



**WMUK**  
UK Point of Contact for  
**Waldenström's  
Macroglobulinaemia**

## November 2016 e-newsletter

**Virgin Money London Marathon places,  
new BTK Clinical Trial, Stem Cell  
Transplant update, Ibrutinib, a WM App,  
Bake4Rory and Cartwheel Database!**

### **London Virgin Money Marathon - CAN YOU RUN FOR TEAM WMUK RORY?**

Because we now have Virgin Money Giving available to us (please try it if you want to make a donation), we have been offered the chance of winning a 4 person VIP team place at the Virgin Money London Marathon 23rd April next year in a Run4Rory Team, this will be led by Sue, Rory Morrison's sister-in-law. This is very competitive as places are very scarce so we are looking for the following potential team members as soon as possible (as in yesterday!) who have some or all of these characteristics:

- 1) **Will run for WMUK** and achieve a substantial sponsorship target for our Rory Registry.
- 2) **Have an interesting back story** - for instance be a patient, carer or doctor, have a relative or interesting celebrity/media connection.
- 3) **Can use social media** (Facebook/Twitter etc) if possible to spread the word, and help us to set up an amazing WMUK Virgin Money Giving page.
- 4) **Has a reasonable chance of finishing** - but speed is not the object!

We have to fill in an application form by the 18th which will give the 'flavour' of the team which will be entered into a competition, and we will know if we have won a team slot by about the 10th December. If our team gains a place we can use the Virgin Lounge in London Haymarket for a fundraising event for free, and any ideas here would be welcome (**from anyone**), as this forms part of the competition entry.

If you or anyone you know are interested in being put on the potential team list, just get back to me at [info@wmuk.org.uk](mailto:info@wmuk.org.uk) ASAP with a brief outline on how you would contribute to the team as above. We will then select a team and a reserve to be led by Sue.

By the way, whilst we talk of fund raising runs, thank you to all of those who helped sponsor **Maddie Hall** in her recent **Great South Run** - her team raised 238% of their target! - £1190 for WMUK: details at <https://www.justgiving.com/fundraising/maddiehallx> You will also find on our JustGiving page some ideas from others who have helped us. Small as we are, we always try to help and support any event you want to undertake. Never underestimate your potential target!

### **New BTK Trial: BGB-3111**

Beigene's BGB-3111 second generation BTK inhibitor is to be trialled for the first time in the UK in WM early in the New Year at several UK centres. BGB-3111 Details are at <http://www.beigene.com/pipeline/clinical-candidates/bgb-3111/> Final details of the trial are awaited, which I'll distribute as soon as available. Places are limited, so if you feel it might be of benefit, ask your doctor now to consider you - for instance, if you have run out of conventional treatment options - but be aware there are always entry conditions. It's taken orally, and earlier trials showed it's well tolerated. Early phase 1 dosing trials were mainly in Australia, and Dr Con Tam presented some encouraging initial data at IWWW9 in Amsterdam.

### **Stem Cell Transplant Saga unresolved**

The saga of transplants in WM rolls on as the NHS attempts to save money. Firstly a huge thanks again for the amazing response supporting Sandra and Harriet whose transplants were held up at UCLH due to the funding row, when some 18 treatments were held back in August whilst NHS Specialised Commissioning challenged the court ruling that PrEP, the AIDS prevention drug, could be NHS funded. This was resolved last week when the high court confirmed it did have the power to fund it. In the meantime Sandra's and Harriet's campaign ranged across the media and the petition in Parliament now stands at 12,300 signatures (please sign it if you haven't yet and get others to do so!- it's at <https://petition.parliament.uk/petitions/167441> along with the NHS response). As a result of the media pressure, NHS Specialised Commissioning in turn pressurised UCLH to fund their transplants, although the

billing is not finally settled, but Harriet is about to leave hospital as I write and Sandra is about to start hers. We wish them both well. They are very brave people to stand up with their families and be counted.

However the basic problem is not solved, and NHS is **still** stalling by 're-evaluating' the cost effectiveness of all the treatments held up (all the others are new, only WM is a current treatment). The evaluation process is totally obscure. I was invited to give evidence at the All Party Parliamentary Group on Transplantation at Westminster and MPs were not impressed with what NHS SC are doing, it seems mainly to involve writing to hospitals asking for up to 20% discount on transplants. As the cost is mainly highly specialist nursing care, this seems unwise. The total projected cost of commissioning transplants properly was an extra £60,000 a year in England - a drop in the ocean, but vital for those with few other options. The government response to the petition was so feeble (basically saying 'all is well') that the Westminster Petitions Committee unusually asked for a better response, but NHS SC still claim it was never commissioned and is still available through Individual Funding requests (IFRs). These take doctors three hours to fill in which are all turned down. Meanwhile patients are being put on a slowly growing queue, or doctors have just given up on the process for now and telling patients it's not available. We have submitted a detailed FOI request to NHS Specialist Commissioning to try and dig into how treatments are prioritised, particularly how these life saving transplants ended up as low priority and why the IFR process is not working. IFRs are at least being looked at and WMUK is going to a consultation meeting at the end of November. The basic reason of course is that high rejection rates save money.

