50TH ANNIVERSARY 1950

THE BULLETIN

Magazine of the Haemophilia Society

WINTER 2000 ISSUE 4

The National Institute for Clinical Excellence (NICE) recommends combination treatment for hepatitis C

On 31 October 2000 NICE issued clinical guidance recommending the use in the NHS of interferon alpha and ribavirin in combination for the treatment of hepatitis C. The guidance was issued after an appraisal which began in May this year, and which involved examining clinical trial data into the effectiveness of the drugs, economic evidence and also evidence supplied by patient organisations including the Haemophilia Society.

- This is good news for people with haemophilia infected with HCV, a number of whom have been waiting for treatment which health authorities were unwilling to fund. **For a full report see page 10**.

NOT RELEVANT

NOT RELEVANT

NOT RELEVANT

Thanks go to the following pharmaceutical companies who are providing valuable support in 2000.
Aventis Behring • Baxter • Bayer • BPL
Grifols • Novo Nordisk • Roche • Schering-Plough • Wyeth/Genetics Institute
Copies of the Haemophilia Society's commercial funding guidelines are available upon request.

CONTACT LIST FOR GROUPS

In order to establish contact with your local Haemophilia Society group you should write in the first instance to the national office. We have groups in the following areas:

ENGLAND

BRISTOL & SOUTHWEST • CAMBRIDGESHIRE & DISTRICT
CORNWALL • HAMPSHIRE • HULL • KENT
LEICESTERSHIRE & RUTLAND • LINCOLN & DISTRICT
NORFOLK & NORWICH • NORTHAMPTON • NORTHERN
NORTH WEST • NOTTINGHAM • OXFORD
SOUTHERN • SOUTH ESSEX • YORKSHIRE

WALES

NORTH WALES

SCOTLAND

PERTH • GRAMPIAN • TAYSIDE
WEST OF SCOTLAND • SOUTH EAST SCOTLAND

NORTHERN IRELAND

NORTHERN IRELAND GROUP

SPECIAL INTEREST

BIRCHGROVE GROUP

SERVICES AVAILABLE FROM THE SOCIETY

- General information about haemophilia and related bleeding disorders
- Information about Social Security benefits
- Information, advice and support on hepatitis and HIV
- Information for parents of newly-diagnosed children
- Volunteer support network
- Hardship grants
- Aventis Alert pager service (previously called Centeon Call)
- Caravan holidays in the UK
- Adventure holidays and weekends for children
- Fundraising support
- Assistance with media enquiries
- Information on treatments
- Travel advice and travel insurance advice
- Haemophilia Days and Family Days
- One-off meetings on specific issues, such as hepatitis
- Hepatitis C Support Network
- C. Issues
- Mild and moderate haemophilia support contacts

For more information about any of the above services, please contact the national office.

Full details of our services are also available on our web site: www.haemophilia.org.uk

Chief Executive's Column

Karin Pappenheim, chief executive

As the year 2000 draws to a close, the Society can look back on a very eventful 12 months. We have marked the charity's 50th anniversary this year in a number of ways.

Parties for children with haemophilia funded by the Roald Dahl Foundation have taken place all over the country, and the House of Lords reception in July brought together our parliamentary and other supporters. These events were a reminder of the extraordinary history of the haemophilia community in this country, and a time to look forward.

No account of this community's history could fail to include the tragedy of contaminated blood products. Yet, here another year has passed and still there has been no official public inquiry into this disaster. The long awaited report of the investigation in Scotland called by Scottish Health Minister Susan Deacon was issued shortly before we went to press, and has proved a sad disappointment.

The report completely failed to examine the political decision making processes in the 70s and 80s - and did not answer questions about whether cost considerations came before safety for the politicians and their advisors who were responsible for blood products policy in that era.

With press and media at the moment filled with revelations from the four year inquiry into BSE, more hitherto buried information about official decision making and public health is now coming out. Also ongoing are a number of court cases against the National Blood Authority involving people infected with hepatitis C through contaminated blood. These findings are likely to renew the debate about past blood safety policy, and provide more grounds for the public inquiry we have been seeking.

In the meantime, the Society's campaigns continue as reported on page 6. We are actively pressing for more provision of recombinant for adults in England - following the policy already adopted in Scotland and Wales of providing recombinant for all.

