



Registered Charity Number 1149692

BRINGING WM PATIENTS AND DOCTORS CLOSER

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4th International WM Forum



Over 150 attended the successful 4th International Forum at the Royal College of GPs in March. Apart from formal and 'Ask the Doctor' sessions on Sunday, there was a doctor/patient networking dinner on Saturday together with a meeting of associates of the European WM network. The Forum was financially supported by the EWMn, IWMF and the Binding Site. The DVD set is still available at £15.00, by donation on our website.

In **2014** the 8th International Workshop on Waldenström's (IWWM8) moves to London on August 13-17th at the Park Plaza Westminster Hotel. This doctor event is organised by Dana Farber (i.e Drs. Steve Treon and Chris Patterson) with UK doctors Shirley D'Sa, Chara Kyriacou and Roger Owen as chair organisers. Details will be published at www.wmworkshop.org. On the last day there is usually a patient/doctor event, and WMUK is working with IWMF to support the programme. We are of course encouraging all UK doctors and scientists with a Waldenström interest to attend the medical event.

Roger Tudor's run for Waldenström's

Roger raised over £1250+ Gift Aid for WMUK in support for his 59-year-old father Andrew at the Great Manchester Run in May. Andrew has been on 'watch and wait' for two years with an IgM of 15 at the Countess of Chester Hospital and is 'immensely proud' of his son's activity. Roger and WMUK worked together to establish a BT MyDonate page (www.btplc.com/mydonate) which is available to anyone else considering sponsored events for WM.



Regional Meetings Planned

In line with its aim of bringing doctors and patients closer, WMUK is considering developing a programme of low key, regional meetings where doctors, carers, patients and nurses can interact, develop local contacts and discuss one or two topics of interest. We would like some feedback from you: should they be evenings or weekends? Do you have suggestions for locations and content? Whilst WMUK would undertake the main organisation, it would also be good if we could have more 'helpful patients' volunteering who could help with hosting. Get in touch with Roger now if you want to help.

Welcome to the second WMUK newsletter. It's a work in progress, and we need your help developing regular features to make it lively, informative and understandable. We'd welcome short contributions, news and links to websites.

Rory Morrison

Those of you at the 2012 Forum can- Association in not fail to remember the succinct his memory. links delivered in that familiar BBC WMUK has accent by Rory when he acted as been consider-M/C. He memorably described WM ing what might as an "orphan disease where they be fitting as a can't even find the orphanage". Short- tribute to Rory ly after the Forum, Rory relapsed (who was on our Advisory Board). from his auto transplant, and was (who was on our Advisory Board). scheduled for an allo transplant from Co-incidentally we had been investi- his brother. Before this could happen, gating the possibility of setting up a his WM transformed into a Diffuse **UK patient medical registry**. This would be a secure, professional da- Large B-cell Lymphoma, which was tabase of medical outcomes of treat- successfully treated and Rory eventu- ment past and current. This would ally underwent his transplant at the encourage research and help find end of this April, but sadly succumbed candidates for drug trials. To create to complications on 11th June at the this would not be a simple or cheap tragically early age of 48. He leaves matter, but it would be a great step his wife Nikki and two children. forward, and a fitting tribute to Rory. WMUK Trustee Phil Manning, visited Dr Helen McCarthy at Bournemouth Rory in hospital and represented us Hospital is currently looking at the at a moving funeral near St Albans. costings and practicality of such a Nikki had generously encouraged do- scheme and further details will be nations to WMUK and the Lymphoma announced when available.



Patient Survey Moves Forward

A volunteer committee under Trustee Phil Manning with Jackie James and David Kerrigan has been drafting our patient questionnaire. The aim is to build up a joined-up picture of your treatment patterns throughout the UK and of the complications of the disease, including side effects of treatment and impact on families. In turn, with your help, we should then be able to identify most UK treatment centres and doctors, allowing us to provide support for both non-specialist doctors and newly diagnosed patients. There are a number of challenges, including keeping it short and access for those not online, but we should see the result this autumn. This is not the same as the professional medical registry which is under consideration (see above), but a good response to the patient questionnaire will help progress on the Registry, particularly by locating treatment centres.

Chemo Corner- Novel Treatments *First UK Ibrutinib Trialist!*

There were a number of questions at the Forum about Ibrutinib, which has been on trial in the USA. One of our supporters joined the USA trial. Here is a report of his experience to date.