## **Ibrutinib Funding**

The second meeting with NICE took place on 15<sup>th</sup> November. The result of the first one was a provisional no, as you previously heard, and we were not impressed with the organisation (or the result). They accepted the benefit of the drug, but refused to stomp up the money. Drs Roger Owen & Shirley D'Sa as well as Mark Barnett and I have responded in detail and will attend, as have Janssen, who have made offers to try and make this available through the Cancer Drugs Fund. Fingers crossed.

I have to say, that at a recent Blood Cancers Coalition meeting, of which we are members, the mood was very pessimistic about the NHS funding situation at the moment and it may get worse. Rarer diseases and blood cancers have had a particularly poor deal recently. In my humble opinion the concept of clinical need is being rapidly lost in the panic to avoid NHS overspend this year.

## **A dedicated WM app?**

We are looking at what a dedicated WM app for smartphones, desktops and tablets might be able to do for us. The Registry system already has the ability to interact by providing a way of regularly entering patient generated data to link with that of your treatment. It will be particularly valuable for clinicians to have these patient reported outcomes (PROs), which are increasingly being demanded by regulators when getting medicines approval and licencing, and especially so when patients are on trials. This bit is relatively simple (but has a cost of course!). However, can you think of what else an app could do to strengthen the WM community? Perhaps you develop apps? For instance it could be part of an online chat forum to replace the Yahoo group- (as Yahoo is going out of fashion rapidly) but what else could it do? I'm sure some of you have experience of apps - or perhaps your children have - so it would be good to have some thought and feedback on this. One thought might be to have a database of common WM questions. The Kings Fund, the health think tank has done a bit of work on this and if you are interested you might look at

[https://www.kingsfund.org.uk/publications/articles/eight-technologies-will-change-health-and-care?qclid=Cj0KEQjw4\\_DABRC1tuPspqXjxZwBEiQAhMIp6\\_CBu-fWZwW9hK-nDFY2Rt7aBX\\_KN0z29DrjvrB5fVAaAhzl8P8HAQ](https://www.kingsfund.org.uk/publications/articles/eight-technologies-will-change-health-and-care?qclid=Cj0KEQjw4_DABRC1tuPspqXjxZwBEiQAhMIp6_CBu-fWZwW9hK-nDFY2Rt7aBX_KN0z29DrjvrB5fVAaAhzl8P8HAQ)

## Bake4Rory rises yet again.

Cakes flew out of the door at the BBC last week as the team of Rory's R4 colleagues and supporters raised a record £4,000, roughly half the annual maintenance cost of the Rory Morrison Clinical Registry in their annual Bake4Rory event - and there were no reported soggy bottoms or double entendres. Thanks to you who added to their total online. We will be putting Phil Manning's photos up on the website but here is one his tweets of Kathy Clugston (L) and Susan Rae with some of the bakes which attracted magnificent donations.



## The Cartwheel database

We have been asked by the Australian WMOzzies and the Whimsical Study team in New South Wales led by Dr Judith Trotman (who came to IWWM9 Amsterdam) to encourage as many UK patients as we can to join the database. This is not a registry, but something like an international version of our recent treatment survey. Here is a link to the study page. <http://www.wmozzies.com.au/index.php/about-us/our-history/australian-research-projects/> this will provide a resource for researchers using your anonymised data. Once you are signed up there is a fair bit of your data to enter, but you can stage it over a period of time, update your data and view it later. Something for those long winter evenings!

## Spring Meeting 2017

We are still looking at Oxford venues and dates for a spring Doctor/Patient meeting. We will let you know before Christmas as soon we have tied this down, but if you have any Oxford venues you can suggest which can take up to 100, let us know.

Finally, a big thank you to those who now make their regular donation by **standing order** - this is of immense importance so we can have the comfort of a steady income as we develop new services. If you want to do this, just let us know and I'll send you our bank details. Virgin Money Giving also allows us to accept monthly payments, and they charge nothing for doing this or reclaiming Gift Aid, unlike some other giving platforms. By the way, our main bank, Lloyds, charges us nothing for cheques and standing orders, and our local branch in N Chingford is being kept open. Much relief!

All the very best,

A handwritten signature in black ink that reads 'Roger Brown'. The signature is written in a cursive style and is underlined with a single horizontal stroke.

Roger Brown

Chair WMUK