NOT RELEVANT

NOT RELEVANT

CONTENTS

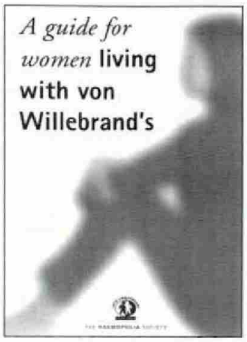
MAGAZINE OF THE HAEMOPHILIA SOCIETY

2000 ISSUE 4

NOT RELEVANT

New von Willebrand's booklet for women

As part of the Society's new project for women affected by bleeding disorders, we have produced our first publication which will be available in the new year from national office. The 36 page booklet has been specially written for women and girls with von Willebrand's, in consultation with an expert advisory group of doctors, nurses and women who have the condition.



Research from the UK and other countries suggests that many women with von Willebrand's may go undiagnosed, in some cases for years, whilst suffering heavy periods and other specifically female bleeding problems which can be treated once diagnosed properly. A study carried out at the Royal Free Hospital of women referred for gynaecological investigations for mennorrhagia (heavy periods) found that 17% were suffering from an undiagnosed bleeding disorder.

In order to reach those as yet undiagnosed women, the Society plans to distribute the booklet through women's health networks (e.g. via health visitors, family planning clinics, midwives) in addition to the normal haemophilia centre distribution. We also hope to achieve some publicity in women's magazines to bring more attention to this hidden problem.

For a copy of the booklet contact head office or ask your haemophilia centre.

NOT RELEVANT

Appointment of trustee to Macfarlane Trust

The Haemophilia Society and the Macfarlane Trust are seeking a trustee to join the Board of the Trust. The vacancy arises following the resignation of GRO-D There are ten members of the Trust's board, four are appointed by the Department of Health and six are appointed by the Society. The new trustee will fill one of the six Haemophilia Society appointed places.

The Society and the Trust have been advised by the Charity Commission that it is possible to appoint a service user as a trustee providing that adequate safeguards are in place for possible conflicts of interest. It has been agreed therefore to seek a service user for this vacancy.

A trustee job description is available from the Macfarlane Trust (GRO-C). Those interested will need to submit a written application indicating that they are able to meet the job description requirements by Monday January 15, 2001.

CAMPAIGNS Update

THE AIMS OF THE SOCIETY'S CAMPAIGN

- **To persuade government to provide financial assistance to meet the needs of all people with haemophilia and related bleeding disorders affected by HCV**
- **To press for the best treatment and care for people with haemophilia and related bleeding disorders infected with HCV**
- **To persuade government to hold a full public inquiry into contaminated blood products**
- **To ensure recombinant is available to all throughout the UK regardless of age or viral status**

Scottish Executive Report

The report of the fact-finding exercise commissioned by Susan Deacon, Minister for Health and Community Care into the heat treatment of blood products in Scotland in the mid 1980s, was issued on 25 October 2000.

The report concluded that the Scottish National Blood Transfusion Service was around 18 months behind the Bio Products Laboratory in England in producing a heat-treated product which was subsequently found to have eliminated the hepatitis C virus but that *'...there were understandable technical reasons why this was the case'* : The report also concluded that *"..there was no evidence of any policy by Haemophilia Centre Directors deliberately to mislead patients about the risks of hepatitis."*

The Minister ruled out providing financial assistance for people with haemophilia and hepatitis in Scotland stating that *"... the NHS should not pay compensation for non-negligent harm...."*

The Society immediately wrote to Susan Deacon seeking an urgent meeting, as well as to members of the Scottish Parliament, including the chair of the Health Committee and opposition health spokespersons. Other actions are being planned by members in Scotland led by the Society vice-chairman Phil Dolan, based in Glasgow, and trustee GRO-D based in Perth.

Responding to the report, Karin Pappenheim, chief executive of the Haemophilia Society, commented: "This report is a very thin, incomplete piece of work which does not represent the full inquiry we were seeking. The report fails almost entirely to address the impact of the contaminated blood products tragedy on the haemophilia community in Scotland - not only the medical, but the emotional and social consequences of the virus which have devastated lives. Significantly there is no commitment to action either in the form of financial recompense or even so much as a promise to ensure that all those infected with hepatitis C receive proper care and support.

