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Sarcoma UK

Sarcoma UK is published by Dragonflair Publishing, PO Box 5, Church Stretton, Shropshire SY6 6ZZ.

It is published free of charge to sarcoma patients, carers, friends and those who treat this group of rare cancers.

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THANK YOU

The response to the first edition of Sarcoma UK has been overwhelming. Thank you to everyone who has written to us. Sarcoma UK is now being incorporated as a company limited by guarantee with a view to applying for charity status. Please join the mailing list (form below) to be kept informed.



Roger Wilson

Second Edition
Summer/Autumn 2003

Email:
info@sarcoma-uk.org

New Drugs, Regulators and the Patients' Voice

New drugs hold the key to treating cancer effectively in the future. There have been some important developments, such as tamoxifen for breast cancer, which have helped patients in ways undreamed of ten years ago. Drug companies, however, are not allowed to market just what they want. The regulatory process is demanding and requires clinical trials to prove the benefits before new treatments can be openly marketed.

Drugs developed specifically to treat rare cancers are few and far between. With such drugs the approval process seems especially challenging when there is clinical benefit but not the quantity of statistical proof that regulators want to see.

The decision by the European Agency for the Evaluation of Medicinal Products (EMA) to ask Pharmamar for further trial proof of effectiveness of Yondelis® illustrates the issues.

The EMA process is rigorous, as you expect. It asks for evidence of manufacturing quality, effectiveness in treating the target disease, and

proofs of safety. Their job is to find the balance between benefits and risks for patients, taking evidence from clinical trials. The approval process draws a line between benefit and risk which the drug company, the clinicians who are running trials and the regulators all have to tread.

Even if a drug is showing clinical benefits the regulator can opt for a safety-first approach in the absence of the statistical proof it requires. In rare diseases reaching these standards can be difficult. Clinical trials start with small numbers of patients to find safe dosage (Phase I) and to look at effectiveness (Phase II). They are followed by larger scale Phase II and Phase III trials comparing standard and proposed new treatments.

With rare cancers like sarcoma large-scale clinical trials can take a long time to show statistically valid results because there are not many patients. If there is growing evidence that the new treatment is clinically effective, then running a trial just to produce regulatory evidence may well be unethical.

It looks as though Yondelis® is

(Continued on page 2)



Thank you

Thank you to everyone who wrote following our first edition earlier in the year. There have been some very moving stories, and some very funny ones. I have found it exciting to be in contact with so many other patients and hope that we can help do something about the loneliness so many of us feel.

Please keep writing.

In this edition we talk to the two doctors who lead sarcoma clinical trials work in the UK. I hope that in future editions we will be able to talk to others around the country and get a full picture of what the challenge of developing new treatments for sarcoma involves.

This edition also tells some more patient stories. Go to pages 6 and 7 and have a look at page 2 to find out what this lady in a funny hat has been doing.

Roger Wilson

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facing this problem. Pharmamar is appealing the EMEA decision but meanwhile the clinicians cannot add it to their armoury of treatments.

While Yondelis is just embarking on its regulatory journey, Glivec®, the revolutionary treatment for GIST developed by Novartis, is further along. Glivec® has EMEA marketing approval and is now awaiting technical approval from the UK's National Institute of Clinical Excellence. If this is gained it will become a standard NHS treatment for advanced GIST.

The NICE process is also rigorous and takes into account the cost of the drug. The Glivec® appraisal for GIST is underway at present and we must hope that the trials results from around the world, which demonstrate what a remarkable treatment it is, will gain NICE support.

There is, however, one distinct difference between the two regulators. EMEA does not seek any patient input while NICE gives patients a voice in its appraisals, and in the appraisal of Glivec® for GIST, it is being heard.

'TANKED UP IN SHROPSHIRE'

The photograph was taken on Sunday 25th May which was when I organised a sponsored walk to raise money for Professor Radford and his team at the Christie who do so much good work in researching sarcoma. Some money also went to The Shropshire and Mid Wales Hospice - where I have received physiotherapy since my first operation, on my leg



The 6 Pubs in 10 miles Walk through rural Shropshire proved to be a great success and besides having a huge amount of fun, great weather, and loads of support from many friends, we raised £2,500.

One of the highlights of the day was the fact that we were led by my partner Phil driving his pride and joy, the Chieftain Tank. Over £500 was raised in the collection buckets on the day. After all, who is going to argue with a tank! My thanks go to everyone who helped on the day.

To any one who is currently going through treatment for Sarcoma, and possibly not feeling too brilliant, I hope this will help by showing that there is light at the end of the tunnel, and you WILL feel better.

So, here's to The 6 Pubs in 10 Miles walk 2004!.