"I was diagnosed in 2009 with IgM paraprotein of 56.2g/litre, Beta 2 microglobulin 2.8, haemoglobin 10.3g/dl. Shortly after, I started chemo - CHOP X3, CVP X1, R-CVP X4 and then 8 cycles of Rituximab. This went on into 2010 when I was put down for an Autograft and started on R-ESHAP for about 3 months, then HD chemo up to transplant in December 2010. I was told that everything went well, but two-monthly blood tests showed my paraprotein level (about 2.5 after the transplant) rising by about 0.5 every two months.

During this time I read quite a bit about Dr Steven Treon's findings identifying a gene mutation in the vast majority of Waldenström's patients. In February 2012 I made an appointment to visit Dr Treon at The Dana - Farber/Harvard Cancer Center in Boston. At the time my health was pretty good and I would not have been considered for a trial unless it was a lot worse, but over the next year I slowly deteriorated and in March 2013 my IgM was back to where it was when I was first diagnosed.

I was then invited to Boston to see if I now met the criteria to be accepted for the clinical trial. I did, and started on an Investigational trial of Ibrutinib. This entails taking a few tablets every day, with no real side effects, quite different to the days, weeks and months of chemo, associated nausea, aches, pains and generally feeling pretty rotten. Ibrutinib is a newly discovered drug that is being developed as an anti-cancer agent. Ibrutinib is a Bruton's tyrosine kinase (Btk) inhibitor drug which interrupts B cell receptor (BCR) signaling in Lymphomas by selectively and irreversibly binding to the Btk protein, which then results in malignant cell death.

The incredible news is within as little as 2 months my IgM is down by over 80%. There is no guarantee that this specific drug will work as well as chemotherapy for Waldenström's but if the results to date are anything to go by the next few months will be very exciting.

This is just the beginning but hopefully the future for the majority of sufferers with Waldenström's and other forms of cancer will be a lot more bearable with this type of drug treatment."

Ed comment: *Ibrutinib is being trialed in several B-cell malignancies in 39 trials in the USA and for CLL in the UK and Europe, and showing very promising results. In the USA it has been fast tracked for approval by the FDA and may be authorised by the year end. Key questions, assuming it is eventually approved in the EU and UK are cost - wild estimates have been put forward, but £60k+ a year has been rumoured, and the fact that it is a maintenance medication, not a cure; so you would be on it for life.*

New Trial should produce more accurate IgM Results

News from Dr Shirley D'Sa at UCLH

A recurring difficulty faced by doctors is the inaccuracy of the IgM level that is obtained by current methods. When the IgM amount is greater than a certain level (which is around as low as 5-10 g/l), the result obtained by conventional methods can become progressively inaccurate as the IgM level rises. Given the prominence placed on repeated measurements of IgM levels in the follow up of WM patients, this is an impediment to progress.

The Binding Site, a UK-based company based in Birmingham has developed a novel method for measuring heavy chains (IgM) and light chains (kappa and lambda) in a test called the **Hevylite test**. This uses antibodies against specific proteins on the surface of the heavy and light chains to detect them directly. This provides a true reading of the actual amount of IgM. Dr Dale Powner of The Binding Site has been working closely with the Dr Anne Dawnay, Consultant Biochemist at UCLH to use the Hevylite test in-house in patients seen in the WM Clinic, so as to validate this method. Patients are being invited to participate in the study, which simply requires one extra tube of blood (taken through the same needle as the routine bloods) at each visit. The results of the study will be analysed later in the year and an update will be provided.

Current tests for the identification of M proteins rely on lengthy and subjective lab tests called serum protein electrophoresis. A serum sample is placed in a gel across which an electric current is applied. This current causes the various serum proteins to move along the gel and become separated according to their relative electrical charges. The quantities of these separated proteins are estimated by a lab technician. Experts interpreting such gels do so with a high degree of accuracy, but a simple numerical result would allow for easier interpretation of the level of the M protein. This is what the Hevylite test may provide. However, it needs to be validated.

The results of this work will provide information to help us to follow patients more accurately. It does not affect the patient's management in any way- it is simply an observational study carried out on blood tests. It is also being trialed at other specialist clinics.

Ed's Note: The Binding Site has been a major sponsor of all three WMUK Fora.



WMUK website update

Due to the generous people who have made donations or rounded up their Forum registration we are improving the website. Work is underway to recode the existing site to make it more flexible and easy to edit, and which will allow us to display more Patient Tales. Please have another look at the WMUK site and

get back to us with what you would like us to add to improve its usefulness. We have a dummy's guide to blood tests in preparation, and want to encourage more doctor-authored content, but I'm sure there are other things that you would value, particularly for newly diagnosed patients.

Do we have your correct email ? If you have changed or acquired a new email, please let us know.

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