"This is a wholly inadequate response to what has been described as one of the greatest treatment disasters in the history of modern medicine and to the effect this has had on the Scottish haemophilia community.

Susan Deacon MSP

"It flatly contradicts the evidence put forward by the Society and our members that patients were not clearly informed of the risks and that many were informed of their diagnoses late or by accident."

The full report is available on Scottish Health on the Web (SHOW) website www.show.scot.nhs.uk

- Full details of the Society's response to the report can be obtained from national office.

For further information
contact the following trustees in Scotland.
Phil Dolan (tel. GRO-C or
GRO-D (tel. GRO-C

CAMPAIGN FOR RECOMBINANT

The Society wrote to Lord Hunt of Kings Heath, Parliamentary Under Secretary of State for Health, on 22 September requesting an urgent meeting on recombinant.

The letter followed the publication in *The Lancet* of more research evidence of the theoretical risk of transmission of variant Creutzfeldt-Jakob disease through blood. This is in addition to concern over non-enveloped viruses, particularly parvovirus B19 and hepatitis A.

The Society pointed out that the recent survey of recombinant provision carried out by Dr Linda Garvican, senior research fellow at the Health Care Evaluation Unit of St George's, London, revealed considerable inequity in recombinant provision in the UK. It showed that NHS commissioners in Scotland, Wales and Northern Ireland have adopted a policy of recombinant for all, while in England health authorities are restricting recombinant largely to the under 16s.

Health Minister Lord Hunt

Key points made in the letter were:

"The provision of recombinant for children has been strongly welcomed within the haemophilia community, and particularly amongst parents. However, the Society believes that with increased availability of recombinant products now, it is time to extend this provision to adults.

"Whilst fully aware that as yet there is no evidence that either classical CJD or new variant CJD has ever been transmitted to people with haemophilia through blood products, the new research shows that ***we cannot be certain*** *that there is no risk of infection in these products.*

".....Considerable anxiety is generated within the community by the fear of blood-borne viruses and diseases which may escape modern inactivation processes used in the manufacture of plasma products. The mere fact that parvovirus and hepatitis A can be transmitted is a cause for concern because this indicates that other potentially dangerous viruses with similar physical properties (ie small and lacking a lipid envelope) may be transmitted in plasma-derived factor concentrates.

".....recombinant clotting factors should to be made available to all people with haemophilia whatever their age and viral status wherever they live in the UK.".

"The current situation with regard to recombinant provision in the UK is inequitable and cannot be medically justified. Current treatment of haemophilia in the UK is discriminatory.

"Choice of plasma derived or recombinant clotting factor is decided by age, postal code and whether you have been infected with other viruses in the past or not.

"A number of the Society's members in England are now challenging their health authorities' refusal to provide recombinant, and we expect this pressure to grow with the continuing focus on BSE and CJD in the press and media."

SOCIETY SUPPORTS BATTLES WITH HEALTH AUTHORITIES

The Society is also supporting a number of individuals in England who are battling with their health authority for recombinant. We have condemned the current situation in the UK as grossly unfair and completely unjustifiable. Some people with haemophilia in Newcastle and the Midlands are refusing treatment which is derived from plasma in protest at this unfairness.

One man who contracted HIV and hepatitis C 15 years ago from his NHS treatment with contaminated blood products, has even resorted to getting friends and relatives abroad to supply him with recombinant clotting factor as he is refusing plasma derived products.

PLEASE NOTE: The Society does not recommend that anyone withholds treatment from themselves because they could put their lives at risk. However, we appreciate the strength of feeling behind these individual actions.

Fundraising

Contact Juliet Harris, Fundraising & Marketing Officer

A Country of Fundraisers!

NOT RELEVANT

NOT RELEVANT

GRO-D

Congratulations to the team of twelve walkers from **Baxter Healthcare** who took part in the Welsh 3000s Baxter Challenge, completing what is regarded as one of the toughest long distance walks

RED RIBBON PAGE

Compiled by
Babs Evans
HIV/HCV Worker

NOT RELEVANT

NOT RELEVANT

Plea from New Zealand

Hi There!