Penny Tong



GETTING INFORMATION

The first stop for information about your case and your treatment should be your doctors. The hospital specialist will have the latest information and will be able to tell you most about the treatment appropriate for you. Your GP will have some information but remember that on average a GP will only ever have one sarcoma patient in his whole career ! Sarcoma is that rare!

General sarcoma information is available from Cancer Bacup. They have good booklets, which have recently been revised, on Soft Tissue Sarcoma and on Understanding Primary Bone Cancer. Your hospital information centre may have them, if not call Cancer Bacup and they will post them to you straight away. Some hospitals also have local produced information in their information centre.

If you have access to the Internet there is a wide range of information available, from around the world. Even if you cannot access the Internet yourself there may be someone who can do it for you and print out the pages they find. It could be quite a lot of paper !

Information can help you decide what questions you want to ask. Doctors or nurses don't mind being asked, they would rather hear all the questions you want answered and they will spend time answering them. Their focus is on you, the patient.

If you have been diagnosed with advanced sarcoma, information is just as important as it is for the newly diagnosed, though it may be of a different kind. Treatments such as chemotherapy have a lot of mystique about them and raise fears of their own. Rather than listening to old stories about side effects it is much more valuable to hear about how treatments have changed and how modern medicine, and some complementary therapies too, can help you through a demanding treatment.

It is also important to understand that if you do not want to learn too much about your disease you don't have to. The choice is yours.

Contact telephone numbers and useful websites

CANCER HELP UK

Cancer Help is Cancer Research UK's patient website. Excellent sarcoma material including "Questions for Your Doctors". Click the link to 'Specific Cancers' and also use the search function as there are further references within the site.

<http://www.cancerhelp.org.uk/>

CANCERBACUP

Cancer Bacup's free helpline is staffed by experienced nurses who can answer many questions. The web link leads to booklets on sarcoma and other cancer related issues - all available free by phoning Bacup.

<http://www.cancerbacup.org.uk/catalogue/sarcomas.htm>

0808 800 1234 - freephone helpline

020 7696 9003 - publications

MACMILLAN

Macmillan works to improve the quality of life for people living with cancer. It offers information and practical advice over the phone and its web site is extensive. Macmillan also has information on local support groups.

<http://www.macmillan.org.uk/>

0808 808 2020 - freephone helpline

UK CHILDREN'S CANCER STUDY GROUP

The UKCCSG is a main contact point for professional treating children's cancer. Support materials and excellent downloadable newsletter are on the website.

<http://www.ukccsg.org/>

SARCOMA UK

We are a new publication and our web presence is being worked on. The main page offers links to a range of further Internet resources and you can download our first edition if you missed it.

<http://www.sarcoma-uk.org>

PHARMAMAR

Pharmamar is developing the specialist sarcoma drug Yondelis®.

<http://www.pharmamar.com>

NOVARTIS

Novartis manufactures Glivec®, the radical new treatment for GIST, an intestinal sarcoma.

<http://www.novartisoncology.com/home.jsp>

Be cautious when using the web to look for information on any cancer. Just because a website exists it does not mean that those responsible have any expertise. Information can only be general and can not be specific to a particular patient.

THE CHALLENGES OF RESEARCH



The National Cancer Research Institute is the partnership created in 2001 between the government, the NHS and the major cancer charities who fund research trials. One of its objectives is to double the number of patients entering clinical trials by 2004. The NCRI has 19 Clinical Studies Groups, responsible for designing and developing trials, and 34 local research networks responsible for implementing them.



Rob Grimer describes taking on the chairmanship of the NCRI Clinical Study Group for Sarcoma as "a very exciting challenge". Bone sarcomas have traditionally had quite a strong base of trials, particularly for children, but for adult soft tissue sarcoma there have been fewer trial

opportunities.

"My job as chair is to get a greater awareness of trials that are open, to increase the trials which are available and to clarify areas where we sarcoma clinicians feel there is uncertainty and a case for a trial. Overall the aim is to increase the quality of care for sarcoma patients."

There is a close relationship with EORTC (see opposite) and other research groups internationally. This is crucial if trials are to be completed quickly. A recent osteosarcoma trial took ten years to gather enough patients to allow the hoped for benefits to be proved, such is the rarity of sarcomas, and even now the final results of that trial are awaited.

"Effectively this means we have been using the same drugs we were ten years ago. So the more numbers we can get into trials with the same treatments in future, across the world, the quicker and the better we build the base for beneficial change."

The job of the Clinical Studies Group is to develop a portfolio of trials. This will mean looking around the

world for trials to adopt, starting UK based trials and finding others from Europe and elsewhere to collaborate.

"That's quite a challenge because there is excellent work around the world in treating sarcomas. For instance the Scandinavians and Italians have some exciting ongoing studies. I am looking for a portfolio which could mean that every patient with a sarcoma could potentially be entered into at least one study."

