DO YOU HAVE A DRUG PROBLEM?

Orphans, Economic Orphans and Expensive Relations

FRANK FIRKIN

We recently approached HSA members for names of useful drugs which are unobtainable, or difficult to obtain in Australia. We did this because subcommittees of both ADEC and the RACP had expressed interest in identifying and improving access to drugs of this type.

While a patient can privately import such medications from overseas as a result of the Baume report recommendations, this is an absurdly difficult way to practise medicine. The replies we received basically represented four types of access problems.

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CONTENTS

Do you have a drug problem 1
Haematology on the Internet 2,8
Council 1995 - 1997 3
ASH Education Program '95 4
New Members 4
Young Achiever's Award 5
State Reports 6
Letters to the Editor 7
Courses at St Mary's Hospital London 9
Future Meetings 10

Orphan Drugs: These are essentially drugs for which a sponsor (i.e. pharmaceutical company) can not be found, or for which sponsorship is withdrawn. One example is the recent deletion of oxymetholone from PBS listing. It would not be excessive to infer that getting to the bottom of why such an event should occur, by talking to the regulators on the one hand, and suppliers on the other, feels at times like playing snakes and ladders. In this instance, it appears that a subsidiary company ceased manufacturing the drug so that it was delisted because of lack of a sponsor. It would also be fair to say that other important medications have from time to time become unavailable for variety of reasons, and that certain helpful people in the interstices of the regulatory processes have found us other sources and ensured continuity of supply. These are the good people.

It is usually far from this, and it is possible to get the impression that the person, branch, or group who could reasonably be expected to have a constructive interest in helping patients disadvantaged by such problems, believe it is someone else's responsibility. It is interesting to see that Peter Baume addressed this very problem in 1991, and we still await an effective response, to recommendation 125 "Responsibility for assuring supply of service items (i.e. orphan drugs) should be delegated to the Health Care Access Division which already has responsibility for the PBS and community/State issues. This additional task should commence immediately." (Page 145 - The Future of Drug Evaluation in Australia.)

Another item that has been delisted is rapid release iron tablets. A reply from one previous supplier was that the base price set by the price agreed for slow release preparations was not enough to break even for the cost of iron-polymaltose (Ferrum H), and the proposition to market it was no longer economically viable.

Economic Orphans: By far the greatest number of drugs mentioned were of established benefit for a specific disorder, but did not either have approval sought by the manufacturer, or granted by the TGA, despite safety and efficacy approval for other purposes. As a
result, the drug can not be considered for PBS or Section 100 listing, and these include:

Danazole for ITP and related autoimmune disorders, Cyclosporin and anti-thymocyte globulin for aplastic anaemia, Orabolin for plasminogen activator deficiency, Chlordeoxyadenosine for lymphoid malignancies other than Hairy Cell Leukaemia, high dose DDAVP nasal spray for mild haemophilia, Interferon for non-Hodgkin’s lymphoma, Acyclovir for BMT patients, and many others.

Nouveau-Riche Relations:
Another category is drugs for which PBS listing was either not taken up by the manufacturer (e.g. Dacarbazine), or withdrawn. These include Bleomycin (only 50% funded), Mustine, BCNU, Procarbazine, and others. The opinion from the PBAC that withdrawal from PBS listing, or partial funding, was due to failure to reach agreement between the PBAC and the supplier on a price, or on a subsequent rise in price. In these instances the supplier chose to withdraw, and place the drug on the open market. In the past, much of the purchase cost was borne by public hospital pharmacies, but the future prospects for this route is not promising because of budget cuts.

Very Rich Relatives:
These include recombinant agents which are very costly to produce and are obviously expensive. They will never be considered for listing on Section 100 or on the PBS until they receive marketing approval, and then they must be shown to be cost effective. It is nonetheless encouraging to see that support has become available in the case of Interferon for CML, is to become available soon for myeloma after obtaining a treatment response, and that marketing approval has been granted for G-CSFS for stem cell mobilisation. It is also heartening to see the PBAC have removed previous limitations on use of tranexamic acid.

Disclaimer:
It is very difficult to present a totally objective answer to each of these problems, as opinions voiced by the parties involved not surprisingly can be stated with feeling, but be very different from that proffered by the other side, and time is just not available to probe every issue.