My name is GRO-A and I am looking for some help and advice. I have two sons born with severe haemophilia, both have Hep C and one has HIV. GRO-A the eldest has HIV and he is now 27 years old. We have reason to believe he was infected in Scotland in August 1980. This has been a 16 year search for me, it was not until 1985 when I was asked to get his blood tested for Hep C that he was diagnosed with both viruses. From the time GRO-A was born in 1973 and GRO-A in 1975, both boys were being treated with cryoprecipitate but started being treated with factor VIII in New Zealand around 1988-1989. We did not know until recently that GRO-A had been given factor VIII in Scotland back in 1980. If there is anyone out there who sued privately for compensation, would you PLEASE help me by giving me advice on what steps to take.

To contact GRO-D please telephone Babs on GRO-C or e-mail GRO-C

Hepatitis Update

Compiled by Lucy McGrath
Hepatitis Worker

NICE Report

Continued from page 1

The National Institute of Clinical Excellence (NICE) has recommended to the NHS that the combination of interferon alpha and ribavirin should be used for the treatment of moderate to severe hepatitis C (i.e. where patients show some evidence of liver damage) for the following patients over the age of 18 years: -

- All patients who have not previously been treated with interferon (alone or in combination) and all patients who have previously been treated with interferon on its own, and have had some "response" (i.e. cleared the virus from the blood while on treatment) but have since relapsed. Such treatment should be continued for 6 months for all patients.
- A further six months combination therapy is recommended only for patients infected with a specific type of hepatitis C virus (genotype 1), who "respond" to the treatment in the first six months. "Response" is measured by the patient's blood becoming clear of the hepatitis C virus, detected by a polymerase chain reaction test (PCR).
- For most patients, the doctors decide whether or not the hepatitis C is moderate to severe by doing a liver biopsy, but the NICE guidance states that people with haemophilia can receive treatment without necessarily needing a liver biopsy.

Whilst we welcome the decision by NICE that the more effective combination therapy should be used for the treatment of hepatitis C, we fear that health authorities may be slow to provide funding to make it available.

We are already aware of a number of our members who have been waiting months for this treatment - refused on the grounds of cost. This is despite repeated government assurances that treatment should be made available to people with haemophilia and hepatitis C on the grounds of clinical need - assurances which have been ignored by some health authorities.

Karin Pappenheim said "It is a disgrace that some people with haemophilia who contracted hepatitis C through contaminated blood products used in their treatment on the NHS have so far been denied the chance of a cure by NHS bureaucracy. Our fear is that health authorities may wait until the next financial year until beginning to make the treatment available, adding a further delay for people who have already been waiting too long for adequate treatment."

If anyone is still experiencing delays in getting this treatment for funding reasons then get in touch with me at the Society - we may be able to help.

Lucy McGrath. Hepatitis Worker

NOT RELEVANT

Disability Living Allowance and Hepatitis C

GRO-D

Ruth Taylor,
Information and Advice Worker

There are various benefits available for those unable to work due to illness and/or on low incomes but Disability Living Allowance DLA is unique in that it is not income related or taxable and it is payable if you are working. An award of DLA is also a condition of entitlement to higher rates of other benefits.DLA is awarded to people who need help with personal care (which does not include shopping or housework) or with getting around or both. In order to qualify for either or both of these components it is not enough to show that you suffer from a particular condition. You have to demonstrate that it is giving rise to particular needs or difficulties and will continue to do so for at least six months.

Many people on interferon/ribavirin therapy or whose condition is reasonably active or advanced should qualify for this benefit. The joint pains, fatigue, night sweats, depression and other symptoms which are characteristic of hepatitis C or side effects of therapy can give rise to many care needs and mobility problems and those suffering from these symptoms should always make a claim.

However, we have found that quite a number of applicants are having their claims turned down. In one case a tribunal told an applicant that he was exaggerating his symptoms. Whilst researching the information required by decision makers who decide applications I was amazed to find that the *Disability Handbook* which is supposed to inform decision makers about the conditions they are dealing with, made no mention of hepatitis C whatsoever.