For adult soft tissue sarcoma it is likely that the major research questions will come from European collaboration. There are some significant issues about controlling local disease and using radiotherapy in reducing recurrences. There is also an important question about improving the quality of the follow-up experience, an anxiety issue for so many patients.

Could the three and six monthly hospital visits many of us face be replaced by a protocol delivered by a GP or local hospital?

"We just don't know," says Rob Grimer, "and that is a starting point for a randomised clinical trial".

Drug company sponsored trials to test new drugs lie outside the remit of the NCRI Clinical Studies Group even though the clinicians running them are also involved in the NCRI portfolio of trials.

"Our aim is better survival for all sarcoma patients," says Rob Grimer, "Virtually every study into any kind of cancer shows that people do better in trials. Just by getting more people into trials should help us improve outcomes for patients."

Rob Grimer became the UK's first consultant orthopaedic oncology surgeon in 1988 after qualifying in London and Birmingham. He heads the Royal Orthopaedic Hospital's cancer unit, based in Birmingham. He has three teenage children. For relaxation he likes to swim, play tennis, and is taking up golf ... "again". The family goes skiing every year and have a cottage in south Wales to escape the urban sprawl of the Midlands.

EORTC



The European Organisation for Research and Treatment of Cancer (EORTC) is responsible for collaborative clinical trials in many cancers. The Soft Tissue and Bone Sarcoma Group (STBSG) is recognised as a world leader in sarcoma research group. **Professor Ian Judson** of The Royal Marsden Hospital has been chair of the Group since October 2002. We asked him to reflect on the challenges facing sarcoma clinicians during the next three years.



"Probably the biggest challenge we face is going to be funding, both of research and treatment. Sarcomas are rare tumours and it is difficult to obtain the backing of the pharmaceutical industry for trials that will not result in a financial return in the market place."

Many licensed products are not approved for the treatment of sarcoma and doctors can find it difficult to get permission from Primary Care Trusts, who fund drugs for patients in their area, for the use of expensive drugs for which the justification may be only one small clinical study.

"On the positive side we are identifying new targets for treatment all the time and this is bound to have an impact on the way that we treat sarcomas over the next 10 years."

The main role of the STBSG is to design and manage large scale clinical trials. Sometimes these will be available in all member countries but sometimes only in a few. The new treatment for gastro-intestinal stromal tumour (GIST) had many of its early (Phase I and II) trials in the larger EORTC centres and the drug, imatinib (Glivec®), is now gaining regulatory approvals.

"I hope to see many more examples of clinical trials with agents that target specific molecular abnormalities in sarcoma, in a similar fashion to imatinib in GIST. This

is likely to require the involvement of the global sarcoma research fraternity and we will have to work hard to overcome the current regulatory problems associated with conducting clinical trials on both sides of the Atlantic".

Increasingly the main UK sarcoma treatment centres are members of the EORTC group and hence are able to participate. Even when they do not take part in it a European clinical trial can help UK clinicians improve sarcoma treatment because the results are available to everyone and can be used to benefit patients.

Treatment centres apply for membership of the EORTC Group. A site visit takes place to assess the number of patients they treat, whether they have a trial infrastructure (for example research nurses and data managers) and to evaluate the expertise of the medical team. Probationary membership is usually granted but full membership requires accrual into clinical trials of five patients a year for 2 years. This is not a trivial hurdle and some centres find it difficult to meet this target

Randomised clinical trials (Phase III) which compare new treatments with standard treatments sometimes meet patient resistance. Professor Judson has been managing trials since the 1980s and has a simple answer for any objections.

"You have to explain that if we knew the answer as to which treatment was superior then we wouldn't be doing the trial. With new drugs there is less of a problem because standard treatment has failed and patients are more willing to try anything"

Professor Ian Judson was appointed consultant medical oncologist in 1989 and has been involved in care of sarcoma patients since the early 1990's. He qualified as a doctor in London. He is now Professor of Clinical Pharmacology at the Royal Marsden Hospital. Married with 3 teenage children, he enjoys choral singing, playing the piano, and cooking. He used to like travel but says he now does too much of it!

GUESS THE WEIGHT OF THE LEG



Cuth with his daughter Evelyn

It was a sunny Friday afternoon in late August 2001 and Ward 19 was unusually quiet. Nearly all the patients due for discharge that weekend had departed; teas had been served, and cleared away, so Elvis and Victoria (names that have been changed to protect the innocent) had time to pop into the cubicle Caroline and I were sharing, for a chat. I know it's unusual for husband and wife to be occupying the same room in hospital, but when it had become clear that the liposarcoma in my leg could not be treated further surgically, and that a modified hind-quarter amputation would be necessary, we had arranged for our four year old daughter, Evelyn, to spend a fortnight in Ibiza with family, and for Caroline to join me in the Freeman Hospital, Newcastle, for our Summer Vacation.

