintain their present services may be politically expedient, but is hardly fair to patients who wonder why they cannot obtain treatment they know has been available to haemophiliacs in other countries for the past five years.

Haemophilia is a rare disorder and as a group the needs of haemophiliacs are easily concealed in terms of the disabled as a whole. The Yorkshire Post has performed a valuable service in bringing the subject of haemophilia treatment to the attention of the public who, in the long run, must be responsible for the development of their Health Service. — Yours faithfully,

(Dr.) PETER JONES,
(Director, Newcastle Haemophilia Centre)
Newcastle upon Tyne.

The cure is ready but GRO-A 's suffering goes on

THREE THOUSAND people in Britain today who suffer from haemophilia urgently need a new blood extract, developed in this country, which can stop their bleeding and its crippling effects. But the treatment is expensive and the Department of Health will not help hospitals to buy thousands of bottles of the extract lying on the shelves of two drug firms. As a result, children are growing up without the full benefit of this discovery and are suffering unnecessary pain and damage. MARJORIE WALLACE reports.



A wheelchair life: six-year-old GRO-A

GRO-A is six and spends much of his time in a wheelchair. Five days a week, on average, he suffers intense pain and has to be rushed to a London hospital from his Essex home. Every minute's delay increases the chance that he may grow up a cripple.

GRO-A has haemophilia. His most obvious symptom is that his blood does not clot normally. It is a hereditary disease, passed on through the mother, but usually affecting only male children. If GRO-A cuts himself or has a nose-bleed, quick action is needed to stop him bleeding to death.

But there is also a more insidious danger: frequent spontaneous internal bleeds, which result in permanent damage to his tissues, muscles and, most often, his joints. Both GRO-A ankles are affected. Like all severe haemophiliacs, he has become the victim of a cruel cycle. Each bleed causes painful swelling and also weakens the joint, making it liable to further bleeding. If this goes on, it is finally eroded and useless. His muscles are in similar danger of being wasted away.

"It is frightening to look into the joint of a person who has been bleeding for even one or two hours," says Dr Peter Jones, director of the Haemophilia Centre in Newcastle, "and see the destruction that has taken place. It is this process we must prevent."

It was eight years ago that doctors at the Churchill Hospital, Oxford, pioneered a new form of treatment that can stop this destructive cycle. They developed a blood extract known as Factor VIII which supplies the missing factor in a haemophiliac's blood. It can halt a bleed within seconds and bring immediate relief from pain. It is freeze-dried so that it can keep for several weeks in a refrigerator, and giving an injection is simple enough for the patient or his family to perform at home.

But little Factor VIII has been made in Britain — partly because the Department of Health was unwilling to put up money for processing facilities, and partly because blood is scarce and a large quantity is needed to make a little extract. Other countries, however, have been making Factor VIII. It is available under the health schemes of many European countries.

In 1973 the directors of British haemophilia centres, annoyed that many British patients were not benefiting from Factor VIII, began to put pressure on the Department of Health. It licensed two drug firms, Serological Products and Travenol, to import 10 million units of the extract from America and Austria — enough to treat all Britain's haemophiliacs for several months. But still there was a snag. The department left it to regional health authorities to pay for the Factor VIII out of their already narrow budgets. And it is expensive: each injection costs £25 for a child and £50 for an adult. It was obvious from the start that they could not buy much Factor VIII at that price—so many experts regarded the department's move as no more than a face-saving gesture.

Thousands of bottles of Factor VIII remain on the shelves of the two drug firms. "There is no need for a child of six or seven to be deformed by haemophilia today," says Dr Charles Rizza of the Churchill Hospital. "He should be able to run about, fall off his bicycle and do anything a normal boy does. We have the know-how but the stuff is just lying there unused while these children risk becoming crippled. It makes me angry to see such mismanagement of a life-saving treatment and it makes nonsense of all the research we have done. It isn't medicine any longer—its politics."

The Department of Health says it is not its policy to provide central help for individual cases. "We do not pre-empt the decisions of the health officers in the field," said a Department of Health spokesman, "and we never allocate money with a tag on it." Later the department modified this statement and admitted that central funds are provided for some drugs with specific users. For example, L-Dopa is bought centrally for the treatment of Parkinson's disease. The spokesman explained: "We never reveal the criteria by which we choose. Let us just say that economics and widespread shortage of a material come into it."

Last year the two drug firms, struck with a large stock of Factor VIII with a limited shelf-life, started to re-export and protested strongly to the Department of Health. It responded last October by buying 500,000 units of Factor VIII from the companies and then offering it to the regional health authorities at the normal commercial price. What this was intended to achieve is uncertain, nor is it known whether all the material was bought up. A senior official at the Department of Health said: "It was certainly not purchased for the exclusive benefit of haemophiliacs. It may have been to promote goodwill towards the drug firms."

The department has since also licensed two other firms.

Even the famous Great Ormond Street Hospital in London is so short of Factor VIII that it cannot give GRO-A 's mother a few days' supply for use at home. "GRO-A will undoubtedly be permanently crippled unless he gets home treatment with factor VIII soon," says Dr Elizabeth Letsky, who treats him. "If we had sufficient Factor VIII, a bright boy like GRO-A could become a valuable member of society. But if he has to continue this way, coming in five or so times a week after an hour's journey, he is in danger of spending his life in a wheelchair and becoming a burden."

There is now a glimmer of hope, following protests by patients and their families, by MPs and by directors of haemophilia centres. Last month David Owen, Secretary for Health, pledged a £500,000 grant to improve the blood transfusion services in order to provide more plasma for the production of Factor VIII. Even here the department is reluctant to appear to be earmarking funds for a particular group of sufferers. "What we are doing," says an official," is not allocating funds for the treatment of haemophiliacs, but for increasing the production of plasma which may help haemophiliacs."

Doctors concerned with haemophilia say this action should have been taken at least eight years ago. The build-up will take at least three or four years.

At present most National Health haemophilic patients are treated with a crude blood extract called "cryoprecipitate." This contains many impurities, which make it dangerous, especially for children. Dr Letsky says: "Each time I inject a child who is allergic to 'cryo' I have a sinking feeling. I never know whether he will develop a serious reaction. There are no such problems with Factor VIII.

While Factor VIII continues to be out of the reach of most regional authorities, children like Jon will spend the next few years under-treated. It would cost the Health Service between £1 million and £3 million a year to treat all haemophiliacs with Factor VIII. Several hospitals have already shown, however, that the cost to the nation is in the end much less than the alternative in-patient treatment, orthopaedic operations to straighten twisted joints, social security payments and time off work—let alone the cost of institutional care.

"The Government's sums just don't add up," says Dr Rizza of Churchill Hospital. "They are thinking in the short term. They don't realise that it is cheaper to keep a haemophiliac fit, quite apart from the humanity of it. I have often heard a boy crying out in pain long before he reaches the building. There is no excuse for his suffering."

GRO-A 's mother says: "Why doesn't the country see economic sense? Each time he has a bleed it takes an ambulance and two ambulance-men—and, if it is at night, calling up a doctor. Even then, I always give the injection —the doctor just looks on. I could easily have done this hours ago at home. It's a daily nightmare. We can have no family life, no holidays and we can never plan anything. The other children get neglected and sometimes I break down altogether.

"We have a wheelchair and a new bicycle which I bought GRO-A for Christmas and they stand side by side in the hall. It's up to the Government which of these he will be able to use."