In order to persuade the publishers of the *Handbook* to remedy this omission, GRO-D and I met with the Disability Living Allowance Advisory Board in July together with one of our members who lives with mild haemophilia and hepatitis C. He has recently had to cut short his interferon/ribavirin therapy because he developed anaemia and described his symptoms and problems to the board very graphically. We also provided a number of medical studies and other information for the board to study.

The result has been that both decision makers and medical examiners working for the Benefits Agency are to receive training in the symptoms and problems of Hepatitis C as related to haemophilia. I also hope that the next addition of the *Disability Handbook* will include information on hepatitis C. So, do put in your claim if you believe you qualify. Good corroborative evidence from a specialist nurse, social worker or physiotherapist who knows you, as well as medical evidence from your doctor, will be helpful.

Contact GRO-D or myself if you have trouble completing the form or are turned down first time and need help with an appeal.

Survey of HIV and/or HCV treatment - initial results

We have now had 57 responses (out of 92) to our survey of the treatment of HIV, and/or HCV in haemophilia centres. The data has not yet been properly analysed by Dr Linda Garvican, an independent researcher, but there is one worrying finding from the initial data. This is that only 16 out of 30 haemophilia centres (not including comprehensive care centres) have taken steps to trace all patients, past and present, who might have been exposed to HCV infection.

Also, out of all comprehensive care centres and haemophilia centres, only 13 (out of 47) have managed to complete the trace of all patients who might be at risk of having contracted HCV.

These findings lend weight to our calls for a proper Department of Health "lookback" (as was done with patients who received contaminated blood transfusions) to trace all those with haemophilia, or a related bleeding disorder, who received clotting factor concentrates before the late 1980s, and to offer them HCV tests.

We will be taking this matter up with the Department of Health.

Treatment Update Compiled by Dr David Evans

The BSE Enquiry

Dr David Evans

The report of the BSE enquiry, chaired by Lord Phillips, has been published, with much media discussion. Eighty of the 85 people affected by new variant CJD have died. There is no evidence that blood products transmit BSE to humans, but the possibility has not been definitely ruled out

The enquiry is more concerned with the way the Government handled the information available at the time than with the precise details of how BSE came to infect human beings. Lord Phillips' committee has concluded that it was probably due to a new prion mutation in cattle, or sheep, in the early 1970s, and spread through the practice of feeding cattle with recycled animal protein.

The Government was preoccupied with preventing an alarmist reaction to BSE because it believed that the risk was remote. As a result the Department of Health was initially able to play only a small role in the discussions.

The needs of the victims have not been met. Care has been variable and not always responsive to the rapidly changing needs of patients. So the government will now set up a new national fund, to improve diagnosis and care, to set up a national network of experts and to purchase appropriate care and equipment. The government would also like to set up a compensation scheme with a special Trust Fund amounting to millions of pounds; but other options are being considered. Whether this will end up with cash payments being made to patients and their families is not yet clear; but we await developments with keen interest.

The CJD surveillance unit has announced that all cases of variant CJD so far have occurred only in people with a particular genetic subgroup. Whether this means that only individuals with this subgroup can become infected, and that those who lack it will be immune, is not known. The possibility remains that those without it may be taking longer to show signs of the disease. Nevertheless it offers some encouragement that many of us may not be at risk of developing CJD.

Can BSE be Transmitted through Blood?

In September, *The Lancet* (Lancet 2000; 356: 999-1000) published a letter from a group at the Institute for Animal Health in Newbury, to say that they had transmitted BSE from one sheep free from symptoms to another by transfusion with whole blood. This provoked a response from Paul Brown of the National Institutes of Health, in the USA, which also appeared in *The Lancet* (Lancet 2000; 356: 955-956). He wondered why a preliminary report about only one sheep had been hurried into publication before the results of inoculations into a total of 19 sheep had been completed. The author remarked that there is scanty evidence, if any, that blood can transmit BSE to humans. So far as BSE, scrapie and similar disorders are concerned:

- Blood and blood components are infectious to experimental animals only when they are inoculated into the brain or abdomen
- In naturally infected animals, all attempts to transmit disease by the inoculation of blood have failed
- Although it was reported that blood from four of 37 human beings with sporadic CJD had transmitted the disease by inoculation into the brain of guinea pigs, mice or hamsters, the results could not be repeated and have been questioned on technical grounds
- There is no evidence that any case of CJD could be attributed to administration of blood or blood products, and no-one with haemophilia has been infected in spite of frequent doses of plasma concentrates

However, it has been shown that lymphoid tissues such as the tonsil may contain the prion protein thought to be responsible for the disease. Lymphocytes are a major component of the tonsil. They are white blood cells and therefore present in blood, some of which can be carried over into plasma concentrates. So theoretically the prion could be transferred by transfusion of blood or plasma concentrates. For this reason, the Blood Transfusion Service now removes the white cells from all donated blood and only uses plasma imported from countries free from BSE.