What can be done?
The preferred option for orphan drugs is for constructive assistance to be provided by the relevant Commonwealth Department as suggested by Peter Baume. As for other issues, one of our members, John Hounsell, actually did something, and wrote to the Commonwealth minister who then broadened the indications for use of Desferral on Section 100. It seems likely that we, as the relevant specialist group, will have to look after our own interests, and that our membership will have to be engaged more in actions of this type, either through contacts in the political process who are approachable, and likewise through helpful members of the pharmaceutical industry.

HAEMATOLOGY ON THE INTERNET

**BLOODLINE (not to be confused with Blood Lines) is a place on the Internet for haematologists.**

**Address**
http://www.kcj.com/blood/
# COUNCIL 1995 - 1997

<table>
<thead>
<tr>
<th>Position</th>
<th>Name</th>
<th>Address</th>
<th>Phone 1</th>
<th>Phone 2</th>
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<tbody>
<tr>
<td>President</td>
<td>Dr Ed Sage</td>
<td>Haematology/Oncology Dept, The Queen Elizabeth Hospital, Woodville, SA 5011</td>
<td>08 222 6645</td>
<td>08 222 6046</td>
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<tr>
<td>Local Organiser 1996</td>
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</tr>
<tr>
<td>Vice President, Newsletter Editor, Organiser 1997</td>
<td>Dr Alison Street</td>
<td>Department of Haematology, Alfred Hospital, Prahran VIC 3181</td>
<td>03 92763120</td>
<td>03 92763781</td>
</tr>
<tr>
<td>Honorary Secretary</td>
<td>A/Prof Ken Bradstock</td>
<td>Haematology Department, ICPMR, Westmead Hospital, Westmead, NSW 2145</td>
<td>02 633 7649</td>
<td>02 689 2331</td>
</tr>
<tr>
<td>Honorary Treasurer</td>
<td>Dr Katherine Marsden</td>
<td>Oncology &amp; Immunology Lab, University of Tasmania Clinical School, 43 Collins Street Hobart, TAS 7000</td>
<td>002 388 899</td>
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</tr>
<tr>
<td>Councillors</td>
<td>Dr Ross Baker</td>
<td>Department of Haematology, Royal Perth Hospital, Box X2213 GPO, Perth, WA 6001</td>
<td>09 224 2897</td>
<td>09 224 3449</td>
</tr>
<tr>
<td>Organiser 1997</td>
<td>Dr Jeffrey Szer</td>
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<tr>
<td></td>
<td>A/Prof Kerry Taylor</td>
<td>Department of Haematology, Mater Public Hospital, South Brisbane, QLD 4101</td>
<td>07 840 8735</td>
<td>07 840 8338</td>
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<tr>
<td></td>
<td>A/Prof Graham Young</td>
<td>Institute of Haematology, Royal Prince Alfred Hospital, Camperdown, NSW 2050</td>
<td>02 515 8829</td>
<td>02 515 6255</td>
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<tr>
<td>Coopted Members</td>
<td>A/Prof John Buchanan</td>
<td>82 Mountain Road, Epsom, Auckland 3, NEW ZEALAND</td>
<td>64 9 630 6168</td>
<td>64 9 623 2904</td>
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Thank you to our

SUPPORTING DONORS 1995, 1996

AMGEN Australia Pty Ltd
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Organon Teknika Pty Ltd
Rhone-Poulenc Rorer Australia Pty Ltd
Roche Products Pty Ltd
Sandoz Australia Pty Ltd
Schering-Plough Pty Ltd
The Boots Company (Australia) P/L

There are a limited number of *Hematology 1995 Education Program, American Society of Hematology, Seattle, Washington - December 2-5 1995* available from the Secretariat.

Please contact
Mrs Ruth Guss, 145 Macquarie Street, Sydney 2000
Tel: 02 256 5456
Fax: 02 252 3310

***************

WELCOME TO OUR NEW MEMBERS

At the last HSA Council meeting in February

SC Yeoh, SJ Wright and PJ Ho were accepted as full members.

MS Ward was accepted as an associate member.

***************
THE ROYAL COLLEGE OF PATHOLOGISTS OF AUSTRALASIA

YOUNG ACHIEVER'S AWARD

The Royal College of Pathologists of Australasia (RCPA) has this year introduced a Young Achiever's Award to be presented at the RCPA Annual Scientific Meeting.

The Criteria

- The presenter must be aged 35 or less at the time of presentation
- The presenter can be any person working in the area of pathological sciences
- The abstract is to be submitted on the prescribed abstract form, a copy of which is available from the HSA Secretariat.