I am convinced that this arrangement played a vital part in my speedy recovery, for with her constant attention and encouragement, I was quickly on my foot and hopping up and down the corridor like a demented, albeit deformed, frog. In addition, her presence freed nursing staff for other duties, as Caroline performed many of the non-medical, time consuming tasks for which I required assistance.

Perhaps because we had been to the limb centre that morning for a preliminary visit the conversation got round to discussing the weight of artificial legs. We had been informed that my prosthesis would weigh round about 6 kilos.

"I wonder how that compares with the one you lost", mused Elvis.

And at about that point in the proceedings, someone proposed the "Guess the Weight of the Leg" sweepstake. I can't now recall who it was but it was obviously a person with a macabre sense of humour. At any rate each of us stumped up the £1 entry fee and wrote down our estimate.

Elvis was despatched to fetch the scales, upon which I had been weighed the day prior to my operation, and the book in which my weight had been recorded. Victoria undertook to ensure a proper scrutiny of the scales to ensure that the "official weigh in" was carried out accurately; after all there was almost a fiver at stake!

For those of you who are a little concerned about how we were to obtain the amputated limb, fear not; its weight was to be calculated using the equation:

Cuth's Weight Prior to Operation minus Cuth's Weight Post Operation = Weight of Leg

Using this formula, it took no time at all to discover that my weight loss was almost exactly 11 kilograms and that this was the weight of the missing limb.

Elvis, who was easily the most accurate in his guess, having scooped the cash, felt a bit guilty about it all and so bought lottery tickets with the proceeds. He promised to share any winnings with the rest of us. It would be very nice to be writing this piece whilst sitting on our luxury yacht in some sun-kissed Caribbean haven, rather than sitting at my computer in rain-lashed Sunderland; but if that were to have been the case, I would almost certainly not have been corresponding with you, and would not have been able share one of the lighter moments of my time in hospital.

Cuth Earl

SARCOMA & SURVIVE

Sarcoma is rare and extremely aggressive. I guess most of you reading this will be aware of that now. But I've been asked to tell just a part of my story, about my experience with this disease.

I know that what I can tell you are, that Sarcoma, whether it be Fibrosarcoma or Leiomyosarcoma or whatever kind of Sarcoma – is survivable.

My own story started back in Glasgow, where I live, in the spring of 1994. I was 29 years old. My Sarcoma was a rare form of Spindle Cell Sarcoma, which affected my left leg. When I went to the Western Infirmary in Glasgow I met the Professor of Oncology who explained what was wrong with me and the treatment that I was about to undergo, which included surgery but before that, chemotherapy.

Nothing that anyone tells you can prepare you for the effect, mentally and physically, that chemotherapy has on you. I appreciate that chemo can take many different forms and affects people differently but when an aggressive cancer is being treated it usually calls for aggressive treatment. My experience with chemo was hellish. I find it difficult, even now to comprehend all that I went through. I felt that no one could help me and that I was on my own. I felt isolated. My family and friends couldn't help. The only person that could help me was me!

The doctors and nurses support you, they give you

drugs that help fight the cancer but the only person that can really make the difference is YOU. It's your body; it's your mind.

I was told I had 2 options: Give in and my survival would be measured in months or Fight it and the medics would do all they could, BUT I had to decide.

The Orthopaedic Professor who was looking after me once told me that the human mind could overcome huge obstacles against all the odds. But that YOU have to drive it. You have to tell yourself, every minute of every day that you are going to fight, that you are going to win. It sounds daft and quite melodramatic but that's how you win, that's how you defeat it.

In your darkest days fight harder; tell yourself that you're winning, don't give in!

The minute that you give in to it, it's won.

John Martin

John recently had his prosthetic knee overhauled. His determination shows through in one of his latest messages: "I guess all the parts in my leg have lasted well. They say 10 years is the point at which they start wearing out. I've been fairly active over the last 10 years, with 3 kids I had to be, so I've had my money's worth. Still in plaster now and I guess I will be for the next 2-3 months but I'm not complaining."

Booklist

Patients often seek something to read which might help with healing or with achieving an inner peace. The following books have been chosen for us by Jacqui Abbott, who has been a great support to me on my cancer journey (Editor). The books are a mixed bunch with spiritual and personal development covered as well as healing. I hope that they prove supportive. I would suggest that if a particular book title resonates with the reader, then that book may be helpful to them.

Title/Author/ISBN#

You Can Heal Your Life Louise L Hay	1870845013
Love Medicine and Miracles Bernie Siegel MD	0099632705
The Journey Brandon Bays	0722538391
A Little Light on the Spiritual Laws Diana Cooper	0340768630
The Road Less Travelled M Scott Peck	0099727404
Conversations with God (Book 1) Neale Donald Walsch	0340693258