The author concluded that the paper added nothing to current thinking about BSE and variant CJD. It was published prematurely because of the need to calm public anxiety about the dangers, due to the fact that the media had exaggerated the risks. There is no evidence that BSE can be transmitted this way to man.

Seven of the individuals who have developed variant CJD had previously been blood donors. Some of their donations had been mixed with plasma from other donors. It is not known how many individuals received blood products from these donors, but no-one with

variant CJD has so far been linked with them. The government is considering more stringent controls on blood plasma used in surgical emergencies. However, each unit of plasma produced in the UK is derived from a single donor, and not from pooled donations; so the risk is minimised.

Gene Therapy for Haemophilia in the UK

The UK Haemophilia Centre Doctors Organisation has recently set up a genetics working party. It will discuss matters of genetics as they affect haemophilia such as standards of practice, the provision of genetic services round the country and gene therapy. It will also hold discussions with people working in Clinical Genetics over matters such as counselling, and the interaction between departments of clinical genetics and haemophilia centres.

It has already discussed the following:
- The difficulties involved with consent for genetic tests for both adults and children

- How and where genetic tests for haemophilia should be undertaken, and

- Counselling for families with congenital bleeding disorders

The working party has also issued a discussion document titled "Gene Therapy Trials for Haemophilia in the UK".

The working party believes that the UK is an ideal site for trials of new treatments, because of the well-established organisation of haemophilia care in the UK. It would like to bring gene therapy trials here, and establish a framework to set up any gene therapy treatment that might develop as a result.

Although the effects of haemophilia can be substantially altered by treatment, past treatments have led to major complications. It remains very expensive and less developed countries cannot afford to provide any treatment at all. So there is good reason to look at gene therapy as an alternative to existing therapies. Unlike many other disorders, haemophilia is due to a defect of a single gene, which should make gene replacement straightforward.

(But it won't be - several other disorders are due to a single gene defect, but the way that the gene affects the individual varies because of other influences, just as the severity of haemophilia does not depend solely on the clotting factor level - Ed). Nevertheless the risks of gene therapy are unknown, whereas the risks of present-day replacement therapy are well recognised.

Some of the questions we particularly want to know the answers to are:

- Will HCV infection exclude patients from trials?
- If gene treatment leads to unexpected complications, will it be possible to switch off or remove the new gene?
- What are the risks?
- How can parents give informed consent for their children to be treated when the long-term effects are unknown?

There are many problems with gene therapy which are quite distinct from the basic problems of how to make it work. In recent years the management of new treatments and clinical trials has become much more highly organised. The ethics of such treatment have to be discussed by various authorities. The government has instituted numerous regulations for the conduct of trials of new drugs and other treatments. In addition, gene therapy is regulated by the Gene Therapy Advisory Committee. Most of the trials approved so far have been aimed at the treatment of cancer, and the committee considers that all gene therapy must be considered to be experimental, and not yet regarded as treatment.

Other organisations with an interest in gene therapy include:

- The Medicines Control Agency, which regulates medicinal products and applications for use in clinical trials
- The Health and Safety Executive, which has to protect the health of people in the workplace who handle the materials used in such trials
- The Department of the Environment, who may need to issue consent if the modified cells or viruses used may spread to the environment
- The NHS itself, which has a responsibility for deciding whether any research programme should take place in its premises.

So you can see how complicated it will be to set up any trial of gene therapy, when all these organisations have to be involved, quite apart from any considerations for the safety of the patient.