- Closing date for abstracts - Monday 3 June 1996

The Award

- The award will include the cost of return economy travel from anywhere in Australasia, 1 nights’ accommodation and 1 day registration to attend the Annual Scientific Meeting to be held from 16 to 19 September 1996 at the Sydney Hilton Hotel
- a monetary prize of $1,500.00
- presentation of the work submitted at the Annual Scientific Meeting.

Further details from Dr D Sullivan.
Tel: 02 515 8312    Fax: 02 515 7931
STATE REPORTS

Could all members please let their State Councillors know if distinguished visitors are attending your department. Perhaps a combined HSA meeting could be planned in order to attract a wider audience.

WESTERN AUSTRALIA

Once again the HSA in conjunction with the ASBT have organised a Margaret River weekend to be held from Friday 3 - Sunday 5 May 1996. We have organised an interesting and varied scientific and social programme to provide a stimulating meeting. We hope that we will give you time, not only to enjoy the meeting, but the good food, wine and environment the Margaret River Region offers.

The invited speakers for the weekend are Dr Bik To from the Institute of Medical and Veterinary Science in Adelaide who will discuss Stem Cell Transplantation; Dr Malcolm Webb, Consultant Haematologist at the Woden Valley Hospital in Canberra who will present his experience on Gene Therapy; Peter Klinken, Professor Clinical Biochemistry at Royal Perth Hospital who will discuss Cell Biology and Cytokines; Professor Alex Gallus from the Flinders Medical Centre in Adelaide who will discuss Target INR Ranges for Oral Anticoagulation; Dr Tom Exner, Research Director Gradipore, who will outline Laboratory Investigation of Thrombophilia. In addition to these invited speakers there will also be a Transfusion Symposium and a Coagulation Symposium. There will also be a Free Communications Session and participants are invited to submit abstracts. A prize will be awarded to the best Free Communication to assist presentation at the national meeting this year.

If you require registration / accommodation or abstract forms, please do not hesitate to contact me.

The Society has also commenced monthly meetings at the Red Cross Blood Bank where invited speakers and local clinical units can present interesting cases and scientific research for general discussion. The next meeting planned is the HSA/AMGEN orator Dr Nigel Russell from Nottingham on the 15th April. Information concerning this meeting will be sent specifically to each member.

Ross Baker for WA

NEW SOUTH WALES

The NSW Branch of the Society remains very active with monthly Scientific presentations from the major hospitals in the State. These meetings are held at the Ritz Carlton Hotel in Macquarie Street in Sydney, and attract about 50 members each month. All members and their guests are welcome.

The Annual State Education weekend is being held this year at the Vineyard Resort in the Hunter Valley, on 20-21 April. At the meeting, the Amgen/HSA Orator for 1996, Dr Nigel Russell, will speak on Allogeneic Blood Stem Cell Transplantation and Autocrine growth stimulation of AML cells. As usual the programme is adorned with a cast of local stars. Details of the programme and accommodation arrangements can be obtained from Judith Salvo, Haematology Dept, Westmead Hospital, Westmead 2145.

Ken Bradstock, Graham Young, Councillors for NSW

TASMANIA

The 20th Annual Weekend Scientific Meeting of THING (Tasmanian Haematology, Immunology and Neoplasia Group) was held at Poatina Holiday Village on the weekend of 9-10 March.

The meeting was very successful and attended by over 50 delegates and their families who also enjoyed golf, tennis, swimming and sightseeing.

Invited speakers were Marianne Berwick from Sloan Kettering Cancer Center who presented her work on risk factors for melanoma, Sue O’Malley, Pharmacoeconomist with Amgen Australia and Jim Wiley who gave an overview and update on the Australian trials of all trans retinoic acid and also contributed in a seminar on monocyte haematology. Local speakers also gave presentations on a wide range of topics.

Katherine Marsden, Councillor for Tasmania

VICTORIA

The annual scientific meeting for the Victorian members of HSA will be held at The Country Place, Kalorama on Saturday April 27th. Our guest speaker will be Nigel Russell, sponsored by AMGEN, and the program includes transfusion, transplantation, training as well as non ‘t’ topics.

The monthly morphology sessions convened by John Catalano and fortnightly inter-hospital meetings preceded the start of the football season which is again upon us.