Haemophilia is a rare disease. Any gene therapy protocols must be suitable for all patients with haemophilia, and not just any particular sub-group of patients. Drug companies are more likely to support gene therapy for common diseases, such as cancer and heart disease, as success will bring greater returns for their investment. It would be wrong to develop trials of gene therapy for haemophilia because it appears to be an easy option, and then to find that the number of patients was too small to make the treatment commercially viable.

The Society is invited to respond to the Centre Directors by the end of December this year, so if you have any views, please write to Karin Pappenheim.

NOT RELEVANT

New Products

- **Bayer** has just launched their new recombinant factor VIII, which uses no human material as a stabiliser. Instead of human albumin, it uses sucrose, the same sugar (properly purified!) that we put in tea. Although human albumin is used in the initial stage of production of factor VIII, it is removed during later stages and only traces remain in the final product, which is then treated to inactivate any potential viruses by a solvent-detergent technique. No bovine protine is used at any stage. It is called Kogenate Bayer. All doses, whatever their strength, are reconstituted into a volume of 2.5 ml, and 25g needles are supplied as routine. The packs contain 250, 500 or 1000 units. Once made up, the material for injection contains 28 mgm of sucrose. For those who are worried about the sugar content 28 mgm is about half a grain, or less than one eight-hundredth of an ounce: the amount is minute.

Trials in North America and Europe have shown that it is as well-tolerated and as effective as Kogenate, the existing product. It is no more or less likely to lead to the development of inhibitors than other concentrates. This new product certainly appears to be an improvement on the existing one.

- **Baxter** has recently gained approval from the FDA (the body which approves drugs and their production in the United States) for their second manufacturing suite at Thousand Oaks, California, where they make their recombinant product, Recombinate. Approval has also been sought for a third suite. Although the USA has approved the facility, approval is still awaited from the European Union. It is expected that approval will come through in a few months time.

Although the products made in this new suite are not yet available in Europe, the fact that they are now available in the USA means that more of the products made in the original production suite will be available for export to the EU; so this should ease supplies. Baxter is also developing a new recombinant concentrate which is free from all human and animal products. No albumin or other protein is added to stabilise the product although the initial stage of the production process, which uses chinese hamster ovary cells, is unchanged. The material is similar to Recombinate, and the initial trials will be starting soon.

NOT RELEVANT

NOT RELEVANT

European Haemophilia Consortium

View of Timisoara, Rumania

The idea of holding a meeting for people with haemophilia right in the heart of Dracula country has a strange irony. But as those of us who attended the European Haemophilia Consortium annual conference in the city of Timisoara in Romania found out, there is a very warm welcome to be found there in the heart of the Transylvania region.

Over 70 delegates representing 26 EHC member organisations attended the Consortium's annual conference in Timsoara, Romania over the weekend of 6-8 October. The UK society was represented by Chris Hodgson and Karin Pappenheim, who was also there as head of the EHC secretariat, which is now managed by the UK Society. Gordon Clarke, also of the UK, is the EHC chairman. Here Chris Hodgson gives his perspective on the meeting.

A fascinating part of our stay was the visit to the Centre for Evaluation and Rehabilitation for haemophilic children and young people at Buzias about 40 kilometres from Timisoara. This is a centre where children with haemophilia can go with their mothers and stay for up to three weeks. Whilst there is little treatment with concentrates, the centre has the facilities to provide physiotherapy, hydrotherapy and an exercise regime administered by a trained staff of physios and doctors. This helps patients regain use of damaged joints and builds up muscles to prevent further bleeds and prepares children to live without the treatment we have come to take for granted in the UK.

Karin and GRO-D facilitated a workshop on the "Present and future role of the Haemophilia Society". We heard from 11 countries in Europe about their Societies. Again, there were concerns from many countries such as Romania where there is still little treatment. Societies in the majority of Western Europe are well developed with paid staff, support services and up to date publications. many have been involved in HIV and hepatitis C campaigns and are using recombinant products for the majority of patients.

It was most humbling to hear GRO-D from the Romanian Society, who worked so hard to host the event, say "Please don't feel sorry for us; we welcome the help which can be provided to our Society".

NOT RELEVANT

PARENT'S CORNER

Compiled by GRO-D
Children & Families Worker

These are some of the questions we are frequently asked by parents and carers and some tips that parents have shared with us.