Alison Street Councillor for VIC

SOUTH AUSTRALIA

The SA community has been single mindedly pursuing the organisation of the Annual Scientific meeting in October. By now you should have received your registration brochure for the meeting. If you haven’t or if you need more abstract forms, please contact the conference organiser, Elisabeth Eaton at Festival City Conventions Ph: 08 363 1307
LETTERS TO THE EDITOR

15 January 1996

Re: Proposal for the HSA to become a direct sponsor of research

I have serious concerns about this proposal, and have already discussed these with Frank Firkin.

This should not be interpreted as lack of support on my part for research. Recognising my own limitations in this area, I am quite dependent on high-quality research for my livelihood.

My concerns are both practical and ethical.

Firstly, I ask how does becoming a funder of research help “to promote improved standards of haematology”? I think it may even have the reverse effect. Instead of pressuring those in government, industry and the community who should be supporting medical research, it lets them off the hook! It sends the message that we in haematology don’t need extra funds for research, because we can raise it among our own membership.

My other concern is much graver. If we are to be a Learned Society, responsible for setting standards for training and the practice of our craft, we cannot then become an employer of our own trainees or graduates. And, if we are employers of some researchers, we certainly cannot claim to have an objective view of the merits of the work done by other researchers in the field. We would have a vested financial interest in the work “our” grant recipients are doing.

There are many more useful ways for the Society to use its surplus assets in ways which will enhance our craft. Here are just a few:

- Professional advocacy of our patients’ interest, to improve access to new therapies.
- A larger and more comprehensive Journal, including research reports, and
- A career counselling service which covers all of the options for young trainees.

We must not allow our mutual enthusiasm for research to cloud our judgement. The society should be promoting the funding and quality of research, not threatening its own integrity by directly competing in the field.

The debate on this subject needs to be developed much more before the Society makes any firm commitments.

With my best wishes

Yours sincerely

Dr Steve Flecknoe-Brown.
LETTERS TO THE EDITOR continued

24th February 1996

Dear Steve

Thanks very much for your letter of 15th January 1996 about the future use of HSA funds. I was pleased to hear from you, as the Blood Lines newsletter article has drawn little response overall. A bit of debate on the future of our profession is certainly in order.

Your letter was discussed at some length at the HSA Council meeting last week, and I hope that some positive moves will result. I think that you have highlighted some genuine concerns.

I think you are correct in stating that it would be wrong for the HSA to become directly involved in research. Council did agree, however, that the most worthwhile use of the interest earned on our capital reserves each year would be to foster the training of our advanced trainees.

A working party fro Council has therefore been formed to develop the concept of establishing one or more fellowships for Haematology trainees. These would be targeted at people near the end of advanced training to provide sufficient funding to permit specialised clinical or laboratory training, probably in US or European centres of excellence. It is frequently difficult for registrars to get funding for overseas jobs, and a fellowship of, say $25,000 would be of great assistance.

A proposal along these lines will be put to Council in July, and if accepted circulated to members for comment prior to the next AGM. This is not to say that other ideas, such as those put forward in your letter, are ruled out. In fact, if you would like to develop a proposal, with or without the support of other Society members, we will be very happy to consider it.

I fully agree that further debate is necessary, and look forward to hearing from you.

Yours sincerely

Ken Bradstock
Honorary Secretary.

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ELECTRONIC COMMUNICATION AND THE HSA?

Should the HSA have a presence ("home page") on the World Wide Web of the Internet? If we had one, this could serve as a mechanism for having a readily available source of information about the activities of the HSA accessible to the world (as well as our own members and prospective members). A particular advantage would be wide spread notification of our Scientific Meetings, which could serve to increase international attendance, particularly from South-East Asia.

A second question is that of electronic mail (email). If the majority of members had access to email (and used it) the routine dissemination of Society materials (including subscription notices, membership lists, notices of meetings etc) would be more timely, less labour intensive and certainly cheaper.

The author welcomes comment and input from members about these matters so that the society can make appropriate arrangements to "get connected". Responses to the Secretariat or to szer@icre.ludwig.edu.au.

Jeff Szer
A ONE DAY COURSE IN
HISTOPATHOLOGY OF THE BONE MARROW
will be held at
St Mary's Hospital Medical School
Wednesday 11th September 1996.

The Course is for Consultant Haematologists, Consultant Histopathologists and advanced trainees in Haematology and Histopathology.

40 places only, CME approved (7 CME credits)

The cost is £80 (light lunch included). Please can you inform us if you require vegetarian food when you register.