Q: What is the best way to stop a mouth bleed? Our son is 9 years old and has a loose tooth,which keeps bleeding, have you got any suggestions?

A: Our main advisors recommend Tranexamic Acid (Cyclokapron) either by tablet/syrup orally or as a mouth wash. It can be given to people with severe haemophilia as it is a very useful addition to FVIII treatment. Mouth bleeding often looks much worse if a child is crying and dribbling and an ice-lolly is an effective treatment, both for calming a distressed child and slowing the bleeding. If the bleeding persists for several hours seek advice from your haemophilia centre.

Q: We want to know if we should get a Port-a-Cath put into our son. He is 15 months old and has severe Haemophilia A. I heard that the risk of infection is high and that infusing is still tricky, requiring two people to do the procedure, I thought it was supposed to make life easier?!! What are the advantages and disadvantages?

A: Veins can be troublesome things in very little people. A Port-A-Cath (PAC) can be inserted so that there is constant venous access and you don't have the stress of having to try and infuse your son whilst holding him still and he is screaming his head off.

A (PAC) is a device that sits underneath the skin of the chest wall in a metal button with a latex rubber membrane through which a needle can be inserted many times. It is put into place through a small cut near the right nipple and another cut through the skin of the neck. The soft, flexible catheter is introduced into one of the neck veins and then into the right atrium (of the heart).

Once the device is in there is a risk of infection but only normally from the external part of the device. It is important to use aseptic techniques when administering treatment. Having a PAC put in is a surgical operation and as such your child would run the normal risks associated with having a general anaesthetic. Many parents say that the device has radically changed their lives, decreasing stress levels, leaving them more free to pursue their lives, however, if your child does get an infection as a result of the PAC he must then take antibiotics and there will be yet more visits to the haemophilia centre! Children can get a blockage in the line. One other thing to note is that a port will still allow your child to do many things such as swimming and bike riding. It is probably worth talking to other parents whose children have had PACs and to talk to a haemophilia nurse specialist as your starting point. PACs are not the right thing for everyone and plenty of people manage by just finding a vein and going in.

Q: I want to have a baby but I know that I am a carrier of the haemophilia gene. My brother has haemophilia and HCV and my uncle died of HIV/AIDS six years ago. I don't know what to do if I get pregnant or if there are any tests that could tell me what sex the baby will be or if he has haemophilia, help?!

A: The best time for you and your partner to seek information from a haemophilia centre is before you become pregnant. If, however, you find youself pregnant before you have sourced this information, don't panic. Contact your haemophilia centre as soon as possible. Being a carrier of haemophilia and making decisions on pregnancy and childbirth is very difficult, particularly if your views on haemophilia may be associated with negative things that have happened in your life. What is important to know is that treatments have changed dramatically in the last 10 - 15 years. Children with severe haemophilia and PUPs (previously untreated patients) will automatically be eligible for treatment with recombinant products (these have never been associated with viral infection) and will be treated prophylactically (not as and when required but before a bleed may occur). For this reason, these children should grow into adults with stronger joints and muscles and be virus-free. However, it is equally important not to play down the facts, and they are: haemophilia is still a life-threatening condition, you will have to be more vigilant with your child and he will still require life-long treatment for his condition. There may be some careers that he won't be able to do and he may still suffer from joint damage. Conversations with people with haemophilia are surprisingly mixed on these issues, some men say "I wouldn't have wanted a child of mine to be born with haemophilia", whilst others say "I live a good life and I love life, thankfully termination and tests for haemophilia were not really an option when my mother was pregnant".

So what are the tests? **In brief they are:**

CVS (chorionic villus sampling) at 10 - 14 weeks. This can detect the sex of the baby and the haemophilia status. If you have a daughter you may be able to find out her carrier status - easier if you have a family haemophilia history and blood has been taken from a certain group of family members. This test does run a risk of miscarriage (around two percent) and it is worth checking out the experience of the clinicians doing the test.

Amniocentesis - taken at 16 weeks, can detect genetic disorders including haemophilia and Down's syndrome, has a similar risk of miscarriage to the CVS test. This test is done at a significantly later date which may affect a woman's decision.

PGD - pre-implantation genetic diagnosis. This is still not widely available but the Haemophilia Society

continued on page 19

NOT RELEVANT

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