Those wishing to participate should apply in writing, enclosing a cheque PAYABLE TO IMPERIAL COLLEGE for the appropriate amount, to:

Dr BJ Bain
Department of Haematology
St Mary's Hospital Medical School
Norfolk Place, London W2 1PG

A TWO DAY COURSE IN
HAEMATOLOGY MORPHOLOGY
will be held at
St Mary's Hospital Medical School
on
Monday and Tuesday 9th and 10th September 1996.

This course, which includes both lectures and work at individual microscopes, is suitable for updating career grade post holders in haematology and is also valuable for trainees in haematology.

40 places only, CME approved (6 + 7 CME credits)

The cost is £120 including light lunches or £105 without lunches. Please can you inform us if you require vegetarian food when you register.

Those wishing to participate should apply in writing, enclosing a cheque PAYABLE TO IMPERIAL COLLEGE for the appropriate amount, to:

Dr BJ Bain
Department of Haematology
St Mary's Hospital Medical School
Norfolk Place, London W2 1PG
# What's On In The World

## Future Meetings

<table>
<thead>
<tr>
<th>Date</th>
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<th>Location</th>
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<tr>
<td>20-21 April</td>
<td>NSW Education Weekend</td>
<td>Pokolbin</td>
<td>Judith Salvo, Haematology Dept, Westmead Hospital, Westmead 2145</td>
<td>02 845 7073</td>
<td>02 689 2331</td>
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<tr>
<td>2-4 May</td>
<td>New Insights in Haematology</td>
<td>Venice, Italy</td>
<td>Congress Secretariat, c/o Interloquia s.n.c., S.Marco 4600, 30124 Venice, Italy</td>
<td>39 41 5209800</td>
<td>39 41 5234942</td>
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<tr>
<td>21-23 Aug</td>
<td>XIV International Fibrinogen Workshop</td>
<td>Canberra</td>
<td>CH Nair, Vascular &amp; Thrombosis Research Unit, Woden Valley Hospital, PO Box 11, Woden ACT 2606</td>
<td>06 244 2831</td>
<td>06 244 2832</td>
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<tr>
<td>12-16 Oct</td>
<td>34th HSA /ASBT ASM</td>
<td>Adelaide</td>
<td>Festival City Conventions, PO Box 986 Kent Town SA</td>
<td>08 363 1307</td>
<td>08 363 1604</td>
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<tr>
<td>17-20 Nov</td>
<td>13th Asian Pacific Cancer Conference</td>
<td>Penang Malaysia</td>
<td>Director, Highland Hospital Cancer Treatment Center, 1000 South Avenue, Box 71, Rochester, NY 14620</td>
<td>716 473 7172</td>
<td>716 461 2719</td>
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<tr>
<td>6-10 Dec</td>
<td>American Society of Haematology</td>
<td>Orlando Florida</td>
<td>ASH, 1200 19th Street Suite 300, Washington DC 20036-2412</td>
<td>202 857 1118</td>
<td>202 857 1164</td>
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<tr>
<td>1997</td>
<td>14th Meeting of ISH, European &amp; African Division</td>
<td>Stockholm</td>
<td>Prof G Gahrton, Huddinge Sjakhus, Karolinska Institute, Dept of Medicine, S-14186 Huddinge, Sweden Tel: 46 8 746 1000</td>
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<tr>
<td>1997</td>
<td>35th HSA /ASBT ASM</td>
<td>Auckland</td>
<td>Jan Tonkin, The Conference Company, PO Box 90-040, Auckland NZ Tel: 64 9 360 1240 Fax: 64 9 360 1242</td>
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<tr>
<td>1999</td>
<td>XXVII Congress of the International Society of Haematology</td>
<td>San Diego California</td>
<td>ASH, 1200 19th Street Suite 300, Washington DC 20036-2422 Tel: 202 857 1118 Fax: 202 857 1164</td>
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<tr>
<td>1999</td>
<td>XVIII International Congress of Genetics</td>
<td>New Orleans Louisiana</td>
<td>ASH, 1200 19th Street Suite 300, Washington DC 20036-2422 Tel: 202 857 1118 Fax: 202 857 1164</td>
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The HSA Secretariat, 145 Macquarie St, Sydney NSW 2000, is attended by Mrs Ruth Guss on Mondays, Wednesdays and Thursdays from 9 am to 3 pm. Tel: 02 256 5456 Fax: 02 252 3310